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Filed Pursuant to Rule 424(6)(4)
Registration No. 333-230822

3,571,428 Shares

Axcella Health Inc.

Common Stock

This is our initial public offering. We are selling 3,571,428 shares of common stock. The initial public offering price is $20.00 per share.

Prior to this offering, there has been no public market for our common stock. Our common stock has been approved for listing on The Nasdaq Global Market under the symbol "AXLA."

Investing in our common stock involves risks. See "Risk Factors" beginning on page 12 of this prospectus.

We are an "emerging growth company" as defined under U.S. federal securities laws and will be subject to reduced public company reporting requirements. See "Prospectus Summary—Implications of being an emerging growth company."

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities, or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial Public Offering Price	$20.00	$71,428,560.00
Underwriting Discounts and Commissions [(1)]	$1.40	$4,999,999.20
Proceeds, before expenses, to us	$18.60	$66,428,560.80

(1) See "Underwriting" beginning on page 217 of this prospectus for additional information regarding total underwriter compensation.

We have granted the underwriters an option for a period of up to 30 days to purchase up to 535,714 additional shares of our common stock.

Certain of our existing stockholders, including entities affiliated with certain of our directors, have indicated an interest in purchasing an aggregate of approximately $39.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any or all of these stockholders, or any or all of these stockholders may determine to purchase more, fewer or no shares in this offering. The underwriters will receive the same underwriting discount on any shares purchased by these stockholders as they will on any other shares sold to the public in this offering.

Delivery of the shares of common stock is expected to be made on or about May 13, 2019.

Joint Book-Running Managers

GOLDMAN SACHS & CO. LLC **J.P. MORGAN** **SVB LEERINK**

Prospectus dated May 8, 2019

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We and the underwriters have not authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses we have prepared. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may provide you. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of the common stock.

Until June 2, 2019 (25 days after the date of this prospectus), all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

For investors outside the United States: Neither we nor the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside the United States.

PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus. Before investing in our common stock, you should carefully read this entire prospectus, including our consolidated financial statements and the related notes included elsewhere in this prospectus. As used in this prospectus, unless the context otherwise requires, references to the "company," "we," "us" and "our" refer to Axcella Health Inc. together with its consolidated subsidiaries. In this prospectus, we use the following defined terms:

"AXA Candidate" to refer to one of our investigational product candidates.

"*AXA Development Platform" to refer to our proprietary human-focused development platform.*

"Clinical Trial" to refer to a human clinical study of a drug product candidate subject to the requirements for an effective Investigational New Drug application, or an IND.

"dose" to refer to the exposure amount of an AXA Candidate in Clinical Trials and Non-IND, IRB-Approved Clinical Studies.

"non-drug" to refer to a non-therapeutic use of an AXA Candidate. Such use may be as a food product or dietary supplement.

"Non-IND, IRB-Approved Clinical Study" to refer to Institutional Review Board-approved, or IRB-Approved, clinical studies conducted in humans with our AXA Candidates under U.S. Food and Drug Administration, or the FDA, regulations and guidance supporting research with food outside of an IND (prior to any decision to develop an AXA Candidate as a drug product candidate under an IND or a non-drug product candidate). In these food studies, based on our understanding of FDA regulations and guidance, we evaluate in humans, including individuals with disease, an AXA Candidate for safety, tolerability and effects on the normal structures and functions of the body. These studies are not designed or intended to evaluate an AXA Candidate's ability to diagnose, cure, mitigate, treat or prevent a disease as these would be evaluated in Clinical Trials if we decide to develop an AXA Candidate as a drug or therapeutic.

Overview

We are a biotechnology company pioneering the research and development of novel multifactorial interventions to support health and address dysregulated metabolism across a broad spectrum of consumers and patients who have limited options. Our AXA Candidates are generated from our proprietary, human-focused AXA Development Platform and harness the power of endogenous metabolic modulators, or EMMs, a broad family of molecules that fundamentally impact and regulate human metabolism.

Using our AXA Development Platform, we have rapidly generated a pipeline of AXA Candidates that are novel compositions of EMMs engineered in distinct ratios and designed to target and maximize the fundamental role that EMMs play in regulating multiple metabolic functions. Our AXA Candidates are administered orally and anchored by amino acids, which have a history of safe use as food. As such, we expect our AXA Candidates may also be combinable with other modalities. We believe our current dataset supports the potential of our AXA Candidates to safely modulate the metabolic pathways they target. These features may make them an attractive development opportunity with significant commercial potential.

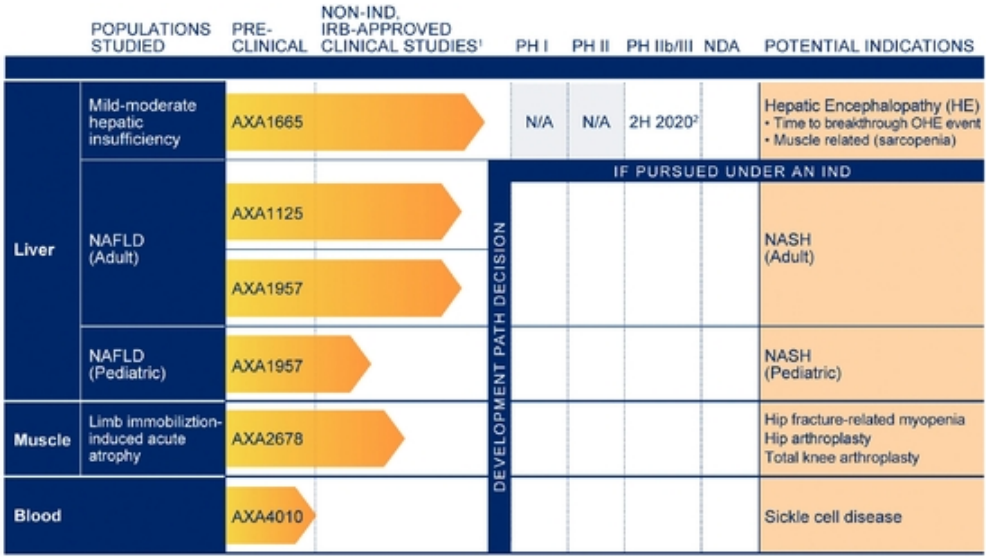
In 2018, we completed three Non-IND, IRB-Approved Clinical Studies. In all three studies, our AXA Candidates were generally found to be well tolerated, and we generated positive normal structure and function data indicating potential metabolic modulation. We believe generating this rich human dataset at this early stage of development (i) eliminates the translational uncertainty associated with transitioning from animal studies to human studies, (ii) enables us to make

high-insight, capital-efficient development decisions and (iii) potentially increases the probability of success for these AXA Candidates. For AXA Candidates we decide to develop under an IND, we will discuss with the FDA our ability to use data generated from our Non-IND, IRB-Approved Clinical Studies to begin Phase II or potential registrational Clinical Trials.

On March 6, 2019, we had a face-to-face pre-IND meeting with the FDA for AXA1665, our lead AXA Candidate, during which we discussed clinical endpoints, assessment tools and other matters relating to a potential IND-opening Clinical Trial for AXA1665 in hepatic encephalopathy, or HE. After meeting with the FDA, we made a decision to pursue a drug development path for AXA1665. Under the planned IND, the initial indication would be for the treatment of HE, with time to breakthrough episode of overt HE event as the primary endpoint and key secondary muscle-related endpoints. We anticipate interacting with the FDA again prior to a formal IND submission for AXA1665. We anticipate initiating a Clinical Trial in the second half of 2020 for AXA1665 that we believe could potentially serve as a registrational (pivotal) trial to support the submission of a New Drug Application, or NDA.

Our Pipeline

Our wholly-owned pipeline currently comprises five programs focused on the metabolic functions of the liver, muscle and blood. An overview of our AXA Candidates and their status is illustrated below.



(1) In the above pipeline chart, "Development Path Decision" reflects the point in a program at which we decide whether to develop an AXA Candidate as a drug product candidate under an IND, develop it as a non-drug product candidate or terminate development. We have made a decision to develop AXA1665 as a drug product candidate and anticipate interacting with the FDA again prior to submitting an IND. We have not made a development path decision for any of our other AXA Candidates.

(2) We believe that this Clinical Trial has the potential to serve as a registrational (pivotal) Clinical Trial, subject to continuing IND discussions and allowance by the FDA.

Definitions: NAFLD = non-alcoholic fatty liver disease; NASH = nonalcoholic steatohepatitis; OHE = overt hepatic encephalopathy.

AXA1665 in Hepatic Encephalopathy

In study AXA1665-001, subjects with mild and moderate hepatic insufficiency (Child-Pugh Class A and B) were divided into two groups: 14.7g/day and 44.1g/day of AXA1665, administered in three divided doses. In subjects receiving the higher dose of AXA1665, we observed a cumulative increase of 40% in the Fischer's ratio, the molar ratio of branched-chain amino acids to aromatic amino acids. These subjects also demonstrated a lower plasma ammonia area under the curve when sampled over five hours (AUC_{0-5h}), and tended to maintain a leaner phenotype (i.e., increase in dry lean mass and decrease in fat mass as assessed by bioimpedance measurements) and muscle function (as assessed using a liver frailty index score) compared to those on a control regimen. Each of these markers are believed to have prognostic significance in assessing liver health, including cirrhosis.

In 2019, prior to making a drug development path decision for AXA1665, we initiated an additional 12-week (with a four-week follow-up), randomized, placebo-controlled, Non-IND, IRB-Approved Clinical Study in approximately 60 mild and moderate hepatic insufficiency (Child-Pugh Class A and B) subjects. We anticipate the data readout from this study in the first half of 2020 and plan to include these data in the IND submission for AXA1665. Under the planned IND, the initial indication would be for the treatment of HE, with time to breakthrough episode of overt HE event as the primary endpoint and key secondary muscle-related endpoints. HE has multiple drivers, namely amino acid imbalance, dysregulated ammonia handling and sarcopenia.

AXA1125 and AXA1957 in Subjects with Non-alcoholic Fatty Liver Disease, or NAFLD

In study AXA1125-002, administration of AXA1125 24g three times a day was associated with a trend of decreased liver fat, improved insulin sensitivity (i.e., lower Homeostatic Model Assessment of Insulin Resistance) and induction of fat oxidation (i.e., increased beta-hydroxybutyrate). In subjects who received AXA1125, mean blood levels of key fibroinflammatory biomarkers tended to decrease compared to the mean pre-administration baseline levels. We believe these data suggest AXA1125 has the potential to impact three critical drivers associated with NAFLD—metabolism, inflammation and fibrosis. Leveraging these findings, we rapidly designed AXA1957 to potentially address additional populations within the liver health and disease spectrum, including pediatrics.

In January 2019, we initiated study AXA1125-003, a 16-week, randomized, single-blind, placebo-controlled, Non-IND, IRB-Approved Clinical Study to assess safety, tolerability and physiological impact on the normal structures and functions of AXA1125 and AXA1957 in approximately 105 adult subjects with NAFLD. We currently anticipate the data readout from this study in the second half of 2020. In the second half of 2019, we also plan to initiate AXA1957-002, a placebo-controlled, single-blind, randomized (2:1), controlled Non-IND, IRB-Approved Clinical Study to assess safety, tolerability and physiological impact on the normal structures and functions of AXA1957 in approximately 30 adolescent subjects with NAFLD.

AXA2678 in Healthy Subjects with Immobilization-Induced Acute Muscle Atrophy

In study AXA2678-001, in subjects receiving AXA2678 24g three times a day, we observed attenuated muscle atrophy during the one-week immobilization period compared to placebo, reflected by a 76% relative difference in average muscle cross sectional area assessed by magnetic resonance imaging. Subjects receiving AXA2678 tended to have muscle fiber architecture preserved during immobilization and also tended to recover muscle strength to pre-immobilization baseline levels as compared to placebo within a short two-week recovery period. We are continuing to evaluate next steps for this program.

We believe that the consistency and nature of the data from our three completed Non-IND, IRB-Approved Clinical Studies indicate that this approach has significant promise. We are focused on actively expanding our pipeline and currently reviewing additional development opportunities in hematology, central nervous system, kidney and pulmonary function. Many diseases are driven by multifactorial dysregulated systemic metabolism, and we have already characterized over 50 diseases for prioritization.

We have attracted a world-class leadership team that has significant experience in the successful development and commercialization of collectively more than 50 drugs across numerous therapeutic areas. We are supported by an industry-leading board of directors and scientific advisory board. Since inception, we have raised approximately $200 million in capital from leading life sciences investors, including our founder, Flagship Pioneering, Fidelity Management & Research Company, Nestlé Health Science US Holdings and Gurnet Point Capital Limited.

Our Strengths

We believe we are well-positioned to execute on our corporate strategy based on the following competitive strengths:

- **Deep understanding of metabolic dysregulation and the modulatory power of EMMs.** Applying our expertise in EMMs and metabolism, we aim to harness the potential of EMMs to directly and simultaneously support and modulate multiple metabolic pathways implicated both in complex diseases and overall health.
- **Predictive, rapid and scalable human-focused AXA Development Platform.** We have built a first-of-its-kind integrated knowledge platform that has proven capable of rapidly designing our novel and proprietary AXA Candidates. To date, we have progressed three programs from biological hypothesis to readout of initial Non-IND, IRB-Approved Clinical Study data in less than 18 months.
- **Informed and capital-efficient AXA Candidate early-stage development model.** In our Non-IND, IRB-Approved Clinical Studies, we have rapidly generated a rich dataset in healthy subjects and subjects with certain disease conditions. We believe that generating human data at this early stage of development enables us to make high-insight, capital-efficient development decisions and potentially increases the probability of success for these candidates.
- **Pipeline with significant potential across a spectrum of disease and health.** We currently have five AXA Candidate programs across multiple target areas, including in liver and muscle, and have characterized over 50 additional diseases for prioritization.
- **Broad intellectual property portfolio with worldwide rights to our AXA Candidates.** As a first mover in our approach to EMMs, we are building a broad intellectual property portfolio to protect our AXA Candidates and AXA Development Platform.
- **Experienced leadership team and board of directors.** Members of our management team and board of directors have extensive experience in product development and commercialization.

Our Strategy

Our goal is to become the preeminent biotechnology company reprogramming metabolism to address a diverse set of complex diseases and support health. To achieve our goal, we are executing a strategy that includes the following core elements:

- Accelerate paths to market for our lead programs.

- Further enhance and leverage our AXA Development Platform to advance multiple new programs per year in attractive markets.
- Expand patient and consumer access to AXA Candidates, if approved, and opportunistically leverage strategic partnerships.
- Establish ourselves as a global, fully-integrated biotechnology company.

Risks associated with our business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk Factors" section of this prospectus immediately following the prospectus summary. These risks include the following:

- For AXA Candidates we decide to develop under an IND, such as AXA1665, the FDA will require substantial additional preclinical and/or clinical development in target diseased populations before we can seek and receive regulatory approval for and launch these products commercially. For any AXA Candidate under an IND, the FDA may reject data from previously completed Non-IND, IRB-Approved Clinical Studies or may require new, previously untested data points to be studied; the resulting Clinical Trial results may not support or be consistent with our prior data, and analysis or expectations for such AXA Candidate or the AXA Candidate may not meet therapeutic efficacy endpoints in Clinical Trials.
- Regulatory requirements for development of our AXA Candidates as non-drugs or drugs are uncertain and evolving. Changes in these laws, including our ability to conduct Non-IND, IRB-Approved Clinical Studies, or the current interpretation or application of these laws could have a significant adverse impact on our ability to research, develop and commercialize our products. Some of these risks include that the FDA may require that we conduct additional preclinical studies before proceeding to Clinical Trials, determine that our AXA Candidates cannot be marketed as a non-drug product, disagree with our determination that our AXA Candidates may be studied in humans without an effective IND, or subject us to litigation or enforcement actions.
- We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.
- Even if we consummate this offering, we will require additional capital to fund our operations and if we fail to obtain necessary financing, we will not be able to complete research or development and commercialization of our AXA Candidates.
- Any negative safety and efficacy data regarding or negative perceptions of EMMs, our novel approach to EMMs or our AXA Candidates could adversely affect our ability to successfully recruit for and complete our human studies. This could adversely affect our ability to conduct our business, obtain regulatory approvals, if required, identify alternate regulatory pathways to market for any AXA Candidates or enter into strategic partnerships, and materially and negatively impact our business and operations.
- Clinical development is a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development of, obtain positive data for or commercialize any AXA Candidates, which could result in an impaired ability or inability to finance or fund the Company in the future.
- There can be no assurance that we will be able to develop in-house sales, distribution and other capabilities needed to commercialize drug or non-drug products or establish or

maintain relationships with third-party collaborators to successfully commercialize any AXA Candidates that receive regulatory approval in the United States or overseas.

- If we are unable to obtain or protect intellectual property rights related to our technology and current or future AXA Candidates and AXA Development Platform, or if our intellectual property rights are inadequate, we may not be able to compete effectively.
- We may not be able to establish a commercial manufacturing source and secure supply chain capacity sufficient to provide AXA Candidates and materials for preclinical studies, Non-IND, IRB-Approved Clinical Studies, Clinical Trials, if we decide to develop any AXA Candidate as a drug product candidate, and commercial quantities of any AXA Candidates.
- We may be unable to attract and retain key scientific or management personnel to successfully execute our business plans.

For additional information about the risks we face, please see the section of this prospectus captioned "Risk Factors."

Recent Developments

As of March 31, 2019, our cash and cash equivalents were $66.7 million.

The information above is based on preliminary unaudited information and management estimates for the three months ended March 31, 2019, is not a comprehensive statement of our financial results, and is subject to completion of our financial closing procedures. Our independent registered public accounting firm has not conducted an audit or review of, and does not express an opinion or any other form of assurance with respect to, this preliminary estimate.

Axcella Health Inc. and Flagship Pioneering

We were founded in 2008 by Flagship Labs, the institutional innovation foundry of Flagship Pioneering. A team comprising innovators and entrepreneurs Dr. David Berry, Dr. Geoffrey von Maltzahn and Dr. Noubar Afeyan started exploring the potential medical applications of diverse, orally-consumed proteins. The work of this team led to the understanding that specific amino acid combinations at different ratios have the potential to drive profound biological effects in both health and disease by impacting dysregulated metabolic pathways and multi-compartmental biology. With this transformational new approach, we are harnessing the potential for orally-administered EMM compositions to drive cellular biology in a specific and reproducible manner with the goal of delivering safe interventions for patients or consumers.

Corporate history

We were incorporated in August 2008 under the laws of the state of Delaware under the name Newco LS16, Inc. Our name was changed to Axcella Health Inc. in June 2016. Our principal executive offices are located at 840 Memorial Drive, Cambridge, MA 02139, and our phone number is (857) 320-2200. Our website address is https://www.axcellahealth.com. The information contained in or accessible from our website is not incorporated into this prospectus, and you should not consider it part of this prospectus.

We own various U.S. federal trademark applications and unregistered trademarks, including our company name and our logo. All other trademarks or trade names referred to in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the symbols ® and ™, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

Implications of being an emerging growth company

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. For so long as we remain an emerging growth company, we are permitted and intend to rely on certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments not previously approved. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

We will remain an emerging growth company until the earlier to occur of (1) December 31, 2024, (2) the last day of the fiscal year in which we have total annual gross revenues of at least $1.07 billion, (3) the last day of the fiscal year in which we are deemed to be a "large accelerated filer," under the rules of the U.S. Securities and Exchange Commission, or SEC, which means the market value of our equity securities that is held by non-affiliates exceeds $700 million as of the prior June 30th and (4) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This provision allows an emerging growth company to delay the adoption of some accounting standards until those standards would otherwise apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company.

The Offering

Common stock offered by us	3,571,428 shares
Common stock to be outstanding immediately after this offering	22,988,359 shares (or 23,524,073 shares if the underwriters exercise their option to purchase additional shares in full)
Option to purchase additional shares offered by us	535,714 shares
Use of proceeds	We estimate that we will receive net proceeds from the sale of shares of our common stock in this offering of approximately $63.3 million, or $73.3 million if the underwriters exercise their option to purchase additional shares in full, based on the initial public offering price of $20.00 per share, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering, together with our existing cash and cash equivalents: (i) to advance our current liver and other programs through additional Non-IND, IRB-Approved Clinical Studies, our planned IND filing for AXA1665 and other potential IND filing(s) and the ensuing Clinical Trials; (ii) to advance our AXA Development Platform and discovery efforts; (iii) to support organizational growth; and (iv) for working capital and other general corporate purposes. For a more complete description of our intended use of the proceeds from this offering, see "Use of Proceeds."
Risk factors	You should carefully read the "Risk Factors" section of this prospectus for a discussion of factors that you should consider before deciding to invest in our common stock.
Nasdaq Global Market symbol	"AXLA"

Certain of our existing stockholders, including entities affiliated with certain of our directors, have indicated an interest in purchasing an aggregate of approximately $39.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any or all of these stockholders, or any or all of these stockholders may determine to purchase more, fewer or no shares in this offering. The underwriters will receive the same underwriting discount on any shares purchased by these stockholders as they will on any other shares sold to the public in this offering.

The number of shares of our common stock to be outstanding after this offering is based on 4,774,934 shares of our common stock outstanding as of December 31, 2018, and gives effect to the conversion of all outstanding shares of our preferred stock into an aggregate of 14,641,997 shares of our common stock upon the completion of this offering, and excludes:

- 61,235 shares of common stock issuable upon the exercise of warrants to purchase shares of Series A preferred stock that will become warrants to purchase common stock outstanding as of December 31, 2018, with a weighted-average exercise price of $3.59 per share;

- 4,039,464 shares of common stock issuable upon the exercise of stock options outstanding as of December 31, 2018 under our 2010 Stock Incentive Plan, or our 2010 Plan, with a weighted-average exercise price of $5.67 per share;
- 41,230 shares of common stock reserved for future issuance as of December 31, 2018 under our 2010 Plan, which ceased to be available for issuance at the time that our 2019 Stock Option and Incentive Plan, or our 2019 Plan, became effective;
- 905,000 shares of our common stock that became available for future issuance under our 2019 Plan upon the effectiveness of the registration statement of which this prospectus forms a part; and
- 237,181 shares of our common stock that became available for future issuance under our 2019 Employee Stock Purchase Plan, or our 2019 ESPP, upon the effectiveness of the registration statement of which this prospectus forms a part.

Unless otherwise indicated, all information in this prospectus reflects or assumes the following:

- the filing of our restated certificate of incorporation and the effectiveness of our amended and restated bylaws in connection with the completion of this offering;
- the conversion of all outstanding shares of preferred stock into an aggregate of 14,641,997 shares of common stock upon the completion of this offering;
- the outstanding warrants to purchase our Series A preferred stock becoming warrants to purchase an aggregate of 61,235 shares of our common stock upon the closing of this offering;
- the payment of a $1.2 million success fee that is payable to Solar Capital Ltd. upon completion of this offering;
- no exercise of outstanding options after April 20, 2019;
- a one-for-1.842 reverse split of our common stock effected on April 29, 2019; and
- no exercise by the underwriters of their option to purchase up to 535,714 additional shares of common stock in this offering.

Summary Consolidated Financial Data

You should read the following summary consolidated financial data together with our consolidated financial statements and the related notes appearing at the end of this prospectus and the "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of this prospectus. We have derived the consolidated statements of operations data for the years ended December 31, 2017 and 2018 from our audited consolidated financial statements appearing at the end of this prospectus. Our historical results are not necessarily indicative of the results that may be expected in the future.

	December 31,	
	2017	2018
	(in thousands, except share and per share data)	
Consolidated Statements of Operations Data:		
Operating expenses:		
Research and development	$ 22,916	$ 25,486
General and administrative	6,005	8,410
Total operating expenses	28,921	33,896
Loss from operations	(28,921)	(33,896)
Other income (expense):		
Change in fair value of preferred stock warrant liability	81	(14)
Interest income (expense), net	(2,100)	(2,159)
Total other income (expense), net	(2,019)	(2,173)
Net loss	$ (30,940)	$ (36,069)
Net loss per share, basic and diluted [(1)]	$ (7.37)	$ (7.97)
Weighted average common shares outstanding, basic and diluted [(1)]	4,211,918	4,546,373
Pro forma net loss per share, basic and diluted (unaudited) [(1)]		$ (2.19)
Pro forma weighted average common shares outstanding, basic and diluted (unaudited) [(1)]		16,528,448

(1) See Note 11 to our consolidated financial statements appearing at the end of this prospectus for details on the calculation of basic and diluted net loss per share and the calculation of basic and diluted pro forma net loss per share.

	As of December 31, 2018		
	Actual	Pro Forma [1]	Pro Forma As Adjusted [2]
	(in thousands)		
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 79,466	$ 79,466	$ 142,785
Working capital [3]	73,390	73,390	136,709
Total assets	81,844	81,844	145,163
Long term debt, net of discount	24,521	24,521	24,521
Preferred stock warrant liability	425	—	—
Other liabilities [4]	1,898	1,898	698
Redeemable convertible preferred stock	197,842	—	—
Total stockholders' equity (deficit)	(149,753)	48,514	113,033

(1) The pro forma consolidated balance sheet data gives effect to (i) the conversion of all outstanding shares of our preferred stock into an aggregate of 14,641,997 shares of common stock upon the closing of this offering and (ii) all outstanding warrants to purchase shares of Series A preferred stock becoming warrants to purchase shares of common stock upon the closing of this offering.

(2) The pro forma as adjusted balance sheet data give further effect to our issuance and sale of shares of our common stock in this offering at the initial public offering price of $20.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

(3) We define working capital as current assets less current liabilities.

(4) Includes a $1.2 million success fee related to our loan and security agreement with Solar Capital Ltd., which is payable upon the consummation of this offering.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this prospectus, including our consolidated financial statements and the related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations," before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline and you may lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.

Risks related to our business, technology and industry

We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.

We are a biotechnology company with a limited operating history. Investment in product development in the healthcare industry, including of biotechnology products, is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and/or become commercially viable. Our AXA Candidates are currently being studied in Non-IND, IRB-Approved Clinical Studies as food products. We have no products approved for commercial sale, have not generated any revenue from product sales to date and continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception in 2008. For the years ended December 31, 2017 and 2018, we reported net losses of $30.9 million and $36.1 million, respectively. As of December 31, 2018, we had an accumulated deficit of $157.0 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and if we choose to develop our AXA Candidates under an Investigational New Drug application, or IND, seek regulatory approvals for, our AXA Candidates. We anticipate that our expenses will increase substantially if, and as, we:

- conduct preclinical studies and additional Non-IND, IRB-Approved Clinical Studies and, for any AXA Candidate we elect to develop as a drug product candidate, initiate Clinical Trials to develop an AXA Candidate under an IND or its equivalent in non-U.S. jurisdictions;
- further develop our proprietary human-focused product development platform, our AXA Development Platform;
- continue to discover and develop our current AXA Candidates as well as additional AXA Candidates;
- maintain, expand and protect our intellectual property portfolio;
- hire or contract additional clinical, scientific, manufacturing and commercial personnel to support our product research, development and commercialization efforts;
- continue to develop, scale and validate a manufacturing process and specifications for our AXA Candidates;
- continue to establish in-house manufacturing capabilities for our research and product development efforts;

- establish a commercial manufacturing source and secure supply chain capacity sufficient to provide preclinical study material, Non-IND, IRB-Approved Clinical Study material, Clinical Trial material for any AXA Candidate we elect to develop as a drug product candidate under an IND, and commercial quantities of any AXA Candidates that we may commercialize as a drug or non-drug product candidate, following receipt of any necessary approvals or authorizations;

- acquire or in-license other candidates and technologies;

- seek various non-drug product marketing pathways and, if applicable, drug regulatory authorizations;

- establish a sales, marketing and distribution infrastructure to commercialize any AXA Candidates for which we may obtain regulatory approval or identify an alternate regulatory pathway to market; and

- add operational, compliance, financial and management information systems and personnel to support our transition to a public company.

To become and remain profitable, we or any potential future collaborator must develop and eventually commercialize products with significant market potential at an adequate profit margin after cost of goods sold and other expenses. This will require us to be successful in a range of challenging activities, including: completing preclinical studies, Non-IND, IRB-Approved Clinical Studies and Clinical Trials for any AXA Candidate we elect to develop as a drug product candidate under an IND; obtaining marketing approval or identifying alternate regulatory pathways for AXA Candidates; manufacturing, marketing and selling products for which we may obtain marketing approval; or successfully identifying alternate regulatory pathways and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenue that is significant enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company, which could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

Even if we succeed in commercializing one or more of our AXA Candidates, we will continue to incur substantial research and development and other expenditures to develop and market additional AXA Candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business, which may be significant. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

Even if we consummate this offering, we will require additional capital to fund our operations and if we fail to obtain necessary financing, we will not be able to complete development and commercialization of our AXA Candidates.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts for our current and future programs: to conduct further research and development, preclinical studies, Non-IND, IRB-Approved Clinical Studies and Clinical Trials for any AXA Candidate we elect to develop as a drug product candidate under an IND, as we plan to do for our AXA1665 product candidate; to validate the manufacturing process and specifications for our AXA Candidates; to seek regulatory approvals for or identify alternate regulatory pathways to market for our AXA Candidates; and to launch and commercialize any

products for which we receive regulatory approval or identify an alternate regulatory pathway to market, including potentially building our own commercial organization. As of December 31, 2018, we had $79.5 million of cash and cash equivalents on hand. Based on our current operating plan, we believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses, capital expenditure requirements and debt service obligations through the second quarter of 2021. However, our future capital requirements and the period for which our existing resources will support our operations may vary significantly from our expectations, and we will in any event require additional capital in order to complete clinical development of any of our current AXA Candidates. Our monthly spending levels will vary based on new and ongoing development and corporate activities. Because the length of time and activities associated with development of our AXA Candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- our decisions regarding the development path under which we will develop our AXA Candidates (i.e., either continuing to develop our AXA Candidates as non-drug products, or initiating development as drug product candidates under an IND);
- the initiation, progress, timing, costs and results of preclinical studies, Non-IND, IRB-Approved Clinical Studies and, depending on our development path decision, Clinical Trials for our AXA Candidates and any need to conduct additional such studies as may be required by a regulator, including additional studies that may be required by a regulator in order to allow the initiation of Clinical Trials under an IND or the non-U.S. equivalent for any of our AXA Candidates;
- the clinical development plans we establish for these AXA Candidates;
- further development of our AXA Development Platform and supporting infrastructure;
- the number and characteristics of AXA Candidates that we develop or may in-license;
- the terms of any collaboration agreements we may choose to initiate or conclude;
- the outcome, timing and cost of meeting regulatory requirements established by the U.S. Food and Drug Administration, or the FDA, any other regulatory authorities in the United States, and, when applicable, comparable foreign regulatory authorities, such as the European Medicines Agency, or the EMA;
- the effect of changes in regulations or policy relating to the development and commercialization of our AXA Candidates by the FDA, any other regulatory authorities in the United States and, when applicable, other comparable foreign regulatory authorities such as the EMA;
- the costs of establishing, maintaining and overseeing a quality system compliant with current Good Manufacturing Practices, or cGMPs, and a supply chain for the development and manufacture of our AXA Candidates;
- the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us, our AXA Candidates or our AXA Development Platform;
- the effect of competing technological and market developments;
- the cost and timing of establishing, expanding and scaling manufacturing capabilities; and

- the cost of establishing sales, marketing and distribution capabilities for any AXA Candidates for which we may receive regulatory approval or identify alternate regulatory pathways in regions where we choose to commercialize our products on our own.

We do not have any committed external source of funds or other support for our development efforts and we cannot be certain that additional funding will be available on acceptable terms, or at all. Until we can generate sufficient product or royalty revenue to finance our cash requirements, which we may never do, we expect to finance our future cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing or distribution arrangements. If we raise additional funds through public or private equity offerings, the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. Further, to the extent that we raise additional capital through the sale of common stock or securities convertible into or exchangeable for common stock, your ownership interest will be diluted. If we raise additional capital through debt financing, we would be subject to fixed payment obligations and may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, declaring dividends or acquiring or licensing intellectual property rights. If we raise additional capital through marketing and distribution arrangements or other collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish certain valuable rights to our AXA Candidates, technologies, future revenue streams or research programs or grant licenses on terms that may not be favorable to us. We also could be required to seek collaborators for one or more of our current or future AXA Candidates at an earlier stage than otherwise would be desirable or relinquish our rights to AXA Candidates or technologies that we otherwise would seek to develop or commercialize ourselves. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our products or AXA Candidates or one or more of our other research and development initiatives. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline, causing you to lose all or part of your investment.

We have a limited operating history, which may make it difficult to evaluate our technology and product development capabilities and predict our future performance.

We are early in our development efforts and we have not initiated Clinical Trials for any of our AXA Candidates to allow for development of such candidates as drug product candidates. With the exception of AXA1665 for which we plan to file an IND, we are currently developing our AXA Candidates as non-drug products under food regulations, although the ultimate pathway under which we will develop our other AXA Candidates is subject to change depending on a number of factors. We were formed in 2008, have no products approved for commercial sale as drugs or marketed via other regulatory pathways (e.g., non-drug products) and have not generated any revenue from product sales. Our ability to generate product revenue or profits, which we do not expect will occur for many years, if ever, will depend on the successful development and eventual commercialization of our AXA Candidates, which may never occur.

Our limited operating history may make it difficult to evaluate our technology and industry and predict our future performance. Specifically, to date we have conducted Non-IND, IRB-Approved Clinical Studies for our AXA Candidates to evaluate safety and tolerability only in healthy subjects or subjects with certain disease conditions. If we decide to develop an AXA Candidate under an IND with patient populations reflecting the desired indication for such AXA Candidate, the physiological structure and function data we observed in our Non-IND, IRB-Approved Clinical Studies for such

AXA Candidate may not be replicated in or consistent with results from Clinical Trials and such AXA Candidate may not meet therapeutic efficacy endpoints in Clinical Trials.

Our short history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early-stage companies in evolving fields. If we do not address these risks successfully, our business will suffer. Similarly, we expect that our financial condition, expenditures and operating results will fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. As a result, our stockholders should not rely upon the results of any quarterly or annual period as an indicator of future operating performance.

In addition, as an early-stage company, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown circumstances, which may be significant. As we advance our AXA Candidates, we will need to transition from a company with a research focus to a company capable of supporting clinical development and, if successful, commercial activities. We may not be successful in such transitions.

Any use of our AXA Candidates to support and maintain homeostasis, which helps support normal structures and functions of the body, or to modulate dysregulated metabolism is a novel approach and negative perception of any AXA Candidates that we develop could adversely affect our ability to conduct our business, obtain regulatory approvals or identify alternate regulatory pathways, to the extent required by applicable laws, to market such AXA Candidates.

Using endogenous metabolic modulators, or EMMs, in the compositions, ratios and formulations we use in our AXA Candidates and the potential drug and non-drug applications of our AXA Candidates represents a novel approach. Our AXA Candidates in general may not be successfully developed or commercialized or gain the acceptance of the public or the medical community. For any AXA Candidate that we choose to develop as a drug product candidate, our success will depend upon physicians who specialize in the treatment of diseases targeted by our AXA Candidates, prescribing potential treatments that involve the use of our AXA Candidates in lieu of, or in addition to, existing treatments with which they are more familiar and for which greater clinical data may be available. For any AXA Candidate that we choose to develop as a non-drug product candidate, our success will depend on finding partners in a non-drug market who can help successfully commercialize AXA Candidates. In addition, our success will also depend on consumer acceptance and adoption of our products that we, or a future partner, commercialize. Adverse events in Non-IND, IRB-Approved Clinical Studies and Clinical Trials of our AXA Candidates or in studies or Clinical Trials of others developing similar products and the resulting publicity, as well as any other adverse events in the field of EMMs and metabolic pathways, could result in a decrease in demand for any product that we may develop. In addition, responses by the U.S. federal, state or foreign governments to negative public perception or ethical concerns may result in new legislation or regulations that could limit our ability to develop or commercialize any AXA Candidates, obtain or maintain regulatory approval, if applicable, identify alternate regulatory pathways to market or otherwise achieve profitability. More restrictive statutory regimes, government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects, and may delay or impair the development and commercialization of our AXA Candidates or demand for any products we may commercialize.

All of our AXA Candidates for which we make a drug development path decision, including any targeting the metabolic pathways of the liver and muscle that if dysregulated could result in loss of health or disease, will require significant additional preclinical and clinical development before we can seek regulatory approval for and launch a drug product commercially.

For any AXA Candidate that we choose to develop as a drug product candidate, such as our AXA1665 drug product candidate for which we plan to submit an IND, our business and future success will depend on our ability to obtain regulatory approval of and then successfully launch and commercialize such AXA Candidate as a drug targeting a disease involving dysregulated metabolism, such as cirrhosis, non-alcoholic steatohepatitis or muscle atrophy. Any future Clinical Trials of AXA1665 and any other AXA Candidates that we decide to develop as drug product candidates may experience preliminary complications in trial execution, such as complexities surrounding regulatory clearance to initiate Clinical Trials, the need for additional preclinical data to support authorization to proceed with Clinical Trials, modifications in trial design and trial protocols, bioanalytical assay method development, dose level and regimen selection, patient recruitment and enrollment, quality and supply of clinical doses or safety issues. Additionally, even if additional preclinical studies, Non-IND, IRB-Approved Clinical Studies or Clinical Trials are successfully executed, there is no guarantee that the results produced by such additional studies will confirm or be consistent with the results obtained in our Non-IND, IRB-Approved Clinical Studies to date for our AXA Candidates.

Any AXA Candidates that we decide to develop as drug product candidates will require significant additional development, including preclinical and clinical development, regulatory review or approval in the jurisdictions that we target for commercialization, identification of appropriate non-drug regulatory pathways to commercialization, substantial investment, access to sufficient validated and cGMP-compliant commercial manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. In addition, if we choose to develop AXA1665, our lead candidate, as a drug product candidate and AXA1665 encounters safety, efficacy, supply or manufacturing problems, developmental delays, regulatory or commercialization issues or other problems, our development plans, including for our other AXA Candidates, and business would be significantly harmed.

The successful development of our AXA Candidates is highly uncertain.

Successful development of our AXA Candidates is highly uncertain and is dependent on numerous factors, many of which are beyond our control. AXA Candidates that appear promising in the early phases of development may fail to reach the market for several reasons, including:

- results from preclinical or Non-IND, IRB-Approved Clinical Studies may demonstrate that our AXA Candidates are not safe, not tolerable or have unanticipated impacts on the normal structure and function of the body and could result in data showing one or more AXA Candidates to have harmful or problematic side effects or toxicities;
- Clinical Trial results may show any AXA Candidate we decide to develop as a drug product candidate to be less effective than expected (e.g., a Clinical Trial could fail to meet its primary endpoint(s)) or to have unacceptable side effects or toxicities;
- failure to execute the Non-IND, IRB-Approved Clinical Studies or Clinical Trials caused by slow enrollment in Non-IND, IRB-Approved Clinical Studies or Clinical Trials, as applicable, study subjects dropping out of Clinical Trials or volunteers or study subjects dropping out of Non-IND, IRB-Approved Clinical Studies, length of time to achieve clinical endpoints, additional time requirements for data analysis, inability to validate the manufacturing process or to achieve cGMP compliance for our AXA Candidates;

- failure to receive the necessary regulatory approvals or authorizations, where applicable, or a delay in receiving such approvals or authorizations for, including, but not limited to, a new drug application, or NDA, or delays in NDA preparation, the need to submit a new dietary ingredient, or NDI, notification or other filings with the FDA, discussions with the FDA and other regulatory authorities in jurisdictions we target or pursue, responding to an FDA request or other regulatory authority for additional preclinical or clinical data or unexpected safety or manufacturing issues;

- manufacturing costs, formulation issues, manufacturing deficiencies or other factors that make our AXA Candidates uneconomical;

- proprietary rights of others and their competing products and technologies that may prevent our product candidates from being commercialized; and

- the length of time necessary to complete Clinical Trials and, for those AXA Candidates that we may decide to develop as drug product candidates, to submit an application for marketing approval of a drug product candidate, where applicable, for a final decision by a regulatory authority may be difficult to predict for any such AXA Candidates, in large part because of their limited regulatory history.

Even if we are successful in obtaining market approval for those AXA Candidates that we decide to develop as drug product candidates, commercial success of any approved drugs will also depend in large part on marketing acceptance, the availability of insurance coverage and adequate reimbursement from third-party payors, including government payors, such as the Medicare and Medicaid programs, and managed care organizations, which may be affected by existing and future healthcare reform measures designed to reduce the cost of healthcare. Third-party payors could require us to conduct additional studies, including post-marketing studies related to the cost-effectiveness of a product, to qualify for reimbursement, which could be costly and divert our resources. If government and other healthcare payors were not to provide adequate insurance coverage and reimbursement levels for one or any of our drug products once approved, market acceptance and commercial success would be reduced.

In addition, if any AXA Candidate we decide to develop as a drug product candidate is approved for marketing, we will be subject to significant regulatory obligations regarding the submission of safety and other post-marketing information and reports and registration. If approved, our drug products may be subject to restrictions on our products' labels and other conditions of regulatory approval that may limit our ability to market our products for therapeutic indications. We will also need to comply (and ensure that our third-party contractors comply) with cGMPs and Good Clinical Practice, or GCP, as we (and our third-party contractors) will be required to comply with cGMPs for products used in Clinical Trials and for any Clinical Trials that we conduct post-approval with cGMPs for either drug or non-drug candidates. In addition, we will need to comply with full GCPs in development efforts for any therapeutic indications we develop for approval and for any additional therapeutic indications we develop after approval of our first drug candidate. In addition, there is always the risk that we or a regulatory authority might identify previously unknown problems with a drug product post-approval or with non-drug products after commercialization, such as adverse events of unanticipated severity or frequency. Compliance with regulatory requirements to address such issues could be costly and any failure to comply or other issues with our drug products post-approval or non-drug products post-commercialization could have a material adverse effect on our business, financial condition and results of operations.

Clinical development is a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of any AXA Candidates, which could impair our ability to fund our operations or obtain financing on acceptable terms, or at all.

To obtain the requisite regulatory approvals to commercialize any AXA Candidate that we decide to develop as a drug product candidate, we must demonstrate through extensive preclinical studies and Clinical Trials that our AXA Candidates are safe and effective in humans for their intended use. Non-IND, IRB-Approved Clinical Studies to commercialize non-drug products also require a significant financial investment to generate data that supports the claims we would make for such products and the safety of the product. Clinical testing is expensive, difficult to design and implement and can take many years to complete, and its outcome is inherently uncertain. We may be unable to establish, where applicable, endpoints, dose levels and regimens or bioanalytical assay methods that applicable regulatory authorities would consider clinically meaningful, and a Clinical Trial can fail at any stage of testing. Additionally, our Non-IND, IRB-Approved Clinical Studies or other studies may not result in data that supports intended claims for non-drug products. The outcome of preclinical studies, Non-IND, IRB-Approved Clinical Studies and early Clinical Trials may not be predictive of the success of later preclinical studies, Non-IND, IRB-Approved Clinical Studies and Clinical Trials, and interim results of these studies or trials do not necessarily predict final results. Differences in trial design between early-stage Clinical Trials and later-stage Clinical Trials make it difficult to extrapolate the results of earlier Clinical Trials to later Clinical Trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and Clinical Trials have nonetheless failed to obtain marketing approval of their product candidates, or have data that supports desirable marketing claims even where marketing approval is not required.

Successful completion of Clinical Trials is a prerequisite to submitting an NDA to the FDA, or its equivalent in other foreign regulatory authorities such as a marketing authorization application to the EMA, for each product candidate for therapeutic indications and, consequently, the ultimate approval and commercial marketing of any product candidates for therapeutic indications. We do not know whether we will be able to initiate or complete Clinical Trials for AXA Candidates we decide to develop as drug product candidates on schedule, if at all.

We may experience delays in completing our preclinical studies and initiating or completing Non-IND, IRB-Approved Clinical Studies and, for those AXA Candidates that we decide to develop as drug product candidates, Clinical Trials. We also may experience numerous unforeseen events during, or as a result of, any future Non-IND, IRB-Approved Clinical Studies or Clinical Trials that we may conduct that could delay or prevent our ability to receive marketing approval or commercialize our AXA Candidates, including:

- we may be unable to generate sufficient preclinical, toxicology or other *in vivo* or *in vitro* data to support the initiation of Clinical Trials for therapeutic indications or the marketing of our products as non-drug products;
- the FDA may not allow us to use data from any Non-IND, IRB-Approved Clinical Studies to support an IND for AXA1665 or any other AXA Candidate we decide to develop as a drug product candidate instead of a non-drug product candidate;
- the FDA or other regulatory authorities may disagree with the design, implementation or results of our Non-IND, IRB-Approved Clinical Studies or Clinical Trials, or require us to submit additional data such as long-term toxicology studies or impose other requirements before permitting us to initiate or complete a Clinical Trial. For example, the FDA could require that we stop a Non-IND, IRB-Approved Clinical Study for an AXA Candidate and

continue such study only under an IND, and we may not be able to obtain such an IND, if at all, without additional study or we may be subject to an enforcement action for conducting a Non-IND, IRB-Approved Clinical Study not under an IND;

- regulators, IRBs or ethics committees may not authorize us or our investigators to commence or conduct a Non-IND, IRB-Approved Clinical Study or Clinical Trial at a prospective study or trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective study or trial sites and prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and study or trial sites;
- Non-IND, IRB-Approved Clinical Studies or Clinical Trials of any AXA Candidates may fail to show safety or effectiveness, or produce negative or inconclusive results and we may decide or be required to conduct additional preclinical studies, Non-IND, IRB-Approved Clinical Studies, Clinical Trials or any other studies, or we may decide to abandon product development programs;
- the number of subjects or patients required for Non-IND, IRB-Approved Clinical Studies or Clinical Trials of any AXA Candidates may be larger than we anticipate, enrollment in these clinical studies or trials may be slower than we anticipate or participants may drop out of these clinical studies or trials or fail to return for required follow-up post study or trial completion at a higher rate than we anticipate;
- we may need to add new or additional Non-IND, IRB-Approved Clinical Study or Clinical Trial sites;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the Non-IND, IRB-Approved Clinical Study or Clinical Trial protocol or drop out of the study or trial, which may require that we add new clinical study or trial sites or investigators;
- the cost of preclinical studies, Non-IND, IRB-Approved Clinical Studies, Clinical Trials or any other studies of any AXA Candidates may be more than we anticipate or more than our available financial resources;
- the supply or quality of our AXA Candidates or other materials necessary to conduct Non-IND, IRB-Approved Clinical Studies and Clinical Trials may be insufficient or inadequate and may not achieve compliance with applicable cGMPs;
- our AXA Candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, IRBs or ethics committees to suspend or terminate Non-IND, IRB-Approved Clinical Studies and Clinical Trials, or reports may arise from preclinical or clinical testing of our AXA Candidates that raise safety or other concerns about one or more of our AXA Candidates; and
- preclinical studies, Non-IND, IRB-Approved Clinical Studies or Clinical Trials of our AXA Candidates may produce negative or inconclusive results, which may result in our deciding, or being required, to conduct additional clinical studies or trials or abandon product development programs.

We could also encounter delays if a preclinical study, Non-IND, IRB-Approved Clinical Study or Clinical Trial is suspended or terminated for any reason. A suspension or termination may be imposed due to a number of factors, including failure to conduct the Non-IND, IRB-Approved Clinical Study or Clinical Trial in accordance with regulatory requirements or our clinical protocols,

inspection of the clinical trial operations or trial site by the FDA, other regulatory authorities or IRB resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product or treatment, failure to establish or achieve clinically meaningful endpoints, changes in governmental regulations or administrative actions or lack of adequate funding to continue the Non-IND, IRB-Approved Clinical Study or Clinical Trial. Many of the factors that cause, or lead to, a delay in the commencement or completion of Non-IND, IRB-Approved Clinical Studies or Clinical Trials may also ultimately lead to the denial of regulatory approval of our AXA Candidates for therapeutic indications, where applicable, or the failure to meet applicable regulatory requirements to support and commercialize non-drug products. Further, the FDA or other regulatory authorities may disagree with our Non-IND, IRB-Approved Clinical Study or Clinical Trial design and our interpretation of data from these clinical studies or trials, or may change the requirements for regulatory approval of a drug even after they have reviewed and commented on the design for our preclinical studies, Non-IND, IRB-Approved Clinical Studies or Clinical Trials.

Our product development costs will increase if we experience delays in clinical testing and marketing approvals, if applicable, or otherwise meeting regulatory requirements to commercialize our AXA Candidates. We do not know whether any of our preclinical studies, Non-IND, IRB-Approved Clinical Studies or Clinical Trials, if applicable, will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant delays in our preclinical studies, Non-IND, IRB-Approved Clinical Studies or Clinical Trial also could shorten any periods during which we may have the exclusive right to commercialize our AXA Candidates and may allow our competitors to bring products to market before we do, potentially impairing our ability to successfully commercialize our AXA Candidates and harming our business and results of operations. Any delays in our preclinical or future clinical development programs may harm our business, financial condition and prospects significantly, and could impair our ability to fund our operations or obtain financing on acceptable terms, or at all.

Our planned Non-IND, IRB-Approved Clinical Studies or any future Clinical Trials, if applicable, or those of our future collaborators may reveal significant adverse events not seen in our preclinical studies, Non-IND, IRB-Approved Clinical Studies or other Clinical Trials and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our AXA Candidates.

Before obtaining regulatory approvals for the commercial sale of any products for therapeutic indications, we must demonstrate through lengthy, complex and expensive preclinical studies and Clinical Trials that our AXA Candidates are both safe and effective for use in each target indication. Preclinical and clinical studies and testing are expensive and can take many years to complete, and their outcome is inherently uncertain. Failure can occur at any time during the preclinical or clinical study or trial process. The results of preclinical studies, Non-IND, IRB-Approved Clinical Studies as well as early Clinical Trials of our AXA Candidates may not be predictive of the results of later-stage clinical studies or trials. In addition, initial results in Non-IND, IRB-Approved Clinical Studies and Clinical Trials, in particular as shown by any interim data, may not be indicative of results obtained when such Non-IND, IRB-Approved Clinical Studies and Clinical Trials are completed. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical studies and trials. Our AXA Candidates have been generally well tolerated in our Non-IND, IRB-Approved Clinical Studies, but we are not certain that we will be able to dose participants at a high enough dose in any future Clinical Trials so as to demonstrate efficacy without unacceptable safety risk.

AXA Candidates in later stages of Non-IND, IRB-Approved Clinical Studies may fail to show the desired safety profile despite having progressed through successful preclinical studies and earlier

Non-IND, IRB-Approved Clinical Studies. AXA Candidates that we decide to develop as drug product candidates and that progress to Clinical Trials may fail to show the desired safety and efficacy profile despite having progressed successfully through preclinical studies, Non-IND, IRB-Approved Clinical Studies and, if applicable, initial Clinical Trials. A number of companies in the healthcare industry have suffered significant setbacks in later development, notwithstanding promising results in earlier trials. Most product candidates that commence Clinical Trials are never approved as products for therapeutic indications and there can be no assurance that any of our current or future Non-IND, IRB-Approved Clinical Studies or Clinical Trials will ultimately be successful or support further clinical development of any of our AXA Candidates.

If significant adverse events or other side effects are observed in any of our current or future Non-IND, IRB-Approved Clinical Studies or Clinical Trials, we may have difficulty recruiting subjects or patients for our Non-IND, IRB-Approved Clinical Studies or Clinical Trials, subjects or patients may drop out of our Non-IND, IRB-Approved Clinical Studies or Clinical Trials or we may be required to significantly redesign or abandon Non-IND, IRB-Approved Clinical Studies or Clinical Trials or our development efforts of one or more AXA Candidates altogether. We, the FDA or other applicable regulatory authorities or an IRB may suspend Non-IND, IRB-Approved Clinical Studies or Clinical Trials of an AXA Candidate at any time for various reasons, including a belief that subjects or patients in such Non-IND, IRB-Approved Clinical Studies or Clinical Trials are being exposed to unacceptable health risks or adverse side effects or that our Non-IND, IRB-Approved Clinical Studies are designed to evaluate our AXA Candidates as drug product candidates. Some potential non-drug products and drug product candidates that initially showed promise for further development in early-stage testing, including in Non-IND, IRB-Approved Clinical Studies or Clinical Trials, have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude an AXA Candidate from obtaining or maintaining marketing approval, if applicable, or being commercialized, undesirable side effects may inhibit market acceptance of the commercialized product due to its tolerability versus other non-drug products or drugs. Any of these developments could materially harm our business, financial condition and prospects.

If we encounter difficulties enrolling study subjects or patients in our Non-IND, IRB-Approved Clinical Studies or any future Clinical Trials, our development activities could be delayed or otherwise adversely affected.

We may experience difficulties in subject and patient enrollment in our Non-IND, IRB-Approved Clinical Studies and Clinical Trials for a variety of reasons. The timely completion of Non-IND, IRB-Approved Clinical Studies or Clinical Trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of subjects or patients who remain in the Non-IND, IRB-Approved Clinical Study or Clinical Trial until its conclusion. The enrollment of subjects or patients depends on many factors, including:

- the severity of the disease or condition under investigation in the case of a Clinical Trial conducted under an IND for an AXA Candidate that we decide to develop as a drug product candidate;
- the subject or patient eligibility and exclusion criteria defined in the protocol;
- the size of the study subject or patient population required for analysis of the primary endpoint(s) of the Non-IND, IRB-Approved Clinical Study or Clinical Trial;
- the proximity of subjects or patients to study and trial sites;
- the design of the clinical study or trial;
- our ability to recruit investigators with the appropriate competencies and experience;

- clinicians', subjects' or patients' perceptions as to the potential advantages and risks of the AXA Candidate being studied in relation to other available drugs or non-drug products, as applicable;
- the efforts to facilitate timely enrollment in clinical studies or trials;
- the subject or patient referral practices of physicians;
- the ability to monitor subjects or patients adequately during and after study product administration;
- our ability to obtain and maintain subject and patient consents; and
- the risk that subjects or patients enrolled in Non-IND, IRB-Approved Clinical Studies or Clinical Trials will drop out of the Non-IND, IRB-Approved Clinical Studies or Clinical Trials before completion.

In addition, our Non-IND, IRB-Approved Clinical Studies and Clinical Trials will compete with other clinical studies or trials for product candidates that are in the same target markets as our AXA Candidates, and this competition will reduce the number and types of subjects or patients available to us, because some individuals who might have opted to enroll in our clinical studies or trials may instead opt to enroll in a study or trial being conducted by one of our competitors. Because the number of qualified clinical investigators is limited, we expect to conduct some of our Non-IND, IRB-Approved Clinical Studies or Clinical Trials at the same sites that some of our competitors use, which will reduce the number of subjects or patients who are available for our Non-IND, IRB-Approved Clinical Studies and Clinical Trials in such sites. Moreover, because our AXA Candidates represent a departure from more commonly used methods in the non-drug and drug areas we may pursue, potential subjects or patients and their doctors may be inclined to use conventional products or therapies, rather than enroll subjects or patients in any future clinical study or trial.

Delays in subject or patient enrollment may result in increased costs or may affect the timing or outcome of our planned Non-IND, IRB-Approved Clinical Studies or future Clinical Trials, which could prevent completion of these clinical studies or trials and adversely affect our ability to advance the development of our AXA Candidates.

Interim and preliminary data from our Non-IND, IRB-Approved Clinical Studies or future Clinical Trials that we announce or publish from time to time may change as more subject or patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

To date, our Non-IND, IRB-Approved Clinical Studies have been exploratory in nature and in small numbers of subjects. Data from larger subsequent studies may not support or may be inconsistent with our observations in our completed Non-IND, IRB-Approved Clinical Studies. From time to time, we may conduct Non-IND, IRB-Approved Clinical Studies and Clinical Trials that result in interim or preliminary data. These data are subject to the risk that one or more of the outcomes may materially change as preclinical studies complete, subject enrollment continues and more subject data become available. Preliminary or top-line data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Material inconsistencies between preliminary or interim data and final data could significantly harm our business prospects.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our research and development activities may involve the use of biological and hazardous materials and may produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may negatively impact our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of biological waste or hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological waste or hazardous waste insurance coverage, workers compensation or property and casualty and general liability insurance policies that include coverage for damages and fines arising from biological or hazardous waste exposure or contamination.

If product liability claims or lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our AXA Candidates.

We face an inherent risk of product liability as a result of testing our AXA Candidates in Non-IND, IRB-Approved Clinical Studies and Clinical Trials, if we decide to develop any AXA Candidate as a drug product candidate, and will face an even greater risk if we commercialize any products. For example, we may be sued, or claims may be made against us, if our informed consents for subjects or patients in any preclinical, Non-IND, IRB-Approved Clinical Studies or Clinical Trials are or are alleged to be inadequate or inaccurate in any way or fail to fully inform subjects or patients of any potential risks involved with their participation or other material or required information. We may also be sued, or claims may be made against us, if our AXA Candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during Non-IND, IRB-Approved Clinical Studies, Clinical Trials, manufacturing, marketing or after sale. Any such product liability claims may include, without limitation, allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, marketing or promotional claims or a breach of warranties. Claims could also be asserted under state consumer protection or other statutes or regulations. If we cannot successfully defend ourselves against product liability claims or any other claims related to our products, we may incur

substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims could have a material adverse effect on our business and operations, and may result in, among other things:

- inability to bring an AXA Candidate to the market;
- decreased demand for our products;
- damage to our reputation;
- withdrawal of Non-IND, IRB-Approved Clinical Study or Clinical Trial participants and inability to enroll future participants or continue Non-IND, IRB-Approved Clinical Studies or Clinical Trials;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- diversion of management's time and our resources;
- substantial monetary awards to participants or patients;
- product recalls, withdrawals or labeling, packaging, marketing or promotional modifications or restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any AXA Candidates via any regulatory pathway; and
- decline in our share price.

We maintain clinical trial insurance. We review our clinical trial insurance policy annually and we believe that our coverage is currently adequate to cover any claims that may arise in connection with our Non-IND, IRB-Approved Clinical Studies or Clinical Trials. There is no guarantee that we will be able to obtain additional clinical trial insurance at an acceptable cost in the future, which could prevent or inhibit the ongoing development of our products.

Since we have not yet commenced marketing of any products we do not yet hold product liability insurance for commercialization of our products. Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. If and when coverage is secured, our insurance policies may also have various exclusions, and we may be subject to a product liability claim for which we have no or inadequate coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

The market opportunities for our AXA Candidates may be limited and our estimates of the incidence and prevalence of our target patient populations may be inaccurate.

Our projections of both the non-drug and drug market sizes we may target and the number of people who have the diseases or conditions we may target, as well as the subset of people with these diseases or conditions in a position to receive any drug or non-drug we develop, if approved, are based on our beliefs and estimates. These estimates have been derived from a variety of

sources, including scientific literature, input from key opinion leaders, patient foundations or secondary market research databases, and may prove to be incorrect. Further, new studies may change the estimated market sizes or the incidence or prevalence of target diseases we may target with AXA Candidates that we decide to develop as drug product candidates. For those AXA Candidates we develop under an IND, regulatory approvals may include limitations for use or contraindications that decrease the addressable patient population. The number of subject individuals may turn out to be lower than expected. Additionally, the potentially addressable patient population for our AXA Candidates that we decide to develop as drugs may be limited or may not be amenable to treatment with our AXA Candidates. For instance, we estimate that there are approximately 633,000 patients currently diagnosed with cirrhosis in the United States and upwards of 518,000 patients undergoing or recovering from procedures that are associated with limb immobilization-induced acute atrophy. Even if we obtain significant market share for our AXA Candidates, because certain potential target populations are small, we may never achieve profitability without obtaining regulatory approval for additional indications for drugs or expanding the target market size for non-drug products.

We are early in our development efforts and may not be successful in our efforts to use our AXA Development Platform to build a pipeline of AXA Candidates and develop marketable products.

We are developing our AXA Candidates and AXA Development Platform to reprogram metabolism and maintain and restore metabolic health. However, our AXA Development Platform has not yet led, and may never lead, to marketable drug or non-drug products. We are developing our initial AXA Candidates and have additional AXA Candidates that we intend to investigate, including in liver, muscle, central nervous system and blood, and in the future, we may decide to develop any AXA Candidate as a drug product candidate. We may have problems applying our technologies to these and other future target areas, and our AXA Candidates may not demonstrate a comparable ability in supporting or maintaining health or treating disease, where applicable. Even if we are successful in identifying additional AXA Candidates, they may not be suitable for clinical development as a result of limited efficacy, unacceptable safety profiles or other characteristics that indicate that they are unlikely to be products that will receive marketing approval or achieve market acceptance. The success of our AXA Candidates will depend on several factors, including the following:

- completion of preclinical studies, Non-IND, IRB-Approved Clinical Studies and Clinical Trials with positive results;
- receipt of marketing approvals from applicable regulatory authorities, if necessary;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our AXA Candidates;
- making arrangements with third-party manufacturers for, or establishing our own, commercial manufacturing capabilities;
- launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- entering into new collaborations throughout the development process as appropriate, from preclinical studies through to commercialization;
- acceptance of our products, if and when approved or launched for commercialization under applicable regulations, by patients, consumers, the medical community and third-party payors;

- effectively competing with other drugs and non-drug products, depending on the development pathway that we choose for an AXA Candidate;
- obtaining and maintaining coverage and adequate reimbursement by third-party payors, including government payors, for our AXA Candidates developed as drug products, if approved by the FDA;
- protecting our rights in our intellectual property portfolio;
- operating without infringing or violating the valid and enforceable patents or other intellectual property of third parties;
- remaining in compliance with applicable laws and regulations that apply to the research, development and commercialization of our AXA Candidates and having productive interactions and positive regulatory decisions that lead to successful product commercialization;
- maintaining a continued acceptable safety profile of the products following approval or commercialization; and
- maintaining and growing an organization of scientists and business people who can develop and commercialize our products and technology.

If we do not successfully develop and commercialize AXA Candidates using our AXA Development Platform, we will not be able to obtain product revenue in future periods, which could result in significant harm to our financial position and adversely affect our stock price.

We face significant competition from other healthcare companies, and our operating results will suffer if we fail to compete effectively.

The healthcare industry is characterized by intense competition and rapid innovation. Our competitors may be able to develop other drug or non-drug products that are able to achieve similar or better results. Our potential competitors include major multinational pharmaceutical, nutritional foods companies, established biotechnology companies, specialty pharmaceutical companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff, experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Established pharmaceutical companies may also invest heavily to accelerate discovery and development of novel drugs or to in-license novel drugs that could make any AXA Candidate that we develop as a drug product candidate obsolete. Mergers and acquisitions in the healthcare industry may result in even more resources being concentrated amongst our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on exclusive basis non-drug products that are more effective, safer, more easily commercialized or less costly than our AXA Candidates or may develop proprietary technologies or secure patent protection that we may need for the development of our technologies and products. We believe the key competitive factors that will affect the development and commercial success of our AXA Candidates are efficacy, safety, tolerability, reliability, convenience of use, price and reimbursement, if applicable depending on the development path we choose. We also anticipate that we will face increased competition in the future as additional companies enter our market and scientific developments surrounding other non-drug products and drugs targeted at metabolic pathways continue to accelerate.

In addition, there are additional companies that are working on modulating specific metabolic pathways involved in various health and disease conditions, although we are not aware of any company creating AXA Candidate-like products with multifactorial activity. Companies with clinical programs that could compete with our AXA Candidates include Madrigal Pharmaceuticals, Inc., Intercept Pharmaceuticals, Inc., Gilead Sciences, Inc., Novartis AG, Bristol-Myers Squibb Co., Esperion Therapeutics, Inc., Viking Therapeutics, Inc., Scholar Rock Holding Corporation, NGM Biopharmaceuticals Inc., Genfit SA and Kaleido Biosciences, Inc., among others.

We also anticipate competing with the largest consumer health companies and nutritional and amino acid companies in the world, such as Nestlé Health Science S.A., Abbott Laboratories, Johnson & Johnson, The Procter & Gamble Company and Ajinomoto Co., Inc., all of which are currently conducting research in competitive indications or may be interested in using amino acids and other EMMs as therapeutics as well as nutritional supplements.

Even if we obtain regulatory approval to market our AXA Candidates as drugs or are successful in identifying alternate regulatory pathways to market our AXA Candidates under regulations that would apply to non-drug products, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our AXA Candidates. We may not be able to implement our business plan if the acceptance of our AXA Candidates is inhibited by price competition or the reluctance of consumers to accept of our AXA Candidates and choose them over other competitive products on the market or, for AXA Candidates we develop as drugs, of physicians to switch from existing methods of treatment to our AXA Candidates, or if physicians switch to other new drug or biologic products or choose to reserve our AXA Candidates for use in limited circumstances. For additional information regarding our competition, see "Business—Competition."

Even if an AXA Candidate we develop as a drug product candidate receives marketing approval, or otherwise is commercialized as a non-drug product, such products may fail to achieve the degree of market acceptance by physicians, patients, third-party payors, consumers and others in the medical or healthcare community or other target markets necessary for commercial success.

If any AXA Candidate we decide to develop as a drug product candidate receives marketing approval or otherwise is commercialized under applicable regulations as a non-drug product, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors, consumers and others in the medical or health community or other target markets. If the AXA Candidates we develop do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of any AXA Candidate, if approved for commercial sale, will depend on a number of factors, including:

- efficacy (for any AXA Candidate developed as a drug product candidate), safety and potential advantages compared to alternative products;
- the labeled uses or limitations for use, including age limitations or contraindications, for our AXA Candidates compared to alternative products;
- convenience and ease of administration compared to alternative products;
- the willingness of the target patient or consumer population to try new drugs or non-drug products, respectively, and, with respect to any AXA Candidates developed as drug product candidates, of physicians to prescribe these therapies or, in the case of non-drug products, the willingness of target consumers in the market of health products to try and healthcare professionals to recommend consumers purchase our products;

- public perception of new drugs and non-drug products, including our AXA Candidates;
- the strength of marketing and distribution support;
- the ability for us to partner in the manufacture and distribution of these products;
- the ability to offer our products, if approved, as applicable, for sale at competitive prices;
- the ability to obtain sufficient third-party insurance coverage and adequate reimbursement, as applicable depending on the development path we pursue; and
- the prevalence and severity of any side effects.

We will need to grow the size of our organization and we may experience difficulties in managing this growth.

