Axcella Announces Publication in The American Journal of Gastroenterology of Positive Results from AXA1125-003 in Subjects With Presumed NASH

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AXA1125 demonstrates meaningful reductions in key measures of liver metabolism, inflammation, and fibrosis

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 16, 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 the publication of results from the company's AXA1125-003 clinical study in *The American Journal of Gastroenterology* entitled "Safety, Tolerability, and Biologic Activity of AXA1125 and AXA1957 in Subjects With Nonalcoholic Fatty Liver Disease." Based on the positive findings from this study, Axcella recently initiated its EMMPACT℠ Phase 2b clinical trial of AXA1125 in patients with biopsy-confirmed nonalcoholic steatohepatitis (NASH).

"As our largest clinical investigation completed to date, AXA1125-003 served as an important confirmation of AXA1125's potential to generate meaningful benefits for patients with NASH utilizing a multi-targeted mechanism," says Dr. Alison Schecter, Axcella's President of Research and Development. "We are pleased to share data in this important publication to broaden awareness about this EMM composition's potential to serve as a compelling first-line treatment for NASH. On behalf of our team, I would like to thank the participants who took part in the study."

AXA1125-003 was a placebo-controlled, randomized, multi-arm clinical study that enrolled 102 subjects with presumed NASH and assessed the impact of AXA1125 and AXA1957 on safety, tolerability and effects on structures and functions of the liver, as measured by a comprehensive panel of imaging and soluble biomarkers related to metabolism, inflammation, and fibrosis. Study subjects were stratified based on the presence or absence of type 2 diabetes.

Results from the study showed that AXA1125 and AXA1957 were generally well-tolerated, with sustained reductions noted for both product candidates versus placebo in key biomarkers of metabolism, inflammation and fibrosis over 16 weeks. Overall, as compared to placebo, AXA1125 demonstrated larger and more consistent reductions in clinically relevant biomarkers than AXA1957. Among subjects receiving AXA1125, 39% achieved a ≥30% relative reduction in liver fat content (MRI-PDFF), 39% achieved a ≥17 U/L reduction in alanine aminotransaminase (ALT; a marker of inflammation), and 35% achieved a ≥80 mSec reduction in corrected T1 (cT1; a marker of fibrosis). Among subjects with type 2 diabetes receiving AXA1125, a greater proportion achieved each of these thresholds. Emerging evidence suggests that these thresholds of activity increase the likelihood of histopathological improvement in NASH subjects. Notably, the above results were seen without impacting mean body weight or serum lipids.

Initiated in April 2021, EMMPACT℠ is an ongoing randomized, double-blind, placebo-controlled, multi-center Phase 2b clinical trial that is evaluating the efficacy and safety of AXA1125 in patients with biopsy-confirmed F2/F3 NASH. Approximately 270 patients are being enrolled and randomized 1:1:1 to receive either 45.2 or 67.8 grams per day of AXA1125 or a matched placebo in two divided doses for 48 weeks, with a four-week safety follow-up period. Patients are stratified based on the presence or absence of type 2 diabetes. Additional information can be found on https://clinicaltrials.gov/ via the identifier NCT04880187.

About Endogenous Metabolic Modulators (EMMs)
EMMs are a broad family of naturally occurring 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 underlying causes of various complex diseases and improve health.

About Axcella's Clinical Development
Each of the clinical investigations that the company has completed to date 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 and when Axcella decides to develop a product candidate as a potential therapeutic, as is the case with AXA1665 and AXA1125, the company will seek an IND to enable the initiation of "clinical trials" in patients.

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 AXA1125 and the company's ability to enroll its EMMPACT clinical trial in a timely manner. 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.

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