Axcella Reports First Quarter Financial Results and Provides Business Update

May 11, 2020

- *Reported positive top-line data from AXA1125-003, with clinically relevant reductions in liver fat content, insulin resistance and fibroinflammation observed in subjects with NAFLD receiving AXA1125*
- *Completed enrollment of AXA1665-002, with data readout expected in Q3 2020*
- *Announced AXA2678 patent issuance and oral presentation at ICSFR*
- *Launched new corporate website at www.axcellahealth.com*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 11, 2020-- Axcella (Nasdaq: AXLA), a clinical-stage biotechnology company focused on leveraging endogenous metabolic modulators (EMMs) to pioneer a new approach for treating complex diseases and improving health, today announced financial results for the first quarter ended March 31, 2020 and provided a business update.

“These past few months have been a particularly exciting period for Axcella, culminating in our recent report of positive top-line data for AXA1125, our NASH product candidate, that we believe further validates our platform,” said Bill Hinshaw, President and Chief Executive Officer of Axcella. “In addition to these recent data, we also completed enrollment in AXA1665-002 in the first quarter and plan to provide a data readout from that ongoing clinical study in the third quarter of 2020 despite the COVID-19 pandemic. With this and other milestones expected in the quarters ahead, including expected regulatory engagements regarding our lead product candidates, 2020 has the potential to be a transformational year for Axcella.”

Recent Highlights

Liver Programs

- Reported positive top-line data from AXA1125-003, a clinical study assessing the impact of AXA1125 and AXA1957 on safety, tolerability and physiology in subjects with non-alcoholic fatty liver disease (NAFLD). Results from the study showed that AXA1125 and AXA1957 were generally well-tolerated, with sustained reductions noted for both product candidates versus placebo in key biomarkers of metabolism, inflammation and fibrosis over 16 weeks. Overall, as compared to both placebo and AXA1957, AXA1125 demonstrated larger and more consistent reductions in clinically relevant biomarkers.
- Completed enrollment of AXA1665-002, an ongoing clinical study assessing the impact of AXA1665 on safety, tolerability and physiology in adult subjects with mild and moderate hepatic insufficiency. AXA1665 has been generally safe and well tolerated in this study to date. The company expects to report data from this study in the third quarter of 2020.

Muscle Program

- Discussed Axcella’s AXA2678 muscle product candidate during an oral presentation at the 2020 International Conference on Frailty and Sarcopenia Research (ICFSR) in March 2020.
- Announced the issuance of U.S. Patent 10,596,136, which broadly covers the use of AXA2678 and related EMM compositions for the reduction of fat infiltration in muscle.

Corporate

- Launched a new corporate website at www.axcellahealth.com that includes additional detail about the company’s platform, product candidates and culture.

Anticipated 2020 Milestones

Liver Programs

- Q3 2020: Report data from AXA1665-002 in adult subjects with mild and moderate hepatic insufficiency.
- Q4 2020: Submit an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) and initiate a potential Phase 2b/3 registrational clinical trial of AXA1665 for the reduction in risk of OHE recurrence.
- 2H 2020: Engage with the FDA to discuss the company’s planned IND application for AXA1125, proposed Phase 2b clinical trial in adults and pediatric development program.

Blood Program

- Q4 2020: Report top-line data from Cohort 1 of AXA4010-001, a clinical study on safety, tolerability and blood physiology in subjects with sickle cell disease.

Financial Results

R&D Expenses: Research and development expenses were $10.3 million and $7.6 million for the quarters ended March 31, 2020 and 2019, respectively. The change was primarily related to greater costs associated with the advancement of the company's product candidates and ongoing clinical studies.

G&A Expenses: General and administrative expenses were $4.1 million and $3.5 million for the quarters ended March 31, 2020 and 2019, respectively. The change was primarily related to higher professional services and employee-related costs associated with being a public company.

Net Loss: Net loss for the quarter ended March 31, 2020 was $15.0 million, or $0.65 per basic and diluted share. This compares with a net loss of $11.6 million, or $2.43 per basic and diluted share, for the quarter ended March 31, 2019.

Cash Position: Cash and cash equivalents at March 31, 2020 were $75.5 million, which compares with $92.1 million at December 31, 2019. Axcella expects that its cash and cash equivalents will be sufficient to meet the company's operating needs into the second quarter of 2021.

About Endogenous Metabolic Modulators (EMMs)
EMMs are a broad family of molecules, including amino acids, that regulate human metabolism. Axcella is developing a range of novel product candidates that are comprised of multiple EMMs engineered in distinct combinations and ratios to simultaneously impact multiple metabolic pathways to modify the root causes of various complex diseases and improve health.