As of December 31, 2018, we had 49 full-time employees. As our research, development, manufacturing and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect to need additional managerial, operational, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, compensating, integrating, maintaining and motivating additional employees;
- managing our internal research and development efforts effectively, including identification of clinical candidates, scaling our manufacturing process and navigating the clinical and the FDA review process for our AXA Candidates; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to commercialize our AXA Candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain organizations, advisors and consultants to provide certain services, including many aspects of regulatory affairs, clinical management and manufacturing. There can be no assurance that the services of these organizations, advisors and consultants will continue to be available to us on a timely basis when needed or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical development may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our AXA Candidates, if required, or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our AXA Candidates and, accordingly, may not achieve our research, development and commercialization goals.

Our current operations are located in Massachusetts; and we or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Our current operations are located in Massachusetts. Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics, power shortage, telecommunication failure or other natural or manmade accidents or incidents that result in us being unable to fully utilize our facilities, or the manufacturing facilities of our third-party contract manufacturers, may have a material and adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Loss of access to these facilities may result in increased costs, delays in the development of our AXA Candidates or interruption of our business operations. Natural disasters could further disrupt our operations, and have a material and adverse effect on our business, financial condition, results of operations and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our research facilities or the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure our investors that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities or the manufacturing facilities of our third-party contract manufacturers are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any business interruption may have a material and adverse effect on our business, financial condition, results of operations and prospects.

If we lose key management personnel, or if we are unable to recruit additional highly skilled personnel, our ability to identify and develop new or next generation AXA Candidates will be impaired, could result in loss of markets or market share and could make us less competitive.

Our ability to compete in the highly competitive biotechnology industry depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel, including David Epstein, our Chairman of the Board, William Hinshaw, our Chief Executive Officer and President, Thomas Leggett, our Chief Financial Officer and Senior Vice President of Finance, Manu Chakravarthy, M.D., Ph.D., our Chief Medical Officer and Senior Vice President of Clinical Development, Tony Tramontin, Ph.D., our Chief Scientific Officer and Senior Vice President of Research and Development, Stephen Mitchener, PharmD, our Chief Business Officer and Senior Vice President and Paul Fehlner, J.D., Ph.D., our Chief Intellectual Property Officer and Senior Vice President. The loss of the services of any of our executive officers, other key employees and other scientific and medical advisors, and our inability to find suitable replacements could result in delays in product development and harm our business.

We conduct our operations in Massachusetts. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock

options that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Employment of our key employees is at-will, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain "key man" insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical personnel.

Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations, and those of our CROs, contract manufacturing organizations, or CMOs, manufacturers of the raw materials used in our AXA Candidates and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. For our Non-IND, IRB-Approved Clinical Studies, we rely on third-party manufacturers to produce our AXA Candidates, on CROs for conducting various portions of such studies and on various consultants throughout the study process. For materials to be used in any future Clinical Trials for AXA Candidates that we decide to develop as drug product candidates, we plan to rely on an external CMO for the entire manufacturing supply chain and plan to continue using CROs and consultants in connection with conducting such trials. Our ability to obtain supplies of our AXA Candidates and services from CROs and consultants could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption.

Our internal computer systems, or those used by our CROs, CMOs or other contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our CROs, CMOs and other contractors and consultants are vulnerable to damage from computer viruses and unauthorized access. While we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of data from any future Clinical Trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we currently rely on third parties for the manufacture of our AXA Candidates and to conduct Non-IND, IRB-Approved Clinical Studies, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our AXA Candidates could be delayed.

Regulators globally are also imposing greater monetary fines for privacy violations. For example, in 2016, the European Union adopted a new regulation governing data practices and privacy called the General Data Protection Regulation, or GDPR, which became effective on May 25, 2018. The GDPR applies to any company that collects and uses personal data in connection with offering goods or services to individuals in the European Union or the monitoring of their behavior. Non-compliance with the GDPR may result in monetary penalties of up to €20 million or 4% of worldwide revenue, whichever is higher. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of personal data, such as healthcare data or other sensitive information, could greatly increase the cost of providing our AXA Candidates, if approved, or even prevent us from offering our AXA Candidates, if approved, in certain jurisdictions.

Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless or negligent conduct that fails to comply with the laws of the FDA and other similar foreign regulatory bodies, provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies, comply with manufacturing standards we have established, comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our AXA Candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. These laws may impact, among other things, our current activities with principal investigators and research subjects, as well as proposed and future sales, marketing and education programs.

A variety of risks associated with testing and developing our AXA Candidates internationally could materially adversely affect our business.

In addition to researching, developing and commercializing our AXA Candidates in the United States, we also intend to engage in these activities outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries, if our AXA Candidates are approved, including:

- differing regulatory requirements in foreign countries;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act, or FCPA, or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism.

Additionally, we intend to contract with third parties to conduct some of our Clinical Trials outside the United States, which will subject us to additional risks and regulations. These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

We currently have no marketing and sales organization and have no experience in marketing products for therapeutic or non-drug uses. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our AXA Candidates, we may not be able to generate product revenue.

We currently have no sales, marketing or distribution capabilities and have no experience in marketing products for therapeutic uses or other non-drug uses. We intend to develop an in-house marketing organization and sales force, which will require significant capital expenditures, management resources and time. We will have to compete with other healthcare companies to recruit, hire, train and retain marketing and sales personnel.

In addition to establishing internal sales, marketing and distribution capabilities, we intend to optimistically pursue collaborative arrangements regarding the sales and marketing of our products, in particular for products we develop as non-drug products; however, there can be no assurance that we will be able to establish or maintain such collaborative arrangements, or if we are able to do so, that they will have effective sales forces. Any revenue we receive will depend upon the efforts of such third parties, which may not be successful. We may have little or no control over the marketing and sales efforts of such third parties and our revenue from product sales may be lower than if we had commercialized our AXA Candidates ourselves. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our AXA Candidates.

There can be no assurance that we will be able to develop in-house sales and distribution capabilities or establish or maintain relationships with third-party collaborators to commercialize any product in the United States or overseas.

Comprehensive tax reform legislation could adversely affect our business and financial condition.

The Tax Cuts and Jobs Act, or the TCJA, that significantly reforms the Internal Revenue Code of 1986, as amended, or the Code. The TCJA, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal tax rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses, or NOLs, to 80% of current year taxable income and elimination of NOL carrybacks, in each case, for losses generated after December 31, 2017 (though any such NOLs may be carried forward indefinitely), and modifying or repealing many business deductions and credits, including reducing the business tax credit for certain clinical testing expenses incurred in the testing of certain drugs for rare diseases or conditions generally referred to as "orphan drug." We continue to examine the impact this tax reform legislation may have on our business. We urge investors to consult with their legal and tax advisers regarding the implications of the TCJA on an investment in our common stock.

Our ability to use NOLs and research and development credits to offset future taxable income may be subject to certain limitations.

As of December 31, 2018, we had U.S. federal and state NOL carryforwards of $140.6 million and $139.4 million, respectively, both of which expire at various dates beginning in 2030. As of December 31, 2018, we also had U.S. federal and state research and development tax credit carryforwards of $4.2 million and $1.6 million respectively, both of which expire at various dates

through 2038. These NOL and tax credit carryforwards could expire unused and be unavailable to offset future taxable income or tax liabilities, respectively. In addition, in general, under Sections 382 and 383 of the Code, and corresponding provisions of state law, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change NOL carryforwards or tax credits, or Credits, to offset future taxable income or taxes. For these purposes, an ownership change generally occurs where the aggregate stock ownership of one or more stockholders or groups of stockholders who owns at least 5% of a corporation's stock increases its ownership by more than 50 percentage points over its lowest ownership percentage within a specified testing period. Our existing NOLs or Credits may be subject to limitations arising from previous ownership changes, and if we undergo an ownership change in connection with or after this offering, our ability to utilize NOLs or Credits could be further limited by Sections 382 and 383 of the Code. In addition, future changes in our stock ownership, many of which are outside of our control, could result in an ownership change under Sections 382 and 383 of the Code. Our NOLs or Credits may also be impaired under state law. Accordingly, we may not be able to utilize a material portion of our NOLs or Credits. Furthermore, our ability to utilize our NOLs or Credits is conditioned upon our attaining profitability and generating U. S. federal and state taxable income. We have incurred significant net losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future; and therefore, we do not know whether or when we will generate the U.S. federal or state taxable income necessary to utilize our NOLs or Credits that are subject to limitation by Sections 382 and 383 of the Code. The reduction of the corporate tax rate under the TCJA caused a reduction in the economic benefit of our NOL carryforwards and other deferred tax assets available to us. Under the TCJA, NOLs generated after December 31, 2018 will not be subject to expiration; however, any NOLs generated after December 31, 2018 may only offset 80% of our annual taxable income.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

As widely reported, global credit and financial markets have experienced extreme volatility and disruptions in the past, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. There can be no assurance that future volatility, disruption or deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. If the current equity and credit markets continue to be volatile or are disrupted or deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget.

As of December 31, 2018, we had cash and cash equivalents of approximately $79.5 million. While we are not aware of any downgrades, material losses or other significant deterioration in the fair value of our cash equivalents since December 31, 2018, no assurance can be given that further deterioration of the global credit and financial markets would not negatively impact our current portfolio of cash equivalents or our ability to meet our financing objectives. Furthermore, our stock price may decline due in part to the volatility of the stock market and the general economic downturn.

Our loan agreement subjects us to operating restrictions and financial covenants and may restrict our business and financing activities.

On January 9, 2018, we entered into a loan and security agreement with Solar Capital Ltd., or Solar, for term loans in an aggregate principal amount of $26 million, which we amended on October 5, 2018 and November 30, 2018. Our obligations under the loan agreement are secured by a first priority security interest in our assets, excluding intellectual property and certain other exceptions. We are subject to a negative pledge covenant with respect to our intellectual property. The loan agreement contains customary representations, as well as customary affirmative and negative covenants. Among other restrictions, the negative covenants, subject to exceptions, prohibit or limit our ability to: declare dividends or redeem or purchase equity interests; incur additional liens; make investments; incur additional indebtedness; engage in mergers, acquisitions and asset sales; transact with affiliates; undergo a change in control; add or change business locations; and engage in businesses that are not related to its existing business. These covenants may restrict our ability to finance our operations and to pursue our business activities and strategies. Our ability to comply with these covenants may be affected by events beyond our control.

Risks related to government regulation

We are very early in our development efforts. AXA Candidates we decide to develop as drug product candidates will require significant additional preclinical and clinical development before we seek regulatory approval. AXA Candidates that we decide to bring to market as non-drug products may also require additional development, and all AXA Candidates may require significant interactions with regulators and investments before their respective commercial launches. If we are unable to advance our AXA Candidates to final development, meet regulatory requirements, including obtaining regulatory approval, where applicable, or ultimately commercialize our AXA Candidates or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development efforts and have invested substantially all of our efforts and financial resources in the identification and early clinical development of AXA Candidates. To date, we have not marketed or commercialized products and, although we have made a development path decision to develop AXA1665 as a drug product candidate under a planned IND submission, we have not done so for any of our other AXA Candidates, including whether to continue their development under food or dietary supplement regulations or to pursue their development as drugs. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend on the successful development and eventual commercialization of our AXA Candidates, which may never occur. We currently generate no revenue from sales of any products, and we may never be able to develop or commercialize a marketable product. The success of our AXA Candidates will depend on several factors, including the following:

- successful completion of preclinical studies, Non-IND, IRB-Approved Clinical Studies and, if applicable, Clinical Trials;
- clearance of INDs for future Clinical Trials for AXA Candidates that we decide to develop as drug product candidates;
- successful enrollment in, and completion of, Non-IND, IRB-Approved Clinical Studies and Clinical Trials, if applicable;
- receipt of regulatory approvals from applicable regulatory authorities for drug product candidates or, alternatively, compliance with regulatory requirements applicable to non-drug products;

- establishing cGMP-compliant supply and commercial manufacturing operations or making arrangements with cGMP-compliant third-party manufacturers for supply and commercial manufacturing;
- obtaining and maintaining patent and trade secret protection or regulatory exclusivity for our AXA Candidates;
- launching commercial sales of our AXA Candidates, if and when approved or allowed for marketing, whether alone or in collaboration with others;
- acceptance of our drug product candidates, if and when approved, by patients, the medical community and third-party payors, if we decide to develop any of our AXA Candidates as drug product candidates, or acceptance of our non-drug products we may market by consumers;
- effectively competing with other drugs for any AXA Candidate developed and approved as a drug or competing with other non-drug products for any AXA Candidate developed and marketed as such;
- obtaining and maintaining third-party insurance coverage and adequate reimbursement, as applicable;
- enforcing and defending intellectual property rights and claims;
- the marketing of our products; and
- maintaining a continued acceptable safety profile of the AXA Candidates following approval or commercialization.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our AXA Candidates, which would materially harm our business. If we do not receive regulatory approvals or identify and execute on alternate regulatory pathways to market for our AXA Candidates, we may not be able to continue our operations.

Regulatory requirements for development of our AXA Candidates as drugs or as non-drug products are uncertain and evolving. Changes in these laws, including our ability to conduct Non-IND, IRB-Approved Clinical Studies, or the current interpretation or application of these laws, or conflicts between us and the FDA on the applicability or interpretation of applicable laws, would have a significant adverse impact on our ability to develop and commercialize our products.

In the United States, under sections 201(s) and 409 of the Federal Food, Drug, and Cosmetic Act, or the FD&C Act, any substance that is reasonably expected to become a component of food is considered to be a food additive, and therefore subject to FDA premarket review and approval, unless the substance is generally recognized, among qualified experts, as having been adequately shown to be safe under the conditions of its intended use. Moreover, under federal law, dietary supplement products must only contain certain permissible dietary ingredients, and any ingredients considered to be an NDI under the FD&C Act will require pre-market notification to the FDA.

Based on the large body of studies and scientific literature on the human exposure to and safety profiles of certain amino acids, the FDA's promulgation of regulations governing the use of certain amino acids under certain conditions as safe and permissible food additives when used as nutrients, our own data on amino acids used in AXA Candidates and the fact that we use amino acids in our AXA Candidates within amounts previously studied safely in humans, we believe we have designed our AXA Candidates to have favorable safety profiles, and we further have evaluated

or will evaluate the safety and tolerability of these AXA Candidates in Non-IND, IRB-Approved Clinical Studies. Under the FDA's framework governing studies of non-drug products, we believe that use of our AXA Candidates containing amino acids may be studied for safety and tolerability without an IND. However, the FDA or comparable regulators may disagree with this approach and determine that our studies should be conducted under an IND, which may result in negative consequences. To date, we have only had a pre-IND meeting with the FDA regarding the potential development of AXA1665 as drug under IND but have not met with FDA in connection with our other programs or at all with other comparable foreign regulatory authorities to discuss our approach or plans for further development.

In prior or future studies or trials of our AXA Candidates, we may have or will expressly or implicitly characterize or classify such candidates as encompassed within a specific regulatory scheme (e.g., as foods or dietary supplements). Regulators may not agree with the regulatory classification of the AXA Candidates used in our Non-IND, IRB-Approved Clinical Studies or any subsequent classification of such candidates prior to commercialization. To date, we have had one pre-IND meeting with FDA regarding our AXA1665 program and have not discussed the development of our other AXA Candidates evaluated in Non-IND, IRB-Approved Clinical Studies or our utilization of specific regulatory pathways for our other AXA Candidates with the FDA or comparable foreign regulatory authorities and any such regulator may not agree with our current activities or future approach or plans. The FDA may determine that our AXA Candidates are not safe or appropriate for use in Non-IND, IRB-Approved Clinical Studies or are not governed by food regulations and therefore may classify any of our AXA Candidates as being ineligible for investigation in clinical studies without an IND. The FDA or other regulatory authorities may also take enforcement action, or otherwise delay or prevent further development or commercialization of our AXA Candidates.

The FDA may determine that our AXA Candidates cannot be marketed as or do not meet the regulatory requirements for marketing or testing as non-drug products. The FDA may take the position that we failed to satisfy the premarket requirements for ingredient compositions, including that the particular product is not generally recognized as safe, or GRAS, is an unapproved food additive, is a NDI requiring premarket review or that our products contain otherwise impermissible ingredients, in which case some or all of our products may be deemed adulterated or misbranded in violation of the FD&C Act. Moreover, if we choose to study a product under an IND before the product candidate has been marketed as a non-drug product, the FD&C Act could prevent us from marketing the product as a non-drug product if we are unable to secure FDA approval as a new drug. Any delay in the regulatory consultation process, or a warning, finding or determination that any of our operations or product candidates do not meet the regulatory requirements of the FDA, including but not limited to any applicable GRAS, food additive or NDI requirements, could subject the company to regulatory enforcement action, and/or cause a delay in or prevent the commercialization of one or more of our product candidates, which may lead to reduced acceptance by the public or others for any products we are able to commercialize and could materially adversely affect our business.

The FDA may determine that the only pathway for conducting studies of our AXA Candidates is under an IND or that our Non-IND, IRB-Approved Clinical Studies already conducted should have been conducted under an IND. Any such determination could prevent our reliance on existing regulatory frameworks to conduct Non-IND, IRB-Approved Clinical Studies for other AXA Candidates or prevent us from relying on or including data from our Non-IND, IRB-Approved Clinical Studies in any regulatory submissions to support further clinical development or marketing approval, and could significantly increase the cost of and delay the development or commercialization of AXA Candidates. If the FDA disagrees with our determination that we may conduct Non-IND, IRB-Approved Clinical Studies without filing an IND, they could require that we halt any Non-IND,

IRB-Approved Clinical Studies or Clinical Trials we have commenced, or we may be subject to enforcement action. Should we choose to commercialize our AXA Candidates as non-drug products and if the FDA determines our AXA Candidates fall outside the food regulations, we may be subject to regulatory enforcement action and we could be required to stop selling, withdraw, recall, re-label or re-package any products we have commercialized as non-drug products on the market. In addition, if new safety issues are raised by Non-IND, IRB-Approved Clinical Studies in advance of deciding whether to file an IND that suggest safety concerns for all of our AXA Candidates, then the FDA could ask us to modify approved labeling for or withdraw from the market any previously approved products for therapeutic uses or products being commercialized for other non-drug uses. A decision by the FDA that we cannot conduct Non-IND, IRB-Approved Clinical Studies without filing an IND would significantly impact our current business model and we may incur significant expense and operational difficulties.

Changes in the legal and regulatory environment could limit our future business activities, increase our operating or regulatory costs, reduce demand for our AXA Candidates or result in litigation.

The conduct of our business, including, but not limited, to the development, testing, production, storage, distribution, sale, display, advertising, marketing, labeling, packaging, health and safety practices and regulatory classification and approval (where necessary) use of many of our AXA Candidates, is subject to various laws and regulations administered by federal, state and local governmental agencies in the United States, as well as to laws and regulations administered by government entities and agencies outside the United States in markets in which we conduct clinical studies or trials under foreign food or drug regulations or in which our AXA Candidates and components thereof (such as packaging) may be manufactured or sold.

These laws and regulations and interpretations thereof may change, sometimes dramatically, as a result of a variety of factors, including political, economic or social events. Such changes may include changes in:

- food and drug laws, including FDA regulations;
- laws related to product labeling;
- advertising and marketing laws and practices;
- laws and programs restricting the sale and advertising of certain of product candidates;
- laws and programs aimed at regulating, restricting or eliminating ingredients present in certain of our AXA Candidates;
- increased regulatory scrutiny of, and increased litigation involving, product claims and concerns regarding the actual or possible effects or side effects of ingredients in, or attributes of, certain of our AXA Candidates;
- state and federal consumer protection and disclosure laws; and
- increased sponsor or company obligations under privacy laws such as the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, and GDPR.

New laws, regulations or governmental policy and their related interpretations, or changes in any of the foregoing, may alter the environment in which we do business and, therefore, may impact our operating results or increase our costs or liabilities.

We may rely on academic and private non-academic institutions to conduct investigator-sponsored Non-IND, IRB-Approved Clinical Studies or Clinical Trials of our AXA Candidates. Any failure by the investigator-sponsor to meet its obligations with respect to the clinical development of our AXA Candidates may delay or impair our ability to obtain regulatory approval or otherwise commercialize the applicable AXA Candidates.

We may rely on academic and private non-academic institutions to conduct and sponsor clinical studies or trials relating to our AXA Candidates. We will not control the design or conduct of the investigator-sponsored trials, and it is possible that the FDA or ex-U.S. regulatory authorities will not view these investigator-sponsored studies or trials as providing adequate support to allow for the initiation of future Clinical Trials for those AXA Candidates that we choose to develop as drug product candidates, whether controlled by us or independent investigators, for any one or more reasons, including elements of the design or execution of the trials or safety concerns or other trial results.

Such arrangements will likely provide us certain information rights with respect to the investigator-sponsored studies or trials, including access to and the ability to use and reference the data, including for our own regulatory filings, resulting from the investigator-sponsored studies or trials. However, we would not have control over the timing and reporting of the data from investigator-sponsored trials, nor would we own the data from the investigator-sponsored studies or trials. If we are unable to confirm or replicate the results from the investigator-sponsored studies or trials or if negative results are obtained, we would likely be further delayed or prevented from advancing further clinical development of our AXA Candidates. Further, if investigators or institutions breach their obligations with respect to the clinical development of our AXA Candidates or if the data proves to be inadequate compared to the first-hand knowledge we might have gained had the investigator-sponsored studies or trials been sponsored and conducted by us, then our ability to design and conduct any future Clinical Trials ourselves may be adversely affected.

Additionally, the FDA or ex-U.S. regulatory authorities may disagree with the sufficiency of our right of reference to the preclinical, manufacturing or clinical data generated by these investigator-sponsored studies or trials or our interpretation of preclinical, manufacturing or clinical data from these investigator-sponsored studies or trials. If so, the FDA or other ex-U.S. regulatory authorities may require us to obtain and submit additional preclinical, manufacturing or clinical data before we may initiate our planned Clinical Trials or may not accept such additional data as adequate to initiate our planned Clinical Trials. In addition, it could limit or prevent our ability to commercialize AXA Candidates for non-drug uses.

Obtaining and maintaining regulatory approval of our AXA Candidates that we decide to develop as drug product candidates for therapeutic indications or the ability to commercialize our AXA Candidates through a non-drug regulatory pathway in one jurisdiction does not mean that we will be successful in obtaining regulatory approval or identifying a similar alternate regulatory pathway for our AXA Candidates in other jurisdictions.

Obtaining and maintaining regulatory approval for AXA Candidates that we decide to develop as drug product candidates or identifying or commercializing our AXA Candidates through non-drug pathways in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval or identify and maintain an alternate regulatory pathway in any other jurisdiction, while a failure or delay in obtaining regulatory approval or an alternate regulatory pathway in one jurisdiction may have a negative effect on the regulatory approval process or path to market in others. For example, even if the FDA grants marketing approval of an AXA Candidate for therapeutic indications, comparable regulatory authorities in foreign jurisdictions could take opposing positions and decline to approve the manufacturing, marketing and promotion of such AXA Candidate in those countries. Approval procedures vary among jurisdictions and can involve

requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies, Non-IND, IRB-Approved Clinical Studies and Clinical Trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval and the approved price may not lead to profitability or acceptable margins.

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the United States may have requirements for approval of product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we fail to comply with the regulatory requirements in international markets or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our AXA Candidates will be harmed.

Preclinical and clinical development is uncertain. Our preclinical programs, Non-IND, IRB-Approved Clinical Studies and Clinical Trials may experience delays or may never advance to the next stage of development, which would adversely affect our ability to obtain regulatory approvals, where necessary, or identify and execute on alternate regulatory pathways to commercialize these programs on a timely basis or at all, which would have an adverse effect on our business.

Our AXA Candidates are in both preclinical and clinical (non-IND) stages of development, and their risk of failure is high. To proceed with our development plans and ultimately commercialization, we may need to conduct and meet regulatory requirements for preclinical, Non-IND, IRB-Approved Clinical Studies or, for AXA Candidates that we decide to develop as drug product candidates, Clinical Trials. For therapeutic applications, the FDA may require additional extensive preclinical and other studies. We cannot be certain of the timely completion or outcome of our preclinical testing and studies and cannot predict if the FDA or other regulatory authorities will accept our proposed clinical programs, including our Non-IND, IRB-Approved Clinical Studies and future Clinical Trials, if any, including their design, dose level, and dose regimen, or if the outcome of our preclinical testing, Non-IND, IRB-Approved Clinical Studies or Clinical Trials, if any, will ultimately support the subsequent development of our clinical programs for therapeutic indications or non-drug applications. As a result, we cannot be sure that we will be able to submit INDs or similar applications in the case of AXA Candidates for which we pursue a drug pathway or comply with any other regulatory requirements where necessary for commercialization and marketing of drugs or non-drug products on the timelines we expect, if at all. We cannot be sure that submission of INDs or similar applications, where necessary, or other regulatory required submissions for our AXA Candidates will result in the FDA or other regulatory authorities allowing our studies or Clinical Trials to begin, be completed or have their data used to support commercialization and required regulatory approvals. We also cannot be certain if our testing and studies will provide support for the further development of AXA Candidates as non-drug products or support for any associated product claims made, and, as a result, we cannot be sure that we will be able to successfully pursue alternative regulatory pathways to commercialization as non-drug product for some or all of our AXA Candidates.

If we are not able to meet certain regulatory requirements for our AXA Candidates or to obtain, or timely obtain, required regulatory approvals for our AXA Candidates that we choose to develop as drugs, we will not be able to commercialize or will be delayed in commercializing, our AXA Candidates, and our ability to generate revenue will be materially impaired.

Our AXA Candidates and the activities associated with their development and commercialization as a drug or non-drug products, including but not limited to their design, testing, manufacture, safety, efficacy, recordkeeping, packaging, labeling, storage, holding, approval, advertising, promotion, sale, distribution, import and export are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Before we can commercialize any of our AXA Candidates as a drug, we must obtain marketing approval. Before we can commercialize any of our AXA Candidates as a non-drug product, we may be required to follow pre- or post-market notification and other applicable regulatory requirements for ingredients and claims. We have not received approval to market any of our AXA Candidates as drugs from regulatory authorities in any jurisdiction nor executed on requirements for commercialization of non-drug products under applicable regulations, and it is possible that none of our current AXA Candidates, or any AXA Candidates we may seek to develop in the future, will ever obtain regulatory approval, where applicable, or meet other applicable regulatory requirements to reach the market. We, as a company, have no experience in filing and supporting the applications necessary to gain regulatory approvals for drugs or in the submission of other petitions, notifications or registrations in the case of non-drug products, where applicable, and expect to work with or rely on third-party CROs or regulatory consultants to assist us in this process. For example, the FDA and Federal Trade Commission, or FTC, require substantiating data or evidence for marketing claims and may require other regulatory submissions, including, for example, NDI submissions for certain product ingredients in certain non-drug products before they can be sold. With respect to AXA Candidates that we decide to develop as drug product candidates, securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the drug candidate's safety and efficacy. If we fail to execute competently on these requirements, as applicable, our AXA Candidates may not make it to market.

Securing regulatory approval for therapeutic indications also requires the submission of information about the drug manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our AXA Candidates that we decide to develop as drug product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining regulatory approvals for therapeutic indications, both in the United States and abroad, is expensive, may take many years if additional Clinical Trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the AXA Candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted IND, NDA or equivalent application types, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process of our AXA Candidates that we decide to commercialize as drugs and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. Our AXA Candidates that we decide to develop as drug product candidates could be

delayed in receiving, or fail to receive, regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design, including study population, dose level, dose regimen and bioanalytical assay methods, or implementation of our Clinical Trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a drug candidate is safe and effective for its proposed indication or a related companion diagnostic is suitable to identify appropriate patient populations;
- the results of our Non-IND, IRB-Approved Clinical Studies and Clinical Trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that an AXA Candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies, Non-IND, IRB-Approved Clinical Studies or Clinical Trials;
- the data collected from our Non-IND, IRB-Approved Clinical Studies and Clinical Trials of our product candidates may not be sufficient to support the submission of an NDA or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of drugs in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future Clinical Trial results may result in our failing to obtain regulatory approval to market our AXA Candidates, which would significantly harm our business, results of operations and prospects.

If we decide to develop an AXA Candidate as a drug product candidate and submit an NDA for such AXA Candidate, the FDA may also require a panel of experts, or an Advisory Committee, to deliberate on the adequacy of the safety and efficacy data to support approval for therapeutic indications. The opinion of the Advisory Committee, although not binding, may have a significant impact on our ability to obtain approval of any AXA Candidates that we decide to develop as drugs based on the completed Clinical Trials.

In addition, even if we were to obtain approval for use of our AXA Candidates as drug product candidates, regulatory authorities may approve any of our AXA Candidates for fewer or more limited therapeutic indications than we request, may include limitations for use or contraindications that limit the suitable patient population, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing Clinical Trials or may approve an AXA Candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that drug product candidate. Similarly, regulatory authorities may limit or prohibit label claims that limit the market, price or other factors that are necessary or desirable for the successful commercialization of candidates developed as non-drug products. Any of the foregoing scenarios could materially harm the commercial prospects for our AXA Candidates.

If we experience delays or failures in obtaining regulatory approvals, where applicable, or otherwise experience delays or failures in complying with regulatory requirements for commercialization of our product candidates, the commercial prospects for our AXA Candidates may be harmed and our ability to generate revenues will be materially impaired.

The FDA and other regulatory authorities such as the EMA may implement additional regulations or restrictions on the development and commercialization of products that act on metabolic pathways, which may be difficult to predict.

The FDA and foreign regulatory authorities such as the EMA have expressed interest in further regulating biotechnology products and product candidates, such as AXA Candidates. Agencies at both the federal and state level in the United States, as well as the U.S. Congressional committees and other governments or governing agencies, have also expressed interest in further regulating the biotechnology industry. Such action may delay or prevent commercialization of some or all of our AXA Candidates. Adverse developments in Non-IND, IRB-Approved Clinical Studies or Clinical Trials of AXA Candidates or similar products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our AXA Candidates. The FDA or other regulatory authorities may impose unexpected, onerous requirements on our products because they are composed of multiple amino acids, requiring a clinical demonstration of the functionality and contribution of each component of our EMMs. Such requirements may include additional studies or analyses. Similarly, the EMA governs the development of AXA Candidates as drugs in the European Union and member state regulatory bodies govern the development of AXA Candidates under non-drug regulations and may issue new guidelines concerning the development and marketing authorization for AXA Candidates and require that we comply with these new guidelines. These regulatory review agencies and committees and the new requirements or guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our AXA Candidates or lead to significant limitations or restrictions. As we advance our AXA Candidates, we will be required to consult with these regulatory agencies and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of such AXA Candidates. These additional processes may, for our AXA Candidates that we decide to develop as drug product candidates, result in a review and approval process that is longer than we otherwise would have expected and delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our AXA Candidates can be costly and could negatively impact our ability to complete Clinical Trials and commercialize our current and future AXA Candidates in a timely manner, if at all.

We may fail to obtain and maintain orphan drug designations from the FDA or, if applicable, other foreign regulatory authorities such as the EMA for AXA Candidates that we decide to develop as drug product candidates.

Our strategy includes filing for orphan drug designation where available for our AXA Candidates we decide to develop as drug product candidates. In the United States, under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug or biologic intended to treat a rare disease or condition, which is defined as one occurring in a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug or biologic will be recovered from sales in the United States. In the United States, orphan drug designation entitles a party to financial incentives, such as opportunities for grant funding toward clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the

product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including an NDA, to market the same drug or biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the original manufacturer is unable to assure sufficient product quantity.

In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the product to meet the needs of patients with the orphan-designated disease or condition. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties may receive and be approved for the same condition, and only the first applicant to receive approval will receive the benefits of marketing exclusivity. Even after an orphan-designated product is approved, the FDA can subsequently approve a later drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior if it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process. In addition, while we may seek orphan drug designation for our AXA Candidates that we decide to develop as drug product candidates, we may never receive such designations.

In the European Union, the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In the European Union, orphan drug designation entitles a party to financial incentives such as reductions of fees or fee waivers. In addition, ten years of market exclusivity is granted following drug product approval, meaning that another application for marketing authorization of a later similar medicinal product for the same indication will generally not be approved in the European Union. This period may be reduced to six years if the orphan designation criteria are no longer met, including where it is shown that the product is not sufficiently profitable to justify maintenance of market exclusivity.

Even if we receive regulatory approval of any AXA Candidates as drugs, or commercialize our AXA Candidates as non-drug products, we will be subject to ongoing regulatory compliance obligations or continued regulatory review, which may result in significant additional expense. Additionally, any of our AXA Candidates, if approved or commercialized, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our AXA Candidates.

If any of our AXA Candidates are developed as drug product candidates and approved for therapeutic indications or are commercialized as non-drug products, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, distribution, quality, safety, sale, marketing, advertising, promotion, sampling, record-keeping, export, import, conduct of post-marketing studies and submission of safety, efficacy or other post-market information. Such

requirements may be imposed as federal and state requirements in the United States or by comparable foreign regulatory authorities. In addition, we will be subject to continued compliance with cGMP requirements as applicable to drugs and non-drug products and GCP requirements for any Clinical Trials that we conduct post-approval, if applicable.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA, and comparable foreign regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to the respective cGMP regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA, if applicable, or other marketing application or submission, and previous responses to inspection observations. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

The FDA has significant post-marketing authority, including, for example, the authority to require labeling or packaging changes based on the use of improper product claims or new safety or other information and, where applicable, to require post-marketing studies or clinical trials to evaluate serious safety risks related to the use of a drug. With respect to products developed as drugs, any regulatory approvals that we receive for our AXA Candidates may be subject to limitations on the approved indicated uses for which a drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase IV clinical trials and surveillance to monitor the safety and efficacy of the AXA Candidate. The FDA may also require a Risk Evaluation and Mitigation Strategy, or REMS, program as a condition of approval of our AXA Candidates that we decide to develop as drugs, which could entail requirements for long-term patient follow-up, a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or a comparable foreign regulatory authority approves our AXA Candidates as a drug for therapeutic uses, we will have to comply with requirements including submissions of safety and other post-marketing information and reports and registration.

The FDA or other regulatory authorities may take regulatory enforcement action or other legal action or, in the case of drugs, impose consent decrees or withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with our AXA Candidates, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in potential consequences, including, among other things:

- in the case of AXA Candidates that we decide to develop as drugs, revisions to the approved labeling to add new safety information and required regulatory submissions; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program;
- restrictions on the marketing or manufacturing of our products, withdrawal of the product from the market or voluntary or mandatory product recalls;
- re-labeling or re-packaging;
- fines, warning or untitled enforcement letters or holds on clinical trials;
- in the case of drugs, refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;

- product seizure or detention or refusal to permit the import or export of our AXA Candidates; and
- injunctions or the imposition of civil or criminal penalties.

The FDA and FTC strictly regulate marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses for drugs, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. Non-drug products are prohibited from making any claims, whether express or implied, that the product is intended to "diagnose, mitigate, treat, cure or prevent disease," and doing so may subject a non-drug product to classification as a drug product and regulatory enforcement action. If the FDA or other regulatory agency determines that any of our AXA Candidates make impermissible claims, we may be subject to any of the aforementioned consequences or other legal challenges that may have an adverse effect on the company's business and operations.

The policies of the FDA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval, where applicable, and commercialization, and continued commercialization, of our AXA Candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained for any drugs, or may no longer be able to market or sell products we develop as non-drug products, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the current administration may impact our business and industry. Namely, the current administration has taken several executive actions, including the issuance of a number of executive orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities, such as implementing statutes through rulemaking, issuance of guidance and review and approval of marketing applications. It is difficult to predict how these executive actions, including any executive orders, will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. In addition, if we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, where applicable, our ability to continue to market and sell our products and we may not achieve or sustain profitability.

Non-compliance by us or any future collaborator with regulatory requirements, including safety monitoring or pharmacovigilance requirements, where applicable, can also result in significant financial penalties.

Healthcare insurance coverage and reimbursement may be limited or unavailable in certain market segments for our AXA Candidates, if developed as a drug and approved, which could make it difficult for us to sell any product profitably.

The success of our AXA Candidates, if approved for therapeutic indications, depends on the availability of adequate coverage and reimbursement from third-party payors, including governmental healthcare programs, such as Medicare and Medicaid, commercial payors and health maintenance organizations. In addition, because our AXA Candidates have the potential to

represent a relatively new approach to the treatment of the diseases, we cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, our AXA Candidates or assure that coverage and reimbursement will be available for any product that we may develop.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs and commercial payors are critical to new product acceptance.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs and treatments they will cover and the amount of reimbursement. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of product candidates. Patients are unlikely to use our AXA Candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our AXA Candidates. Because our AXA Candidates may have a higher cost of goods than conventional therapies, and may require long-term follow-up evaluations, the risk that coverage and reimbursement rates may be inadequate for us to achieve profitability may be greater. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our AXA Candidates.

The pricing of prescription pharmaceuticals is also subject to governmental control outside the United States. In these other countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our product candidates to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our ability to generate revenues and become profitable could be impaired.

Healthcare insurance often does not cover non-drug products administered outside of the hospital setting. This may impact our AXA Candidates if we decide to commercialize them as non-drug products.

For those AXA Candidates that we decide to develop as drug product candidates, our relationships with healthcare providers, physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, or FCA, which may constrain the business or financial arrangements and relationships through which such companies sell, market and distribute pharmaceutical products. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for Clinical Trials. The applicable federal and state healthcare laws and regulations laws that may affect our ability to operate include, but are not limited to:

- The federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity can be found guilty of violating the statute without actual knowledge of the statute or specific intent to violate it. In addition, a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution.

- Federal civil and criminal false claims laws and civil monetary penalty laws, including the FCA, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, false or fraudulent claims for payment to, or approval by Medicare, Medicaid or other federal healthcare programs, knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim or an obligation to pay or transmit money to the federal government, or knowingly concealing or knowingly and improperly avoiding or decreasing or concealing an obligation to pay money to the federal government. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims. The FCA also permits a private individual acting as a "whistleblower" to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery.

- HIPAA created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or

property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it.

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose, among other things, requirements on certain covered healthcare providers, health plans and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.

- The federal Physician Payment Sunshine Act, created under the Patient Protection and Affordable Care Act, or the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the United States Department of Health and Human Services information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

- Federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

- Analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and may be broader in scope than their federal equivalents; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of

interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention from the business.

The failure to comply with any of these laws or regulatory requirements subjects entities to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in significant civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, exclusion from participation in federal and state funded healthcare programs (such as Medicare and Medicaid), contractual damages and the curtailment or restructuring of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Any action for violation of these laws, even if successfully defended, could cause a pharmaceutical manufacturer to incur significant legal expenses and divert management's attention from the operation of the business. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, that person or entity may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Prohibitions or restrictions on sales or withdrawal of future marketed products could materially affect business in an adverse way.

Effective upon the completion of this offering, we will adopt a code of business conduct and ethics, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our AXA Candidates outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

Failure to comply with health and data protection laws and regulations could lead to government enforcement actions (which could include civil or criminal penalties), private litigation or adverse publicity and could negatively affect our operating results and business.

We and any potential collaborators may be subject to federal, state and foreign data protection laws and regulations (i.e., laws and regulations that address privacy and data security). In the United States, numerous federal and state laws and regulations, including federal health information privacy laws, state data breach notification laws, state health information privacy laws and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure and protection of health-related and other personal information could apply to our operations or the operations of our collaborators. In addition, we

may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA, as amended by HITECH. Depending on the facts and circumstances, we could be subject to civil, criminal and administrative penalties if we knowingly obtain, use or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

Compliance with U.S. and international data protection laws and regulations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use and disclose data or, in some cases, impact our ability to operate in certain jurisdictions. Failure to comply with these laws and regulations could result in government enforcement actions (which could include civil, criminal and administrative penalties), private litigation or adverse publicity and could negatively affect our operating results and business. Moreover, clinical trial subjects, employees and other individuals about whom we or our potential collaborators obtain personal information, as well as the providers who share this information with us, may limit our ability to collect, use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

European data collection is governed by restrictive regulations governing the use, processing and cross-border transfer of personal information.

In the event we decide to conduct Clinical Trials in the European Union, we may be subject to additional privacy restrictions. The collection and use of personal health data in the European Union is governed by the provisions of the GDPR. This directive imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The GDPR also imposes strict rules on the transfer of personal data out of the European Union to the United States. Failure to comply with the requirements of the Data Protection Directive, the GDPR, and the related national data protection laws of the European Union Member States may result in fines and other administrative penalties. The GDPR introduces new data protection requirements in the European Union and substantial fines for breaches of the data protection rules. The GDPR regulations may impose additional responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with these or new data protection rules. This may be onerous and adversely affect our business, financial condition, prospects and results of operations.

Ongoing healthcare legislative and regulatory reform measures may have a material adverse effect on our business and results of operations.

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of our AXA Candidates or any future AXA Candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product for which we obtain marketing approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the ACA was passed, which substantially changes the way healthcare is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The ACA, among other things, subjects biological products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, and creates a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% (as of 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Since January 2017, the Trump administration has signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. One Executive Order directs federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers or manufacturers of pharmaceuticals or medical devices. The second Executive Order terminates the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12 billion in ACA risk corridor payments to third-party payors who argued were owed to them. The effects of this gap in reimbursement on third-party payors, the viability of the ACA marketplace, providers and potentially our business, are not yet known.

Since its enactment, some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to judicial, congressional or executive challenges. As a result, there have been delays in the implementation of, and action taken to repeal or replace, certain aspects of the ACA. The U.S. Supreme Court has upheld certain key aspects of the legislation, including a tax-based shared responsibility payment imposed on certain individuals who fail to maintain qualifying health coverage for all or part of a year or pay a penalty, which is commonly known as the "individual mandate." However, as a result of tax reform legislation passed in December 2017, the individual mandate has been eliminated effective January 1, 2019. On January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018, or the BBA, among other things, amended the ACA to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole."

In July 2018, the Centers for Medicare and Medicaid Services, or CMS, published a final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. In addition, CMS has recently published a final rule that would give states greater flexibility, starting in 2020, in setting benchmarks for insurers in the individual and small group marketplaces, which may

have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or the Texas District Court Judge, ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Cuts and Jobs Act of 2017, the remaining provisions of the ACA are invalid as well. While the Trump Administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal, it is unclear how this decision, subsequent appeals and other efforts to repeal and replace the ACA Act will impact the ACA and our business.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction, which triggered the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of, on average, two percent per fiscal year through 2025 unless Congress takes additional action. These reductions were extended through 2027 under the BBA. In January 2013, the American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Additionally, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The U.S. Department of Health and Human Services has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example, in September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. Although a number of these and other proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that the healthcare reform measures that have been adopted and may be adopted in the future may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product and could seriously harm our future revenues. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private third-party payors.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our product. Such reforms could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain regulatory approval and may affect our overall financial condition and ability to develop product candidates.

European Union drug marketing and reimbursement regulations may materially affect our ability to market and receive coverage for any AXA Candidate that we decide to develop as a drug candidate in the European member states.

We intend to seek approval to market our AXA Candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our AXA Candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the European Union, the pricing of pharmaceutical products is subject to governmental control and other market regulations which could put pressure on the pricing and usage of our AXA Candidates. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval of an AXA Candidate. In addition, market acceptance and sales of our AXA Candidates will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for our AXA Candidates and may be affected by existing and future healthcare reform measures.

Much like the federal Anti-Kickback Statute prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the European Union. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

In addition, in most foreign countries, including the European Economic Area, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Reference pricing used by various European Union Member States and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company

placing the medicinal product on the market. In some countries, we may be required to conduct a Clinical Trial or other studies that compare the cost-effectiveness of any of our AXA Candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the United States and generally prices tend to be significantly lower. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales by us or our strategic partners and the potential profitability of any of our product candidates in those countries would be negatively affected.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain products outside of the United States and require us to develop and implement costly compliance programs.

If we expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The FCPA prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain ex-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions and other trade laws and regulations. We can face serious consequences for violations.

Among other matters, U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions and other trade laws and regulations, which are collectively referred to as Trade Laws, prohibit companies and their employees, agents, clinical research organizations, legal counsel, accountants, consultants, contractors and other partners from authorizing, promising, offering, providing, soliciting or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of Trade Laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. We also expect our ex-U.S. activities to increase in time. We plan to engage third parties for Clinical Trials or to obtain necessary permits, licenses, patent registrations and other regulatory approvals and we can be held liable for the corrupt or other illegal activities of our personnel, agents or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

Changes in funding for the FDA, the SEC and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products or take action with respect to other regulatory matters can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept payment of user fees and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed or approved, or for other actions to be taken, by relevant government agencies, which would adversely affect our business. For example, over the last several years, including beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Similarly, a prolonged government shutdown could prevent the timely review of our patent applications by the United States Patent and Trademark Office, or USPTO, which could delay the issuance of any U.S. patents to which we might otherwise be entitled. Further, upon completion of this offering and in our operations as a public company, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly

Risks related to our intellectual property

If we are unable to obtain and maintain patent protection for any AXA Candidates we develop or for our AXA Development Platform, our competitors could develop and commercialize products or technology similar or identical to ours, and our ability to successfully commercialize any AXA Candidates we may develop, and our technology may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our AXA Candidates, AXA Development Platform and other technologies we may develop. We seek to protect our proprietary position by filing patent applications in the United States and abroad relating to our AXA Candidates and AXA Development Platform, as well as other technologies that are important to our business. Given that the development of our technology and AXA Candidates is at an early stage, our intellectual property portfolio with respect to certain aspects of our technology and AXA Candidates is also at an early stage. We have filed or intend to file patent applications on these aspects of our technology and our AXA Candidates; however, there can be no assurance that any such patent applications will issue as granted patents. Furthermore, in some cases, we have only filed provisional patent applications on certain aspects of our technology and AXA Candidates and each of these provisional patent applications is not eligible to become an issued patent until, among other things, we file a non-provisional patent application within 12 months of the filing date of the applicable provisional patent application. Any failure to file a non-provisional patent application within this timeline could cause us to lose the ability to obtain patent protection for the inventions disclosed in the associated provisional patent applications.

Composition of matter patents for biological and pharmaceutical products are generally considered to be the strongest form of intellectual property protection for those types of products, as such patents provide protection without regard to any method of use. We cannot be certain, however, that the claims in our pending patent applications covering the composition of matter of our AXA Candidates will be considered patentable by the USPTO or by patent offices in foreign countries, or that the claims in any of our issued patents will be considered valid and enforceable by courts in the United States or foreign countries. Furthermore, in some cases, we may not be able to obtain issued claims covering compositions of matter relating to our AXA Candidates and proprietary product platform, as well as other technologies that are important to our business, and instead may need to rely on filing patent applications with claims covering a method of use or method of manufacture. Method of use patents protect the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their products for our targeted indications, physicians may prescribe these products "off-label" for those uses that are covered by our method of use patents. Although off-label prescriptions may infringe or contribute to the infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute. There can be no assurance that any such patent applications will issue as granted patents, and even if they do issue, such patent claims may be insufficient to prevent third parties, such as our competitors, from utilizing our technology. Any failure to obtain or maintain patent protection with respect to our AXA Candidates and AXA Development Platform could have a material adverse effect on our business, financial condition, results of operations and prospects.

If any of our owned patent applications do not issue as patents in any jurisdiction, we may not be able to compete effectively.

Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our inventions, obtain, maintain and enforce our

intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned patents. With respect to our patent portfolio, as of April 1, 2019, our AXA Candidate-related patent portfolio consists of 18 patent families, including two granted U.S. patents, 22 U.S. pending patent applications (including provisional applications) and 13 owned pending patent applications in jurisdictions outside of the United States (including Patent Cooperation Treaty applications) that, in many cases, are counterparts to the foregoing U.S. patents and patent applications, which include claims directed to compositions, methods of use, treatment of indications, dosing, formulations and methods of manufacturing. With respect to owned intellectual property, we cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors or other third parties.

The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patents and patent applications at a reasonable cost or in a timely manner. Disruptions at the USPTO or other government agencies may also slow the time necessary for patent applications to be reviewed by the USPTO, which could adversely affect our patent portfolio. It is also possible that we will fail to identify patentable aspects of our research and development output in time to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. In addition, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our inventions and the prior art allow our inventions to be patentable over the prior art. Furthermore, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in any of our owned or pending patent applications, or that we were the first to file for patent protection of such inventions.

If the scope of any patent protection we obtain is not sufficiently broad, or if we lose any of our patent protection, our ability to prevent our competitors from commercializing similar or identical technology and AXA Candidates would be adversely affected.

The patent position of healthcare companies generally is highly uncertain, involves complex legal and factual questions and has been the subject of much litigation in recent years. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our AXA Candidates, AXA Development Platform or other technologies or which effectively prevent others from commercializing competitive technologies and AXA Candidates.

No consistent policy regarding the scope of claims allowable in patents in the biotechnology field has emerged in the United States. The patent situation outside of the United States is similarly uncertain. Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our inventions and enforce our intellectual property rights, and more generally could affect the value of our intellectual property. In particular, our ability to stop third parties from making, using, selling, offering to sell or importing products that infringe our intellectual property will depend in part on our success in obtaining and enforcing patent claims that cover our technology, inventions and improvements. With respect to intellectual property that we own, we cannot be sure that patents will be granted with respect to any of our pending patent

applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our products and the methods used to manufacture those products. Moreover, even our issued patents do not guarantee us the right to practice our technology in relation to the commercialization of our products. The area of patent and other intellectual property rights in biotechnology is an evolving one with many risks and uncertainties, and third parties may have blocking patents that could be used to prevent us from commercializing our patented AXA Candidates and practicing our proprietary technology. Our issued patents and those that may issue in the future may be challenged, invalidated or circumvented, which could limit our ability to stop competitors from marketing related products or limit the length of the term of patent protection that we may have for our AXA Candidates. In addition, the rights granted under any issued patents may not provide us with protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may independently develop similar technologies. For these reasons, we may have competition for our AXA Candidates. Moreover, because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that, before any particular AXA Candidate can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby reducing any advantage of the patent.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we own issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we own may be challenged, narrowed, circumvented or invalidated by third parties. Consequently, we do not know whether our AXA Candidates or other technologies will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and patents that we own may be challenged in the courts or patent offices in the United States and abroad. We may be subject to a third party preissuance submission of prior art to the USPTO or to foreign patent authorities or become involved in opposition, derivation, revocation, reexamination, post-grant and *inter partes* review or interference proceedings or other similar proceedings challenging our owned patent rights. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate or render unenforceable, our owned patent rights, allow third parties to commercialize our AXA Candidates, AXA Development Platform or other technologies and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Moreover, we may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as *inter partes* reviews, post-grant reviews or derivation proceedings at the USPTO or oppositions in a foreign patent office, that challenge our priority of invention or other features of patentability with respect to our owned patents and patent applications. Such challenges may result in loss of patent rights, loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our AXA Candidates, AXA Development Platform and other technologies. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us.

In addition, given the amount of time required for the development, testing and regulatory review of new AXA Candidates, patents protecting such AXA Candidates might expire before or shortly after such AXA Candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

We may in the future co-own patent rights relating to future AXA Candidates and our AXA Development Platform with third parties. We may need the cooperation of any such co-owners of our patent rights in order to enforce such patent rights against third parties, and such cooperation may not be provided to us. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

Our rights to develop and commercialize our AXA Candidates and AXA Development Platform may be subject, in part, to the terms and conditions of future licenses granted to us by others.

We may rely upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our AXA Candidates and AXA Development Platform. Patent rights that we in-license in the future may be subject to a reservation of rights by one or more third parties. As a result, any such third parties may have certain rights to such intellectual property.

In addition, subject to the terms of any such license agreements, we may not have the right to control the preparation, filing, prosecution and maintenance, and we may not have the right to control the enforcement, and defense of patents and patent applications covering the technology that we license from third parties. We cannot be certain that any in-licensed patent applications (and any patents issuing therefrom) that are controlled by any potential licensors will be prepared, filed, prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to prosecute, maintain, enforce and defend such patent rights, or lose rights to those patent applications (or any patents issuing therefrom), the rights we have licensed may be reduced or eliminated, our right to develop and commercialize any of our AXA Candidates and AXA Development Platform technologies that are subject of such licensed rights could be adversely affected and we may not be able to prevent competitors from making, using and selling competing products. Moreover, we cannot be certain that such activities by our potential future licensors will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents or other intellectual property rights. In addition, even where we may have the right to control patent prosecution of patents and patent applications that we may license to and from third parties, we may still be adversely affected or prejudiced by actions or inactions of our potential future licensees, licensors and their counsel that took place prior to the date of assumption of control over patent prosecution.

We may not be able to protect our intellectual property and proprietary rights throughout the world.

Filing, prosecuting and defending patents on our AXA Candidates, AXA Development Platform technologies and other technologies in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may export otherwise infringing products to territories where we have patent protection but enforcement is not as strong as that in the United States. These products may compete with our products, and our patents or other intellectual property rights may not be

effective or sufficient to prevent them from competing. Furthermore, the amino acids that we expect to incorporate into our products are available for purchase separately from a variety of retail outlets, and our intellectual property rights will not prevent these sales from continuing in the future.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Obtaining and maintaining our patent protection depends on compliance with various procedural requirements, document submission, fee payment and other requirements imposed by government patent agencies and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our owned patents and applications. The USPTO and various ex-U.S. government agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. In some cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in a partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market with similar or identical products or technology, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act, or the America

Invents Act, enacted in September 2011, the United States transitioned to a first-inventor-to-file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before we do could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we were the first to file any patent application related to our AXA Candidates, AXA Development Platform or other technologies.

The America Invents Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, *inter partes* review and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned patent applications and the enforcement or defense of our owned issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

From time-to-time the U.S. Supreme Court, other federal courts, the U.S. Congress or the USPTO, may change the standards of patentability and any such changes could have a negative impact on our business. For instance, on June 13, 2013, in *Association for Molecular Pathology v. Myriad Genetics*, the Supreme Court held that a naturally occurring DNA segment is a product of nature and not patent eligible merely because it has been isolated. The Supreme Court did not address the patentability of any innovative method claims involving the manipulation of isolated genes. On January 7, 2019, the USPTO released guidance entitled "2019 Revised Subject Matter Eligibility Guidance." This memorandum provides guidelines for the USPTO's new examination procedure for subject matter eligibility under 35 U.S.C. §101 for claims embracing natural products or natural principles. Although the guidelines do not have the force of law, patent examiners have been instructed to follow them. Some aspects of our technology involve processes or molecules that may be subject to this evolving standard and we cannot guarantee that any of our pending process claims will be patent eligible, or issued claims will remain patent eligible, as a result of such evolving standards. Changes in either the patent laws or in interpretations of patent laws in the United States or other countries could weaken our ability to obtain new patents or to enforce

our existing patents and patents that we might obtain in the future. We cannot predict the breadth of claims that may be allowed or enforced in our patents or in third-party patents. We may not develop additional proprietary products, methods and technologies that are patentable.

Issued patents covering our AXA Candidates and any patents that may issue covering our AXA Development Platform and other technologies, could be found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad.

If we initiated legal proceedings against a third party to enforce a patent covering our AXA Candidates, AXA Development Platform or other technologies, the defendant could counterclaim that such patent is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may raise claims challenging the validity or enforceability of our owned patents before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in the revocation of, cancellation of, or amendment to our patents in such a way that they no longer cover our AXA Candidates, AXA Development Platform or other technologies. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our AXA Candidates, AXA Development Platform or other technologies. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations and prospects.

If we do not obtain patent term extension and/or data exclusivity for any AXA Candidates we decide to develop as drug product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any AXA Candidates we decide to develop as drug product candidates, one or more of our owned U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act, also known as the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent term extension of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. Similar extensions as compensation for patent term lost during regulatory review processes are also available in certain foreign countries and territories, such as in Europe under a Supplementary Protection Certificate. However, we may not be granted an extension in the United States and/or foreign countries and territories because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is shorter than what we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations and prospects could be materially harmed.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an interest in our owned patent rights, trade secrets or other intellectual property as an inventor or co-inventor. For example, we may have disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our AXA Candidates, AXA Development Platform or other technologies. Litigation may be necessary to defend against these and other claims challenging inventorship or our ownership of our owned patent rights, trade secrets or other intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our AXA Candidates, AXA Development Platform and other technologies. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for our AXA Candidates, AXA Development Platform and other technologies, we also rely on trade secrets and confidentiality agreements to protect our unpatented know-how, technology and other proprietary information and to maintain our competitive position. Trade secrets and know-how can be difficult to protect. We expect our trade secrets and know-how to over time be disseminated within the industry through independent development, the publication of journal articles describing the methodology and the movement of personnel from academic to industry scientific positions.

We currently, and may in the future continue to, rely on third parties to assist us in developing and manufacturing our AXA Candidates. Accordingly, we must, at times, share know-how and trade secrets, including those related to our AXA Development Platform, with them. We may in the future also enter into research and development collaborations with third parties that may require us to share know-how and trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our know-how, trade secrets and other proprietary technology, in part, by entering into non-disclosure and confidentiality agreements, and including in our vendor and service agreements terms protecting our confidential information, know-how and trade secrets, with parties who have access to such information, such as our employees, scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants as well as train our employees not to bring or use proprietary information or technology from former employers to us or in their work, and we remind former employees when they leave their employment of their confidentiality obligations. However, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and other confidential information by maintaining physical security of our premises and physical and electronic security of our information technology systems.

Despite our efforts, any of the aforementioned parties may breach the agreements and disclose our proprietary information, including our trade secrets, or there may be lapses or failures in our physical and electronic security systems that lead to our proprietary information being disclosed, and we may not be able to obtain adequate remedies in the event of any such breaches. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of our scientific advisors,

employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a result. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators or others is inadvertently disclosed or subject to a breach or violation, we may be exposed to liability to the owner of that confidential information. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third party, our competitive position would be materially and adversely harmed.

We rely on our AXA Development Platform to identify AXA Candidates. Our competitive position could be materially harmed if our competitors develop a similar platform and develop rival product candidates.

We rely on know-how, inventions and other proprietary information to strengthen our competitive position. We consider know-how to be our primary intellectual property with respect to our AXA Development Platform. Our Non-IND, IRB-Approved Clinical Studies allow us to collect clinical data, which we use in a feedback loop to make improvements to our AXA Development Platform. In particular, we anticipate that, with respect to this platform, this data may over time be disseminated within the industry through independent development, the publication of journal articles describing the method and the movement of skilled personnel.

We cannot rule out that our competitors may have or will obtain the knowledge necessary to analyze and characterize similar data to our known data for the purpose of identifying and developing products that could compete with any of our AXA Candidates. Our competitors may also have significantly greater financial, product development, technical and human resources and access to data. Further, our competitors may have significantly greater experience in using translational science methods to identify and develop product candidates.

We may not be able to prohibit our competitors from using technology or methods that are the same as or similar to our AXA Development Platform to develop their own product candidates. If our competitors develop associated therapies, our ability to develop and market a promising product or product candidate may diminish substantially, which could have a material adverse effect on our business, financial condition, prospects and results of operations.

We may not be successful in obtaining, through acquisitions, in-licenses or otherwise, necessary rights to our AXA Candidates, AXA Development Platform technologies or other technologies.

We may need to, or want to for strategic purposes, acquire rights to certain intellectual property, through licenses from third parties, to create new products or advancements to our AXA Development Platform or further develop our AXA Candidates and AXA Development Platform technologies. Some healthcare companies and academic institutions are competing with us in the field of EMMs and metabolic pathways and may have patents and have filed and are likely filing patent applications potentially relevant to our business. In order to avoid infringing these third-party patents, we may find it necessary or prudent to obtain licenses to such patents from such third party intellectual property holders. We may also require licenses from third parties for certain technologies that we may evaluate for use with our current or future AXA Candidates. However, we may be unable to secure such licenses or otherwise acquire or in-license any compositions,

methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our current or future AXA Candidates and our AXA Development Platform at a reasonable cost or on reasonable terms, if at all. The licensing or acquisition of third party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all.

In the event that we try to obtain rights to required third party intellectual property rights, and are ultimately unsuccessful, we may be required to expend significant time and resources to redesign our technology, AXA Candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected AXA Candidates or continue to utilize our existing AXA Development Platform technology, which could harm our business, financial condition, results of operations and prospects significantly.

We may be subject to claims that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants and advisors are currently or were previously employed at universities or other healthcare companies, including our competitors and potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these individuals have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations and prospects.

Third-party claims of intellectual property infringement, misappropriation or other violation against us or our collaborators may prevent or delay the development and commercialization of our AXA Candidates, AXA Development Platform and other technologies.

The field of developing drug or non-drug products that target metabolic pathways is competitive and dynamic. Due to the focused research and development that is taking place by several companies, including us and our competitors, in this field, the intellectual property landscape is in flux, and it may remain uncertain in the future. As such, there may be significant intellectual property related litigation and proceedings relating to our owned, and other third party, intellectual property and proprietary rights in the future.

Our commercial success depends in part on our and our collaborators' ability to avoid infringing, misappropriating and otherwise violating the patents and other intellectual property rights of third parties. There is a substantial amount of complex litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. As discussed above, recently, due to changes in U.S. law referred to as patent reform, new procedures including *inter partes* review and post-grant review have been implemented. As stated above, this reform adds uncertainty to the possibility of challenge to our patents in the future.

Numerous U.S. and foreign issued patents and pending patent applications owned by third parties exist relating to technologies and fields in which we are developing our AXA Candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our AXA Candidates, AXA Development Platform and other technologies may give rise to claims of infringement of the patent rights of others. We cannot assure you that our AXA Candidates, proprietary product platform technologies and other technologies that we have developed, are developing or may develop in the future will not infringe existing or future patents owned by third parties. We may not be aware of patents that have already been issued and that a third party, for example, a competitor in the fields in which we are developing our AXA Candidates, AXA Development Platform and other technologies might assert are infringed by our current or future AXA Candidates, AXA Development Platform or other technologies, including claims to compositions, formulations, methods of manufacture or methods of use or treatment that cover our AXA Candidates, AXA Development Platform or other technologies. It is also possible that patents owned by third parties of which we are aware, but which we do not believe are relevant to our AXA Candidates, AXA Development Platform or other technologies, could be found to be infringed by our AXA Candidates, AXA Development Platform or other technologies. In addition, because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our AXA Candidates, AXA Development Platform or other technologies may infringe. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, including our AXA Development Platform technologies, manufacturing methods, AXA Candidates or future methods or products resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

Third parties may have patents or obtain patents in the future and claim that the manufacture, use or sale of our AXA Candidates, AXA Development Platform or other technologies infringes upon these patents. In the event that any third party claims that we infringe their patents or that we are otherwise employing their proprietary technology without authorization and initiates litigation against us, even if we believe such claims are without merit, a court of competent jurisdiction could hold that such patents are valid, enforceable and infringed by our AXA Candidates, AXA Development Platform or other technologies. In this case, the holders of such patents may be able to block our ability to commercialize the applicable AXA Candidate or technology unless we obtain a license under the applicable patents, or until such patents expire or are finally determined to be held invalid or unenforceable. Such a license may not be available on commercially reasonable terms or at all. Even if we are able to obtain a license, the license would likely obligate us to pay license fees or royalties or both, and the rights granted to us might be non-exclusive, which could result in our competitors gaining access to the same intellectual property. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our AXA Candidates, AXA Development Platform or other technologies, or such

commercialization efforts may be significantly delayed, which could in turn significantly harm our business.

Defense of infringement claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of management and other employee resources from our business, and may impact our reputation. In the event of a successful claim of infringement against us, we may be enjoined from further developing or commercializing our infringing AXA Candidates, AXA Development Platform or other technologies. In addition, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties and/or redesign our infringing AXA Candidates or technologies, which may be impossible or require substantial time and monetary expenditure. In that event, we would be unable to further develop and commercialize our AXA Candidates, AXA Development Platform or other technologies, which could harm our business significantly.

Engaging in litigation to defend against third parties alleging that we have infringed, misappropriated or otherwise violated their patents or other intellectual property rights is very expensive, particularly for a company of our size, and time-consuming. Some of our competitors may be able to sustain the costs of litigation or administrative proceedings more effectively than we can because of greater financial resources. Patent litigation and other proceedings may also absorb significant management time. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings against us could impair our ability to compete in the marketplace. The occurrence of any of the foregoing could have a material adverse effect on our business, financial condition or results of operations.

We may become involved in lawsuits to protect or enforce our patents and other intellectual property rights, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents, or we may be required to defend against claims of infringement. In addition, our patents also may become involved in inventorship, priority or validity disputes. To counter or defend against such claims can be expensive and time-consuming. In an infringement proceeding, a court may decide that a patent owned by us is invalid or unenforceable, the other party's use of our patented technology falls under the safe harbor to patent infringement under 35 U.S.C. §27(I)(1) or may refuse to stop the other party from using the technology at issue on the grounds that our owned patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our owned patents at risk of being invalidated or interpreted narrowly. Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties

resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our business, financial condition, results of operations and prospects.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make products that are similar to our AXA Candidates or utilize similar technology but that are not covered by the claims of the patents that we may own;
- we, or our future licensors or collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we own now or in the future;
- we, or our future licensors or collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned intellectual property rights;
- it is possible that our current or future pending owned patent applications will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors or other third parties;
- our competitors or other third parties might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;

- the patents of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks related to our reliance on third parties

Third-party relationships are important to our business. If we are unable to enter into and maintain strategic collaborations or if these relationships are not successful, our business could be adversely affected.

We have limited capabilities for product development and do not yet have any capability for sales, marketing or distribution. Accordingly, we may need to enter into relationships with other companies to provide us with important technologies, and we may receive additional technologies and funding under these and other collaborations in the future. Relationships we enter into may pose a number of risks, including the following:

- third parties have, and future third-party collaborators may have, significant discretion in determining the efforts and resources that they will apply;
- third parties may not perform their obligations as expected;
- third parties may not pursue development and commercialization of any AXA Candidates that we decide to develop as drugs and that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical study or trial results, changes in the third parties' strategic focus or available funding, or external factors, such as a strategic transaction that may divert resources or create competing priorities;
- third parties may delay Non-IND, IRB-Approved Clinical Studies or Clinical Trials, provide insufficient funding for a Non-IND, IRB-Approved Clinical Study or Clinical Trial program, stop a Non-IND, IRB-Approved Clinical Study or Clinical Trial or abandon an AXA Candidate, repeat or conduct Non-IND, IRB-Approved Clinical Studies or new Clinical Trials or require a new formulation of an AXA Candidate for clinical testing;
- third parties could independently develop, or develop with other third parties, products that compete directly or indirectly with our products and AXA Candidates if the third parties believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- AXA Candidates discovered in collaboration with us may be viewed by our current or future third parties as competitive with their own product candidates or products, which may cause such third parties to cease to devote resources to the commercialization of our AXA Candidates;
- third parties may fail to comply with applicable regulatory requirements regarding the development, manufacture, packaging, labeling, holding, distribution and/or marketing of an AXA Candidate or product;
- third parties with marketing and distribution rights to one or more of our AXA Candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;

- disagreements with third parties, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of AXA Candidates, might lead to additional responsibilities for us with respect to AXA Candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;

- third parties may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

- third parties may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;

- if one of our third parties is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any AXA Candidate licensed to it by us; and

- relationships may be terminated by the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable AXA Candidates.

