Axcella Therapeutics Reports Fourth Quarter and Full Year 2021 Financial Results and Provides Business Update

March 30, 2022

- *Completed $25 million registered direct stock offering priced at the market*
- *Initiated Phase 2a Long COVID clinical trial*
- *Fast Track designation received from FDA for AXA1125 in NASH with liver fibrosis*
- *Enhanced management team*
- *Long COVID top-line data and NASH interim data expected in the third quarter of 2022*
- *Company to host conference call at 8:30 a.m. ET today*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 30, 2022-- 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 fourth quarter and full year ended December 31, 2021 and provided a business update.

"The year 2021 was one of significant accomplishment for Axcella Therapeutics that was highlighted by the initiation of three distinct Phase 2 trials in non-alcoholic steatohepatitis (NASH), overt hepatic encephalopathy (OHE), and our newest area of focus, Long COVID. We were pleased to continue our momentum in early 2022 by obtaining Fast Track designation for AXA1125 in NASH with liver fibrosis," said Bill Hinshaw, President and Chief Executive Officer of Axcella. "A data-rich period is now fast approaching, with both our Long COVID Phase 2a top-line results and NASH Phase 2b interim results anticipated in the third quarter. With these steps, a recently strengthened balance sheet and other potential value drivers on the near-term horizon, we believe the stage has been set for a transformational 2022."

Recent Accomplishments and Developments

- **Initiated Long COVID Phase 2a Clinical Trial:** Axcella initiated patient enrollment and dosing in a Phase 2a trial that is investigating AXA1125 as a potential oral treatment for patients with exertional fatigue related to Long COVID. Fatigue is the most common symptom associated with Long COVID, impacting a majority of patients. This randomized, double-blind, placebo-controlled trial is enrolling approximately 40 patients at the University of Oxford in the United Kingdom who are receiving AXA1125 or a matched placebo for 28 days with a one-week safety follow-up period.
- **Obtained Fast Track Designation for AXA1125:** The U.S. Food and Drug Administration (FDA) recently granted a Fast Track designation to AXA1125 for the treatment of NASH with liver fibrosis. Fast Track is a process designed by the FDA to facilitate the development and expedite the review of drugs to treat serious or life-threatening conditions with unmet medical needs.
- **Presented Data at The Liver Meeting® 2021:** Posters were presented about Axcella's programs at The Liver Meeting® 2021, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD), which took place virtually November 12-15, 2021. One of the presentations focused on the amino acid signature of AXA1665 and the other featured predictive metabolic modeling for AXA1125.
- **Progressed Screening and Enrollment in EMMPACT℠ and EMMPOWER℠ Clinical Trials:**
  - EMMPACT is a global 48-week, randomized, double-blind, placebo-controlled Phase 2b clinical trial that is evaluating the efficacy and safety of AXA1125 in approximately 270 patients with biopsy-confirmed F2/F3 NASH.
  - EMMPOWER is a global 24-week, randomized, double-blind, placebo-controlled Phase 2 clinical 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.
- **Strengthened Management**: Margaret Koziel, M.D., was promoted to the role of Chief Medical Officer. Dr. Koziel, who previously served as Axcella's Vice President, Clinical Development, has a wealth of leadership experience within both biopharma and academia. Additionally, Robert Crane was appointed as the company's new Chief Financial Officer. Mr. Crane brings to Axcella over 35 years of experience building therapeutics, diagnostics and medical device companies.
- **Enhanced Balance Sheet**: Axcella completed a registered direct offering in March 2022. In this transaction, 13,089,002 shares of the company's common stock were sold at the market for a purchase price of $1.91 per share, yielding gross proceeds of approximately $25.0 million.

Financial Results

Cash Position: As of December 31, 2021, cash, cash equivalents, and marketable securities totaled $55.0 million, compared to $107.3 million as of December 31, 2020. As mentioned above, subsequent to the close of 2021, the company received approximately $25.0 million in gross proceeds from a registered direct offering of common stock. Axcella expects that its current cash balance will be sufficient to meet its operating needs into 2023.

R&D Expenses: Research and development expenses for the quarter and year ended December 31, 2021 were $12.5 million and $43.1 million,

respectively. Research and development expenses for the same periods ended December 31, 2020 were $10.6 million and $37.0 million. These increases are primarily the result of the initiation of the company's EMMPOWER, EMMPACT and Long COVID Phase 2 clinical trials.

G&A Expenses: General and administrative expenses for the quarter and year ended December 31, 2021 were $4.7 million and $18.7 million, respectively. General and administrative expenses for the same periods ended December 31, 2020 were $3.9 million and $16.8 million. These increases are primarily the result of greater non-cash stock-based compensation expenses.

Net Loss: Net loss for the quarter and year ended December 31, 2021 was $17.9 million, or $0.46 per basic and diluted share, and $64.6 million, or $1.70 per basic and diluted share, respectively. This compares with a net loss of $15.2 million, or $0.40 per basic and diluted share, and $56.5 million, or $1.78 per basic and diluted share, for the quarter and year ended December 31, 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.axcellatx.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.

Internet Posting of Information

Axcella uses the "Investors and News" section of its website, www.axcellatx.com, as a means of disclosing material nonpublic information, to communicate with investors and the public, and for complying with its disclosure obligations under Regulation FD. Such disclosures include, but may not be limited to, investor presentations and FAQs, Securities and Exchange Commission filings, press releases, and public conference calls and webcasts. The information that we post on our website could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

About Axcella Therapeutics (Nasdaq: AXLA)

Axcella is a clinical-stage biotechnology company pioneering a new approach to treat complex diseases using compositions of endogenous metabolic modulators (EMMs). 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 treatment of Long COVID and non-alcoholic steatohepatitis (NASH), and the reduction in risk of overt hepatic encephalopathy (OHE) recurrence. 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 the company's clinical trial data readouts and its 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)

	December 31, 2021	December 31, 2020
Assets:		
Cash and cash equivalents	$ 23,574	$ 71,590
Marketable securities	31,474	35,739
Other assets	2,679	2,263
Total assets	$ 57,727	$ 109,592
Liabilities and stockholders' equity:		
Liabilities	$ 35,719	$ 34,211
Stockholders' equity	22,008	75,381

Total liabilities and stockholders' equity	$ 57,727	$ 109,592

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

	Three Months Ended December 31,		Year Ended December 31,	
	2021	**2020**	**2021**	**2020**
Operating expenses:				
Research and development	$ 12,467	$ 10,598	$ 43,135	$ 37,039
General and administrative	4,736	3,869	18,711	16,797
Total operating expenses	17,203	14,467	61,846	53,836
Loss from operations	(17,203)	(14,467)	(61,846)	(53,836)
Other income (expense):				
Interest income (expense) and other income (expense), net	(688)	(722)	(2,782)	(2,691)
Total other income (expense), net	(688)	(722)	(2,782)	(2,691)
Net loss	$ (17,891)	$ (15,189)	$ (64,628)	$ (56,527)
Net loss per share, basic and diluted	$ (0.46)	$ (0.40)	$ (1.70)	$ (1.78)
Weighted average common shares outstanding, basic and diluted	38,847,669	37,536,350	38,110,420	31,747,676

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