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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

FOR THE QUARTERLY PERIOD ENDED March 31, 2019
OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

FOR THE TRANSITION PERIOD FROM _ TO _

COMMISSION FILE NUMBER 001-38501

AXCELLA HEALTH INC.
(Exact name of registrant as specified in its charter)

Delaware	26-3321056
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
840 Memorial Drive Cambridge, Massachusetts	02139
(Address of principal executive offices)	**(Zip Code)**

(857) 320-2200
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock	AXLA	The Nasdaq Global Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☐ No ☒

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒ (Do not check if a smaller reporting company)	Smaller reporting company	☐
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of June 18, 2019, the registrant had 23,008,283 shares of common stock, $0.001 par value per share, outstanding.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

In this Quarterly Report on Form 10-Q, or Quarterly Report, 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.

"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.

"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.

"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.

This Quarterly Report contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “may”, “will”, “should”, “expects”, “intends”, “plans”, “anticipates”, “believes”, “estimates”, “predicts”, “potential”, “continue” or the negative of these terms or other comparable terminology. These forward-looking statements 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, the European Medicines Agency, or the EMA, and other comparable 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 type 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 for both product development and as a public company, future revenue, capital requirements and needs for additional financing;
- the potential for faults in our internal controls; and
- other risks and uncertainties, including those discussed in Part II, Item 1A, Risk Factors in this Quarterly Report.

Any forward-looking statements in this Quarterly Report reflect our current views with respect to future events and with respect to our future financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those described under Part II, Item 1A, Risk Factors and elsewhere in this Quarterly Report. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

We may from time to time provide estimates, projections and other information concerning our industry, the general business environment, and the markets for certain diseases, including estimates regarding the potential size of those markets and the estimated incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties, and actual events, circumstances or numbers, including actual disease prevalence rates and market size, may differ materially from the information reflected in this Quarterly Report. Unless otherwise expressly stated, we obtained this industry, business information, market data, prevalence information and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources, in some cases applying our own assumptions and analysis that may, in the future, prove not to have been accurate.

AXCELLA HEALTH INC.
FORM 10-Q
FOR THE QUARTER ENDED MARCH 31, 2019
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PART I - FINANCIAL INFORMATION

Item I. Condensed Consolidated Financial Statements (Unaudited)

AXCELLA HEALTH INC.
Condensed Consolidated Balance Sheets (Unaudited)
(in thousands, except share and per share data)

	As of	
	March 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 66,769	$ 79,466
Prepaid expenses and other current assets	1,679	835
Total current assets	68,448	80,301
Property and equipment, net	920	1,076
Security deposits and other assets	216	216
Deferred offering costs	1,334	251
Total assets	$ 70,918	$ 81,844
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 2,470	$ 1,612
Accrued expenses	3,735	5,299
Total current liabilities	6,205	6,911
Long term debt, net of discount	24,624	24,521
Other liabilities	1,948	1,898
Preferred stock warrant liability	476	425
Total liabilities	33,253	33,755
Commitments and contingencies (Note 8)	—	—
Redeemable convertible preferred stock (Note 7)	197,888	197,842
Stockholders' equity (deficit):		
Common stock, $0.001 par value; 47,000,000 and 47,000,000 shares authorized, 5,195,250 and 5,193,915 shares issued and 4,776,269 and 4,774,934 shares outstanding at March 31, 2019 and December 31, 2018, respectively	6	6
Additional paid-in-capital	8,393	7,290
Treasury stock, 418,981 shares at cost	—	—
Accumulated deficit	(168,622)	(157,049)
Total stockholders' equity (deficit)	(160,223)	(149,753)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 70,918	$ 81,844

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AXCELLA HEALTH INC.
Condensed Consolidated Statements of Operations (Unaudited)
(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2019	2018
Operating expenses:		
Research and development	$ 7,563	$ 5,455
General and administrative	3,468	2,136
Total operating expenses	11,031	7,591
Loss from operations	(11,031)	(7,591)
Other income (expense):		
Change in fair value of preferred stock warrant liability	(51)	37
Interest income (expense), net	(491)	(546)
Total other income (expense), net	(542)	(509)
Net loss	$ (11,573)	$ (8,100)
Net loss per share, basic and diluted	$ (2.43)	$ (1.92)
Weighted average common shares outstanding, basic and diluted	4,775,828	4,229,118

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AXCELLA HEALTH INC.
Condensed Consolidated Statements of Cash Flows (Unaudited)
(in thousands)

	Three Months Ended March 31,	
	2019	2018
Cash flows from operating activities:		
Net loss	$ (11,573)	$ (8,100)
Adjustment to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	203	355
Stock-based compensation expense	1,137	368
Change in fair value of preferred stock warrant liability	51	(37)
Non-cash interest expense	153	75
Changes in current assets and liabilities:		
Prepaid expenses and other current assets	(844)	92
Accounts payable	858	291
Accrued expenses	(2,182)	(1,169)
Net cash used in operating activities	(12,197)	(8,125)
Cash flows from investing activities:		
Purchases of property and equipment	(47)	(288)
Net cash used in investing activities	(47)	(288)
Cash flows from financing activities:		
Payment of deferred offering costs	(465)	—
Proceeds from issuance of long term debt	—	1,000
Payment of debt issuance costs	—	(844)
Proceeds from exercise of common stock options	12	—
Net cash (used in) provided by financing activities	(453)	156
Net decrease in cash and cash equivalents	(12,697)	(8,257)
Cash and cash equivalents, beginning of period	79,466	46,817
Cash and cash equivalents, end of period	$ 66,769	$ 38,560
Supplemental cash flow information:		
Cash paid for interest	$ 715	$ 535
Deferred offering costs included in accounts payable and accrued expenses	$ 618	$ —

