Axcella Therapeutics Announces FDA Fast Track Designation for AXA1125 in NASH

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- *Designation provides the potential for an expedited drug development path*
- *EMMPACT℠ Phase 2b clinical trial enrolling well, with interim data expected in mid-2022*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 14, 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 that the U.S. Food and Drug Administration (FDA) has granted a Fast Track Designation to AXA1125 for the treatment of non-alcoholic steatohepatitis (NASH) with liver fibrosis. AXA1125 is a multi-targeted oral drug candidate that is currently being investigated in the EMMPACT Phase 2b clinical trial in NASH (NCT04880187) and a separate Phase 2a clinical trial in Long COVID (NCT05152849).

"Despite the fact that NASH is an epidemic that is impacting tens of millions of people here in the U.S. alone, there are no approved treatments today," said Axcella President and Chief Executive Officer Bill Hinshaw. "We are pleased that, after reviewing the compelling data from our NASH program including those from two prior clinical studies, the FDA recognized AXA1125's potential to address the needs of patients with this serious and chronic disease. We plan to leverage Fast Track to expedite our development path as we seek to provide an important first-line treatment option."

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. Drugs receiving Fast Track Designation may benefit from:

- More frequent meetings and written communications with FDA to discuss development plans,
- Accelerated Approval and Priority Review, assuming relevant criteria are met, and
- A Rolling Review of the drug's New Drug Application (NDA) submission.

NASH is the most severe form of fatty liver disease that is driven by multifactorial systemic dysregulation of pathways associated with metabolism, inflammation and fibrosis. If left untreated, NASH may ultimately lead to life-threatening conditions such as cirrhosis or liver cancer. According to the Global Liver Institute's U.S. NASH Action Plan, up to 40 million people in the U.S. alone are living with NASH and approximately 10% of U.S. children are afflicted with this disease. Incidence is expected to continue increasing in parallel with the obesity and type 2 diabetes epidemics.

About Axcella Therapeutics (Nasdaq: AXLA)

Axcella is a clinical-stage biotechnology company pioneering a new approach to treat complex diseases using endogenous metabolic modulator (EMM) compositions. The company's product candidates are comprised of EMMs and derivatives that are engineered in distinct combinations and ratios to restore cellular homeostasis in multiple key biological pathways and improve cellular energetic efficiency. Axcella's pipeline includes lead therapeutic candidates in Phase 2 development for the reduction in risk of overt hepatic encephalopathy (OHE) recurrence, the treatment of Long COVID, and the treatment of non-alcoholic steatohepatitis (NASH). 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, the potential for AXA1125 to serve as a first-line treatment option. 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 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.

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