Axcella Reports First Quarter Financial Results and Provides Business Update

May 6, 2021

- *AXA1665 IND application cleared by FDA for the reduction in risk of recurrent OHE*
- *AXA1125 IND application cleared by FDA for the treatment of NASH*
- *Initiation of AXA1665 Phase 2 and AXA1125 Phase 2b clinical trials expected shortly*
- *Company to host conference call at 8:30 a.m. ET today*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 6, 2021-- Axcella (Nasdaq: AXLA), a clinical-stage biotechnology company pioneering a new approach to treat complex diseases and improve health using endogenous metabolic modulator (EMM) compositions, today announced financial results for the first quarter ended March 31, 2021 and provided a business update.

“Already in 2021, we have made tremendous progress in advancing our EMM platform and our mission to bring new multi-targeted therapies to patients with complex diseases and conditions,” said Bill Hinshaw, President and Chief Executive Officer of Axcella. “The recent clearance of our first two investigational new drug (IND) applications has extended our strong track record of execution. Additionally, these milestones validate how we can efficiently compile a significant amount of clinical data and then rapidly progress into later-stage development. We expect to continue our momentum as we get our later-stage clinical trials in overt hepatic encephalopathy (OHE) and nonalcoholic steatohepatitis (NASH) underway this quarter, ramp up enrollment leveraging a physician network that is excited by our approach and modality, and expand Axcella’s pipeline of EMM compositions.”

Recent Accomplishments and Next Steps

AXA1665 for the Reduction in Risk of Recurrent OHE

- **Cleared IND:** In January 2021, Axcella’s IND application was cleared by the U.S. Food and Drug Administration (FDA) for AXA1665, the company’s oral product candidate for OHE.
- **Readied for Phase 2 Clinical Trial:** During the first quarter of 2021, Axcella finalized plans for a 24-week Phase 2 clinical trial of AXA1665 that will enroll patients with a history of OHE. Initiation of this trial is expected in the near future.
- **Accepted for DDW 2021 Oral Presentation:** Data from Axcella’s AXA1665-002 clinical study were accepted for an oral presentation at the Digestive Disease Week (DDW) 2021 Annual Meeting, which will be held virtually from May 21 to 23, 2021.

AXA1125 for the Treatment of NASH

- **Cleared IND:** In April 2021, Axcella’s investigational new drug (IND) application was cleared by the FDA for AXA1125, the company’s oral product candidate for NASH.
- **Readied for Phase 2b Clinical Trial:** During the first quarter of 2021, Axcella finalized plans for a 48-week Phase 2b serial biopsy clinical trial of AXA1125 that will enroll adult patients with NASH, with primary and secondary endpoints based on liver histology. Initiation of this trial is expected in the near future.
- **Presented Data at NASH-TAG 2021:** In March 2021, multiple presentations regarding AXA1125 were included in NASH-TAG 2021, an event bringing clinicians and researchers together to focus on the diagnosis and treatment of NASH and liver fibrosis. Among them was a poster presentation providing novel insights regarding AXA1125’s multi-targeted mechanism of action and a AXA1125-003 clinical study data presentation that was recognized with a Distinguished Abstract and Poster Award.

Management Team

- **Added Dr. Alison Schecter as President of R&D:** In March 2021, Axcella announced the appointment of Alison D. Schecter, M.D., as the company’s President of Research and Development. In this role, Dr. Schecter oversees all of the company’s research, product candidate design, clinical and regulatory efforts. She brings more than 20 years of research, clinical and regulatory experience to Axcella at companies such as Sanofi-Genzyme, Johnson & Johnson, Selecta Biosciences, and Novartis.

Financial Results

Cash Position: As of March 31, 2021, cash, cash equivalents, and marketable securities totaled $93.0 million, compared to $107.3 million at December 31, 2020. Axcella expects that its cash balance will be sufficient to meet the company’s operating needs into the third quarter of 2022.

R&D Expenses: Research and development expenses were $10.2 million and $10.3 million for the quarters ended March 31, 2021 and 2020,

respectively.

G&A Expenses: General and administrative expenses were $4.3 million and $4.1 million for the quarters ended March 31, 2021 and 2020, respectively.

Net Loss: Net loss for the quarter ended March 31, 2021 was $15.2 million, or $0.40 per basic and diluted share. This compares with a net loss of $15.0 million, or $0.65 per basic and diluted share, for the quarter ended March 31, 2020.

Conference Call Reminder

Axcella will host a conference call today at 8:30 a.m. ET to discuss the company's financial results and other recent business updates. The conference call webcast will be accessible in the Investors & News section on the company's website at www.axcellahealth.com. To access the call via telephone, please dial (844) 808-7139 (U.S. toll free) or (412) 902-0127 (international) five minutes prior to the start time. For those unable to listen in live, a webcast archive will be available on the company's website for 90 days following the call.

About Endogenous Metabolic Modulators (EMMs)

EMMs are a broad family of naturally occurring 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 underlying causes of various complex diseases and improve health.

About Axcella's Clinical Studies

Each of the company's clinical studies to date are or have been conducted as non-investigational new drug application (IND) 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 were not designed or intended to evaluate a product candidate's ability to diagnose, cure, mitigate, treat or prevent a disease. INDs were recently cleared for Axcella's lead product candidates, AXA1665 and AXA1125, allowing for the investigation of efficacy, safety and tolerability in subsequent clinical trials.

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 this portion 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 pioneering a new approach to treat complex diseases and improve health using endogenous metabolic modulator (EMM) compositions. 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. 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 AXA1665, AXA1125 and potential future EMM compositions, the potential for our product candidates to improve the quality of life for patients with complex diseases, the status and timing of the company's planned Phase 2 clinical trial of AXA1665 and planned Phase 2b clinical trial of AXA1125, and the company's expected cash runway into the third quarter of 2022. 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 clinical trials 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 the company is able to collect in its clinical trials of AXA1665 and AXA1125, other potential impacts of COVID-19 on the company's business and financial results, including with respect to its ability to raise additional capital and operational disruptions or delays, changes in law, regulations, or interpretations and enforcement of regulatory guidance, whether data readouts support the company's clinical trial plans and timing, clinical trial design and target indications for AXA1665 and AXA1125, the clinical development and safety profile of AXA1665 and AXA1125 and their therapeutic potential, whether and when, if at all, the company's product candidates will receive approval from the FDA or other comparable regulatory authorities, potential competition from other biopharma companies in the company's target indications, 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, 2021	December 31, 2020
Assets:		
Cash and cash equivalents	$ 43,049	$ 71,590
Marketable securities	49,909	35,739
Other assets	1,522	2,263
Total assets	$ 94,480	$ 109,592
Liabilities and stockholders' equity:		
Liabilities	$ 32,822	$ 34,211
Stockholders' equity	61,658	75,381
Total liabilities and stockholders' equity	$ 94,480	$ 109,592

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

	Three Months Ended March 31,	
	2021	2020
Operating expenses:		
Research and development	$ 10,240	$ 10,335
General and administrative	4,256	4,125
Total operating expenses	14,496	14,460
Loss from operations	(14,496)	(14,460)
Other income (expense):		
Interest income (expense) and other income (expense), net	(693)	(549)
Total other income (expense), net	(693)	(549)
Net loss	$ (15,189)	$ (15,009)
Net loss per share, basic and diluted	$ (0.40)	$ (0.65)
Weighted average common shares outstanding, basic and diluted	37,652,158	23,188,816

View source version on businesswire.com: https://www.businesswire.com/news/home/20210506005368/en/

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