Axcella Reports Fourth Quarter and Full Year 2020 Financial Results and Provides Business Update

March 17, 2021

- *First IND application cleared by FDA for AXA1665*
- *Initiation of AXA1665 Phase 2 clinical trial in OHE expected in Q2 2021*
- *Initiation of AXA1125 Phase 2b clinical trial in NASH expected in Q2 2021*
- *Company to host conference call at 8:30 a.m. ET today*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 17, 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 fourth quarter and full year ended December 31, 2020 and provided a business update.

"The year 2020 was a key inflection point for Axcella as we completed multiple clinical studies with positive results and shifted our full attention to our upcoming later-stage clinical trials," said Bill Hinshaw, President and Chief Executive Officer of Axcella. "We were pleased to advance our preparations in the fourth quarter with a successful Type B pre-IND meeting with the U.S. Food and Drug Administration (FDA) regarding our nonalcoholic steatohepatitis (NASH) candidate, AXA1125, and began 2021 with our first investigational new drug (IND) clearance for AXA1665, our candidate for the reduction in risk of recurrent overt hepatic encephalopathy (OHE). Axcella's strong execution has positioned us for an exciting year ahead as we seek to swiftly enroll our upcoming trials, expand our pipeline of EMM compositions and further our mission to improve the lives of patients with complex diseases."

Recent Accomplishments and Next Steps

AXA1665 for OHE

- **Cleared IND Application:** In January 2021, Axcella's IND application was cleared by the FDA for AXA1665. This first IND clearance marks a key milestone and validates the company's clinical approach with its EMM compositions.
- **Planned Phase 2 Clinical Trial Initiation:** Axcella plans to initiate a 24-week Phase 2 clinical trial of AXA1665 in the second quarter of 2021. This will be a randomized, double-blind, placebo-controlled, multi-center trial evaluating the efficacy and safety of AXA1665 in patients who have experienced at least one prior OHE event and have neurocognitive dysfunction at screening. Approximately 150 patients on lactulose ± rifaximin (stratified by rifaximin use) will be enrolled and randomized 1:1 to receive either 53.8 grams per day of AXA1665 or a calorie-matched placebo in three divided doses for 24 weeks, with a four-week safety follow-up period. The trial will be conducted globally with a primary endpoint assessing the proportion of subjects with a ≥2 point increase in the psychometric hepatic encephalopathy score (PHES) after the 24-week treatment period. Secondary endpoints will include the proportion of patients experiencing an OHE breakthrough event; time to first OHE breakthrough event, including time to hospitalization; changes in physical function; and patient-reported outcomes. Other endpoints include measures of circulating ammonia, amino acids, and inflammation-related markers.

AXA1125 for NASH

- **Advanced IND Preparations:** Axcella participated in a Type B pre-IND meeting with the FDA regarding AXA1125 in the fourth quarter of 2020. This engagement provided the insight required for the company to advance its Phase 2b clinical trial design and finalize its preparation of an IND submission.
- **Planned Phase 2b Clinical Trial:** Following IND clearance by the FDA, Axcella plans to proceed directly into a 48-week Phase 2b serial biopsy clinical trial of AXA1125 enrolling adult patients with NASH, with a primary endpoint based on liver histology. This trial is expected to be initiated in the second quarter of 2021.
- **Presented Data at The Liver Meeting 2020:** Two posters regarding AXA1125 were presented in November 2020 at The Liver Meeting® 2020, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD). Both presentations featured data from AXA1125-003, a placebo-controlled, randomized clinical study that enrolled 102 subjects with presumed nonalcoholic steatohepatitis (NASH) to assess the impact of AXA1125 and AXA1957 on safety, tolerability and effects on structures and functions of the liver for 16 weeks.
- **Presented Data at NASH-TAG 2021:** Last week, multiple presentations regarding AXA1125 were included in NASH-TAG 2021, an event bringing clinicians and researchers together to focus on the diagnosis and therapy 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 President of R&D:** 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 Selecta Biosciences, Sanofi-Genzyme, Baxalta, Novartis and Johnson & Johnson.

Financial Results

Cash Position: As of December 31, 2020, cash, cash equivalents, and marketable securities totaled $107.3 million, compared to $92.1 million at 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. 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 $10.6 million and $10.8 million for the quarters ended December 31, 2020 and 2019, respectively. For the years ended December 31, 2020 and 2019, research and development expenses were $37.0 million and $41.7 million, respectively. The year-over-year decrease is primarily due to the completion of the company's AXA1665-002 and AXA1125-003 clinical studies.

G&A Expenses: General and administrative expenses were $3.9 million and $4.6 million for the quarters ended December 31, 2020 and 2019, respectively. For the years ended December 31, 2020 and 2019, general and administrative expenses were $16.8 million and $15.8 million, respectively. The year-over-year increase is primarily due to greater costs associated with becoming a public company.

Net Loss: Net loss for the quarter ended December 31, 2020 was $15.2 million, or $0.40 per basic and diluted share. This compares with a net loss of $15.7 million, or $0.68 per basic and diluted share, for the quarter ended December 31, 2019. Net loss for the year ended December 31, 2020 was $56.5 million, or $1.78 per basic and diluted share. This compares with a net loss of $59.0 million, or $3.55 per basic and diluted share, for the year ended December 31, 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 (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 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 trials 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. 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 Phased 2b clinical trial of AXA1125, the timing and outcome of IND application submissions 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 planned 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 initiation 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)

	December 31, 2020	December 31, 2019
Assets:		
Cash and cash equivalents	$ 71,590	$ 92,053
Marketable securities	35,739	—
Other assets	2,263	2,306
Total assets	$ 109,592	$ 94,359
Liabilities and stockholders' equity:		
Liabilities	$ 34,211	$ 34,135
Stockholders' equity	75,381	60,224
Total liabilities and stockholders' equity	$ 109,592	$ 94,359

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

	Three Months Ended December 31,		Year Ended December 31,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 10,598	$ 10,758	$ 37,039	$ 41,658
General and administrative	3,869	4,582	16,797	15,781
Total operating expenses	14,467	15,340	53,836	57,439
Loss from operations	(14,467)	(15,340)	(53,836)	(57,439)
Other income (expense):				
Interest income (expense) and other income (expense), net	(722)	(373)	(2,691)	(1,598)
Total other income (expense), net	(722)	(373)	(2,691)	(1,598)
Net loss	$ (15,189)	$ (15,713)	$ (56,527)	$ (59,037)
Net loss per share, basic and diluted	$ (0.40)	$ (0.68)	$ (1.78)	$ (3.55)
Weighted average common shares outstanding, basic and diluted	37,536,350	23,137,014	31,747,676	16,624,941

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Source: Axcella