About Axcella's Clinical Studies
Each of the company's ongoing clinical studies are being conducted as non-investigational new drug (IND) application clinical studies under U.S. Food and Drug Administration regulations and guidance supporting research with food. These studies evaluate product candidates for safety, tolerability and effects on the normal structures and functions in humans, including in individuals with disease. They are not designed or intended to evaluate a product candidate's ability to diagnose, cure, mitigate, treat or prevent a disease. If Axcella decides to further develop a product candidate as a potential therapeutic, as is the case with AXA1665 and AXA1125, subsequent studies will be conducted under an IND.

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 on the company's website in the "Investors and News" section. Accordingly, investors should monitor such portions of the company's website, in addition to following its press releases, SEC filings and public conference calls and webcasts.

About Axcella
Axcella is a clinical-stage biotechnology company focused on leveraging endogenous metabolic modulators (EMMs) to pioneer a new approach for treating complex diseases and improving health. The company's product candidates are comprised of EMMs and their derivatives that are engineered in distinct combinations and ratios to simultaneously impact multiple biological pathways. Axcella's pipeline includes lead therapeutic candidates for non-alcoholic steatohepatitis (NASH) and the reduction in risk of overt hepatic encephalopathy (OHE) recurrence. Additional muscle- and blood-related programs are in earlier-stage development. For more information, please visit www.axcellahealth.com.

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 characteristics, competitive position and development potential of the company's EMM product candidates, including for AXA1125 and AXA1665, the design, status and timing of the company's ongoing clinical studies and planned IND-enabled clinical trials, the company's anticipated program milestones, including the timing of AXA1665-002 and AXA4010-001 data readouts, the subject and timing of the company's planned interactions with the FDA on the AXA1665 and AXA1125 programs including potential timing of IND application submissions, and the potential of the company's product candidates to impact health and/or disease, including AXA1125's potential in NASH and AXA1665 potential in OHE. 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 potential impact of COVID-19 on the company's ability to conduct and complete its ongoing or planned clinical studies and IND enabled clinical trials and planned interactions and submissions to FDA or other regulatory authorities in a timely manner or at all due to patient or principal investigator recruitment or availability challenges, clinical trial site shutdowns or other interruptions and potential limitations on the quality, completeness and interpretability of data we are able to collect in our ongoing AXA1665-002 and AXA4010-001 clinical studies and potential delays in disclosure of the same, other potential impacts of COVID-19 on our business and financial results, including with respect to our ability to raise additional capital and operational disruptions or delays, changes in law, regulations, or interpretations and enforcement of regulatory guidance, whether data readouts and/or FDA feedback support our IND submission and clinical trial initiation plans and timing, clinical trial design and target indication for AXA1125 and AXA1665, the clinical development and safety profile of the company's product candidates and their health or therapeutic potential, whether and when, if at all, the company's product candidates will receive approval from the FDA or other comparable regulatory authorities, and for which, if any, indications, competition from other biotechnology companies, past results from clinical studies not being representative of future results, and other risks identified in the company's SEC filings, including Axcella's Annual Report on Form 10-K, Quarterly Report on Form 10-Q and subsequent filings with the SEC. The company cautions you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. Axcella disclaims 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 the company's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. The company explicitly disclaims any obligation to update any forward-looking statements.

Axcella Health Inc.
Unaudited Condensed Consolidated Balance Sheets
(in thousands)

	March 31, 2020	March 31, 2019
Assets:		
Cash and cash equivalents	$ 75,522	$ 66,769
Other assets	2,060	$ 4,149
Total assets	$ 77,582	$ 70,918
Liabilities and stockholders' equity (deficit):		
Liabilities	$ 30,768	$ 33,253
Preferred stock	—	$ 197,888
Stockholders' equity (deficit)	46,814	$(160,223)
Total liabilities and stockholders' equity	$ 77,582	$ 70,918

Axcella Health Inc.
Unaudited Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2020	2019
Operating expenses:		
Research and development	$ 10,335	$ 7,563
General and administrative	4,125	3,468
Total operating expenses	14,460	11,031
Loss from operations	(14,460)	(11,031)
Other income (expense), net	(549)	(542)
Net loss and comprehensive loss	$ (15,009)	$ (11,573)
Net loss per share, basic and diluted	$ (0.65)	$ (2.43)
Weighted average common shares outstanding, basic and diluted	23,188,816	4,775,828

View source version on businesswire.com: https://www.businesswire.com/news/home/20200511005106/en/

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

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

Source: Axcella Health Inc.