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

November 12, 2019

- *Completed enrollment of an ongoing non-IND clinical study of AXA1125/AXA1957 in more than 100 adult subjects with NAFLD*
- *Announced intent to conduct future studies of AXA1125/AXA1957 under IND as therapeutic candidates for NASH*
- *On track to file an IND and initiate a registrational trial of AXA1665 for OHE in 2020*
- *Initiated a non-IND clinical study of hematology candidate AXA4010*
- *Closed third quarter with $105 million in cash and cash equivalents, providing runway through the second quarter of 2021*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 12, 2019-- Axcella Health Inc. (Nasdaq: AXLA), 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 third quarter ended September 30, 2019 and provided a company update.

"We are excited by our team's execution and are pleased to report back on the great strides we have made across our programs in recent months," said Bill Hinshaw, President and Chief Executive Officer of Axcella. "During the third quarter, we continued enrolling patients in our ongoing non-IND clinical study of AXA1665 in subjects with mild and moderate hepatic insufficiency, achieved significant milestones related to our clinical development of AXA1125 and AXA1957 and also announced our intent to conduct any future studies of these latter product candidates in non-alcoholic steatohepatitis (NASH) under an IND. Additionally, we recently initiated a non-IND clinical study of our hematology product candidate AXA4010. With readouts from all four of our ongoing non-IND clinical studies and a first IND submission to the U.S. Food and Drug Administration planned for 2020, the year ahead is expected to be a particularly catalyst-rich period for Axcella."

Recent Highlights

Liver Programs

- Presented data from non-IND clinical studies at The Liver Meeting™ on the multifactorial effects of AXA1665 observed in subjects with mild and moderate hepatic insufficiency and AXA1125 in subjects with Non-Alcoholic Fatty Liver Disease (NAFLD)
- Completed enrollment of a non-IND clinical study to assess the impact of AXA1125/AXA1957 on safety, tolerability and physiological impact in more than 100 adult subjects with NAFLD
- Initiated enrollment of a non-IND clinical study to assess the impact of AXA1957 on safety, tolerability and physiology in approximately 30 adolescent subjects with NAFLD
- Declared the company's intent to conduct future studies of AXA1125/AXA1957 in patients with NASH under an IND

Blood Program

- Announced an upcoming presentation of mechanistic data on the company's hematology candidate AXA4010 at the 61st American Society of Hematology (ASH) Annual Meeting and Exposition
- Initiated a non-IND clinical study to assess the impact of AXA4010 on safety, tolerability and blood physiology in subjects with sickle cell disease

Muscle Program

- Announced a collaboration with CYTOO aimed at informing the development of new product candidates that promote skeletal muscle growth and function

Organization

- Appointed Catherine A. Sohn, Pharm.D. to replace Christopher A. Viehbacher on the company's Board of Directors

Anticipated 2020 Milestones

Liver Programs

- Early 2020: Complete enrollment in the company's non-IND clinical study to assess the impact of AXA1665 on safety, tolerability and physiology in subjects with mild and moderate hepatic insufficiency
- First Half of 2020: Report data from the aforementioned non-IND clinical study of AXA1665
- Mid 2020: Report data from the company's non-IND clinical study to assess the impact of AXA1125/AXA1957 on safety, tolerability and physiology in adult subjects with NAFLD
- Second Half of 2020: Submit an IND application to the U.S. Food and Drug Administration (FDA) to study AXA1665 in patients with overt hepatic encephalopathy (OHE)
- Second Half of 2020: Report data from the company's non-IND clinical study to assess the impact of AXA1957 on safety, tolerability and physiology in adolescent subjects with NAFLD

Blood Program

- First Half of 2020: Complete enrollment in the company's non-IND clinical study to assess the impact of AXA4010 on safety, tolerability and blood physiology in subjects with sickle cell disease
- Second Half of 2020: Report data from the aforementioned non-IND clinical study of AXA4010

Third Quarter Financial Results

For the third quarter ended September 30, 2019, Axcella reported:

- Research and development expenses were $12.2 million. This compares with $6.1 million for the third quarter ended September 30, 2018. The increase in R&D expenses was primarily related to greater costs related to the company's non-IND clinical studies and other expenses associated with the development of the company's product candidates.
- General and administrative expenses were $4.8 million. This compares with $2.1 million for the quarter ended September 30, 2018. The increase in G&A expenses was primarily related to higher professional services and employee-related costs associated with the company's infrastructure growth.
- A net loss of approximately $17.3 million, or $0.75 per basic and diluted share. This compares with a net loss of $8.8 million, or $1.85 per basic and diluted share, for the quarter ended September 30, 2018.
- Cash and cash equivalents at September 30, 2019 were $105.4 million, which compares with $79.5 million at December 31, 2018. The increase was the result of proceeds from the company's May 2019 initial public offering, partially offset by operating expenditures. Axcella continues to expect that its existing balance of cash and equivalents is sufficient to meet the company's operating needs through the second quarter of 2021.

About Non-IND 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.

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. For more information, visit www.axcellahealth.com.

Internet Posting of Information

Axcella uses its website, www.axcellahealth.com, as a means of disclosing material nonpublic information and for complying with its disclosure obligations under Regulation FD. Such disclosures will be included in the "Investors and News" section of the company's website. Accordingly, investors should monitor this portion of the website, in addition to following Axcella's press releases, SEC filings and public conference calls and webcasts.

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 September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	$ 12,157	$ 6,147	$ 29,063	$ 17,609
General and administrative	4,840	2,087	13,036	7,124
Total operating expenses	16,997	8,234	42,099	24,733
Loss from operations	(16,997)	(8,234)	(42,099)	(24,733)
Other income (expense):				
Change in fair value of preferred stock warrant liability	—	5	(51)	46
Interest income (expense), net	(307)	(555)	(1,174)	(1,620)
Total other income (expense), net	(307)	(550)	(1,225)	(1,574)
Net loss	$ (17,304)	$ (8,784)	$ (43,324)	$ (26,307)
Net loss per share, basic and diluted	$ (0.75)	$ (1.85)	$ (3.01)	$ (5.92)
Weighted average common shares outstanding, basic and diluted	23,083,367	4,769,387	14,430,397	4,470,013

Axcella Health Inc.

Condensed Consolidated Balance Sheet Data (Unaudited)

(in thousands)

	September 30, 2019	December 31, 2018
Assets:		
Cash and cash equivalents	$ 105,355	$ 79,466
Other assets	3,832	2,378
Total assets	**$ 109,187**	**$ 81,844**
Liabilities and stockholders' (deficit) equity		
Liabilities	$ 34,843	$ 33,755
Preferred stock	—	197,842
Stockholders' equity (deficit) equity	74,344	(149,753)
Total liabilities and stockholders' equity	**$ 109,187**	**$ 81,844**

View source version on businesswire.com: https://www.businesswire.com/news/home/20191112005946/en/

Source: Axcella Health

Company/Investor Contact
Jason Fredette
jfredette@axcellahealth.com
(857) 320-2236

Media Contact
Azeem Zeekrya
azeem.zeekrya@hdmz.com
(312) 506-5244