If our relationships do not result in the successful discovery, development and commercialization of products or if a third party terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under any third party agreements we enter into, our development of our technology and AXA Candidates could be delayed and we may need additional resources to develop AXA Candidates and our technology. All of the risks relating to product development, regulatory compliance and/or approval and commercialization described in this prospectus also apply to the activities of any drug and non-drug collaborators we enter into relationships or agreements with in the future. Additionally, if any third party terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

Relationships are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. We face significant competition in seeking appropriate collaborators. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. If we are unable to reach agreements with suitable third parties on a timely basis, on acceptable terms, or at all, we may have to curtail the development of an AXA Candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms, or at all. If we fail to enter into relationships or do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our AXA Candidates, bring them to market and generate revenue from sales of drugs or continue to develop our technology, and our business may be materially and adversely affected.

We will rely on third parties to conduct our clinical trials for any AXA Candidate that we decide to develop as a drug product candidate and to assist us in meeting the regulatory requirements applicable to development and marketing of non-drug products. If these third parties do not successfully carry out their contractual duties or meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval of or commercialize any potential AXA Candidates.

We will depend upon third parties, including independent investigators, to conduct preclinical studies, Non-IND, IRB-Approved Clinical Studies and/or Clinical Trials under agreements with universities, medical institutions, CROs, strategic partners and others. We expect to have to negotiate budgets and contracts with CROs and study or trial sites, which may result in delays to our development timelines and increased costs.

We have, and will have to, rely heavily on third parties over the course of our Non-IND, IRB-Approved Clinical Studies and Clinical Trials and, as a result, will have limited control over the clinical investigators and limited visibility into their day-to-day activities, including with respect to their compliance with the approved clinical protocol. Nevertheless, we are responsible for ensuring that each of our Non-IND, IRB-Approved Clinical Studies and Clinical Trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with GCP requirements, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCP requirements through periodic inspections of study or trial sponsors, clinical investigators and trial sites. If we or any of these third parties fail to comply with applicable GCP requirements, the clinical data generated in our Non-IND, IRB-Approved Clinical Studies or Clinical Trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to suspend or terminate these Non-IND, IRB-Approved Clinical Studies or Clinical Trials or perform additional Non-IND, IRB-Approved Clinical Studies or Clinical Trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our Non-IND, IRB-Approved Clinical Studies or Clinical Trials comply with the GCP or other applicable requirements. In addition, our Clinical Trials for therapeutic indications must be conducted with drug product produced under cGMP requirements and may require a large number of patients.

Our failure or any failure by these third parties to comply with these regulations or to recruit a sufficient number of subjects or patients may require us to repeat Non-IND, IRB-Approved Clinical Studies or Clinical Trials, which would delay the regulatory approval or commercialization process. Moreover, our business may be implicated if any of these third parties violates federal or state laws or regulations including fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Any parties conducting our future Non-IND, IRB-Approved Clinical Studies or Clinical Trials, if any, generally will not be our employees and, except for remedies that may be available to us under our agreements with the third parties conducting such Non-IND, IRB-Approved Clinical Studies or Clinical Trials, if any, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting Non-IND, IRB-Approved Clinical Studies or Clinical Trials or other product development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our Non-IND, IRB-Approved Clinical Studies and Clinical Trials may be extended, delayed or

terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our AXA Candidates. As a result, our financial results and the commercial prospects for our AXA Candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

If any of our relationships with these third-party CROs or others terminate, we may not be able to enter into contractual and other arrangements with alternative CROs or other third parties in a timely manner to meet projected clinical development deadlines or to do so on commercially reasonable terms. Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO begins work. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Further, we expect to work with and/or rely upon third-party CROs and/or regulatory consultants to assist us with meeting regulatory requirements applicable to non-drug products. If we experience delays in meeting or fail to meet the regulatory requirements for commercialization of our AXA Candidates, the commercial prospects for our AXA Candidates may be harmed and our ability to generate revenues will be materially impaired.

We expect to rely on third parties to manufacture our supply of AXA Candidates, and we intend to rely on third parties to produce and process our products, if approved or commercialized.

We currently rely on outside vendors to supply raw materials and other important components, such as the amino acids and excipients that make up our AXA Candidates. We have not yet caused any AXA Candidates to be manufactured or processed on a large clinical or commercial scale and may not be able to do so for any of our AXA Candidates. We will make changes as we work to optimize the manufacturing process for our AXA Candidates, and we cannot be sure that even minor changes in the process will result in products that are safe and, where applicable, effective.

The facilities used to manufacture our AXA Candidates that we develop as drug product candidates must be approved by the FDA or other foreign regulatory agencies pursuant to inspections that will be conducted after we submit a marketing application to the FDA or other foreign regulatory agencies. Additionally, any facilities used for the manufacture of AXA Candidates commercialized for non-drug uses will be subject to registration and inspection by the FDA and foreign regulatory authorities. We do not currently control all aspects of the manufacturing process of, and are currently largely dependent on, our contract manufacturing partners for compliance with regulatory requirements, known as cGMP requirements, for manufacture of our AXA Candidates. If we ever decide to open a manufacturing facility, we will be responsible for compliance with cGMP requirements. If we or our contract manufacturers cannot successfully manufacture in conformance with our specifications and the strict regulatory requirements of the FDA or other regulatory authorities, we and they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities with respect to the manufacture of our AXA Candidates. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our AXA Candidates, where applicable, or if it withdraws any such approval in the future, or if it otherwise finds that a manufacturing facility is out of regulatory compliance, we may need to find alternative manufacturing facilities, which would significantly impact our ability to research, develop, obtain regulatory approval, where necessary, for and/or market our AXA Candidates.

For more information, see "Risk Factors — Risks related to manufacturing and supply" below.

Risks related to manufacturing and supply

Our AXA Candidates rely on the availability of specialty raw materials, including significant quantities of amino acids, which may not be available to us on acceptable terms or at all.

Our AXA Candidates require certain specialty raw materials, including significant quantities of amino acids, some of which we may obtain from small companies with limited resources and experience to support a commercial product. The suppliers may be ill-equipped to support our needs, especially in non-routine circumstances like an FDA or foreign regulatory inspection or medical crisis, such as widespread contamination. Additionally, our suppliers may fail inspections or have other compliance issues with regulatory authorities that, even if unrelated to our supply chain and materials, may impact or cause delays in their ability to deliver agreed upon supplies in a timely manner which can have negative impacts on our business plans, including delays in initiating or continuing Non-IND, IRB-Approved Clinical Studies or Clinical Trials. We do not currently have supply contracts in place with all of the suppliers that we may need at any point in time in the future, and if needed, may not be able to contract with them on acceptable terms or at all, in particular for large quantities of pharmaceutical grade raw materials, including amino acids. Accordingly, we may experience delays in receiving key raw materials to support clinical or commercial manufacturing.

Our AXA Candidates require precise, high-quality manufacturing capabilities. If any of our third-party manufacturers encounter difficulties in manufacturing our AXA Candidates, our ability to provide supply of our AXA Candidates for Non-IND, IRB-Approved Clinical Studies or Clinical Trials or future commercial supply of products we bring to market under applicable regulatory requirements and approvals, could be delayed or stopped, or we may be unable to maintain a commercially viable cost structure.

We do not currently operate manufacturing facilities and rely on third parties under our existing contracts to produce our AXA Candidates. The manufacturing process used to produce our AXA Candidates has not been validated for clinical and commercial production. We combine multiple EMMs in novel combinations and ratios in our manufacturing process for AXA Candidates. These combinations may result in unanticipated manufacturing and product quality issues that we may not be able to resolve without incurring significant expense or delays in our Non-IND Clinical Studies or Clinical Trials, or at all. Furthermore, our cGMP manufacturing process development and scale-up is at an early stage. The actual cost to manufacture and process our AXA Candidates could be greater than we expect and could materially and adversely affect the commercial viability of our AXA Candidates.

Our manufacturing process may be susceptible to manufacturing issues associated with interruptions in the manufacturing process, contamination, equipment or reagent failure, improper installation or operation of equipment, vendor or operator error and variability in product characteristics. Even minor deviations from normal manufacturing processes could result in reduced production yields, lot failures, product defects, product recalls, product liability claims and other supply disruptions. If microbial, viral or other contaminations are discovered in our AXA Candidates or in the manufacturing facilities in which our AXA Candidates are made, production at such manufacturing facilities may be interrupted for an extended period of time to investigate and remedy the contamination. Further, as AXA Candidates are developed through preclinical, Non-IND, IRB-Approved Clinical Studies and, if we decide to develop any AXA Candidates as a drug product candidate, like we have done with AXA1665, Clinical Trials toward approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods, are altered along the way in an effort to scale-up and optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives, and any of these changes could cause our AXA Candidates to perform differently and affect the results of planned Clinical Trials or other future Clinical Trials.

Although we continue to optimize our manufacturing process for our AXA Candidates, doing so is a difficult and uncertain task, and there are risks associated with scaling to the level required for advanced Non-IND, IRB-Approved Clinical Studies and Clinical Trials or commercialization, including, among others, cost overruns, potential problems with process scale-up, process reproducibility, stability issues, lot consistency, supplier manufacturing capacity and timely availability of reagents and/or raw materials. We ultimately may not be successful in transferring our production system from our contract manufacturers to any manufacturing facilities we may establish ourselves or other contract manufacturers who can provide cost and process efficiencies, or our contract manufacturer may not have the necessary capabilities to complete the implementation and development process. If we are unable to adequately validate or scale-up the manufacturing process for our AXA Candidates with each of our current manufacturers, we will need to transfer to other manufacturers and complete the manufacturing validation and scale-up processes, which can be lengthy. If we are able to adequately validate and scale-up the manufacturing process for our AXA Candidates with a contract manufacturer, we will still need to negotiate with such contract manufacturer an agreement for commercial supply and it is not certain we will be able to come to agreement on terms acceptable to us. As a result, we may ultimately be unable to reduce the cost of goods for our AXA Candidates to levels that will allow for an attractive return on investment if and when those AXA Candidates are commercialized.

The manufacturing process for any AXA Candidates that we decide to develop as drug product candidates is subject to the FDA and foreign regulatory authority approval process, and extensive oversight of manufacturing facilities and changes to manufacturing processes. Non-drug products that we may develop will also be subject to extensive legal and regulatory requirements, including those with respect to the manufacturing, packaging, labeling, holding, processing and distribution of such products under appropriate cGMPs, as indicated in other risk factor sections herein. As such, we will need to contract with manufacturers who can meet all applicable FDA, foreign or other regulatory authority requirements on an ongoing basis, including with respect to quality systems and standards. If we or our CMOs are unable to reliably produce products under conditions and to specifications acceptable to the company and/or the FDA or other regulatory authorities, we may not obtain or maintain the ability or, in the case of drugs, the requisite approvals to commercialize such products. There is no assurance that our CMOs will be able to manufacture the approved product to specifications acceptable to us, the FDA, foreign or other regulatory authorities, even if we obtain regulatory approval for any of our AXA Candidates for therapeutic indications, to produce product in sufficient quantities to meet the requirements for the potential launch of the product or to meet potential future demand. In the case of AXA Candidates for which a therapeutic pathway is pursued, any of these challenges could delay completion of Clinical Trials, require bridging Clinical Trials or the repetition of one or more Clinical Trials, increase Clinical Trial costs and delay approval of our AXA Candidates. In the case of all AXA Candidates that we choose to commercialize, any of these challenges could delay and/or impair commercialization efforts, increase our cost of goods, and have an adverse effect on our business, financial condition, results of operations and growth prospects. Our future success depends on our ability to manufacture our AXA Candidates on a timely basis with acceptable manufacturing costs, while at the same time maintaining good quality control and complying with applicable regulatory requirements, and an inability to do so could have a material adverse effect on our business, financial condition and results of operations. In addition, we could incur higher manufacturing costs if manufacturing processes or standards change, and we could need to replace, modify, design or build and install equipment, all of which would require additional capital expenditures. Specifically, because our AXA Candidates may have a higher cost of goods than other drugs and/or non-drug products, the risk that coverage and reimbursement rates may be inadequate for us to achieve profitability may be greater.

In addition to raw materials and CMOs, we depend on third parties for clinical product supplies (e.g., clinical labeling and secondary packaging materials) and will likely need to do the same for any future commercial supply, including, in some instances, a single supplier.

In addition to raw materials and CMOs, we depend on third-party suppliers for labeling secondary packaging and other materials needed to produce Non-IND, IRB-Approved Clinical Study ready supplies of our AXA Candidates and will likely need to do the same for any future supplies for Clinical Trials or commercial supplies. These supplies may not always be available to us at the standards we require or on terms acceptable to us, or at all, and we may not be able to locate alternative suppliers in a timely manner, or at all. If we are unable to obtain necessary clinical or commercial supplies, our manufacturing operations and Non-IND, IRB-Approved Clinical Studies and Clinical Trials and the clinical studies and trials of our collaborators may be delayed or disrupted and our business and prospects may be materially and adversely affected as a result.

We may rely on a sole supplier for certain of our supplies. If this sole suppliers is unable to supply to us in the quantities we require, or at all, or otherwise defaults on its supply obligations to us, we may not be able to obtain alternative supplies from other suppliers on acceptable terms, in a timely manner, or at all.

We have limited experience manufacturing our AXA Candidates for purposes of Non-IND, IRB-Approved Clinical Studies, and have no experience manufacturing our AXA Candidates for the purposes of Clinical Trials, or at commercial scale, and if we decide to establish our own manufacturing facility for our AXA Candidates, we cannot assure you that we can manufacture our AXA Candidates in compliance with regulations at a cost or in quantities necessary to make them commercially viable.

We may establish a manufacturing facility for our AXA Candidates for use in Non-IND, IRB-Approved Clinical Studies, Clinical Trials, if any, or commercial sale. We have limited experience in cGMP compliant manufacturing of our AXA Candidates for purposes of Non-IND, IRB-Approved Clinical Studies and no experience manufacturing for Clinical Trials. We similarly have limited experience with the manufacturing requirements for non-drug products at a commercial scale. In the future, we may develop internal manufacturing capacity in part by expanding our own facilities or building additional facilities. This activity will require substantial additional funds and we would need to invest such funds in creating the proper manufacturing infrastructure and to hire and train a significant number of qualified employees to staff these facilities. We may not be able to develop cGMP-compliant manufacturing facilities that are adequate to produce materials for additional later-stage Non-IND, IRB-Approved Clinical Studies, Clinical Trials or commercialization.

The equipment and facilities employed in the manufacture of pharmaceuticals and non-drug products are subject to stringent qualification requirements by regulatory agencies, including validation of facility, equipment, systems, processes and analytics. We may be subject to lengthy delays and expense in conducting validation studies, if we can meet the requirements at all.

Risks related to our common stock and this offering

We do not know whether an active, liquid and orderly trading market will develop for our common stock or what the market price of our common stock will be and, as a result, it may be difficult for you to sell your shares of our common stock.

Prior to this offering there has been no public market for shares of our common stock. Although our common stock has been approved for listing on The Nasdaq Global Market, an active trading market for our shares may never develop or be sustained following this offering. You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not active. The initial public offering price for our common stock has been determined through negotiations with the underwriters, and the negotiated price may not be indicative of the

market price of the common stock after the offering. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the initial public offering price. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

The price of our stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock following this offering is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this prospectus, these factors include:

- the commencement, enrollment or results of our ongoing and planned Non-IND, IRB-Approved Clinical Studies, or any future Non-IND, IRB-Approved Clinical Studies or Clinical Trials we may conduct, or changes in the development status of our AXA Candidates;
- any delay in our regulatory filings for our AXA Candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings;
- adverse results from or delays in Non-IND, IRB-Approved Clinical Studies or Clinical Trials of our AXA Candidates, including as a result of clinical holds, safety events, enrollment or study or trial protocol amendments;
- our decision to initiate a Non-IND, IRB-Approved Clinical Studies or Clinical Trial, not to initiate a Non-IND, IRB-Approved Clinical Study or Clinical Trial or to terminate an existing Non-IND, IRB-Approved Clinical Study or Clinical Trial;
- adverse regulatory decisions, including the FDA's disagreeing with our interpretation and application of applicable rules and regulations and any government actions that may arise from such disagreement and our failure to receive regulatory approval of our AXA Candidates for therapeutic indications or to proceed on alternate regulatory pathways to market for our AXA Candidates;
- changes in laws or regulations applicable to our products, including, but not limited to, clinical trial requirements for approvals of drugs or marketing of non-drug products;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved product or inability to do so at acceptable prices;
- our inability to establish collaborations, if needed;
- our failure to commercialize our AXA Candidates;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of our AXA Candidates;
- introduction of new products or services by our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- the size and growth of our initial target markets;
- actual or anticipated variations in quarterly or annual operating results;
- our cash position;

- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- adoption of new accounting standards;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and the market for healthcare companies in particular, has experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. If the market price of our common stock after this offering does not exceed the initial public offering price, you may not realize any return on your investment in us and may lose some or all of your investment. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, our ability to pay cash dividends is currently restricted by the terms of our loan and security agreement with Solar, and future debt or other financing arrangements may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Any return to stockholders will therefore be limited in the foreseeable future to the appreciation of their stock.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Immediately following the completion of this offering, based on shares of common stock outstanding as of December 31, 2018 on a pro forma basis, our current executive officers, directors and their affiliates and 5% stockholders will hold, in the aggregate, approximately 64.7% of our outstanding voting stock (assuming no exercise of the underwriters' option to purchase additional shares). Therefore, even after this offering, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents or approval of any merger, sale of assets

or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that stockholders may feel are otherwise in their best interests.

Certain of our existing stockholders, including entities affiliated with certain of our directors, have indicated an interest in purchasing an aggregate of approximately $39.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any or all of these stockholders, or any or all of these stockholders may determine to purchase more, fewer or no shares in this offering. The foregoing ownership percentage does not give effect to any purchases of shares of our common stock by the stockholders.

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the net tangible book value of your shares.

The initial public offering price is substantially higher than the pro forma as adjusted net tangible book value per share of our common stock after this offering. Investors purchasing common stock in this offering will pay a price per share that substantially exceeds the pro forma as adjusted net tangible book value per share after this offering. As a result, investors purchasing common stock in this offering will incur immediate dilution of $15.08 per share, based on the initial public offering price of $20.00 per share, representing the difference between our pro forma as adjusted net tangible book value per share after giving effect to this offering and the initial public offering price. Further, investors purchasing common stock in this offering will contribute approximately 26.5% of the total amount invested by stockholders since our inception, but will own only approximately 15.5% of the shares of common stock outstanding after this offering.

This dilution is due to our investors who purchased shares prior to this offering having paid substantially less when they purchased their shares than the price offered to the public in this offering. To the extent outstanding stock options or warrants are exercised, new stock options or warrants are issued or we issue additional shares of common stock in the future, there will be further dilution to new investors. As a result of the dilution to investors purchasing common stock in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation. For a further description of the dilution that you will experience immediately after this offering, see "Dilution."

We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an emerging growth company, as defined in the JOBS Act, enacted in April 2012. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years following the year in which we complete this offering, although circumstances could cause us to lose that status earlier. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering; (b) in which we have total annual gross revenue of at least $1.07 billion; or (c) in which we are deemed to be a large accelerated filer, which requires the market value of our common stock that is held by non-affiliates to exceed $700 million as of the

prior June 30th; and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we may adopt the new or revised standard at the time private companies adopt the new or revised standard and may do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company. This may make comparison of our financial statements with the financial statements of another public company that is not an emerging growth company, or an emerging growth company that has opted out of using the extended transition period, difficult or impossible because of the potential differences in accounting standards used.

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, which will require, among other things, that we file with the SEC, annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and The Nasdaq Global Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas, such as "say on pay" and proxy access. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period and up to five years from the pricing of this offering. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements.

The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market could cause our stock price to fall.

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the trading price of our common stock could decline. Based on shares of common stock outstanding as of April 20, 2019, upon the closing of this offering we will have outstanding a total of 22,998,513 shares of common stock. Of these shares, only the shares of common stock sold in this offering by us, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable without restriction in the public market immediately following this offering.

The lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus, subject to earlier release of all or a portion of the shares subject to such agreements by the representatives of the underwriters in their sole discretion. After the lock-up agreements expire, based upon the number of shares of common stock, on an as-converted basis, outstanding as of April 20, 2019, up to an additional 19,416,931 shares of common stock will be eligible for sale in the public market. Approximately 59.5% of these additional shares are held by directors, current executive officers and other affiliates and will be subject to certain limitations of Rule 144 under the Securities Act of 1933, as amended, or the Securities Act. If these additional shares of common stock are sold upon the expiration of the lockup and market stand-off agreements, or if it is perceived that they will be sold, or the early release therefrom, the trading price of our common stock could decline and it may be more difficult for you to sell your common stock at a time and price that you deem appropriate.

In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our existing equity compensation plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline. Additionally, the number of shares of our common stock reserved for issuance under our 2019 Stock Option and Incentive Plan will automatically increase on January 1, 2020 and each January 1 thereafter by 4% of the number of shares of common stock outstanding on the immediately preceding December 31 or such lesser number of shares determined by our compensation committee. Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution.

After this offering, the holders of 17,356,437 shares of our common stock as of April 20, 2019 will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the 180-day lock-up agreements described above. See "Description of capital stock — Registration rights." Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act, where offers and sales by affiliates are not registered. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

We have broad discretion in the use of our existing cash, cash equivalents and the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of our existing cash, cash equivalents and the net proceeds from this offering, including for any of the purposes described in

the section entitled "Use of Proceeds," and you will not have the right or opportunity as part of your investment decision to assess whether such proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of our existing cash, cash equivalents and the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. Our management might not apply our existing cash, cash equivalents and the net proceeds from this offering in ways that ultimately increase the value of your investment. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders. If we do not invest or apply the net proceeds from this offering in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or our guidance.

Our quarterly and annual operating results may fluctuate significantly in the future, which makes it difficult for us to predict our future operating results. From time to time, we may enter into license or collaboration agreements with other companies that include development funding and significant upfront and milestone payments and/or royalties, which may become an important source of our revenue. Accordingly, our revenue may depend on development funding and the achievement of development and clinical milestones under current and any potential future license and collaboration agreements and sales of our products, if approved. These upfront and milestone payments may vary significantly from period to period and any such variance could cause a significant fluctuation in our operating results from one period to the next.

In addition, we measure compensation cost for stock-based awards made to employees, directors and non-employee consultants based on the fair value of the award on either the grant date or service completion date, and we recognize the cost as an expense over the recipient's service period. Because the variables that we use as a basis for valuing stock-based awards change over time, including our underlying stock price and stock price volatility, the magnitude of the expense that we must recognize may vary significantly.

Furthermore, our operating results may fluctuate due to a variety of other factors, many of which are outside of our control and may be difficult to predict, including the following:

- the timing and cost of, and level of investment in, research and development activities relating to our current and any future AXA Candidates, which will change from time to time;
- our ability to enroll subjects in Non-IND, IRB-Approved Clinical Studies or Clinical Trials and the timing of enrollment;
- the cost of manufacturing our current and any future AXA Candidates, which may vary depending on FDA guidelines and requirements, the quantity of production and the terms of our agreements with manufacturers;
- expenditures that we may incur to acquire or develop additional AXA Candidates and technologies;
- the timing and outcomes of Clinical Trials for our current AXA Candidates and any other future AXA Candidates or competing product candidates;
- competition from existing and potential future products that compete with our current AXA Candidates and any other future AXA Candidates, and changes in the competitive landscape of our industry, including consolidation among our competitors or partners;

- any delays in regulatory review or approval or commercialization of our current AXA Candidates or any other future AXA Candidates;
- the level of demand for our current AXA Candidates and any other future AXA Candidates, if approved, which may fluctuate significantly and be difficult to predict;
- the risk/benefit profile, cost and reimbursement policies with respect to our products, if approved, and existing and potential future products that compete with our current AXA Candidates and any other future AXA Candidates;
- our ability to commercialize our current AXA Candidates and any other future AXA Candidates inside and outside of the United States, either independently or working with third parties;
- our ability to adequately support future growth;
- potential unforeseen business disruptions that increase our costs or expenses;
- future accounting pronouncements or changes in our accounting policies; and
- the changing and volatile global economic environment.

The cumulative effect of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue and/or earnings guidance we may provide.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control, which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our restated certificate of incorporation and amended and restated bylaws, which are to become effective at or prior to the closing of this offering, contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;

- a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and

- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock, if it ever develops, will depend in part on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no securities or industry analysts commence coverage of our company, the trading price for our stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who cover us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

If we fail to maintain proper and effective internal control over financial reporting, our ability to produce accurate and timely financial statements could be impaired, investors may lose confidence in our financial reporting and the trading price of our common stock may decline.

Pursuant to Section 404 of Sarbanes-Oxley, our management will be required to report upon the effectiveness of our internal control over financial reporting beginning with the annual report for our fiscal year ending December 31, 2020. When we lose our status as an "emerging growth company," our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting. The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. To comply with the requirements of being a reporting company under the Exchange Act, we may need to implement additional financial and management controls, reporting systems and procedures and may need to hire additional accounting and finance staff.

We cannot assure you that there will not be material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting,

investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by The Nasdaq Global Market, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

If we engage in future acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We may evaluate various acquisition opportunities and strategic partnerships, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any potential acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- the issuance of our equity securities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing product programs and initiatives in pursuing such a strategic merger or acquisition;
- retention of key employees, the loss of key personnel and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and marketing approvals; and
- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs.

In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities, and this inability could impair our ability to grow or obtain access to technology or products that may be important to the development of our business.

Our amended and restated bylaws will designate specific courts as the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us.

Pursuant to our amended and restated bylaws, as will be in effect upon the completion of this offering, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Chancery Court does not have jurisdiction, the federal district court for the District of Delaware or other state courts of the State of Delaware) will be the sole and exclusive forum for state law claims for (i) any derivative action or proceeding brought on our behalf; (ii) any action asserting a claim of breach of a fiduciary duty or other wrongdoing by any of our directors, officers, employees or agents to us or our stockholders; (iii) any action asserting a claim against us arising pursuant to any provision of the Delaware General Corporation Law or our certificate of incorporation or bylaws; (iv) any action to interpret, apply, enforce or determine the validity of our certificate of incorporation or bylaws; or (v) any action asserting a claim governed by the internal affairs doctrine. This exclusive forum provision will not apply to any causes of action arising under the Securities Act or the Exchange Act. The forum selection clauses in our amended and restated bylaws may limit our stockholders' ability to obtain a favorable judicial forum for disputes with us.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that are based on management's beliefs and assumptions and on information currently available to management. Some of the statements in the section captioned "Prospectus summary," "Risk factors," "Management's discussion and analysis of financial condition and results of operations," "Business" and elsewhere in this prospectus contain forward-looking statements. In some cases, you can identify forward-looking statements by the following words: "may," "will," "could," "would," "should," "expect," "intend," "plan," "anticipate," "believe," "estimate," "predict," "project," "potential," "continue," "ongoing" or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words.

These statements involve risks, uncertainties and other factors that may cause actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained in this prospectus, we caution you that these statements are based on a combination of facts and factors currently known by us and our projections of the future, about which we cannot be certain. Forward-looking statements in this prospectus include, but are not limited to, statements about:

- the success, cost and timing of our product development activities, preclinical studies, Non-IND, IRB-Approved Clinical Studies and Clinical Trials, including statements regarding the timing of initiation and completion of preclinical studies, Non-IND, IRB-Approved Clinical Studies or Clinical Trials and related preparatory work, and the timing of the availability of the results of these preclinical studies, Non-IND, IRB-Approved Clinical Studies and Clinical Trials;
- our ability to obtain funding for our operations, including funding necessary to complete further development of our initial AXA Candidates, and if successful, commercialization of these candidates as drug or non-drug products;
- the potential for our identified research priorities to advance our AXA Development Platform, development programs or AXA Candidates;
- our ability to obtain and maintain regulatory approval or find alternate regulatory commercialization pathways from the FDA, EMA and other regulatory authorities for our AXA Candidates, and any related restrictions, limitations or warnings in the label of an approved AXA Candidate;
- our expectations regarding our ability to obtain and maintain intellectual property protection for our AXA Candidates, AXA Development Platform and the direction of such protection;
- our ability and the potential to successfully manufacture our AXA Candidates for preclinical studies, Non-IND, IRB-Approved Clinical Studies and Clinical Trials and for commercial use, if approved;
- the size and growth potential of the markets for our AXA Candidates and our ability to serve those markets, either alone or in combination with others;
- the rate and degree of market acceptance of our AXA Candidates, if approved;
- regulatory developments in the United States and foreign countries;
- our ability to enter into a partnership or other agreement with a third party on reasonable terms or at all to commercialize any of our AXA Candidates, if approved;
- our ability to secure sufficient manufacturing and supply chain capacity;

- the success of competing products or therapies that are or may become available;
- our ability to attract and retain key scientific or management personnel;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing; and
- our use of the proceeds from this offering.

In addition, you should refer to the "Risk Factors" section of this prospectus for a discussion of other important factors that may cause actual results to differ materially from those expressed or implied by the forward-looking statements. As a result of these factors, we cannot assure you that the forward-looking statements in this prospectus will prove to be accurate. Furthermore, if the forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all. The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

INDUSTRY AND MARKET DATA

The market data and certain other statistical information used throughout this prospectus are based on independent industry publications, governmental publications, reports by market research firms or other independent sources that we believe to be reliable sources. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. We are responsible for all of the disclosure contained in this prospectus, and we believe these industry publications and third-party research, surveys and studies are reliable. While we are not aware of any misstatements regarding any third-party information presented in this prospectus, their estimates, in particular, as they relate to projections, involve numerous assumptions, are subject to risks and uncertainties and are subject to change based on various factors, including those discussed under the section entitled "Risk Factors" and elsewhere in this prospectus. Some data are also based on our good faith estimates.

USE OF PROCEEDS

We estimate that the net proceeds to us from the sale of the shares of our common stock in this offering will be approximately $63.3 million, or approximately $73.3 million if the underwriters exercise their option to purchase additional shares in full, based upon the initial public offering price of $20.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

We currently expect to use the net proceeds from this offering, together with our cash and cash equivalents as of March 31, 2019, as follows:

- approximately $90.0 million to advance our current liver (AXA1665, AXA1125 and AXA1957) and other programs, including through additional Non-IND, IRB-Approved Clinical Studies, potential IND filing(s) and the ensuing Clinical Trials, including our planned IND filing and Clinical Trial for AXA1665, and infrastructure to support our pipeline;

- approximately $30.0 million to advance our AXA Development Platform discovery efforts, intellectual property and associated infrastructure; and

- the remainder, if any, for working capital and other general corporate purposes.

As of March 31, 2019, we had $66.7 million of cash and cash equivalents on hand, based on preliminary unaudited information and management estimates for the three months ended March 31, 2019. Based on our current plans, we believe our cash and cash equivalents as of March 31, 2019, together with the net proceeds from this offering, will be sufficient to fund our operating expenses and capital expenditure requirements through the second quarter of 2021. With our cash and cash equivalents as of March 31, 2019 and the net proceeds of this offering, we expect to be able to complete ongoing and planned Non-IND IRB-Approved Clinical Studies numbered AXA1665-002, AXA1125-003, AXA1957-002 and AXA4010-001; and file an IND for a Phase IIb/III AXA1665 Clinical Trial in HE, including if it is a registrational study. We have based these estimates on assumptions that may prove to be incorrect, and we could use our available capital resources sooner than we currently expect. In any event, we will require additional funding to be able to complete any Clinical Trial in HE, and we do not yet have any committed source of funding for this Clinical Trial. We may satisfy our future cash needs through the sale of equity securities, debt financings, working capital lines of credit, corporate collaborations or license agreements, grant funding, interest income earned on invested cash balances, or a combination of one or more of these sources.

We cannot specify with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering. Due to uncertainties inherent in the development process, it is difficult to estimate the exact amounts of the net proceeds that will be used for any particular purpose. We may use our existing cash, cash equivalents and the future payments, if any, generated from any future collaboration agreements to fund our operations, either of which may alter the amount of net proceeds used for a particular purpose. In addition, the amount, allocation and timing of our actual expenditures will depend upon numerous factors, including the results of our research and development efforts, the timing and success of our Non-IND, IRB-Approved Clinical Studies and Clinical Trials and the timing of regulatory submissions. Accordingly, we will have broad discretion in using these proceeds.

Pending their uses, we plan to invest the net proceeds of this offering in short-term, interest-bearing, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

DIVIDEND POLICY

We have never declared or paid any cash dividends on our common stock or any other securities. We anticipate that we will retain all available funds and any future earnings, if any, for use in the operation of our business and do not anticipate paying cash dividends in the foreseeable future. In addition, our ability to pay cash dividends is currently restricted by the terms of our loan and security agreement with Solar, and future debt or other financing arrangements may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock.

CAPITALIZATION

The following table sets forth our cash and cash equivalents and our capitalization as of December 31, 2018:

- on an actual basis;
- on a pro forma basis to give effect to (i) the conversion of all outstanding shares of our preferred stock into an aggregate of 14,641,997 shares of common stock upon the closing of this offering, (ii) all outstanding warrants to purchase shares of Series A preferred stock becoming warrants to purchase shares of common stock upon the closing of this offering and (iii) the filing and effectiveness of our restated certificate of incorporation in connection with this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of 3,571,428 shares of common stock in this offering at the initial public offering price of $20.00 per share, after deducting underwriting discounts, commissions and estimated offering expenses payable by us and the payment of the $1.2 million success fee that is payable to Solar in connection with our loan and security agreement upon completion of this offering.

You should read the information in this table together with our consolidated financial statements and the related notes appearing at the end of this prospectus and the "Selected

Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of this prospectus.

	As of December 31, 2018		
	Actual	Pro Forma	Pro Forma As Adjusted
	(in thousands, except share and per share data)		
Cash and cash equivalents	$ 79,466	$ 79,466	$ 142,785
Long term debt, net of discount	24,521	24,521	24,521
Other liabilities [1]	1,898	1,898	698
Warrant liability	425	—	—
Redeemable convertible preferred stock (Series A, B, B-1, C, D and E), $0.001 par value; 27,928,825 shares authorized, 26,831,246 issued and outstanding, actual; no shares authorized, no issued or outstanding, pro forma and pro forma as adjusted	197,842	—	—
Stockholders' equity (deficit):			
Preferred stock, $0.001 par value; no shares authorized, issued or outstanding, actual; 10,000,000 shares authorized; no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	—
Common stock, $0.001 par value; 47,000,000 shares authorized, 5,193,915 shares issued and 4,774,934 shares outstanding, actual; 47,000,000 shares authorized, 19,835,912 shares issued and 19,416,931 shares outstanding, pro forma; 150,000,000 shares authorized, 23,407,340 shares issued and 22,988,359 shares outstanding, pro forma as adjusted	6	19	23
Additional paid-in capital	7,290	205,544	270,059
Treasury stock, 418,981 shares at cost	—	—	—
Accumulated deficit	(157,049)	(157,049)	(157,049)
Total stockholders' equity (deficit)	(149,753)	48,514	113,033
Total capitalization	$ 74,933	$ 74,933	$ 138,252

(1) Includes a $1.2 million success fee relating to our loan and security agreement with Solar, which is payable upon the consummation of this offering.

The table above does not include:

- 4,039,464 shares of common stock issuable upon the exercise of stock options outstanding as of December 31, 2018, at a weighted average exercise price of $5.67 per share;
- 61,235 shares of common stock issuable upon the exercise of warrants outstanding as of December 31, 2018 to purchase shares of Series A preferred stock that will become warrants to purchase shares of common stock, at a weighted average exercise price of $3.59 per share, in connection with this offering;
- 41,230 shares of common stock available for future issuance as of December 31, 2018 under our 2010 Plan, which ceased to be available for issuance at the time that our 2019 Plan became effective;

- 905,000 shares of common stock that became available for future issuance under our 2019 Plan in connection with the effectiveness of the registration statement of which this prospectus is a part; and
- 237,181 shares of common stock that became available for future issuance under our 2019 Employee Stock Purchase Plan in connection with the effectiveness of the registration statement of which this prospectus is a part.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

Our historical net tangible book value (deficit) as of December 31, 2018 was $(149.8) million, or $(31.36) per share of our common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities and the carrying values of our preferred stock, which is not included within stockholders' equity (deficit). Historical net tangible book value (deficit) per share represents historical net tangible book value (deficit) divided by the 4,774,934 shares of our common stock outstanding as December 31, 2018.

Our pro forma net tangible book value as of December 31, 2018 was $48.5 million, or $2.50 per share of our common stock. Pro forma net tangible book value (deficit) represents the amount of our total tangible assets less our total liabilities, after giving effect to (i) the conversion of all outstanding shares of our preferred stock into an aggregate of 14,641,997 shares of common stock upon the closing of this offering and (ii) all outstanding warrants to purchase shares of Series A preferred stock becoming warrants to purchase shares of common stock upon the closing of this offering. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of December 31, 2018, after giving effect to the pro forma adjustments described above.

After giving further effect to our issuance and sale of 3,571,428 shares of our common stock in this offering at the initial public offering price of $20.00 per share and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of December 31, 2018 would have been $113.0 million, or $4.92 per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $2.42 to existing stockholders and immediate dilution of $15.08 in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Assumed initial public offering price per share		$ 20.00
Historical net tangible book value (deficit) per share as of December 31, 2018	$ (31.36)	
Increase per share attributable to the pro forma adjustments described above	33.86	
Pro forma net tangible book value per share as of December 31, 2018	2.50	
Increase in pro forma as adjusted net tangible book value per share attributable to new investors purchasing common stock in this offering	2.42	
Pro forma as adjusted net tangible book value per share after this offering		4.92
Dilution per share to new investors purchasing common stock in this offering		$ 15.08

If the underwriters exercise their option to purchase additional shares in full, our pro forma as adjusted net tangible book value per share after this offering would be $5.23, representing an immediate increase in pro forma as adjusted net tangible book value per share of $2.73 to existing stockholders and immediate dilution in pro forma as adjusted net tangible book value per share of $14.77 to new investors purchasing common stock in this offering, based on the initial public offering price of $20.00 per share and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

The following table summarizes, as of December 31, 2018, on the pro forma as adjusted basis described above, the total number of shares of common stock purchased from us on an as converted to common stock basis, the total consideration paid or to be paid, and the average price per share paid or to be paid by existing stockholders and by new investors in this offering at the initial public offering price of $20.00 per share, before deducting underwriting discounts and commissions and estimated offering expenses payable by us. As the table shows, new investors purchasing common stock in this offering will pay an average price per share substantially higher than our existing stockholders paid.

	Shares Purchased		Total Consideration		Average Price
	Number	Percentage	Amount	Percentage	Per Share
Existing shareholders	19,416,931	84.5%	$ 198,012,594	73.5%	$ 10.20
New investors[1]	3,571,428	15.5	71,428,560	26.5	$ 20.00
Total	22,988,359	100.0%	$ 269,441,154	100.0%	

(1) Certain of our existing stockholders, including entities affiliated with certain of our directors, have indicated an interest in purchasing an aggregate of approximately $39.0 million in shares of our common stock in this offering at the initial public offering price. The presentation in this table regarding ownership by existing stockholders does not give effect to any purchases in this offering by such stockholders.

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to 82.5% of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors purchasing common stock in this offering would be increased to 17.5% of the total number of shares of our common stock outstanding after this offering.

The tables and discussion above are based on the number of shares of our common stock outstanding as of December 31, 2018, and exclude:

- 4,039,464 shares of common stock issuable upon the exercise of stock options outstanding as of December 31, 2018, at a weighted average exercise price of $5.67 per share;
- 61,235 shares of common stock issuable upon the exercise of warrants outstanding as of December 31, 2018 to purchase shares of Series A preferred stock that will become warrants to purchase shares of common stock, at a weighted average exercise price of $3.59 per share, in connection with this offering;
- 41,230 shares of common stock available for future issuance as of December 31, 2018 under our 2010 Plan, which ceased to be available for issuance at the time that our 2019 Plan became effective;
- 905,000 shares of common stock that became available for future issuance under our 2019 Plan in connection with the effectiveness of the registration statement of which this prospectus is a part; and
- 237,181 shares of common stock that became available for future issuance under our 2019 Employee Stock Purchase Plan in connection with the effectiveness of the registration statement of which this prospectus is a part.

To the extent that outstanding stock options or warrants are exercised, new stock options or warrants are issued or we issue additional shares of common stock in the future, there will be further dilution to new investors. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

SELECTED CONSOLIDATED FINANCIAL DATA

You should read the following selected consolidated financial data together with our consolidated financial statements and the related notes appearing at the end of this prospectus and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this prospectus. We have derived the consolidated statements of operations data for the years ended December 31, 2017 and 2018 and the consolidated balance sheet data as of December 31, 2018 from our audited consolidated financial statements appearing at the end of this prospectus. Our historical results are not necessarily indicative of the results that may be expected in the future.

	December 31,	
	2017	2018
	(in thousands, except share and per share data)	
Consolidated Statements of Operations Data:		
Operating expenses:		
Research and development	$ 22,916	$ 25,486
General and administrative	6,005	8,410
Total operating expenses	28,921	33,896
Loss from operations	(28,921)	(33,896)
Other income (expense):		
Change in fair value of preferred stock warrant liability	81	(14)
Interest income (expense), net	(2,100)	(2,159)
Total other income (expense), net	(2,019)	(2,173)
Net loss	$ (30,940)	$ (36,069)
Net loss per share, basic and diluted [1]	$ (7.37)	$ (7.97)
Weighted average common shares outstanding, basic and diluted [1]	4,211,918	4,546,373
Pro forma net loss per share, basic and diluted (unaudited) [1]		$ (2.19)
Pro forma weighted average common shares outstanding, basic and diluted (unaudited) [1]		16,528,448

(1) See Note 11 to our consolidated financial statements appearing at the end of this prospectus for details on the calculation of basic and diluted net loss per share and the calculation of basic and diluted pro forma net loss per share.

	December 31,	
	2017	2018
	(in thousands)	
Consolidated Balance Sheet Data:		
Cash and cash equivalents	$ 46,817	$ 79,466
Working capital [1]	41,977	73,390
Total assets	48,813	81,844
Long term debt, net of discount	19,557	24,521
Other liabilities [2]	1,276	1,898
Preferred stock warrant liability	411	425
Redeemable convertible preferred stock	138,828	197,842
Total stockholders' equity (deficit)	(116,354)	(149,753)

(1) We define working capital as current assets less current liabilities.

(2) Includes a $1.2 million success fee relating to our loan and security agreement with Solar, which is payable upon the consummation of this offering.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the "Selected Consolidated Financial Data" section of this prospectus and our consolidated financial statements and related notes appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a biotechnology company pioneering the research and development of novel multifactorial interventions to support health and address dysregulated metabolism across a broad spectrum of consumers and patients who have limited options. Our AXA Candidates are generated from our proprietary, human-focused AXA Development Platform and harness the power of EMMs, a broad family of molecules that fundamentally impact and regulate human metabolism.

Using our AXA Development Platform, we have rapidly generated a pipeline of AXA Candidates that are novel compositions of EMMs engineered in distinct ratios and designed to target and maximize the fundamental role that EMMs play in regulating multiple metabolic functions. Our AXA Candidates are administered orally and anchored by amino acids, which have a history of safe use as food. As such, we expect our AXA Candidates may also be combinable with other modalities. We believe our current dataset supports the potential of our AXA Candidates to support and modulate the metabolic pathways they target with favorable safety profiles. These features may make them an attractive development opportunity with significant commercial potential.

To date, we have funded our operations with proceeds from the sale of preferred stock and borrowing of debt. Through December 31, 2018, we had received gross proceeds of $197.8 million from sales of our preferred stock and $26.0 million from borrowings under a loan and security agreement. Since our inception, we have incurred significant operating losses. Our ability to generate product revenue sufficient to achieve profitability or even any product revenue will depend on the successful development and eventual commercialization of one or more of our AXA Candidates. We reported net losses of $30.9 million and $36.1 million for the years ended December 31, 2017 and 2018, respectively. We expect to continue to incur significant expenses and increasing operating losses and capital requirements for at least the next several years in connection with our ongoing activities, particularly if and as we conduct clinical trials for our AXA Candidates as we:

- further develop our AXA Development Platform;
- continue to discover and develop additional AXA Candidates;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, scientific manufacturing and commercial personnel;
- establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any AXA Candidates for which we may obtain regulatory approval;

- seek regulatory approvals for any AXA Candidates for which we make a drug development path decision, as we have done with AXA1665, and that successfully complete Clinical Trials;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval;
- potentially acquire or in-license other product candidates and technologies; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts, as well as to support our transition to being a public company.

Regulatory agencies, including the FDA, regulate many aspects of a product candidate's life cycle, including research and development and clinical testing. We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our AXA Candidates or choose to market an AXA Candidate as a non-drug product. If we obtain regulatory approval for any of our AXA Candidates, we expect to incur significant expenses related to developing our commercialization capabilities to support product sales, marketing and distribution. Further, following the closing of this offering, we expect to incur additional costs associated with operating as a public company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, strategic alliances and marketing, distribution or licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our AXA Candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

We believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses, capital expenditure requirements, and debt service payments through the second quarter of 2021. See "—Liquidity and capital resources."

Components of our results of operations

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the near future. If our development efforts for our AXA Candidates are successful and result in regulatory approval or we execute license or collaboration agreements with third parties, we may generate revenue in the future from product sales, payments from collaborations or license agreements that we may enter into with third parties, or any combination thereof.

Operating expenses

Research and development expenses

Our research and development expenses consist primarily of costs incurred in connection with our research activities, including our drug discovery efforts, and the development of our AXA Candidates, which include:

- employee-related expenses, including salaries, related benefits and stock-based compensation expense for employees engaged in research and development functions;
- expenses incurred in connection with the preclinical and clinical development of our AXA Candidates, including any Non-IND, IRB-Approved Clinical Studies and research programs, including under agreements with third parties, such as consultants, contractors and contract research organizations, or CROs;
- the cost of developing and scaling our manufacturing process and manufacturing products for use in our preclinical studies and Non-IND, IRB-Approved Clinical Studies, including under agreements with third parties, such as consultants, contractors and contract manufacturing organizations, or CMOs;
- laboratory supplies and research materials;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities and insurance; and
- costs related to compliance with regulatory requirements as well as costs to protect our intellectual property.

We expense research and development expenses as incurred. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Our direct external research and development expenses are tracked on a program-by-program basis and consist of costs that include fees, reimbursed materials and other costs paid to consultants, contractors, CMOs and CROs in connection with our preclinical and clinical development and manufacturing activities. We do not allocate employee costs, costs associated with our discovery efforts, laboratory supplies and facilities expenses, including depreciation or other indirect costs, to specific product development programs because these costs are deployed across multiple programs, as well as our platform technology and, as such, are not separately classified.

AXA Candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size, scope and duration of later-stage Clinical Trials. We expect that our research and development expenses will increase substantially in connection with our planned preclinical and clinical development activities both in the near-term and beyond. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any of our AXA Candidates, as the successful development and commercialization of our AXA Candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with product development and commercialization, including the following:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;

- the progress of the development efforts of parties with whom we may enter into collaboration arrangements;
- our ability to maintain our current research and development programs and to establish new ones;
- our ability to establish new licensing or collaboration arrangements for both drug and non-drug products;
- FDA clinical and other requirements for the receipt and related terms of regulatory approval;
- the successful initiation and completion of Non-IND, IRB-Approved Clinical Studies or Clinical Trials, with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- the receipt and related terms of regulatory approvals from applicable regulatory authorities;
- the availability of specialty raw materials for use in production of our AXA Candidates;
- our ability to consistently manufacture our AXA Candidates for use in Non-IND, IRB-Approved Clinical Studies or Clinical Trials;
- our ability to establish and secure manufacturing supply through relationships with third parties;
- our ability to protect our rights in our intellectual property portfolio and maintain trade secret protection and regulatory exclusivity, both in the United States and internationally;
- the commercialization of our AXA Candidates, if and when approved;
- obtaining and maintaining third-party insurance coverage and adequate reimbursement;
- the acceptance of our AXA Candidates, if approved, by patients, the medical community, third-party payors or other parties, such as consumers;
- our ability to raise additional funds necessary to complete preclinical and clinical development of and commercialize our AXA Candidates;
- competition with other products; and
- a continued acceptable safety profile of our therapies following approval.

A change in the outcome of any of these variables with respect to the development of any of our AXA Candidates could significantly change the costs and timing associated with the development of that AXA Candidate. We may never succeed in obtaining regulatory approval for any of our AXA Candidates.

General and administrative expenses

General and administrative expenses consist primarily of salaries and related costs, including stock-based compensation, for personnel in executive, finance and administrative functions. General and administrative expenses also include direct and allocated facility-related costs including information technology, as well as professional fees for legal, consulting, investor and public relations, accounting and audit services. We anticipate that our general and administrative expenses will increase in the future as we expand our organization to support the continued research and development activities of our AXA Candidates. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses associated with operating as a public company.

Other income (expense)

Interest expense, net

Interest expense consists of interest expense on outstanding borrowings under our loan and security agreement as well as amortization of debt discount and debt issuance costs. Interest income has historically been insignificant and consists of interest earned on our invested cash balances. We expect our interest income to increase compared to the year ended December 31, 2018 as we invest the cash received from the sale of Series E Preferred Stock in November 2018 and the net proceeds from this offering.

Change in fair value of preferred stock warrant liability

In connection with the issuance of debt in 2012, we issued warrants to purchase Series A preferred stock. We classify these warrants as a liability on our consolidated balance sheet that we remeasure to fair value as of each reporting date, and we recognize changes in the fair value of the warrant liability as a component of other income (expense) in our consolidated statements of operations. We will continue to recognize changes in the fair value of the warrant liability until the warrants are exercised, expire or qualify for equity classification.

Upon the closing of this offering, the preferred stock warrants will become exercisable for common stock instead of preferred stock, and the fair value of the warrant liability at that time will be reclassified to additional paid-in capital. As a result, subsequent to the closing of this offering, we will no longer remeasure the fair value of the warrant liability at each reporting date.

Income taxes

Since our inception, we have not recorded any income tax benefits for the net losses we have incurred in each year or for our research and development tax credits, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our net operating loss carryforwards and tax credits will not be realized. As of December 31, 2018, we had U.S. federal and state net operating loss carryforwards of $140.6 million and $139.4 million, respectively, which may be available to offset future taxable income and begin to expire in 2030. As of December 31, 2018, we also had U.S. federal and state research and development tax credit carryforwards of $4.2 million and $1.6 million, respectively, which may be available to offset future tax liabilities and will expire at various dates through 2038. We have recorded a full valuation allowance against our net deferred tax assets at each balance sheet date.

On December 22, 2017, the Tax Cuts and Jobs Act, or the TCJA, was signed into United States law. The TCJA includes a number of changes to existing tax law, including, among other things, a permanent reduction in the federal corporate income tax rate from a top marginal tax rate of 35% to a flat rate of 21%, effective as of January 1, 2018, as well as limitation of the deduction for net operating losses to 80% of annual taxable income and elimination of net operating loss carrybacks, in each case, for losses arising in taxable years beginning after December 31, 2017, although any such net operating losses may be carried forward indefinitely.

Results of operations

Comparison of the years ended December 31, 2017 and 2018

The following table summarizes our results of operations for the years ended December 31, 2017 and 2018:

	2017	2018	Change
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	22,916	25,486	2,570
General and administrative	6,005	8,410	2,405
Total operating expenses	28,921	33,896	4,975
Loss from operations	(28,921)	(33,896)	(4,975)
Other income (expense):			
Change in fair value of preferred stock warrant liability	81	(14)	(95)
Interest income (expense), net	(2,100)	(2,159)	(59)
Total other expense, net	(2,019)	(2,173)	(154)
Net loss	$ (30,940)	$ (36,069)	$ (5,129)

Research and development expenses

	Year ended December 31,		
	2017	2018	Change
	(in thousands)		
Direct research and development expenses by program:			
AXA1665	$ —	$ 2,012	$ 2,012
AXA1125/1957	1,747	3,689	1,942
AXA2678	657	356	(301)
Platform development, early-stage research and unallocated expenses:			
Personnel related	9,375	9,265	(110)
Stock-based compensation expense	604	1,091	487
External manufacturing and research	4,919	3,018	(1,901)
Laboratory supplies and research materials	825	1,031	206
Facility related and other	4,789	5,024	235
Total research and development expenses	$ 22,916	$ 25,486	$ 2,570

Research and development expenses were $22.9 million for the year ended December 31, 2017, compared to $25.5 million for the year ended December 31, 2018. The increase in direct costs related to our AXA1665 program of $2.0 million was primarily due to costs incurred with external CROs and CMOs for the Non-IND, IRB-Approved Clinical Studies conducted in 2018. The increase in direct costs related to our AXA1125 and AXA1957 program was due to the Non-IND, IRB-Approved Clinical Study achieving full enrollment and completing during 2018. The increase in stock-based compensation expense of $0.5 million was primarily due to an increase in the number of awards granted and the per share fair value of such awards. The decrease in external manufacturing and research costs of $1.9 million was a result of lower use of material to support our Non-IND, IRB-Approved Clinical Studies. The increase in laboratory supplies and research materials of $0.2 million was primarily due to expansion of our *in vitro* capabilities. The increase in

facility-related and other expenses of $0.2 million was primarily due to increased rent and expanded operations.

General and administrative expenses

	Year ended December 31,		
	2017	2018	Change
	(in thousands)		
Personnel related	$ 3,792	$ 4,462	$ 670
Stock-based compensation expense	642	1,690	1,048
Professional and consultant fees	1,105	1,848	743
Facility related and other	466	410	(56)
Total general and administrative expenses	$ 6,005	$ 8,410	$ 2,405

General and administrative expenses for the year ended December 31, 2017 were $6.0 million, compared to $8.4 million for the year ended December 31, 2018. The increase in general and administrative expenses of $2.4 million was primarily due to growth of operations and clinical programs and required personnel and professional services to support that growth. The increase in stock-based compensation expense of $1.0 million was primarily due to an increase in the number of awards granted and the per share fair value of such awards. Personnel-related costs increased by $0.7 million primarily due to the hiring of key executives in 2018, including our Chief Executive Officer and Chief Business Officer, as well as additional personnel in our general and administrative functions as we continued to expand our operations to support the organization. Professional and consultant fees increased by $0.7 million primarily due to expanded operations.

Liquidity and capital resources

Since our inception, we have incurred significant operating losses. We have not yet commercialized any of our AXA Candidates, and we do not expect to generate revenue from sales of any AXA Candidates for several years, if at all. To date, we have funded our operations with proceeds from the sales of preferred stock and borrowings under our loan and security agreement. Through December 31, 2018, we had received gross proceeds of $197.8 million from sales of our preferred stock and $26.0 million from borrowings under a loan and security agreement. As of December 31, 2018, we had cash and cash equivalents of $79.5 million.

Cash flows

The following table summarizes our sources and uses of cash for each of the periods presented:

	Year ended December 31,	
	2017	2018
Cash used in operating activities	$ (26,861)	$ (30,712)
Cash used in investing activities	(915)	(586)
Cash provided by financing activities	25,697	63,947
Net increase (decrease) in cash and cash equivalents	$ (2,079)	$ 32,649

Operating activities

During the year ended December 31, 2018, operating activities used $30.7 million of cash, primarily resulting from our net loss of $36.9 million, partially offset by net non-cash charges of $4.4 million, primarily consisting of stock-based compensation expense, and net cash provided by

changes in our operating assets and liabilities of $1.0 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2018 consisted primarily of a $1.6 million increase in accounts payable and accrued expenses, partially offset by a $0.6 million increase in prepaid expenses and other current assets.

During the year ended December 31, 2017, operating activities used $26.9 million of cash, primarily resulting from our net loss of $30.9 million, partially offset by net non-cash charges of $2.9 million and net cash provided by changes in our operating assets and liabilities of $1.2 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2017 consisted primarily of a $1.1 million increase in accounts payable and accrued expenses.

Investing activities

During the years ended December 31, 2017 and 2018, net cash used in investing activities was $0.9 million and $0.6 million, respectively, due to purchases of property and equipment. The purchases of property and equipment during the year ended December 31, 2018 related to equipment purchases associated with the expansion of our AXA Development Platform and other research and development capabilities.

Financing activities

During the year ended December 31, 2018, net cash provided by financing activities was $63.9 million, consisting primarily of proceeds from the issuance of preferred stock of $58.9 million and borrowings of $6.0 million under our current loan and security agreement.

During the year ended December 31, 2017, net cash provided by financing activities was $25.7 million, consisting primarily of proceeds from the issuance of Series C Preferred Stock.

Loan and security agreement

At December 31, 2018, we had $26.0 million in outstanding long term debt. In October 2018, we amended the 2018 Facility, or the Amended 2018 Facility, to extend the interest only period through July 2020 or January 2021 and the Maturity Date to July 2022 or January 2023 if certain conditions are met. The Amended 2018 Facility provides additional funding in the amounts of $5.0 million, in the Term B Loan, and $4.0 million, in the Term C Loan, if certain conditions are met. The Term B Loan of $5.0 million was drawn in December 2018. Monthly principal payments of $1.1 million will commence August 2020 for 24 months. The terminal interest fee was amended to 5.35% ($1.4 million) and is due with the final principal payment of the loan. We granted the lender a first security interest in all of our assets, excluding intellectual property and granted a negative pledge on such intellectual property. The interest rate and success fee were not changed through the amendment.

Funding requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities, Non-IND, IRB-Approved Clinical Studies and Clinical Trials for our AXA Candidates in development. In addition, upon the closing of this offering, we expect to incur additional costs associated with becoming and operating as a public company. The timing and amount of our operating and capital expenditures will depend largely on:

- the timing and progress of preclinical and clinical development activities;
- the commencement, enrollment or results of the planned Non-IND, IRB-Approved Clinical Studies of our AXA Candidates or any future or additional Non-IND, IRB-Approved Clinical Studies or Clinical Trials we may conduct or may be required to conduct, or changes in the development status of our AXA Candidates;

- the timing and outcome of regulatory review of our AXA Candidates;
- our decision to initiate a Non-IND, IRB-Approved Clinical Study or Clinical Trial, not to initiate a Non-IND, IRB-Approved Clinical Study or Clinical Trial or to terminate an existing Non-IND, IRB-Approved Clinical Study or Clinical Trial;
- changes in laws or regulations applicable to our AXA Candidates, including but not limited to Clinical Trial requirements for approvals;
- developments concerning our CMOs;
- our ability to obtain materials to produce adequate product supply for any approved product or inability to do so at acceptable prices;
- our ability to establish a manufacturing and a supply chain for supply of AXA Candidates for Non-IND, IRB-Approved Clinical Studies and Clinical Trials and for commercial supply;
- our ability to establish collaborations if needed;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our AXA Candidates for which we obtain marketing approval as a drug or market as a non-drug product;
- the legal patent costs involved in prosecuting patent applications and enforcing patent claims and other intellectual property claims;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of our AXA Candidates; and
- the terms and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder.

We believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses, capital expenditure requirements, and debt service payments through the second quarter of 2021. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making acquisitions or capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or drug candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed, we may be required to delay, limit, reduce or terminate our research, product development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual obligations and commitments

The following table summarizes our contractual obligations as of December 31, 2018 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

	Payments due by period				
	Total	Less than 1 year	1 to 3 years	4 to 5 years	More than 5 years
	(in thousands)				
Operating lease commitments [1]	$ 2,828	$ 1,187	$ 1,641	$ —	$ —
Debt obligations [2]	33,192	2,740	22,603	7,849	—
Total	$ 36,020	$ 3,927	$ 24,244	$ 7,849	$ —

(1) Amounts in table reflect payments due for our leases of office and laboratory space in Cambridge, Massachusetts under one operating lease agreement that expires in April 2021.

(2) Amounts in table reflect the contractually required principal and interest payments payable under our credit facility. For purposes of this table, the interest due under the 2018 Credit Facility was calculated using an assumed interest rate of 10.54% (LIBOR plus 8.50%) per annum, which was the interest rate in effect as of December 31, 2018.

We enter into contracts in the normal course of business with CROs, CMOs and other third parties for preclinical research studies, Non-IND, IRB-Approved Clinical Studies, Clinical Trials and testing and manufacturing services. These contracts do not contain minimum purchase commitments and are cancelable by us upon prior written notice. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation. These payments are not included in the table above as the amount and timing of such payments are not known.

Critical accounting policies and significant judgments and estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue, costs and expenses and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements appearing at the end of this prospectus, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our financial statements.

Research and development costs

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed,

on a pre-determined schedule or when contractual milestones are met; however, some service providers require advance payments. Examples of estimated accrued research and development expenses include fees paid to:

- CROs in connection with Non-IND, IRB-Approved Clinical Studies;
- investigative sites in connection with Non-IND, IRB-Approved Clinical Studies;
- CMOs in connection with the process development and scale-up activities and the production of preclinical Non-IND, IRB-Approved Clinical Study and Clinical Trial materials; and
- vendors in connection with preclinical and clinical development activities (e.g., data analytics).

We base our expenses related to Non-IND, IRB-Approved Clinical Studies on our estimates of the services received and efforts expended pursuant to contracts with CROs that conduct and manage Non-IND, IRB-Approved Clinical Studies on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of subjects and the completion of clinical study milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period and adjust accordingly.

We make estimates of our research and development accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We recognize costs for certain development activities based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites, such as number of sites activated, number of patient enrollments and visits and patient duration. We determine accrual estimates through financial models that take into account discussion with applicable personnel and service providers as to the progress or state of completion of trials. We periodically confirm the accuracy of these estimates with the service providers and make adjustments, if necessary. Upon settlement, these costs may differ materially from the amounts accrued in our consolidated financial statements. Our historical accrual estimates have not been materially different from our actual costs. However, due to the nature of estimates, we cannot provide assurance that we will not make changes to our estimates in the future as we become aware of additional information about the status or conduct of our Non-IND, IRB-Approved Clinical Studies and other research activities.

Stock-based compensation

We issue stock-based awards to employees and non-employees, generally in the form of stock options and unvested stock. We measure and recognize compensation expense for our stock-based awards granted to our employees and non-employee directors based on the estimated grant date fair value in accordance with FASB ASC Topic 718, *Compensation — Stock Compensation*, or ASC 718. We account for stock-based awards to non-employees in accordance with FASB ASC Topic 505-50, *Equity-Based Payments to Non-Employees*, which requires the fair value of the unvested portion of the equity awards granted to non-employees to be re-measured as of each reporting date.

Our stock-based awards are subject to either service or performance-based vesting conditions. We recognize compensation expense related to awards to employees and directors with service-based vesting on a straight-line basis based on the grant date fair value over the requisite service

period, which is generally the vesting period. Compensation expense related to awards to employees with performance-based vesting conditions is recognized based on the grant date fair value over the requisite service period using an accelerated attribution method to the extent the achievement of the performance condition is probable. Compensation expense related to awards to non-employees with service-based vesting conditions is recognized on the then-current fair value at each reporting date prior to the measurement date over the requisite service period, which is generally the vesting period. We recognize forfeitures as they occur.

We classify stock-based compensation expense in our consolidated statements of operations in the same manner in which the award recipient's salary and related costs are classified or in which the award recipient's service payments are classified. In future periods, we expect stock-based compensation expense to increase, due in part to our existing unrecognized stock-based compensation expense and as we grant additional stock-based awards to continue to attract and retain our employees.

Fair value of stock-based awards

We estimate the fair value of our stock options using the Black-Scholes option pricing model, which requires inputs of subjective assumptions, including: (i) the expected volatility of our stock; (ii) the expected term of the award; (iii) the risk-free interest rate; (iv) expected dividends; and (v) the fair value of common stock. Due to the lack of company specific historical and implied volatility data, we based our estimate of expected volatility on the estimate and expected volatilities of a guideline group of publicly traded companies. For these analyses, we select companies with comparable characteristics to ours including enterprise value, risk profiles, and with historical share price information sufficient to meet the expected life of the stock-based awards. We compute the historical volatility data using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of our stock-based awards. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available. We estimate the expected term of our stock options granted to employees and non-employee directors using the simplified method, whereby, the expected term equals the midpoint of the vesting term and the original contractual term of the option. We utilize this method as we do not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. For the determination of the risk-free interest rates we utilize the U.S. Treasury yield curve for instruments in effect at the time of measurement with a term commensurate with the expected term assumption. The expected dividend yield is assumed to be zero as we have never paid dividends and do not have current plans to pay any dividends on our common stock. Historically, for periods prior to this initial public offering, the fair value of our common stock underlying our stock-based awards were determined on each grant date by our board of directors based on valuation estimates from management considering our most recently available independent third-party valuation of our equity instruments.

We determine the fair value of unvested stock based on the fair value of our common stock. In addition, for unvested stock awards under which unvested common stock is purchased by the holder with a promissory note treated as a nonrecourse note for accounting purposes, we measure the fair value of the award using the Black-Scholes option-pricing model.

