Axcella Reports Fourth Quarter and Full Year 2019 Financial Results and Provides Business Update

March 23, 2020

- *Announced positive interim findings from ongoing clinical study of AXA1125 and AXA1957; expect to report top-line data in second quarter of 2020*
- *Completed enrollment in ongoing clinical study of AXA1665; expect to report top-line data in third quarter of 2020*
- *Bolstered intellectual property portfolio with new patent issuance for AXA1125 and AXA1957*
- *Strengthened management team*
- *Company provides COVID-19 update*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 23, 2020-- Axcella Health Inc. (Nasdaq: AXLA), a clinical-stage biotechnology company focused on leveraging endogenous metabolic modulators (EMMs) to pioneer a new approach for treating complex diseases and improving health, today announced financial results for the fourth quarter and full year ended December 31, 2019 and provided a business update.

“Axcella made great strides in 2019 as we presented data at preeminent medical congresses, bolstered our intellectual property portfolio, completed our IPO and strengthened our management team and Board of Directors. We also initiated four clinical studies during the year to provide us with significant human data to inform our clinical development plans and regulatory engagements,” said Bill Hinshaw, President and Chief Executive Officer of Axcella. “As we entered 2020, this strong momentum continued as we completed enrollment of our clinical study of AXA1665 and announced positive interim findings from our clinical study of AXA1125 and AXA1957. We look forward to multiple planned readouts during the remainder of the year and remain on track to report top-line data for our lead candidates for nonalcoholic steatohepatitis (NASH) and overt hepatic encephalopathy (OHE) in mid-2020.

“Meanwhile, the impacts of the COVID-19 outbreak on our society and in the medical community are far-reaching and rapidly evolving,” continued Mr. Hinshaw. “As it relates to Axcella, we have taken a series of actions aimed at safeguarding our staff and business associates, including implementing a work-at-home policy, providing flexibility for working parents and suspending all business-related travel. We also are keeping in close virtual contact with our service providers and clinical sites in order to assess and minimize impact to our programs.”

Recent Highlights

Liver Programs

- Announced interim findings from AXA1125-003, the company’s ongoing clinical study assessing the impact of AXA1125 and AXA1957 on safety, tolerability and physiology in more than 100 adult subjects with non-alcoholic fatty liver disease (NAFLD). The analysis showed that AXA1125 and both doses of AXA1957 had been well tolerated to date. Additionally, both AXA1125 and AXA1957 demonstrated clinically relevant responses on the three biological nodes fundamental to liver health and disease: metabolism, inflammation and fibrogenesis. The onset of response in some biomarkers was seen as early as the eight-week, post-baseline assessment with continued improvement through 16 weeks. These findings were highlighted at the 2020 NASH-TAG Conference in January 2020.
- Completed enrollment of AXA1665-002, an ongoing clinical study to assess the impact of AXA1665 on safety, tolerability and physiology in adult subjects with mild and moderate hepatic insufficiency. AXA1665 has been generally safe and well tolerated in this study to date.
- Announced the issuance of U.S. Patent 10,471,034, which covers an array of EMM compositions, including AXA1125 and AXA1957, the company’s product candidates for NASH.

Blood Program

- Presented mechanistic data on the company’s hematology product candidate AXA4010 at the 61st American Society of Hematology (ASH) Annual Meeting and Exposition.

Muscle Program

- Discussed Axcella’s AXA2678 muscle product candidate during an oral presentation at the 2020 International Conference on Frailty and Sarcopenia Research (ICFSR).

Organization

- Appointed Laurent Chardonnet as the company's Chief Financial Officer and promoted Heidy King-Jones to General Counsel.

Anticipated 2020 Milestones

Liver Programs

- Q2 2020: Report top-line data from AXA1125-003 in adult subjects with NAFLD. Since enrollment in this clinical study was completed in October 2019, Axcella does not currently anticipate the COVID-19 outbreak to impact the timing of its readout.
- Q3 2020: Report top-line data from AXA1665-002 in adult subjects with mild and moderate hepatic insufficiency. Since enrollment in this clinical study was completed in February 2020, Axcella does not currently anticipate the COVID-19 outbreak to impact the timing of its readout.
- Q4 2020: Submit an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) and initiate a potential Phase 2b/3 registrational clinical trial of AXA1665 for the reduction in risk of OHE recurrence.
- AXA1957 Update: As a result of a resource reallocation from studies and trials toward COVID-19 detection and treatment at clinical sites involved in AXA1957-002, enrollment and dosing in this pediatric clinical study has been temporarily suspended. Axcella continues to view pediatric NASH as a significant area of unmet need. Utilizing information gathered thus far from AXA1957-002 and the upcoming top-line readout from AXA1125-003, the company plans to provide an update on its plans for this program at a future date.

Blood Program

- Q4 2020: Report top-line data from Cohort 1 of AXA4010-001, a clinical study on safety, tolerability and blood physiology in subjects with sickle cell disease.