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AXCELLA HEALTH INC.
Condensed Consolidated Statements of Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit) (Unaudited)
(in thousands, except share data)

	Redeemable convertible preferred stock		Common stock			Treasury stock			Total
	Shares	Amount	Shares	Par Value	Additional paid-in capital	Shares	Amount	Accumulated deficit	stockholders' equity (deficit)
BALANCE - December 31, 2017	21,549,244	$138,828	4,648,078	$ 5	$ 4,621	418,981	$ —	$ (120,980)	$ (116,354)
Accretion of preferred stock to redemption value		38			(38)				(38)
Stock-based compensation					368				368
Net loss								(8,100)	(8,100)
BALANCE - March 31, 2018	21,549,244	138,866	4,648,078	5	4,951	418,981	—	(129,080)	(124,124)
BALANCE - December 31, 2018	26,831,246	$197,842	5,193,915	$ 6	$ 7,290	418,981	$ —	$ (157,049)	$ (149,753)
Exercise of common stock options			1,335		12				12
Accretion of preferred stock to redemption value		46			(46)				(46)
Stock-based compensation					1,137				1,137
Net loss								(11,573)	(11,573)
BALANCE - March 31, 2019	26,831,246	$197,888	5,195,250	$ 6	$ 8,393	418,981	$ —	$ (168,622)	$ (160,223)

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AXCELLA HEALTH INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (Unaudited)

1. NATURE OF BUSINESS

Axcella Health Inc. and subsidiaries ("Axcella," 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 early-stage 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, if required, 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 preclinical and clinical testing and any necessary regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

On April 29, 2019, the Company filed an amended and restated certificate of incorporation to effect a one-for-1.842 reverse stock split of the Company’s common stock. All share and per share data shown in the accompanying condensed consolidated financial statements and related notes have been retroactively revised to reflect the reverse stock split.

On May 13, 2019, the Company completed an initial public offering (the “IPO”) of 3,571,428 shares of its common stock for aggregate gross proceeds of $71.4 million and its shares started trading on The Nasdaq Global Market under the ticker symbol “AXLA.” The Company received approximately $64.5 million in net proceeds after deducting underwriting discounts and commissions and other estimated offering expenses payable by the Company. Upon closing of the IPO, all of the Company's outstanding shares of redeemable convertible preferred stock automatically converted into 14,641,997 shares of common stock.

The accompanying condensed 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 historically funded its operations with proceeds from sales of preferred stock and borrowings under a loan and security agreement. As of March 31, 2019, the Company had an accumulated deficit of $168.6 million. The Company expects to continue to generate operating losses in the foreseeable future. The Company expects that its cash and cash equivalents at March 31, 2019, along with the net proceeds it received from completion of the IPO, will be sufficient to fund its operations for at least the next twelve months from the date of the issuance of the interim condensed consolidated financial statements.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation and Principles of Consolidation

The Company's condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("US GAAP").

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany accounts and transactions have been eliminated in consolidation.

Unaudited Interim Financial Information

The condensed consolidated financial statements of the Company included herein have been prepared, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission (the “SEC”). Certain information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP") have been condensed or omitted from this report, as is permitted by such rules and regulations. Accordingly, these condensed consolidated financial statements should be read in conjunction with the financial statements and notes thereto included in the Company’s Prospectus that forms a part of the Company’s Registration Statement on Form S-1 (File No. 333-230822), which was filed with the SEC pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended ("Securities Act"), on May 9, 2019 (the “Prospectus”). The results for any interim period are not necessarily indicative of results for any future period.

Use of Estimates

The preparation of the condensed 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 condensed 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.

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, which closed on May 13, 2019. As of March 31, 2019 and December 31, 2018, the Company capitalized $1.3 million and $0.3 million, respectively, of deferred issuance costs related to the IPO.

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 general and administrative expenses.

Summary of Significant Accounting Policies

The Company’s significant accounting policies are described in Note 2 *“Summary of Significant Accounting Policies,”* in the Prospectus. There have been no material changes to the significant accounting policies during the period ended March 31, 2019.

Accounting Pronouncements Issued and Not Adopted

In February 2016, the Financial Accounting Standards Board issued Accounting Standards Update, or 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. The Company is assessing the impact the adoption of ASU 2016-02 will have on its condensed consolidated financial statements and will recognize a lease obligation and right of use asset for its existing operating leases upon adoption. See additional information regarding the Company's lease obligations in Note 8.

3. PROPERTY AND EQUIPMENT

Property and equipment consist of the following (in thousands):

	March 31, 2019	December 31, 2018
Laboratory equipment	$ 3,536	$ 3,489
Leasehold improvements	564	564
Office and computer equipment	294	294
Furniture and fixtures	122	122
Property and equipment	4,516	4,469
Less: accumulated depreciation and amortization	(3,596)	(3,393)
Property and equipment, net	$ 920	$ 1,076

Depreciation and amortization expense for the three months ended March 31, 2019 and 2018 was $0.2 million and $0.4 million, respectively.

