Axcella Reports Third Quarter 2020 Financial Results and Provides Business Update

November 12, 2020

- *Plan to advance AXA1125 directly into a Phase 2b biopsy clinical trial in NASH under IND following successful Type B pre-IND meeting with FDA*
- *Plan to advance AXA1665 directly into a Phase 2 clinical trial in OHE under IND following positive top-line data from AXA1665-002*
- *Published peer-reviewed manuscripts highlighting therapeutic benefit of EMMs and results from AXA1665-001 clinical study*
- *Presented AXA1125 late-breaker at EASL Digital International Liver Congress*
- *Company to hold conference call today at 8:30 a.m. ET*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 12, 2020-- 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 third quarter ended September 30, 2020 and provided a business update.

“Recent accomplishments serve as further validation of the strength of Axcella’s execution amid the COVID-19 pandemic as well as its clinical and regulatory approach,” said Bill Hinshaw, President and Chief Executive Officer of Axcella. “We are pleased to report that we recently completed a successful Type B pre-IND meeting with the U.S. Food and Drug Administration (FDA) that enabled us to affirm our plans to submit an Investigational New Drug (IND) application and initiate a Phase 2b biopsy clinical trial of AXA1125, our product candidate for nonalcoholic steatohepatitis (NASH). We also presented positive top-line data in the third quarter for AXA1665, our product candidate for the reduction in risk of overt hepatic encephalopathy (OHE) recurrence. Having already completed our pre-IND FDA engagement for AXA1665, preparations for its IND submission and Phase 2 clinical trial are now underway.”

“Just three years ago, Axcella finalized the designs of these differentiated, multi-targeted product candidates, and we now are prepared to enter later-stage clinical trials. Better still, we have compiled far more human data than would normally be expected at this stage of development, which we believe increases the probability of success in our next phase of development as we seek to make a difference for patients,” Mr. Hinshaw continued. “With key catalysts for AXA1125 and AXA1665 as well as our decision regarding enrollment expansion for our AXA4010-001 clinical study on the near-term horizon, our excitement continues to build.”

Recent Accomplishments and Next Steps

AXA1125

- Completed Successful Type B Meeting: Axcella recently participated in a Type B pre-IND meeting with the FDA regarding AXA1125, focusing on key elements of the company’s development strategy. This engagement enables Axcella to proceed with its IND submission and Phase 2b clinical trial design as planned.
- Planned Phase 2b Clinical Trial: Axcella plans to submit an IND for AXA1125 and proceed directly into a 48-week Phase 2b serial biopsy clinical trial enrolling adult patients with NASH, with a primary endpoint based on liver histology. This trial is expected to be initiated in the first half of 2021.
- Presented Late-Breaker at EASL for AXA1125: A late-breaker poster containing key findings from Axcella’s AXA1125-003 clinical study was presented at the EASL Digital International Liver Congress by Stephen A. Harrison, M.D., Medical Director of Pinnacle Clinical Research in San Antonio, TX and visiting professor of Hepatology at the University of Oxford, UK.
- Planned Presentations at AASLD: Posters regarding AXA1125-003 will be presented at The Liver Meeting® 2020, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD), which is taking place virtually November 13-16, 2020.

AXA1665

- Reported Positive Top-Line Data: Axcella reported top-line data from AXA1665-002, a 12-week clinical study assessing the impact of AXA1665 on safety, tolerability and physiology in subjects with mild and moderate hepatic insufficiency. Results from the study showed that AXA1665 was generally well-tolerated, with dose dependent changes noted across measures of amino acid metabolism and neurocognition. These included statistically significant ($p < 0.05$) improvements in the Fischer Ratio and the psychometric hepatic encephalopathy score (PHES) in the AXA1665 high dose arm vs. placebo. Additionally, clinically relevant trends were seen in certain measures of nitrogen/ammonia handling and physical function in the AXA1665 arms versus placebo.
- Planned Phase 2 Clinical Trial Initiation: Axcella plans to submit an IND for AXA1665 and proceed directly into a 24-week

Phase 2 clinical trial enrolling subjects with liver cirrhosis who have experienced at least one prior OHE event. This trial is expected to be initiated in the first half of 2021.

- Published Findings from Initial Investigation of AXA1665: *Clinical and Translational Gastroenterology* published a peer-reviewed report detailing results from Axcella's AXA1665-001 clinical study, the initial clinical investigation of AXA1665's effect on safety, tolerability, and clinically relevant biomarkers related to hepatic and muscle metabolism and function.

