Axcella Therapeutics Reports Third Quarter Financial Results and Provides Update on Long COVID, OHE and NASH Clinical Trials

November 10, 2021

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 10, 2021-- Axcella Therapeutics (Nasdaq: AXLA), a clinical-stage biotechnology company pioneering a new approach to treat complex diseases using multi-targeted endogenous metabolic modulator (EMM) compositions, today announced financial results for the third quarter ended September 30, 2021 and provided a business update.

“The third quarter of 2021 was a time in which Axcella focused heavily on both execution and expansion,” said Bill Hinshaw, President and Chief Executive Officer of Axcella. “From an executional standpoint, we opened dozens of clinical sites and rapidly increased patient screening activities in our recently initiated EMMPACT Phase 2b and EMMPOWER Phase 2 clinical trials. Additionally, based upon AXA1125’s mechanistic underpinnings and emerging science implicating mitochondrial dysfunction as a core driver of Long COVID fatigue, we moved aggressively to advance a new program in this area and are prepared to conduct a highly efficient Phase 2a clinical trial with researchers at Oxford University that is set to begin in the weeks ahead. These accomplishments position us well for multiple potentially transformational catalysts ahead.”

Recent Accomplishments and Developments

- **Launched Long COVID Clinical Development Program:** Axcella announced a new clinical program to investigate AXA1125 as a potential treatment for patients with Long COVID, a complex condition also known as Post COVID-19 Condition and Post-Acute Sequelae of COVID-19 (PASC). Following the recent acceptance of a clinical trial authorization (CTA) submission by the United Kingdom’s Medicines and Healthcare products Regulatory Agency (MHRA) and Ethics Committee approval, a Phase 2a clinical trial led by researchers at the Radcliffe Department of Medicine at the University of Oxford (U.K.) is planned to begin later in 2021, with top-line data expected by mid-2022. This will be a 28-day, randomized, double-blind, placebo-controlled investigation to evaluate the efficacy and safety of AXA1125 in approximately 40 patients with exertional fatigue related to Long COVID.
- **Progressed Screening and Enrollment in EMMPOWER℠ and EMMPACT℠ Clinical Trials:** Patient screening and enrollment continues in Axcella’s recently initiated EMMPOWER Phase 2 clinical trial in overt hepatic encephalopathy (OHE) and EMMPACT Phase 2b clinical trial in nonalcoholic steatohepatitis (NASH). EMMPOWER is a global 24-week, randomized, double-blind, placebo-controlled trial that is evaluating the efficacy and safety of AXA1665 in approximately 150 patients who have experienced at least one prior OHE event and have neurocognitive dysfunction at screening. EMMPACT is a global 48-week, randomized, double-blind, placebo-controlled trial that is evaluating the efficacy and safety of AXA1125 in approximately 270 patients with biopsy-confirmed F2/F3 NASH.
- **Published AXA1125 Data in The American Journal of Gastroenterology**: The American Journal of Gastroenterology recently published results from Axcella’s AXA1125-003 clinical study. The publication, entitled “Safety, Tolerability, and Biologic Activity of AXA1125 and AXA1957 in Subjects With Nonalcoholic Fatty Liver Disease,” highlighted the effects seen with AXA1125 versus placebo across markers of metabolism, inflammation and fibrosis over 16 weeks in subjects with presumed NASH.
- **Accepted for Presentations at The Liver Meeting® 2021:** Posters were accepted for presentation 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 12-15, 2021. One of the presentations focuses on the amino acid signature of AXA1665 and the other features predictive metabolic modeling for AXA1125.
- **Announced Organizational Change:** Axcella today announced that Chief Financial Officer Laurent Chardonnet will be departing the company for personal reasons and to pursue other interests, effective November 28, 2021. Mr. Chardonnet plans to continue to serve as a consultant to the company to ensure a smooth transition. “On behalf of management, Axcella employees and the Board of Directors, I would like to thank Laurent for his contributions over the past two years and for his ongoing support,” said Mr. Hinshaw.

Financial Results

Cash Position: As of September 30, 2021, cash, cash equivalents, and marketable securities totaled $66.1 million, compared to $107.3 million as of December 31, 2020. Axcella continues to expect that its existing 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 for the quarter and nine months ended September 30, 2021 were $10.1 million and $30.7 million, respectively. Research and development expenses for the same periods ended September 30, 2020 were $7.5 million and $26.4 million. These increases are primarily the result of work related to the initiation of the company’s EMMPOWER and EMMPACT clinical trials.

G&A Expenses: General and administrative expenses for the quarter and nine months ended September 30, 2021 were $4.8 million and $14.0

million, respectively. General and administrative expenses for the same periods ended September 30, 2020 were $4.2 million and $12.9 million. These increases are primarily the result of greater non-cash stock-based compensation expenses.

Net Loss: Net loss for the quarter and nine months ended September 30, 2021 was $15.6 million, or $0.41 per basic and diluted share, and $46.7 million, or $1.23 per basic and diluted share, respectively. This compares with a net loss of $12.4 million, or $0.34 per basic and diluted share, and $41.3 million, or $1.39 per basic and diluted share, for the quarter and nine months ended September 30, 2020.

Internet Posting of Information

Axcella uses its website, www.axcellatx.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 Therapeutics (Nasdaq: AXLA)

Axcella is a clinical-stage biotechnology company pioneering a new approach to treat complex diseases using endogenous metabolic modulator (EMM) compositions. The company's product candidates are comprised of EMMs and derivatives that are engineered in distinct combinations and ratios to reset multiple biological pathways, improve cellular energetics, and restore homeostasis. Axcella's pipeline includes lead therapeutic candidates in Phase 2 development for the reduction in risk of overt hepatic encephalopathy (OHE) recurrence, the treatment of Long COVID, and the treatment of non-alcoholic steatohepatitis (NASH). The company's unique model allows for the evaluation of its EMM compositions through non-IND clinical studies or IND clinical trials. For more information, please visit www.axcellatx.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 timing of initiation, enrollment and completion of the company's clinical trials, the potential for transformational catalysts, and the company's expected cash runway. 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 Therapeutics
Unaudited Condensed Consolidated Balance Sheets
(in thousands)

	September 30, 2021	December 31, 2020
Assets:		
Cash and cash equivalents	$ 26,091	$ 71,590
Marketable securities	40,055	35,739
Other assets	2,675	2,263
Total assets	$ 68,821	$ 109,592
Liabilities and stockholders' equity:		
Liabilities	$ 33,260	$ 34,211
Stockholders' equity	35,561	75,381
Total liabilities and stockholders' equity	$ 68,821	$ 109,592

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 10,130	$ 7,541	$ 30,668	$ 26,441
General and administrative	4,773	4,184	13,975	12,928
Total operating expenses	14,903	11,725	44,643	39,369
Loss from operations	(14,903)	(11,725)	(44,643)	(39,369)
Other income (expense):				
Interest income (expense) and other income (expense), net	(710)	(712)	(2,094)	(1,969)
Total other income (expense), net	(710)	(712)	(2,094)	(1,969)
Net loss	$ (15,613)	$ (12,437)	$ (46,737)	$ (41,338)
Net loss per share, basic and diluted	$ (0.41)	$ (0.34)	$ (1.23)	$ (1.39)
Weighted average common shares outstanding, basic and diluted	38,195,583	36,942,475	37,861,970	29,804,034

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