4. FAIR VALUE MEASUREMENTS

The following table sets forth by level, within the fair value hierarchy, the assets and liabilities carried at fair value on a recurring basis (in thousands):

	Fair value measurements at March 31, 2019 using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash Equivalents	$ 66,519	$ —	$ —	$ 66,519
Total	$ 66,519	$ —	$ —	$ 66,519
Liabilities:				
Success Fee Liability	$ —	$ —	$ 1,220	$ 1,220
Preferred Stock Warrant Liability	—	—	476	476
Total	$ —	$ —	$ 1,696	$ 1,696

	Fair value measurements at December 31, 2018 using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash Equivalents	$ 79,216	$ —	$ —	$ 79,216
Total	$ 79,216	$ —	$ —	$ 79,216
Liabilities:				
Success Fee Liability	$ —	$ —	$ 1,220	$ 1,220
Preferred Stock Warrant Liability	—	—	425	425
Total	$ —	$ —	$ 1,645	$ 1,645

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-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 paying the success fee.

As a result of the IPO, the preferred stock warrants were converted to warrants to purchase common stock and the fair value of the warrant liability was reclassified to stockholders' equity and the success fee liability was settled. As such, in the periods subsequent to the IPO, the carrying value of these instruments will no longer be adjusted.

A roll forward of the fair value of the success fee liability and preferred stock warrant liability categorized with Level 3 inputs for the period ended March 31, 2019 is as follows (in thousands):

	Success fee	Preferred stock warrant liability
Balance — January 1, 2019	$ 1,220	$ 425
Increase in warrant fair value included in other expense	—	51
Balance — March 31, 2019	$ 1,220	$ 476

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.

5. ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

Accrued expenses and other liabilities consisted of the following (in thousands):

	March 31, 2019	December 31, 2018
Accrued employee compensation and benefits	$ 527	$ 1,957
Accrued external research and development expenses	2,007	1,679
Accrued professional fees	376	678
Other	825	985
Total accrued expenses and other current liabilities	$ 3,735	$ 5,299

6. DEBT FINANCING

Long term debt consisted of the following (in thousands):

	March 31, 2019	December 31, 2018
Principal amount of long term debt	$ 26,000	$ 26,000
Debt discount	(569)	(612)
Deferred financing fees	(807)	(867)
Long term debt, net of discount	$ 24,624	$ 24,521

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 required interest only payments through January 2019 with the ability to extend the interest only payment period through January 2020 if certain conditions were 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 (10.99% as of March 31, 2019) payable monthly and a $1.1 million success fee which is payable upon the occurrence of certain events, including the IPO. 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 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. 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.

Upon completion of the IPO in May 2019, the interest only period was extended through January 2021 and the Maturity Date was extended to January 2023. Monthly principal payments of $1.1 million are to commence February 2021 for 24 months. The $1.2 million success fee was also paid.

For the three months ended March 31, 2019 and 2018, interest expense arising from the amortization of the debt discount and deferred financing fees was $0.2 million and $0.1 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. There was no change in the terminal interest fee during the 3 months ended March 31, 2019. The carrying value of the terminal interest fee was $0.7 million and $0.7 million at March 31, 2019 and December 31, 2018, respectively.

The scheduled principal maturity of the long-term debt, reflecting the 2018 Amended Facility and the Term B Loan draw offers, as of March 31, 2019 is as follows (in thousands):

Year Ending December 31,	
2020	$ 5,417
2021	13,000
2022	7,583
	$ 26,000

7. STOCKHOLDERS' EQUITY

At March 31, 2019, the redeemable convertible preferred stock (the “Preferred Stock”) consisted of the following (in thousands, except for share data):

	March 31, 2019				
	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	3,000	3,000	588,730
Series C Preferred Stock	6,969,044	6,969,044	70,091	70,248	3,783,401
Series D Preferred Stock	2,997,179	2,997,179	42,442	42,500	1,702,785
Series E Preferred Stock	6,266,786	5,282,002	58,870	59,000	2,867,532
	27,928,825	26,831,246	$ 197,888	$ 198,233	14,641,997

Upon closing of the IPO in May 2019, all of the Preferred Stock converted into 14,641,997 shares of common stock.

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 and an expiration date of April 13, 2020. The fair value of the warrants has been estimated using a Black-Scholes option-pricing model with the following assumptions:

	March 31,	
	2019	2018
Expected volatility	73.41%	65%
Risk-free interest rate	2.22%	2.48%
Weighted-average remaining contractual term (in years)	1.03	2.03
Expected dividend yield	0%	0%
Fair value of underlying Series A Preferred Stock	$6.06	$4.98

Upon closing of the IPO on May 13, 2019, the outstanding warrants to purchase Series A Preferred Stock became outstanding warrants to purchase an aggregate of 61,235 shares of common stock at a weighted average exercise price of $3.59 per share.

The Company typically grants stock options to employees and non-employees at exercise prices deemed by the Company's board of directors (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 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 three months ended March 31, 2019 and 2018, were as follows:

	2019	2018
Risk-free interest rate	2.30% - 2.50	2.05% - 2.68%
Expected term (in years)	6.25	1.83 - 6.25
Expected dividend yield	0%	0%
Expected volatility of underlying common stock	73.4%	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 three months ended March 31, 2019, was $5.02 per share. As of March 31, 2019, there was $12.8 million of unrecognized compensation expense related to unvested stock options that is expected to be recognized over a weighted-average period of 3.1 years. Stock-based compensation related to stock options and unvested stock awards are classified as follows (in thousands):

	Three Months Ended March 31,	
	2019	2018
Research and development	$ 482	$ 211
General and administrative	655	157
	$ 1,137	$ 368

The following table summarizes the option activity under the 2010 Stock Incentive Plan:

	Options	Weighted Average Exercise Price	Weighted Average Remaining Life (in Years)	Intrinsic Value (in thousands)
Outstanding January 1, 2019	4,039,487	$ 5.67	8.6	$ 5,331
Granted	542,996	13.83		
Exercised	(1,336)	6.16		
Canceled	(8,028)	6.63		
Outstanding March 31, 2019	4,573,119	$ 6.55	7.6	
Options vested or expected to vest as of March 31, 2019	4,402,433	$ 6.55	7.6	$ 32,050
Options exercisable as of March 31, 2019	1,330,837	$ 4.41	5.8	$ 12,536

The intrinsic value of options exercised during the three months ended March 31, 2019 was nominal.