Determination of fair value of common stock

As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined by our board of directors as of the date of each option grant, with input from management, considering our most recently available third-party valuations of common stock, and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent

valuation through the date of the grant. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Our common stock valuations were prepared using either an option pricing method, or OPM, or a hybrid method, both of which used market approaches to estimate our enterprise value. The OPM treats common stock and preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceeded the value of the preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. A discount for lack of marketability of the common stock is then applied to arrive at an indication of value for the common stock. The hybrid method is a probability-weighted expected return method, PWERM, where the equity value in one or more scenarios is calculated using an OPM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for the company, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock. These third-party valuations were performed at various dates, which resulted in valuations of our common stock of $3.54 per share as of February 10, 2017, $3.37 per share as of February 10, 2018, $4.64 per share as of November 30, 2018, and $7.51 per share as of January 31, 2019. In addition to considering the results of these third-party valuations, our board of directors considered various objective and subjective factors to determine the fair value of our common stock as of each grant date, including:

- the prices at which we sold shares of preferred stock and the superior rights and preferences of the preferred stock relative to our common stock at the time of each grant;
- the progress of our research and development programs, including the status and results of preclinical studies for our AXA Candidates and progress of our development of manufacturing processes;
- our stage of development and commercialization and our business strategy;
- external market conditions affecting the biopharmaceutical industry and trends within the biopharmaceutical industry;
- our financial position, including cash on hand, and our historical and forecasted performance and operating results;
- the lack of an active public market for our common stock and our preferred stock;
- the likelihood of achieving a liquidity event, such as an initial public offering, or IPO, or sale of our company in light of prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in the biopharmaceutical industry.

The assumptions underlying these valuations represented management's best estimate, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common stock and our stock-based compensation expense could have been materially different.

Once a public trading market for our common stock has been established in connection with the closing of this offering, it will no longer be necessary for our board of directors to estimate the

fair value of our common stock in connection with our accounting for granted stock options and other such awards we may grant, as the fair value of our common stock will be determined based on the quoted market price of our common stock.

The following table summarizes by grant date the number of shares subject to option awards granted between January 1, 2017 and March 22, 2019, the per share exercise price of the options, the fair value of common stock on each grant date, and the per share estimated fair value of the awards:

Grant date	Number of shares subject to award	Per share exercise price of options ($)	Per share fair value of common stock on grant date ($)[1]	Per share estimated fair value of award on grant date ($)
June 13, 2017	799,932	6.52	6.52	4.46
August 10, 2017	262,277	6.52	6.52	4.46
October 13, 2017	11,128	6.52	6.52	4.47
December 19, 2017	5,428	6.52	6.52	4.46
December 21, 2017	782,646	6.52	6.52	3.74
February 7, 2018	67,316	6.52	6.52	3.76
April 26, 2018	267,258	6.20	6.20	3.84
June 21, 2018	69,488	6.20	6.20	3.84
June 22, 2018	1,053,573	6.20	6.20	3.84
August 29, 2018	125,681	6.20	6.20	3.84
October 2, 2018	224,498	6.20	6.20	3.85
March 11, 2019	482,166	13.83	13.83	9.24
March 22, 2019	60,803	13.83	13.83	9.21

(1) Independent third-party valuations of our equity instruments were performed annually in February or when material changes in the business occurred and these were considered by the board of directors in determining the exercise price for stock-based awards. We granted options with an exercise price equal to or above the fair value of the common stock based on the most recent independent third-party valuation, at discretion of the board of directors.

Off-balance sheet arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Recently adopted and issued accounting pronouncements

A description of recently adopted and issued accounting pronouncements that may potentially impact our financial position, results of operations or cash flows is disclosed in Note 2 to our consolidated financial statements appearing at the end of this prospectus.

Quantitative and qualitative disclosures about market risks

As of December 31, 2018, we had cash, cash equivalents of $79.5 million, which consisted of cash, and money market funds. Interest income is sensitive to changes in the general level of interest rates; however, due to the nature of these investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio.

As of December 31, 2018 we had $26.0 million of borrowings outstanding under the Amended 2018 Debt Facility. An immediate 10% change in the LIBOR rate would not have had a material impact on our debt-related obligations, financial position or results of operations.

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with foreign vendors that are located in Europe and Australia. Our operations may be subject to fluctuations in foreign currency exchange rates in the future.

Inflation generally affects us by increasing our cost of labor. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2017 and 2018.

Emerging growth company status

The Jumpstart Our Business Startups Act of 2012 permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company.

BUSINESS

Overview

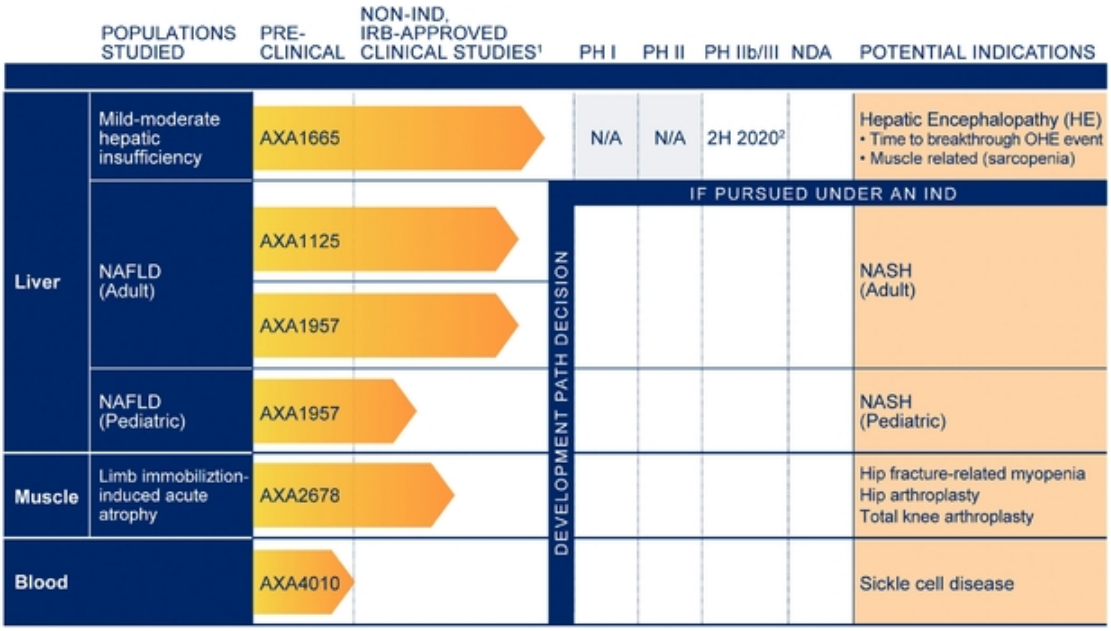
We are a biotechnology company pioneering the research and development of novel multifactorial interventions to support health and address dysregulated metabolism across a broad spectrum of consumers and patients who have limited options. Our AXA Candidates are generated from our proprietary, human-focused AXA Development Platform and harness the power of endogenous metabolic modulators, or EMMs, a broad family of molecules that fundamentally impact and regulate human metabolism.

Using our AXA Development Platform, we have rapidly generated a pipeline of AXA Candidates that are novel compositions of EMMs engineered in distinct ratios and designed to target and maximize the fundamental role that EMMs play in regulating multiple metabolic functions. Our AXA Candidates are administered orally and anchored by amino acids, which have a history of safe use as food. As such, we expect our AXA Candidates may also be combinable with other modalities. We believe our current dataset supports the potential of our AXA Candidates to support and modulate the metabolic pathways they target with favorable safety profiles. These features may make them an attractive development opportunity with significant commercial potential.

In 2018, we completed three Non-IND, IRB-Approved Clinical Studies. In all three studies, our AXA Candidates were generally found to be well tolerated, and we generated positive normal structure and function data indicating potential metabolic modulation. We believe generating this rich human dataset at this early stage of development (i) eliminates the translational uncertainty associated with transitioning from animal studies to human studies (ii) enables us to make high-insight, capital-efficient development decisions and (iii) potentially increases the probability of success for these AXA Candidates. For AXA Candidates we decide to develop under an Investigational New Drug application, or IND, we will discuss with the U.S. Food and Drug Administration, or FDA our ability to use data generated from our Non-IND, IRB-Approved Clinical Studies to begin Phase II or potential registrational (pivotal) Clinical Trials.

On March 6, 2019, we had a face-to-face pre-IND meeting with the FDA for AXA1665, our lead AXA Candidate, during which we discussed clinical endpoints, assessment tools and other matters relating to a potential IND-opening Clinical Trial for AXA1665 in hepatic encephalopathy, or HE. After meeting with the FDA, we made a decision to pursue a drug development path for AXA1665. Under the planned IND, the initial indication would be for the treatment of HE, with time to breakthrough episode of overt HE event as the primary endpoint and key secondary muscle-related endpoints. We anticipate interacting with the FDA again prior to a formal IND submission for AXA1665. We anticipate initiating a Clinical Trial in the second half of 2020 for AXA1665 that we believe could potentially serve as a registrational (pivotal) trial to support the submission of a New Drug Application, or NDA.

Our wholly-owned pipeline currently comprises five programs focused on the metabolic functions of the liver, muscle and blood. An overview of our AXA Candidate and their status is illustrated below.



(1) In the above pipeline chart, "Development Path Decision" reflects the point in a program at which we decide whether to develop an AXA Candidate as a drug product candidate under an IND, develop it as a non-drug product candidate or terminate development. We have made a decision to develop AXA1665 as a drug product candidate and anticipate interacting with the FDA again prior to submitting an IND. We have not made a development path decision for any of our other AXA Candidates.

(2) We believe that this Clinical Trial has the potential to serve as a registrational (pivotal) Clinical Trial, subject to continuing IND discussions and allowance by the FDA.

Definitions: NAFLD = non-alcoholic fatty liver disease; NASH = nonalcoholic steatohepatitis; OHE = overt hepatic encephalopathy.

AXA1665 in Hepatic Encephalopathy

In study AXA1665-001, in subjects with mild and moderate hepatic insufficiency (Child-Pugh Class A and B), receiving the higher dose of AXA1665, we observed a cumulative increase of 40% in the Fischer's ratio, or FR, the molar ratio of branched-chain amino acids, or BCAAs, to aromatic amino acids, or AAAs. These subjects also demonstrated a lower plasma ammonia area under the curve when sampled over five hours ($AUC_{0\text{-}5h}$), and tended to maintain a leaner phenotype (i.e., increase in dry lean mass and decrease in fat mass as assessed by bioimpedance measurements) and muscle function (as assessed using a liver frailty index score) compared to those on a control regimen. Each of these markers appear to have prognostic significance in assessing liver health, including in cirrhosis.

AXA1125 in Subjects with Non-alcoholic Fatty Liver Disease, or NAFLD

In study AXA1125-002, administration of AXA1125 24g three times a day was associated with a trend of decreased liver fat, improved insulin sensitivity (i.e., lower Homeostatic Model Assessment of Insulin Resistance, or HOMA-IR) and induction of fat oxidation (i.e., increased beta-hydroxybutyrate, or BHB). In subjects who received AXA1125, mean blood levels of key

fibroinflammatory biomarkers reflective of normal function tended to decrease compared to the mean pre-administration baseline levels. We believe these data suggest AXA1125 has the potential to impact three critical drivers associated with NAFLD-metabolism, inflammation and fibrosis.

AXA2678 in Healthy Subjects with Immobilization-Induced Acute Muscle Atrophy

In study AXA2678-001, in subjects receiving AXA2678 24g three times a day, we observed attenuated muscle atrophy during the one week immobilization period compared to placebo, reflected by a 76% relative difference in average muscle cross sectional area, or CSA, assessed by magnetic resonance imaging, or MRI. Subjects receiving AXA2678 tended to have muscle fiber architecture preserved during immobilization and also tended to recover muscle strength to pre-immobilization baseline levels as compared to placebo within a short two-week recovery period.

Once we have generated sufficient data for an AXA Candidate, we will decide whether to develop such candidate as a drug or non-drug product candidate. This development path decision will be based on the (i) strength of data from our Non-IND, IRB-Approved Clinical Studies, (ii) the unmet need, (iii) market opportunity and (iv) availability of accelerated paths to market under applicable regulations. To date, we have not submitted any INDs to conduct Clinical Trials with our AXA Candidates to evaluate their therapeutic potential, and while we plan to pursue the development of AXA1665 as a drug product candidate under IND, we have not yet filed such IND nor made decisions to pursue future development of any of our other AXA Candidates as drug product candidates. For AXA Candidates developed under an IND, we will discuss with the FDA our ability to use data generated from our Non-IND, IRB-Approved Clinical Studies to begin Phase II or potential registrational Clinical Trials.

We believe that the consistency and nature of the data from our three completed Non-IND, IRB-Approved Clinical Studies indicate that this approach has significant promise. We are focused on actively expanding our pipeline and currently reviewing additional development opportunities in hematology, central nervous system, or CNS, kidney and pulmonary function. Many diseases are driven by multifactorial dysregulated systemic metabolism, and we have already characterized over 50 diseases for prioritization.

We have attracted a world-class leadership team that has significant experience in the successful development and commercialization of collectively more than 50 drugs across numerous therapeutic areas. We are supported by an industry-leading board of directors and scientific advisory board. Since inception, we have raised approximately $200 million in capital from leading life sciences investors, including Flagship Pioneering, our founder, Fidelity Management & Research Company, Nestlé Health Science US Holdings and Gurnet Point Capital Limited.

Our Strengths

We believe we are well-positioned to execute on our corporate strategy based on the following competitive strengths:

- **Deep understanding of metabolic dysregulation and the modulatory power of EMMs.** We are pioneers of a novel approach that we believe can address metabolic health and dysregulation. Applying our expertise in EMMs and metabolism, we aim to harness the potential of EMMs to directly and simultaneously support and modulate multiple metabolic pathways implicated both in complex diseases and overall health.

- **Predictive, rapid and scalable human-focused AXA Development Platform.** We have built a first-of-its-kind integrated knowledge platform that has proven capable of rapidly designing our novel and proprietary AXA Candidates. Our AXA1665, AXA1125 and AXA2678 programs progressed from biological hypothesis to readout of initial Non-IND, IRB-Approved

Clinical Study data in less than 18 months. We believe that the data derived from testing these three AXA Candidates in a total of more than 160 normal structure and function assessments support the potential that our AXA Candidates may have the ability to modulate the metabolic pathways they target with favorable safety profiles.

- **Informed and capital-efficient AXA Candidate early-stage development model.** In our Non-IND, IRB-Approved Clinical Studies, we have rapidly generated a rich dataset in healthy subjects and subjects with certain disease conditions. We believe that generating human data at this early stage of development enables us to make high-insight, capital-efficient development decisions and potentially increases the probability of success for these candidates.

- **Pipeline with significant potential across a spectrum of disease and health.** We currently have five AXA Candidate programs across multiple target areas, including in liver and muscle. Our three lead programs are in large, growing and commercially attractive areas with notable unmet needs. We have already characterized over 50 additional diseases for prioritization.

- **Broad intellectual property portfolio with worldwide rights to our AXA Candidates.** As a first mover in our approach to EMMs, we are building a broad intellectual property portfolio to protect our AXA Candidates and AXA Development Platform. We believe our patents, trademarks and trade secrets will help to maintain our competitive advantage. We have full commercial rights to all of our AXA Candidates.

- **Experienced leadership team and board of directors.** We have attracted a world-class leadership team that has significant experience in the successful development and commercialization of collectively more than 50 drugs across numerous therapeutic areas. We are supported by an industry-leading board of directors and scientific advisory board. Since inception, we have raised approximately $200 million in capital from leading life sciences investors, including Flagship Pioneering, our founder, Fidelity Management & Research Company, Nestlé Health Science US Holdings and Gurnet Point Capital Limited.

Our Strategy

Our goal is to become the preeminent biotechnology company reprogramming metabolism to address a diverse set of complex diseases and support health. To achieve our goal, we are executing a strategy that includes the following core elements:

- **Accelerate paths to market for our lead programs.** For AXA Candidates we decide to develop under an IND, we will discuss with the FDA our ability to use data generated from our Non-IND, IRB-Approved Clinical Studies to begin Phase II or potential registrational Clinical Trials.

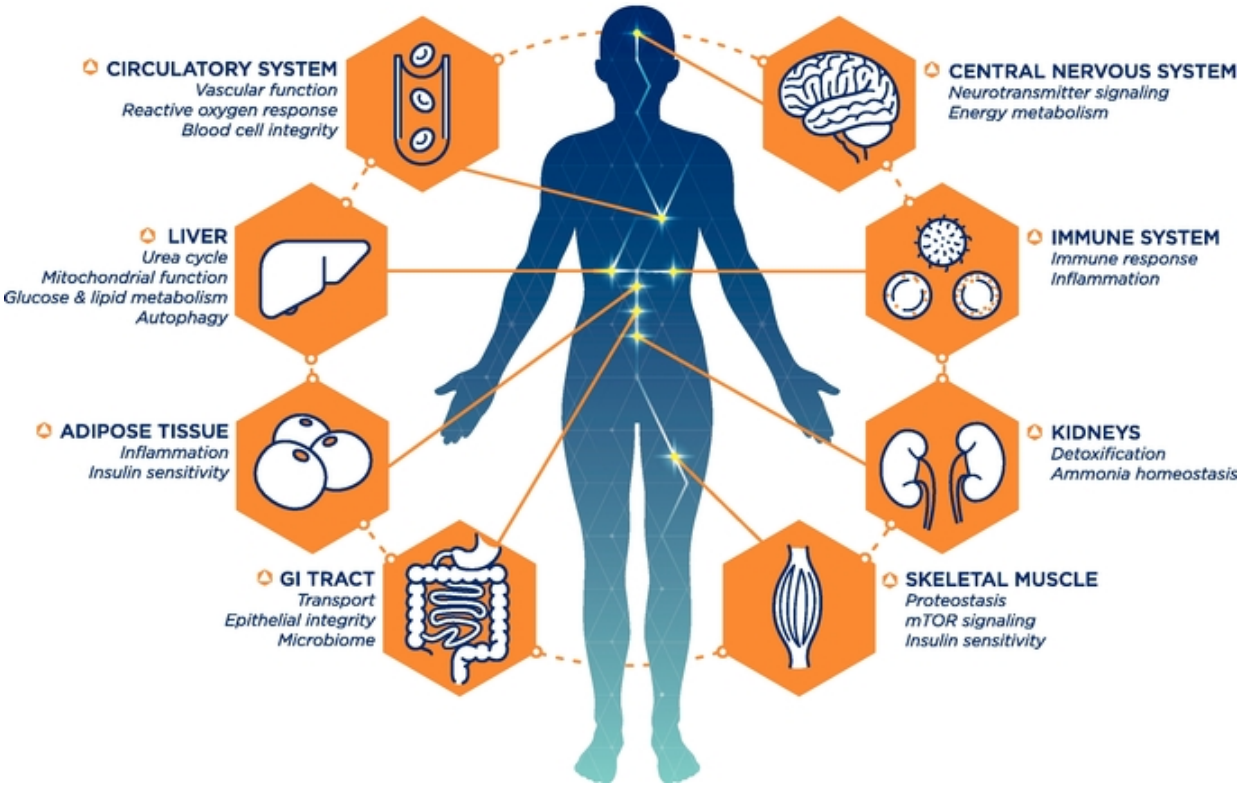
- **Further enhance and leverage our AXA Development Platform to advance multiple new programs per year in attractive markets.** We intend to continue to enhance the predictive capability of our AXA Development Platform, which is underpinned by a continuous feedback loop between our AXA Candidate design process and the data generated from our Non-IND, IRB-Approved Clinical Studies and Clinical Trials. We will prioritize attractive markets with high unmet need where our AXA Candidates have the potential to address the underlying metabolic dysregulation.

- **Expand patient and consumer access to AXA Candidates, if approved, and opportunistically leverage strategic partnerships.** Our AXA Candidates are administered orally and anchored by amino acids, which have a history of safe use as food. As such, we expect our AXA Candidates may also be combinable with other modalities. We believe such

characteristics may make them an attractive development opportunity with significant commercial potential. We will opportunistically evaluate strategic partnerships to optimally commercialize our products in large markets.

- **Establish ourselves as a global, fully-integrated biotechnology company.** We believe that building a fully-integrated company will allow us to more rapidly and efficiently develop our AXA Candidates. We will continue to bring together world-class scientific talent with experienced, innovative leaders who are focused on building an independent research, development, clinical and potentially commercial organization to realize the value of our AXA Development Platform.

Our Approach to the Development of AXA Candidates



Schematic representation of the numerous organs and systems effected by EMMs.

Our approach to the development of our AXA Candidates is based on the fundamental thesis of translating complex human biology into potential drug and non-drug product candidates that modulate metabolism. Metabolic dysregulation results from a disruption in human homeostasis that is core to optimal functioning and consequently, health. Maintenance of this dynamic state of equilibrium requires an orchestration of multiple metabolic pathways and inter-organ signaling, evolved over billions of years, and is carried out by signaling intermediates and endogenous mediators. EMMs are a critical subset of such endogenous elements that regulate metabolic function.

EMMs encompass a broad set of molecular families, including amino acids, bile acids other intermediary substrates and hormones. Together, these molecules drive a myriad of biological pathways to elicit multifactorial effects that integrate basic cellular functioning to impact fundamental biologies. Such biologies include cellular bioenergetics (e.g., tricarboxylic acid cycle and electron transport chain), nutrient handling (e.g., *de novo* lipogenesis, or metabolic formation of fat,

gluconeogenesis, or the generation of glucose from certain non-carbohydrate carbon substrates, and proteogenesis, or protein formation), nutrient sensing via master regulators (e.g., mammalian target of rapamycin, or mTOR, 5' AMP-activated protein kinase, or AMPK, fibroblast growth factor 21, or FGF21, and peroxisome proliferator-activated receptors, or PPARs), immune response and inflammation, reactive oxygen response, vascular function, neurotransmitter signaling, tissue repair, autophagy, or recycling of damaged and defective parts into basic elements thereby allowing the cells to remodel itself, and detoxification.

Dysregulation in the endogenous modulators, their signaling pathways or the metabolic processes they control can all lead to the loss of homeostasis, which can be manifested within specific organs and their sequelae, such as Type 2 diabetes, or T2D, NAFLD and muscle atrophy. As an example, dysregulation in the metabolic processes and pathways controlled by the liver such as *de novo* lipogenesis or gluconeogenesis can lead to an inability to adequately handle fuel substrates such as fats or carbohydrates. This ultimately results in fatty liver, insulin resistance and buildup of toxic waste products, such as ammonia, as the liver loses its detoxification capabilities. Similarly, a complex cascade of fuel dysregulation within skeletal muscles, a key organ involved in glucose disposal and utilization of amino acids as substrates for protein synthesis, can result in insulin resistance, intramuscular fat infiltration and muscle mass loss, thus decreasing muscle function. Muscle mass loss, or sarcopenia, is being increasingly recognized as a critical determinant of end organ function, and is linked to clinical outcomes and overall survival, such as in end-stage liver disease (cirrhotic sarcopenia) or in end-stage renal disease (uremic sarcopenia).

Complex diseases impact multiple biological pathways. Loss of health can be the direct result of metabolic pathways and functions that are not being maintained or supported. Consequently, restoring homeostasis and maintaining health requires multifactorial approaches. We believe that EMMs, anchored by amino acids, have the potential to serve as interventional candidates to address the systems-wide impact of dysregulated metabolism to support and maintain homeostasis, which helps support normal structures and functions of the body. Amino acids have decades of data supporting their role as fundamental building blocks of life. More recently, their roles in cellular signaling have become elucidated not only with individual amino acids, such as a leucine sensor on mTOR, but also in combinations, with a long history of general safe use in foods and other dietary supplements. We believe our approach with the use of EMMs has the potential to bring about a transformation in health and medicine by harnessing the body's own molecules and intrinsic chemistry to maintain or restore health.

Our Human-Focused AXA Development Platform

Our AXA Development Platform allows us to efficiently design and test AXA Candidates that simultaneously target multiple biologies and metabolic pathways by integrating advanced analytics of metabolism regulation and dysregulation with correlative-reasoning algorithms to interrogate data in our proprietary databases, which we refer to as AxcellaDB and AxcellaKB. Our proprietary human primary cell systems then directly test the multiple biologies that drive particular disease-related or metabolic dysregulation. We leverage predictive combinatorial drug metabolism and pharmacokinetics, or DMPK, analytics to inform dose-exposure relationships. All of this is supported by what we believe to be the world's leading EMM safety database. The data and learnings generated from our AXA Candidate design process further inform the design pathway, increasing the AXA Development Platform's efficiency.

Dysregulated Metabolism Data and Analytics: AxcellaDB and AxcellaKB

AxcellaDB, our proprietary database, synthesizes a combination of data from published literature and data from our proprietary models and human studies. AxcellaDB also includes key

data and analytics that are integral to our AXA Candidate design and development process, including:

- Metabolic profiles derived from a range of human plasma and specific tissue compartments, such as muscle biopsies.
- Characterization of over 50 diseases driven by multifactorial dysregulated systemic metabolism, including liver, renal, metabolic, CNS, muscle, inborn errors of metabolism and immune disorders.
- Clinical precedent using EMMs published across various public clinical trial databases and literature.
- Our proprietary EMM Safety Database, which synthesizes our own and third-party data on safety information for dose ranges for each EMM or combinations of EMM previously studied in humans.

We leverage AxcellaDB to apply internal modeling of EMM and metabolic pathways to generate hypotheses around the biological drivers of regulation and dysregulation in the human body. In the AXA Candidate design process, we use AxcellaDB's EMM safety database to determine the ranges within which individual or combinations of EMMs have been found to be tolerable with no significant safety concerns. We synthesize this information to determine the specific EMM components and proprietary composition ratios for an AXA Candidate and then test them in our studies, including in proprietary *in vitro* models and in Non-IND, IRB-Approved Clinical Studies.

We are currently building our internal machine learning capabilities, which we refer to as Axcella Knowledge Base, or AxcellaKB, to further probe novel, causal connections contained within the information and data contained in AxcellaDB. This will allow us to take a systems biology approach to AXA Candidate discovery and development. We plan to use a number of advanced approaches for network and pathway analysis of big data. Ultimately, AxcellaKB uses could include identifying AXA Candidate combinations, predicting effects of our AXA Candidates, use of causal reasoning to identify new target areas for our technology and novel biology.

Human Primary Cell Systems

We test hypothesized EMM mechanisms, synergies and dis-synergies in normal and disease-specific human primary cell models. We conduct our model systems in environments that aim to simulate physiological levels of biofluids and nutrients. These models include multiple cell types that we use to deconstruct dysregulated metabolism or disease conditions to isolate effects of AXA Candidates and EMMs on subsets of metabolic pathways. The high throughput of these models enables us to test AXA Candidates as well as combinations of the individual constituents to identify, better understand and incorporate their synergistic and dis-synergistic interactions.

Predictive Combinatorial DMPK

The designed AXA Candidate components, amounts and ratios are subsequently refined using pharmacokinetic, or PK, and pharmacodynamic modeling that evaluates EMM plasma exposure, supra-physiological exposures, windows of exposure administration amounts, the characterization of critical PK behaviors across molecule classes and the implications of physiological compartmental distribution. We believe these data allow us to highly refine AXA Candidates based on the variability among each AXA Candidate's individual constituents.

Axcella Health and Flagship Pioneering

We were founded in 2008 by Flagship Labs, the institutional innovation foundry of Flagship Pioneering. A team comprising innovators and entrepreneurs Dr. David Berry, Dr. Geoffrey von Maltzahn and Dr. Noubar Afeyan started exploring the potential medical applications of diverse, orally-consumed proteins. The work of this team led to the understanding that specific amino acid combinations at different ratios have the potential to drive profound biological effects in both health and disease by impacting dysregulated metabolic pathways and multi-compartmental biology. With this transformational new approach, we are harnessing the potential for orally-administered EMM compositions to drive cellular biology in a specific and reproducible manner, with the goal of delivering safe interventions for patients or consumers.

Regulatory Landscape and Our AXA Candidate Development Framework

Regulatory Status of Our AXA Candidates

Under the FDA's September 2013 Guidance for Clinical Investigators, Sponsors, and IRBs entitled "*Investigational New Drug Applications (INDs) — Determining Whether Human Research Studies Can Be Conducted Without an IND*," we believe Non-IND, IRB-Approved Clinical Studies of food products may be conducted to study the safety, tolerability, effect on normal structure or function in humans. We believe such Non-IND, IRB-Approved Clinical Studies may also be conducted to characterize the mechanism by which such product acts to maintain such structure or function, including in individuals with disease conditions, as long as the assessment is not intended to evaluate the non-drug product candidate's ability to diagnose, cure, mitigate, treat or prevent a disease. Studies specifically intended to assess the potential therapeutic use of a product candidate, such as its ability to diagnose, cure, mitigate, treat or prevent disease, including effects on an abnormal and uncommon or serious condition, must be done under an IND. Initially, we are generally studying our AXA Candidates as non-drug product candidates, and in particular, as food for humans in Non-IND, IRB-Approved Clinical Studies. The EMM constituents of our AXA Candidates generally fall under the food category. They are based on combinations of amino acids and their natural derivatives, which are all naturally occurring in the body and generally in the foods we eat. Based on our large database of studies and scientific literature on human exposure to and safety profiles of amino acids, the FDA's food additive regulations authorizing the use of specific amino acids under certain conditions as safe and permissible food additives when used as nutrients, our own data on amino acids used in AXA Candidates, and the fact that we use amino acids in our AXA Candidates within amounts previously studied in humans in doses found to be tolerable with no significant safety concerns, we believe we can and have studied our AXA Candidates in Non-IND, IRB-Approved Clinical Studies as foods and not as drug product candidates under an IND.

Our Non-IND, IRB-Approved Clinical Studies for AXA Candidates

In our Non-IND, IRB-Approved Clinical Studies for our AXA Candidates, we evaluate their safety and tolerability, effect on the normal structures or functions of the human body, and characterize the mechanism by which AXA Candidates act to maintain such structure or function. Such a study may be performed in individuals with disease for purposes of evaluating safety and tolerability. For example, in AXA1125-002, in addition to the primary endpoint of safety and tolerability, we included as secondary assessments normal liver structure assessments (e.g., liver fat) and normal liver functional assessments (e.g., alanine aminotransferase, or ALT, monocyte chemoattractant protein-1, or MCP-1 and N-terminal type III collagen propeptide, or Pro-C3, which are markers of changes in inflammation and fibrosis). The protocols for Non-IND, IRB-Approved Clinical Studies, which include detailed rationale and descriptions of the composition of amino acids in our AXA Candidates, are approved by IRBs at the various sites and conducted consistent

with Good Clinical Practices, or GCP. To date, our Non-IND, IRB-Approved Clinical Studies have been exploratory in nature and conducted in small numbers of subjects. We conduct Non-IND, IRB-Approved Clinical Studies for our AXA Candidates in both healthy subjects and subjects with specific disease conditions to maximize interpretability and accuracy of data on safety, tolerability and physiological impact on normal structures and functions of the body given that some of these changes may not be in healthy populations. The learnings from these studies inform the evolution of the AXA Candidate design and our AXA Development Platform. Armed with the deep scientific knowledge and understandings from all of our studies for each AXA Candidate, we believe that we will be able to make a highly informed development path decision for each AXA Candidate. For example, we made our first drug development path decision for AXA1665 after assessing AXA1665 Non-IND, IRB-Approved Clinical Study data and having a pre-IND meeting with the FDA on March 6, 2019 on a potential IND development path for AXA1665.

Development Path Decisions for AXA Candidates

The development path decision for an AXA Candidate, based on data from our Non-IND, IRB-Approved Clinical Studies and other data and market analyses, includes (i) exploring the AXA Candidate's therapeutic potential for a disease or disorder in Clinical Trials, (ii) developing the AXA Candidate as a non-drug product or (iii) terminating development efforts for such AXA Candidate. The development path decision we make for an AXA Candidate will also determine the appropriate market channel. For AXA Candidates we decide to develop under an IND, as we have done with AXA1665, we will discuss with the FDA our ability to use data generated from our Non-IND, IRB-Approved Clinical Studies to begin Phase II or potential registrational Clinical Trials.

In the context of further developing an AXA Candidate as a non-drug product, we anticipate that we would look to partner with third parties. Once a formulation for commercialization is finalized, we would expect that either we or our partner would comply with all applicable regulatory requirements applicable to the chosen commercialization path and market (e.g., submitting a notification to the FDA that the product is generally recognized as safe and effective, or GRAS, or, in the case of a dietary supplement, confirmation that it contains permissible dietary ingredients or file a new dietary ingredient filing, if necessary).

Our AXA Candidates

Key Characteristics of Our AXA Candidates

Our AXA Candidates are novel compositions of EMMs engineered in distinct ratios, with the objective of maximizing the fundamental role that EMMs play in regulating metabolic function. Our AXA Candidates have the following characteristics:

- **Multifactorial:** Our AXA Candidates are designed to simultaneously target multiple pathways that if not maintained, or dysregulated, can lead to disease or loss of health.
- **Safety Precedent:** Our AXA Candidates are anchored by amino acids, which have a history of use as foods without significant safety concerns.
- **Combinable:** We believe the EMM constituents in our AXA Candidates, due to their favorable safety profiles based on our own and third-party data to date, may enable us to combine them with other modalities. This could potentially further expand our market opportunity.
- **Oral Administration:** Our AXA Candidates are supplied in a dry powder form for oral consumption after being mixed with water, which we believe increases convenience of administration.

- **Rapidly Designed and Scalable:** We have produced human study-ready product in less than three months from initial biological hypothesis generation. Our AXA Candidates are produced using third-party manufacturers that employ readily and widely available clinical-grade materials. We believe that manufacturing will be both scalable and cost-effective.

- **Novel and Proprietary:** We own full commercial rights to our AXA Candidates, which are protected by our expanding patent estate, trade secrets and know-how.

AXA Candidates that Target Liver Metabolism

The liver has many critical functions relating to metabolism, including detoxification of a variety of substances, synthesis of key proteins and other intermediary substrates impacting nutrient homeostasis to coagulation, lipid metabolism and amino acid regulation. Hepatic insufficiency can lead to the loss of health and consequently to disease. We are investigating our AXA Candidates for safety, tolerability and physiological effects across a spectrum of normal liver structures and functions in our Non-IND, IRB-Approved Clinical Studies. Our AXA Candidates may have potential for development under an IND as drug product candidates with applications in conditions from non-alcoholic steatohepatitis, or NASH, to certain complications of cirrhosis. While the pathologies of NAFLD, NASH and cirrhosis may manifest primarily in the liver, these are heterogeneous and systemic diseases that affect multiple biological pathways impacting nearly every important organ system within the body, namely skeletal muscle, the heart, kidney, brain and gut.

AXA1665 Program in Hepatic Encephalopathy

Overview

On March 6, 2019, we had a face-to-face pre-IND meeting with the FDA for AXA1665, our lead AXA Candidate, during which we discussed clinical endpoints, assessment tools and other matters relating to a potential IND-opening Clinical Trial for AXA1665. Among other feedback received, the FDA indicated that (i) our proposal to proceed with the proposed IND-opening Clinical Trial without conducting additional toxicology studies appears to be acceptable, (ii) our drug product development approach with respect to chemistry, manufacturing and controls, or CMC, including analytical methods, microbial tests and specification to control the quality of AXA1665 appear reasonable for this stage of the product development, and (iii) the FDA requested we provide the relevant CMC and other information with the initial IND submission package in a Drug Master File, or DMF. After meeting with the FDA, we made a decision to pursue a drug development path for AXA1665 for HE. Under the planned IND, the initial indication would be for the treatment of HE, with time to breakthrough episode of overt HE event as the primary endpoint and key secondary muscle-related endpoints. We anticipate interacting with the FDA again prior to a formal IND submission for AXA1665. We anticipate initiating a Clinical Trial in the second half of 2020 for AXA1665 that we believe could potentially serve as a registrational (pivotal) trial to support the submission of a New Drug Application, or NDA.

To date, we have completed one Non-IND, IRB-Approved Clinical Study, AXA1665-001, in subjects with mild and moderate hepatic insufficiency to assess AXA1665's safety, tolerability and physiological effects on normal structures and functions. We did not observe any significant safety issues with AXA1665, and it was generally well-tolerated in this population over 15 days across two periods. Secondary assessments suggest that subjects administered AXA1665 tended to maintain a leaner phenotype as observed by changes in body composition and clinically relevant functional parameters.

In 2019, prior to making a drug development path decision for AXA1665, we initiated a 12-week (with a four-week follow-up), randomized, placebo-controlled, Non-IND, IRB-Approved Clinical Study, AXA1665-002, in approximately 60 mild and moderate hepatic insufficiency (Child-Pugh Class A and B) subjects to extend the initial observations from AXA1665-001 and further characterize the safety, tolerability and physiological effects on normal structure and function over this longer duration.

AXA1665 Design Rationale

AXA1665 has been designed to target multiple metabolic pathways intersecting key organ systems (liver, muscle and gut) to maintain liver health.

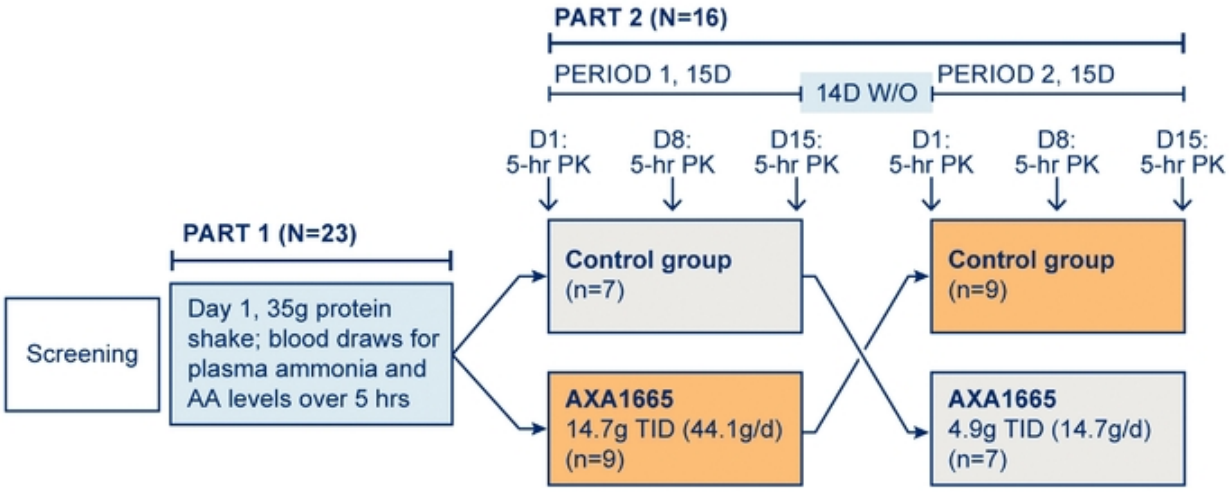
Underlying Biology	**Design Objectives**
Plasma amino acid imbalance	Maximize proteogenesis and reduce systemic AAAs
Ammonia handling	Stimulate urea cycle function, intestinal and renal nitrogen metabolism, and induce intramuscular ammonia detoxification
Muscle wasting	Increase muscle protein synthesis by addressing metabolic demand and stimulating mTORC1

Completed AXA1665-001 Non-IND, IRB-Approved Clinical Study

Study Design

AXA1665-001 was a two-part, 15-day controlled crossover Non-IND, IRB-Approved Clinical Study to assess the safety, tolerability and physiological impact on the normal structures and functions of the liver in subjects with mild and moderate hepatic insufficiency. The purpose of Part 1 was to evaluate the response (i.e., tolerability, changes in endogenous amino acid levels and plasma ammonia) to a standard 35-gram protein shake. A total of 23 subjects participated in Part 1. Part 2 utilized a crossover design comprising two 15-day periods separated by a 14-day washout. Subjects were domiciled in the Clinical Research Unit during each period, but not during the washout. Sixteen subjects participated in Part 2. All subjects in Part 2 were provided with well-balanced protein rich meals, a daily exercise regimen of 30 minutes of walking at their usual pace per day (other than on Days 1, 8, and 15), and a daily mandatory late-evening snack, or LES. LES alone has been shown in previous studies to reverse anabolic resistance and sarcopenia of cirrhosis with improved quality of life parameters in cirrhotic patients. The 16 subjects in Part 2 were split into two groups: Group 1 (n=7) received only LES in Period 1 and, after a 14-day washout, received AXA1665 4.9g three times a day, or TID (14.7g/day) and LES in Period 2; Group 2

(n=9) received AXA1665 14.7g TID (44.1g/day) and LES in Period 1 and, after a 14-day washout, received only LES in Period 2.



Study design for AXA1665-001, a two-part, 15-day controlled crossover Non-IND, IRB-Approved Clinical Study to assess the safety, tolerability and physiological impact of AXA1665 on the normal structures and functions of the liver in subjects with mild and moderate hepatic insufficiency (Child-Pugh Class A and B). AA=amino acid; D=day; W/O=washout.

Study Assessments in AXA1665-001

Primary Safety & tolerability	Clinical adverse events, or AEs, vital signs, electrocardiograms, or ECGs, clinical laboratory parameters, including standard chemistry and hematology panels, plasma ammonia, albumin, total protein and other liver function tests
Secondary PK of AXA1665 constituents and endogenous amino acid levels Physiological assessments	FR* Valine-to-phenylalanine ratio, or VPR* Normal Structure • Body composition via bioimpedance to assess lean and fat mass compartments Normal Function • Handgrip strength, timed chair stands and balance to derive a composite score called the liver frailty index, or the LFI*

* FR, VPR and LFI are believed to have prognostic significance in subjects with cirrhosis and end-stage liver disease based on emerging scientific literature.

Study Results

Primary

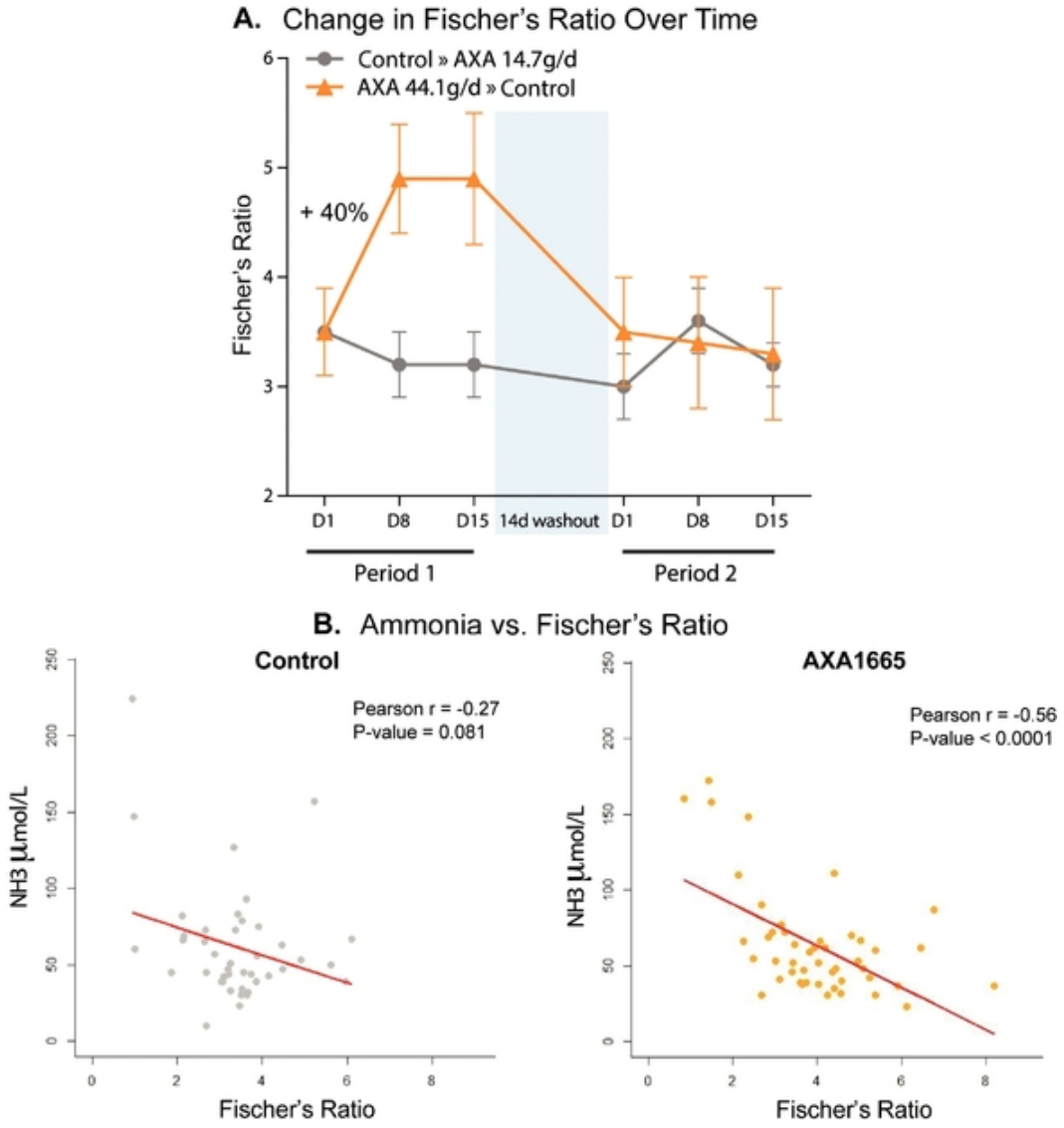
Daily consumption of AXA1665 at both the 14.7g/day and 44.1g/day amounts for 15 days was well-tolerated. There were no study product-related AEs observed in Part 1. There were five AEs, specifically neck pain, posterior shoulder pain, gastroesophageal reflux disease, or GERD, rib pain and headache, observed in four subjects in Part 2, all of which were considered unlikely to be related to the study food product. Three of these AEs were observed while subjects were consuming the LES and two, GERD and headache, were observed when subjects were consuming AXA1665. Four of the AEs were mild, and GERD was of moderate intensity. None were serious or led to discontinuation from the study. All the AEs resolved spontaneously, including GERD, which self-resolved within a day. There were no clinically significant changes in vital signs, ECGs or standard clinical laboratory parameters, including plasma ammonia, albumin, total protein, creatinine or blood urea nitrogen.

Secondary

Consumption of AXA1665 resulted in supra-physiological plasma concentrations of the constituent amino acids within AXA1665. These changes in amino acids resulted in a 40% increase in the basal (fasted) FR as early as Day 8 and maintained to Day 15, as compared to Day 1 baseline. These changes were markedly diminished once the 44.1g/day amount of AXA1665 in Period 1 was washed out over 14 days. The same subjects were then re-baselined and placed on the control regimen for 15 additional days in Period 2. A similar pattern of change was also seen in VPR.

We observed a statistically significant inverse correlation between basal (fasted) plasma ammonia and basal (fasted) FR with AXA1665 administration, but not in the control group, suggesting a potential ammonia dampening effect of AXA1665. Despite the provision of additional nitrogen via amino acids within AXA1665, the mean plasma ammonia area under the curve ($AUC_{0\text{-}5h}$) decreased by an average of approximately 10-25% on Day 15 compared to Day 1 in both

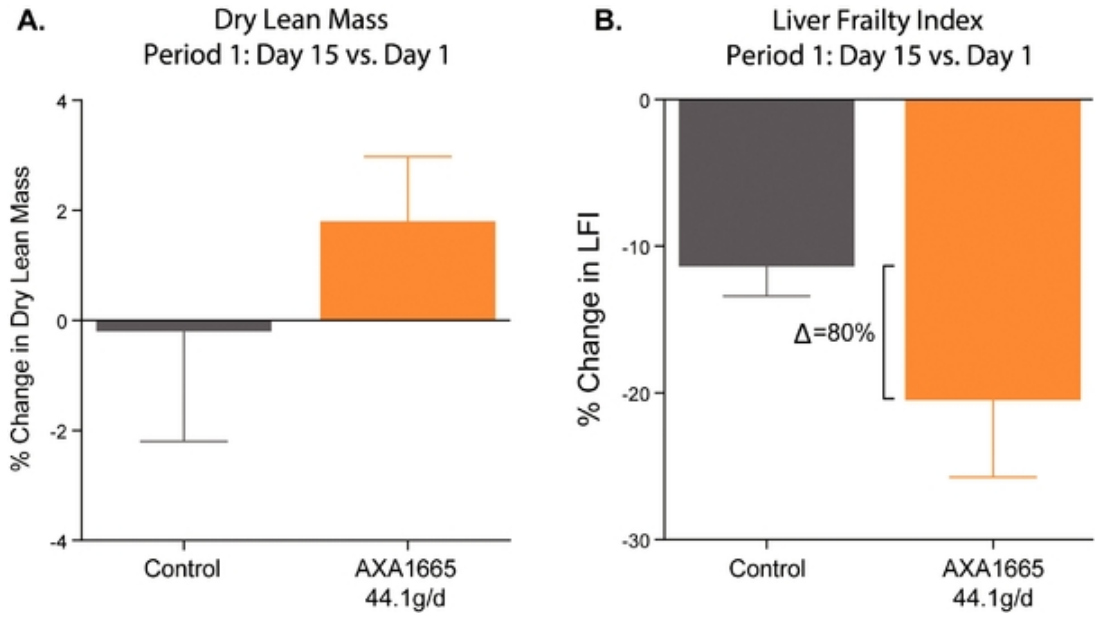
the 44.1g/day and 14.7g/day AXA1665 groups. We believe these findings are notable given that prior studies showed protein meals or BCAA mixtures by themselves increased plasma ammonia.



(A) Change in the basal (fasted) FR over time in both Periods 1 and 2 of Part 2 of the Non-IND, IRB-Approved Clinical Study of AXA1665. Data represent mean +/- standard error of the mean, or SEM; control, or CTRL à AXA 14.7g/d (n=7); AXA 44.1g/d à CTRL (n=9); (B) Inverse correlation between basal plasma ammonia and corresponding FR; p-value determined using Pearson correlation of basal plasma ammonia and basal plasma FR values from the D1, D8 and D15 fasted samples from the control and AXA1665 treated groups. In these figures g/day means grams per day and D means study Day. p-value is a statistical calculation that relates to the probability that the difference between groups happened by chance, with a p-value of less than 0.05 (i.e., less than 5% probability that the difference happened by chance) generally being used as the threshold to indicate statistical significance.

Clinical observations indicated that subjects consuming the higher amounts of AXA1665 (44.1g/day), but not the lower amount (14.7g/day), generally tended to maintain or increase their lean body compartments (dry lean mass, lean body mass and skeletal muscle mass), with a concomitant decrease in fat mass from baseline levels, with no overall change in body

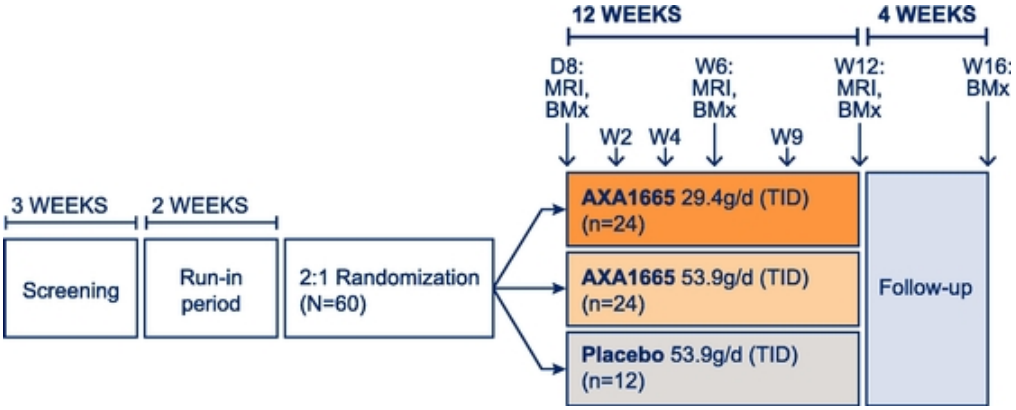
weight. These structure-related findings suggest alteration of the body composition toward a leaner phenotype, a key indicator of potential healthy muscle gain. Functional assessments included measurements of handgrip strength, timed chair stands and balance, from which the LFI composite score was derived. The average LFI score directionally moved toward less frailty in those consuming AXA1665 44.1g/day, but not in those consuming 14.7g/day AXA1665, nor in LES alone. A relative difference of 80% between the control and 44.1g/day AXA1665 groups was observed at the end of the 15-day administration period. We believe this finding is notable because LES alone appears to favorably impact sarcopenia in subjects with cirrhosis. Therefore, we believe an observed relative difference of this magnitude, even within this relatively short duration, warrants continued investigation of these effects in a longer duration study. Both the structural and functional changes were diminished once the 44.1g/day amount of AXA1665 in Period 1 was washed out over 14 days. The same subjects were then re-baselined and placed on the control regimen for 15 additional days in Period 2.



Percentage change in Day 15 compared to Day 1 values of (A) Dry lean mass and (B) LFI from Period 1 of Part 2 of the Non-IND, IRB-Approved Clinical Study of AXA1665. Values represent mean +/- SEM; Control (n=7) and AXA1665 44.1g/day (n=9). In these figures g/d means grams per day.

On-Going AXA1665-002 Non-IND, IRB-Approved Clinical Study

In 2019, prior to making a drug development path decision for AXA1665 we initiated AXA1665-002, a 12-week (with a four week follow-up), randomized placebo-controlled, Non-IND, IRB-Approved Clinical Study to assess AXA1665's safety, tolerability and physiological impact on normal liver and muscle structures and functions in approximately 60 subjects with mild and moderate hepatic insufficiency (Child-Pugh Class A and B) to further extend the findings from AXA1665-001. We anticipate the data readout from this study in the first half of 2020.



Study design of AXA1665-002, a 12-week (with a four-week follow-up), randomized placebo-controlled, Non-IND, IRB-Approved Clinical Study to assess the safety, tolerability and physiological impact on normal liver and muscle structures and functions of AXA1665 in subjects with mild and moderate hepatic insufficiency (Child-Pugh Class A and B). W=week; g/d=grams per day; BMx=biomarkers.

Study Assessments in AXA1665-002

Primary Safety & tolerability	Clinical AEs, vital signs, ECGs, clinical laboratory parameters, including standard chemistry and hematology panels, plasma ammonia, albumin, total protein and other liver function tests
Secondary PK of AXA1665 constituents and endogenous amino acid levels	FR* and VPR*
Physiological assessments	Normal Structure • Body composition via MRI to assess lean and fat mass compartments, including thigh muscle volumes, intramuscular fat Normal Function • Physical (LFI*; gait speed) • Cognitive (Stroop test; PHES; CFF) • Health-related questionnaires

* FR, VPR and LFI are believed to have prognostic significance in subjects with cirrhosis and end-stage liver disease based on emerging scientific literature. PHES = psychometric hepatic encephalopathy score; CFF = critical flicker frequency.

AXA1665 Market Opportunities

Overview of HE

Under our planned IND for AXA1665, the initial indication would be for the treatment of HE, with time to breakthrough episode of overt HE as the primary endpoint and key secondary muscle-related endpoints. HE has multiple precipitating factors, such as amino acid imbalance, ammonia handling, muscle wasting, infections and constipation. Emerging data suggest that muscle mass and function are key independent factors associated with progression to and severity of HE.

Long-term damage to the liver from various causes, such as alcohol, hepatitis B or C viral infections, NASH or autoimmune hepatitis, can lead to permanent scarring, a condition called cirrhosis. Prevalence of cirrhosis in the United States is approximately 0.27%, corresponding to approximately 633,000 adults, of which 69% reported that they were unaware of having liver disease. Transition from compensated cirrhosis to decompensated cirrhosis occurs at a rate of approximately 5% to 7% per year.

Decompensated cirrhosis is a serious systemic disease with multi-organ dysfunction, resulting in a variety of significant complications, including HE, bacterial infections, gastrointestinal bleeding, renal impairment and ascites, which is the build-up of excess fluid in the abdomen. Presence of sarcopenia, which affects approximately 40% of patients with cirrhosis being evaluated for liver transplant, independently worsens many of these complications, results in more hospital stays and is becoming increasingly recognized as a complication of cirrhosis. The presence of complications decreases median survival to approximately two years versus approximately 12 years without complications.

HE is a common complication of cirrhosis, with an estimated prevalence of overt HE of 10% to 14% in the general cirrhotic population and 16% to 21% in those with decompensated cirrhosis. Overt HE refers to the evidence of neurological abnormalities without specialized psychometric testing. By contrast, minimal or covert HE requires specialized testing for its diagnosis, including psychometric tests. HE is a type of metabolic encephalopathy with a multifactorial etiopathogenesis, and including dysregulated amino acid metabolism and neurotransmitter signaling, inability of the failing liver to clear toxins such as ammonia and microbiome changes, all of which ultimately result in diminished brain function. Consequently, HE is well-established as a significant cause of morbidity and mortality in the cirrhotic population and represents a significant unmet medical need.

Muscle depletion and muscle fat infiltration independently increase the risk of both overt and minimal HE with increased mortality. Decline in muscle mass can also hamper the alternative pathway of ammonia detoxification, and hyperammonemia may further worsen sarcopenia, generating a vicious cycle. The highly interdependent complications of cirrhosis, sarcopenia and HE, constitute a significant disease burden and can have an impact on irreversible morbidity and mortality in cirrhotic patients. We estimate that approximately 63,000 to 130,000 individuals may be affected at any given time with both cirrhotic sarcopenia and overt HE in the United States.

We believe that therapeutic strategies that simultaneously address both sarcopenia and ammonia detoxification will clinically improve outcomes, such as maintaining remission of HE in those with prior overt HE and/or reduce rates of other cirrhosis complications, and ultimately mortality. Thus, under an IND, we propose to develop AXA1665 to treat either or both complications of cirrhosis, specifically HE and sarcopenia.

Limitations of Current Therapies for HE

There are currently no treatment options that directly address the multifactorial drivers of HE in an integrated manner. There is a significant unmet need for approved medications to reverse and

resolve the hepatic as well as systemic effects (e.g., muscle and gut) of HE. There are currently three main approved therapies for the treatment of HE: lactulose, rifaximin and HepatAmine.

- Lactulose is a non-absorbed disaccharide that has been used for several decades to reduce hyperammonemia in cirrhotic patients. For maximum clinical effect, lactulose must be titrated to cause two to four loose stools per day. The resulting diarrhea and fecal urgency, as well as other adverse gastrointestinal events, significantly limits patient compliance. Because of the variability in individual response, patients are often instructed to self-regulate dosing, which may lead to noncompliance and, in some cases, additional complications, including aspiration, dehydration, hypernatremia, and severe perianal skin irritation.

- Rifaximin is an antibiotic derived from rifamycin that impacts gut microbiota and likely indirectly influences ammonia generation by the gut flora. It is prescribed for the secondary prophylaxis of HE as a tablet twice per day, although caution is advised for patients with very advanced liver disease due to increased systemic absorption.

- HepatAmine is an intravenous administered amino acid composition, which is used in patients who are malnourished and unable to consume normal proteins or diets orally. In a Phase II/III Clinical Trial, HepatAmine reversed the abnormal plasma amino acid pattern characterized by decreased levels of BCAAs and elevated levels of AAAs, as compared to neomycin, which was used as an active control. The trend towards normalization of these amino acids was generally associated with an improvement in mental status and electroencephalogram, or EEG, patterns in a majority of patients, including a reduction in mortality.

Despite these treatment options for HE, none are adequate to directly address the multifactorial etiology, complexity, severity and sequelae of HE. This is either because the existing treatment options do not sufficiently and comprehensively address the underlying pathophysiologic drivers such as amino acid dysregulation, ammonia detoxification and sarcopenia, or they fail to pre-empt the cascading series of events leading to HE, such as altered metabolic signaling, or combinations thereof. Based on the evidence to date, sarcopenia is not only an effect-modifier of HE but also an independent complication of cirrhosis itself, impacting overall morbidity and mortality of cirrhotic patients. Consequently, new approaches are warranted that can adequately and directly address sarcopenia, a critical driver of the multifactorial pathogenesis of HE and other complications in cirrhotic patients. Based on our data to date, we believe that AXA1665 may have the potential to address the aforementioned multiple drivers of HE.

AXA1125 Program

Overview

AXA1125 is an AXA Candidate that is also being evaluated in liver health. To date, we have conducted Non-IND, IRB-Approved Clinical Studies of AXA1125 in healthy subjects in AXA1125-001 and in T2D subjects with NAFLD in AXA1125-002. In both studies, the safety, tolerability and physiological effects on normal structures and functions were assessed. We did not observe any significant safety issues with AXA1125, and it was generally well tolerated in these studies. In AXA1125-002, we observed that administration of AXA1125 tended to impact normal liver structure (as measured by average fat content) and function (e.g., plasma ALT, other markers of inflammation and fibrosis and mediators of glucose and lipid homeostasis). In 2019, we initiated a 16-week Non-IND, IRB-Approved Clinical Study, AXA1125-003, to extend the initial observations from AXA1125-002 and further characterize the safety, tolerability and physiological effects on normal structure and function over this longer period and in approximately 105 adult NAFLD subjects.

AXA1125 Design Rationale

AXA1125 has been designed to target multiple pathways (metabolism, inflammation and fibrosis) intersecting key organ systems (liver, muscle, adipose and gut) to maintain liver health.

Underlying Biology	**Design Objectives**
Metabolism	Lower lipotoxicity, improve insulin sensitivity and maximize mitochondrial function by enhancing fatty acid beta-oxidation
Inflammation	Modulate macrophage function, reduce hepatic inflammatory mediators and improve gut epithelial integrity
Fibrosis	Reduce hepatic stellate cell activation and proliferation to decrease hepatic fibrogenesis

Completed AXA1125-001 Non-IND, IRB-Approved Clinical Study

AXA1125-001 was a two-part study in normal healthy subjects. Part 1 included ten subjects administered a single 24g amount of AXA1125 either 30 minutes before meal (fasted), 30 minutes after meal (fed) or two hours after meal (fed) to assess the effect of meal timing on plasma concentration and exposure of AXA1125 amino acid constituents. Part 2 included ten subjects administered 24g TID (72g/day) for six weeks to assess the safety and tolerability at these amounts over this duration in healthy subjects.

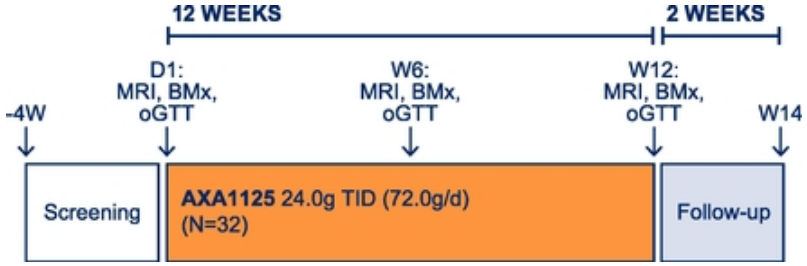
In Part 1, the PK data suggested well-behaved PK of the complex amino acid mixture with all of the constituents achieving supra-physiologic concentrations as well as no meaningful impact of meal timing on the amino acid profiles. Average peak amino acid plasma concentrations, or Cmax, and total exposure, or AUC, values were generally similar (within 20% to 30%) and sequentially increased with each AXA1125 consumption regardless of meal timing, with the exception of glutamine. Consumption of AXA1125 in the fed state, either 30 minutes or two hours after a meal, resulted in a 1.1 to 2.5-fold increased AUC of glutamine relative to when AXA1125 was consumed in the fasted state.

In both Parts 1 and 2, we did not observe any significant safety issues with AXA1125 and it was generally well tolerated with no clinically significant changes in clinical laboratory values, vital signs or ECGs. There were no product-related AEs in Part 1. In Part 2, mild gastrointestinal symptoms of abdominal pain, distention and diarrhea were noted in three of ten subjects, considered by the study investigator to be study product-related. All AEs were transient, self-limited and resolved with no intervention, and none were serious or led to discontinuation.

Completed AXA1125-002 Non-IND, IRB-Approved Clinical Study

Study Design

AXA1125-002 was a pilot, single-arm, open-label, 12-week (with a two-week follow-up) multicenter, Non-IND, IRB-Approved Clinical Study to assess the safety, tolerability and physiological impact of AXA1125 24g TID (72g/day) on the normal liver structures and functions in subjects with T2D and NAFLD.



Study design of AXA1125-002, a single-arm, pilot, open-label, 12-week (with a two-week follow-up), multicenter, Non-IND, IRB-Approved Clinical Study of AXA1125 in subjects with T2D and NAFLD. W=week; D=day; BMx=biomarkers; oGTT=oral glucose tolerance test.

Study Subject Baseline Demographics and Disposition

A total of 32 subjects were enrolled, of which 50% were males and 50% were females. Subjects were an average age of 51 years, weight of 105.3 kg, body mass index of 38.6 kg/m^2 and hemoglobin A1c, or HbA1c, of 8%. Subjects with stable chronic disease (i.e., T2D, hypertension and hyperlipidemia) were maintained on their usual standard of care therapies on a stable regimen for the duration of treatment. Of these 32 subjects, 23 received AXA1125 24g TID and had baseline MRI proton density fat fraction, or PDFF, greater than 10%, five received AXA1125 24g TID with baseline MRI PDFF less than 10% (i.e., a total of 28 subjects received AXA1125 24g TID) and four received AXA1125 6g TID (an error in study product administration at one study site). In the subgroup that received AXA1125 24g TID with baseline MRI PDFF greater than 10% (n=23), there were a total of nine early terminations: four between Day 1 and Week 6, and five between Week 6 and Week 12. Discontinuations were due to a combination of withdrawal of consent (n=2), lost to follow up (n=1) and AEs (n=6). Of the six AE-related reasons for early termination, only one AE (diarrhea, prior to Week 6 visit) was considered as possibly related to study product. One subject was removed from the analysis of the physiological assessments because the subject missed several study product administrations and was noncompliant during the study. In addition, one subject had no Week 6 MRI due to poor image quality. Consequently, sample sizes vary at the indicated time points due to subject attrition, discontinuation and availability of the corresponding data at those time points.

Study Assessments in AXA1125-002

Primary Safety & tolerability	Clinical AEs, vital signs, ECGs, clinical laboratory parameters, including standard chemistry and hematology panels, other liver function tests (e.g., ALT, AST)
Secondary Basal levels of endogenous amino acids and amino acids within AXA1125	Amino acid levels
Physiological assessments	Normal Structure • MRI-PDFF assessment of liver fat Normal Function • Metabolism — glucose (FPG, HbA1c, oGTT, insulin and HOMA-IR) and lipid (BHB, FAO markers, lipid panel) homeostasis assessments • Inflammation — apoptosis (e.g., CK-18) and macrophage (e.g., MCP-1) markers • Fibrosis — fibrogenic (e.g., Pro-C3) markers

FPG: fasting plasma glucose; oGTT: oral glucose tolerance test; FAO: fatty acid oxidation; HOMA-IR: Homeostatic Model Assessment of Insulin Resistance; BHB: beta-hydroxybutyrate; CK: cytokeratin; MCP: monocyte chemoattractant protein; Pro-C3: N-terminal type III collagen propeptide.

