Axcella Announces Regulatory Path to Registration of AXA1125 for Long COVID Fatigue

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MHRA guidance aligns on key measurements for a registration trial, including primary endpoint and trial design elements

IND for phase 2b/3 trial submitted to the FDA

Axcella to host a conference call Tuesday, January 24 at 8:00 a.m. ET; To register, click here

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 23, 2023-- Axcella Therapeutics (Nasdaq: AXLA), a clinical-stage biotechnology company pioneering novel approaches to treating complex diseases using multi-targeted endogenous metabolic modulator (EMM) compositions, announced a regulatory path to registration of AXA1125 in the treatment of Long COVID Fatigue. The company reported that it had received regulatory guidance from The Medicines and Healthcare products Regulatory Agency (MHRA), the U.K.'s regulatory agency, supporting a single trial that could serve as the registration trial for patients with Long COVID fatigue, and aligning on key measurements, including primary endpoint and trial design. Axcella will be meeting with the MHRA in the near term to discuss the Innovative Licensing and Access Pathway (ILAP) application. The company further reported submission of an Investigational New Drug (IND) Application to the U.S. Food and Drug Administration (FDA) for a Phase 2b/3 trial.

These efforts follow submission of materials to both regulatory agencies including results from the Phase 2a randomized, double-blind, placebo-controlled investigation to evaluate the efficacy and safety of AXA1125 in patients with fatigue related to Long COVID. Notably, in this study subjects who received AXA1125 experienced clinically and statistically significant improvement in mental (p=0.0097) and physical (p=0.0097) fatigue scores compared to placebo subjects.

Long COVID is a persistent and growing long-term part of the pandemic, affecting an estimated one hundred million patients worldwide, with fatigue as the most commonly reported symptom. Recent estimates indicate that 15-20% of Americans with COVID have persisting health issues,[i] up to four million Americans are out of work due to Long COVID symptoms, and that Long COVID has contributed to approximately $1 trillion in lost earnings and $544 billion in increased medical spending.[ii]

"At Axcella, we are gratified and validated that there is a clear path to advance the Long COVID program into a potential registration trial with a leading regulator," said Bill Hinshaw, CEO of Axcella. "This treatment could help millions of people around the world and there are no other agents that have demonstrated impact on the level of fatigue in a controlled trial. Many stakeholders have been eagerly anticipating a regulatory path in this new, important, and widespread disease and it is exciting to have this milestone achieved. With additional resources or collaboration, this program has the potential to advance and quickly reach patients in need."

"We were pleased to have such constructive engagement with the MHRA, who have consistently recognized the urgent needs of patients and the healthcare system in the U.K. and have taken a rigorous and engaged, forward-looking approach to addressing Long COVID," said Margaret Koziel, M.D., Chief Medical Officer of Axcella. "The tenor of the MHRA response and the results of our trial informed our recently completed FDA IND submission for our phase 2b/3 Long COVID trial. AXA1125 is the most advanced clinical-stage program for this devastating disease and we look forward to the opportunity to conduct a global trial that has the potential to rapidly enroll and submit for approval as the leading program in the field."

"The statistically significant improvement in reported mental and physical fatigue among study participants receiving AXA1125 is a very encouraging finding for Long COVID patients, who often experience extreme and constant fatigue throughout their days," added Betty Raman, M.D., Associate Professor of Cardiovascular Medicine at the Radcliffe Department of Medicine, University of Oxford, who led the phase 2a study.

"Given the devastating health and economic impact that Long COVID is having on millions of patients and their families, there is an urgent need for new treatments developed specifically for this population," said Oved Amitay, Chief Executive Officer of Solve M.E., a non-profit organization that serves as a catalyst for critical research into diagnostics, treatments, and cures for myalgic encephalomyelitis/chronic fatigue syndrome (ME/CFS), Long COVID and other post-infection diseases. "We are encouraged by data from Axcella's phase 2 study in Long COVID chronic fatigue patients and the hope that AXA1125 provides to these patients, and by the opportunity to advance the program to a pivotal clinical trial." Amitay, who also is the co-founder of the Long COVID Alliance, a network of patient-advocates, scientists, disease experts, and drug developers focused on educating policy makers and accelerating research into post-viral illnesses, added, "I urge the US government to use all the available mechanisms, including the RECOVER study infrastructure, to advance a clinical study of Axcella's investigational drug in the U.S. as rapidly as possible. Patients are waiting."

Investor Conference Call Information

Axcella will host a live conference call and webcast at 8:00 a.m. ET on Tuesday, January 24, 2023. To access the live conference call, please dial 844-808-7139 (domestic) or 412-902-0127 (international) and refer to "Axcella Health." A webcast of the call will also be available under "Events and Presentations" in the Investors section of the Axcella Health website at https://ir.axcellatx.com/. The archived webcast will be available on Axcella's website approximately two hours after the conference call and will be available for 90 days following the call.

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 for the treatment of Long COVID, NASH, and the reduction in risk of 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 potential utility of AXA1125 as a treatment of Long COVID and the Company's anticipated regulatory pathway for AXA1125 and the timing and potential success thereof. 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 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 AXA1125, the clinical development and safety profile of AXA1125 and its 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.

[i] Bull-Otterson L, Baca S, Saydah S, et al. Post–COVID Conditions Among Adult COVID-19 Survivors Aged 18–64 and ≥65 Years — United States, March 2020–November 2021. MMWR Morb Mortal Wkly Rep 2022;71:713–717. DOI: http://dx.doi.org/10.15585/mmwr.mm7121e1external icon

[ii] Bach K. New data shows long Covid is keeping as many as 4 million people out of work. Brookings Institute. August 24, 2022. https://www.brookings.edu/research/new-data-shows-long-covid-is-keeping-as-many-as-4-million-people-out-of-work/#footnote-3

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