8. 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 March 31, 2019 and 2018. The Company is obligated to make minimum lease payments under the facility lease as follows (in thousands):

Years Ending December 31,	
2019	$ 896
2020	1,226
2021	415
Total	$ 2,537

Rent expense for the three months ended March 31, 2019 and 2018 was $0.3 million and $0.3 million, respectively.

We enter into contracts in the normal course of business with contract research organizations ("CROs"), contract manufacturing organizations ("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 upon prior written notice. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including noncancelable obligations of service providers, up to the date of cancellation.

9. 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.

10. 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):

	Three Months Ended March 31,	
	2019	2018
Numerator:		
Net loss	$ (11,573)	$ (8,100)
Accretion of redeemable convertible preferred stock	(46)	(38)
Net loss attributable to common stockholders	$ (11,619)	$ (8,138)
Denominator:		
Weighted average common shares outstanding, basic and diluted	4,775,828	4,229,118
Net loss per share, basic and diluted	$ (2.43)	$ (1.92)

The Company's potential dilutive securities, which include redeemable convertible preferred stock, warrants to purchase redeemable convertible preferred stock, vested and 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:

	Three Months Ended March 31,	
	2019	2018
Redeemable convertible preferred stock (as converted to common stock)	14,641,997	11,735,178
Warrants to purchase redeemable convertible preferred stock (as converted to common stock)	61,235	61,235
Options to purchase common stock	4,573,119	2,673,453
	19,276,351	14,469,866

Upon closing of the IPO, all of the Preferred Stock converted into 14,641,997 shares of common stock. 3,571,428 shares of common stock were issued as part of the IPO.

11. SUBSEQUENT EVENTS

The Company has evaluated subsequent events for financial statement purposes occurring through June 20, 2019, the date that these condensed consolidated financial statements were issued.

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 became effective immediately prior to May 9, 2019, which was the date the registration statement for the Company's proposed IPO was declared effective. The 2019 Plan provides for the grant of incentive stock options, nonqualified stock options, stock appreciation rights, restricted stock units, 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 became effective immediately prior to May 9, 2019, which was the date the registration statement for the Company's proposed IPO was 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.

* * * * * *

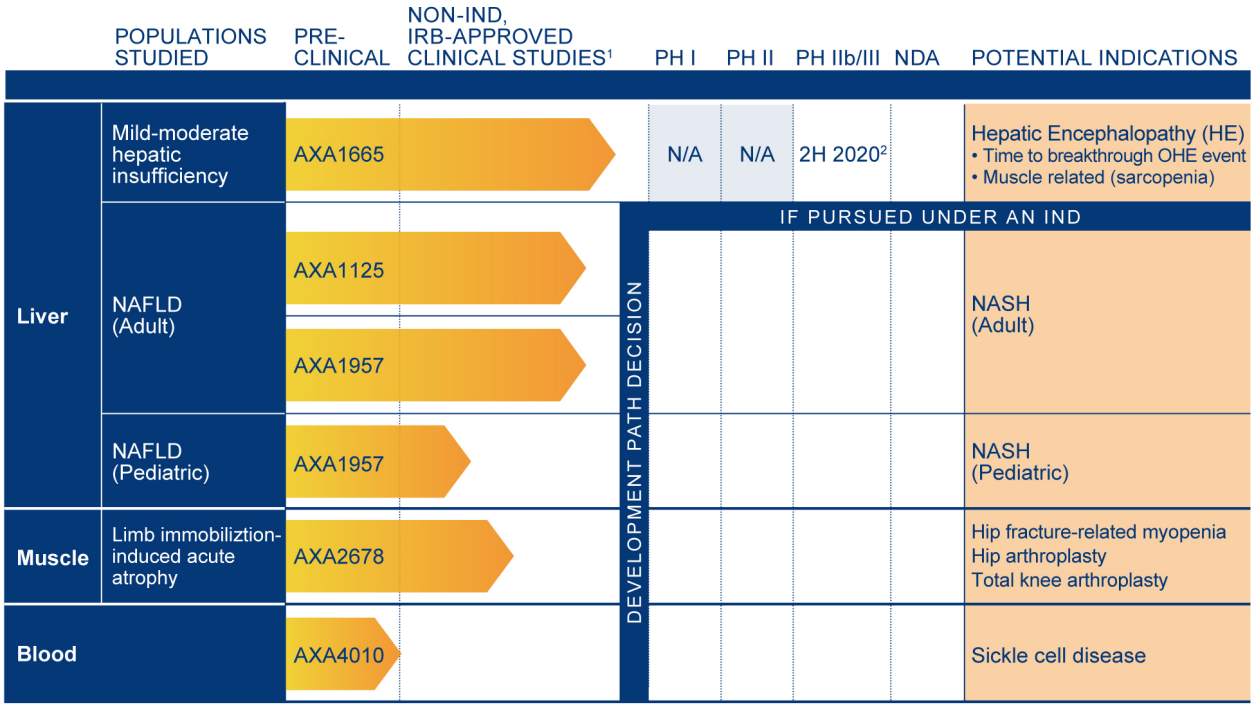
Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion of the financial condition and results of operations of Axcella Health Inc. should be read in conjunction with the condensed consolidated financial statements and the related notes thereto included elsewhere in this Quarterly Report, and the audited financial statements and notes included in our Prospectus filed pursuant to Rule 424(b) under the Securities Act, with the SEC, on May 9, 2019. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. We caution you that forward-looking statements are not guarantees of future performance, and that our actual results of operations, financial condition and liquidity, and the developments in our business and the industry in which we operate, may differ materially from the results discussed or projected in the forward-looking statements contained in this Quarterly Report. We discuss risks and other factors that we believe could cause or contribute to these potential differences elsewhere in this Quarterly Report, including under Item 1A. "Risk Factors" and under "Special Note Regarding Forward-Looking Statements." In addition, even if our results of operations, financial condition and liquidity, and the developments in our business and the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report, they may not be predictive of results or developments in future periods. We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