Study Results

Primary

We did not observe any significant safety issues with AXA1125 and it was generally well tolerated. The rates and patterns of the observed AEs in this study appear to be consistent with the typical rates and patterns observed generally in clinical studies with T2D and NAFLD subjects. The most common AEs were diarrhea (in seven subjects) and headache (in six subjects). There were two serious AEs reported, acute cholecystitis and right toe infection, which were deemed not reasonably associated with the study product, and more likely due to subject's preexisting conditions as these complications are quite common with underlying obesity and T2D. A total of six subjects discontinued from the study due to an AE. These were diarrhea (n=2), cholecystitis (n=1), stomach flu (n=1), hyperglycemia (n=1) and right toe infection (n=1). Of these six AEs leading to discontinuation, only one (diarrhea, prior to the Week 6 study visit) was considered as possibly related to the study product. All AEs, except the two serious AEs, generally resolved without intervention. There were no clinically significant abnormalities in physical examination, body weight, vital signs, ECG and standard clinical safety laboratory (chemistry and hematology) parameters over the 12-week duration.

Secondary

Pharmacokinetic changes

Only basal (fasted) levels of amino acids were measured in this study. The average plasma concentration of the individual amino acid constituents within AXA1125 in all subjects receiving 24g

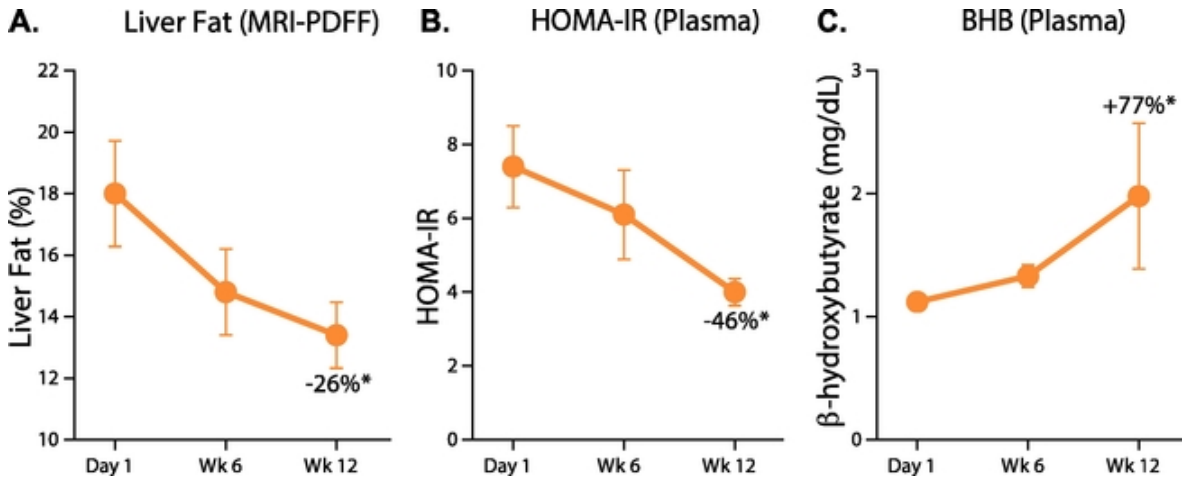
TID AXA1125 in the basal state (i.e., before ingestion of AXA1125) were not meaningfully changed over the course of the 12-week study, as compared to baseline (Day 1) levels.

Physiologic Assessments

Metabolism

Lipotoxicity and insulin resistance are generally regarded as metabolic drivers of NAFLD. AXA1125 24g TID administration for 12 weeks tended to decrease the mean liver fat content, and the average directional change over this period tended to be 26% lower compared to subjects' corresponding Day 1 baseline values in those with a baseline PDFF greater than 10%. Measures of glucose homeostasis revealed no changes in FPG, HbA1c or in glucose AUC during oGTT with AXA1125 administration. However, after 12 weeks of AXA1125 24g TID administration, HOMA-IR (–46%) and plasma insulin in both the fasted (–38%) and prandial states (i.e., after a glucose bolus in the setting of an oGTT, –19%), tended to decrease as compared to the corresponding mean pre-administration baseline levels in those with a baseline PDFF greater than 10%. We believe these results suggest a potential insulin sensitizing effect of AXA1125 as lower amounts of insulin are needed to maintain the same glucose level and/or an enhanced insulin clearance effect.

Effects on both liver fat and HOMA-IR were observed without any accompanying body weight changes despite the provision of approximately 496 kcal/day via AXA1125 daily administrations. Subjects did not drastically alter their usual dietary or physical activity patterns during the 12-week study period. Thus, the observation of body weight neutrality under these circumstances suggests a likely change in nutrient utilization, implicating increased fat oxidation. Assessment of the fasting lipid profile revealed no changes in mean serum levels of total cholesterol, HDL-C, and nonesterified fatty acids, with trends toward slight decreases in LDL-C and triglyceride, or TG, levels. However, beta hydroxybutyrate, or BHB, tended to directionally increase by 77% as compared to the corresponding mean pre-administration baseline levels in those with a baseline PDFF greater than 10% suggestive of a mild-to-moderate ketotic state. A systematic plasma lipidomic profiling indicated a tendency to upregulate acylcarnitines, a marker of mitochondrial fatty acid oxidation, and downregulate toxic lipid species such as ceramides, sphingomyelins and long-chain TGs. Taken together, we believe these results suggest that AXA1125 over 12 weeks tended to impact lipid handling (lowered liver fat and raised BHB), and insulin sensitivity (lowered HOMA-IR and plasma insulin).



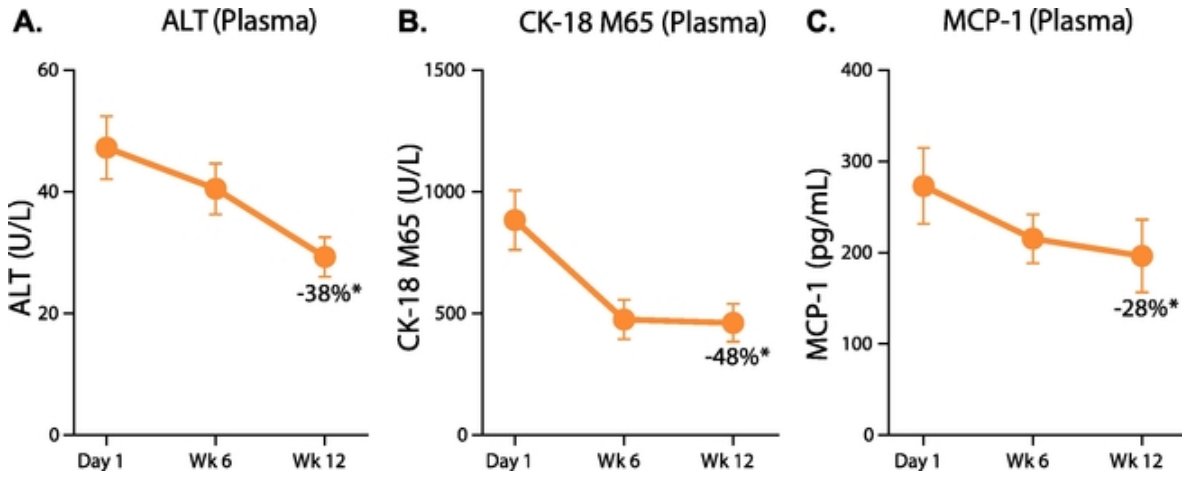
*(A) Liver fat content (n=22, day 1; n=17, week 6; n=13, week 12); (B) Plasma HOMA-IR (n=22, day 1; n=19, week 6; n=13, week 12); (C) Plasma beta-hydroxybutyrate (n=22, day 1; n=20, week 6; n=13, week 12) levels at baseline (day 1), week 6, and week 12 in the Non-IND, IRB-Approved Clinical Study of AXA1125. * denotes the relative directional percentage change trend over the 12-week administration period. Values represent mean ± SEM from subjects administered AXA1125 24g TID with Day 1 PDFF greater than 10%. Wk=week.*

The clinical observations of a decreasing trend in liver fat in subjects with T2D and NAFLD were followed up with cell-based experiments in primary human hepatocytes. In these *in vitro* experiments, AXA1125 attenuated the saturated fatty acid and tumor necrosis factor alpha mediated increase in lipid droplet accumulation, as well as induced a microsteatotic phenotype.

Inflammation

After 12 weeks of AXA1125 24g TID administration, mean blood levels of key inflammation biomarkers (ALT, CK-18 M65 and MCP-1) were directionally decreased by –38%, –48%, and –28%, respectively, as compared to their corresponding mean pre-administration baseline levels in those with a baseline PDFF greater than 10%. Changes in AST were also directionally consistent with that seen in ALT.

In previous studies, decreases in CK-18 have been associated with improvements in ballooning on liver histology, and decreases in MCP-1 have also been observed with thiazolidinediones, statins and angiotensin-converting-enzyme inhibitor treatments.



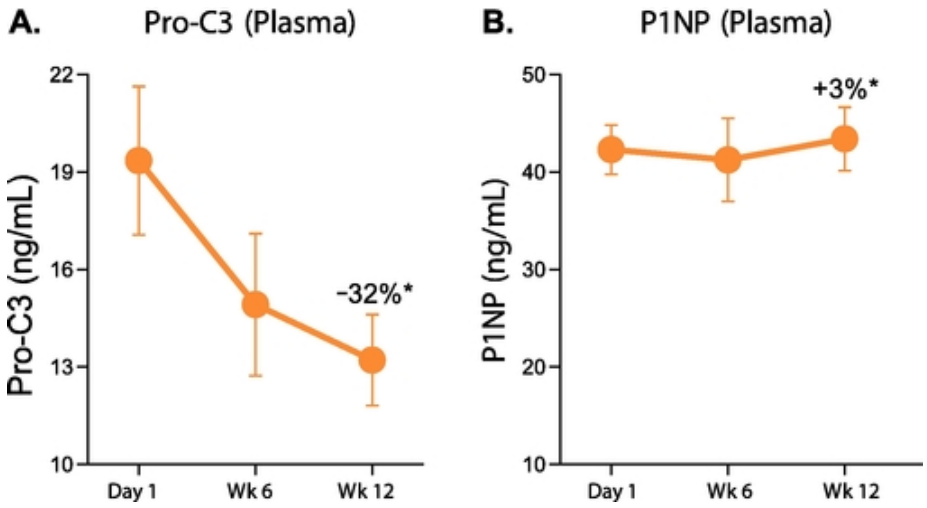
*(A) Plasma ALT (n=22, Day 1; n=20, week 6; n=13, week 12); (B) Plasma CK-18 M65 (n=22, Day 1; n=14, week 6; n=13, week 12); (C) Plasma MCP-1 (n=22, Day 1; n=19 , week 6; n=13, week 12) levels at baseline (Day 1), week 6, and week 12 in the Non-IND, IRB-Approved Clinical Study of AXA1125. * denotes the relative directional percentage change trend over the 12-week administration period. Values represent mean ± SEM from subjects administered AXA1125 24g TID with Day 1 PDFF greater than 10%. Some of the CK-18 M65 and MCP-1 values were below the limit of detection for these assays; hence, the lower limit of the detection values were used for the analysis. Wk means week.*

The clinical observations of a decreasing trend in plasma MCP-1 in subjects with T2D and NAFLD were followed up with cell-based experiments in primary human macrophages. In these *in vitro* experiments, AXA1125 suppressed lipopolysaccharide, or LPS-induced tumor necrosis factor alpha, or TNFa, production from M1 macrophages, and simultaneously increased the anti-inflammatory cytokine, CCL18, in interleukin-4 treated M2 macrophages, both in a dose-dependent manner. Based on additional experiments interrogating cellular energetics, we believe one way in which AXA1125 exerts its potential anti-inflammatory effects is by reducing glycolytic adenosine triphosphate, or Glyco-ATP, while maintaining mitochondrial and total ATP levels within the macrophage.

Fibrosis

Changes in a key hepatic fibrogenic marker, Pro-C3, were assessed after 12 weeks of AXA1125 24g TID administration. Levels of Pro-C3 in the blood correlate with the NAFLD Activity Score on histology as well as to liver-related clinical outcomes. There was a directional trend to

decrease Pro-C3 by –32% as compared to its corresponding mean pre-administration baseline levels in those with a baseline PDFF greater than 10%. Baseline values of Pro-C3 in three subjects were greater than three standard deviations above the mean, i.e., greater than 60 ng/mL to as high as 78 ng/mL and are considered as outliers. One subject had a baseline Pro-C3 of 9.9 ng/mL, experienced a serious AE of acute cholecystitis, stopped taking AXA1125 at the end of Week 1 and then discontinued from the study on Week 5. The subject's end-of-study Pro-C3 level was 31.9 ng/mL, reflecting both the acute inflammatory state (i.e., acute cholecystitis) and also likely a washout effect of AXA1125. In light of this clinical circumstance, Pro-C3 data from this subject were excluded. We believe changes in the pro-collagen markers were specific to those that indicate a change in hepatic fibrogenesis (i.e., Pro-C3) versus a more generalized change because total procollagen type 1 N-terminal propeptide, or P1NP, considered to be a sensitive marker of bone turnover was not affected (+3%) by AXA1125. We believe these observations suggest that AXA1125 may selectively impact hepatic fibrogenesis.



*(A) Plasma Pro-C3 (n=19, Day 1; n=13, week 6; n=16, week 12); (B) Plasma P1NP (n=22, Day 1; n=14, week 6; n=19, week 12) levels at baseline (Day 1), week 6, and week 12 in the Non-IND, IRB-Approved Clinical Study of AXA1125. * denotes the relative directional percentage change trend over the 12 week administration period. Values represent mean ± SEM from subjects administered AXA1125 24g TID with Day 1 PDFF greater than 10%. Wk means week.*

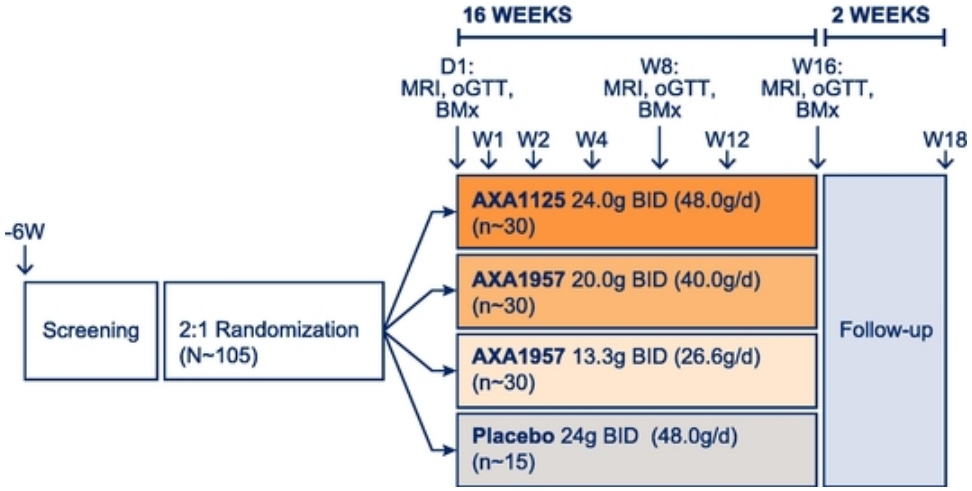
We followed up the clinical observation of a decreasing trend in plasma Pro-C3 in subjects with T2D and NAFLD with cell-based experiments in primary human stellate cells. These *in vitro* experiments revealed that AXA1125 suppressed Pro-C3 protein secretion in the transforming growth factor beta, or TGFb-activated stellate cells, and also reduced gene expression of heat-shock protein 47, a molecular chaperone required for collagen maturation, in a dose-dependent manner. Effect of AXA1125 was specific to stellate cell activation and proliferation as indicated by both a dose-dependent suppression of alpha-smooth muscle actin (a marker of stellate cell activation) and a concomitant decrease in EdU (a marker of cell proliferation).

Acknowledging the limitation of a small sample size, we believe this pilot food study also suggests a dose response effect of AXA1125, as the lower amount (6g TID; n=4) of AXA1125 yielded little to no change in the above physiological liver structure and function parameters.

Overall, we believe the weight of evidence from these results support the directional consistency of changes across both the structural and functional markers associated with liver health.

Ongoing Non-IND, IRB-Approved Clinical Study — AXA1125-003

In January 2019, we initiated AXA1125-003, a 16-week (with a two-week follow-up), randomized, single-blind, placebo-controlled, dose-ranging, Non-IND, IRB-Approved Clinical Study to assess safety, tolerability and physiological impact on the normal structures and functions of the body in approximately 105 adult subjects with NAFLD. A few key features of this study design include administration of two different AXA Candidates, AXA1125 and AXA1957, a calorie-matched placebo control, two different amounts of AXA1957 and twice a day, or BID, regimen. We anticipate data readout from this study in the second half of 2020.



Study design for AXA1125-003, a 16-week (with a two-week follow-up), randomized, single-blind, placebo-controlled, dose-ranging, Non-IND, IRB-Approved Clinical Study. W=week; BMx=biomarkers; MRI: magnetic resonance imaging; oGTT=oral glucose tolerance test.

Study Assessments in AXA1125-003

Primary Safety & tolerability	Clinical AEs, vital signs, ECGs, clinical laboratory parameters, including standard chemistry and hematology panels, and other liver function tests
Secondary PK of AXA1125 constituents and endogenous amino acid levels	Amino acid levels
Physiological assessments	Normal Structure • MRI-PDFF and corrected T1, or cT1 assessment of liver fat and fibroinflammation Normal Function • Metabolism — glucose (FPG, HbA1c, oGTT, insulin, HOMA-IR) and lipid (BHB, FAO markers, lipid panel) homeostasis assessments • Inflammation — apoptosis (e.g., CK-18) and macrophage (e.g., MCP-1) markers • Fibrosis — fibrogenic (e.g., Pro-C3) markers

FPG: fasting plasma glucose; oGTT: oral glucose tolerance test; FAO: fatty acid oxidation; HOMA-IR: Homeostatic Model Assessment of Insulin Resistance; BHB: beta-hydroxybutyrate; CK: cytokeratin; MCP: monocyte chemoattractant protein; Pro-C3: N-terminal type III collagen propeptide.

AXA1125 Potential Product Development Paths and Market Opportunities

Potential Drug Development Path

Overview of NAFLD and NASH

The treatment of NASH is a potential indication if we elect to further develop either AXA1125 or AXA1957 or both under an IND. While the pathologies of NAFLD and NASH manifest primarily in the liver, they are systemic diseases driven by multifactorial systemic dysregulation of pathways associated with metabolism, inflammation and fibrosis. Dysfunctional lipid metabolism associated with insulin resistance and hepatocyte lipotoxicity increases liver cell death. Systemic and chronic inflammation at the cellular and cytokine level drives tissue damage and activates fibrogenic pathways. Activation of stellate cells then causes accumulation of collagen in the liver and leads to progressive fibrosis.

NAFLD is one of the most common causes of liver disease in the United States. NAFLD is characterized by excess fat accumulation in the liver, typically resulting from obesity, insulin resistance and diabetes. NAFLD in children is often more severe than in adults.

NAFLD can progress to NASH, which is characterized by necroinflammation and fibrosis, and may ultimately lead to life threatening conditions such as cirrhosis or liver cancer requiring liver transplant. With a U.S. prevalence of over 15 million people, and similar prevalence in the EU5 (France, Germany, Italy, Spain and the United Kingdom), NASH represents a substantial public health issue and thus burden on the overall healthcare system. Incidence is expected to continue increasing in parallel with the obesity and T2D epidemics.

Limitations of Current Therapies

Currently, there is no drug therapy approved to treat NAFLD or NASH. A combination of dietary modifications and increased physical activity remains the standard of care for management of NAFLD and NASH. Weight loss decreases diabetes risk and can also improve liver health. Additionally, a cornerstone of treatment of NASH is management of comorbidities. Off-label use of certain medications is outlined in most leading clinical practice guidelines.

The complex pathophysiology of NASH and the events that contribute to the advancement of the disease towards cirrhosis and liver cancer has led to a consensus that efficacious pharmacological therapies will likely require simultaneous modulation of metabolic dysfunction, inflammation and fibrosis. The current pipeline of third-party products under development includes product candidates across multiple mechanisms of action. These candidates are mostly designed against specific targets, rather than a systemic, multifactorial approach that can target the central nodes of the disease in a highly coordinated manner. Clinicians and biopharmaceutical companies are increasingly evaluating combination approaches to address the broad spectrum of the disease, improve response rates and expand the size of the treatable population. However, significant challenges to this approach include the potential compounding of overlapping side effect profiles and high development and treatment costs. Additionally, there are limited programs in development for pediatric patients.

We believe that an effective interventional approach to NASH should be intrinsically combinable, multifactorial and safe. Based on our data to date and the focus of other approaches outlined above, we believe there are important differentiated product development opportunities for both AXA1125 and AXA1957.

Potential Non-Drug Development Path

If we decide to develop AXA1125 and/or AXA1957 as a non-drug product, the target market would be defined by, or with input from, a strategic partner and supporting data from our current or additional studies. We believe a non-drug pathway could also represent a significant market opportunity given the potential benefits to generally healthy people that could result from supporting normal liver structures and functions, including when the liver may come under stress from common activities such as exposure to alcohol, heavy metals, and high caloric loads. Under a non-drug development path, we or a strategic partner may decide to develop AXA1125 and/or AXA1957 as a dietary supplement to maintain liver health.

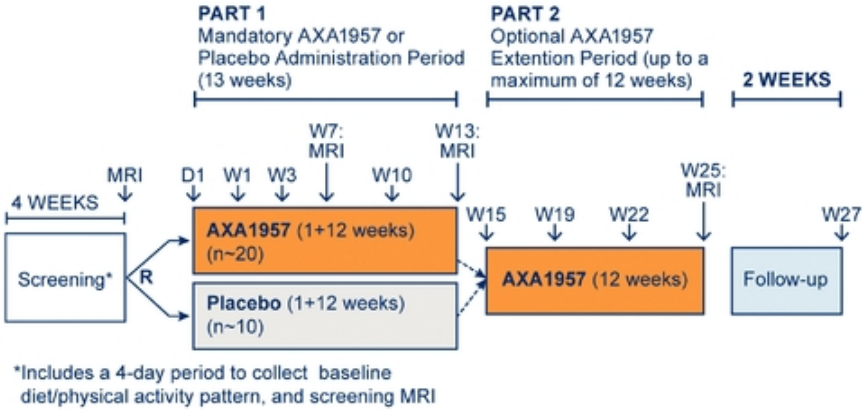
AXA1957 Program

Based on recent scientific publications in the NAFLD field, as well as our own learnings and insights from AXA1125-002 and our AXA Development Platform, we designed and developed AXA1957 with the potential to address additional biologies and populations, including pediatrics. There are no approved pharmacologic treatments and few pipeline programs targeting pediatric NASH. Recommended treatment guidelines currently center around lifestyle interventions.

Planned Non-IND, IRB Approved Clinical Study

In the second half of 2019, we plan to initiate AXA1957-002, a placebo-controlled, single-blind, randomized (2:1) controlled study to assess safety and tolerability and the physiological impact of AXA1957 on the normal structures and functions of the liver in approximately 30 adolescent

(12-17 year old) subjects with NAFLD. We anticipate data readout from this study in the second half of 2020.



Study design for the planned AXA1957-002, a placebo-controlled, single-blind, randomized (2:1) controlled Non-IND, IRB-Approved Clinical Study with up to 25 weeks of administration in 12-17 year old adolescent subjects with NAFLD.

AXA2678 Muscle Program

Overview

AXA2678 is our AXA Candidate being evaluated in muscle health. To date, we have conducted two Non-IND, IRB-Approved Clinical studies of AXA2678 in healthy subjects. AXA2678-001 was a 28-day study in healthy subjects with immobilization-induced acute muscle atrophy to assess safety, tolerability and physiological effects on normal muscle structures and functions. AXA2678-002 was a one-day study in healthy subjects to assess the plasma PK of a 24g amount of AXA2678 when administered two hours after standardized breakfast, lunch and dinner.

In both studies, we did not observe any significant safety issues with AXA2678 and it was generally well tolerated. In AXA2678-002, the average plasma concentrations of the constituent amino acids within AXA2678 increased above endogenous baseline levels in response to AXA2678 administration in all three meal periods with similar area under the curve, or AUC, and maximum serum concentration, or Cmax, and had a median time after administration of approximately two hours, suggesting a consistent PK profile of the constituent amino acids after administration. Additional observations in AXA2678-001 revealed a 76% relative difference in the average muscle cross-sectional area between placebo and AXA2678 groups during the seven-day period of unilateral limb immobilization. We are continuing to evaluate next steps for this program. One potential study design being considered is a placebo-controlled, dose-ranging randomized control trial in subjects undergoing hip arthroplasty. Subjects would receive approximately one week of treatment prior to their elective surgery and up to 12 weeks afterwards in a controlled rehabilitation setting. The potential study endpoints would assess safety, tolerability, muscle mass and function.

AXA2678 Design Rationale

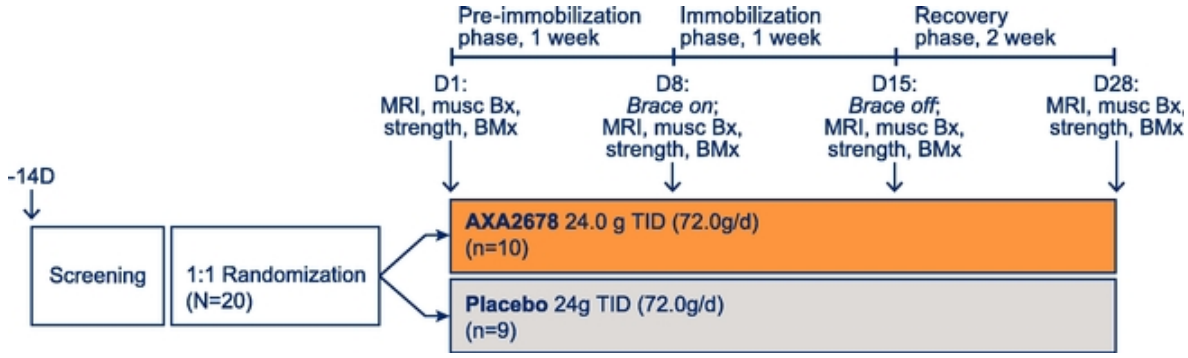
AXA2678 has been designed to target multiple biological pathways with the goal of maintaining normal muscle structure and function, especially in states of disuse atrophy.

Underlying Biology	**Design Objectives**
Proteostasis and anabolic resistance	Optimize the balance between muscle anabolism via increased muscle protein synthesis and catabolism via decreased muscle protein breakdown Improve muscle insulin resistance Stimulate mitochondrial function and provide anaplerotic substrates for the TCA cycle
Inflammation and vasodilation	Decrease inflammation cascades and reactive oxygen species
Defective myogenesis, the process of forming skeletal muscle fibers, and regeneration	Activate stem cells for muscle growth and differentiation

Completed AXA2678-001 Non-IND, IRB-Approved Clinical Study

Study Design

AXA2678-001 was a four-week, randomized, placebo-controlled, double-blind, Non-IND, IRB-Approved Clinical Study to assess the safety, tolerability and physiological impact on the normal muscle structure and functions in 20 healthy subjects undergoing one week of unilateral limb immobilization. The 28-day study consisted of a screening visit, a seven-day pre-immobilization period, a seven-day immobilization period and a 14-day recovery period. Subjects had their dominant leg immobilized by means of a knee brace. Ten subjects received AXA2678 and the other ten received a non-proteinaceous placebo, or maltodextrin, which was color- and excipient-matched, with a similar caloric content to AXA2678. Administration amounts were 24g TID or 72g/day for 28 days to be taken between meals. Subjects' daily meals were standardized with a set protein content of 1g/kg per day for the full 28-day duration of the study.



Study design of AXA2678-001, a four-week, randomized, placebo-controlled, double-blind Non-IND, IRB-Approved Clinical Study evaluating the safety, tolerability and physiological effects of AXA2678 on muscle structure and function. D=day; musc Bx=muscle biopsy; BMx=biomarkers.

Study Assessments in AXA2678-001

Primary Safety & tolerability	Clinical AEs, vital signs, ECGs, clinical laboratory parameters, including standard chemistry and hematology panels
Secondary Physiological assessments	Normal Structure • Muscle MRI for CSA, Mvol, IMF • Muscle biopsy for fiber type assessments Normal Function • Strength, activity, electrical impedance myography and biomarkers of muscle biology, inflammation and myokine signaling

CSA: cross sectional area; MVol: muscle volume; IMF: intramuscular fat fraction

Study Results

Twenty subjects were enrolled in the study (n=10 per group). All 20 subjects were male, with an average age of 21.8 years and an average weight of 82.7 kg. Subjects in the two groups had similar age and similar mean weight. However, one subject in the placebo group had a body weight of 135kg, resulting in an inadequate bracing of his knee, and consequently, removed from the secondary analyses. Therefore, the analysis set for secondary muscle structure and function assessments are nine subjects in the placebo group and ten subjects in the AXA2678 group.

Primary

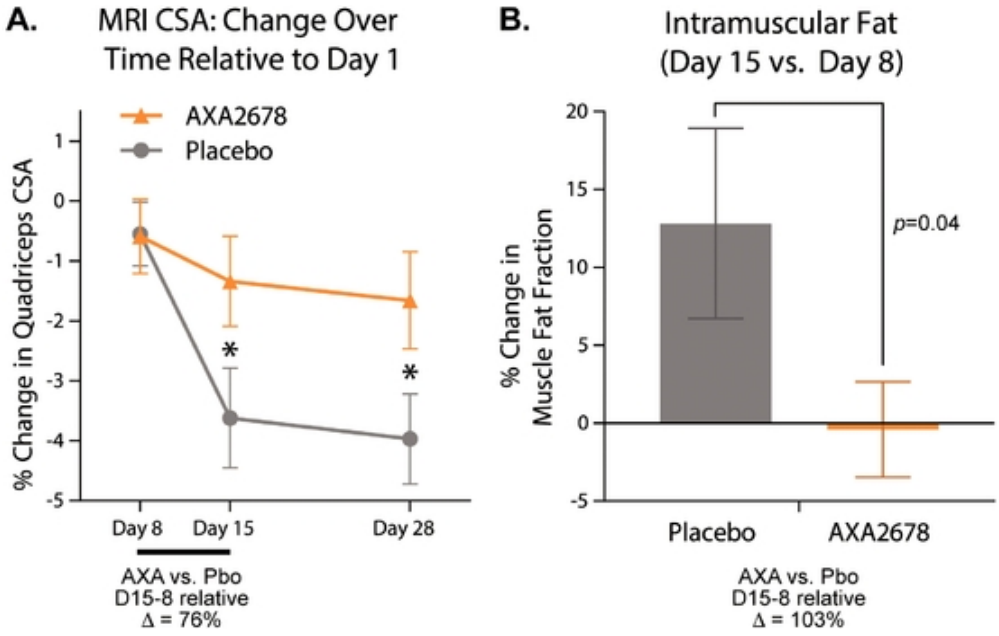
Daily consumption of AXA2678 at 24g TID (72 g/day) was well-tolerated and no significant safety issues were observed over the 28-day administration period. Two AEs of sore throat were reported in one subject on two separate occasions (on Day 6, and then again on Day 22) taking AXA2678. Another two AEs of stomach gas and nausea were reported simultaneously in one subject taking AXA2678 on Day 12. These AEs were also mild, self-resolving within one day and noted as possibly related to AXA2678 administration. No other AEs were reported with AXA2678 or with placebo. There were no clinically meaningful changes noted in routine safety labs (chemistry, hematology), vital signs or ECG.

Secondary

Changes in CSA, MVol and IMF by MRI

In the placebo group, average quadriceps peak CSA declined significantly during immobilization (Day 15 versus Day 8 CSA: –3.1% ($p<0.05$)). By > contrast, CSA was preserved with AXA2678 (Day 15 versus Day 8 CSA: –0.75% ($p>0.3$)), a relative difference of 76% between the two groups during this one-week immobilization period. We believe these findings indicate that AXA2678 administration prevented the loss of muscle mass during disuse. The magnitude of quadriceps CSA loss seen in the placebo group over the seven-day period is consistent with that observed in published studies (3% to 5% loss, typically 0.5% per day). A similar pattern was also observed in the quadriceps muscle volume (Day 15 versus Day 8 Mvol: –2.4% ($p<0.05$) in the placebo group versus Mvol: –0.67% ($p>0.3$) in the AXA2678 group) indicating the attenuation of Mvol loss during disuse with AXA2678 administration. MRI images were subjected to a third-party unbiased and independent analysis to confirm the MRI findings. The independent analysis

confirmed the muscle CSA and Mvol changes and also revealed attenuation of intramuscular fat fraction during immobilization in the AXA2678 group compared to placebo. The relative percentage change in quadriceps muscle fat fraction after one-week of immobilization (Day 15 versus Day 8) was 103% in the immobilized leg (p=0.04). We believe these findings imply a likely impact of AXA2678 on insulin sensitization as insulin resistance has been closely associated with increased muscle fat infiltration. The aforementioned analyses are not materially affected even when including data from the excluded outlier subject.



*(A) Percentage change in quadriceps cross sectional area in the immobilized leg over 28 days compared to Day 1 in AXA2678 (n=10) and placebo (n=9) administered groups. Values represent mean ± SEM. * denotes a statistically significant reduction in muscle CSA in the placebo group during immobilization and recovery relative to Day 1 by repeated measures Analysis of Variance (p<0.05). By contrast, muscle CSA did not significantly change in the AXA2678 group during the study. (B) Percentage change in intramuscular fat fraction within the immobilized quadriceps during one week of immobilization (Day 15 vs. Day 8) in AXA2678 (n=9) and placebo (n=8) administered groups. Values represent mean ± SEM.; p-value obtained using an unpaired t-test with Welch's correction.*

Muscle fiber type changes

Immunohistochemical analysis of muscle fibers within the vastus lateralis did not reveal baseline (pre-immobilization) differences in fiber CSA or fiber type distribution between placebo and AXA2678 treated groups. Immobilization did not result in a statistically significant reduction of fiber CSA in either type I or type II fibers; however, there was a trend toward the preservation of type I fibers with AXA2678 treatment as reflected by a decreasing trend in the percentage of type I fibers in the placebo, but not the AXA2678 group.

Changes in strength, activity and electrical impedance myography

At baseline, no differences in muscle strength were observed between groups in peak knee extensor isometric torque or time to peak torque. Immobilization (Day 15 vs. Day 8) resulted in equivalent relative declines in peak torque in both groups (placebo: –7.5 ± 17.5%; AXA2678: –6.7 ± 15.1%); however, at Day 28, the peak isometric torque tended to be higher in the AXA2678 administered group as compared to the placebo group, suggesting recovery of strength to pre-immobilization baseline levels even within a relatively short two week period. Time to peak torque tended to be faster with AXA2678 administration as compared to placebo during both immobilization and recovery periods. We believe the faster average time to peak torque observed in the AXA2678 group may reflect preservation of muscle fibers and potentially an enhancement in muscle perfusion.

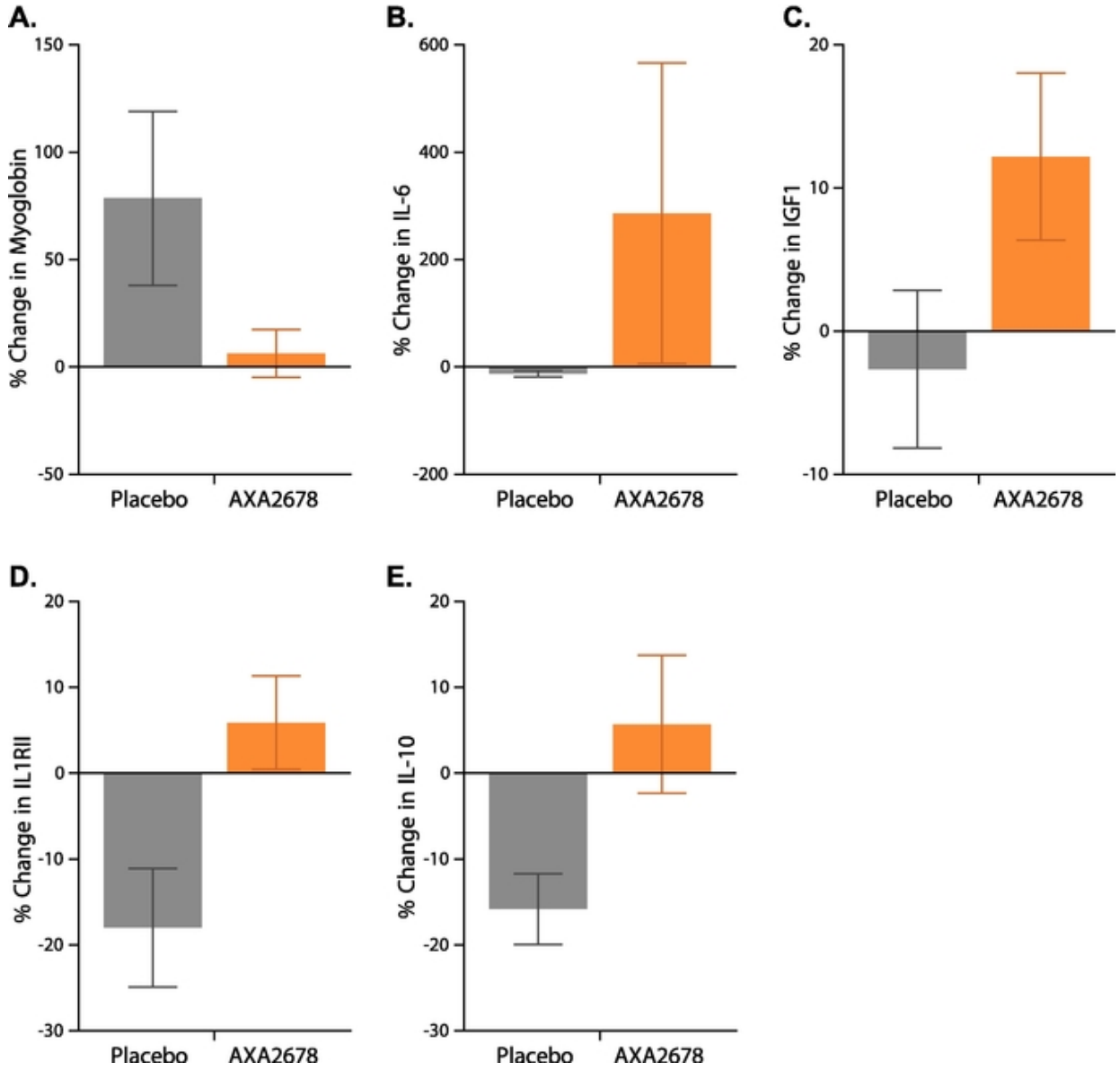
As expected, activity (steps per day) substantially decreased with limb immobilization in both groups. Between Day 8 and Day 15 there was no statistically significant correlation between the percentage change in mean CSA and the percentage change in mean activity in either group. At the end of the recovery period, as expected, activity in both groups had returned toward their respective pre-immobilization levels with no significant differences between the two groups.

Normally, immobilization-induced atrophy results in a decrease in key electrical impedance myographic parameters such as phase, maximum reactance and reactance slope, all of which were decreased as expected in the atrophied limb of the placebo group. These parameters, considered to be reflective of muscle health, tended to increase and were preserved with AXA2678 administration during immobilization, with relative differences versus placebo ranging from +115% to +155%. We believe these findings are consistent with the anti-atrophic effect of AXA2678, thereby reflecting a potentially healthier muscle even during an acute insult of limb disuse.

Changes in key biomarkers of muscle biology, myokine signaling, and inflammation

A preliminary analysis of various biomarkers associated with muscle biology, myokine signaling, and inflammation revealed several important patterns of change. Myoglobin levels, suggestive of muscle breakdown and shown to be increased in blood during immobilization, tended to increase as expected in the placebo group. By contrast, AXA2678 administration tended to attenuate this increase consistent with its anti-atrophic effect and suggests a possible attenuation of muscle breakdown during disuse. AXA2678 administration tended to increase the plasma levels of both IL-6 (myokine associated with myogenic differentiation, insulin-mediated glucose disposal, fatty acid oxidation), and IGF-1 (anabolic hormone that stimulates muscle hypertrophy and insulin sensitivity). Taken together, we believe these preliminary findings suggest AXA2678 may coordinately improve anabolism and insulin sensitivity during the immobilized period.

Subjects administered AXA2678 tended to maintain plasma levels of IL1R2, a soluble receptor that acts as an IL-1 decoy, reducing IL-1-mediated inflammation, during immobilization, and concomitantly tended to maintain plasma IL-10, an anti-inflammatory cytokine expressed during muscle regeneration, during both the immobilization and recovery periods. Thus, we believe that AXA2678 has the potential to impact inflammation associated with muscle disuse and atrophy.



Day 15 versus Day 8 relative percentage changes in plasma myoglobin (A), IL-6 (B), IGF-1 (C), IL1R2 (D), and IL-10 (E) levels during the immobilization period. Values represent mean ± SEM in placebo (n=9) and AXA2678 (n=10) groups.

Subjects receiving AXA2678 also had a decreased loss in muscle mass and strength even in the non-immobilized leg, although these effects were smaller than those observed in the immobilized leg. The milder effects observed in the non-immobilized leg show that AXA2678 affected all muscles whether immobilized or not, albeit with a larger effect on immobilized muscles. These findings are consistent with the coordinated systemic effects observed with AXA2678 on the anabolic, inflammatory and metabolic blood markers described above.

The above clinical observations prompted additional cell-based experiments in cultured C2C12 muscle cells to gain further mechanistic insights into AXA2678's effect on muscle mass and function. These experiments sought to study myogenesis, which is important for recovery from muscle injury and to induce muscle growth. During muscle injury, satellite cells, or skeletal muscle stem cells, are activated, differentiate and fuse with damaged myofibers to induce expression of the

cytoskeletal complexes required for muscle contraction and contribute myonuclei supporting muscle repair. The results of the experiments in C2C12 cultured muscle cells revealed that AXA2678 directly increased myoblast differentiation into myotubes, i.e., enhanced myogenesis, as compared to untreated control cells.

AXA2678 Potential Product Development Paths and Market Opportunities

Potential Drug Development Path

Overview: Disuse-related muscle atrophy

Beyond its role in generating contractile force for movement, skeletal muscle is the primary site of glucose disposal, is a large contributor to circulating lipid oxidation, accounts for a large portion of resting metabolic rate, which is highly predictive of independent living with advancing age, and is an independent predictor of morbidity and mortality in several chronic diseases of the liver, kidney and heart. As a result, atrophy, or a decrease in muscle mass in an area of the body, leads to impaired functional capacity, the onset of insulin resistance and a heightened risk for morbidity and mortality. When a muscle remains unused, even for a period of several days, it begins to waste away and lose functionality, the pathophysiology of which is complex and multifactorial. A hallmark of muscle wasting is the inability to maintain proteostasis, the balance between protein synthesis and breakdown. Atrophy is then driven by an inability to maintain muscle protein synthesis. Defective myogenesis, or differentiation of muscle stem cells into myofibers, limits muscle cell growth. Chronic inflammation and related cytokines (tumor necrosis factor alpha) promote muscle wasting by decreasing protein synthesis and increasing protein breakdown. Muscle fat infiltration increases with age and impacts outcomes for certain immobilization atrophy conditions, including the outcomes in liver-related conditions.

Disuse related muscle wasting is most common in people suffering temporary disablement, such as hospitalization or an immobilized limb following injury. Limb immobilization-induced acute atrophy leads to weakness, which further complicates recovery, because movement, especially load-bearing activity, is one of the ways to induce muscle growth. Elective surgeries, including hip replacement, hip fractures, rotator cuff repair or injury, and surgical intervention addresses only the primary injury and has no effect on muscle mass or function that might have occurred prior to surgery. The preexisting presence of atrophy impacts the prognosis after surgery.

The increased recovery burden resulting from limb immobilization-induced acute muscle atrophy leads to longer patient recovery times, impaired functional capacity for independent living and time away from work and activities of daily living. With significant unmet global need, no commercialized products and few assets in development, disuse-related muscle atrophy is an attractive market opportunity. If we pursue development of AXA2678 under an IND, we believe it could potentially be investigated for multiple indications characterized by such disuse-related muscle atrophy, including total knee arthroplasty, or TKA, total hip arthroplasty, or THA, hip fracture related myopenia, or HFRM, rotator cuff repairs or injury, and other musculoskeletal atrophy or injuries, including osteoarthritis induced myopenia. In the United States, the market for these conditions each year is upwards of 140,000 patients undergoing TKA, 50,000 undergoing THA, 133,000 recovering from HFRM and 195,000 recovering from RCI annually.

Limitations of Current Therapies and Available Products

It has been challenging to effectively demonstrate the functional impact of interventions following a change in muscle mass, because mass and function are often decoupled from each other. Study design limitations have also posed a hurdle for fully assessing the efficacy of a treatment. Clinical studies frequently end shortly after immobilization and there is often limited information on recovery post-immobilization, despite recovery having a significant clinical and health

economic relevance. Thus, the clinical utility of any intervention needs to be characterized during the recovery period following injury. Because pre-operative lean body mass and nutrition state are significantly associated with surgical outcomes and hospital stay, pre-immobilization treatment could drive more significant improvements in muscle mass and strength versus post-immobilization treatment alone. For instance, it is now well known that peri-operative exercise improves post-surgery outcomes in elderly patients undergoing major surgery. We believe that a treatment for muscle disuse atrophy should be applied before, during and after immobilization (peri-immobilization) for optimal effects.

Based on emerging research in this area and our insights generated using the AXA Development Platform, we believe a multifactorial pathway-based approach is required to modulate proteostasis, overcome anabolic resistance and induce myogenesis and anti-inflammation simultaneously and in a highly coordinated manner to re-couple mass and function. We believe such an approach may hold significant promise to meaningfully impact clinical outcomes.

Potential Non-Drug Development Path

If we decide to develop AXA2678 as a non-drug product, the target market would be defined by, or with input from, a strategic partner and supporting data from our current or additional studies. We believe a non-drug pathway could also represent a significant market opportunity given the potential benefits to overall health by supporting the body's ability to maintain normal muscle mass and strength. Under a non-drug development path, we or a strategic partner may decide to develop AXA2678 as a dietary supplement to support healthy muscle structure and function.

AXA4010 Program

Overview

We believe that EMMs have the potential to support and maintain blood health, which is critical to a multitude of metabolic functions throughout the body. Using insights from our Non-IND, IRB-Approved Clinical Studies and preclinical studies of EMM compositions, published clinical studies of amino acid interventions, published and in-house metabolic profiling data, and a mechanistic understanding of blood health, we identified key metabolic paths where we believe amino acid biologies drive key aspects of normal blood health.

AXA4010 Design Rationale

AXA4010 has been designed to target multiple biological pathways with the goal of supporting normal structures and functions of the blood.

Underlying Biology	**Design Objective**
Blood Production & Integrity	Promote proliferation and maturation of blood cells during hematopoietic demand Maintain red blood cell, or RBC, form and function against dehydration, rigidity and support oxygen transport
Plasma and RBC amino acid imbalance	Restore amino acid balance to support RBC metabolism, including substrates for glutathione and nitric oxide synthesis, improve reactive oxygen species and delivery of amino acids to peripheral tissue
Vascular health & Inflammation	Improve defense against vascular adhesion, inflammation and stasis

Preclinical Research and Analysis on Sickled Cells To Date

To investigate the effect of certain amino acids on normal blood biology, we performed preclinical experiments on sickle cell blood from subjects. In order to study the effects of reactive oxygen species, or ROS, we challenged the blood cells with a ROS-inducing agent and attempted to rescue the phenotype with various concentrations of amino acids. The data from these preclinical cell experiments demonstrate the impact on ROS in sickled blood when exposed to certain individual amino acids and across various concentrations. We were able to observe positive effects of cysteine and N-acetylcysteine, or NAC, while also noting a negative impact from tryptophan. This observation highlighted the importance of combining only those amino acids that support the targeted biologies, while being cautious of including others that can be detrimental to the biology of interest.

In studies on macrophages and hepatocytes, we explored the relationship between both individual and combinations of amino acids on inflammation. Across both cell model systems, we observed that combinations of certain amino acids had greater anti-inflammatory activity than each amino acid alone. We also observed that combinations and certain individual amino acids had anti-inflammatory activity above baseline. This was demonstrated by both an increase in CCL18, an anti-inflammatory cytokine, in M2 macrophages as well as a decrease in MCP-1, a pro-inflammatory cytokine, in hepatocytes. We incorporated this knowledge about anti-inflammatory combinations of amino acids into the design of AXA4010.

Additionally, we incorporated insights from our pharmacokinetic and pharmacodynamic studies to determine the appropriate relative levels of AXA4010's individual constituents. We then leveraged insights related to the first-pass metabolism of amino acids to ensure sufficient exposure of amino acids to the blood.

Published amino acid profiles in blood disorders, such as sickle cell disease, typically show reductions in essential amino acids such as leucine, histidine, valine, and lysine, and sometimes also non-essential amino acids. To enhance our understanding of metabolic profiles in blood

disorders, we analyzed the concentration of amino acids in plasma and red blood cells of pediatric sickle cell patients versus healthy age-matched controls. Our results in the RBCs and plasma of patients corroborate key findings from the literature such as depleted arginine, while also generating new insights in cysteine biology and other essential amino acids.

Planned Non-IND, IRB Approved Clinical Study

We plan to initiate AXA4010-001, a Non-IND, IRB-Approved Clinical Study of AXA4010, in the second half of 2019 and anticipate the data readout(s) from this study in the second half of 2020. Based on our findings, we will make the appropriate development path decision to develop AXA4010 as a drug or as a non-drug product candidate.

AXA4010 Potential Product Development Paths and Market Opportunities

Potential Drug Development Path

In addition to supporting normal structures and functions of blood in the body, we believe EMMs may have the potential to target and address diseases of the blood such as sickle cell disease, or SCD. Single amino acid based approaches have been tested and approved for SCD. Glutamine was approved by the FDA in 2017 for the treatment of SCD.

SCD is an inherited disorder of hemoglobin affecting approximately 100,000 individuals in the United States and 4.4 million worldwide. For many patients, SCD is defined by chronic organ failure punctuated by acute complications and early mortality. Care for sickle cell patients is limited, with only two FDA-approved drugs that seek to reduce the probability of vaso-occlusion events leading to "sickle cell crises," ischemic injury and severe pain. We believe AXA4010 could be studied under an IND for its potential impact on the management of the complex and multifactorial pathology of SCD.

Non-Drug Development Path

If we decide to develop AXA4010 as a non-drug product candidate, the target market would be defined by, or with input from, a strategic partner. We believe a non-drug pathway could also represent a significant market opportunity given the potential benefits to overall health by supporting blood health and modulating inflammation. Under a non-drug development path, we or a strategic partner may decide to develop AXA4010 as a dietary supplement to support blood-related structure and function.

Preclinical Program

Many diseases are driven by multifactorial dysregulated systemic metabolism, and we have already characterized over 50 diseases for prioritization. We believe that there are also additional potential applications of our AXA Development Platform and have expanded our pool of potential disease areas by importing additional public databases, such as the Human Metabolome Database. We intend to continue to target diseases driven by dysregulated metabolic pathways and in which we can potentially leverage the vital role that EMMs play in regulating metabolic function.

Additional Potential Opportunities

Central Nervous System

Amino acids are intimately involved in the proper functioning of the CNS, which is composed of the brain and spinal cord. Many neurotransmitters, both excitatory and inhibitory, are either amino acids themselves or close metabolites thereof. The transport and metabolism of these

compounds can extend across multiple neuronal cell types, adding to the specialization and complexity of CNS function.

As a result, dysregulation of amino acid biology in the CNS contributes to conditions such as mild traumatic brain injury and highlights how an acute head injury can lead to multi-modal neurological dysfunction through energy crisis, oxidative stress, inflammation and a temporary disruption of the blood-brain barrier. Thus, the complexity of the CNS and spectrum of neuropathology in disease creates a vast potential for targeted and combinatorial EMM interventions.

Other Areas

Additionally, we are looking at the application of EMMs in other organ systems affected by metabolic dysfunction, including targets relating to kidney and pulmonary function.

Competition

The biotechnology and pharmaceutical industries utilize rapidly advancing technologies and are characterized by intense competition. There is also a strong emphasis on intellectual property and proprietary products.

We believe that our pioneering research in establishing EMM compositions for drug or non-drug pathways such as non-drug products, coupled with our capabilities across our AXA Candidate platform technology, product design, development pathway and manufacturing provide us with a competitive advantage. However, we will continue to face competition from different sources, including major pharmaceutical companies, biotechnology companies, consumer health product companies, academic institutions, government agencies, public and private research institutions, consumer nutritional supplement or conventional consumer health manufacturers and manufacturers of amino acids and other EMMs. For any products that we eventually commercialize, we will not only compete with existing products but also with new products that may become available in the future, such as microbiome products.

We compete in the consumer health, pharmaceutical and biotechnology industries. There are additional companies that are working on modulating specific metabolic pathways involved in various health and disease conditions, although we are not aware of any company creating AXA Candidate-like products with multifactorial activity. Companies with clinical programs that could compete with our AXA Candidates include Madrigal Pharmaceuticals, Inc., Intercept Pharmaceuticals, Inc., Gilead Sciences, Inc., Novartis AG, Bristol-Myers Squibb Co., Esperion Therapeutics, Inc., Viking Therapeutics, Inc., Scholar Rock Holding Corporation, NGM Biopharmaceuticals Inc., Genfit SA and Kaleido Biosciences, Inc., among others.

Finally, we anticipate competing with the largest consumer health companies and nutritional pharmaceutical and amino acid companies in the world, such as Nestlé Health Science S.A., Abbott Laboratories, Johnson & Johnson, The Procter & Gamble Company, and Ajinomoto Co., Inc., all of which are currently conducting research in competitive indications or may be interested in using amino acids and other EMMs as drugs or nutritional supplements.

Intellectual Property

As a first mover in this approach to EMMs, we are establishing a broad, global intellectual property portfolio for our pipeline and have already received our first notice of allowance for an AXA1125 patent application. This is notable considering the speed of our development model and establishes a foundation for the large number of submitted applications for our pipeline (35 patent applications filed as of April 1, 2019). Our matrixed approach to IP, trade secrets, and capabilities

that we take across the AXA Candidate, the indication, the AXA Development Platform, manufacturing processes and technologies; formulations; and amounts provides an important asset for the company.

We seek to create a multi-dimensional intellectual property portfolio as a strategic asset that has the potential to provide us with a significant competitive advantage. We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to our business, including seeking, maintaining and defending patent rights, whether developed internally or through collaborations, or licensed from third parties. Our policy is to file patent applications related to our proprietary technology, inventions, improvements and AXA Candidates that are important to the development and implementation of our business in the United States and in jurisdictions outside of the United States. We also rely on trade secrets and know-how relating to our proprietary technology and AXA Candidates, continuing innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of rebalancing dysregulated metabolism. We additionally rely on regulatory-related protections such as data exclusivity, market exclusivity and patent term extensions when available, and where appropriate, plan to seek and rely on regulatory protection afforded through Orphan Drug designations. Our commercial success may depend in part on our ability to obtain and maintain patent and other proprietary protection for our technology, inventions and improvements; to preserve the confidentiality of our trade secrets; to maintain our licenses to use intellectual property owned by third parties; to defend and enforce our proprietary rights, including our patents; and to operate without infringing on the valid and enforceable patents and other proprietary rights of third parties.

We believe that we have a strong global intellectual property position anchored by our matrix of patents directed to proprietary AXA Candidates, technology, AXA Development Platform, related manufacturing processes and technology, unique formulations, and knowledge about amounts, uses and features established as standards for AXA Candidates. As for our AXA Candidates, AXA Development Platform, and the processes we develop and commercialize, in the normal course of business, we pursue, as appropriate, patent protection or trade secret protection relating to compositions, methods of use, treatment of indications, dosing, formulations and methods of manufacturing. As of April 1, 2019, our AXA Candidate-related patent portfolio consisted of 18 patent families, two granted U.S. patents, 22 U.S. pending patent applications (including provisional applications) and 13 pending patent applications in jurisdictions outside of the United States (including Patent Cooperation Treaty, or PCT, applications) that, in many cases, are counterparts to the foregoing U.S. patent applications. To date all of our patent rights are owned by us. Our objective is to continue to expand our portfolio of patent applications to protect our AXA Candidates and certain aspects of our AXA Development Platform, manufacturing processes, formulations, and insights into amounts, uses, and features of AXA Candidate composition.

In addition, we own a portfolio of legacy patents and patent applications related to recombinant proteins for nutrition and therapeutics, including nine granted and three pending U.S. patents, and five granted and five pending foreign patents.

Examples of the AXA Candidates and technology areas covered by our intellectual property portfolio are described below.

Indication-Related Intellectual Property

The indication-related patent rights in our intellectual property portfolio relate to conditions and disorders associated with dysregulated metabolism and provide coverage for AXA Candidates to specifically address those conditions and the associated disease states, as well as structure and function of normal organs in the context of dysregulated metabolism. The indication-related patent applications for our lead programs cover novel AXA Candidate compositions and their uses broadly

and, with respect to individual AXA Candidates, in detail. Often, we are able to exemplify even our earliest AXA Candidate inventions with human as well as animal and *in vitro* data. Each of the indication-related patent rights and applications described below are owned by us and are not licensed from any third party.

Notably, while our intellectual property covers drug and non-drug areas, each initial AXA Candidate is first tested as a food in order to better understand the impact on normal human physiology and metabolic pathways. After such initial testing, a decision may be made to deem the AXA Candidate a drug product candidate, and subsequent studies on disease endpoints are conducted under INDs. Thus, the descriptions of patent rights should not be considered as suggesting a current intent to pursue the AXA Candidate as a drug candidate.

AXA Candidate Compositions and Methods for the Treatment of Cirrhosis

Our patent applications cover a class of AXA Candidate compositions for cirrhosis, including clinical candidate AXA1665. Currently, patent rights relating to cirrhosis include a U.S. patent application, a PCT patent application, and two foreign national patent applications. We expect any granted patent based on this family to expire in 2038, excluding any patent term adjustments or extensions.

AXA Candidate Compositions and Methods for NAFLD/NASH

Our patent applications cover a class of AXA Candidate compositions for fatty liver disease, including clinical candidate AXA1125. Currently, patent rights relating to fatty liver disease include two U.S. patent applications and two granted U.S. patents, a PCT patent application, and two foreign national patent applications. We expect any granted patent based on this family to expire in 2037, excluding any patent term adjustments or extensions.

AXA Candidate Compositions and Methods for Acute Muscle Atrophy

Our patent applications cover a class of AXA Candidate compositions for muscle atrophy, including clinical candidate AXA2678. Currently, patent rights relating to muscle atrophy include three U.S. patent applications, a PCT patent application, and two foreign national patent applications. We expect any granted patent based on this family to expire in 2037, excluding any patent term adjustments or extensions.

AXA Candidate Compositions and Methods for Treatment of Traumatic Brain Injury and Stroke

Our patent applications cover a class of AXA Candidate compositions for traumatic brain injury and stroke. Currently, patent rights relating to traumatic brain injury include one U.S. patent application, two PCT patent applications, and two foreign national patent applications. We expect any granted patent based on this family to expire in 2038, excluding any patent term adjustments or extensions.

Additional Indication-Related Patents

We have filed six provisional patent applications directed to additional indications associated with dysregulated metabolism. We expect the patent applications in this portfolio, if issued, to expire in 2039, without taking into account any patent term adjustments or extensions we may obtain. Our AXA Development Platform allows us to rapidly identify and file on AXA Candidates, often including human data in the earliest patent application.

Additional AXA Combination-Related Patents

We have filed three provisional patent applications directed to combinations of particular AXAs (AXA1125, AXA1665 and AXA1957) with particular NAFLD/NASH drugs and drug candidates. We expect the patent applications in this portfolio, if issued, to expire in 2040, without taking into account any patent term adjustments or extensions we may obtain.

Platform-Related Intellectual Property

In addition to the indication-related intellectual property, our intellectual property portfolio also includes know-how and patent applications directed to our AXA Development Platform and other technologies developed internally. Exemplary platform technologies that are the subject of such patent applications include:

- manufacturing processes for complex AXA Candidate mixtures;
- taste formulations; and
- pharmacological characteristics of AXA Candidate compositions.

Our AXA Development Platform iterates and integrates data from literature, including patents, *in vitro* experiments, animal studies, and Non-IND, IRB-Approved Clinical Studies, giving us significant competitive advantages. Our AXA Development Platform, which is protected by trade secrets, is core to maintaining our first mover advantage. These advantages translate into a unique understanding of metabolism, development of many new AXA Candidates, and creation of intellectual property around AXA Candidates and our AXA Development Platform technologies.

These AXA Development Platform technologies, and our intellectual property protection related thereto, are broadly applicable to our AXA Candidates. Our patent applications directed to platform-related technologies, if issued, would expire in 2039, without taking into account any patent term adjustment or extensions we may obtain.

Therapeutic Modality Intellectual Property

Our proprietary knowledge and insights into the behavior of EMMs have yielded inventions related to categories of metabolic dysfunction, such as insulin resistance, inflammation, and fibrosis. Our patent applications directed to these underlying modalities, if issued, would expire in 2039, without taking into account any patent term adjustment or extensions we may obtain.

We continually assess and refine our intellectual property strategy as we develop new platform technologies and AXA Candidates. To that end, we are prepared to file additional patent applications if our intellectual property strategy requires such filings, or where we seek to adapt to competition or seize business opportunities. Further, we are prepared to file patent applications, as we consider appropriate under the circumstances, relating to the new technologies that we develop. In addition to filing and prosecuting patent applications in the United States, we plan to file counterpart patent applications in additional countries where we believe such foreign filing is likely to be beneficial, including, but not limited to, Australia, Brazil, Canada, China, Europe, Hong Kong, India, Israel, Japan, and South Korea.

Individual patent terms depend upon the date of filing of the patent application, the date of patent issuance and the legal term of patents in the countries in which they are obtained. Generally, patents issued for applications filed in the United States and most other countries have patent terms that expire 20 years from the earliest effective filing date. In certain instances, a patent term can be extended to recapture a portion of the term effectively lost as a result of a regulatory review period, although patent term restoration in the United States applies to a new chemical entity, so may not apply to some AXA Candidate compositions. Furthermore, any restoration period is limited

to a maximum restoration time (five years in the United States and Europe) and total effective patent life (14 years in the United States and 15 years in Europe). The actual protection afforded by a patent varies on a product-by-product basis, from country-to-country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country, and local decisions about the validity and enforceability of the patent.

Regulatory Exclusivity

Under certain circumstances, approval of a drug product by a health authority will result in a period of data exclusivity and/or market exclusivity for the product. Data exclusivity means that no party can file for approval of a drug product based on the original drug approval application data. Market exclusivity (such as Orphan Drug exclusivity) means that the health authority may not be permitted to give final approval to a drug product for a defined period of time, absent certain conditions. In the United States, five-year data exclusivity is only available upon approval of a new chemical entity, which may not apply to some AXA Candidate compositions, and market exclusivity for new indications supported by or clinical studies essential to the approval is limited to three years from approval. In Europe, the period of regulatory exclusivity for a drug product approved through the full process is 10 years (eight years of data exclusivity and two years of market exclusivity, with an additional year of market exclusivity available for a substantial new indication). We believe that AXA products, if approved, should be entitled to the full 10 years of regulatory exclusivity in Europe.

Trademark Protection

As of April 1, 2019, our trademark portfolio contains more than 50 registrations and pending applications. We have trademarks in over 12 countries, including European Union. For the marks AXCELLA and the Axcella LOGO, we have pending applications in the United States, Canada and Brazil as well as International Registrations designating China, the European Union, India, Japan and Russia. In addition, we have a pending U.S. application for AXCELLA HEALTH.

Trade Secrets

We may also rely, in some circumstances, on trade secrets to protect our technology and aspects of our AXA Development Platform. However, trade secrets are difficult to protect. We seek to protect our trade secret technology, e.g., AxcellaKB, confidential AXA Candidates, and commercial plans in part by entering into confidentiality agreements with those who have access to our confidential information, including our employees, contractors, consultants, collaborators and advisors. We also seek to preserve the integrity and confidentiality of our proprietary technology and processes by maintaining physical security of our premises and physical and electronic security of our information technology systems. Although we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or may be independently discovered by competitors. We have policies in place to ensure that our employees, contractors, consultants, collaborators and advisors do not use intellectual property owned by others in their work for us. Nevertheless, disputes may arise as to the rights in related or resulting know-how and inventions. For this and more comprehensive risks related to our proprietary technology, inventions, improvements and AXA products, if approved, please see the section on "Risk factors — Risks related to our intellectual property."

Manufacturing

To date, we have rapidly designed and efficiently manufactured AXA Candidates and believe our process is readily scalable. Under our existing agreements with our manufacturers, we have been able to secure initial AXA Candidates to initiate our Non-IND, IRB-Approved Clinical Studies in less than three months from identifying the product amount needed. We are making great strides in our goal to create and design product candidates that are consumer-friendly, including in their formulation (e.g., taste), packaging (e.g., easy to open sachets) and administration (e.g., water soluble). Our AXA Candidates are supplied in a dry powder form, which is dissolved in water and then administered orally as an orange-flavored drink. Our formulation development expertise enables us to deliver high concentrations of raw materials at appropriate administration volumes. This expertise covers a wide range of raw material characteristics, and we believe will enable us to deliver multiple oral dosage forms to meet Non-IND, IRB-Approved Clinical Study and clinical needs, as well as commercial needs, if we decide to pursue development of any AXA Candidate under an IND.

The raw materials in our AXA Candidates are used in the pharmaceutical and non-drug product industries. These materials are sourced from commercial manufacturers who are subject to current Good Manufacturing Practice, or cGMP, requirements and who have active drug master files with the FDA wherever possible. We have identified multiple manufacturers of the raw materials. The raw materials are tested and released per defined product specifications for physical, chemical and microbiological, where applicable, properties by the commercial manufacturers using the current edition of United States Pharmacopeia, or USP, and validated methods per the commercial manufacturer's established quality system, for non-USP materials.

