Positive Clinical Data About Axcella's AXA1665 Candidate for Overt Hepatic Encephalopathy Highlighted in Oral Presentation at DDW 2021

May 24, 2021

Activity demonstrated across targeted biologies, including amino acid balance, ammonia handling, muscle function and neurocognition

Presentation includes new subject-specific neurocognition and muscle function data

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 24, 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 that positive data from the company's AXA1665-002 clinical study were presented last night at the Digestive Disease Week (DDW) 2021 Annual Meeting by Dr. Arun Sanyal, Professor in the Virginia Commonwealth University (VCU) Department of Internal Medicine and Education Core Director in the VCU Center for Clinical and Translational Research.

AXA1665-002 was a placebo-controlled, randomized clinical study that investigated the safety, tolerability and physiological impact of AXA1665 in 60 subjects with mild (Child Pugh A) and moderate (Child Pugh B) hepatic insufficiency. Subjects in the study were randomized in a 2:2:1 ratio to receive either 29.4 g or 53.8 g of AXA1665 or a matched placebo in three divided doses per day for 12 weeks with a four-week follow up.

Both doses of AXA1665 were shown to be safe and well tolerated in the study. Additionally, activity was demonstrated across multiple targeted biologies, including amino acid balance, ammonia handling and muscle structure and function. Most importantly, dose dependent, directionally consistent changes were noted across all three psychometric tests that were utilized in AXA1665-002. These included a statistically significant change in the psychometric hepatic encephalopathy score (PHES) for patients receiving the 53.8 g dose of AXA1665 versus placebo. PHES is a highly specific assessment to diagnose hepatic encephalopathy.

The DDW oral presentation included new subject-level data in certain measures of neurocognition and muscle function. Specifically, a positive change in PHES was seen from baseline to week 12 in a dose-proportional manner in subjects receiving AXA1665 compared with placebo, with a majority of subjects in the high dose arm achieving a clinically relevant ≥2 point improvement in PHES. Additionally, while this study primarily enrolled a non-sarcopenic population, a higher proportion of AXA1665 treated subjects (26-40%) versus placebo (14%) achieved a ≥0.3 unit decrease in the liver frailty index. Previous studies suggest that a ≥0.3 reduction in the LFI score may correlate with an improved ability to conduct activities of daily living in subjects with end-stage liver disease.

"Given the clinical evidence supporting the role of muscle dysfunction and sarcopenia in neurocognitive impairment and morbidity in cirrhosis, there is a need for treatments that can provide OHE patients with benefits beyond those provided by conventional approaches," said Dr. Sanyal. "Based on the finding from this most recent clinical study, I believe AXA1665 provides a novel approach to the treatment of OHE and holds the potential to address important unmet needs."

A video recording of Dr. Sanyal's presentation at DDW 2021 is now available in the Publications section of Axcella's website at https://axcellahealth.com/publications/.

Axcella is now initiating a 24-week Phase 2 clinical trial that will compare the 53.8 g/day dose of AXA1665 versus placebo in approximately 150 patients with more advanced liver disease who have experienced at least one prior OHE event and have neurocognitive dysfunction at screening. Additional trial information can be found on https://clinicaltrials.gov/ via the identifier NCT04816916.

About AXA1665 and Overt Hepatic Encephalopathy (OHE)

Hepatic encephalopathy (HE), one of the most common complications of cirrhosis, is a condition involving amino acid imbalance, ammonia toxicity and muscle wasting, all of which contribute to diminished brain function. OHE refers to the presence of neurological abnormalities that are clinically apparent and do not require specialized psychometric testing. OHE is well established as a significant cause of morbidity and mortality in the cirrhotic population and is an area that continues to have unmet medical needs.

AXA1665, Axcella's product candidate for reduction in risk of recurrent OHE, is a composition of eight amino acids and derivatives that is designed to target multiple metabolic pathways intersecting key organ systems, including the liver, muscle and gut. In prior clinical studies, this oral product candidate has been safe, well tolerated and has demonstrated the potential to improve ammonia handling, physical function, amino acid balance and neurocognition with a safe and well tolerated profile. AXA1665 is now in Phase 2 development.

About Axcella's Clinical Studies

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 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 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 AXA1665, the potential for AXA1665 to improve upon the standard of care for OHE patients and address unmet patient needs, and the company's ability to address other complex diseases utilizing EMM compositions. 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 planned clinical trial of AXA1665, other potential impacts of COVID-19 on the company's our business and financial results, including with respect to the company's 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 initiation plans and timing, clinical trial design and target indications for AXA1665, the clinical development and safety profile of AXA1665 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20210524005208/en/

Company Contact
Jason Fredette
jfredette@axcellahealth.com
(857) 320-2236

Source: Axcella