Axcella Reports Second Quarter Financial Results and Provides Business Update

July 29, 2021

- *Initiated EMMPOWER Phase 2 clinical trial of AXA1665 for the reduction in risk of recurrent OHE*
- *Initiated EMMPACT Phase 2b clinical trial of AXA1125 for the treatment of NASH*
- *Presented data at key medical congresses*
- *Enhanced management team with the addition of Chief People Officer*
- *Company to host conference call at 8:30 a.m. ET today*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 29, 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 second quarter ended June 30, 2021 and provided a business update.

“Thanks to solid execution on the part of our team, Axcella has rapidly advanced the development of its multi-targeted EMM compositions in order to address significant unmet needs for patients with complex diseases,” said Bill Hinshaw, President and Chief Executive Officer of Axcella. “Our progress continued in the second quarter as we initiated the EMMPOWER Phase 2 clinical trial of AXA1665 in overt hepatic encephalopathy (OHE) as well as the EMMPACT Phase 2b clinical trial of AXA1125 in nonalcoholic steatohepatitis (NASH). With a high level of interest and engagement from the medical community and many clinical sites already activated for each of these global trials, I am pleased to report that we are off to a strong start. As we enter the second half of 2021, maximizing clinical trial enrollment and expanding Axcella’s pipeline are our primary areas of focus.”

Recent Accomplishments

AXA1665 for the Reduction in Risk of Recurrent OHE

- **Initiated EMMPOWER Clinical Trial:** Axcella recently initiated its EMMPOWER Phase 2 clinical trial. This global 24-week, randomized, double-blind, placebo-controlled trial 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.
- **Presented Orally at DDW 2021:** Data from Axcella’s AXA1665-002 clinical study were highlighted in an oral presentation at the Digestive Disease Week (DDW) 2021 Annual Meeting by Dr. Arun Sanyal, Professor in the Virginia Commonwealth University (VCU) Department of Internal Medicine and Education Core Director in the VCU Center for Clinical and Translational Research.

AXA1125 for the Treatment of NASH

- **Initiated EMMPACT Clinical Trial:** In April, the U.S. Food and Drug Administration (FDA) cleared an Investigational New Drug (IND) application for AXA1125. Shortly thereafter, Axcella initiated its EMMPACT Phase 2b clinical trial. This global 48-week, randomized, double-blind, placebo-controlled trial is evaluating the efficacy and safety of AXA1125 in approximately 270 patients with biopsy-confirmed F2/F3 NASH.
- **Published Findings in Nature’s *Scientific Reports***: Nature’s *Scientific Reports* published findings from a systematic evaluation of AXA1125’s EMM constituents across multiple primary human cell model systems demonstrating that they consistently and simultaneously impacted NASH-relevant metabolic, inflammatory and fibrotic processes.
- **Presented at ADA 81st Scientific Sessions:** Clinical and nonclinical data regarding the effects from AXA1125 and its EMM constituents on insulin sensitivity were included in a poster presentation at the American Diabetes Association (ADA) 81st Scientific Sessions.

Management Team

- **Added Virginia Dean as Chief People Officer:** In June 2021, Axcella appointed Virginia Dean as the company’s Senior Vice President and Chief People Officer. In this role, Ms. Dean is leading the company’s organizational and cultural development initiatives with responsibility for all human resources functions. She previously headed up ClearSight Leadership, a consulting firm specializing in HR services, and led the human resources functions for several high-growth companies, including TESARO, Inc. and ARIAD Pharmaceuticals.

Financial Results

Cash Position: As of June 30, 2021, cash, cash equivalents, and marketable securities totaled $78.9 million, compared to $107.3 million at 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 six months ended June 30, 2021 were $10.3 million and $20.5 million, respectively. Research and development expenses for the same periods ended June 30, 2020 were $8.6 million and $18.9 million. These increases are primarily the result of work related to the initiations of the company's EMMPOWER and EMMPACT clinical trials.

G&A Expenses: General and administrative expenses for the quarter and six months ended June 30, 2021 were $4.9 million and $9.2 million, respectively. General and administrative expenses for the same periods ended June 30, 2020 were $4.6 million and $8.7 million. These increases are primarily the result of greater non-cash stock-based compensation expenses and benefit-related costs.

Net Loss: Net loss for the quarter and six months ended June 30, 2021 was $15.9 million, or $0.42 per basic and diluted share, and $31.1 million, or $0.83 per basic and diluted share, respectively. This compares with a net loss of $13.9 million, or $0.48 per basic and diluted share, and $28.9 million, or $1.10 per basic and diluted share, for the quarter and six months ended June 30, 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 Development

Each of the clinical investigations that the company has completed to date 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 and when Axcella decides to develop a product candidate as a potential therapeutic, as is the case with AXA1665 and AXA1125, the company will seek an IND to enable the initiation of "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 company's ability to enroll its EMMPOWER and EMMPACT clinical trials in a timely manner, its ability to expand the company's pipeline, 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 Health Inc.
Unaudited Condensed Consolidated Balance Sheets
(in thousands)

	June 30, 2021	December 31, 2020
Assets:		
Cash and cash equivalents	$ 33,940	$ 71,590
Marketable securities	45,003	35,739
Other assets	2,541	2,263
Total assets	$ 81,484	$ 109,592
Liabilities and stockholders' equity:		
Liabilities	$ 33,397	$ 34,211
Stockholders' equity	48,087	75,381
Total liabilities and stockholders' equity	$ 81,484	$ 109,592

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 10,298	$ 8,565	$ 20,538	$ 18,900
General and administrative	4,946	4,619	9,202	8,744
Total operating expenses	15,244	13,184	29,740	27,644
Loss from operations	(15,244)	(13,184)	(29,740)	(27,644)
Other income (expense):				
Interest income (expense) and other income (expense), net	(691)	(708)	(1,384)	(1,257)
Total other income (expense), net	(691)	(708)	(1,384)	(1,257)
Net loss	$ (15,935)	$ (13,892)	$ (31,124)	$ (28,901)
Net loss per share, basic and diluted	$ (0.42)	$ (0.48)	$ (0.83)	$ (1.10)
Weighted average common shares outstanding, basic and diluted	37,732,196	29,202,367	37,692,398	26,195,591

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

Source: Axcella