Our AXA Candidates are manufactured through a series of proprietary processing steps at a contract manufacturing organization, or CMO, to produce the dry powder forms in sealed foil stick packs. Each batch is tested per its defined product specification for chemical and microbiological properties, and a Certificate of Analysis, or CoA, is issued for each batch. Chemical testing is performed both in-house and at a contract research organization, or CRO, while microbiological testing is outsourced to a specialty CRO. Chemical and microbiological testing is performed using qualified test methods.

The accuracy, precision and technical robustness of our in-house test methods are confirmed through formal method validation performed by a specialty CRO. This confirmation of our quality-based assays ensures that the quality of our AXA Candidates remains consistent through the shelf life of the AXA Candidate.

Any AXA Candidates that we decide to develop as drug candidates under INDs will be manufactured through a series of proprietary processing steps at a different, well-known CMO under pharmaceutical cGMPs to produce the dry powder forms in sealed foil sachets. Chemical and microbiological testing of AXA Candidates would be performed by this same CMO per defined product specifications and CoAs will be issued for each batch. Chemical and microbiological testing will be performed using validated test methods. We believe these processing steps would enable us to readily provide high quality Clinical Trial Material, or CTM, product to support multiple Clinical Trials conducted under INDs and that are readily scalable to support a greater amount of CTM to commercial drug product requirements, if any of our AXA Candidates regulated as drugs receive regulatory approval.

Suppliers and CMOs

We have entered into clinical supply and quality agreements with our CMOs and have established manufacturing redundancy within our supply chain. The CMO for our AXA Candidates

has the capability and capacity to support clinical to commercial scale quantities of our AXA Candidates, if approved, including manufacturing redundancy between North America and Europe.

We have also entered into clinical supply agreements with well-known clinical drug product labeling, packaging and distribution organizations to support post-IND global clinical studies, as required, if we were to decide to pursue the development of any AXA Candidate as a drug. These agreements provide further redundancy within our supply chain.

At the appropriate time, we will enter into appropriate supply agreements with key commercial raw material manufacturers and additional CMOs to ensure raw material sourcing and additional AXA Candidate product supply redundancy within the supply chain, if any of our AXA Candidates is approved.

Government regulation

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval (when required), advertising, promotion, marketing, post-approval monitoring and post-approval reporting of drugs as well as non-drug products such as foods, dietary supplements, and medical foods. We, along with our contract research organizations and contract manufacturers, will be required to navigate the various preclinical, clinical, manufacturing and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval for some or all of our product candidates, depending on the development pathway pursued in each case. The process of obtaining regulatory approvals of drugs for therapeutic indications or commercialization of non-drug products and ensuring subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources.

In the United States, the FDA regulates drug products as well as non-drug products under the Federal Food, Drug and Cosmetic Act, or the FD&C Act, as amended, its implementing regulations and other laws. None of our product candidates has been approved by the FDA for marketing for therapeutic indications in the United States, and we have not marketed any product as a food (including dietary supplements) or medical food. If we fail to comply with applicable FDA or other requirements at any time with respect to product development, clinical testing, or any approval process, where applicable, or any other legal requirements relating to product manufacture, processing, handling, storage, quality control, safety, marketing, advertising, promotion, packaging, labeling, export, import, distribution, or sale, we may become subject to administrative or judicial sanctions or other legal consequences. These sanctions or consequences could include, among other things, the FDA's refusal to approve pending applications, issuance of clinical holds for ongoing studies, suspension or revocation of approved applications, warning or untitled letters, product withdrawals or recalls, product seizures, relabeling or repackaging, total or partial suspensions of manufacturing or distribution, injunctions, fines, civil penalties or criminal prosecution.

If we decide to pursue development of our AXA Candidates as drugs for therapeutic indications, the process required by the FDA before our EMM product candidates as drugs for therapeutic indications may be marketed in the United States generally involves the following:

- completion of extensive preclinical studies in accordance with applicable regulations, including studies conducted in accordance with good laboratory practice, or GLP, requirements;

- submission to the FDA of an IND application, which must become effective before human Clinical Trials may begin;
- approval by an IRB or independent ethics committee at each Clinical Trial site before each trial may be initiated;
- performance of adequate and well-controlled human Clinical Trials in accordance with applicable IND regulations, GCP requirements and other Clinical Trial-related regulations to establish the safety and efficacy of the investigational product for each proposed indication;
- submission to the FDA of an NDA;
- a determination by the FDA within 60 days of its receipt of a New Drug Application, or NDA, to accept the filing for review;
- satisfactory completion of one or more FDA pre-approval inspections of the manufacturing facility or facilities where the drug will be produced to assess compliance with cGMP requirements to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- potential FDA audit of the Clinical Trial sites that generated the data in support of the NDA;
- payment of user fees for FDA review of the NDA;
- FDA review and approval of the NDA, including consideration of the views of any FDA advisory committee, prior to any commercial marketing or sale of the drug in the United States; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post-approval studies.

The testing and approval process requires substantial time, effort and financial resources, and we cannot be certain that any approvals for our product candidates will be granted on a timely basis, if at all.

Regulatory status of AXA Candidates

We are currently developing our EMM product candidates as Non-Drug Products, and in particular, as food for humans. AXA Candidates in our pipeline are predominantly comprised of amino acids. There is a long history of safe use of many amino acids as food additives and the FDA's food regulations concerning special dietary and nutritional additives specifically provide that "certain food additive amino acids may be safely used as nutrients added to foods" when certain conditions are met. Based on the large body of studies and scientific literature on the human exposure to and safety information on amino acids, the FDA's issuance of regulations authorizing use of specific amino acids under certain conditions as safe and permissible food additives when used as nutrients, our own data on amino acids used in AXA Candidates, and the fact that we use amino acids in our AXA Candidates within amounts previously studied in humans with no significant safety concerns or issues observed, we believe we design AXA Candidates to have favorable safety profiles, but we further characterize the safety and tolerability of these AXA Candidates in our Non-IND, IRB-Approved Clinical Studies.

We believe that the use of AXA Candidates containing amino acids may be studied as a Non-Drug Product for certain non-therapeutic uses without an IND. Specifically, under FDA's September 2013 Guidance for clinical investigators, sponsors, and IRBs entitled "Investigational New Drug Applications (INDs) — Determining Whether Human Research Studies Can Be Conducted Without an IND," we believe Non-IND, IRB-Approved Clinical Studies of Non-Drug

Products may be conducted to study the safety, tolerability, effect on normal structure or function in humans and characterize the mechanism by which a substance acts to maintain such structure or function, including in individuals with disease (as long as the study is not intended to evaluate the Non-Drug Product's ability to diagnose, cure, mitigate, treat, or prevent a disease). However, if such formulations are being studied or developed for therapeutic or drug uses, studies designed to assess the potential therapeutic use of a product candidate, such as its ability to diagnose, cure, mitigate, treat or prevent disease, including effects on an abnormal and uncommon or serious condition, then such product candidate is regulated as a drug and any clinical studies in the United States must be conducted under an IND. Similarly, an investigation intended to evaluate the effects of a medical food on a disease would require an IND, unless the medical food is simply being fed to subjects for nutritional purposes during a study examining the effects of another intervention.

Depending on the results observed for our AXA Candidates in our Non-IND, IRB-Approved Clinical Studies, and other factors related to the potential market for the candidates, we may elect to pursue development of our candidates under one of four regulatory pathways: to continue development as a conventional food or food additive, dietary supplement, medical food, or drug. After meeting with the FDA in March 2019, we made a decision to pursue a drug development path for AXA1665 and plan to file an IND submission after potentially having additional interactions with the FDA. We have not yet made a development path decision for any of our other AXA Candidates, and may never successfully develop and market an AXA Candidate under either drug or non-drug paths.

The development path decision we make for an AXA Candidate will also influence our strategy in bringing an AXA Candidate to market in the appropriate channel, such as a drug product intended to cure, mitigate, treat or prevent a target disease, a Non-Drug Product intended to support the normal structures and functions of the body and to maintain health, or a consumer health product intended for the dietary management of a disease or condition for which there are distinctive nutritional requirements. We intend to focus on and carry forward development of AXA Candidates that we believe may have the potential for therapeutic benefit.

FDA regulation of conventional food

Conventional food products are subject to regulation by the FDA and other regulatory authorities, including the Federal Trade Commission, or FTC, which regulate the manufacturing, preparation, quality control, import, export, packaging, labeling, marketing, advertising, promotion, distribution, safety, and/or adverse event reporting of conventional foods. Among other things, manufacturers of conventional foods must meet applicable current good manufacturing practices, or cGMPs, and certain requirements that govern the manufacturing, packaging, labeling and holding of foods.

The FD&C Act requires that all food products be safe, meaning a reasonably scientific certainty that the substance is not harmful for its intended use. The FD&C Act prohibits the introduction into interstate commerce of a food to which has been an added an approved drug or biologic, or a drug or biologic for which substantial clinical investigations have been instituted and made public, unless such a drug was marketed as a food before approval or meets other certain exceptions.

Under sections 201(s) and 409 of the FD&C Act, any substance that is reasonably expected to become a component of food or added to food is considered to be a "food additive", with a few exceptions, and is therefore subject to FDA premarket review and approval, unless the substance is generally recognized among experts qualified by scientific training and experience to evaluate its safety, as having been adequately shown through scientific procedures or, in the case of a substance used prior to January 1, 1958 through experience based on common use in food, to be

safe under the conditions of its intended use, a standard referred to as "generally recognized as safe," or GRAS. A food additive must either already be included within one of the number of FDA regulations authorizing the use of certain food additives under certain conditions of use or be approved for use by the FDA. To obtain approval for use of a food additive, a manufacturer must submit a petition to the FDA with sufficient data to demonstrate reasonable certainty of no harm at the intended levels of use. Any food that contains an unapproved food additive is considered adulterated under section 402(a)(2)(C) of the FD&C Act.

Ingredients that are determined to be GRAS (as described below) do not fall within the definition of a food additive, which, as noted above, requires mandatory premarket approval. Under sections 201(s) of the FD&C Act, and FDA's implementing regulations in 21 CFR § 170.3 and 21 CFR § 170.30, the use of a food substance may be GRAS either through scientific procedures or, for a substance used in food before 1958, through experience based on common use in food.

General recognition of safety through scientific procedures requires the same quantity and quality of scientific evidence as is required to obtain approval of the substance as a food additive and must be based upon the application of generally available and accepted scientific data, information, or methods, which are ordinarily published, as well as the application of scientific principles, and may be corroborated by unpublished studies and other data and information. General recognition of safety through experience based on common use in foods requires a substantial history of consumption of a substance for food use by a significant number of consumers. If an ingredient is GRAS for one use or in one form, it is not necessarily GRAS for all uses or forms. Under section 201(s) of the FD&C Act, it is the intended use of a substance, rather than the substance itself, that is eligible for classification as GRAS.

Manufacturers of GRAS substances may notify the FDA of their view that a substance is GRAS and thus not subject to the premarket approval requirements of section 409 of the FD&C Act. The notification must include, among other things, a description of the substance, the applicable conditions of use, the dietary exposure, an explanation of the basis for the determination that the substance was determined to be safe for the intended use and supporting data and information. Upon review of such a notification, the FDA may respond with a "no questions" letter stating that while it has not made its own GRAS determination, it has no questions at the time regarding the applications' own GRAS determination. Alternatively, manufacturers may elect to "self-affirm" a given substance is GRAS without the voluntary FDA notification but should retain all applicable safety data used for the GRAS determination in the case of inquiry by the FDA. However, in neither case does this constitute an approval equivalent to that achieved through the food additive process. A manufacturer's use of such food additive is at its own risk and is dependent upon adequate substantiation and/or scientific support demonstrating safe use.

With certain exceptions, clinical investigations in which an investigational drug is administered to human subjects must be conducted under an IND, as required by FDA regulations. The FDA published a guidance document in September 2013 for clinical investigators, sponsors, and IRBs, "Investigational New Drug Applications (INDs) — Determining Whether Human Research Studies Can Be Conducted without an IND," that provides the FDA's thinking on when an IND is required for human research studies. Based on this guidance, we understand and believe that the FDA's interpretation of its regulations is that they do not require human testing of food ingredients or dietary supplements to be conducted under an IND unless such testing is intended to evaluate the product's ability to diagnose, cure, mitigate, treat, or prevent a disease or condition. In this guidance, the FDA specifically recognizes an IND will not be required when a study is designed to "evaluate the tolerability of a food in a specific susceptible population, including individuals with a disease in a diseased population," provided the study is not designed to assess the impact of the food or medical food on the disease. There is no assurance that our understanding of the FDA's guidance is accurate or that the FDA's thinking on this matter will not change. If it does, the FDA

may decide to take enforcement action to prohibit the Non-IND testing of substances that it believes should be conducted under an IND. For any products that we ultimately develop as drugs, the FDA may delay or deny an IND submitted with supporting data from human studies with such products not conducted under an IND or require alternate or additional data to support the IND before authorizing an applicant to proceed.

Additionally, depending on the circumstances, the use of a substance in certain clinical investigations under an IND may restrict the marketing of such substance in food. Section 301(II) of the FD&C Act prohibits the marketing of any food containing a drug substance for which substantial clinical investigations have been instituted and for which the existence of such investigations has been made public, unless the substance was marketed in food before any substantial clinical investigations involving the drug were instituted or one of the other exceptions in section 301(II) applies. Marketing the substance of interest in food before seeking an IND or beginning any clinical investigations preserves the option to continue to market the substance in those forms after substantial clinical investigations have been instituted and their existence has been made public.

The FDA may classify some or all of our potential product candidates as containing a food additive that is not GRAS. Such classification would cause these product candidates to require pre-market approval for a food additive, or else it would need to be subject to an existing food additive regulation authorizing its use, which could substantially delay or prevent the commercialization of these product candidates for non-drug uses. Any delay in the regulatory consultation process or a determination that any of our drug or food product candidates do not meet regulatory requirements of the FDA, including any applicable GRAS requirements, could cause a delay in or prevent the commercialization of our product candidates, which may lead to reduced acceptance by the public or others or an inability to commercialize those candidates at all. Moreover, if the FDA determines that a product candidate marketed as a conventional food contains a non-GRAS additive after the product has already been commercialized, then the FDA may take enforcement or other legal consequences, including, but not limited to, warning or untitled letters, product withdrawals or recalls, product seizures, relabeling or repackaging, total or partial suspensions of manufacturing or distribution, injunctions, fines, civil penalties or criminal prosecution.

FDA regulation of dietary supplements

The Dietary Supplement Health and Education Act of 1994, defines dietary supplement products to be "foods" under the FD&C Act, and they are regulated as such by the FDA. The FDA and other regulatory authorities, including the Federal Trade Commission, or FTC, similarly with conventional foods, regulate the manufacturing, preparation, quality control, import, export, packaging, labeling, marketing, advertising, promotion, distribution, safety, and/or adverse event reporting of dietary supplements. Among other things, manufacturers of dietary supplements must meet applicable cGMPs, and certain requirements that govern the manufacturing, packaging, labeling and holding of dietary supplements.

Under federal law, dietary supplements are defined in relevant part as a product (other than tobacco) intended to supplement the diet that bears or contains one or more dietary ingredients, which include any of the following: a vitamin; a mineral; an herb or other botanical; an amino acid; a dietary substance for use by man to supplement the diet by increasing the total dietary intake; or a concentrate, metabolite, constituent, or extract, or any combination of those substances. Dietary supplements may not include articles that are approved as new drugs or biologics or that have been authorized for investigation as new drugs or biologics for which substantial clinical investigations have been instituted and made public, unless the article was marketed as a dietary supplement or food prior to such approval or authorization.

The manufacturer of a dietary supplement is responsible for ensuring the safety of its product and must demonstrate either that each dietary ingredient was marketed as a dietary supplement in the United States before October 15, 1994, or if the dietary ingredient was not marketed as a dietary supplement in the United States before October 15, 1994, referred to as a "new dietary ingredient," or NDI, then the dietary supplement contains only dietary ingredients that have been present in the food supply as an article used for food in a form in which the food has not been chemically altered. For any supplement containing a new dietary ingredient for which the dietary ingredients have not been present in the food supply as an article used for food in a form in which the food has not been chemically altered, the manufacturer or distributor of the dietary ingredient or the supplement must submit pre-market notification to the FDA at least 75 days before the initial marketing of the dietary ingredient when used under the conditions or suggested in the labeling of the dietary supplement. Even to the extent the NDI was present in the food supply prior to October 15, 1994 or is used in conventional foods, if there are any changes to the ingredient's manufacturing or form as it was present in the food supply at that time or from how it exists in its conventional food form, then the ingredient may also be considered a NDI requiring notification.

Notification of use of a "New Dietary Ingredient" must inform the FDA of the basis on which the manufacturer has concluded that the supplement containing an NDI is reasonably expected to be safe under the recommended conditions of use. The FDA may not respond to such notification, but no response does not mean the FDA has determined that the ingredient is safe or permissible for use in a dietary supplement.

If the FDA determines that a product candidate which has already been brought to market and marketed as a dietary supplement contains a NDI without prior notification or contains some other substance not permitted in supplements, it may determine that the product is adulterated and/or misbranded in violation of federal law. In such a case, the FDA may take enforcement or other legal actions, including, but not limited to, warning or untitled enforcement letters, product withdrawals or recalls, product seizures, relabeling or repackaging, total or partial suspensions of manufacturing or distribution, injunctions, fines, civil penalties or criminal prosecution. Any such enforcement actions or other consequences could have a material adverse effect on the firm and its current and future ability to development the product candidates through the selected pathway or other pathways.

In addition, manufacturers of dietary supplements must ensure that ingredients in their products that are not defined as dietary ingredients comply with all the requirements applicable to foods. For example, fillers and other constituents of the product must be approved as food additives or must be deemed GRAS for the conditions of use in order to be sold.

Permissible claims for conventional foods and dietary supplements

The FDA and other regulatory authorities, including the FTC, heavily regulate any express or implied claims made about food and dietary supplement products, and/or their ingredients, including, but not limited to, any claims made in product labeling, marketing, promotion, on social media, or on the firm's website. FTC seeks to ensure the truth, accuracy, and substantiation of dietary supplement claims, and the FDA regulates the type and specific content of such claims.

With respect to dietary supplements, products are only allowed to make certain truthful and non-misleading claims without prior FDA-approval, and may not make health or qualified health claims, or any other claims that expressly or implicitly characterize the relationship between a substance and a disease or health-related condition (e.g., drug claims). Specifically, 21 C.F.R. § 101.93(g) prohibits claims, whether express or implied, that a dietary supplement is intended to "diagnose, mitigate, treat, cure, or prevent disease" and the use of such claims could subject a dietary supplement product to regulation as a drug. The FDA defines "disease" at 21 C.F.R. § 101.93(g)(1) to include any "damage to an organ, part, structure, or system of the body such that

it does not function properly . . ., or a state of health leading to such dysfunctioning," though excludes diseases resulting from deficiencies in essential nutrients. For these types of claims, there must be significant scientific support and approval by the FDA prior to marketing the product. In other words, products marketed as dietary supplements cannot make health claims or drug claims (i.e., that the product is intended to diagnose, prevent, or cure any disease or condition) without prior FDA-approval of such claims. Dietary supplement products are permitted to make certain claims subject to post-market notification to the FDA and inclusion of an FDA-required disclaimer. These requirements apply to multiple types of claims, including "structure/function" claims that refer to a product's ability to maintain healthy bodily structure or function or claims describing the role of a nutrient or dietary ingredient or characterizing the way in which a nutrient or dietary ingredient supports or maintains such structure or function.

In determining whether or not a particular claim is permissible, the FDA considers the overall context in which that claim is made. As such, the direct recommendation of particular products to users based on deficiencies or other characteristics in their nutritional or health profiles may be viewed by the FDA as an implied disease claim and thus impermissible (except for specific allowed health claims or qualified health claims for supplements or foods or allowed nutrient content claims in foods or structure/function claims for dietary supplements).

The use of product claims on product candidates developed as non-drug products always carries a risk of regulatory enforcement action or other legal consequences, which may include class action litigation. Claims and other marketing or promotional activities also are potentially subject to federal and state consumer protection and unfair competition laws. Any such actions could materially affect us in an adverse way.

FDA regulation of medical food uses

Medical foods are a category of foods distinct from conventional food and dietary supplements. The FDA and other regulatory authorities, including the FTC, also regulate the manufacturing, preparation, quality control, import, export, packaging, labeling, marketing, advertising, promotion, distribution, safety, and/or adverse event reporting of medical foods. Among other things, manufacturers of medical foods must meet relevant cGMPs, and certain requirements that govern the manufacturing, packaging, labeling and holding of foods.

As defined in section 5(b)(3) of the Orphan Drug Act (21 U.S.C. 360ee(b)(3)), a medical food is "a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation." The FDA has established a regulation at 21 C.F.R. 101.9(j)(8) that further defines a medical food as a product that is (1) is specially formulated and processed product (as opposed to a naturally occurring foodstuff used in its natural state) for the partial or exclusive feeding of a patient by means of oral intake or enteral feeding by tube; (2) is intended for the dietary management of a patient who, because of therapeutic or chronic medical needs, has limited or impaired capacity to ingest, digest, absorb, or metabolize ordinary foodstuffs or certain nutrients, or who has other special medically determined nutrient requirements, the dietary management of which cannot be achieved by the modification of the normal diet alone; (3) provides nutritional support specifically modified for the management of the unique nutrient needs that result from the specific disease or condition, as determined by medical evaluation; (4) is intended to be used under medical supervision; and (5) is intended only for a patient receiving active and ongoing medical supervision wherein the patient requires medical care on a recurring basis for, among other things, instructions on the use of the medical food.

Because the marketing of medical foods generally does not require FDA pre-market approval, the medical food category may offer promising opportunities for our products should we pursue that development path. However, we understand that the FDA considers the statutory definition of medical foods to narrowly constrain the types of products that fit within this category of food. There can be no assurance we will be able to develop the data that are needed to substantiate the positioning of the product as a medical food or that the FDA would concur the product meets the definition of medical food. In such cases, the commercialization of such product candidates may be delayed or prevented. To the extent that any product candidate is marketed as a medical food and subsequently determined to not fall within the proper regulatory category or are considered to be misbranded or adulterated, the company may be subject to enforcement action or other legal consequences, including, but not limited to, warning or untitled letters, product withdrawals or recalls, product seizures, relabeling or repackaging, total or partial suspensions of manufacturing or distribution, injunctions, fines, civil penalties or criminal prosecution. Any such actions or other consequences could have a material adverse effect on the firm and its current and future ability to development the product candidates through the selected pathway or other pathways.

Moreover, the ingredients and additives used in medical foods are subject to the same regulations as conventional foods and must be GRAS or otherwise covered by an existing food additive regulation, approved food additive petition, or authorized by a prior sanction. The FDA may determine that some or all of our potential product candidates contain ingredients that are not GRAS and are therefore food additives. Such classification would cause these product candidates to require pre-market approval for a food additive, which could substantially delay or prevent the commercialization of these product candidates for medical food uses. If the FDA determines that a product candidate marketed as a medical food contains a substance that is not GRAS and is therefore a food additive after the product has already been commercialized, and such food additive is not approved or authorized by the FDA pursuant to a food additive regulation, then the firm may face enforcement or other legal consequences including, but not limited to, those mentioned above.

Preclinical and Clinical Trials for drugs

Once a product candidate is identified for development as a drug, it generally enters the preclinical testing stage. Preclinical studies include laboratory evaluations of drug chemistry, formulation and stability, as well as *in vitro* and animal studies to evaluate the potential for adverse events, which must be conducted in accordance with federal regulations and requirements, including GLP requirements. The results of the preclinical studies, together with manufacturing information and analytical data as well as the results of our Non-IND, IRB-Approved Clinical Studies, would be submitted to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational product to humans for a therapeutic indication and must become effective before human Clinical Trials for such purpose may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises concerns or questions about the conduct of the Clinical Trial, including concerns that human research subjects will be exposed to unreasonable health risks, and imposes a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the Clinical Trial can begin. Submission of an IND may result in the FDA not allowing Clinical Trials to commence or not allowing Clinical Trials to commence on the terms originally specified in the IND. A separate submission to an existing IND must also be made for each successive Clinical Trial conducted during product development, and the FDA must grant permission, either explicitly or implicitly by not objecting, before each Clinical Trial can begin.

Such Clinical Trials involve the administration of the product candidate to human volunteers under the supervision of qualified investigators. Clinical Trials are conducted under protocols

detailing, among other things, the objectives of the Clinical Trial, dosing procedures, subject selection and exclusion criteria and the parameters and criteria to be used in monitoring safety and evaluating effectiveness. Each protocol for our product candidates which we decide to market through the drug development pathway for therapeutic indications must be submitted to the FDA as part of the IND. An IRB for each investigator site proposing to participate in a Clinical Trial must also review and approve the Clinical Trial before it can begin at that site, and the IRB must monitor the Clinical Trial until it is completed. The FDA, the IRB, or the sponsor may suspend or discontinue a Clinical Trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk. Clinical testing of product candidates for therapeutic indications also must satisfy extensive GCP requirements, including requirements for informed consent.

Human Clinical Trials to evaluate therapeutic indications to support New Drug Applications, or NDAs, for marketing approval are typically conducted in three sequential phases, which may overlap or be combined. In certain circumstances, where sufficient evidence of safety and tolerability are collected from preclinical studies and other human experience with a product, subject to discussions and acceptance by the FDA, such as our non-IND human clinical studies, we believe that for the development of such drug candidate, a human Clinical Trial may begin at Phase II rather than starting at Phase I. We would expect to discuss with the FDA such proposal to initiate the clinical development program of a drug candidate in a later phase study without first conducting a Phase I Clinical Trial or Trials.

- *Phase I* — Phase I Clinical Trials involve initial introduction of the investigational product into healthy human volunteers or patients with the target disease or condition. These studies are typically designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, excretion the side effects associated with increasing Doses, and, if possible, to gain early evidence of effectiveness.

- *Phase II* — Phase II Clinical Trials typically involve administration of the investigational product to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks.

- *Phase III* — Phase III Clinical Trials typically involve administration of the investigational product to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed Clinical Trial sites. These Clinical Trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval and physician labeling.

Post-approval trials, sometimes referred to as Phase IV Clinical Trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication and are commonly intended to generate additional safety data regarding use of the product in a clinical setting. In certain instances, the FDA may mandate the performance of Phase IV Clinical Trials as a condition of approval of an NDA.

Progress reports detailing the results of the Clinical Trials for therapeutic indications, among other information, must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected suspected adverse events, findings from other studies or animal or *in vitro* testing that suggest a significant risk for human volunteers and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure.

Concurrent with Clinical Trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing the drug product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and manufacturers must develop, among other things, methods for testing the identity, strength, quality and purity of the final drug product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making the product available in the United States for the disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting an NDA. Orphan designation does not convey any advantage in or shorten the duration of the regulatory review and approval process, though companies developing orphan products are eligible for certain incentives, including tax credits for qualified clinical testing and waiver of application fees.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity during which the FDA may not approve any other applications to market the same therapeutic agent for the same indication, except in limited circumstances, such as a subsequent product's showing of clinical superiority over the product with orphan exclusivity or where the original applicant cannot produce sufficient quantities of product. Competitors, however, may receive approval of different therapeutic agents for the indication for which the orphan product has exclusivity or obtain approval for the same therapeutic agent for a different indication than that for which the orphan product has exclusivity. Orphan product exclusivity could block the approval of one of our products for seven years if a competitor obtains approval for the same therapeutic agent for the same indication before we do, unless we are able to demonstrate that our product is clinically superior. If an orphan designated product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan exclusivity. Further, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Expedited development and review programs for drugs

The FDA maintains several programs intended to facilitate and expedite development and review of new drugs to address unmet medical needs in the treatment of serious or life-threatening diseases or conditions. These programs include Fast Track designation, Breakthrough Therapy designation, Priority Review and Accelerated Approval, and the purpose of these programs is to either expedite the development or review of important new drugs to get them to patients earlier than under standard FDA development and review procedures.

A new drug is eligible for Fast Track designation if it is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address unmet medical needs for such disease or condition. Fast Track designation provides increased opportunities for sponsor interactions with the FDA during preclinical and clinical development, in addition to the

potential for rolling review once a marketing application is filed, meaning that the agency may review portions of the marketing application before the sponsor submits the complete application, as well as Priority Review, discussed below. In addition, a new drug may be eligible for Breakthrough Therapy designation if it is intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Breakthrough Therapy designation provides all the features of Fast Track designation in addition to intensive guidance on an efficient drug development program beginning as early as Phase I, and FDA organizational commitment to expedited development, including involvement of senior managers and experienced review staff in a cross-disciplinary review, where appropriate.

Any product submitted to the FDA for approval, including a product with Fast Track or Breakthrough Therapy designation, may also be eligible for additional FDA programs intended to expedite the review and approval process, including Priority Review designation and accelerated approval. A product is eligible for Priority Review if it has the potential to provide a significant improvement in safety or effectiveness in the treatment, diagnosis or prevention of a serious disease or condition. Under priority review, the FDA must review an application in six months compared to ten months for a standard review. Additionally, products are eligible for accelerated approval if they can be shown to have an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or an effect on a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality which is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

Accelerated approval is usually contingent on a sponsor's agreement to conduct additional post-approval studies to verify and describe the product's clinical benefit. The FDA may withdraw approval of a drug or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. In addition, unless otherwise informed by the FDA, the FDA currently requires, as a condition for accelerated approval, that all advertising and promotional materials that are intended for dissemination or publication within 120 days following marketing approval be submitted to the agency for review during the pre-approval review period, and that after 120 days following marketing approval, all advertising and promotional materials must be submitted at least 30 days prior to the intended time of initial dissemination or publication.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or the time period for FDA review or approval may not be shortened. Furthermore, Fast Track designation, Breakthrough Therapy designation, Priority Review and Accelerated Approval do not change the scientific or medical standards for approval or the quality of evidence necessary to support approval but may expedite the development or review process.

U.S. marketing approval for drugs

Assuming successful completion of the required clinical testing of our product candidates for drug uses, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. An NDA is a request for approval to market a new drug for one or more specified indications and must contain proof of the drug's safety and efficacy. The marketing application may include both negative and ambiguous results of preclinical studies and Clinical

Trials, as well as positive findings. Data may come from company-sponsored Clinical Trials intended to test the safety and efficacy of a product's use or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational product to the satisfaction of the FDA. FDA approval of an NDA must be obtained before a drug may be marketed in the United States. Under the Prescription Drug User Fee Act guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA, for a new molecular entity to review and act on the submission. This review typically takes twelve months from the date the NDA is submitted to the FDA because the FDA has approximately two months to make a "filing" decision on whether to accept the application for review.

In addition, under the Pediatric Research Equity Act of 2003, as amended and reauthorized, certain NDAs or supplements to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. An Agreed Initial Pediatric Study Plan requesting a waiver from the requirement to conduct clinical studies has been submitted to the FDA.

The FDA also may require submission of a REMS, plan to ensure that the benefits of the drug outweigh its risks. The REMS plan could include medication guides, physician communication plans, assessment plans, and/or elements to assure safe use, such as restricted distribution methods, patient registries, or other risk-minimization tools.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

The FDA may refer an application for a novel drug to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the Sponsor product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more Clinical Trial sites to assure compliance with GCP and other requirements and the integrity of the clinical data submitted to the FDA.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and Clinical Trial sites, the FDA may issue an approval letter, or, in some cases, a complete response letter. A complete response letter generally contains a statement of specific conditions that must be met in

order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for the FDA to reconsider the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, depending on the specific risk(s) to be addressed it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase IV Clinical Trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

U.S. post-approval requirements for drugs

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. There is also a continuing, annual prescription drug product program user fee.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-market testing, including Phase IV Clinical Trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

In addition, drug manufacturers and their subcontractors involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with ongoing regulatory requirements, including cGMP, which impose certain procedural and documentation requirements upon us and our contract manufacturers. Failure to comply with statutory and regulatory requirements can subject a manufacturer to possible legal or regulatory action, such as warning letters, suspension of manufacturing, product seizures, injunctions, civil penalties or criminal prosecution.

Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, requirements for post-market studies or Clinical Trials to assess new safety risks, or imposition of distribution or other restrictions under a REMS. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- fines, warning letters or holds on post-approval Clinical Trials;

- refusal of the FDA to approve applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties; and
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs; or mandated modification of promotional materials and labeling and issuance of corrective information.

Other regulatory matters

Manufacturing, sales, promotion and other activities of product candidates following product approval, where applicable, or commercialization are also subject to regulation by numerous regulatory authorities in the United States in addition to the FDA, which may include the Centers for Medicare & Medicaid Services, or CMS, other divisions of the Department of Health and Human Services, the Department of Justice, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments and governmental agencies.

Other healthcare laws

Healthcare providers, physicians, and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our business operations and any current or future arrangements with third-party payors, healthcare providers and physicians may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we develop, market, sell and distribute any drugs for which we obtain marketing approval. In the United States, these laws include, without limitation, state and federal anti-kickback, false claims, physician transparency, and patient data privacy and security laws and regulations, including but not limited to those described below.

- The Anti-Kickback Statute prohibits for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration, directly or indirectly, in cash or in kind, that is intended to induce or reward referrals, including the purchase, recommendation, order or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to ten years in prison, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it.
- Federal civil and criminal false claims laws, and civil monetary penalty laws including the federal False Claims Act, which imposes civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities (including manufacturers) for, among other things, knowingly presenting, or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government. The government may deem manufacturers to have "caused" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers, with respect to drug products, purportedly concealing price concessions in the pricing information submitted to the government for government price reporting purposes, allegedly providing free product to customers with the expectation

that the customers would bill federal healthcare programs for the product, or promoting a product off-label. Claims that include items or services resulting from a violation of the federal Anti-Kickback Statute are false or fraudulent claims for purposes of the False Claims Act. Any future marketing and activities relating to the reporting of wholesaler or estimated retail prices for drug products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, and the sale and marketing of our product and any future product candidates, are subject to scrutiny under this law.

- The Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for knowingly and willfully executing a scheme, or attempting to execute a scheme, to defraud any healthcare benefit program, including private payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, or falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services.

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, imposes, among other things, specified requirements on covered entities and their business associates relating to the privacy and security of individually identifiable health information including mandatory contractual terms and required implementation of technical safeguards of such information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates in some cases, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.

- The Physician Payments Sunshine Act, enacted as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, imposed new annual reporting requirements for certain manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, for certain payments and "transfers of value" provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

- Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, which may be broader in scope and apply regardless of payor. These laws are enforced by various state agencies and through private actions. Some state laws require pharmaceutical companies implement compliance to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant federal government compliance guidance, require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, and restrict marketing practices or require disclosure of marketing expenditures and pricing information. State and foreign laws also govern the privacy and security of health information in some circumstances. These data privacy and security laws may differ from each other in significant ways and often are not pre-empted by HIPAA, which may complicate compliance efforts.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of

investigations, prosecutions, convictions and settlements in the healthcare industry. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other related governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, disgorgement, exclusion from government funded healthcare programs, such as Medicare and Medicaid, reputational harm, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar settlement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to similar actions, penalties and sanctions. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention from its business.

Current and future healthcare reform legislation

In the United States and foreign jurisdictions, there have been and continue to be a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and additional downward pressure on the price that we, or any collaborators, may receive for any approved products.

The ACA, for example, may reduce the profitability of drug products through increased rebates for drugs reimbursed by Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. With the current Administration and Congress, there will likely be additional administrative or legislative changes, including modification, repeal, or replacement of all, or certain provisions of, the ACA, which may impact reimbursement for drugs. Since January 2017, President Trump has signed two executive orders and other directives designed to delay, circumvent or loosen certain requirements mandated by the ACA. While Congress has not passed repeal legislation, the Tax Reform Act includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. While the current administration and CMS have both stated that the ruling will have no immediate effect, it is unclear how this decision, subsequent appeals, if any, and other efforts to repeal and replace the ACA will impact the ACA. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." Congress may consider other legislation to repeal and replace elements of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Additionally, other federal health reform measures have been proposed and adopted in the United States since the ACA was enacted:

- The Budget Control Act of 2011, among other things, included aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013 and, due to subsequent legislative amendments to the statute, including the BBA, will remain in effect through 2027, unless additional Congressional action is taken.

- The American Taxpayer Relief Act of 2012, among other things, also reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers.

Further, there has been heightened governmental scrutiny over the manner in which drug manufacturers set prices for their marketed products, which have resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. In addition, the United States government, state legislatures, and foreign governments have shown significant interest in implementing cost containment programs, including price-controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs to limit the growth of government paid health care costs. For example, the United States government has passed legislation requiring pharmaceutical manufacturers to provide rebates and discounts to certain entities and governmental payors to participate in federal healthcare programs. Further, Congress and the current administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs, and the current administration recently released a "Blueprint," or plan, to reduce the cost of drugs. The current administrations' Blueprint contains certain measures that the U.S. Department of Health and Human Services is already working to implement. Individual states in the United States have also been increasingly passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Compliance with other federal and state laws or requirements; Changing Legal requirements

If any products that we may develop are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Products must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. Manufacturing, labeling, packaging, distribution, sales, promotion and other activities also are potentially subject to federal and state consumer protection and unfair competition laws, among other requirements to which the firm or its products may be subject.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The failure to comply with any of these laws or regulatory requirements subjects firms to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, exclusion from federal healthcare programs, requests for recall, seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, relabeling or repackaging, or refusal to allow a firm to enter into supply contracts, including government contracts. Any claim or action against us for violation of these laws, even if we successfully defend against it, could cause

us to incur significant legal expenses and divert our management's attention from the operation of our business. Prohibitions or restrictions on marketing, sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling or packaging; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

Other U.S. environmental, health and safety laws and regulations

We may be subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

Government regulation of drugs outside of the United States

To market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, Clinical Trials, marketing authorization or identification of an alternate regulatory pathway, manufacturing, commercial sales and distribution of our products. For instance, in the European Economic Area, or the EEA (comprised of the 28 EU Member States plus Iceland, Liechtenstein and Norway), medicinal products must be authorized for marketing by using either the centralized authorization procedure or national authorization procedures.

- *Centralized procedure* — If pursuing marketing authorization of a product candidate for a therapeutic indication under the centralized procedure, following the opining of the EMA's Committee for Medicinal Products for Human Use, or, CHMP, the European Commission issues a single marketing authorization valid across the EEA. The centralized procedure is compulsory for human medicines derived from biotechnology processes or advanced therapy medicinal products (such as gene therapy, somatic cell therapy and tissue engineered products), products that contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, neurodegenerative disorders, diabetes, autoimmune diseases and other immune dysfunctions, viral diseases, and officially designated orphan medicines. For medicines that do not fall within these categories, an

applicant has the option of submitting an application for a centralized marketing authorization to the EMA, as long as the medicine concerned contains a new active substance not yet authorized in the EEA, is a significant therapeutic, scientific or technical innovation, or if its authorization would be in the interest of public health in the EEA. Under the centralized procedure the maximum timeframe for the evaluation of an MAA by the EMA is 210 days, excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP. Accelerated assessment might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, particularly from the point of view of therapeutic innovation. The timeframe for the evaluation of an MAA under the accelerated assessment procedure is 150 days, excluding clock stops.

- *National authorization procedures* — There are also two other possible routes to authorize products for therapeutic indications in several countries, which are available for products that fall outside the scope of the centralized procedure:

  - *Decentralized procedure* — Using the decentralized procedure, an applicant may apply for simultaneous authorization in more than one EU country of medicinal products that have not yet been authorized in any EU country and that do not fall within the mandatory scope of the centralized procedure.

  - *Mutual recognition procedure* — In the mutual recognition procedure, a medicine is first authorized in one EU Member State, in accordance with the national procedures of that country. Following this, additional marketing authorizations can be sought from other EU countries in a procedure whereby the countries concerned recognize the validity of the original, national marketing authorization.

In the EEA, new products for therapeutic indications that are authorized for marketing (i.e., reference products) qualify for eight years of data exclusivity and an additional two years of market exclusivity upon marketing authorization. The data exclusivity period prevents generic or biosimilar applicants from relying on the preclinical and Clinical Trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization in the EU during a period of eight years from the date on which the reference product was first authorized in the EU. The market exclusivity period prevents a successful generic or biosimilar applicant from commercializing its product in the EU until ten years have elapsed from the initial authorization of the reference product in the EU. The ten-year market exclusivity period can be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies.

The criteria for designating an "orphan medicinal product" in the EEA are similar in principle to those in the United States. In the EEA a medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the product will be of significant benefit to those affected by the condition. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. During this ten-year orphan market exclusivity period, no marketing authorization application shall be accepted, and no marketing authorization shall be granted for a similar medicinal product for the same indication. An orphan product can

also obtain an additional two years of market exclusivity in the EU for pediatric studies. The ten-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product for the same indication at any time if (i) the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior; (ii) the applicant consents to a second orphan medicinal product application; or (iii) the applicant cannot supply enough orphan medicinal product.

Similar to the United States, the various phases of non-clinical and clinical research in the European Union are subject to significant regulatory controls.

The Clinical Trials Directive 2001/20/EC, the Directive 2005/28/EC on GCP and the related national implementing provisions of the individual EU Member States govern the system for the approval of Clinical Trials in the European Union. Under this system, an applicant must obtain prior approval from the competent national authority of the EU Member States in which the Clinical Trial is to be conducted. Furthermore, the applicant may only start a Clinical Trial at a specific study site after the competent ethics committee has issued a favorable opinion. The Clinical Trial application must be accompanied by, among other documents, an investigational medicinal product dossier (the Common Technical Document) with supporting information prescribed by Directive 2001/20/EC, Directive 2005/28/EC, where relevant the implementing national provisions of the individual EU Member States and further detailed in applicable guidance documents.

In April 2014, the new Clinical Trials Regulation, (EU) No 536/2014 (Clinical Trials Regulation) was adopted. The Regulation is anticipated to come into application in 2019. The Clinical Trials Regulation will be directly applicable in all the EU Member States, repealing the current Clinical Trials Directive 2001/20/EC. Conduct of all Clinical Trials performed in the European Union will continue to be bound by currently applicable provisions until the new Clinical Trials Regulation becomes applicable. The extent to which ongoing Clinical Trials will be governed by the Clinical Trials Regulation will depend on when the Clinical Trials Regulation becomes applicable and on the duration of the individual Clinical Trial. If a Clinical Trial continues for more than three years from the day on which the Clinical Trials Regulation becomes applicable the Clinical Trials Regulation will at that time begin to apply to the Clinical Trial. The new Clinical Trials Regulation aims to simplify and streamline the approval of Clinical Trials in the European Union. The main characteristics of the regulation include: a streamlined application procedure via a single-entry point, the "EU portal"; a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for Clinical Trial sponsors; and a harmonized procedure for the assessment of applications for Clinical Trials, which is divided in two parts. Part I is assessed by the competent authorities of all EU Member States in which an application for authorization of a Clinical Trial has been submitted (Member States concerned). Part II is assessed separately by each Member State concerned. Strict leadlines have been established for the assessment of Clinical Trial applications. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the concerned EU Member State. However, overall related timelines will be defined by the Clinical Trials Regulation.

The collection and use of personal health data in the European Union, previously governed by the provisions of the Data Protection Directive, is now governed by the GDPR, which became effective on May 25, 2018. While the Data Protection Directive did not apply to organizations based outside the EU, the GDPR has expanded its reach to include any business, regardless of its location, that provides goods or services to residents in the EU. This expansion would incorporate any Clinical Trial activities in EU members states. The GDPR imposes strict requirements on controllers and processors of personal data, including special protections for "sensitive information" which includes health and genetic information of data subjects residing in the EU. GDPR grants

individuals the opportunity to object to the processing of their personal information, allows them to request deletion of personal information in certain circumstances, and provides the individual with an express right to seek legal remedies in the event the individual believes his or her rights have been violated. Further, the GDPR imposes strict rules on the transfer of personal data out of the European Union to the United States or other regions that have not been deemed to offer "adequate" privacy protections. Failure to comply with the requirements of the GDPR and the related national data protection laws of the European Union Member States, which may deviate slightly from the GDPR, may result in fines of up to 4% of global revenues, or € 20,000,000, whichever is greater. As a result of the implementation of the GDPR, we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules.

There is significant uncertainty related to the manner in which data protection authorities will seek to enforce compliance with GDPR. For example, it is not clear if the authorities will conduct random audits of companies doing business in the EU, or if the authorities will wait for complaints to be filed by individuals who claim their rights have been violated. Enforcement uncertainty and the costs associated with ensuring GDPR compliance are onerous and may adversely affect our business, financial condition, results of operations and prospects.

Additionally, should we elect one or more product candidates to develop and market as non-drug products in foreign countries, such products would also be subject to regulation under various national, local, and international laws that include provision governing, among other things, the formulation, manufacturing, packaging, labeling, advertising. These regulations may prevent or delay entry into the market or prevent or delay the introduction, or require the reformulation, of certain of our non-drug product candidates.

The regulatory environment outside the United States varies and in general is less developed then in the United States, but some exceptions do exist. The regulatory requirements for nutritional non-drug products and food products outside of the United States varies greatly from jurisdiction to jurisdiction. Each jurisdiction may have its own regulatory framework regarding nutritional non-drug products and food products. The two leading jurisdictions, the United States and the Europe, currently have and may continue to in the future to have distinctly different regulatory regimes with different rules and requirements nutritional non-drug products and food products, with, for example, the European Union having a stronger process for claims review and preapproval for nutritional products. Regulation in Europe is exercised primarily through the European Union, which regulates the combined market of each of its member states. Other European countries, such as Switzerland, have voluntarily adopted laws and regulations that mirror those of the European Union with respect to dietary products.

We cannot predict how the global regulatory landscape regarding our possible nutritional non-drug products or food products, if any, will evolve and we may incur increased regulatory costs as regulations in the jurisdictions in which we operate evolve or change. We cannot predict whether or when any jurisdiction will change its regulations with respect to any of our product candidates.

Should we utilize third part distributors, compliance with such foreign governmental regulations would generally be the responsibility of such distributors, who may be independent contractors over whom we have limited control.

Government regulation of food for special medical purpose in the European Union

The regulatory requirements for foods for special medical purposes, or FSMPs, in the European Union cover FSMP development and commercialization.

In the European Union, FSMPs are designed to feed patients who, because of a particular disease, disorder or medical condition, have nutritional needs that cannot be met by consuming standard foodstuffs. European Union Regulation defines *food for special medical purposes'* as food

specially processed or formulated and intended for the dietary management of patients, including infants, to be used under medical supervision; it is intended for the exclusive or partial feeding of patients with a limited, impaired or disturbed capacity to take, digest, absorb, metabolize or excrete ordinary food or certain nutrients contained therein, or metabolites, or with other medically-determined nutrient requirements, whose dietary management cannot be achieved by modification of the normal diet alone.

Businesses intending to commercialize FSMPs in the European Union are required to register their FSMPs by submitting notifications regarding FSMP use, demonstrating compliance with applicable European Union rules, prior to market commercialization. These notifications to competent authority of each European Union Member State include information appearing on the label, and any other information the competent authority may reasonably request to establish compliance with this Regulation.

The European Commission may decide, by means of implementing acts (a) whether a given food falls within the scope of this Regulation; and (b) to which specific category of food a given food belongs. European Food Safety Authority Guidance provides, among other requirements, that the dossier must include an explanation of the scientific and medical basis on which it has been concluded that the use of the specific food product is necessary or is more practical or safer than the exclusive use of non-FSMP foodstuffs.

FSMPs can also fall within the scope of the novel food legislation in the European Union. Where an ingredient used in the FSMP to be marketed in the European Union falls within the definition of a novel food ingredient' prior authorization for use of the ingredient needs to be sought. A "novel" food or food ingredients as food that has not been consumed to a significant degree by humans in the European Union before May 15, 1997 and that falls within one of the ten food categories listed. Novel foods and novel food ingredients can only be authorized if they do not pose a safety risk to human health, their intended use does not mislead the consumer and they do not differ from the food they are intended to replace in such a way that its normal consumption would be nutritionally disadvantageous for the consumer. The authorization procedure is likely to take between 12 and 18 months.

In accordance with European Union Clinical Trials directives, before a Clinical Trial site is allowed to start enrolling patients in a Clinical Trial, the IRB/independent ethics committee, or IEC, must provide a positive opinion concerning the study protocol and all study-related materials. The competent authorities of the relevant European Union Member State must also provide their related authorization. Clinical Trials involving the investigation of the action of non-medicinal products (e.g., foods, such as many FSMPs), are not covered and are not required to register the Clinical Trial or to complete a Clinical Trial application (CTA) for approval by an European Union Member State.

Employees

As of December 31, 2018, we had 49 full-time employees. Of those employees, 23 have Ph.D. or M.D. degrees and 32 are engaged in research and development activities. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Facilities

We lease a facility containing 19,200 square feet of laboratory and office space, which is located at 840 Memorial Drive, Cambridge, Massachusetts. The lease expires in April 2021, subject to two options to extend the lease for a total of six years. We believe that our current facilities are sufficient to meet our current and near-term needs.

Legal proceedings

We are not currently a party to any material legal proceedings.

MANAGEMENT

Executive officers

The following table sets forth certain information, as of April 1, 2019, concerning our executive officers who, subject to rights pursuant to any employment agreements, serve at the pleasure of our board of directors:

Name	Age	Position
William Hinshaw	50	President and Chief Executive Officer, Director
Thomas Leggett	42	Senior Vice President of Finance, Chief Financial Officer
Stephen Mitchener, PharmD.	40	Senior Vice President, Chief Business Officer
Manu Chakravarthy, M.D., Ph.D.	45	Senior Vice President, Chief Medical Officer
Tony Tramontin, Ph.D.	51	Senior Vice President of Research & Development, Chief Scientific Officer
Paul Fehlner, J.D., Ph.D.	55	Senior Vice President, Chief Intellectual Property Officer

The following is a biographical summary of the experience of our executive officers.

William Hinshaw has served as our President and Chief Executive Officer and as a member of our board of directors since June 2018. Prior to joining us, Mr. Hinshaw served in increasing roles of responsibility at Novartis Pharmaceuticals Corporation, a pharmaceutical company, from December 2003 until November 2017, most recently as the Executive Vice President and Head of U.S. Oncology. Mr. Hinshaw holds a B.S. in molecular biology from the University of Wisconsin. We believe that Mr. Hinshaw's experience, qualifications, attributes and skills, including experience in operations management and executive leadership qualify him to serve on our board of directors.

Thomas Leggett has served as our Senior Vice President of Finance and Chief Financial Officer since January 2017. Prior to joining us, Mr. Leggett served as the Treasurer and Head of Business Development Finance at Purdue Pharma L.P., a pharmaceutical company, from May 2015 to December 2016. From November 2009 to May 2015, Mr. Leggett was an Executive Director at UBS Securities LLC, an investment bank, in the Global Healthcare Group with a primary focus in the biopharmaceutical sector. Mr. Leggett holds an M.B.A. in finance from The Wharton School at the University of Pennsylvania and a B.A. in economics from Columbia University.

Stephen Mitchener, PharmD. has served as our Senior Vice President and Chief Business Officer since August 2018. Prior to joining us, Dr. Mitchener served in increasing roles of responsibility at Novartis Pharmaceuticals Corporation, a pharmaceutical company, from April 2005 to August 2018, most recently as Head of U.S. Oncology Strategy, Partnering and Operations. He previously served as a board observer for COTA Healthcare on behalf of Novartis Oncology. Dr. Mitchener holds a Post-Doctorate from Rutgers University and a PharmD. from the University of North Carolina at Chapel Hill.

Manu Chakravarthy, M.D., Ph.D. has served as our Senior Vice President of Clinical Development and Chief Medical Officer since August 2017. Prior to joining us, Dr. Chakravarthy served as the Global Head of Innovation Strategy and External R&D in diabetes and cardiovascular research at Eli Lilly & Company, a pharmaceutical company, from August 2015 to June 2017. Previously, Dr. Chakravarthy spent more than seven years at Merck & Co., from September 2008 to August 2015, where he assumed positions of increasing responsibility and leadership within the company, most recently as a Distinguished Scientist and leader of the Discovery Medicine group for diabetes and cardiometabolic diseases within Translational Medicine. He is also Board-certified in Internal Medicine and Endocrinology, Diabetes & Metabolism and an Adjunct Clinical Assistant Professor of Medicine at the Rutgers School of Medicine, New Jersey. Dr. Chakravarthy holds an M.D. from the University of Texas Houston Medical School, a Ph.D. in cell biology and physiology

from the University of Texas Graduate School of Biomedical Sciences and the MD Anderson Cancer Center and a B.A. in biology and chemistry from St. John's University.

Tony Tramontin, Ph.D. has served as our Senior Vice President of Research & Development and Chief Scientific Officer since June 2017. Prior to joining us, Dr. Tramontin served as a Partner at McKinsey & Company, a consulting firm, in its Global Healthcare Practice from 2003 until May 2017. Dr. Tramontin holds a Ph.D. in biology (neuroscience and endocrinology) from the University of Washington and a B.A. in business from the University of Notre Dame.

Paul Fehlner, J.D., Ph.D. has served as our Senior Vice President and Chief Intellectual Property Officer since April 2018. In addition, Dr. Fehlner has served as a Principal at Life Sciences Innovation LLC, an intellectual property and legal consulting firm, since December 2017. From November 2008 through November 2017, Dr. Fehlner served as Global Head of Intellectual Property at Novartis Pharma AG in Basel, Switzerland. Dr. Fehlner holds a J.D. from Fordham University School of Law, a Ph.D. in immunology and biochemistry from The Rockefeller University and a B.S. in chemistry from Haverford College.

Non-employee directors

The following table sets forth certain information, as of April 1, 2019, concerning our non-employees who serve on our board of directors:

Name	Age	Position
Non-employee directors:		
David R. Epstein[2][3]	57	Chairman, Director
William D. Baird III[1][2]	47	Director
Grégory Behar[1]	49	Director
David A. Berry, M.D., Ph.D.[3]	41	Director
Stephen Hoge, M.D.[2]	43	Director
Gary Pisano, Ph.D.[1]	57	Director
Cristina M. Rondinone, Ph.D.[3]	58	Director
Christopher A. Viehbacher	58	Director

(1) Member of audit committee.

(2) Member of compensation committee.

(3) Member of nominating and corporate governance committee.

The following is a biographical summary of the experience of our non-employee directors.

David R. Epstein has served as our Chairman and as a member of our board of directors since December 2017. Mr. Epstein has also served as Executive Partner at Flagship Pioneering, a private equity and venture capital firm, since January 2017. From January 2010 to July 2016, Mr. Epstein served as Chief Executive Officer of Novartis Pharmaceuticals Corporation, a pharmaceutical company. He currently serves on the boards of directors of International Flavors and Fragrances, Inc. (NYSE: IFF), Evelo Biosciences, Inc. (Nasdaq: EVLO) and of Rubius Therapeutics, Inc. (Nasdaq: RUBY), as Executive Chairman. Mr. Epstein holds an M.B.A. in finance and marketing from Columbia Business School and a B.S. in pharmacy from Rutgers University. We believe that Mr. Epstein's extensive experience serving in executive roles in the life sciences industry and leading the development and commercialization of numerous therapeutics qualify him to serve on our board of directors.

William D. Baird III has served as a member of our board of directors since June 2018. He joined bluebird bio, Inc. as Chief Financial Officer in February 2019 and previously served as Chief

Financial Officer of Amicus Therapeutics, Inc., a pharmaceutical company, from April 2012 to December 2018. Mr. Baird holds an M.B.A. in finance from The Wharton School of the University of Pennsylvania and a B.S.F.S. in international affairs from the Edmund A. Walsh School of Foreign Service of Georgetown University. We believe Mr. Baird's broad experience in pharmaceutical finance and executive management roles qualify him to serve on our board of directors.

Grégory Behar has served as a member of our board of directors since February 2016. Mr. Behar has also served as Chief Executive Officer of Nestlé Health Science S.A., a health sciences company, since October 2014. He currently serves on the board of directors of Aimmune Therapeutics, Inc. (Nasdaq: AIMT) and Seres Therapeutics, Inc. (Nasdaq: MCRB). Mr. Behar holds an M.B.A. from INSEAD in France, a M.S. in mechanical engineering and manufacturing from EPFL in Switzerland, and a B.S. in mechanical engineering from the University of California, Los Angeles. We believe that Mr. Behar's extensive business experience in the health sciences and pharmaceutical industries qualifies him to serve on our board of directors.

David A. Berry, M.D., Ph.D. helped co-found the company in 2008 and has served as a member of our board of directors since February 2011. Dr. Berry has also served in roles of increasing responsibility at Flagship Pioneering Inc. since January 2005 most recently as General Partner. He previously served as a director of Seres Therapeutics, Inc. (Nasdaq: MCRB). He holds an M.D. from Harvard Medical School, a Ph.D. in biological engineering from the Massachusetts Institute of Technology Biological Engineering Division and a B.S. in brain and cognitive sciences from the Massachusetts Institute of Technology. We believe that Dr. Berry's extensive experience in the life sciences industry qualifies him to serve on our board of directors.

Stephen Hoge, M.D. has served as a member of our board of directors since May 2014. He has also served in roles of increasing responsibility at Moderna Therapeutics, Inc., a biopharmaceutical company, since December 2012, most recently as President. Dr. Hoge holds an M.D. from the University of California, San Francisco and a B.A. in neuroscience from Amherst College. We believe that Dr. Hoge's medical knowledge and extensive experiences in the pharmaceutical industry as a management consultant and executive at a biotechnology company qualify him to serve on our board of directors.

Gary Pisano, Ph.D. has served as a member of our board of directors since October 2011. Dr. Pisano is the Harry E. Figgie, Jr. Professor of Business Administration and Senior Associate Dean for Faculty Development at the Harvard Business School. He has served on the Harvard faculty since 1988. Dr. Pisano's research and teaching focus on technology and operations strategy, the management of innovation and intellectual property, and competitive strategy. For more than two decades, he has consulted extensively on these issues with companies in the pharmaceutical, biotechnology, medical device, specialty chemical and healthcare industries. He has previously served as a director of Axovant Sciences Ltd. (Nasdaq: AXON) and Patheon NV (NYSE: PTHN). Dr. Pisano holds a Ph.D. in business administration from the University of California, Berkeley and a B.A. in economics from Yale University. We believe that Dr. Pisano's knowledge of business administration, innovation, and strategy, particularly within the healthcare industry, qualifies him to serve on our board of directors.

Cristina M. Rondinone, Ph.D. has served as a member of our board since June 2018. Dr. Rondinone has also served in roles of increasing responsibility at MedImmune, LLC, a wholly owned subsidiary of AstraZeneca, since March 2011, most recently as Senior Vice President R&D, Head of Cardiovascular, Renal and Metabolic Diseases Innovative Medicines and Early Development. Dr. Rondinone holds a Docent in molecular medicine from the University of Goteborg, Sweden School of Medicine and a M.Sc. and Ph.D. in biological sciences from the University of Buenos Aires. We believe that Dr. Rondinone's extensive industry experience qualifies her to serve on our board of directors.

Christopher A. Viehbacher has served as a member of our board of directors since September 2015. Mr. Viehbacher has also served as the Managing Partner of Gurnet Point Capital, an investment fund, since 2015. He currently serves on the boards of directors of PureTech Health plc. From 2011 to 2104, Mr. Viehbacher served as the Chief Executive Officer of Sanofi S.A., a French pharmaceutical company. He holds a B.C. from Queen's University. We believe that Mr. Viehbacher's extensive experience in the pharmaceutical and healthcare industries qualifies him to serve on our board of directors.

Our Board of Directors

As of December 31, 2018, our board of directors consisted of nine members, each of whom is a member pursuant to the board composition provisions of our certificate of incorporation and agreements with our stockholders. These board composition provisions will terminate upon the completion of this offering. Upon the termination of these provisions, there will be no further contractual obligations regarding the election of our directors. Our nominating and corporate governance committee and our board of directors may therefore consider a broad range of factors relating to the qualifications and background of nominees. Our nominating and corporate governance committee's and our board of directors' priority in selecting board members is the identification of persons who will further the interests of our stockholders through their established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business, understanding of the competitive landscape, professional and personal experiences and expertise relevant to our growth strategy. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal. Our restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least two-thirds of the votes that all of our stockholders would be entitled to cast in an annual election of directors, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

Director independence

Our board of directors has determined that all members of the board of directors, except William Hinshaw, are independent directors, including for purposes of the rules of The Nasdaq Global Market and the SEC. In making such independence determination, our board of directors considered the relationships that each non-employee director has with us and all other facts and circumstances that our board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director. In considering the independence of the directors listed above, our board of directors considered the association of our directors with the holders of more than 5% of our common stock. Upon the completion of this offering, we expect that the composition and functioning of our board of directors and each of our committees will comply with all applicable requirements of The Nasdaq Global Market and the rules and regulations of the SEC, subject to applicable phase-in periods. Under Nasdaq listing rule 5615(b)(1) a company listing in connection with its initial public offering is permitted to phase in its compliance with the independent committee requirements, the committee composition requirements and the majority independent board requirement. There are no family relationships among any of our directors or executive officers. William Hinshaw is not an independent director under these rules because he is an executive officer of our company.

Staggered board

In accordance with the terms of our restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering, our board of directors will be divided into three staggered classes of directors and each director will be assigned to one of the three classes. At each annual meeting of the stockholders, a class of directors will be elected for a three-year term to succeed the directors of the same class whose terms are then expiring. The terms of the directors will expire upon the election and qualification of successor directors at the annual meeting of stockholders to be held during the years 2020 for Class I directors, 2021 for Class II directors and 2022 for Class III directors.

- Our Class I directors will be Christopher Viehbacher, David Epstein and David Berry;
- Our Class II directors will be Grégory Behar, Gary Pisano and Cristina M. Rondinone; and
- Our Class III directors will be William D. Baird III, William Hinshaw and Stephen Hoge.

Our restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering will provide that the number of directors shall be fixed from time to time by a resolution of the majority of our board of directors.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change in control.

Board leadership structure and board's role in risk oversight

David R. Epstein is the current Chairman of our board of directors and William Hinshaw is our current Chief Executive Officer, hence the roles of Chairman of our board of directors and Chief Executive Officer are separated. We believe that separating these positions allows our Chief Executive Officer to focus on our day-to-day business, while allowing our Chairman of the board to lead the board of directors in its fundamental role of providing advice to and independent oversight of management. Our board of directors recognizes the time, effort and energy that the Chief Executive Officer is required to devote to his position in the current business environment, as well as the commitment required to serve as our Chairman, particularly as the board of directors' oversight responsibilities continue to grow. While our amended and restated bylaws and corporate governance guidelines do not require that our Chairman and Chief Executive Officer positions be separate, our board of directors believes that having separate positions is the appropriate leadership structure for us at this time and demonstrates our commitment to good corporate governance.

Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including risks relating to our financial condition, development and commercialization activities, operations, strategic direction and intellectual property as more fully discussed in the section entitled "Risk Factors" appearing elsewhere in this prospectus. Management is responsible for the day-to-day management of risks we face, while our board of directors, as a whole and through its committees, has responsibility for the oversight of risk management. In its risk oversight role, our board of directors has the responsibility to satisfy itself that the risk management processes designed and implemented by management are adequate and functioning as designed.

The role of the board of directors in overseeing the management of our risks is conducted primarily through committees of the board of directors, as disclosed in the descriptions of each of the committees below and in the charters of each of the committees. The full board of directors (or the appropriate board committee in the case of risks that are under the purview of a particular committee) discusses with management our major risk exposures, their potential impact on us, and

the steps we take to manage them. When a board committee is responsible for evaluating and overseeing the management of a particular risk or risks, the chairman of the relevant committee reports on the discussion to the full board of directors during the committee reports portion of the next board meeting. This enables the board of directors and its committees to coordinate the risk oversight role, particularly with respect to risk interrelationships.

Committees of our board of directors

Our board of directors has established an audit committee and a compensation committee, and intends to establish a nominating and corporate governance committee, each of which will operate pursuant to a charter adopted by our board of directors and will be effective upon the effectiveness of the registration statement of which this prospectus is a part. Upon the effectiveness of the registration statement of which this prospectus is a part, the composition and functioning of all of our committees will comply with all applicable requirements of the Sarbanes-Oxley Act of 2002, Nasdaq and SEC rules and regulations.

Audit committee

Effective upon the closing of this offering Grégory Behar, William D. Baird III, and Gary Pisano will serve on the audit committee, which will be chaired by Mr. Baird. Our board of directors has determined that each of the members of the audit committee "independent" for audit committee purposes as that term is defined in the rules of the SEC and the applicable Nasdaq rules, and each of the committee members has sufficient knowledge in financial and auditing matters to serve on the audit committee. Our board of directors has designated Mr. Baird as an "audit committee financial expert," as defined under the applicable rules of the SEC. The audit committee's responsibilities include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- pre-approving auditing and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the overall audit plan with our independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- coordinating the oversight and reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending based upon the audit committee's review and discussions with management and our independent registered public accounting firm whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;
- reviewing all related person transactions for potential conflict of interest situations and approving all such transactions; and
- reviewing quarterly earnings releases.

Compensation committee

Effective upon the closing of this offering David R. Epstein, William D. Baird III, and Stephen Hoge will serve on the compensation committee, which will be chaired by Mr. Epstein. Our board of directors has determined that each member of the compensation committee is "independent" as defined in the applicable Nasdaq rules. The compensation committee's responsibilities include:

- annually reviewing and recommending to the board of directors the corporate goals and objectives relevant to the compensation of our Chief Executive Officer;
- evaluating the performance of our Chief Executive Officer in light of such corporate goals and objectives and based on such evaluation: (i) recommending to the board of directors the cash compensation of our Chief Executive Officer and (ii) recommending grants and awards to our Chief Executive Officer under equity-based plans;
- reviewing and approving or recommending to the board of directors the cash compensation of our other executive officers;
- reviewing and establishing our overall management compensation, philosophy and policy;
- overseeing and administering our compensation and similar plans;
- evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable Nasdaq rules;
- reviewing and approving our policies and procedures for the grant of equity-based awards;
- reviewing and recommending to the board of directors the compensation of our directors;
- preparing the compensation committee report required by SEC rules, if and when required, to be included in our annual proxy statement; and
- reviewing and approving the retention, termination or compensation of any consulting firm or outside advisor to assist in the evaluation of compensation matters.