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.



(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.

On May 13, 2019, we completed our IPO, of 3,571,428 shares of our common stock at a public offering price of $20.00 per share. The gross proceeds from the IPO were $71.4 million and the net proceeds were $64.5 million, after deducting underwriting discounts and commissions and other estimated offering expenses.

To date, we have funded our operations with proceeds from the sale of redeemable convertible preferred stock and borrowing of debt. Through March 31, 2019, we had received gross proceeds of $197.8 million from sales of our redeemable convertible 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. For the three months ended March 31, 2019, our net loss was $11.6 million. As of March 31, 2019, we had an accumulated deficit of $168.6 million. 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 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;
- complete Non-IND, IRB-Approved Clinical Studies for AXA Candidates for which we have not yet made a drug development path decision or for which we have elected to develop as a non-drug product candidate;

- complete Clinical Trials for any AXA Candidates for which we make a drug development path decision, as we have done with AXA1665, and seek regulatory approvals for any drug product candidate that successfully completes Clinical Trials;
- 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;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval or identify an alternate regulatory pathway to market;
- 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.

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, 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 even terminate our operations.

As of March 31, 2019, our cash and cash equivalents totaled $66.8 million. We expect that our existing cash and cash equivalents together with the net proceeds from the IPO, will enable us to fund our operating expenses, capital expenditure requirements and debt service payments through the second quarter of 2021. We have based these estimates on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. 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 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 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 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;
- 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;
- the progress of the development efforts of parties with whom we may enter into collaboration arrangements;
- 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 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;
- the acceptance of our AXA Candidates, if approved, by patients, the medical community, third-party payors or other parties, such as consumers;
- obtaining and maintaining third-party insurance coverage and adequate reimbursement;
- 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 products 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 expect research and development expenses to increase as compared to 2018 as we develop new AXA Candidates and advance existing AXA Candidates. 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, intellectual property, corporate development, 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 as we invest the net proceeds from our IPO.

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 our IPO, the preferred stock warrants became exercisable for common stock instead of preferred stock, and the fair value of the warrant liability at that time was reclassified to additional paid-in capital. As a result, 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, or 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, or NOLs, carryforwards and Credits will not be realized.

Results of Operations

Comparison of the Three Months Ended March 31, 2019 and 2018

The following table summarizes our results of operations for the three months ended March 31, 2019 and 2018:

	Three Months Ended March 31,		
	2019	2018	Change
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	7,563	5,455	2,108
General and administrative	3,468	2,136	1,332
Total operating expenses	11,031	7,591	3,440
Loss from operations	(11,031)	(7,591)	(3,440)
Other income (expense):			
Change in fair value of preferred stock warrant liability	(51)	37	(88)
Interest income (expense), net	(491)	(546)	55
Total other expense, net	(542)	(509)	(33)
Net loss	$ (11,573)	$ (8,100)	$ (3,473)

Research and Development

	Three Months Ended March 31,		
	2019	2018	Change
		(in thousands)	
Direct research and development expenses by program:			
AXA1665	$ 1,062	$ 259	$ 803
AXA1125/1957	1,321	770	551
AXA2678	—	188	(188)
Platform development, early-stage research and unallocated expenses:			
Personnel related	2,644	1,990	654
Stock-based compensation expense	482	211	271
External manufacturing and research	816	762	54
Laboratory supplies and research materials	247	241	6
Facility related and other	991	1,034	(43)
Total research and development expenses	$ 7,563	$ 5,455	$ 2,108

Research and development expenses were $7.6 million for the three months ended March 31, 2019, compared to $5.5 million for the three months ended March 31, 2018. The increase in direct costs related to our AXA1665 program of $0.8 million and to our AXA1125 and AXA1957 program of $0.6 million was primarily due to costs incurred with external CROs and CMOs for the ongoing Non-IND, IRB-Approved Clinical Studies. The increase in personnel related expense of $0.7 million and Stock-based compensation expense of $0.3 million was primarily due to an increase in personnel to support the growth in research, development and clinical programs.

General and Administrative

	Three Months Ended March 31,		
	2019	2018	Change
		(in thousands)	
Personnel related	$ 1,750	$ 1,131	$ 619
Stock-based compensation expense	655	157	498
Professional and consultant fees	900	740	160
Facility related and other	163	108	55
Total general and administrative expenses	$ 3,468	$ 2,136	$ 1,332

General and administrative expenses were $3.5 million for the three months ended March 31, 2019, compared to $2.1 million for the three months ended March 31, 2018. The increase in general and administrative expenses of $1.3 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 $0.5 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 $0.6 million primarily due to the hiring of additional personnel in our general and administrative functions as we continued to expand our operations and to support operating as a public company. Professional and consultant fees increased by $0.2 million primarily due to increases in legal fees related to business development, regulatory and intellectual property costs, accounting and audit fees and public and investor relations fees.

