Axcella Health Reports Second Quarter 2019 Financial Results and Provides Company Update

August 12, 2019

Initiated a Non-IND, IRB-Approved Clinical Study for AXA1957 in adolescent subjects with NAFLD

Announced the publication in Frontiers of data describing attenuation of muscle atrophy observed with AXA2678 in short-term muscle disuse study

Finished the second quarter with $117.9 million in cash and cash equivalents, which provides cash runway through the middle of 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 12, 2019-- Axcella Health Inc. (Nasdaq: AXLA) ("Axcella" or the "Company"), a biotechnology company pioneering the research and development of novel multifactorial interventions to address dysregulated metabolism and support health, today announced financial results for the second quarter ended June 30, 2019 and provided a company update.

"Our Company continued to make headway in the clinic with the initiation of our first study in adolescents. This Non-IND, IRB-Approved Clinical Study is being conducted in subjects with Non-Alcoholic Fatty Liver Disease or NAFLD. NAFLD is driven by metabolic dysregulation which is increasing in incidence. We believe our unique development model and ability to generate human data prior making a development path decision for our AXA Candidates provides the potential to make rapid and well-informed product development decisions. Our ongoing clinical programs, including AXA1665 for Hepatic Encephalopathy and AXA1125/AXA1957 in liver are progressing well and we look forward to completing enrollment and anticipate reporting additional human data next year," said Bill Hinshaw, President and Chief Executive Officer of Axcella. "We completed the second quarter with $117.9 million in cash and cash equivalents which we believe positions us well to continue developing our current pipeline while expanding into new potential areas."

Corporate Highlights

- In July 2019, Axcella initiated a Non-IND, IRB-Approved Clinical Study for AXA1957 in adolescent subjects with NAFLD
- In July 2019, Axcella announced the publication of data describing attenuation of muscle atrophy observed with AXA2678 in short-term muscle disuse study
- In May 2019, Axcella completed its initial public offering, raising gross proceeds of $71.4 million through the sale of 3,571,428 shares of common stock at an initial public offering price of $20.00 per share and commenced trading on The Nasdaq Global Market under the ticker symbol AXLA

Anticipated Milestones

- Initiate a Non-IND, IRB-Approved Clinical Study of AXA4010 in subjects with sickle cell disease in the second half of 2019
- Report data from our Non-IND, IRB-Approved Clinical Study of AXA1665 in subjects with hepatic insufficiency in the first half of 2020
- Report data from our Non-IND, IRB-Approved Clinical Study of AXA1125 and AXA1957 in adult subjects with NAFLD in the second half of 2020
- Report data from our Non-IND, IRB-Approved Clinical Study of AXA1957 in adolescent subjects with NAFLD in the second half of 2020
- Report data from our Non-IND, IRB-Approved Clinical Study of AXA4010 in subjects with sickle cell disease in the second half of 2020

Second Quarter Financial Results
For the second quarter ended June 30, 2019, Axcella reported a net loss of approximately $14.4 million, or $0.95 per share, basic and diluted, compared to a net loss for the quarter ended June 30, 2018 of $9.4 million, or $2.15 per share, basic and diluted.

Research and development expenses for the quarter ended June 30, 2019 were $9.3 million, compared to $6.0 million for the quarter ended June 30, 2018. The increase in expense for the quarter was driven by an increase in costs related to the conduct of Non-IND, IRB-Approved Clinical Studies and other expenses associated with the development of AXA candidates in 2019.

General and administrative expenses were $4.7 million for the quarter ended June 30, 2019, compared to $2.9 million for the quarter ended June 30, 2018. The increase in general and administrative expenses for the year was driven by increased professional services and employee-related costs as the Company continues to increase headcount and expand infrastructure to support its growth.

Cash and cash equivalents were $117.9 million as of June 30, 2019.

Six Month Financial Results
Net loss for the first six months of 2019 was $26.0 million, or $2.60 per share, basic and diluted, compared to a net loss of $17.5 million, or $4.08 per share, basic and diluted, for the first six months of 2018. In the six months ended June 30, 2019, Axcella invested $16.9 million in research and development expenses related the conduct of Non-IND, IRB-Approved Clinical Studies and other expenses associated with the development of AXA

candidates in 2019, compared to $11.5 million in the first six months of 2018. General and administrative expenses were $8.2 million during the first six months of 2019, as compared to $5.0 million for the same period in 2018.