Nominating and corporate governance committee

Effective upon the closing of this offering David R. Epstein, David A. Berry, and Christina M. Rondinone will serve on the nominating and corporate governance committee, which will be chaired by Mr. Epstein. Our board of directors has determined that each of the members of the nominating and corporate governance committee are "independent" as defined in the applicable Nasdaq rules. The nominating and corporate governance committee's responsibilities include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- reviewing the composition of the board of directors to ensure that it is composed of members containing the appropriate skills and expertise to advise us;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board's committees;
- developing and recommending to the board of directors a code of business conduct and ethics and a set of corporate governance guidelines; and

- overseeing the evaluation of our board of directors and management. Our board of directors may from time to time establish other committees.

Compensation committee interlocks and insider participation

None of the members of our compensation committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee.

Corporate governance

We have adopted a written code of business conduct and ethics, which became effective upon the effectiveness of the registration statement of which this prospectus is a part, that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. Following the effectiveness of the registration statement of which this prospectus is a part, a current copy of the code is posted on the investor relations section of our website, which is located at http://www.axcellahealth.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

EXECUTIVE COMPENSATION

Executive compensation overview

Our executive compensation program has reflected our growth and development-oriented corporate culture. To date, the compensation of the individuals listed below, whom we refer to as our named executive officers, has primarily consisted of a combination of base salary, bonuses and long-term incentive compensation. Our named executive officers, like all of our full-time employees, are eligible to participate in our health and welfare benefit plans. As we transition from a private company to a publicly traded company, we will evaluate our compensation values and philosophy and compensation plans and arrangements as circumstances require. At a minimum, we expect to review executive compensation annually with input from a compensation consultant. As part of this review process, we expect the board of directors and the compensation committee to apply our values and philosophy, while considering the compensation levels needed to ensure our executive compensation program remains competitive. We will also review whether we are meeting our retention objectives and the potential cost of replacing a key employee.

2018 Summary compensation table

The following table presents information regarding the total compensation awarded to, earned by, and paid to our named executive officers for services rendered to us in all capacities for the year indicated.

Name and principal position	Year	Salary ($)	Bonus ($)	Non-Equity Incentive Plan Compensation ($) [1]	Option awards ($) [2]	All other compen-sation ($)	Total ($)
William Hinshaw, *President and CEO (Principal Executive Officer)*[3]	2018	278,910[4]		231,563	3,608,347	160,000[7]	4,278,820
Thomas Leggett, *CFO (Principal Financial Officer)*	2018	341,700		137,534	392,188	—	871,422
Stephen Mitchener, PharmD., *Chief Business Officer*	2018	127,563[5]	15,000[6]	53,969	674,923	21,210[8]	892,665
Robert Connelly, *Former President and CEO (Principal Executive Officer)*[3]	2018	206,291		—	—	355,578[9]	561,869

(1) The amounts reported represent cash incentive compensation based on the Board's assessment of the achievement of company and individual performance objectives for the year ended December 31, 2018, which were paid in February 2019.

(2) The amounts reported represent the aggregate grant-date fair value of stock options awarded in 2018, calculated in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant-date fair value are set forth in Note 7 to our audited consolidated financial statements appearing at the end of this prospectus.

(3) Mr. Hinshaw currently serves as our President and Chief Executive Officer and commenced employment with us in June 2018. Previously, Mr. Connelly served as our President and Chief Executive Officer until May 2018.

(4) Mr. Hinshaw commenced employment with us in June 2018. His annual base salary for 2018 was $475,000.

(5) Dr. Mitchener commenced employment with us in August 2018. His annual base salary for 2018 was $330,000.

(6) The amounts reported represent a one-time sign-on bonus.

(7) The amounts reported represent a one-time relocation stipend.

(8) The amounts reported represent a housing stipend of $15,000 and commuting expenses of $6,210.

(9) The amounts reported represent $337,500 in severance payments and $18,078 in COBRA premiums.

Narrative disclosure to 2018 summary compensation table

Base Salary — Each named executive officer's base salary is a fixed component of annual compensation for performing specific duties and functions, and has been established by our board of directors taking into account each individual's role, responsibilities, skills and experience.

Cash Bonus — Our annual bonus program is intended to reward our named executive officers for meeting objective or subjective performance goals for a fiscal year.

Long-Term Equity Incentives — Our equity grant program is intended to align the interests of our named executive officers with those of our stockholders and to motivate them to make important contributions to our performance.

Employment and consulting arrangements with our named executive officers

William Hinshaw

Under our Amended and Restated Employment Agreement with Mr. Hinshaw, dated December 20, 2018, or the Hinshaw Employment Agreement, he will continue to serve as our President and Chief Executive Officer on an at will basis. Mr. Hinshaw currently receives a base salary of $475,000 per year, which is subject to periodic review and adjustment. Mr. Hinshaw is also eligible for an annual performance bonus targeted at 50% of his base salary and is eligible to participate in the employee benefit plans generally available to our employees, subject to the terms of those plans. Effective upon the completion of this offering, Mr. Hinshaw's base salary will increase to $500,000 per year and his annual performance bonus target will increase to 55% of his base salary. In addition, in March 2019, we approved a grant to Mr. Hinshaw of a performance option to purchase 60,803 shares of our common stock, contingent upon the completion of an initial public offering that the board of directors deems successful (the "Vesting Start Date"). The option will vest 25% on the first anniversary of the Vesting Start Date, with the remainder vesting in equal quarterly installments over the three years following the first anniversary of the Vesting Start Date.

The Hinshaw Employment Agreement further provides that if Mr. Hinshaw's employment is terminated by us without Cause (as defined in the Hinshaw Employment Agreement) or Mr. Hinshaw resigns for Good Reason (as defined in the Hinshaw Employment Agreement), he will be entitled to receive: (i) base salary continuation for 12 months following termination, or the Hinshaw Severance Amount, and, (ii) if Mr. Hinshaw is enrolled in our health care program immediately prior to the date of termination and properly elects to receive COBRA benefits, 12 months of COBRA premiums for himself and his eligible dependents at our normal rate of contribution for employees for coverage at the level in effect immediately prior to the date of termination (or a monthly cash payment in lieu thereof if we determine we cannot pay such amounts without potentially violating applicable law). Payment of the Hinshaw Severance Amount shall immediately cease if Mr. Hinshaw breaches the terms of the Restrictive Covenants Agreement between him and us. In lieu of the severance payments and benefits set forth above, in the event Mr. Hinshaw's employment is by us without Cause or he resigns for Good Reason, in either case within 12 months following a Change in Control (as defined in the Hinshaw Employment Agreement), he will be entitled to receive: (i) a lump sum cash amount equal to 1.5 times the sum of (A) his current base salary (or his base salary in effect prior to the Change in Control, if higher) plus (B) his target annual cash incentive compensation for the year of termination, (ii) if Mr. Hinshaw is enrolled in our health care program immediately prior to the date of termination and properly elects to receive COBRA benefits, 18 months of COBRA premiums for himself and his eligible dependents at our normal rate of contribution for employees for coverage at the level in effect immediately prior to the date of termination (or a monthly cash payment in lieu thereof if we determine we cannot pay such amounts without potentially violating applicable law), and

(iii) notwithstanding anything to the contrary provided in the applicable award agreement, accelerated vesting of 100% of all Time-Based Equity Awards held by Mr. Hinshaw.

Thomas Leggett

Under our Amended and Restated Employment Agreement with Mr. Leggett, dated December 31, 2018, or the Leggett Employment Agreement, he will continue to serve as our Senior Vice President and Chief Financial Officer on an at-will basis. Mr. Leggett currently receives a base salary of $341,700 per year, which is subject to periodic review and adjustment. Mr. Leggett is also eligible for an annual performance bonus targeted at 40% of his base salary and is eligible to participate in the employee benefit plans generally available to our employees, subject to the terms of those plans.

The Leggett Employment Agreement further provides that if Mr. Leggett's employment is terminated by us without Cause (as defined in the Leggett Employment Agreement) or Mr. Leggett resigns for Good Reason (as defined in the Leggett Employment Agreement), he will be entitled to receive: (i) base salary continuation for nine months following termination, or the Leggett Severance Amount, and, (ii) if Mr. Leggett is enrolled in our health care program immediately prior to the date of termination and properly elects to receive COBRA benefits, nine months of COBRA premiums for himself and his eligible dependents at our normal rate of contribution for employees for coverage at the level in effect immediately prior to the date of termination (or a monthly cash payment in lieu thereof if we determine we cannot pay such amounts without potentially violating applicable law). Payment of the Leggett Severance Amount shall immediately cease if Mr. Leggett breaches the terms of the Restrictive Covenants Agreement between him and us. In lieu of the severance payments and benefits set forth above, in the event Mr. Leggett's employment is by us without Cause or he resigns for Good Reason, in either case within 12 months following a Change in Control (as defined in the Leggett Employment Agreement), he will be entitled to receive: (i) a lump sum cash amount equal to 1 times the sum of (A) his current base salary (or his base salary in effect prior to the Change in Control, if higher) plus (B) his target annual cash incentive compensation for the year of termination, (ii) if Mr. Leggett is enrolled in our health care program immediately prior to the date of termination and properly elects to receive COBRA benefits, 12 months of COBRA premiums for himself and his eligible dependents at our normal rate of contribution for employees for coverage at the level in effect immediately prior to the date of termination (or a monthly cash payment in lieu thereof if we determine we cannot pay such amounts without potentially violating applicable law), and (iii) except as otherwise provided in the applicable award agreement, accelerated vesting of 100% of all Time-Based Equity Awards held by Mr. Leggett.

Stephen Mitchener

Under our Amended and Restated Employment Agreement with Dr. Mitchener, dated December 29, 2018, or the Mitchener Employment Agreement, he will continue to serve as our Senior Vice President and Chief Business Officer on an at-will basis. Dr. Mitchener currently receives a base salary of $330,000 per year, which is subject to periodic review and adjustment. Dr. Mitchener is also eligible for an annual performance bonus targeted at 40% of his base salary and is eligible to participate in the employee benefit plans generally available to our employees, subject to the terms of those plans.

Under the Mitchener Employment Agreement, Dr. Mitchener is entitled to a housing stipend of up to $3,000 per month as long as his main domicile remains in New Jersey. The Company will review the housing stipend two years after Dr. Mitchener's start date and may extend or terminate it in its sole discretion. Dr. Mitchener is also entitled to receive reimbursements of up to $600 per week for commuting expenses as long as his main domicile remains in New Jersey. In addition,

under the Mitchener Employment Agreement, Dr. Mitchener is also obligated to repay the entire signing bonus he received from the Company (equal to $15,000 less taxes and withholding) in the event that, prior to February 13, 2019, he resigns other than for Good Reasons or his employment is terminated for Cause (as such terms are defined in the Mitchener Employment Agreement).

The Mitchener Employment Agreement also provides for the grant of the following equity awards subject to final approval by the board of directors or the compensation committee: (i) an option to purchase the Company's common stock in an amount equal to approximately 0.6% of the Company's stock on a fully diluted basis following the closing of the Company's Series E financing round (the "New Hire Options"), and (ii) if during the 12 month period after August 13, 2018, which may be extended to 24 months with modified terms at the discretion of the Compensation Committee, the Company signs a partnership agreement with an upfront cash payment of at least $10 million with a third party to develop one or more therapeutic products an additional option to purchase the Company's common stock in an amount equal to approximately 0.25% of the Company's stock on a fully diluted basis following the closing of the Company's Series E financing round (the "Performance Options"). The New Hire Options will vest 25% on the first anniversary of Dr. Mitchener's start date, with the remainder vesting in equal quarterly installments over the three years following the first anniversary of Dr. Mitchener's start date. The Performance Options will vest 25% on the first anniversary of the closing of the partnership satisfying the performance criteria, with the remainder vesting in equal quarterly installments over the three years following the first anniversary of closing the partnership.

The Mitchener Employment Agreement further provides that if Dr. Mitchener's employment is terminated by us without Cause (as defined in the Mitchener Employment Agreement) or Dr. Mitchener resigns for Good Reason (as defined in the Mitchener Employment Agreement), he will be entitled to receive: (i) base salary continuation for nine months following termination, or the Mitchener Severance Amount, and, (ii) if Dr. Mitchener is enrolled in our health care program immediately prior to the date of termination and properly elects to receive COBRA benefits, nine months of COBRA premiums for himself and his eligible dependents at our normal rate of contribution for employees for coverage at the level in effect immediately prior to the date of termination (or a monthly cash payment in lieu thereof if we determine we cannot pay such amounts without potentially violating applicable law). Payment of the Mitchener Severance Amount shall immediately cease if Dr. Mitchener breaches the terms of the Restrictive Covenants Agreement between him and us. In lieu of the severance payments and benefits set forth above, in the event Dr. Mitchener's employment is by us without Cause or he resigns for Good Reason, in either case within 12 months following a Change in Control (as defined in the Mitchener Employment Agreement), he will be entitled to receive: (i) a lump sum cash amount equal to one times the sum of (A) his current base salary (or his base salary in effect prior to the Change in Control, if higher) plus (B) his target annual cash incentive compensation for the year of termination, (ii) if Dr. Mitchener is enrolled in our health care program immediately prior to the date of termination and properly elects to receive COBRA benefits, 12 months of COBRA premiums for himself and his eligible dependents at our normal rate of contribution for employees for coverage at the level in effect immediately prior to the date of termination (or a monthly cash payment in lieu thereof if we determine we cannot pay such amounts without potentially violating applicable law), and (iii) notwithstanding anything to the contrary provided in the applicable award agreement, accelerated vesting of 100% of all Time-Based Equity Awards held by Dr. Mitchener.

Robert Connelly

In connection with Mr. Connelly's employment relationship ending on May 31, 2018, or the Separation Date, we entered into a Separation Agreement with Mr. Connelly dated May 7, 2018. Under this Separation Agreement, subject to Mr. Connelly's compliance with his obligations under

the agreement, including a general release of any potential claims against the Company, Mr. Connelly received severance benefits that included COBRA coverage and base salary payments for nine months and an extension of the applicable exercise period for 27,766 options that were vested as of the Separation Date through the earlier of (i) a date that is six months after the closing an initial public offering by us or (ii) December 31, 2019. All unvested options were forfeited and void as of the Separation Date. In addition, we entered into a Stock Repurchase Agreement with Mr. Connelly dated May 31, 2019 in which we repurchased 91,527 shares of the Company owned by Mr. Connelly at a cost of $6.21 per share, resulting in an aggregate repurchase price of $568,162, which amount was applied to settle a balance of $568,159, including principal and accrued interest, owed by Mr. Connelly under a loan granted by us under a Promissory Note dated September 5, 2013.

Other agreements

We have also entered into employee confidentiality, inventions, non-solicitation and non-competition agreements with each of our named executive officers. Under such agreements, each named executive officer has agreed (1) not to compete with us during his or her employment and for a period of one year after the termination of such employment, (2) not to solicit our employees during his or her employment and for a period of one year after the termination of such employment, (3) to protect our confidential and proprietary information and (4) to assign to us related intellectual property developed during the course of his or her employment.

Outstanding equity awards at 2018 fiscal year-end

The following table sets forth information concerning outstanding equity awards held by each of our named executive officers as of December 31, 2018. All equity awards set forth in the table below were granted under our 2010 Stock Incentive Plan, as amended, or the 2010 Plan.

	Option Awards					Stock Awards	
Name	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Equity Incentive Plan Awards: Number of Securities Underlying Unexercised Unearned Options (#)	Option Exercise Price ($)	Option Expiration Date	Number of Shares or Units of Stock That Have Not Vested (#)	Market Value of Shares or Units of Stock That Have Not Vested ($)
William Hinshaw, President and CEO	—	939,028[1]	—	6.21	6/21/2028	—	—
Thomas Leggett, CFO	84,817[2]	84,817[2]	—	6.52	6/13/2027	—	—
	7,204[2]	7,204[2]	—	6.52	8/9/2027	—	—
			51,033[3]	6.21	8/28/2028	—	—
		51,033[4]		6.21	8/28/2028	—	—
Stephen Mitchener, PharmD, Chief Business Officer	—	124,011[5]		6.21	10/1/2028	—	—
			51,628[6]	6.21	10/1/2028	—	—
Robert Connelly, President and CEO	27,766		—	6.52	6/13/2027	—	—

(1) The shares underlying these awards vest 25% on May 31, 2019 and then in 12 equal quarterly installments thereafter.

(2) The shares underlying these awards vested 25% on January 3, 2018 and then in 12 equal quarterly installments thereafter.

(3) These awards may be earned upon the closing of the initial public offering of our common stock under an effective registration statement Filed on Form S-1 with the Securities Exchange Commission based on a pre-money valuation in excess of $500,000,000 (the "Vesting Commencement Date"). The shares underlying these awards vest 25% one year after the Vesting Commencement Date and then in 12 equal quarterly installments thereafter.

(4) The shares underlying these awards vest 25% on November 30, 2019 and then in 12 equal quarterly installments thereafter.

(5) The shares underlying these awards vest 25% on August 13, 2019 and then in 12 equal quarterly installments thereafter.

(6) These awards may be earned upon the Company entering into a partnership agreement with a third party to develop one or more therapeutic products that results in at least $10,000,000 in upfront cash to the Company, only if such event occurs prior to August 13, 2019 or, at the discretion of the Company's Compensation Committee, prior to August 13, 2020 (the "Vesting Commencement Date"). The shares underlying these awards vest 25% one year after the Vesting Commencement Date and then in 12 equal quarterly installments thereafter.

Employee benefit and equity compensation plans

2019 Stock Option and Incentive Plan

Our 2019 Stock Option and Incentive Plan, or 2019 Plan, was adopted by our board of directors on April 29, 2019, and approved by our stockholders on April 29, 2019, and became effective on the date immediately prior to the date on which the registration statement of which this prospectus is part is declared effective by the SEC. The 2019 Plan replaced our 2010 Plan, as our board of directors has determined not to make additional awards under that plan following the consummation of our initial public offering. The 2019 Plan allows the board of directors' compensation committee to make equity-based incentive awards to our officers, employees, directors and other key persons (including consultants).

We have initially reserved 905,000 shares of our common stock for the issuance of awards under the 2019 Plan, the Initial Limit. The 2019 Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2020, by 4% of the outstanding number of shares of our common stock on the immediately preceding December 31 or such lesser number of shares as determined by our compensation committee, or the Annual Increase. These limits are subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares of common stock underlying any awards that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without the issuance of stock, expire or are otherwise terminated (other than by exercise) under the 2019 Plan and 2010 Plan will be added back to the shares of common stock available for issuance under the 2019 Plan.

The maximum aggregate number of shares that may be issued in the form of incentive stock options shall not exceed the Initial Limit cumulatively increased on January 1, 2020 and on each January 1 thereafter by the lesser of the Annual Increase for such year or 905,000 shares.

The 2019 Plan will be administered by our compensation committee. Our compensation committee has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, to make any combination of awards to participants, and to determine the specific terms and conditions of each award, subject to the provisions of the 2019 Plan. Persons eligible to participate in the 2019 Plan will be those full or part-time officers, employees, non-employee directors and other key persons (including consultants) as selected from time to time by our compensation committee in its discretion.

The 2019 Plan permits the granting of both options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Code and options that do not so qualify. The option exercise price of each option will be determined by our compensation committee but may not be less than 100% of the fair market value of our common stock on the date of grant. The term of each option will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each option may be exercised.

Our compensation committee may award stock appreciation rights subject to such conditions and restrictions as it may determine. Stock appreciation rights entitle the recipient to shares of common stock, or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price may not be less than 100% of the fair market value of our common stock on the date of grant. The term of each stock appreciation right will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each stock appreciation right may be exercised.

Our compensation committee may award restricted shares of common stock and restricted stock units to participants subject to such conditions and restrictions as it may determine. These conditions and restrictions may include the achievement of certain performance goals and/or continued employment with us through a specified vesting period. Our compensation committee may also grant shares of common stock that are free from any restrictions under the 2019 Plan. Unrestricted stock may be granted to participants in recognition of past services or for other valid consideration and may be issued in lieu of cash compensation due to such participant. Our compensation committee may grant dividend equivalent rights to participants that entitle the recipient to receive credits for dividends that would be paid if the recipient had held a specified number of shares of common stock.

Our compensation committee may grant cash bonuses under the 2019 Plan to participants, subject to the achievement of certain performance goals.

The 2019 Plan provides that upon the effectiveness of a "sale event," as defined in the 2019 Plan, an acquirer or successor entity may assume, continue or substitute outstanding awards under the 2019 Plan. To the extent that awards granted under the 2019 Plan are not assumed or continued or substituted by the successor entity, upon the effective time of the sale event, such awards under the 2019 Plan shall terminate. In such a case, all options and stock appreciation rights that are not exercisable immediately prior to the effective time of the sale event will become fully exercisable as of the effective time of the sale event, all other awards with time-based vesting, conditions or restrictions, will become fully vested and nonforfeitable as of the effective time of the sale event and all awards with conditions and restrictions relating to the attainment of performance goals may become vested and nonforfeitable in the discretion of our compensation committee. In the event of such termination, individuals holding options and stock appreciation rights will be permitted to exercise such options and stock appreciation rights (to the extent exercisable) within a specified period of time prior to the sale event. In addition, in connection with the termination of the 2019 Plan upon a sale event, we may make or provide for a cash payment to participants holding vested and exercisable options and stock appreciation rights equal to the difference between the per share cash consideration payable to stockholders in the sale event and the exercise price of the options or stock appreciation rights and we may make or provide for a cash payment to participants holding other vested awards.

Our board of directors may amend or discontinue the 2019 Plan and our compensation committee may amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose, but no such action may adversely affect rights under an award without the holder's consent. Certain amendments to the 2019 Plan require the approval of our stockholders.

No awards may be granted under the 2019 Plan after the date that is ten years from the date of stockholder approval of the 2019 Plan. No awards under the 2019 Plan have been made prior to the date hereof.

2019 Employee Stock Purchase Plan

On April 29, 2019, our board of directors adopted the 2019 Employee Stock Purchase Plan, or 2019 ESPP, and on April 29, 2019, our stockholders approved the 2019 ESPP. The 2019 ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code. The 2019 ESPP initially reserves and authorizes the issuance of up to a total of 237,181 shares of common stock to participating employees. The ESPP provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2020, by the lesser of (i) 237,181 shares of common stock, (ii) 1% of the outstanding number of shares of our common stock on the immediately preceding December 31 or (iii) such lesser number of shares as determined by the 2019 ESPP administrator. The number of shares reserved under the 2019 ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

All employees whose customary employment is for more than 20 hours per week are eligible to participate in the ESPP. However, any participating employee who would own 5% or more of the total combined voting power or value of all classes of stock after an option were granted under the ESPP would not be eligible to purchase shares under the 2019 ESPP.

We will make one or more offerings each year to our employees to purchase shares under the ESPP. Offerings will usually begin on each January 1 and July 1 and will continue for six-month periods, referred to as offering periods. Each eligible employee may elect to participate in any offering by submitting an enrollment form at least 15 business days before the relevant offering date.

Each employee who is a participant in the 2019 ESPP may purchase shares by authorizing payroll deductions of up to 15% of his or her base compensation during an offering period. Unless the participating employee has previously withdrawn from the offering, his or her accumulated payroll deductions will be used to purchase shares on the last business day of the offering period at a price equal to 85% of the fair market value of the shares on the first business day or the last business day of the offering period, whichever is lower. Under applicable tax rules, an employee may purchase no more than $25,000 worth of shares of common stock, valued at the start of the purchase period, under the ESPP in any calendar year.

The accumulated payroll deductions of any employee who is not a participant on the last day of an offering period will be refunded. An employee's rights under the 2019 ESPP terminate upon voluntary withdrawal from the plan or when the employee ceases employment with us for any reason.

The 2019 ESPP may be terminated or amended by our board of directors at any time. An amendment that increases the number of shares of common stock authorized under the ESPP and certain other amendments require the approval of our stockholders.

Senior Executive Cash Incentive Bonus Plan

On April 29, 2019, our board of directors adopted the Senior Executive Cash Incentive Bonus Plan, or the Bonus Plan. The Bonus Plan provides for cash bonus payments based upon the attainment of performance targets established by our compensation committee. The payment targets will be related to financial and operational measures or objectives with respect to our company, or Corporate Performance Goals, as well as individual performance objectives.

Our compensation committee may select Corporate Performance Goals from among the following: cash flow (including, but not limited to, operating cash flow and free cash flow); sales or revenue; corporate revenue; earnings before interest, taxes, depreciation and amortization; net income (loss) (either before or after interest, taxes, depreciation and/or amortization); changes in

the market price of our common stock; economic value-added; development, clinical, regulatory or commercial milestones; acquisitions or strategic transactions, partnerships or joint ventures; operating income (loss); return on capital, assets, equity, or investment; stockholder returns; return on sales; gross or net profit levels; productivity; expense efficiency; margins; operating efficiency; customer satisfaction; working capital; earnings (loss) per share of our common stock; sales or market shares; number of customers; operating income and/or other strategic, financial or operational objectives, any of which may be measured in absolute terms, as compared to any incremental increase, in terms of growth, as compared to results of a peer group, against the market as a whole, compared to applicable market indices and/or measured on a pre-tax or post-tax basis.

Each executive officer who is selected to participate in the Bonus Plan will have a target bonus opportunity set for each performance period. The bonus formulas will be adopted in each performance period by the compensation committee and communicated to each executive. The Corporate Performance Goals will be measured at the end of each performance period after our financial reports have been published or such other appropriate time as the compensation committee determines. If the Corporate Performance Goals and individual performance objectives are met, payments will be made as soon as practicable following the end of each performance period. The Bonus Plan also permits the compensation committee to approve additional bonuses to executive officers in its sole discretion.

2010 Stock Incentive Plan

Our 2010 Plan was approved and adopted by our board of directors on December 23, 2010, and approved by our stockholders on December 23, 2010. Under the 2010 Plan, we initially reserved for issuance an aggregate of 135,722 shares of our common stock, and most recently increased the shares reserved and available for issuance to 6,019,414 shares of our common stock on March 22, 2019. The number of shares of common stock reserved for issuance under the 2010 Plan, as amended and restated through the date hereof, is subject to adjustment in the event of a stock split, reverse stock split, stock dividend, reorganization, recapitalization, combination of shares, reclassification of shares, spin-off, or other similar change in our capitalization.

The shares of common stock underlying awards that are forfeited, cancelled, reacquired prior to vesting, satisfied without the issuance of shares of common stock, withheld to cover the exercise price or tax withholding or otherwise terminated (other than by exercise) are added back to the shares of common stock available for issuance under the 2010 Plan.

Our board of directors has acted as administrator of the 2010 Plan. The administrator has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, and to determine the specific terms and conditions of each award, subject to the provisions of the 2010 Plan. Persons eligible to participate in the 2010 Plan are those employees, full or part-time officers and directors, as well as consultants and key persons to our company as selected from time to time by the administrator in its discretion.

The 2010 Plan permits the granting of (1) options, including incentive stock options, to purchase common stock, (2) restricted stock, (3) unrestricted stock and (4) restricted stock units. For stock options, the administrator will determine the per share option exercise price and at what time or times each option may be exercised.

The 2010 Plan provides that in the case of and subject to the consummation of (i) the dissolution or liquidation of our company, (ii) the sale of all or substantially all of the assets of our company on a consolidated basis to an unrelated person or entity, (iii) a merger, reorganization or consolidation involving our company in which the shares of voting stock of our company outstanding immediately prior to such transaction represent or are converted into or exchanged for

securities of the surviving or resulting entity immediately upon completion of such transaction which represent less than 50 percent of the outstanding voting power of such surviving or resulting entity, (iv) the acquisition of all or a majority of the outstanding voting stock of our company in a single transaction or a series of related transactions, or (v) any other acquisition of the business of our company, as determined by our board of directors, or taken together, a Sale Event, an acquirer or successor entity may assume, continue or substitute outstanding awards under the 2010 Plan. To the extent that awards granted under the 2010 Plan are not assumed, continued or substituted by the successor entity, upon the effective time of the Sale Event, the 2010 Plan and such awards under the 2010 Plan shall terminate or be forfeited. In the event of such termination, individuals holding options will be permitted to exercise such options (to the extent exercisable) within a specified period of time prior to the Sale Event. In addition, in connection with the termination of the 2010 Plan upon a Sale Event, we may make or provide for a cash payment to participants holding vested and exercisable options equal to the difference between the per share cash consideration payable to stockholders in the Sale Event and the exercise price of the options. In the event of the forfeiture of shares of restricted stock, such shares shall be repurchased from the holder thereof at a per share price equal to the lower of the original per share purchase price or the current fair market value of such shares immediately prior to the effective time of the Sale Event. In addition in connection with the termination of the 2010 Plan upon a Sale Event, we may make or provide for a cash payment to participants holding restricted stock or restricted stock units equal to the per share cash consideration payable to stockholders in the Sale Event.

Our board of directors may amend or discontinue the 2010 Plan at any time, subject to approval by the Company stockholders entitled to vote at a meeting of stockholders where such approval is required by applicable law. Our board of directors may also amend or cancel any outstanding award, provided that no amendment to an award may materially and adversely affect a participant's rights without his or her consent.

The 2010 Plan will terminate upon the closing of this offering; however, awards previously granted may extend beyond that date. As of December 31, 2018, 845,450 shares of common stock and options to purchase 4,039,464 shares of common stock were outstanding under the 2010 Plan. Our board of directors has determined not to make any further awards under the 2010 Plan following the completion of this offering.

401(k) plan

We maintain a 401(k) retirement savings plan for the benefit of our employees, including our named executive officers, who satisfy certain eligibility requirements. Under the 401(k) plan, eligible employees may elect to defer a portion of their compensation, within the limits prescribed by the Code, on a pre-tax or after-tax (Roth) basis through contributions to the 401(k) plan. The Company is permitted to make discretionary profit sharing contributions to the Plan. The 401(k) plan is intended to qualify under Section 401(a) of the Code. As a tax-qualified retirement plan, pre-tax contributions to the 401(k) plan and earnings on those pre-tax contributions are not taxable to the employees until distributed from the 401(k) plan, and earnings on Roth contributions are not taxable when distributed from the 401(k) plan.

Health and welfare plans

All of our full-time employees, including our named executive officers, are eligible to participate in our health and welfare plans, including medical and dental benefits, short-term and long-term disability insurance, and life insurance. We believe these perquisites are necessary and appropriate to provide a competitive compensation package to our named executive officers.

Limitations on liability and indemnification matters

Our restated certificate of incorporation contains provisions that limit the liability of our directors for monetary damages to the fullest extent permitted by the Delaware General Corporation Law, or DGCL. Consequently, our directors are not personally liable to us or our stockholders for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the DGCL; or
- any transaction from which the director derived an improper personal benefit.

Our amended and restated bylaws require us to indemnify our directors and officers to the maximum extent not prohibited by the DGCL and allow us to indemnify other employees and agents as set forth in the DGCL. Subject to certain limitations, our amended and restated bylaws also require us to advance expenses incurred by our directors and officers for the defense of any action for which indemnification is required or permitted.

We have entered, and intend to continue to enter, into separate indemnification agreements with our directors, officers and certain of our key employees, in addition to the indemnification provided for in our restated certificate of incorporation and amended and restated bylaws. These agreements, among other things, require us to indemnify our directors, officers and key employees for certain expenses, including attorneys' fees, judgments, penalties, fines and settlement amounts actually incurred by these individuals in any action or proceeding arising out of their service to us or any of our subsidiaries or any other company or enterprise to which these individuals provide services at our request. Subject to certain limitations, our indemnification agreements also require us to advance expenses incurred by our directors, officers and key employees for the defense of any action for which indemnification is required or permitted

We believe that provisions of our restated certificate of incorporation, amended and restated bylaws and indemnification agreements are necessary to attract and retain qualified directors, officers and key employees. We also maintain directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against our directors and officers for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions.

At present, there is no pending litigation or proceeding involving any of our directors or executive officers as to which indemnification is required or permitted, and we are not aware of any threatened litigation or proceeding that may result in a claim for indemnification.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, executive officers or persons controlling us, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

DIRECTOR COMPENSATION

The following table presents the total compensation for each person who served as a non-employee member of our board of directors and received compensation for such service during the year ended December 31, 2018. Other than as set forth in the table and described more fully below, we did not pay any compensation, make any additional equity awards or non-equity awards to, or pay any other compensation to any of the non-employee members of our board of directors in 2018. We reimburse non-employee members of our board of directors for reasonable travel and out-of-pocket expenses incurred in attending meetings of our board of directors and committees of our board of directors.

We also do not, and do not expect to, provide separate compensation to our directors who are also our employees, such as Mr. Hinshaw, our Chief Executive Officer and President.

Name	Fees Earned or Paid in Cash ($)	Option Awards ($)[1]	Total ($)
William D. Baird III[2]	—	196,460	196,460
Grégory Behar[3]	—	—	—
David A. Berry, M.D., Ph.D.[4]	—	—	—
David R. Epstein[5]	225,000	—	225,000
Stephen Hoge, M.D.[6]	—	—	—
Gary Pisano, Ph.D.[7]	—	—	—
Cristina M. Rondinone, Ph.D.[8]	—	177,650	177,650
Christopher A. Viehbacher[9]	—	—	—

(1) The amounts reported represent the aggregate grant date fair value of the stock options awarded to the non-employee directors in the fiscal year ended December 31, 2018, calculated in accordance with FASB ASC Topic 718. Such grant date fair values do not take into account any estimated forfeitures. The assumptions used in calculating the grant date fair value of the stock options reported in this column are set forth in Note 7 to our Consolidated Financial Statements included at the end of this prospectus. The amounts reported in this column reflect the accounting cost for these stock options and do not correspond to the actual economic value actually received by the non-employee directors or that may be received by the non-employee directors upon the exercise of the stock options or any sale of the underlying shares of common stock.

(2) As of December 31, 2018, Mr. Baird held options to purchase 51,031 shares of our common stock. Such award vests 25% on the first anniversary of June 22, 2018 and then in 12 equal quarterly installments thereafter, subject to continued service — 0 shares subject to each such option were vested as of December 31, 2018.

(3) As of December 31, 2018, Mr. Behar did not hold any outstanding equity awards.

(4) As of December 31, 2018, Dr. Berry held 760,042 shares of stock.

(5) As of December 31, 2018, Mr. Epstein held options to purchase 782,646 shares of our common stock. Such award vests 25% on the first anniversary of December 21, 2017 and then in 12 equal quarterly installments thereafter, subject to continued service — 195,661 shares subject to each such option were vested as of December 31, 2018. As of December 31, 2018, $225,000 was accrued related to a $450,000 retainer payment to Mr. Epstein for service rendered in 2018 and 2019 as Chairman of the board of directors.

(6) As of December 31, 2018, Dr. Hoge held options to purchase 54,288 shares of our common stock, all of which were vested as of December 31, 2018.

(7) As of December 31, 2018, Dr. Pisano held options to purchase 81,433 shares of our common stock, all of which were vested as of December 31, 2018.

(8) As of December 31, 2018, Dr. Rondinone held options to purchase 46,144 shares of our common stock, 40,716 of which were awarded to Dr. Rondinone as compensation for her role on our board of directors and 5,428 for her role on our Scientific Advisory Board. Such awards vest 25% on the first anniversary of June 22, 2018 and July 13, 2018, respectively, and then in 12 equal quarterly installments thereafter, subject to continued service — 0 shares subject to each such option were vested as of December 31, 2018.

(9) As of December 31, 2018, Mr. Viehbacher did not hold any outstanding equity awards.

Non-employee director compensation policy

Prior to the effectiveness of the registration statement of which this prospectus forms a part, we did not have a formal policy to compensate our non-employee directors. Prior to the effectiveness of the registration statement of which this prospectus forms a part, our board of directors intends to adopt a non-employee director compensation policy, to be effective upon effectiveness of the registration statement of which this prospectus forms a part, that is designed to enable us to attract and retain, on a long-term basis, highly qualified non-employee directors. Under the policy, each director who is not an employee will be paid cash compensation from and after the completion of this offering, as set forth below:

	Member Annual Fee	Chairman Additional Annual Fee
Board of Directors	$ 35,000	$ —
Audit Committee	7,500	15,000
Compensation Committee	5,000	10,000
Nominating and Corporate Governance Committee	4,000	8,000

In addition, each non-employee director serving on our board of directors upon the closing of this offering and each non-employee director elected or appointed to our board of directors following the completion of this offering will be granted a one-time equity award of 22,000 shares on the date of such director's election or appointment to the board of directors, which will vest annually over three years, subject to continued service through such vesting dates. On the date of each annual meeting of stockholders of our company, each non-employee director will be granted an annual equity award of 11,000 shares, which will vest in full on the earlier to occur of the first anniversary of the date of grant or the next annual meeting, subject to continued service as a director through such vesting date.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

Other than the compensation agreements and other arrangements described under "Executive Compensation" and "Director Compensation" in this prospectus and the transactions described below, since January 1, 2016, there has not been and there is not currently proposed, any transaction or series of similar transactions to which we were, or will be, a party in which the amount involved exceeded, or will exceed, $120,000 and in which any director, executive officer, holder of 5% or more of any class of our capital stock or any member of the immediate family of, or entities affiliated with, any of the foregoing persons, had, or will have, a direct or indirect material interest.

Stockholder	Common Stock	Shares of Series A Preferred Stock	Shares of Series B Preferred Stock	Shares of Series B-1 Preferred Stock	Shares of Series C Preferred Stock	Shares of Series D Preferred Stock	Shares of Series E Preferred Stock	Total Purchase Price ($)
Flagship Pioneering Funds[1]	2,714,440	5,538,462	2,320,184		992,063		1,342,882	41,800,000
Fidelity Investments[2]					3,799,602		895,255	48,300,000
Nestlé Health Sciences US Holdings, Inc.						2,997,179	581,915	49,000,000
Gurnet Point L.P.					1,488,095		895,255	25,000,000

(1) Flagship Pioneering Funds consists of Flagship VentureLabs IV, LLC, Flagship Ventures Fund 2007, L.P., Flagship Ventures Fund IV, L.P., Flagship Ventures Fund IV-Rx, L.P. and Flagship Ventures Opportunity Fund I, L.P.

(2) Fidelity Investments consists of Fidelity Select Portfolios: Biotechnology Portfolio, Fidelity Advisor Series VII: Fidelity Advisor Biotechnology Fund, Fidelity Securities Fund: Fidelity Series Blue Chip Growth Fund, Fidelity Growth Company Commingled Pool, Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund and FIAM Target Date Blue Chip Growth Commingled Pool.

Sales of securities

Series C preferred stock financing

From January 2015 to August 2017, we issued and sold to investors in a private placement an aggregate of 6,969,044 shares of our Series C Preferred Stock at a purchase price of $10.08 per share, for aggregate consideration of approximately $70.3 million.

Series D preferred stock financing

In February 2016, we issued and sold to investors in a private placement an aggregate of 2,997,179 shares of our Series D Preferred Stock at a purchase price of $14.18 per share, for aggregate consideration of approximately $42.5 million.

Series E preferred stock financing

In November 2018, we issued and sold to investors in a private placement an aggregate of 5,282,002 shares of our Series E Preferred Stock at a purchase price of $11.17 per share, for aggregate consideration of approximately $59.0 million.

Services agreement

In December 2008, we entered into a services agreement with Flagship Ventures Management, Inc., now known as Flagship Pioneering, Inc., an affiliate of the Flagship Pioneering Funds, under which Flagship Pioneering provides us with advisory and administrative services on an as-needed basis. The agreement, which is invoiced monthly, may be terminated by either party upon 30 days' prior written notice. For the years ended December 31, 2017 and 2018, we paid Flagship Pioneering, Inc. an aggregate of approximately $104,000 and $8,000, respectively, for services provided under the services agreement, inclusive of the services provided under the services agreement.

Fifth amended and restated investors' rights agreement

In connection with the initial closing of our Series E Preferred Stock financing on November 30, 2018, we entered into a fifth amended and restated investors' rights agreement, or investors' rights agreement, with certain of our stockholders, including affiliates of Flagship Pioneering Funds, or Flagship. The investors' rights agreement, among other things:

- grants investors party thereto certain registration rights with respect to shares of our common stock, including shares of common stock issued or issuable upon conversion of our convertible preferred stock;
- obligates us to deliver periodic financial statements to Flagship, Fidelity Investments, Gurnet Point L.P. and its affiliates and any investor who, individually or together with affiliates, holds at least 350,000 shares of our preferred stock, each of whom we refer to as a "Major Investor"; and
- grants a right of first offer with respect to sales of our shares by us, subject to specified exclusions (which exclusions include the sale of the shares in connection with this offering), to Major Investors.

For more information regarding the registration rights provided in this agreement, please refer to the section of this prospectus titled "Description of Capital Stock — Registration rights."

Certain provisions of this agreement, including certain of the covenants described above, will terminate automatically prior to the completion of this offering. This is not a complete description of the investors' rights agreement and is qualified by the full text of the investors' rights agreement filed as an exhibit to the registration statement of which this prospectus is a part.

Fifth amended and restated voting agreement

In connection with the initial closing of our Series E Preferred Stock financing on November 30, 2018, we entered into a fifth amended and restated voting agreement, or the voting agreement, with certain of our stockholders, including affiliates of Flagship. The voting agreement, among other things, provides the terms for the voting of shares with respect to the constituency of our directors. Pursuant to the terms of the voting agreement, the following directors were elected to serve as members on our board of directors and, as of the date of this prospectus, continue to so serve: David R. Epstein, William Hinshaw, Gary Pisano, Cristina M. Rondinone, William D. Baird III, Stephen Hoge, David A. Berry, Christopher A. Viehbacher, and Grégory Behar. Mr. Epstein was selected to serve on our board of directors as designated by Flagship Ventures Fund IV, L.P., Mr. Hinshaw was selected to serve on our board of directors as our CEO, Dr. Berry was selected to serve on our board of directors as designated by Flagship, Mr. Behar was selected to serve on our board of directors by Nestlé Health Sciences US Holdings, Inc. and Dr. Pisano, Dr. Rondinone, Mr. Baird and Dr. Hoge were selected to serve on our board of directors as directors who are not affiliated with any investor, possess relevant industry experience and are acceptable to a majority of the investors party to the voting agreement. Mr. Viehbacher was selected to serve on our board of directors as a director who is acceptable to a majority of the other members of our board of directors.

This voting agreement will terminate automatically upon the closing of this offering.

Fifth amended and restated right of first refusal and co-sale agreement

In connection with initial closing of our Series E Preferred Stock financing on November 30, 2018, we entered into a right of first refusal and co-sale agreement with certain of our stockholders, including Flagship. The right of first refusal and co-sale agreement, among other things:

- grants our investors certain rights of first refusal and co-sale with respect to proposed transfers of our securities by certain stockholders; and
- grants us certain rights of first refusal with respect to proposed transfers of our securities by certain stockholders.

The right of first refusal and co-sale agreement will terminate automatically immediately prior to the completion of this offering.

Promissory notes

We entered into a stock repurchase agreement with Robert Connelly, our former CEO, dated May 31, 2018, in which we repurchased 91,527 shares of our common stock owned by Mr. Connelly at a cost of $6.21 per share, resulting in an aggregate repurchase price of $568,162, which amount was applied to settle a balance of $568,159, including principal and accrued interest, owed to us by Mr. Connelly under a Promissory Note dated September 5, 2013.

Indemnification agreements

In connection with this offering, we intend to enter into new agreements to indemnify our directors and executive officers. These agreements will, among other things, require us to indemnify these individuals for certain expenses (including attorneys' fees), judgments, fines and settlement amounts reasonably incurred by such person in any action or proceeding, including any action by or in our right, on account of any services undertaken by such person on behalf of our company or that person's status as a member of our board of directors to the maximum extent allowed under Delaware law.

Participation in this Offering

Certain of our existing stockholders, including entities affiliated with certain of our directors, have indicated an interest in purchasing an aggregate of approximately $39.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any or all of these stockholders, or any or all of these stockholders may determine to purchase more, fewer or no shares in this offering. The underwriters will receive the same underwriting discount on any shares purchased by these stockholders as they will on any other shares sold to the public in this offering.

Policies for approval of related party transactions

Our board of directors reviews and approves transactions with directors, officers and holders of five percent or more of our voting securities and their affiliates, each a related party. Prior to this offering, upon consideration of a potential related party transaction, the material facts as to the related party's relationship or interest in the transaction are disclosed to our board of directors prior to their consideration of such transaction, and the transaction is not considered approved by our board of directors unless a majority of the directors who are not interested in the transaction approve the transaction. Further, when stockholders are entitled to vote on a transaction with a related party, the material facts of the related party's relationship or interest in the transaction are disclosed to the stockholders, who must approve the transaction in good faith.

We have adopted a written related party transactions policy that will become effective on the date on which the registration statement of which this prospectus is part is declared effective by the SEC. Pursuant to this policy, the audit committee has the primary responsibility for reviewing and approving or disapproving "related party transactions," which are transactions between us and related persons in which the aggregate amount involved exceeds or may be expected to exceed $120,000 and in which a related person has or will have a direct or indirect material interest. For purposes of this policy, a related person will be defined as a director, executive officer, nominee for director, or greater than 5% beneficial owner of our common stock, in each case since the beginning of the most recently completed year, and their immediate family members.

PRINCIPAL STOCKHOLDERS

The following table sets forth certain information known to us regarding beneficial ownership of our capital stock as of December 31, 2018, as adjusted to reflect the sale of common stock offered by us in this offering, for:

- each person or group of affiliated persons known by us to be the beneficial owner of more than 5% of our capital stock;
- each of our named executive officers;
- each of our directors; and
- all of our executive officers and directors as a group.

Beneficial ownership is determined in accordance with the rules of the SEC and generally includes voting or investment power with respect to securities. Under those rules, beneficial ownership includes any shares as to which the individual or entity has sole or shared voting power or investment power and includes securities that the individual or entity has the right to acquire, such as through the exercise of stock options, within 60 days of December 31, 2018. Except as noted by footnote, and subject to community property laws where applicable, we believe, based on the information provided to us, that the persons and entities named in the table below have sole voting and investment power with respect to all common stock shown as beneficially owned by them.

The percentage of beneficial ownership prior to this offering in the table below is based on 19,416,931 shares of common stock deemed to be outstanding as of December 31, 2018, assuming the conversion of all outstanding shares of our preferred stock upon the completion of this offering into an aggregate of 14,641,997 shares of common stock upon the completion of this offering, and the percentage of beneficial ownership at this offering in the table below is based on 22,988,359 shares of common stock assumed to be outstanding after the completion of the offering.

Certain of our existing stockholders, including entities affiliated with certain of our directors, have indicated an interest in purchasing an aggregate of approximately $39.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, fewer or no shares in this offering to any or all of these stockholders, or any or all of these stockholders may determine to purchase more, fewer or no shares in this offering. The underwriters will receive the same underwriting discount on any shares purchased by these stockholders as they will on any other shares sold to the public in this offering. The following table does not reflect any such potential purchases by these existing stockholders or their affiliated entities. If any shares are purchased by these stockholders, the number of shares of common stock

beneficially owned after this offering and the percentage of common stock beneficially owned after this offering would increase from that set forth in the table below.

Name and address of beneficial owner	Number of shares beneficially owned prior to offering	Percentage of shares beneficially owned	
		Prior to offering	After offering
5% Stockholders:			
Flagship Pioneering Funds (1)	8,248,414	42.5%	35.9%
Fidelity Investments (2)	2,548,778	13.1%	11.1%
Nestlé Health Sciences US Holdings, Inc. (3)	2,018,699	10.4%	8.8%
Gurnet Point L.P. (4)	1,293,891	6.7%	5.6%
Named executive officers and directors:			
Thomas Leggett (5)	92,021	*	*
Stephen Mitchener	—	*	*
Robert Connelly (6)	563,086	2.9%	2.4%
William D. Baird III	—	*	*
Grégory Behar, M.B.A.	—	*	*
David A. Berry, M.D., Ph.D.	760,042	3.9%	3.3%
David R. Epstein (7)	195,661	1.0%	0.8%
William Hinshaw (8)	—	*	*
Stephen Hoge, M.D. (9)	54,288	*	*
Gary Pisano, Ph.D. (10)	81,433	*	*
Cristina M. Rondinone, Ph.D.	—	*	*
Christopher A. Viehbacher	—	*	*
All executive officers and directors as a group (12 persons)	1,746,531	8.8%	7.5%

* Represents beneficial ownership of less than 1%.

(1) Consists of (a) 678,610 shares of common stock held by Flagship Ventures Fund 2007, L.P. ("Flagship Fund 2007"), (b) 2,035,830 shares of common stock held by Flagship Ventures Fund IV, L.P. ("Flagship Fund IV"), (c) 751,691 shares of common stock issuable upon conversion of the Series A Preferred Stock held by Flagship Fund 2007, (d) 209,933 shares of common stock issuable upon conversion of the Series B/B1 Preferred Stock held by Flagship Fund 2007, (e) 120,795 shares of common stock issuable upon conversion of the Series C Preferred Stock held by Flagship Fund 2007, (f) 2,255,074 shares of common stock issuable upon conversion of the Series A Preferred Stock held by Flagship Fund IV, (g) 503,840 shares of common stock issuable upon conversion of the Series B/B1 Preferred Stock held by Flagship Fund IV, (h) 335,457 shares of common stock issuable upon conversion of the Series C Preferred Stock held by Flagship Fund IV, (i) 194,409 shares of common stock issuable upon conversion of the Series E Preferred Stock held by Flagship Fund IV (j) 545,826 shares of common stock issuable upon conversion of the Series B/B1 Preferred Stock held by Flagship Ventures Fund IV-Rx, L.P. ("Flagship Fund IV-Rx"), (k) 82,324 shares of common stock issuable upon conversion of the Series C Preferred Stock held by Flagship Fund IV-Rx, and (l) 48,602 shares of common stock issuable upon conversion of the Series E Preferred Stock held by Flagship Fund IV-Rx; and (m) 486,023 shares of common stock issuable upon conversion of the Series E Preferred Stock held by Flagship Ventures Opportunities Fund I, L.P. The General Partner of Flagship Fund 2007 is Flagship Ventures 2007 General Partner LLC ("Fund 2007 GP"). Flagship Fund IV is a member of VentureLabs IV and also serves as its manager. The General Partner of Flagship Fund IV is Flagship Ventures Fund IV General Partner LLC ("Fund IV GP"). Noubar B. Afeyan, Ph.D. and Edwin M. Kania, Jr. are the managers of Fund IV GP and Fund 2007 GP and each of these individuals may be deemed to share voting and investment power with respect to all shares held by Flagship Fund IV, VentureLabs IV, and Flagship Fund 2007. While Mr. Kania is retired from Flagship Pioneering, he continues to serve as a manager of the Fund 2007 GP and Fund IV GP. None of the Flagship General Partners, Dr. Afeyan, or Mr. Kania directly own any of the shares held by the Flagship Funds, and each of the Flagship General Partners, Dr. Afeyan, and Mr. Kania disclaims beneficial ownership of such shares except to the extent of its or his pecuniary

interest therein. The mailing address of the Flagship Funds is 55 Cambridge Parkway, Suite 800E, Cambridge, MA 02142.

(2) Consists of (a) 891,570 shares of common stock issuable upon conversion of the Series C Preferred Stock held by Fidelity Select Portfolios: Select Biotechnology Portfolio, (b) 185,590 shares of common stock issuable upon conversion of the Series C Preferred Stock held by Fidelity Advisor Series VII: Fidelity Advisor Biotechnology Fund, (c) 296,218 shares of common stock issuable upon conversion of the Series C Preferred Stock held by Fidelity Securities Fund: Fidelity Blue Chip Growth Fund, (d) 134,644 shares of common stock issuable upon conversion of the Series C Preferred Stock held by Fidelity Securities Fund: Fidelity Series Blue Chip Growth Fund, (e) 186,693 shares of common stock issuable upon conversion of the Series C Preferred Stock and 221,318 shares of common stock issuable upon conversion of the Series E Preferred Stock held by Fidelity Growth Company Comingled Pool, (f) 60,576 shares of common stock issuable upon conversion of the Series C Preferred Stock and 65,545 shares of common stock issuable upon conversion of the Series E Preferred Stock held by Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund, (g) 291,309 shares of common stock issuable upon conversion of the Series C Preferred Stock and 199,159 shares of common stock issuable upon conversion of the Series E Preferred Stock held by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund , and (h) 16,156 shares of common stock issuable upon conversion of the Series C Preferred Stock held by FIAM Target Date Blue Chip Growth Commingled Pool. Fidelity Select Portfolios, Fidelity Advisor Biotechnology Fund, Fidelity Mt. Vernon Street Trust, Fidelity Variable Insurance and Fidelity Advisor Series VII are managed by direct or indirect subsidiaries of FMR LLC. Edward C. Johnson 3d is a Director and the Chairman of FMR LLC and Abigail P. Johnson is a Director, the Vice Chairman and the President of FMR LLC. Members of the family of Edward C. Johnson 3d, including Abigail P. Johnson, are the predominant owners, directly or through trusts, of Series B voting common shares of FMR LLC, representing 49% of the voting power of FMR LLC. The Johnson family group and all other Series B shareholders have entered into a shareholders' voting agreement under which all Series B voting common shares will be voted in accordance with the majority vote of Series B voting common shares. Accordingly, through their ownership of voting common shares and the execution of the shareholders' voting agreement, members of the Johnson family may be deemed, under the Investment Company Act of 1940, to form a controlling group with respect to FMR LLC. Neither FMR LLC nor Edward C. Johnson 3d nor Abigail P. Johnson has the sole power to vote or direct the voting of the shares owned directly by the various investment companies registered under the Investment Company Act (Fidelity Funds) advised by Fidelity Management & Research Company (FMR Co), a wholly owned subsidiary of FMR LLC, which power resides with the Fidelity Funds' Boards of Trustees. Fidelity Management & Research Company carries out the voting of the shares under written guidelines established by the Fidelity Funds' Boards of Trustees.

(3) Consists of (a) 1,702,785 shares of common stock issuable upon conversion of the Series D Preferred Stock and (b) 315,914 shares of common stock issuable upon conversion of the Series E Preferred Stock.

(4) Consists of (a) 807,868 shares of common stock issuable upon conversion of the Series C Preferred Stock and (b) 486,023 shares of common stock issuable upon conversion of the Series E Preferred Stock.

(5) 92,021 shares of common stock underlying options exercisable within 60 days of December 31, 2018.

(6) Consists of (a) 535,320 shares held by Mr. Connelly and (b) 27,766 shares of common stock underlying options exercisable within 60 days of December 31, 2018.

(7) 195,661 shares of common stock underlying options exercisable within 60 days of December 31, 2018.

(8) Mr. Hinshaw currently serves as our President and Chief Executive Officer and commenced employment with us in June 2018. Previously, Mr. Connelly served as our President and Chief Executive Officer until May 2018.

(9) 54,288 shares of common stock underlying options exercisable within 60 days of December 31, 2018.

(10) 81,433 shares of common stock underlying options exercisable within 60 days of December 31, 2018.

DESCRIPTION OF CAPITAL STOCK

The following descriptions are summaries of the material terms of our restated certificate of incorporation, which will be effective upon the completion of this offering and amended and restated bylaws, which will be effective upon the effectiveness of the registration statement of which this prospectus is a part. The descriptions of the common stock and preferred stock give effect to changes to our capital structure that will occur immediately prior to the completion of this offering.

General

Upon the closing of this offering and after giving effect to the conversion of Series A, B, B-1, C, D and E Preferred Stock into common stock, the filing of an amended and restated certificate of incorporation solely for the purpose of eliminating any remaining designated, but unissued, shares of Series A Preferred Stock from our certificate of incorporation, and the retirement of all outstanding shares of our Series B, B-1, C, D and E preferred stock, our authorized capital stock will consist of 150,000,000 shares of common stock, par value $0.001 per share, and 10,000,000 shares of preferred stock, par value $0.001 per share, all of which shares of preferred stock will be undesignated.

In March 2019 and April 2019, respectively, our board of directors and our stockholders approved two amended and restated certificates of incorporation. The first amended and restated certificate of incorporation, or the First A&R Charter, contains all of the provisions that are set forth in the final restated certificate of incorporation, but also includes all of the terms of the Series A, B, B-1, C, D and E preferred stock. The First A&R Charter will be in effect immediately prior to the completion of this offering. The second amended and restated certificate of incorporation, or the Second A&R Charter, is identical to the First A&R Charter, except that it eliminates all provisions relating to the Series A preferred stock. The Second A&R Charter cannot, and will not, be filed until all outstanding shares of our Series A, B, B-1, C, D and E preferred stock have converted into common stock. Also in April 2019, our board of directors approved the retirement, following conversion of all shares of Series A, B, B-1, C, D and E preferred stock into common stock in connection with the completion of this offering, of all shares of Series B, B-1, C, D and E preferred stock following the filing of the Second A&R Charter. The certificate of retirement, or the Certificate of Retirement, will have the effect of eliminating from our certificate of incorporation all provisions relating to our Series B, B-1, C, D and E preferred stock. Finally, also in April 2019, our board of directors approved the restated certificate of incorporation, to be filed after the Certificate of Retirement eliminating the terms of our Series B, B-1, C, D and E preferred stock, which retirement occurs after the elimination of our Series A preferred stock pursuant to the filing of the Second A&R Charter, and which restated certificate of incorporation will govern the company following this offering until amended and/or restated in accordance with applicable law. The purpose of the filings described above is simply to allow us to eliminate the terms of our Series A, B, B-1, C, D and E preferred stock from our certificate of incorporation and to integrate and restate our certificate of incorporation into one operative document, namely the restated certificate of incorporation. The First A&R Charter will be filed and effective prior to the closing of this offering. The Second A&R Charter will be filed in manner that it will become effective on the date of the closing of this offering and after the effective time of the conversion our Series A, B, B-1, C, D and E preferred stock into common stock. The Certificate of Retirement will be filed in manner that it will become effective on the date of the closing of this offering and after the effective time of the Second A&R Charter. The restated certificate will be filed in a manner that it will become effective on the date of the closing and after the effective time of the Certificate of Retirement.

As of April 20, 2019, 4,785,088 shares of our common stock and 26,831,246 shares of preferred stock were outstanding and held by 64 stockholders of record. This amount does not take into account the conversion of all outstanding shares of our preferred stock into common stock upon the completion of this offering.

Common stock

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Preferred stock

Upon the closing of this offering, all outstanding shares of our Series A, B, B-1, C, D and E preferred stock will be converted into shares of our common stock, and all such shares of Series A, B, B-1, C, D and E preferred stock will be retired and will no longer be subject to reissuance. As described above, our board of directors and stockholders have approved a First A&R Charter to authorize the shares of common stock to be issued in this offering and in connection with the conversion of our shares of Series A, B, B-1, C, D and E preferred stock into common stock and to otherwise include the governance and other provisions which will govern the company immediately following this offering. The Second A&R Charter and the Certificate of Retirement have been authorized simply for purposes of allowing us to eliminate the terms of our existing Series A, B, B-1, C, D and E preferred stock. Finally, the restated certificate of incorporation simply reflects the elimination of the terms of our existing Series A, B, B-1, C, D and E preferred stock from our certificate of incorporation. Upon the consummation of this offering and after giving effect to the conversion and retirement of all Series A, B, B-1, C, D and E preferred stock, we will have no shares of Series A, B, B-1, C, D and E preferred stock designated for future issuance, and our board of directors will have the authority, without further action by our stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately upon the closing of this offering, no shares of preferred stock will be outstanding and we have no present plan to issue any shares of preferred stock.

Stock options

As of April 20, 2019, options to purchase 4,876,830 shares of our common stock were outstanding.

Warrants

As of April 20, 2019, warrants to purchase up to 112,795 shares of our Series A preferred stock, at an exercise price of $1.95 per share, were outstanding.

Upon the closing of this offering, and after giving effect to the conversion of our preferred stock into common stock, the warrants to purchase our Series A preferred stock will become exercisable for an aggregate of up to 61,235 shares of our common stock, at a weighted average exercise price of $3.59. All of the warrants provide for adjustments in the event of specified mergers, reorganization, reclassification, stock dividends, stock splits or other changes in our corporate structure. The warrants to purchase shares of our Series A preferred stock expire on the earlier of the close of business April 13, 2020 or one year after the closing of our initial public offering.

Registration rights

Upon the completion of this offering, the holders of 17,356,437 shares of our common stock including those issuable upon the conversion of our preferred stock, which shares we refer to as "registrable securities," will be entitled to rights with respect to the registration of these registrable securities under the Securities Act. These rights are provided under the terms of an investors' rights agreement between us and holders of our preferred stock. The investors' rights agreement includes demand registration rights, short-form registration rights and piggyback registration rights. All fees, costs and expenses of underwritten registrations under this agreement will be borne by us and all selling expenses, including underwriting discounts and selling commissions, will be borne by the holders of the shares being registered.

Demand registration rights

Beginning 180 days after the effective date of this registration statement, the holders of registrable securities are entitled to demand registration rights under certain conditions. Under the terms of the investors' rights agreement, we will be required, upon the request of holders of a majority of the registrable securities to register registrable securities that would result in an aggregate offering price of at least $5.0 million, to file a registration statement of all or a portion of these registrable securities for public resale. We are required to effect only two such registrations pursuant to this provision of the investors' rights agreement.

Short-form registration rights

Pursuant to the investors' rights agreement, if we are eligible to file a registration statement on Form S-3, upon the written request of holders of at least 20% of the registrable securities to register registrable securities that would result in an aggregate offering price of at least $2.5 million, we will be required to file a registration statement on Form S-3 for such registrable securities subject to certain limitations. We are required to effect only two registrations in any twelve month period pursuant to this provision of the investors' rights agreement. The right to have such shares registered on Form S-3 is further subject to other specified conditions and limitations.

Piggyback registration rights

Pursuant to the investors' rights agreement, if we register any of our securities either for our own account or for the account of other security holders, subject to certain exceptions, the holders of these shares are entitled to include their shares in the registration. Subject to certain exceptions contained in the investors' rights agreement, we and the underwriters may limit the number of shares included in the underwritten offering to the number of shares which we and the underwriters determine in our sole discretion will not jeopardize the success of the offering. However, in such circumstances, the number of shares to be included in the offering that are held by holders of registrable shares shall only be excluded if all shares proposed to be sold by us are excluded.

Indemnification

Our investors' rights agreement contains customary cross-indemnification provisions, under which we are obligated to indemnify holders of registrable securities in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions attributable to them.

Expiration of registration rights

The demand registration rights and short form registration rights granted to any holder of registrable securities under the investors' rights agreement will terminate upon the earliest to occur of (i) a deemed liquidation event (as defined in our certificate of incorporation), (ii) the fifth anniversary of the completion of this offering or (iii) such time after this offering when the holders' shares may be sold without restriction pursuant to Rule 144 within a three-month period.

Anti-takeover effects of our restated certificate of incorporation and amended and restated bylaws and Delaware Law

Our restated certificate of incorporation and amended and restated bylaws include a number of provisions that may have the effect of delaying, deferring or preventing another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Board composition and filling vacancies

Our restated certificate of incorporation provides for the division of our board of directors into three classes serving staggered three-year terms, with one class being elected each year. Our restated certificate of incorporation also provides that directors may be removed only for cause and then only by the affirmative vote of the holders of two-thirds or more of the shares then entitled to vote at an election of directors. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by the affirmative vote of a majority of our directors then in office even if less than a quorum. The classification of directors, together with the limitations on removal of directors and treatment of vacancies, has the effect of making it more difficult for stockholders to change the composition of our board of directors.

No written consent of stockholders

Our restated certificate of incorporation provides that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting. This limit may lengthen the amount of time required to take stockholder actions and would prevent the amendment of our amended and restated bylaws or removal of directors by our stockholders without holding a meeting of stockholders.

Meetings of stockholders

Our restated certificate of incorporation and amended and restated bylaws provide that only a majority of the members of our board of directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our amended and restated bylaws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

Advance notice requirements

Our amended and restated bylaws establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days nor more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. Our bylaws specify the requirements as to form and content of all stockholders' notices. These requirements may preclude stockholders from bringing matters before the stockholders at an annual or special meeting.

Amendment to restated certificate of incorporation and amended and restated bylaws

Any amendment of our restated certificate of incorporation must first be approved by a majority of our board of directors, and if required by law or our restated certificate of incorporation, must thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to stockholder action, board composition, limitation of liability and the amendment of our bylaws and certificate of incorporation must be approved by not less than two-thirds of the outstanding shares entitled to vote on the amendment, and not less than two-thirds of the outstanding shares of each class entitled to vote thereon as a class. Our amended and restated bylaws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the amended and restated bylaws, and may also be amended by the affirmative vote of at least two-thirds of the outstanding shares entitled to vote on the amendment, or, if our board of directors recommends that the stockholders approve the amendment, by the affirmative vote of the majority of the outstanding shares entitled to vote on the amendment, in each case voting together as a single class.

Undesignated preferred stock

Our restated certificate of incorporation provides for 10,000,000 authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our restated certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

Choice of forum

Our amended and restated bylaws that will become effective upon the closing of this offering will provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any state law claim for: (1) any derivative action or proceeding brought on our behalf; (2) any action asserting a claim of breach of a fiduciary duty or other wrongdoing by any of our directors, officers, employees or

agents to us or our stockholders; (3) any action asserting a claim against us arising pursuant to any provision of the Delaware General Corporation Law or our certificate of incorporation or bylaws; (4) any action to interpret, apply, enforce or determine the validity of our certificate of incorporation or bylaws or (5) any action asserting a claim governed by the internal affairs doctrine. The choice of forum provision does not apply to any actions arising under the Securities Act or the Exchange Act.

Section 203 of the Delaware General Corporation Law

Upon the closing of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or
- at or after the time the stockholder became interested, the business combination was approved by our board of directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, lease, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;
- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

Nasdaq Global Market listing

Our common stock has been approved for listing on The Nasdaq Global Market under the symbol "AXLA."

Transfer agent and registrar

The transfer agent and registrar for our common stock will be Computershare Trust company, N.A. The transfer agent and registrar's address is 250 Royall Street, Canton, Massachusetts 02021.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our shares. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of April 20, 2019, upon the completion of this offering, 22,998,513 shares of our common stock will be outstanding. Of the outstanding shares, all of the shares sold in this offering will be freely tradable, except that any shares held by our affiliates, as that term is defined in Rule 144 under the Securities Act, may only be sold in compliance with the limitations described below. All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us, or will be issued and sold by us, in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, summarized below.