Financial Results

R&D Expenses: Research and development expenses were $10.8 million and $7.9 million for the quarters ended December 31, 2019 and 2018, respectively. Research and development expenses were $41.7 million and $25.5 million for the years ended December 31, 2019 and 2018, respectively. The increase in both periods was primarily related to greater costs associated with the advancement of the company's product candidates and ongoing clinical studies.

G&A Expenses: General and administrative expenses were $4.6 million and $1.3 million for the quarters ended December 31, 2019 and 2018, respectively. General and administrative expenses were $15.8 million and $8.4 million for the years ended December 31, 2019 and 2018, respectively. The increase in both periods was primarily related to higher professional services and employee-related costs associated with being a public company.

Net Loss: Net loss for the quarter ended December 31, 2019 was $15.7 million, or $0.68 per basic and diluted share. This compares with a net loss of $9.8 million, or $2.05 per basic and diluted share, for the quarter ended December 31, 2018. Net loss for the year ended December 31, 2019 was $59.0 million, or $3.55 per basic and diluted share. This compares with a net loss of $36.1 million, or $7.97 per basic and diluted share, for the year ended December 31, 2018.

Cash Position: Cash and cash equivalents at December 31, 2019 were $92.1 million, which compares with $79.5 million at December 31, 2018. The increase was the result of proceeds from the company's May 2019 initial public offering, partially offset by operating expenditures. Axcella expects that its cash and cash equivalents will be sufficient to meet the company's operating needs into the second quarter of 2021.

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 ongoing clinical studies are being conducted as non-investigational new drug (IND) application 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 AXA1125/1957, subsequent 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 such portions 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 focused on leveraging endogenous metabolic modulators (EMMs) to pioneer a new approach for

treating complex diseases and improving health. 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 and development potential of the company's EMM product candidates, the design, status and timing of the company's ongoing clinical studies and planned IND-enabled clinical trials and the results, including the quality, completeness, and interpretability of results, and the timing of receipt and disclosure of data results from the same, including with respect to the company's ongoing clinical studies for AXA1125, AXA1957 and AXA1665 and planned clinical trial for AXA1665, the subject and timing of the company's interactions with the FDA, including with respect to the potential filing of an IND for AXA1665, the sufficiency of the company's cash and cash equivalents to meet operating needs, the potential of the Company's product candidates to impact health and/or disease, including AXA1125 and AXA1957's potential in NASH and AXA1665 potential in OHE, and the effect of the COVID-19 outbreak on any of the foregoing. 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 clinical study recruitment, including on clinical site, staff and subject availability, potential clinical study delays and holds, regulatory limitations and operations and the completeness and quality of data we are able to collect from ongoing clinical studies, and the company's ability to conduct and complete its ongoing clinical studies and planned clinical trials in a time manner or at all, other potential impacts of COVID-19 on our business and financial results, including with respect to our ability to raise additional capital and operational disruptions or delays, the breadth and potential uses of the company's pipeline of product candidates, including the potential for AXA1665 to benefit OHE patients, whether planned data readouts and disclosures are positive and support our beliefs regarding EMMs and AXA1125/AXA1957 and AXA1665's potential ability to benefit not just healthy but also diseased patients, including patients with NASH and OHE, respectively, and the planned timing of our disclosures regarding data readouts, whether data readouts and/or FDA feedback support our planned timing for an IND filing, clinical trial design and target indication for AXA1125/ AXA1957 and AXA1665, the potential for the planned AXA1665 IND clinical trial to be registrational, the strength of the company's development platform, the efficiency of the company's discovery and development approach, 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 U.S. Food and Drug Administration, or other comparable regulatory authorities, and for which, if any, indications, competition from other biotechnology companies, the company's liquidity, its ability to successfully develop product candidates through current and future milestones on the anticipated timeline, if at all, past results from non-IND clinical studies not being representative of future results, and other risks identified in the company's SEC filings, including Axcella's 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, 2019	December 31, 2018
Assets:		
Cash and cash equivalents	$ 92,053	$ 79,466
Other assets	2,306	2,378
Total assets	$ 94,359	$ 81,844
Liabilities and stockholders' equity (deficit):		
Liabilities	$ 34,135	$ 33,755
Preferred stock	—	197,842
Stockholders' equity (deficit)	60,224	(149,753)
Total liabilities and stockholders' equity	$ 94,359	$ 81,844

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,	
	2019	2018	2019	2018
Operating expenses:				
Research and development	$ 10,758	$ 7,877	$ 41,658	$ 25,486
General and administrative	4,582	1,286	15,781	8,410
Total operating expenses	15,340	9,163	57,439	33,896

Loss from operations	(15,340)	(9,163)	(57,439)	(33,896)
Other income (expense):				
Change in fair value of preferred stock warrant liability	—	(60)	(51)	(14)
Interest income (expense), net	(373)	(539)	(1,547)	(2,159)
Total other income (expense), net	(373)	(599)	(1,598)	(2,173)
Net loss	$ (15,713)	$ (9,762)	$ (59,037)	$ (36,069)
Net loss per share, basic and diluted	$ (0.68)	$ (2.05)	$ (3.55)	$ (7.97)
Weighted average common shares outstanding, basic and diluted	23,137,014	4,772,964	16,624,941	4,546,373

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