Interest Income (Expense), Net

The decrease in net interest expense during the three months ended March 31, 2019 as compared to the three months ended March 31, 2018 was attributable to increased income earned on our invested cash, which was driven by a larger cash balance during the three months ended March 31, 2019 as compared to the three months ended March 31, 2018.

Liquidity and Capital Resources

Sources of Liquidity

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 any AXA Candidates for several years, if at all. To date, we have funded our operations with proceeds from the sales of redeemable convertible preferred stock and borrowings under our loan and security agreement. Through March 31, 2019, we had received gross proceeds of $197.8 million from sales of our redeemable convertible preferred stock and $26.0 million from borrowings under a loan and security agreement. As of March 31, 2019, we had cash and cash equivalents of $66.8 million.

Cash Flows

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

	Three Months Ended March 31,	
	2019	2018
Cash used in operating activities	$ (12,197)	$ (8,125)
Cash used in investing activities	(47)	(288)
Cash (used in) provided by financing activities	(453)	156
Net increase (decrease) in cash and cash equivalents	$ (12,697)	$ (8,257)

Net Cash Used in Operating Activities

Net cash used in operating activities for the three months ended March 31, 2019 was $12.2 million, which was primarily due to our net loss of $11.6 million, a decrease in prepaid expense of $0.8 million and a decrease in accrued expense of $2.2 million. This was partially offset by non-cash charges, including stock-based compensation expense of $1.1 million, depreciation expense of $0.2 million and an increase in accounts payable of $0.9 million.

Net cash used in operating activities for the three months ended March 31, 2018 was $8.1 million, which was primarily due to our net loss of $8.1 million and a decrease in accrued expenses of $1.2 million. This was partially offset by non-cash charges, including stock-based compensation expense of $0.4 million, depreciation of $0.4 million and an increase in accounts payable of $0.3 million.

Net Cash Used in Investing Activities

Net cash used in investing activities was $0.1 million for the three months ended March 31, 2019 compared to net cash used in investing activities of $0.3 million for the three months ended March 31, 2018. Net cash used in investing activities for the three months ended March 31, 2019 and 2018 consisted of the purchase of capital equipment used during the period.

Net Cash (Used in) Provided by Financing Activities

Net cash used in financing activities for the three months ended March 31, 2019 was $0.5 million, which primarily consisted of payments of deferred offering costs.

Net cash provided by financing activities for the three months ended March 31, 2018 was $0.2 million, which consisted of the net proceeds from the additional draw down from our debt facility.

Loan and security agreement

At March 31, 2019, we had $26.0 million in outstanding long-term debt. In October 2018, we entered into 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 provided 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.

Upon completion of the IPO in May 2019, the interest only period was extended through January 2021 and the Maturity Date was extended to January 2023. Monthly principal payments of $1.1 million are to commence February 2021 for 24 months. The $1.2 million success fee was also paid.

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, if applicable, for our AXA Candidates in development. In addition, we expect to incur additional costs associated with 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;
- 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;
- the timing and outcome of regulatory review of our AXA Candidates;
- 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 and maintain 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 terms and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder;

- 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 costs involved in prosecuting patent applications and enforcing patent claims and other intellectual property claims;
- additions or departures of key scientific or management personnel; and
- unanticipated serious safety concerns related to the use of our AXA Candidates.

We believe that the net proceeds from our IPO, 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.

Critical Accounting Policies and Use of Estimates

Our management's discussion and analysis is based on our condensed consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these condensed consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts and experience. The effects of material revisions in estimates, if any, will be reflected in the condensed consolidated financial statements prospectively from the date of change in estimates.

Our critical accounting policies are described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations-Critical Accounting Policies and Use of Estimates" in our Prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on May 9, 2019. During the three months ended March 31, 2019, there were no material changes to our critical accounting policies from those previously disclosed.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not have, any off-balance sheet arrangements, as defined under applicable SEC rules.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our condensed consolidated financial statements appearing elsewhere in this Quarterly Report.

Contractual Obligations

The following table summarizes our contractual obligations as of March 31, 2019 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]	$ 2,537	$ 896	$ 1,641	$ —	$ —
Debt obligations [2]	32,788	2,144	22,783	7,861	—
Total	$ 35,325	$ 3,040	$ 24,424	$ 7,861	$ —

(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 the Amended 2018 Facility. For purposes of this table, the interest due under the Amended 2018 Facility was calculated using an assumed interest rate of 10.99% (LIBOR plus 8.50%) per annum, which was the interest rate in effect as of March 31, 2019.

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.

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. 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 our IPO; (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 March 31st; and (2) the date on which we issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Item 3. Quantitative and Qualitative Disclosure About Market Risk

As of March 31, 2019, we had cash and, cash equivalents of $66.8 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 impact on the fair market value of our investment portfolio.

As of March 31, 2019, 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 three months ended March 31, 2019 and 2018.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rule 13a-15(e) of the Securities Exchange Act of 1934, as amended, or the Exchange Act) as of the end of the period covered by this report. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures as of the end of the period covered by this report were effective at a reasonable assurance level in ensuring that information required to be disclosed by us in reports that we file or submit under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms; and (ii) accumulated and communicated to management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely discussions regarding required disclosure. We believe that a control system, no matter how well designed and operated, cannot provide absolute assurance that the objectives of the control system are met, and no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within a company have been detected.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting (as defined in Rule 13a-15(f) of the Exchange Act) that occurred during the period covered by this report that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Part II. Other Information

Item 1. Legal Proceedings

From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any material legal proceedings. The outcome of litigation cannot be predicted with certainty and some lawsuits, claims or proceedings may be disposed of unfavorably to us, which could materially affect our financial condition or results of operations.

