



Intellia Presents Positive Results from the Phase 2 Study of NTLA-2002, an Investigational In Vivo CRISPR Gene Editing Treatment for Hereditary Angioedema (HAE)

October 24, 2024

- *Deep attack rate reductions achieved in both dose levels tested; a single 50 mg dose resulted in a mean monthly attack rate reduction of 77% and 81% compared to placebo during weeks 1-16 and 5-16, respectively*
- *Eight of 11 patients in the 50 mg arm were completely attack free following a one-time infusion through the latest follow-up; data support NTLA-2002's potential to be a functional cure for hereditary angioedema (HAE)*
- *NTLA-2002 demonstrated an encouraging safety and tolerability profile*
- *Data published in The New England Journal of Medicine and will be presented at the 2024 ACAAI Scientific Meeting*
- *Actively screening patients in the global pivotal Phase 3 HAELO study evaluating the 50 mg dose of NTLA-2002*
- *Intellia to host investor webcast on Thursday, October 24 at 8:30 a.m. ET*

CAMBRIDGE, Mass., Oct. 24, 2024 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies, today announced positive Phase 2 data from the ongoing Phase 1/2 study of NTLA-2002 in patients with hereditary angioedema (HAE), with results continuing to indicate that NTLA-2002 has the potential to eliminate HAE attacks following a one-time infusion. NTLA-2002 is an investigational *in vivo* CRISPR-based gene editing therapy in development as a one-time treatment for HAE, a rare genetic condition that leads to potentially life-threatening swelling attacks. Results were published [online](#) today in *The New England Journal of Medicine* and will be presented on Saturday, October 26 at the 2024 American College of Allergy, Asthma & Immunology (ACAAI) Scientific Meeting in Boston, Massachusetts.

"These positive NTLA-2002 Phase 2 results underscore the tremendous potential of our *in vivo* CRISPR gene editing therapy to be a functional cure and redefine the treatment paradigm for HAE," said Intellia President and Chief Executive Officer John Leonard, M.D. "The Phase 2 data demonstrated that a majority of patients in the 50 mg arm experienced a complete response — no attacks at all and no further treatment needed — after a one-time infusion of NTLA-2002 through the latest follow-up, consistent with the long-term Phase 1 data. We are highly encouraged by these results, which we believe sets NTLA-2002 apart from other prophylaxis treatments. What was previously an unimaginable potential to be free of chronic therapy is one step closer to becoming a reality for the HAE community."

"Approved HAE therapies can reduce but frequently do not eliminate all angioedema attacks and require chronic administration, resulting in a significant treatment burden and a major impact on the quality of life for people living with HAE," said Danny Cohn, M.D., Ph.D., Internist, Department of Vascular Medicine, Amsterdam University Medical Center and the Phase 2 study's lead principal investigator. "These NTLA-2002 Phase 2 data are remarkable, showing this investigational therapy could permanently stop swelling attacks with a single infusion. I am optimistic that NTLA-2002 will change the way we treat HAE and put an end to the need for a lifetime of chronic treatment."

The Phase 2 study is a randomized, double-blind, placebo-controlled study to evaluate the efficacy, safety, pharmacodynamics and pharmacokinetics of NTLA-2002. A total of 27 participants were enrolled and randomized to receive one of two single doses of NTLA-2002 (25 mg or 50 mg) or placebo via intravenous infusion. The data cut-off date for the analysis was April 4, 2024, when the 25th patient completed the 16-week primary observation period.

Single dose of NTLA-2002 led to deep attack rate reductions during the primary observation period. The mean monthly attack rates relative to placebo were reduced by 75% and 77% for the 25 mg and 50 mg arms during weeks 1 – 16, and by 80% and 81% during weeks 5 – 16, respectively. In the 50 mg arm, eight of 11 patients experienced a complete response after a single dose of NTLA-2002, with no attacks at all during the 16-week primary observation period; these eight patients continued to be attack-free through the latest follow-up (median of eight months) and no subsequent treatment has been required. In contrast, four of the 10 patients in the 25 mg arm experienced a complete response and zero patients in the placebo arm. Similarly, patients who received the 50 mg dose achieved a greater kallikrein protein reduction, with an 86% mean reduction from baseline compared to 55% in the 25 mg arm at week 16.