Cash Flows used in operating activities for the six months ended June 30, 2019 were $25.4 million.

About Axcella Health
Axcella is designing and developing AXA Candidates, compositions of endogenous metabolic modulators, or EMMs, engineered in distinct ratios, designed to target and maximize the fundamental role that EMMs play in regulating multiple metabolic functions. Axcella's AXA Candidates are generated from its proprietary, human-focused AXA Development Platform. Axcella believes its expertise and capabilities in EMMs position it to become a preeminent biotechnology company reprogramming metabolism to address a diverse set of complex diseases and support health. Axcella's AXA Development Platform has already produced a pipeline of product candidates in programs targeting liver, muscle and blood. Axcella was founded by Flagship Pioneering.

About Non-IND, IRB-Approved Clinical Studies
Axcella conducts non-investigational new drug application (Non-IND), Institutional Review Board (IRB)-approved clinical studies in humans with its AXA Candidates under U.S. Food and Drug Administration regulations and guidance supporting research with food outside of an IND. In these studies, Axcella evaluates in humans, including in individuals with disease, AXA Candidates for safety, tolerability and effects on the normal structures and functions of the body. Non-IND, IRB-Approved Clinical Studies are not designed or intended to evaluate an AXA Candidate's ability to diagnose, cure, mitigate, treat or prevent a disease. If Axcella decides to further develop an AXA Candidate as a potential therapeutic, subsequent studies will be conducted under an IND.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding the development potential and development pathway of our current AXA Candidates, potential expansion into new therapeutic fields, the timing of our clinical studies and the timing of receipt of data from the same, our liquidity, including our expected cash runway, and our strategy, business plans and focus. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, those related to the breadth of our pipeline of product candidates, the strength of our proprietary product platform, the efficiency of our discovery and development approach, the clinical development and safety profile of our AXA Candidates and their health or therapeutic potential, whether and when, if at all, our AXA Candidates will become commercial products or, if applicable, receive approval from the U.S. Food and Drug Administration and for which, if any, indications, competition from other biotechnology and other health companies, our liquidity, our ability to successfully develop our AXA Candidates through current and future milestones on the anticipated timeline, if at all, past results from Non-IND, IRB-Approved Clinical Studies not being representative of future results, and other risks identified in our SEC filings, including our final prospectus for our initial public offering, and subsequent filings with the SEC. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in 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. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

Axcella Health Inc.

Condensed Consolidated Statements of Operations (Unaudited)

(in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	$ 9,343	$ 6,007	$ 16,906	$ 11,462
General and administrative	4,728	2,901	8,196	5,037
Total operating expenses	14,071	8,908	25,102	16,499
Loss from operations	(14,071)	(8,908)	(25,102)	(16,499)

Other income (expense):				
Change in fair value of preferred stock warrant liability	—	4	(51)	41
Interest income (expense), net	(376)	(519)	(867)	(1,065)
Total other income (expense), net	(376)	(515)	(918)	(1,024)
Net loss	$ (14,447)	$ (9,423)	$ (26,020)	$ (17,523)
Net loss per share, basic and diluted	$ (0.95)	$ (2.15)	$ (2.60)	$ (4.08)
Weighted average common shares outstanding, basic and diluted	15,230,815	4,405,597	10,032,202	4,317,845

Axcella Health Inc.

Condensed Consolidated Balance Sheet Data (Unaudited)

(in thousands)

	June 30, 2019	**December 31, 2018**
Assets:		
Cash and cash equivalents	$ 117,910	$ 79,466
Other assets	4,497	2,378
Total assets	**$ 122,407**	**$ 81,844**
Liabilities and stockholders' (deficit) equity		
Liabilities	$ 32,463	$ 33,755
Preferred stock	—	197,842
Stockholders' equity (deficit) equity	89,944	(149,753)
Total liabilities and stockholders' equity	**$ 122,407**	**$ 81,844**

View source version on businesswire.com: https://www.businesswire.com/news/home/20190812005606/en/

Source: Axcella Health Inc.

Investor Contact
Lauren Stival
ir@axcellahealth.com
(212) 698-8646

Company Contact

Alison Williams
awilliams@axcellahealth.com
(857) 320-2204