Axcella's Late-Breaker Data from AXA1125-003 Clinical Study Presented at the EASL Digital International Liver Congress

August 27, 2020

Poster presentation contains data demonstrating AXA1125's multifactorial effects on markers of metabolism, inflammation and fibrosis in subjects with NAFLD

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 27, 2020-- Axcella (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, announced that data are being presented today in a late-breaker poster regarding key findings from its AXA1125-003 clinical study by Stephen A. Harrison, M.D., Medical Director of Pinnacle Clinical Research in San Antonio, TX, visiting professor of Hepatology at the University of Oxford, UK. The poster presentation is entitled "Multifactorial Effects of AXA1125 and AXA1957 Observed on Markers of Metabolism, Inflammation and Fibrosis: A 16-Week Randomized Placebo-Controlled Study in Subjects With Nonalcoholic Fatty Liver Disease (NAFLD) With and Without Type 2 Diabetes (T2D)."

"Based on the multifactorial effects seen in initial clinical investigations and its well-tolerated profile to date, AXA1125 is among the most intriguing candidates in development for nonalcoholic steatohepatitis (NASH)," said Dr. Harrison. "Notable reductions in virtually all non-invasive biomarkers – including liver fat content (MRI-PDFF), alanine aminotransferase (ALT) and corrected T1 (cT1) – were seen in subjects receiving AXA1125 versus placebo in the 003 clinical study. Interestingly, mean reductions in these measures were even greater among subjects with T2D, providing the potential for a highly differentiated profile within the NASH field."

AXA1125-003 was a placebo-controlled, randomized, multi-arm clinical study assessing the impact of AXA1125 and AXA1957 on safety, tolerability and effects on structures and functions of the liver. A total of 102 adult subjects with presumed NASH were enrolled and dosed in a 2:2:2:1 ratio to receive AXA1125, one of two AXA1957 doses, or placebo administered twice daily for 16 weeks. Study subjects were stratified based on the presence or absence of T2D, with approximately 40 percent of the subjects having T2D.

Highlights from the presentation include:

- The AXA1125 study arm consistently achieved numerically greater reductions from baseline in biomarkers of liver fat and fibroinflammation versus placebo, with AXA1957 showing activity in a number of key biomarkers, but with less consistent directional change than AXA1125
- A reduction from baseline was seen in the AXA1125 arm compared with placebo at weeks 8 and 16 in:
    - Liver fat biomarker MRI-PDFF
    - HOMA-IR
    - ALT
    - CK-18 M65
    - ProC3
    - cT1
- In the AXA1125 arm, 39 percent of subjects achieved a ≥30 percent relative reduction in MRI-PDFF, 39 percent achieved a ≥17 U/L reduction in ALT from baseline, and 54 percent achieved a ≥40 millisecond absolute reduction in cT1
- AXA1125 and AXA1957 were both generally well tolerated in the study, with low rates of discontinuations and with all product-emergent adverse events being mild or moderate

As announced earlier this year in conjunction with Axcella's top-line data announcement , the company plans to advance AXA1125 into a Phase 2b clinical trial in adult NASH in the first half 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 Ongoing Clinical Studies

Each of the company's clinical studies to date are or 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 Axcella decides to further develop a product candidate as a potential therapeutic, as is the case with AXA1665 and AXA1125, any subsequent clinical 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 & 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 and the company's characterization of the results from its clinical studies and future clinical trials, including for AXA1125, the company's anticipated program milestones, including the design, status and timing of the planned Phase 2b clinical trial of AXA1125 in adult NASH, the subject and timing of the company's planned interactions with the FDA, including the timing of IND submissions, and the potential of the company's product candidates to impact health and/or disease, including AXA1125's potential in NASH. 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 IND-enabled 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 studies or IND-enabled clinical trials, other potential impacts of COVID-19 on business and financial results, including with respect to the ability to raise additional capital, make planned interactions and submissions to FDA or other regulatory authorities in a timely manner or at all and operational disruptions or delays, changes in law, regulations, or interpretations and enforcement of regulatory guidance, whether data readouts and/or FDA feedback support the company's IND submission and clinical trial initiation plans and timing, whether and when, if at all, the company's product candidates will receive approval from the FDA or other comparable regulatory authorities, and for which, if any, indications, past results from clinical studies not being representative of future results, 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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