At both dose levels, NTLA-2002 was well tolerated. The most frequent adverse events (AEs) were headache, fatigue and nasopharyngitis. There have been no serious AEs and all AEs were either Grade 1 or 2, except for one patient in the placebo arm who experienced a serious AE of Grade 4 edema of the tongue with breathing impairment that was attributed to their underlying HAE. No clinically significant laboratory abnormalities were observed.

The safety, tolerability and efficacy data from the Phase 2 study are consistent with the long-term Phase 1 data [presented](#) at the European Academy of Allergy and Clinical Immunology (EAACI) Congress in Valencia, Spain on June 2, 2024.

Based on these results, Intellia selected 50 mg for evaluation in the global, pivotal Phase 3 HAELO study, which is actively screening patients. For more information on HAELO (NCT06634420), please visit [clinicaltrials.gov](#).

Intellia Therapeutics Investor Webcast Information

Intellia will host a live webcast, today, October 24, 2024, at 8:30 a.m. ET to discuss the NTLA-2002 Phase 2 data. Joining the Intellia management team will be Dr. Danny Cohn, Internist, Department of Vascular Medicine, Amsterdam University Medical Center, and the Phase 2 study's lead principal investigator as well as Dr. Paula Busse, Professor of Medicine, Division of Clinical Immunology, Icahn School of Medicine at Mount Sinai.

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 NTLA-2002

Based on Nobel-prize winning CRISPR/Cas9 technology, NTLA-2002 has the potential to become the first one-time treatment for hereditary

angioedema (HAE). NTLA-2002 is designed to prevent HAE attacks by inactivating the *kallikrein B1 (KLKB1)* gene, which encodes for prekallikrein, the kallikrein precursor protein. NTLA-2002 has received five notable regulatory designations, including Orphan Drug and RMAT Designation by the U.S. Food and Drug Administration, the Innovation Passport by the U.K. Medicines and Healthcare products Regulatory Agency (MHRA), Priority Medicines (PRIME) Designation by the European Medicines Agency, as well as Orphan Drug Designation by the European Commission.

About Hereditary Angioedema

Hereditary angioedema (HAE) is a rare, genetic disease 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. Although there is no known cure for HAE, there are preventative and on-demand treatment options to help manage the condition, including long- and short-term prophylaxis used to prevent swelling attacks. 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 gene editing company focused on revolutionizing medicine with CRISPR-based therapies. The company's *in vivo* programs use CRISPR to enable precise editing of disease-causing genes directly inside the human body. Intellia's *ex vivo* programs use CRISPR to engineer human cells outside the body for the treatment of cancer and autoimmune diseases. Intellia's deep scientific, technical and clinical development experience, along with its people, is helping set the standard for a new class of medicine. To harness the full potential of gene editing, Intellia continues to expand the capabilities of its CRISPR-based platform with novel editing and delivery technologies. Learn more at intelliatx.com and follow us [@intelliatx](https://www.instagram.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 (HAE) pursuant to its clinical trial applications and investigational new drug application, including the potential for NTLA-2002 to become the first one-time treatment for HAE, the potential for NTLA-2002 to be a functional cure for people living with HAE and the potential of NTLA-2002 to redefine the treatment paradigm for HAE.

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; uncertainties related to the authorization, initiation, enrollment and conduct of studies and other development requirements for its product candidates, including NTLA-2002; the risk that NTLA-2002 will not be successfully developed and commercialized; and the risk that the results of preclinical studies or clinical studies, such as the clinical study of NTLA-2002, will not be predictive of future results in connection with future studies for the same product candidate or Intellia's other product candidates. 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 and quarterly report on Form 10-Q, 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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