Item 1A. Risk Factors

Careful consideration should be given to the following risk factors, in addition to the other information set forth in this Quarterly Report and in other documents that we file with the SEC, in evaluating the Company and our business. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and are not the only risks facing the Company. New risk factors can emerge from time to time, and it is not possible to predict the impact that any factor or combination of factors may have on our business, prospects, financial condition and results of operations.

Risks related to our financial position and capital needs

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. Our net loss was $11.6 million for the three months ended March 31, 2019 and $36.1 million for the year ended December 31, 2018, respectively. As of March 31, 2019, we had an accumulated deficit of $168.6 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 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.

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 March 31, 2019, we had $66.8 million of cash and cash equivalents on hand. Based on our current operating plan, we believe that the net proceeds from our IPO, 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 FDA, any other regulatory authorities in the United States, and, when applicable, comparable foreign regulatory authorities, such as 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.

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 a new drug application, or 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 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.

Risks related to our business, technology and industry

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 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 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.

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.

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 March 31, 2019, we had 52 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 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 NOLs to 80% of current year taxable income and elimination of NOL carrybacks, in each case, for losses generated after December 31, 2018 (though any such NOLs may be carried forward indefinitely), and modification or repeal of many business deductions and Credits, including reduction of 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 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 our IPO, 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. 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 and may not be carried back.

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 March 31, 2019, we had cash and cash equivalents of $66.8 million. While we are not aware of any downgrades, material losses or other significant deterioration in the fair value of our cash equivalents since March 31, 2019, 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.

We adopted 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 FTC 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 TCJA, 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 for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 legislative session 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. 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. On May 10, 2019, CMS announced a new pricing transparency rule, which goes into effect on July 9, 2019. This final rule requires direct-to-consumer television advertisements for prescription drugs and biological products for which reimbursement is available, directly or indirectly, through or under Medicare or Medicaid to include the list price of that product, except for a prescription drug or biological product that has a list price of less than $35 per month for a 30-day supply or typical course of treatment. The pricing transparency rule could have a negative effect on our business. 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 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, 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 10Q 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

An active trading market for our common stock may not be sustainable, and you may not be able to resell your shares at or above the purchase price.

In May 2019, we closed our initial public offering. Prior to that offering, there was no public market for our common stock. Although we have completed our initial public offering and shares of our common stock are listed on The Nasdaq Global Market, an active trading market for our shares may not be sustained. 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. As a result of these and other factors, it may be difficult for our stockholders to resell their shares of our common stock at or above the prices at which they acquired their shares or sell their shares at the time they would like to sell. 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 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 Quarterly Report, 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 does not exceed the IPO 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 our IPO, based on shares of common stock outstanding as of March 31, 2019 on a pro forma basis, our current executive officers, directors and their affiliates and 5% stockholders held, 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 our IPO, these stockholders 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.

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 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 completed our IPO, 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 our IPO; (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 March 31st; 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 are subject to the reporting requirements of the Exchange Act, as amended, which requires, 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 our IPO. 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.

Following the May 2019 closing of our IPO, we had outstanding 22,988,359 shares of common stock, of which 20,832,417 shares are subject to restrictions on transfer under 180-day lock-up arrangements with the underwriters of our IPO. These restrictions are due to expire on November 4, 2019, resulting in the majority of these shares becoming eligible for public sale on November 4, 2019 if they are registered under the Securities Act, or if they qualify for an exemption from registration under the Securities Act including under Rules 144 or 701. 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 arrangements and other legal restrictions on resale lapse, the trading price of our common stock could 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 became effective upon the closing of our IPO, 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 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, which were effective upon the closing of our IPO, 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.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

Set forth below is information regarding shares of equity securities sold, and options granted, by us during the three months ended March 31, 2019 that were not registered under the Securities Act.

Recent Sales of Unregistered Equity Securities

On May 13, 2019, upon the closing of our IPO all 26,831,246 shares of our then-outstanding redeemable convertible preferred stock automatically converted into 14,641,997 shares of common stock. The issuance of such common stock was exempt from the registration requirements of the Securities Act, pursuant to Section 3(a)(9) of the Securities Act, involving an exchange of securities exchanged by the issuer with its existing security holders exclusively where no commission or other remuneration is paid or given directly or indirectly for soliciting such exchange. No underwriters were involved in this issuance of shares.

During the period between January 1, 2019 and March 31, 2019, we issued to certain of our employees and advisors, options to purchase an aggregate of 542,969 shares of our common stock at an exercise price of $13.83 per share. We deemed these issuances to be exempt from registration under the Securities Act either in reliance on Rule 701 of the Securities Act as sales and offers under compensatory benefit.

Use of Proceeds from IPO of Common Stock

On May 13, 2019, we completed the IPO of our common stock pursuant to which we issued and sold 3,571,428 shares of our common stock at a price to the public of $20.00 per share.

The offer and sale of all of the shares of our common stock in our IPO were registered under the Securities Act pursuant to a registration statement on Form S-1, as amended (File No. 333- 230822), which was declared effective by the SEC on May 8, 2019. Goldman Sachs & Co. LLC, J.P. Morgan Securities LLC and SVB Leerink LLC acted as joint book-running managers of our IPO.

