



Intellia Therapeutics to Present Updated Interim Data from Ongoing Phase 1/2 Study of NTLA-2002 for the Treatment of Hereditary Angioedema (HAE) at the EAACI Hybrid Congress 2023

May 31, 2023

- *Late-breaking presentation will include new safety, kallikrein reduction and attack rate data across all dose cohorts in the Phase 1 portion of the study*
- *Intellia to host investor webcast on Monday, June 12, at 8 a.m. ET*

CAMBRIDGE, Mass., May 31, 2023 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing potentially curative therapies leveraging CRISPR-based technologies, today announced the acceptance of a late-breaking abstract from the Phase 1 portion of the ongoing NTLA-2002 Phase 1/2 study for a presentation at the European Academy of Allergy and Clinical Immunology (EAACI) Hybrid Congress 2023, taking place June 9-11 in Hamburg, Germany, and virtually. NTLA-2002 is an *in vivo* genome editing candidate designed to inactivate the target gene *kallikrein B1 (KLKB1)* to permanently reduce plasma kallikrein protein levels and activity and thus prevent hereditary angioedema (HAE) attacks after a single-dose treatment. The presentation will include updated safety and efficacy results from the Phase 1 portion of the study across all three dose cohorts (25 mg, 50 mg and 75 mg).

Presentation Details

Title: Updated safety and efficacy of NTLA-2002, a CRISPR/Cas9-based gene editing therapy targeting *KLKB1*, in a Phase 1 study of patients with hereditary angioedema

Session: Flash talks on immune-mediated diseases

Date and Time: Sunday, June 11, 2023, from 2:30 – 3:30 p.m. CET

Presenter: Remy Petersen, M.D., and Ph.D. candidate, Amsterdam University Medical Center

Intellia Therapeutics Investor Webcast Information

Intellia will host a live webcast on Monday, June 12, 2023, at 8:00 a.m. ET to review the new data. Joining the Intellia management team will be Timothy J. Craig, D.O., tenured professor of Medicine, Pediatrics and Biomedical Sciences at Penn State University, to provide an overview of the current treatment landscape and unmet medical need for people living with HAE.

To join the webcast, please visit this [link](#), or the Events and Presentations page of the Investors & Media section of the company's website at www.intelliatx.com. A replay of the webcast will be available on Intellia's website for at least 30 days following the call.

About the NTLA-2002 Clinical Program

Intellia's multi-national Phase 1/2 study is evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of NTLA-2002 in adults with Type I or Type II hereditary angioedema (HAE). This includes the measurement of plasma kallikrein protein levels and activity, as well as HAE attack rate. The Phase 1 portion of the study is an open-label, single-ascending dose design used to identify two dose levels of NTLA-2002 for further evaluation in the Phase 2, randomized, placebo-controlled portion of the study. The Phase 1/2 study will identify the dose of NTLA-2002 for use in future studies. In 2022, Intellia [reported](#) positive interim results from the Phase 1 study demonstrating deep, dose-dependent reductions in plasma kallikrein and robust reductions in patient HAE attacks. Patient screening and dosing in the Phase 2 portion of the study is ongoing. Visit clinicaltrials.gov (NCT05120830) for more details.

About NTLA-2002

Based on Nobel-prize winning CRISPR/Cas9 technology, NTLA-2002 is the first single-dose investigational treatment being explored in clinical trials for the potential to continuously reduce kallikrein activity and prevent attacks in people living with hereditary angioedema (HAE). NTLA-2002 is a wholly owned investigational CRISPR therapeutic candidate designed to inactivate the *kallikrein B1 (KLKB1)* gene, which encodes for prekallikrein, the kallikrein precursor protein. NTLA-2002 is Intellia's second investigational CRISPR therapeutic candidate to be administered systemically, by intravenous infusion, to edit disease-causing genes inside the human body with a single dose of treatment. Intellia's proprietary non-viral platform deploys lipid nanoparticles to deliver to the liver a two-part genome editing system: guide RNA specific to the disease-causing gene and messenger RNA that encodes the Cas9 enzyme, which together carry out the precision editing.

About Hereditary Angioedema

Hereditary angioedema (HAE) is a rare, genetic disorder characterized by severe, recurring and unpredictable inflammatory attacks in various organs and tissues of the body, which can be painful, debilitating and life-threatening. It is estimated that one in 50,000 people are affected by HAE, and current treatment options often include life-long therapies, which may require chronic intravenous (IV) or subcutaneous (SC) administration as often as twice per week, or daily oral administration to ensure constant pathway suppression for disease control. Despite chronic administration, breakthrough attacks still occur. Kallikrein inhibition is a clinically validated strategy for the preventive treatment of HAE attacks.

About Intellia Therapeutics

Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics leveraging

CRISPR-based technologies. To fully realize the transformative potential of CRISPR-based technologies, Intellia is pursuing two primary approaches. The company's *in vivo* programs use intravenously administered CRISPR as the therapy, in which proprietary delivery technology enables highly precise editing of disease-causing genes directly within specific target tissues. Intellia's *ex vivo* programs use CRISPR to create the therapy by using engineered human cells to treat cancer and autoimmune diseases. Intellia's deep scientific, technical and clinical development experience, along with its robust intellectual property portfolio, have enabled the company to take a leadership role in harnessing the full potential of genome editing to create new classes of genetic medicine. Learn more at intelliatx.com. Follow us on Twitter [@intelliatx](https://twitter.com/intelliatx).

Forward-Looking Statements

This press release contains "forward-looking statements" of Intellia Therapeutics, Inc. ("Intellia" or the "Company") within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements include, but are not limited to, express or implied statements regarding Intellia's beliefs and expectations regarding: the safety, efficacy, success and advancement of its clinical program for NTLA-2002 for the treatment of hereditary angioedema pursuant to its clinical trial applications and investigational new drug application and the expected timing of data releases such as the presentation of additional data from the Phase 1 portion of the study at the EAACI Hybrid Congress on June 12, 2023.

Any forward-looking statements in this press release are based on management's current expectations and beliefs of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: risks related to Intellia's ability to protect and maintain its intellectual property position; risks related to Intellia's relationship with third parties, including its licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; and uncertainties related to the authorization, initiation, enrollment and conduct of studies and other development requirements for its product candidates, including NTLA-2002.. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause Intellia's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in Intellia's most recent annual report on Form 10-K as well as discussions of potential risks, uncertainties, and other important factors in Intellia's other filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Intellia undertakes no duty to update this information unless required by law.

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