



New Publication Highlights Therapeutic Potential of Endogenous Metabolic Modulators (EMMs)

September 30, 2020

iScience review paper details use of amino acids in medicine

Axcella focused on designing novel EMM compositions with the potential to tackle complex diseases; plans to advance lead candidates into later-stage clinical trials

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 30, 2020-- [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 a new publication detailing the potential for the development of EMM compositions that simultaneously target multiple biological pathways and address unmet needs in a range of complex diseases. The publication in *iScience* is [now available online](#).

Amino acids and related metabolites and precursors are a class of EMMs that have diverse biological functions. Entitled "Endogenous Metabolic Modulators: Emerging Therapeutic Potential of Amino Acids," the new publication reviews literature on the use of this EMM class to treat diseases, with a focus on single and combination agents targeted to a single biology.

"This manuscript sheds light on a wealth of clinical precedents demonstrating the ability to utilize single and simple combinations of EMMs to reprogram biological pathways," said Michael Hamill, Head of Research at Axcella and the publication's lead author. "It also describes how, utilizing a rational design approach, compositions of EMMs provide the potential for compounding multifactorial effects on a range of complex diseases."

Complex diseases, which tend to involve the dysregulation of a variety of underlying pathways and biologies, also are discussed in the publication. These diseases would be best addressed by safe multifactorial treatments rather than combination therapies that can raise the risk of drug-drug interactions and adverse effects.

Axcella's EMM compositions are designed to enable a comprehensive approach, with the potential to simultaneously and safely target multiple pathways underlying diseases and regulate biological processes to foster overall health.

"Individual EMMs have been utilized for decades to drive therapeutic activity with a well-established safety track record," said Bill Hinshaw, President and Chief Executive Officer of Axcella. "At Axcella, we are seeking to harness the full power of EMMs by developing multi-targeted agents with the potential to tackle complex diseases like non-alcoholic steatohepatitis (NASH) and serious complications such as overt hepatic encephalopathy (OHE). Each of our five clinical studies to date investigating multiple product candidates have generated positive results, validating our approach and providing us with confidence in our ability to address unmet needs for patients with a range of diseases. We look forward to furthering our leadership in EMMs as we advance [our product candidates](#) into later-stage clinical trials."

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 Clinical Approach

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 this portion of the company's website, in addition to 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. 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, as well as compositions of EMMs in general, the company's characterization of the results from its clinical studies and future clinical trials, and the potential of the company's product candidates to impact health and/or treat disease, including NASH and OHE. 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 submissions 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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Source: Axcella