AXA4010

- Planned Enrollment Decision for AXA4010-001: Following Axcella's receipt of Cohort 1 data in December 2020 from the ongoing AXA4010-001 clinical study, the company expects to communicate its decision about whether to enroll additional subjects with sickle cell disease in the study by early 2021.

EMM Platform

- Published Manuscript Elucidating the Therapeutic Potential of EMMs: *iScience* published a peer-reviewed manuscript entitled "Endogenous Metabolic Modulators: Emerging Therapeutic Potential of Amino Acids" that detailed clinical precedents for EMMs as therapeutics and discussed the potential to develop EMM compositions that simultaneously target multiple biological pathways to address unmet needs in a range of complex diseases.

Financial Results

Cash Position: As of September 30, 2020, cash, cash equivalents and marketable securities totaled $117.3 million, compared to $92.1 million as of December 31, 2019. The increase is primarily the result of net proceeds from the company's follow-on stock offering that was completed in May 2020.

R&D Expenses: Research and development expenses for the quarter and nine months ended September 30, 2020 were $7.5 million and $26.4 million, respectively. Research and development expenses for the same periods ended September 30, 2019 were $12.2 million and $29.1 million. The decrease for the three and nine months ended September 30, 2020 is primarily due to the completion of the company's AXA1125-003 and AXA1665-002 clinical studies.

G&A Expenses: General and administrative expenses for the quarter and nine months ended September 30, 2020 were $4.2 million and $12.9 million, respectively. General and administrative expenses for the same periods ended September 30, 2019 were $4.8 million and $13.0 million.

Net Loss: Net loss for the quarter and nine months ended September 30, 2020 was $12.4 million, or $0.34 per basic and diluted share, and $41.3 million, or $1.39 per basic and diluted share, respectively. This compares with a net loss of $17.3 million, or $0.75 per basic and diluted share, and $43.3 million, or $3.01 per basic and diluted share, for the quarter and nine months ended September 30, 2019. Included in the net loss for the quarter and the nine months ended September 30, 2020 was $1.4 million and $4.9 million, respectively, of non-cash expense related to stock-based compensation, as compared to $1.7 million and $4.3 million, respectively, for the same periods in 2019.

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 (866) 652-5200 (U.S. toll free) or (412) 317-6060 (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 30 days following the call.

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 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 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, any subsequent clinical 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 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. 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 product candidates and the company's characterization of the results from its clinical studies and future clinical trials, including for AXA1125 and AXA1665, the design, status and timing of the company's ongoing clinical study and planned IND-enabled clinical trials, the company's anticipated program milestones, including the timing of data readout from Cohort 1 of AXA4010-001, the subject and timing of the company's planned interactions with the FDA on the AXA1665 and AXA1125 programs, including the potential timing of IND application submissions for its product candidates, including AXA1125 and AXA1665, the potential of the company's product candidates to impact health and/or disease, including AXA1125's potential in NASH and AXA1665's potential in OHE, and the importance of any intellectual rights granted to the company. 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, including planned IND submissions for AXA1125 and AXA1665, 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 ongoing AXA4010-001 clinical study and potential delays in disclosure of the same, other potential impacts of COVID-19 on the company's our business and financial results, including with respect to the company's 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 the company's IND submission and clinical trial initiation plans and timing, clinical trial design and target indications 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 in clinical studies or IND-enabled clinical trials, 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)

	September 30, 2020	December 31, 2019
Assets:		
Cash and cash equivalents	$ 114,063	$ 92,053
Marketable securities	3,192	—
Other assets	2,898	2,306
Total assets	$ 120,153	$ 94,359
Liabilities and stockholders' equity:		
Liabilities	$ 31,391	$ 34,135
Stockholders' equity	88,762	60,224
Total liabilities and stockholders' equity	$ 120,153	$ 94,359

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 7,541	$ 12,157	$ 26,441	$ 29,063
General and administrative	4,184	4,840	12,928	13,036
Total operating expenses	11,725	16,997	39,369	42,099
Loss from operations	(11,725)	(16,997)	(39,369)	(42,099)
Other income (expense), net	(712)	(307)	(1,969)	(1,225)
Net loss	$ (12,437)	$ (17,304)	$ (41,338)	$ (43,324)
Net loss per share, basic and diluted	$ (0.34)	$ (0.75)	$ (1.39)	$ (3.01)

Weighted average common shares outstanding, basic and diluted	36,942,475	23,083,367	29,804,034	14,430,397

View source version on businesswire.com: https://www.businesswire.com/news/home/20201112005213/en/

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

Source: Axcella