Rule 144

In general, a person who has beneficially owned restricted stock for at least six months would be entitled to sell their securities provided that (i) such person is not deemed to have been one of our affiliates at the time of, or at any time during the 90 days preceding, a sale and (ii) we are subject to the Exchange Act, periodic reporting requirements for at least 90 days before the sale. Persons who have beneficially owned restricted shares for at least six months but who are our affiliates at the time of, or any time during the 90 days preceding, a sale, would be subject to additional restrictions, by which such person would be entitled to sell within any three-month period only a number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares then outstanding, which will equal approximately 229,985 shares immediately after this offering, assuming no exercise of the underwriters' option to purchase additional shares, based on the number of shares outstanding as of April 20, 2019; or

- the average weekly trading volume of our common stock on The Nasdaq Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale;

provided, in each case, that we are subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale. Such sales both by affiliates and by non-affiliates must also comply with the manner of sale, current public information and notice provisions of Rule 144.

Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares.

However, substantially all Rule 701 shares are subject to lock-up agreements as described below and under "Underwriting" included elsewhere in this prospectus and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Lock-up agreements

We, our directors and executive officers and holders of substantially all of our common stock have signed a lock-up agreement that prevent us and them from selling any of our common stock or any securities convertible into or exercisable or exchangeable for common stock for a period of not less than 180 days from the date of this prospectus without the prior written consent of the representatives of the underwriters, subject to certain exceptions. See the section entitled "Underwriting" appearing elsewhere in this prospectus for more information.

Registration rights

Upon the closing of this offering, certain holders of our securities will be entitled to various rights with respect to registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See the section entitled "Description of Capital Stock—Registration rights" appearing elsewhere in this prospectus for more information.

Equity incentive plans

We intend to file one or more registration statements on Form S-8 under the Securities Act to register our shares issued or reserved for issuance under our equity incentive plans. The first such registration statement is expected to be filed soon after the date of this prospectus and will automatically become effective upon filing with the SEC. Accordingly, shares registered under such registration statement will be available for sale in the open market, unless such shares are subject to vesting restrictions with us or the lock-up restrictions described above. As of April 20, 2019, we estimate that such registration statement on Form S-8 will cover approximately 5,702,331 shares.

CERTAIN MATERIAL U.S. FEDERAL INCOME AND ESTATE TAX CONSIDERATIONS FOR NON-U.S. HOLDERS

The following discussion is a summary of the material U.S. federal income tax considerations applicable to non-U.S. holders (as defined below) with respect to their ownership and disposition of shares of our common stock issued pursuant to this offering. For purposes of this discussion, a non-U.S. holder means a beneficial owner of our common stock that is for U.S. federal income tax purposes:

- a non-resident alien individual;
- a foreign corporation or any other foreign organization taxable as a corporation for U.S. federal income tax purposes; or
- a foreign estate or trust, the income of which is not subject to U.S. federal income tax on a net income basis.

This discussion does not address the tax treatment of partnerships or other entities that are pass-through entities for U.S. federal income tax purposes or persons that hold their common stock through partnerships or other pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its tax advisor regarding the tax consequences of acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any such change or differing interpretation could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, which we refer to as the IRS, will not challenge one or more of the tax consequences described herein. We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset within the meaning of Section 1221 of the Code, generally property held for investment.

This discussion does not address all aspects of U.S. federal income taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances including the alternative minimum tax, or the Medicare tax on net investment income and any election to apply Section 1400Z-2 of the Code to gains recognized with respect to shares of our common stock. This discussion also does not address any U.S. state, local or non-U.S. taxes or any other aspect of any U.S. federal tax other than the income tax. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt or governmental organizations;
- financial institutions;
- brokers or dealers in securities;
- regulated investment companies;
- pension plans;
- "controlled foreign corporations," "passive foreign investment companies," and corporations that accumulate earnings to avoid U.S. federal income tax;

- "qualified foreign pension funds," or entities wholly owned by a "qualified foreign pension fund";
- partnerships or other entities or arrangements treated as partnerships for U.S. federal income tax purposes (and partners and investors therein);
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment;
- persons who have elected to mark securities to market;
- persons who have a functional currency other than the U.S. dollar;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation;
- persons for whom our stock constitutes "qualified small business stock" within the meaning of Section 1202 of the Code;
- certain U.S. expatriates; and
- persons subject to special tax accounting rules as a result of any item of gross income with respect to the common stock being taken into account in an applicable financial statement under Section 451(b) of the Code.

This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders of our common stock should consult their tax advisors with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of our common stock.

Distributions on our common stock

Distributions, if any, on our common stock will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such holder's tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below in "Gain on sale or other taxable disposition of our common stock." Any such distributions will also be subject to the discussions below under the sections titled "Backup withholding and information reporting" and "Withholding and information reporting requirements—FATCA."

Subject to the discussion in the following two paragraphs in this section, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence. If we or another withholding agent apply over-withholding or if a non-U.S. holder does not timely provide us with the required certification, the non-U.S. holder may be entitled to a refund or credit of any excess tax withheld by timely filing an appropriate claim with the IRS.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder

satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to U.S. persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) to the applicable withholding agent and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty. A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

Gain on sale or other taxable disposition of our common stock

Subject to the discussions below under "Backup withholding and information reporting" and "Withholding and information reporting requirements—FATCA," a non-U.S. holder generally will not be subject to any U.S. federal income tax on any gain realized upon such holder's sale or other taxable disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a U.S. trade or business and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed-base maintained by such non-U.S. holder in the United States, in which case the non-U.S. holder generally will be taxed on a net income basis at the graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code) and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above in "Distributions on Our Common Stock" also may apply;

- the non-U.S. holder is a nonresident alien individual who is present in the United States for 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the net gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder, if any (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses; or

- we are, or have been, at any time during the five-year period preceding such sale of other taxable disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder holds no more than 5% of our outstanding common stock, directly or indirectly, actually or constructively, during the shorter of the 5-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. Generally, a corporation is a U.S. real property holding corporation only if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, or that we are likely to become one in the future. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rules described above.

Backup withholding and information reporting

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a U.S. person (as defined in the Code) in order to avoid backup withholding at the applicable rate with respect to dividends on our common stock. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above in "Distributions on Our Common Stock," generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and backup withholding rules to them. Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement. Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is filed with the IRS in a timely manner.

Withholding and information reporting requirements — FATCA

Provisions of the Code commonly referred to as the Foreign Account Tax Compliance Act, or FATCA, generally imposes a U.S. federal withholding tax at a rate of 30% on payments of dividends on, or gross proceeds from the sale or other disposition of, our common stock paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," such foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," such foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA. Under applicable U.S. Treasury regulations, withholding under FATCA currently applies to payments of dividends on our common stock. Currently proposed U.S. Treasury Regulations provide that FATCA withholding does not apply to gross proceeds from the disposition of property of a type that can produce U.S. source dividends or interest; however, prior versions of the rules would have made such gross proceeds subject to FATCA withholding. Taxpayers (including withholding agents) can currently rely on the proposed Treasury Regulations. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of this withholding tax. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this paragraph. Non-U.S. holders should consult their tax advisors regarding the possible implications of this legislation on their investment in our common stock and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of the 30% withholding tax under FATCA.

UNDERWRITING

The company and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. Goldman Sachs & Co. LLC, J.P. Morgan Securities LLC and SVB Leerink LLC are the representatives of the underwriters.

Underwriters	Number of Shares
Goldman Sachs & Co. LLC	1,428,571
J.P. Morgan Securities LLC	1,428,571
SVB Leerink LLC	714,286
Total	3,571,428

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised.

The underwriters have an option to buy up to an additional 535,714 shares from the company to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. They may exercise that option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above.

The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters by the company. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase 535,714 additional shares.

Paid by the Company	No Exercise	Full Exercise
Per Share	$ 1.40	$ 1.40
Total	$ 4,999,999.20	$ 5,749,998.80

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to $0.84 per share from the initial public offering price. After the initial offering of the shares, the representatives may change the offering price and the other selling terms. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part.

The Company has agreed that, subject to certain limited exceptions, it will not: (1) offer, pledge, sell, contract to sell, any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise transfer or dispose of, directly or indirectly, or file with, or submit to, the SEC a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition, submission or filing, or (2) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of common stock or any such other securities (in either case, regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), in each case without the prior written consent of the representatives for a period of 180 days after the date of this prospectus.

The directors and executive officers of the company and its significant shareholders have entered into lock-up agreements with the underwriters prior to the commencement of this offering

pursuant to which each of these persons or entities, subject to certain limited exceptions, for a period of 180 days after the date of this prospectus, may not, without the prior written consent of the representatives: (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such directors, executive officers, managers and members in accordance with the rules and regulations of the Securities and Exchange Commission and securities which may be issued upon exercise of a stock option or warrant); or (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of the common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise; or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock.

Prior to the offering, there has been no public market for the shares. The initial public offering price has been negotiated among the company and the representatives. Among the factors considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, were the company's historical performance, estimates of the business potential and earnings prospects of the company, an assessment of the company's management and the consideration of the above factors in relation to market valuation of companies in related businesses.

Our common stock has been approved for listing on The Nasdaq Global Market under the symbol "AXLA."

In connection with the offering, the underwriters may purchase and sell shares of common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A "covered short position" is a short position that is not greater than the amount of additional shares for which the underwriters' option described above may be exercised. The underwriters may cover any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option described above. "Naked" short sales are any short sales that create a short position greater than the amount of additional shares for which the option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in

the market price of the company's stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on The Nasdaq Global Market, in the over-the-counter market or otherwise.

The company estimates that its share of the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately $1.9 million. The company has agreed to reimburse the underwriters for certain of their expenses in an amount up to $50,000.

The company has agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates have provided, and may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively trade securities, derivatives, loans, commodities, currencies, credit default swaps and other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities or instruments of the issuer (directly, as collateral securing other obligations or otherwise) or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such assets, securities or instruments and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

European Economic Area

In relation to each Member State of the EEA which has implemented the Prospectus Directive (each, a "Relative Member State") an offer to the public of shares of our common stock may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of shares of our common stock may be made at any time under the following exemptions under the Prospectus Directive:

- To any legal entity which is a qualified investor as defined in the Prospectus Directive;
- To fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), subject to obtaining the prior consent of the Representatives for any such offer; or
- In any other circumstances falling within Article 3(2) of the Prospectus Directive;

provided that no such offer or shares of our common stock shall result in a requirement for the publication by us or any Brazilian placement agent of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to public" in relation to shares of our common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and shares of our common stock to be offered so as to enable an investor to decide to purchase shares of our common stock, as the

same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (as amended), including by Directive 2010/73/EU and includes any relevant implementing measure in the Relevant Member State.

This EEA selling restriction is in addition to any other selling restrictions set out below.

United Kingdom

In the United Kingdom, this prospectus is only addressed to and directed as qualified investors who are (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (the Order); or (ii) high net worth entities and other persons to whom it may lawfully be communicated, falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). Any investment or investment activity to which this prospectus relates is available only to relevant persons and will only be engaged with relevant persons. Any person who is not a relevant person should not act or rely on this prospectus or any of its contents.

Canada

The shares may be sold in Canada only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions, and Ongoing Registrant Obligations. Any resale of the shares must be made in accordance with an exemption form, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this offering memorandum (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Hong Kong

The shares may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32 of the Laws of Hong Kong) ("Companies (Winding Up and Miscellaneous Provisions) Ordinance") or which do not constitute an invitation to the public within the meaning of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong) ("Securities and Futures Ordinance"), or (ii) to "professional investors" as defined in the Securities and Futures Ordinance and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance, and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to

shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" in Hong Kong as defined in the Securities and Futures Ordinance and any rules made thereunder.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor (as defined under Section 4A of the Securities and Futures Act, Chapter 289 of Singapore (the "SFA")) under Section 274 of the SFA, (ii) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to conditions set forth in the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor, the securities (as defined in Section 239(1) of the SFA) of that corporation shall not be transferable for 6 months after that corporation has acquired the shares under Section 275 of the SFA except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (2) where such transfer arises from an offer in that corporation's securities pursuant to Section 275(1A) of the SFA, (3) where no consideration is or will be given for the transfer, (4) where the transfer is by operation of law, (5) as specified in Section 276(7) of the SFA, or (6) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore ("Regulation 32").

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a trust (where the trustee is not an accredited investor (as defined in Section 4A of the SFA)) whose sole purpose is to hold investments and each beneficiary of the trust is an accredited investor, the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferable for 6 months after that trust has acquired the shares under Section 275 of the SFA except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (2) where such transfer arises from an offer that is made on terms that such rights or interest are acquired at a consideration of not less than S$200,000 (or its equivalent in a foreign currency) for each transaction (whether such amount is to be paid for in cash or by exchange of securities or other assets), (3) where no consideration is or will be given for the transfer, (4) where the transfer is by operation of law, (5) as specified in Section 276(7) of the SFA, or (6) as specified in Regulation 32.

Japan

The shares have not been and will not be registered under the Financial Instruments and Exchange Act of Japan (Act No. 25 of 1948, as amended), or the FIEA. The shares may not be offered or sold, directly or indirectly, in Japan or to or for the benefit of any resident of Japan (including any person resident in Japan or any corporation or other entity organized under the laws of Japan) or to others for reoffering or resale, directly or indirectly, in Japan or to or for the benefit of any resident of Japan, except pursuant to an exemption from the registration requirements of the FIEA and otherwise in compliance with any relevant laws and regulations of Japan.

LEGAL MATTERS

The validity of the shares of common stock offered by this prospectus will be passed upon for us by Goodwin Procter LLP, Boston, Massachusetts. Certain legal matters related to this offering will be passed upon for the underwriters by Latham & Watkins LLP.

EXPERTS

The consolidated financial statements included in this prospectus have been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report appearing herein. Such consolidated financial statements have been so included in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 (File Number 333-230822) under the Securities Act with respect to the common stock we are offering by this prospectus. This prospectus does not contain all of the information included in the registration statement. For further information pertaining to us and our common stock, you should refer to the registration statement and to its exhibits. Whenever we make reference in this prospectus to any of our contracts, agreements or other documents, the references are not necessarily complete, and you should refer to the exhibits attached to the registration statement for copies of the actual contract, agreement or other document.

Upon the closing of the offering, we will be subject to the informational requirements of the Exchange Act and will file annual, quarterly and current reports, proxy statements and other information with the SEC. You can read our SEC filings, including the registration statement, at the SEC's website at www.sec.gov. We also maintain a website at https://www.axcellahealth.com/. Upon completion of the offering, you may access, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendment to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

AXCELLA HEALTH INC. AND SUBSIDIARIES

INDEX TO THE CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and Board of Directors of Axcella Health Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheet of Axcella Health Inc. and subsidiaries (the "Company") as of December 31, 2017 and 2018, and the related consolidated statements of operations, redeemable convertible preferred stock and stockholders' equity (deficit), and cash flows for the years then ended and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and 2018, and the results of its operations and its cash flows for the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulation of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

Boston, Massachusetts

March 15, 2019 (April 29, 2019 as to the effects of the reverse stock split described in Note 13)

We have served as the Company's auditor since 2012.

Axcella Health Inc.

Consolidated Balance Sheets

(in thousands, except share and per share data)

	December 31,		Pro Forma December 31,
	2017	2018	2018
			(Unaudited)
Assets			
Current assets:			
Cash and cash equivalents	$ 46,817	$ 79,466	$ 79,466
Prepaid expenses and other current assets	255	835	835
Total current assets	47,072	80,301	80,301
Property and equipment, net	1,525	1,076	1,076
Security deposits and other assets	216	216	216
Deferred offering costs	—	251	251
Total assets	$ 48,813	$ 81,844	$ 81,844
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)			
Current liabilities:			
Accounts payable	$ 1,019	$ 1,612	$ 1,612
Accrued expenses	4,076	5,299	5,299
Total current liabilities	5,095	6,911	6,911
Long term debt, net of discount	19,557	24,521	24,521
Other liabilities	1,276	1,898	1,898
Preferred stock warrant liability	411	425	—
Total liabilities	26,339	33,755	33,330
Commitments and contingencies (Note 9)	—	—	—
Redeemable convertible preferred stock (Note 7)	138,828	197,842	—
Stockholders' equity (deficit):			
Common stock, $0.001 par value; 45,000,000 and 47,000,000 shares authorized, 5,274,926 and 5,193,915 shares issued and 4,855,945 and 4,774,934 shares outstanding at December 31, 2017 and 2018, respectively, actual; 47,000,000 shares authorized, 19,835,912 shares issued and 19,416,931 shares outstanding, pro forma as of December 31, 2018 (unaudited)	5	6	19
Additional paid-in-capital	4,621	7,290	205,544
Treasury stock, 418,981 shares at cost	—	—	—
Accumulated deficit	(120,980)	(157,049)	(157,049)
Total stockholders' equity (deficit)	(116,354)	(149,753)	48,514
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 48,813	$ 81,844	$ 81,844

The accompanying notes are an integral part of these consolidated financial statements

Axcella Health Inc.

Consolidated Statements of Operations

(in thousands, except share and per share data)

	December 31,	
	2017	2018
Operating expenses:		
Research and development	$ 22,916	$ 25,486
General and administrative	6,005	8,410
Total operating expenses	28,921	33,896
Loss from operations	(28,921)	(33,896)
Other income (expense):		
Change in fair value of preferred stock warrant liability	81	(14)
Interest income (expense), net	(2,100)	(2,159)
Total other income (expense), net	(2,019)	(2,173)
Net loss	$ (30,940)	$ (36,069)
Net loss per share, basic and diluted	$ (7.37)	$ (7.97)
Weighted average common shares outstanding, basic and diluted	4,211,918	4,546,373
Pro forma net loss per share, basic and diluted (unaudited)		$ (2.19)
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)		16,528,448

The accompanying notes are an integral part of these consolidated financial statements

Axcella Health Inc.

Consolidated Statements of Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)

(in thousands, except share data)

	Redeemable convertible preferred stock		Common stock		Additional	Treasury stock			Total stockholders'
	Shares	Amount	Shares	Par Value	paid-in capital	Shares	Amount	Accumulated deficit	equity (deficit)
BALANCE — January 1, 2017	18,976,824	$113,040	4,624,196	$ 5	$ 3,466	418,981	$ —	$ (90,040)	$ (86,569)
Issuance of Series C redeemable convertible preferred stock (net of issuance costs of $259)	2,572,420	25,671							—
Exercise of common stock options			23,882		26				26
Accretion of preferred stock to redemption value		117			(117)				(117)
Stock-based compensation					1,246				1,246
Net loss								(30,940)	(30,940)
BALANCE — December 31, 2017	21,549,244	138,828	4,648,078	5	4,621	418,981	—	(120,980)	(116,354)
Issuance of Series E redeemable convertible preferred stock (net of issuance costs of $139)	5,282,002	58,861							—
Exercise of common stock options			545,837	1	44				45
Accretion of preferred stock to redemption value		153			(153)				(153)
Stock-based compensation					2,778				2,778
Net loss								(36,069)	(36,069)
BALANCE — December 31, 2018	26,831,246	$197,842	5,193,915	$ 6	$ 7,290	418,981	$ —	$ (157,049)	$ (149,753)

The accompanying notes are an integral part of these consolidated financial statements

Axcella Health Inc.

Consolidated Statements of Cash Flows

(in thousands)

	Year Ended December 31,	
	2017	2018
Cash flows from operating activities:		
Net loss	$ (30,940)	$ (36,069)
Adjustment to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,389	1,071
Stock-based compensation expense	1,246	2,778
Change in fair value of preferred stock warrant liability	(81)	14
Non-cash interest expense	509	529
Gain on sale of property and equipment	(175)	(36)
Changes in current assets and liabilities:		
Prepaid expenses and other current assets	132	(580)
Accounts payable	40	595
Accrued expense	1,019	986
Net cash used in operating activities	(26,861)	(30,712)
Cash flows from investing activities:		
Purchases of property and equipment	(1,090)	(659)
Proceeds from the sale of property and equipment	175	73
Net cash used in investing activities	(915)	(586)
Cash flows from financing activities:		
Payment of deferred offering costs	—	(14)
Proceeds from issuance of long term debt	—	6,000
Payment of debt issuance costs	—	(945)
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	25,671	58,861
Proceeds from exercise of common stock options	26	45
Net cash provided by financing activities	25,697	63,947
Net increase (decrease) in cash and cash equivalents	(2,079)	32,649
Cash and cash equivalents, beginning of year	48,896	46,817
Cash and cash equivalents, end of year	$ 46,817	$ 79,466
Supplemental cash flow information:		
Cash paid during the year for interest	$ 2,002	$ 2,255
Deferred offering costs included in accounts payable and accrued expenses	$ —	$ 237

The accompanying notes are an integral part of these consolidated financial statements

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. NATURE OF BUSINESS

Axcella Health Inc. and subsidiaries (the "Company" or "we") is a biotechnology company that was incorporated in Delaware on August 27, 2008 and has a principal place of business in Cambridge, Massachusetts. The Company is a biotechnology company pioneering the research and development of novel multifactorial interventions to support health and address dysregulated metabolism across a broad spectrum of consumers and patients who have limited options.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, successful development of technology, obtaining additional funding, protection of proprietary technology, compliance with government regulations, risks of failure of preclinical studies, clinical studies and clinical trials, the need to obtain marketing approval for its product candidates and successfully market consumer products, fluctuations in operating results, economic pressure impacting therapeutic pricing, dependence on key personnel, risks associated with changes in technologies, development by competitors of technological innovations and the ability to scale manufacturing to large scale production. Product candidates currently under development will require significant additional research and development efforts, including clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

The accompanying consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities and commitments in the ordinary course of business. The Company has funded its operations with proceeds from sales of preferred stock and borrowings under a loan and security agreement. Since its inception, the Company has incurred recurring losses, including net losses of $30.9 million and $36.1 million for the years ended December 31, 2017 and 2018, respectively. As of December 31, 2018, the Company had an accumulated deficit of $157.0 million. The Company expects to continue to generate operating losses in the foreseeable future. The Company expects that its cash and cash equivalents as of December 31, 2018 will be sufficient to fund its operations for at least the next twelve months from the date of the issuance of the financial statements.

The Company is seeking to complete an initial public offering ("IPO") of its common stock. Upon completion of a qualified public offering on specified terms, the Company's outstanding convertible preferred stock will automatically convert into shares of common stock (see Note 7). In the event the Company does not complete an IPO, the Company expects to seek additional funding through private equity financings, debt financings, strategic alliances and marketing, distribution, or licensing arrangements. The Company may not be able to obtain financing on acceptable terms, or at all, and may not be able to enter into collaborations or other arrangements. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. If the Company is unable to secure funding, it could be forced to delay, reduce, or eliminate some or all of its research development programs, product portfolio expansion or commercialization efforts, which could adversely affect its business prospects. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation and Principles of Consolidation

The Company's consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("US GAAP").

The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany accounts and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of the consolidated financial statements in accordance with US GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of expenses during the reporting period. The Company bases its estimates on historical experience, known trends and other market-specific or relevant factors that it believes to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates as there are changes in circumstances, facts and experience. Actual results may differ from those estimates or assumptions.

Unaudited pro forma

On December 18, 2018, the Company's board of directors (the "Board") authorized the Company to submit a confidential draft registration statement with the Securities and Exchange Commission to sell shares of its common stock to the public. Upon the closing of an IPO, all of the Company's outstanding shares of redeemable convertible preferred stock will automatically convert into shares of common stock. The unaudited pro forma consolidated balance sheet information as of December 31, 2018 reflects the conversion of all outstanding shares of redeemable convertible preferred stock into common stock upon the closing of an IPO and the conversion of the outstanding warrants to purchase shares of redeemable convertible preferred stock as of December 31, 2018 into warrants to purchase shares of common stock as if the proposed IPO had occurred on December 31, 2018.

For purposes of calculating pro forma basic and diluted loss per share, all shares of redeemable convertible preferred stock outstanding as of the last reporting date have been treated as if they had been converted to common stock as of the beginning of the year or on the issuance date of the preferred stock, if later.

Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but before the final financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure.

Cash and Cash Equivalents

Cash includes cash in readily available checking and money market accounts. Cash equivalents include all highly liquid investments maturing within 90 days from the date of purchase. The cash equivalents consisted of money market funds.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

Concentrations of Credit Risk

Financial instruments that subject the Company to significant concentrations of credit risk consist primarily of cash and cash equivalents. The Company's cash deposits on hand at one financial institution often exceed federally insured limits. The Company places its cash in a financial institution that management believes to be of high credit quality.

Deferred offering costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with the proposed IPO as deferred offering costs. The deferred offering costs will be offset against the IPO proceeds upon the consummation of the IPO. In the event the IPO is abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the statements of operations. The Company did not record any deferred offering costs as of December 31, 2017. As of December 31, 2018, the Company capitalized $0.3 million of deferred issuance costs related to the proposed IPO.

Property and Equipment

Property and equipment are stated at cost and depreciated using the straight-line method over the estimated useful lives of the respective assets as follows:

	Estimated useful life
Laboratory equipment	3 - 5 years
Production Equipment	3 - 15 years
Furniture and fixtures	3 - 5 years
Office and computer equipment	3 - 5 years
Leasehold improvements	Shorter of the asset's estimated useful life or the remaining lease term

Major additions and betterments are capitalized; expenditures for repairs and maintenance, which do not improve or extend the life of the respective assets, are charged to operating expense as incurred.

Impairment of Long-Lived Assets

Long-lived assets to be held and used, including property and equipment, are tested for impairment whenever events or changes in circumstances indicate that the carrying amount of the asset may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. Evaluation of the recoverability of the asset or asset group is based on an estimate of undiscounted future cash flows resulting from the use of the asset or asset group and its eventual disposition. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the asset or asset group, the assets are written down to their estimated fair values. The impairment loss would be based on the excess of the carrying value

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

of the impaired asset over its fair value, determined based on discounted cash flows. The Company did not record any impairment losses on long-lived assets during the periods presented.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under US GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the user of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- **Level 1** — Unadjusted quoted prices in active markets that are accessible to the reporting entity at the measurement date for identical assets and liabilities.
- **Level 2** — Inputs other than quoted prices in active markets for identical assets and liabilities that are observable either directly or indirectly for substantially the full term of the asset or liability. Level 2 inputs include the following:
  - quoted prices for similar assets and liabilities in active markets
  - quoted prices for identical or similar assets or liabilities in markets that are not active
  - observable inputs other than quoted prices that are used in the valuation of the asset or liabilities (e.g., interest rate and yield curve quotes at commonly quoted intervals)
  - inputs that are derived principally from or corroborated by observable market data by correlation or other means
- **Level 3** — Unobservable inputs for the assets or liability (i.e., supported by little or no market activity). Level 3 inputs include management's own assumptions about the assumptions that market participants would use in pricing the asset or liability (including assumptions about risk).

Research and Development Costs

Research and development costs are expensed as incurred. Research and development costs consist of expenses incurred in performing research and development activities, including salaries, bonuses, stock-based compensation and benefits of employees, lab supplies and materials, depreciation, manufacturing expenses, facilities expenses, overhead expenses, and external costs of vendors engaged to conduct preclinical development activities and clinical trials. Research and development costs that are paid in advance of performance (if any) are capitalized as a prepaid expense and expensed as the goods are delivered or services performed.

Research and manufacturing contract costs and accruals

The Company has entered into various research and development and manufacturing contracts. These agreements are generally cancelable, and related payments are recorded as the

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

corresponding expenses are incurred. The Company records accruals for estimated ongoing costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the research studies or clinical trials and manufacturing activities, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as research and development expenses.

Income Taxes

Deferred tax assets and liabilities are recognized for the expected future tax consequences of events that have been included in the consolidated financial statements or tax returns. Under this method, deferred tax assets and liabilities are determined based on the difference between the consolidated financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided to reduce the deferred tax asset to an amount, which, more likely than not, will be realized.

The Company recognizes the tax benefit from any uncertain tax positions only if it is more likely than not that the tax position will be sustained on examination by the taxing authorities, based on the technical merits of the position. The tax benefits recognized in the consolidated financial statements from such a position are measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate settlement. Interest and penalties associated with uncertain tax positions are recorded as a component of income tax expense. As of December 31, 2017 and 2018, the Company has not identified any uncertain tax positions for which reserves would be required.

Segment Information

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's one business segment is the development of our products. All of the Company's assets are held in the United States.

Redeemable Convertible Preferred Stock

The Company classifies stock that is redeemable in circumstances outside of the Company's control outside of permanent equity. The Company records redeemable convertible preferred stock at fair value upon issuance, net of any issuance costs, and the carrying value is increased by periodic accretion to its redemption value at the earliest redemption date, when the events that give rise to redemption are deemed probable of occurrence. These increases are effected through charges against retained earnings, if any, and then to additional paid-in capital. In the absence of paid-in capital, the accretion is charged to the accumulated deficit. The Company's redeemable

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

convertible preferred stock is subject to a dividend when and if declared by the Board. From inception through December 31, 2018, no dividend has been declared.

Stock-Based Compensation

The Company accounts for stock-based awards granted to employees and non-employees at fair value, which is measured using the Black-Scholes option-pricing model. The measurement date for employee awards is generally the date of grant. Prior to the adoption of ASU No. 2018-07, *Compensation — Stock Compensation (Topic 718)*("ASU 2018-07"), which simplifies the accounting for non-employee share based payment transactions and is discussed below under "Recently Issued Accounting Pronouncements," the fair value measurement date for non-employee awards was the date the performance of services was completed. Stock-based compensation costs are recognized as expense over the requisite service period, which is generally the vesting period, on a straight-line basis for all time-vested awards.

Upon adoption of ASU 2018-07 on January 1, 2018, the measurement date for non-employee awards is the date of grant. The compensation expense for non-employees is recognized, without changes in the fair value of the award, over the requisite service period, which is the vesting period of the respective award. The impact on its consolidated financial statements was immaterial.

The Company classifies equity-based compensation expense in its consolidated statement of operations in the same manner in which the award recipient's salary and related costs are classified or in which the award recipient's service payments are classified.

Preferred Stock Warrant Liability

Warrants to purchase shares of redeemable or contingently redeemable preferred stock are classified as a liability on the consolidated balance sheets and carried at fair value with the change in fair value recorded in the consolidated statements of operations. The liability has been classified as long-term as the warrants are not expected to be settled in the next 12 months. The warrants will expire the earlier of the close of business April 13, 2020 or one year after closing an IPO.

Upon the closing of an IPO, the preferred stock warrants will become exercisable for common stock instead of preferred stock, and the fair value of the warrant liability at that time will be reclassified to additional paid-in capital. As a result, subsequent to the closing of this IPO, the Company will no longer remeasure the fair value of the warrant liability at each reporting date.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity (deficit) that result from transactions and economic events other than those with stockholders. For the years ended December 31, 2017 and 2018, there was no difference between net loss and comprehensive loss in the accompanying consolidated financial statements.

Net Income (Loss) Per Share

Basic net loss per share attributable to common stockholders is calculated by dividing net loss attributable to common stockholders by the weighted average shares outstanding during the period. During periods of income, the Company allocates participating securities a proportional

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

share of income determined by dividing total weighted average participating securities by the sum of the total weighted average common shares and participating securities (the "two class method"). The Company's redeemable convertible preferred stock participates in any dividends declared by the Company and are therefore considered to be participating securities. Participating securities have the effect of diluting both basic and diluted earnings per share during periods of income. During periods of loss, the Company allocates no loss to participating securities because they have no contractual obligation to share in the losses of the Company. Diluted net income (loss) per share attributable to common stockholders is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock and if-converted methods. For purposes of the diluted net income (loss) per share attributable to common stockholders' calculation, redeemable convertible preferred stock, stock options, and preferred stock warrants are considered to be common stock equivalents. All common stock equivalents have been excluded from the calculation of diluted net loss per share attributable to common stockholders, as their effect would be anti-dilutive for all periods presented. Therefore, basic and diluted net loss per share were the same for all periods presented.

Recently Issued Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, *Leases*, which alters the accounting model and financial statement presentation and disclosure of leases. The new standard increases the transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet and disclosing key information about leasing arrangements. ASU 2016-02 is effective for the Company's fiscal years beginning January 1, 2020. We are assessing the impact the adoption of ASU 2016-02 will have on our consolidated financial statements and will recognize a lease obligation and right of use asset for our existing operating leases upon adoption. See additional information regarding the Company's lease obligations in Note 9.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation — Stock Compensation (Topic 718)*. ASU 2018-07 simplifies the accounting for non-employee share-based payment transactions. The Company adopted this ASU on January 1, 2018 and applied the standard prospectively in accordance with the guidance. The impact on the Company's consolidated financial statements was immaterial.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

3. PROPERTY AND EQUIPMENT

Property and equipment consist of the following (in thousands):

	December 31,	
	2017	2018
Laboratory equipment	$ 3,319	$ 3,489
Production equipment	119	—
Leasehold improvements	601	564
Office and computer equipment	299	294
Furniture and fixtures	122	122
Property and equipment	4,460	4,469
Less: accumulated depreciation and amortization	(2,935)	(3,393)
Property and equipment, net	$ 1,525	$ 1,076

Depreciation and amortization expense for the years ended December 31, 2017 and 2018 was $1.4 million and $1.1 million, respectively.

4. FAIR VALUE MEASUREMENTS

The following table sets forth by level, within the fair value hierarchy, the assets (liabilities) carried at fair value as of December 31, 2018 (in thousands):

	Fair value measurements at December 31, 2018 using:			
	Level 1	Level 2	Level 3	Total
Cash Equivalents	$ 79,216	$ —	$ —	$ 79,216
Success Fee Liability	—	—	1,220	1,220
Preferred Stock Warrant Liability	—	—	425	425
Total	$ 79,216	$ —	$ 1,645	$ 80,861

The following table sets forth by level, within the fair value hierarchy, the assets (liabilities) carried at fair value as of December 31, 2017 (in thousands):

	Fair value measurements at December 31, 2017 using:			
	Level 1	Level 2	Level 3	Total
Cash Equivalents	$ 46,567	$ —	$ —	$ 46,567
Success Fee Liability	—	—	700	700
Preferred Stock Warrant Liability	—	—	411	411
Total	$ 46,567	$ —	$ 1,111	$ 47,678

The cash equivalents are comprised of funds held in an exchange traded money market fund and the fair value of the cash equivalents is determined based upon quoted market price for that fund. The fair value of the preferred stock warrant was determined using the Black-Scholes option-

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

4. FAIR VALUE MEASUREMENTS (Continued)

pricing model with the assumptions as disclosed in Note 7. These assumptions include significant judgments including the fair value of the underlying preferred stock. An increase or decrease in the estimated fair value will result in increases or decreases in the fair value of the warrant liability and such changes could be material.

The fair value of the success fee liability was determined using a probability weighted present value of cash flows. The Company has projected that 100% of the liability will be paid and that the time value of discounting those cash flows does not have a material impact on the fair value measurement due to the expected term. The liability could decrease if the Company changes its assessment of the probability of the paying the success fee.

A roll forward of the fair value of the success fee liability and preferred stock warrant liability categorized with Level 3 inputs for the years ended December 31, 2017 and 2018 is as follows (in thousands):

	Success fee	Preferred stock warrant liability
Balance — January 1, 2017	$ 700	$ 492
Decrease in warrant fair value included in other expense	—	(81)
Balance — December 31, 2017	700	411
Increase in success fee included in other liabilities	520	—
Increase in warrant fair value included in other expense	—	14
Balance — December 31, 2018	$ 1,220	$ 425

There were no transfers among Level 1, Level 2, or Level 3 categories in the periods presented.

The carrying value of cash, cash equivalents, accounts payable and accrued expenses that are reported on the consolidated balance sheets approximate their fair value due to the short-term nature of these assets and liabilities. The carrying value of the long-term debt approximates fair value as evidenced by the recent amendment.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

5. ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

Accrued expenses and other liabilities consisted of the following (in thousands):

	December 31,	
	2017	2018
Accrued employee compensation and benefits	$ 1,765	$ 1,957
Accrued external research and development expenses	1,537	1,679
Accrued professional fees	252	678
Other	522	985
Total accrued expenses and other current liabilities	$ 4,076	$ 5,299

6. DEBT FINANCING

Long-term debt consisted of the following (in thousands):

	December 31,	
	2017	2018
Principal amount of long term debt	$ 20,000	$ 26,000
Debt discount	(257)	(612)
Deferred financing fees	(186)	(867)
Long term debt, net of discount	$ 19,557	$ 24,521

In February 2017, the Company amended and restated the August 2015 debt facility (the "February 2017 amended facility") to extend the interest only period through February 2018. Monthly principal payments of $1.0 million were to commence March 2018 for 10 months and then increase to $1.3 million for the eight months thereafter. The loan had an interest rate equal to the LIBOR rate plus 8.80% per annum (9.97% as of December 31, 2017) payable monthly and a $0.7 million success fee, payable upon the occurrence of certain events, including the completion of an IPO. The initial fair value of the success fee was recorded as an obligation to the lenders and creates a debt discount, which is then amortized to interest expense through the term of the loan.

In January 2018, the Company entered into a new secured debt facility (the "2018 Facility") with the existing lender that replaced the February 2017 amended facility. The 2018 Facility provided funding of $21.0 million. The Company paid a transaction fee of $0.9 million to the lender in connection with the 2018 Facility, and that fee was recognized as debt discount. The 2018 Facility requires interest only payments through January 2019 with the ability to extend the interest only payment period through January 2020 if certain conditions are met. Monthly principal payments of $0.6 million were to commence in February 2019 for 36 months. The 2018 Facility has an interest rate equal to the LIBOR plus 8.50% per annum (10.54% as of December 31, 2018) payable monthly and a $1.1 million success fee which is payable upon the occurrence of certain events. The success fee is comprised of $0.7 million associated with the February 2017 amended facility and an additional $0.4 million arising from the January 2018 amendment. The fair value of the additional success fee was recorded as an obligation to the lenders and created an additional debt discount. The Company granted the lender a first security interest in all assets of the

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

6. DEBT FINANCING (Continued)

Company, excluding intellectual property and granted a negative pledge on such intellectual property.

In October 2018, the Company amended the 2018 Facility (the "Amended 2018 Facility") to extend the interest only period through July 2020 or January 2021 and the Maturity Date to July 2022 or January 2023 if certain conditions are met. The Amended 2018 Facility provides additional funding in the amounts of $5.0 million ("Term B Loan") and $4.0 million ("Term C Loan") if certain conditions are met. The Term B Loan of $5.0 million was drawn in December 2018. Monthly principal payments of $1.1 million are to commence August 2020 for 24 months. The success fee increased to $1.2 million, which increased the debt discount by $0.1 million. Deferred financing costs of $0.1 million were incurred related to the amendment. The interest rate was not changed through the amendment.

For the years ended December 31, 2017 and 2018, interest expense arising from the amortization of the debt discount and deferred financing fees was $0.2 million and $0.4 million, respectively.

Terminal Interest Fee

The Company's August 2015 debt facility, as amended and the 2018 Facility, included a terminal interest fee obligation, which is due with the final principal payment of the loan and has been modified from time to time as the facilities were amended. The Company is accruing the terminal fee obligation over the term of the facility. At December 31, 2017, the terminal fee obligation was $1.1 million. The October 2018 amendment increased the terminal interest fee to $1.4 million.

The following summarizes the accrued terminal fee payable activity (in thousands):

	December 31,	
	2017	2018
Balance at beginning of year	$ 266	$ 576
Terminal interest fees accrued during the year	310	101
Balance at end of year	$ 576	$ 677

The scheduled principal maturity of the long-term debt, reflecting the 2018 Amended Facility and the Term B Loan draw offers, is as follows (in thousands):

Year Ended December 31,	
2020	$ 5,417
2021	13,000
2022	7,583
	$ 26,000

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. STOCKHOLDERS' EQUITY

The following summarizes the significant terms of the Company's capital stock and stock- based compensation plans.

Series A Preferred Stock

On April 8, 2011, the Company entered into the Series A Preferred Stock Purchase Agreement with its founding investor committing $10.8 million in Series A Preferred Stock equity financing. During 2011 and 2012, the Company issued an aggregate of 5,538,462 shares of Series A Redeemable Convertible Preferred Stock ("Series A Preferred Stock") at $1.95 per share. In 2013, an additional 223,077 shares of Series A Preferred Stock was issued. Issuance costs associated with the transaction were $30,000.

Series B Preferred Stock

On October 10, 2013 and December 11, 2013, the Company entered into the Series B Preferred Stock Purchase Agreements with its founding investor and other investors providing $12.3 million in Series B Preferred Stock equity financing (the "Series B Agreement"). In connection with entering into the Series B Agreement, the Company issued 4,737,041 shares of Series B Redeemable Convertible Preferred Stock ("Series B Preferred Stock") at $2.59 per share for gross cash proceeds of $12.3 million (inclusive of $4.1 million in convertible notes and accrued interest that were converted). Issuance costs associated with the transaction were $57,000.

Series B-1 Preferred Stock

On March 12, 2014, the Company entered into the Series B-1 Preferred Stock Purchase Agreement with a new investor providing $3.0 million in Series B-1 Preferred Stock equity financing (the "Series B-1 Agreement"). In connection with entering into the Series B-1 Agreement, the Company issued 1,084,441 shares of Series B-1 Redeemable Convertible Preferred Stock ("Series B-1 Preferred Stock") at $2.77 per share for gross cash proceeds of $3.0 million. Issuance costs associated with the transaction were $38,000.

Series C Preferred Stock

On each of January 30, 2015, April 28, 2015 and September 16, 2015, the Company entered into the Series C Preferred Stock Purchase Agreements with its founding investor and other investors providing $44.3 million in Series C Preferred Stock equity financing (the "Series C Agreement"). In connection with entering into the Series C Agreement, the Company issued 4,396,624 shares of Series C Redeemable Convertible Preferred Stock ("Series C Preferred Stock") at $10.08 per share for gross cash proceeds of $44.3 million. Issuance costs associated with the transaction were $175,000.

On August 11, 2017, the Company extended the Series C Agreement with existing investors providing an additional $25.9 million in Series C Preferred Stock equity financing (the "Series C Extension Agreement"). In connection with the Series C Extension Agreement, the Company issued 2,572,420 shares of Series C Preferred Stock at $10.08 per share for gross cash proceeds of $25.9 million. Issuance costs associated with the transaction were $259,000.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. STOCKHOLDERS' EQUITY (Continued)

Series D Preferred Stock

On February 10, 2016, the Company entered into the Series D Preferred Stock Purchase Agreement with a new strategic investor providing $42.5 million in Series D Preferred Stock equity financing (the "Series D Agreement"). In connection with entering into the Series D Agreement, the Company issued 2,997,179 shares of Series D Redeemable Convertible Preferred Stock ("Series D Preferred Stock") at $14.18 per share for gross cash proceeds of $42.5 million. Issuance costs associated with the transaction were $150,000.

Series E Preferred Stock

On November 30, 2018, the Company entered into the Series E Preferred Stock Purchase Agreement with a new strategic investor and existing investors providing $59.0 million in Series E Preferred Stock equity financing (the "Series E Agreement"). In connection with entering into the Series E Agreement, the Company issued 5,282,002 shares of Series E Redeemable Convertible Preferred Stock ("Series E Preferred Stock") at $11.17 per share for gross cash proceeds of $59.0 million. Issuance costs associated with the transaction were $139,000.

As of each balance sheet date, the Preferred Stock consisted of the following (in thousands, except for share data):

	December 31, 2018				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Common stock issuable upon conversion
Series A preferred stock	5,874,334	5,761,539	$ 11,235	$ 11,235	3,127,870
Series B preferred stock	4,737,041	4,737,041	12,250	12,250	2,571,679
Series B-1 preferred stock	1,084,441	1,084,441	2,998	3,000	588,730
Series C preferred stock	6,969,044	6,969,044	70,062	70,248	3,783,401
Series D preferred stock	2,997,179	2,997,179	42,434	42,500	1,702,785
Series E preferred stock	6,266,786	5,282,002	58,863	59,000	2,867,532
	27,928,825	26,831,246	$ 197,842	$ 198,233	14,641,997

	December 31, 2017				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Common stock issuable upon conversion
Series A preferred stock	5,874,334	5,761,539	$ 11,235	$ 11,235	3,127,870
Series B preferred stock	4,737,041	4,737,041	12,244	12,250	2,571,679
Series B-1 preferred stock	1,084,441	1,084,441	2,990	3,000	588,730
Series C preferred stock	9,356,941	6,969,044	69,950	70,248	3,783,401
Series D preferred stock	2,997,179	2,997,179	42,409	42,500	1,663,498
	24,049,936	21,549,244	$ 138,828	$ 139,233	11,735,178

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. STOCKHOLDERS' EQUITY (Continued)

The terms and conditions of the Series A, Series B, Series B-1, Series C, Series D and Series E Preferred Stock are as follows:

Dividends — Dividends shall accrue at the rate of 8% annually and are non-cumulative in nature. Dividends are payable only when and if declared by the Board. The Company shall not declare, pay, or set aside any dividends on shares of any class of common stock, unless the holders of the preferred stock shall first receive dividends on each outstanding share of preferred stock in the amount of the accrued dividends unpaid as of such date. No dividends have been declared or paid.

Liquidation — In the event of any liquidation, dissolution, or winding-up of the Company, which would include the sale of the Company, Series E Preferred Stock is senior to the Series A, Series B, and Series B-1 Preferred Stock, and *pari-passu* with the Series C and Series D Preferred Stock. The Series C, Series D, and Series E Preferred stockholders would be entitled to be paid before any payment shall be made to the holders of common stock, Series A Preferred Stock, Series B Preferred Stock, or Series B-1 Preferred Stock an amount per share equal to (i) in the case of the Series C Preferred Stock, the greater of the Series C original issue price of $10.08, plus any dividends declared but unpaid or such amount per share as would have been payable had all shares of Series C Preferred Stock been converted into Common Stock immediately prior to such liquidation, dissolution, or winding up of the Company, (ii) in the case of the Series D Preferred Stock, the greater of the Series D original issue price of $14.18, plus any dividends declared but unpaid or such amount per share as would have been payable had all shares of Series D Preferred Stock been converted into Common Stock immediately prior to such liquidation, dissolution, or winding up of the Company, and (iii) in the case of the Series E Preferred Stock, the greater of the Series E original issue price of $11.17, plus any dividends declared but unpaid or such amount per share as would have been payable had all shares of Series E Preferred Stock been converted into Common Stock immediately prior to such liquidation, dissolution, or winding up of the Company. Following the payment of the Series C, Series D, and Series E liquidation amount, the Series A Preferred stockholders would be entitled to be paid *pari-passu* with the Series B Preferred stockholders and Series B-1 Preferred Stockholders and before any distribution or payment is made to the holders of the common stock. The amount to be paid to the holders of Series A, Series B and Series B-1 Preferred Stock is an amount equal to the greater of the original purchase price per share, plus all declared but unpaid dividends thereon or such amount per share as would have been payable had all shares of Series A, Series B, Series B-1, and Series C Preferred Stock been converted to common stock prior to such liquidation, dissolution, or winding up of the Company. The original issue purchase price per share of the Series A Preferred Stock was $1.95 per share, the original issue price of the Series B Preferred Stock was $2.59 per share and the original issue price of the Series B-1 Preferred Stock was $2.77 per share. Any assets remaining following the preferential distribution to the holders of Series A, Series B, and Series B-1 Preferred Stock would be available for distribution ratably among the holders of common stock.

Voting — The preferred stockholders are entitled to the number of votes equal to the number of shares of common stock into which the shares of preferred stock held by each holder are then convertible.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. STOCKHOLDERS' EQUITY (Continued)

Conversion — Each share of Series A, Series B, Series B-1, Series C, Series D and Series E Preferred Stock is convertible at any time at the option of the holder. The number of shares into which the preferred stock converts is equal to the issuance price divided by the conversion price. The conversion price shall initially be $3.59 per share for the Series A Preferred Stock, $4.76 per share for the Series B Preferred Stock, $5.10 per share for the Series B-1 Preferred Stock, $18.57 for the Series C Preferred Stock, $26.12 for the Series D Preferred Stock, and $20.58 for the Series E Preferred Stock and may be adjusted for certain dilutive events. Conversion to common stock shall be mandatory upon the closing of an IPO resulting in net proceeds of at least $35.0 million or upon the decision of the holders of at least a majority of the outstanding preferred stock shares and the holders of at least a majority of the outstanding shares of Series C Preferred Stock. The conversion price at December 31, 2018 was $3.59 per share for the Series A Preferred Stock, $4.76 per share for the Series B Preferred Stock, $5.10 per share for the Series B-1 Preferred Stock, $18.57 for the Series C Preferred Stock, $24.96 for the Series D Preferred Stock and $20.58 for the Series E Preferred Stock.

Redemption — The preferred stock may be redeemed at the option of the majority of the holders in three annual installments on or after February 10, 2021, at a price per share equal to $1.95 per share for the Series A Preferred Stock, $2.59 per share for the Series B Preferred Stock, $2.77 per share for the Series B-1 Preferred Stock, $10.08 per share for the Series C Preferred Stock, $14.18 per share for the Series D Preferred Stock and $11.17 per share for the Series E Preferred Stock plus all declared but unpaid dividends.

Preferred Stock Warrants — In connection with the issuance of debt in 2012, the Company issued warrants to purchase 112,795 shares of Series A Preferred Stock with an exercise price of $1.95 per share. The warrants expire upon the earliest of April 13, 2020 or the first anniversary of the consummation of the initial public offering. Fair value has been estimated using a Black-Scholes option-pricing model with the following assumptions:

	December 31,	
	2017	2018
Expected volatility	71%	65%
Risk-free interest rate	2.09%	2.49%
Weighted-average remaining contractual term (in years)	2.28	1.28
Expected dividend yield	0%	0%
Fair value of underlying Series A Preferred Stock	$5.23	$5.59

Unvested Common Stock — The Company has entered into stock purchase agreements with certain senior executives of the Company.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. STOCKHOLDERS' EQUITY (Continued)

In August 2013, the Company issued 626,848 shares of nonvested common stock to a then-executive with an aggregate fair value of $0.5 million, or $0.85 per share, in exchange for $1,155 in cash and $0.5 million in a promissory note. The note bears interest at a rate of 1.22% per annum and has partial recourse for up to 50% of the value of the note. The promissory note is collateralized by all of the underlying shares of common stock (626,848 shares) and is due within 60 days of termination of the executive, immediately prior to a liquidity or liquidation event, or five years from the date of issuance. The vesting period for the shares is over a four-year period and reflects the period the stock-based compensation expense is recognized for the award. The outstanding principal balance on the promissory note as of December 31, 2017 and December 31, 2018, totaled $0.5 million and $0.0 million, respectively.

The Company determined that the promissory note did not represent a substantive exercise of an option as the employee does not bear the risks and rewards of ownership as the note is partial recourse and, therefore, was accounted for as a nonrecourse note. The promissory note was accounted for as a stock option grant and the note receivable and related shares of common stock are not recorded until such time as they are repaid. The Company has estimated the aggregate grant date fair value of the awards at $0.3 million using the Black-Scholes option-pricing model with assumptions consistent with the assumptions outlined below. During the years ended December 31, 2017 and 2018, the Company recognized $0.03 million and $0.0 million, respectively, of stock-based compensation relating to these awards.

The shares of common stock securing the promissory notes are not considered outstanding shares for accounting purposes until the shares vest, the repurchase right lapses and the holders have repaid the promissory notes. At December 31, 2017 and 2018, 626,848 and 0 shares of stock, respectively, were subject to repurchase agreements and partial recourse promissory notes.

In 2018, upon termination of the executive, the Company withheld 91,527 shares of vested common stock in exchange for the settlement of the outstanding promissory note. The former executive retained ownership of the remaining 535,320 shares of vested common stock.

No other shares of stock of the Company were subject to repurchase agreements or partial recourse promissory notes at December 31, 2017 or December 31, 2018.

Stock Option Plan — On December 23, 2010, the Company adopted the 2010 Stock Option and Grant Plan (the "2010 Plan"). The 2010 Plan, as amended, provides for the issuance of up to 5,205,082 shares of common stock to employees, officers, directors, consultants, and advisors in the form of nonqualified and incentive stock options, unvested stock awards, and other stock-based awards. In general, options typically vest over four years. An option's maximum term is 10 years. At December 31, 2018, there were 41,230 shares of common stock available for issuance under the 2010 Plan.

The Company typically grants stock options to employees and non-employees at exercise prices deemed by the Board to be equal to the fair value of the common stock at the time of grant. The fair value of the common stock has been determined by the Board at each measurement date based on a variety of different factors, including the results obtained from independent third-party appraisals, the Company's financial position and historical financial performance, the status of development of the Company's services, the current climate in the marketplace, the illiquid nature

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. STOCKHOLDERS' EQUITY (Continued)

of the common stock, the effect of the rights and preferences of the preferred stockholders, and the prospects of a liquidity event, among others.

The Company utilized the Black-Scholes option-pricing model to estimate the fair value of stock options awarded to employees. The Black-Scholes option-pricing model requires several key assumptions. The key assumptions used to apply this pricing model during the years ended December 31, 2017 and 2018, were as follows:

	2017	2018
Risk-free interest rate	1.93% - 2.34%	1.93% - 3.02%
Expected term (in years)	6.25	0.25 - 6.25
Expected dividend yield	0%	0%
Expected volatility of underlying common stock	65%	65%

The risk-free interest rate was based on rates associated with U.S. Treasury issues approximating the expected life of the stock options. The expected term of options granted to employees was determined using the simplified method, which represents the midpoint of the contractual term of the option and the weighted-average vesting period of the option. The Company uses the simplified method because it does not have sufficient historical option exercise data to provide a reasonable basis upon which to estimate the expected term. The expected dividend-yield assumption was based on the Company's expectation of no future dividend payments. The expected volatility of the underlying stock was based on the average historical volatility of comparable publicly traded companies based on weekly price returns as reported by a pricing service, as the Company does not have a trading history for its stock.

The weighted-average grant date fair value of the options granted during the years ended December 31, 2017 and 2018, was $4.46 per share and $3.63 per share, respectively. As of December 31, 2018, there was $9.7 million of unrecognized compensation expense related to unvested stock options that is expected to be recognized over a weighted-average period of 3.0 years. Stock-based compensation related to stock options and unvested stock awards are classified as follows (in thousands):

	December 31,	
	2017	2018
Research and development	$ 604	$ 1,088
General and administrative	642	1,690
	$ 1,246	$ 2,778

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. STOCKHOLDERS' EQUITY (Continued)

The following table summarizes the option activity under the 2010 Plan:

	Options	Weighted Average Exercise Price	Weighted Average Remaining Life (in Years)	Intrinsic Value (in thousands)
Outstanding January 1, 2018	2,624,321	$ 5.41	8.7	$ 2,900
Granted	1,807,814	5.87		
Exercised	(10,517)	4.31		
Canceled	(382,154)	6.38		
Outstanding December 31, 2018	4,039,464	$ 5.67	8.6	
Options vested or expected to vest as of December 31, 2018	3,936,746	$ 5.67	8.6	$ 11,328
Options exercisable as of December 31, 2018	1,201,096	$ 4.11	6.7	$ 5,331

The intrinsic value of options exercised during the years ended December 31, 2017 and 2018 was nominal.

Common Stock Reserved — As of December 31, 2018, the Company had authorized 47,000,000 shares of common stock. The number of shares of common stock has been reserved for the potential conversion of Series A, Series B, Series B-1, Series C, Series D and Series E Preferred Stock and exercise of stock options as of December 31, 2018, is as follows:

	As of December 31, 2018
Series A Preferred Stock	3,127,870
Series B Preferred Stock	2,571,679
Series B-1 Preferred Stock	588,730
Series C Preferred Stock	3,783,401
Series D Preferred Stock	1,702,785
Series E Preferred Stock	2,867,532
Common Stock Options	4,039,464
Total shares reserved for issuance	18,681,461

8. INCOME TAXES

There is no provision for income taxes because the Company has historically incurred operating losses and maintains a full valuation allowance against its deferred tax assets. A

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

8. INCOME TAXES (Continued)

reconciliation of income taxes computed using the U.S. federal statutory rate to that reflected in operations as of December 31, 2017 and 2018 are as follows:

	December 31,	
	2017	2018
Tax at U.S. statutory rate	34.0 %	21.0 %
State taxes, net of federal benefit	5.4 %	6.2 %
Permanent differences	(2.2)%	(1.5)%
Tax credits	3.3 %	3.8 %
U.S. tax reform impact	(44.9)%	0.0 %
Change in valuation allowance	4.4 %	(29.5)%
Effective income tax rate	0.0 %	0.0 %

With enactment of the Tax Cuts and Jobs Act (the "Act"), which was signed by President Trump on December 22, 2017, Congress substantially amended the Internal Revenue Code, which, among other things, permanently reduced the U.S. corporate income tax rate from 34% to 21% effective for tax years including or commencing January 1, 2018. As such in 2017, the Company remeasured its existing cumulative temporary differences at the enacted rate of 21% as compared to the previous rate of 34%. The remeasurement of the Company's deferred tax balances of $13.9 million was offset by the application of its valuation allowance. The change in the U.S. corporate income tax rate is reflected in the Company's deferred tax table. The Company finalized its assessment of the Act during the fourth quarter of 2018 and did not record any changes from its initial remeasurement.

Significant components of the Company's deferred tax asset at December 31, 2017 and 2018 are as follows (in thousands):

	December 31,	
	2017	2018
Net operating loss carryforwards	$ 29,456	$ 38,337
Research and development tax credit carryforwards	4,107	5,470
Start-up costs	50	43
Capitalized research and development costs	384	277
Depreciation	457	479
Accrued expenses	725	811
Stock-based compensation	412	600
Other items	499	710
Total deferred tax assets	36,090	46,727
Valuation allowance	(36,090)	(46,727)
Net deferred tax asset	$ —	$ —

As of December 31, 2018, the Company had federal and state net operating loss carryforwards of $140.6 million and $139.4 million, respectively, which may be used to offset future taxable income, if any. These amounts begin to expire in 2030. The federal net operating losses

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

8. INCOME TAXES (Continued)

generated in 2018 can be carried forward indefinitely. As of December 31, 2018, the Company had federal and state research and development tax credit carryforwards of $4.2 million and $1.6 million, respectively. These amounts expire at various dates through 2038. Due to the degree of uncertainty related to the ultimate use of the deferred tax assets, the Company has fully reserved these tax benefits, as the determination of the realization of the deferred tax benefits was not determined to be more likely than not. The valuation allowance increased in 2018 by $10.6 million, due to the increase in deferred tax assets by the same amount (primarily due to net operating loss carryforwards) and the Company's recording of a full valuation allowance. In 2017, the valuation allowance decreased by $1.4 million which was driven by the $13.9 million reduction in the U.S. corporate income tax rate discussed above and partially offset by the current period net operating loss.

Utilization of the net operating loss and research and development credit carryforwards may be subject to a substantial annual limitation under Sections 382 and 383 of the Internal Revenue Code of 1986 due to ownership change limitations that have occurred previously or that could occur in the future. These ownership changes may limit the amount of net operating loss and research and development credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. As of December 31, 2018, the Company had not yet completed an analysis of whether its net operating loss and research and development credit carryforwards may be limited.

At December 31, 2017 and 2018, the Company had no unrecognized tax benefits. As of December 31, 2017 and 2018, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in our consolidated statements of operations. The Company will recognize interest and penalties related to uncertain tax positions in income tax expense.

For years through December 31, 2018, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carryforwards and the valuation allowance.

The Company files income tax returns in the U.S. federal and Massachusetts and New Jersey jurisdictions. The statute of limitations for assessment by the Internal Revenue Service, or IRS, and state tax authorities is closed for tax years prior to 2015, although carryforward attributes that were generated prior to tax year 2015 may still be adjusted upon examination by the IRS or state tax authorities if they either have been or will be used in a future period. There are no federal or state audits in progress.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

9. COMMITMENTS AND CONTINGENCIES

Leases

The Company entered into a facility lease agreement for laboratory and office space with an expiration date of April 1, 2021. The lease agreement and most recent amendment contained escalating rent payments. Rent expense is recorded on a straight-line basis. The Company had deferred rent of $0.1 million as of both December 31, 2017 and 2018. The Company is obligated to make minimum lease payments under the facility lease as follows (in thousands):

Years Ending December 31,	
2019	$ 1,187
2020	1,226
2021	415
Total	$ 2,828

Rent expense for the years ended December 31, 2017 and 2018 was $1.1 million and $1.2 million, respectively.

10. RETIREMENT PLAN

The Company has a 401(k) retirement and savings plan (the "Plan") covering all qualified employees. The Plan allows each participant to contribute a portion of his or her base wages up to an amount not to exceed an annual statutory maximum. The Company is permitted to make discretionary matching contributions to the Plan. The Company has not made any discretionary contributions.

11. NET LOSS AND PRO FORMA NET LOSS PER SHARE

Net Loss Per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

	Year ended December 31,	
	2017	2018
Numerator:		
Net loss	$ (30,940)	$ (36,069)
Accretion of redeemable convertible preferred stock	(117)	(153)
Net loss attributable to common stockholders	$ (31,057)	$ (36,222)
Denominator:		
Weighted average common shares outstanding, basic and diluted	4,211,918	4,546,373
Net loss per share attributable to common stockholders, basic and diluted	$ (7.37)	$ (7.97)

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

11. NET LOSS AND PRO FORMA NET LOSS PER SHARE (Continued)

The Company's potential dilutive securities, which include redeemable convertible preferred stock, warrants to purchase redeemable convertible preferred stock, vested unvested stock purchased with promissory notes and common stock options, have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	Year ended December 31,	
	2017	2018
Redeemable convertible preferred stock (as converted to common stock)	11,735,178	14,641,997
Warrants to purchase redeemable convertible preferred stock (as converted to common stock)	61,235	61,235
Options to purchase common stock	2,624,321	4,039,464
	14,420,734	18,742,696

Pro Forma Net Loss Per Share (Unaudited)

The pro forma basic and diluted net loss per share for the year ended December 31, 2018 has been prepared to give effect to adjustments arising upon the completion of a qualified IPO. The pro forma net loss used in the calculation of pro forma basic and diluted net loss per share attributable to common stockholders does not include the change in the fair value of the warrant liability because the calculation gives effect to all warrants to purchase redeemable convertible preferred stock becoming warrants to purchase common stock as if the proposed IPO had occurred on the later of January 1, 2018 or the date of issuance of the preferred stock. The pro forma basic and diluted weighted average common shares outstanding used in the calculation of pro forma basic and diluted net loss per share for the year ended December 31, 2018 has been prepared to give effect, upon a qualified IPO, to the automatic conversion of all outstanding shares of redeemable convertible preferred stock into common stock as if the proposed IPO had occurred on the later of January 1, 2018 or the issuance date of the redeemable convertible preferred stock.

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

11. NET LOSS AND PRO FORMA NET LOSS PER SHARE (Continued)

Pro forma basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

	Year ended December 31, 2018 (unaudited)
Numerator:	
Net loss attributable to common stockholders	$ (36,222)
Accretion of redeemable convertible preferred stock	153
Net loss	$ (36,069)
Change in fair value of preferred stock warrant liability	14
Pro forma net loss attributable to common stockholders	$ (36,055)
Denominator:	
Weighted average common shares outstanding, basic and diluted	4,546,373
Pro forma adjustment to reflect automatic conversion of redeemable convertible preferred stock into common stock upon the completion of the proposed IPO	11,982,075
Pro forma weighted average common shares outstanding, basic and diluted	16,528,448
Pro forma net loss per share, basic and diluted	$ (2.19)

12. RELATED-PARTY TRANSACTIONS

In April 2013, the Company entered into a services agreement with Flagship Ventures Management, Inc. ("Flagship"), an affiliate of one of the Company's principal stockholders, to provide various strategic consulting services to the Company. The total expense under the agreement for the years ended December 31, 2017 and 2018 was $0.1 million and $0, respectively. As of December 31, 2017 and 2018, there were no amounts payable to Flagship for costs related to the services agreement.

13. SUBSEQUENT EVENTS

The Company has evaluated subsequent events for financial statement purposes occurring through March 15, 2019, the date that these consolidated financial statements were originally issued, and April 29, 2019, the date on which the retrospectively revised consolidated financial statements were reissued (as a result of the reverse stock split discussed below), and determined that no additional subsequent events had occurred that would require recognition in these consolidated financial statements and that all subsequent events that require disclosure have been disclosed.

2019 Stock Option and Incentive Plan — The 2019 Stock Option and Incentive Plan (the "2019 Plan") was approved by our board of directors on April 29, 2019, and will become effective on the date immediately prior to the date on which the registration statement for the Company's proposed initial public offering is declared effective. The 2019 Plan provides for the grant of incentive stock options, nonqualified stock options, stock appreciation rights, restricted stock units,

AXCELLA HEALTH INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

13. SUBSEQUENT EVENTS (Continued)

restricted stock awards, unrestricted stock awards, cash-based awards and dividend equivalent rights to the Company's officers, employees, directors and consultants. The number of shares initially reserved for issuance under the 2019 Plan is 905,000, which shall be cumulatively increased on January 1, 2020 and each January 1 thereafter by 4% of the number of shares of the Company's common stock outstanding on the immediately preceding December 31 or such lesser number of shares determined by the Company's board of directors or compensation committee of the board of directors.

2019 Employee Stock Purchase Plan — The 2019 Employee Stock Purchase Plan (the "2019 ESPP") was approved by our board of directors on April 29, 2019, and will become effective on the date immediately prior to the date on which the registration statement for the Company's proposed initial public offering is declared effective. A total of 237,181 shares of common stock were initially reserved for issuance under this plan, which shall be cumulatively increased on January 1, 2020 and each January 1 thereafter by 1% of the number of shares of the Company's common stock outstanding on the immediately preceding December 31 or such lesser number of shares determined by the Company's board of directors or compensation committee of the board of directors.

Reverse Stock Split — On April 29, 2019, the Company effected a 1-for-1.842 reverse stock split of the Company's common stock. All shares, stock options, warrants and per share information presented in the consolidated financial statements have been adjusted to reflect the reverse stock split on a retroactive basis for all periods presented. There was no change in the par value of the Company's common stock. The ratio by which shares of preferred stock are convertible into shares of common stock was adjusted to reflect the effects of the reverse stock split.

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3,571,428 Shares

Axcella Health Inc.

Common Stock

PRELIMINARY PROSPECTUS

Joint Book-Running Managers

GOLDMAN SACHS & CO. LLC

J.P. MORGAN

SVB LEERINK

Until June 2, 2019 (25 days after the date of this prospectus), all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.