We received aggregate gross proceeds from our IPO of $71.4 million, or aggregate net proceeds of $64.5 million after deducting underwriting discounts and commissions and other offering costs. None of the underwriting discounts and commissions or offering expenses were incurred or paid, directly or indirectly, to any of our directors or officers or their associates or to persons owning 10% or more of our common stock or to any of our affiliates.

There has been no material change in our planned use of the net proceeds from the IPO as described in our Prospectus dated May 8, 2019.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

Not applicable.

Item 6. Exhibits

The exhibits listed on the Exhibit Index immediately preceding such exhibits, which is incorporated herein by reference, are filed or furnished as part of this Quarterly Report.

Exhibit No.	Exhibit Index
3.1	Restated Certificate of Incorporation of the Registrant (Incorporated by reference to Exhibit 3.3 to the Registrant's Current Report on Form 8-K (File No. 001-38901) filed with the Securities and Exchange Commission on May 13, 2019).
3.2	Amended and Restated Bylaws of Registrant (Incorporated by reference to Exhibit 3.4 to the Registrant's Current Report on Form 8-K (File No. 001-38901) filed with the Securities and Exchange Commission on May 13, 2019).
4.1	Specimen Stock Certificate evidencing shares of common stock (Incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1/A (File No. 333-230822) filed with the Securities and Exchange Commission on April 30, 2019).
4.2	Fifth Amended and Restated Investors' Rights Agreement among the Registrant and certain of its stockholders, dated November 30, 2018 (Incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1 (File No. 333-230822) filed with the Securities and Exchange Commission on April 12, 2019).
10.1#	2019 Stock Option and Incentive Plan and forms of award agreements thereunder (Incorporated by reference to Exhibit 10.2 to the Registrant's Registration Statement on Form S-1/A (File No. 333-230822) filed with the Securities and Exchange Commission on May 6, 2019).
10.2#	2019 Employee Stock Purchase Plan (Incorporated by reference to Exhibit 10.3 to the Registrant's Registration Statement on Form S-1/A (File No. 333-230822) filed with the Securities and Exchange Commission on April 30, 2019).
10.3#	Senior Executive Cash Incentive Bonus Plan (Incorporated by reference to Exhibit 10.4 to the Registrant's Registration Statement on Form S-1/A (File No. 333-230822) filed with the Securities and Exchange Commission on April 30, 2019).
10.4#	Amended and Restated Employment Agreement between the Registrant and William Hinshaw, dated December 20, 2018 (Incorporated by reference to Exhibit 10.5 to the Registrant's Registration Statement on Form S-1/A (File No. 333-230822) filed with the Securities and Exchange Commission on May 6, 2019).
10.5#	Amended and Restated Employment Agreement between the Registrant and Thomas Leggett, dated December 31, 2018 (Incorporated by reference to Exhibit 10.6 to the Registrant's Registration Statement on Form S-1/A (File No. 333-230822) filed with the Securities and Exchange Commission on May 6, 2019).
10.6#	Amended and Restated Employment Agreement between the Registrant and Stephen Mitchener, dated December 29, 2018 (Incorporated by reference to Exhibit 10.7 to the Registrant's Registration Statement on Form S-1/A (File No. 333-230822) filed with the Securities and Exchange Commission on May 6, 2019).
31.1*	Certification of Principal Executive Officer Pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer Pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101INS	XBRL Instance Document.
101SCH	XBRL Taxonomy Extension Schema Document.
101CAL	XBRL Taxonomy Extension Calculation Linkbase Document.
101LAB	XBRL Taxonomy Extension Labels Linkbase Document.
101PRE	XBRL Taxonomy Extension Presentation Linkbase Document.
101DEF	XBRL Taxonomy Extension Definition Linkbase Document.

#	Indicates a management contract or any compensatory plan, contract or arrangement.
*	Filed herewith.
**	The certification furnished in Exhibit 32.1 hereto is deemed to accompany this Form 10-Q and will not be deemed "filed" for purposes of Section 18 of the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

AXCELLA HEALTH INC.

Date: June 20, 2019

By: /s/ William R. Hinshaw, Jr.

William R. Hinshaw, Jr.
President, Chief Executive Officer and Director

AXCELLA HEALTH INC.

Date: June 20, 2019

By: /s/ Thomas Leggett

Thomas Leggett
Senior Vice President of Finance, Chief Financial Officer

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO RULE 13a-14(a) OR 15d-14(a)
OF THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF
THE SARBANES-OXLEY ACT OF 2002

I, William R. Hinshaw, Jr., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended March 31, 2019 of Axcella Health Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
    b. (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: June 20, 2019

By: /s/ William R. Hinshaw, Jr.

William R. Hinshaw, Jr.
President, Chief Executive Officer
and Director
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO RULE 13a-14(a) OR 15d-14(a)
OF THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF
THE SARBANES-OXLEY ACT OF 2002

I, Thomas Leggett, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended March 31, 2019 of Axcella Health Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
    b. (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: June 20, 2019

By: /s/ Thomas Leggett

Thomas Leggett
Senior Vice President of Finance, Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER AND CHIEF FINANCIAL OFFICER
PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Axcella Health Inc. (the "Company") for the quarterly period ended March 31, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned, William R. Hinshaw, Jr., Chief Executive Officer, President and Director of the Company, and Thomas Leggett, Senior Vice President of Finance and Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: June 20, 2019 By: /s/ William R. Hinshaw, Jr.

William R. Hinshaw, Jr.
President, Chief Executive Officer
and Director
(Principal Executive Officer)

Date: June 20, 2019 By: /s/ Thomas Leggett

Thomas Leggett
Senior Vice President of Finance, Chief Financial Officer
(Principal Financial Officer)