



Intellia Therapeutics Receives Priority Medicines (PRIME) Designation From the European Medicines Agency for NTLA-2002, an Investigational In Vivo CRISPR Genome Editing Treatment for Hereditary Angioedema

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CAMBRIDGE, Mass., Oct. 13, 2023 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing potentially curative therapeutics leveraging CRISPR-based technologies, today announced that the European Medicines Agency (EMA) has granted Priority Medicine (PRIME) designation to NTLA-2002 for the treatment of hereditary angioedema (HAE). NTLA-2002 is an *in vivo* CRISPR-based investigational therapy designed to prevent potentially life-threatening swelling attacks in people with HAE.

"We are very pleased the EMA has granted PRIME designation to NTLA-2002. This designation provides valuable regulatory benefits and highlights the potential of our novel *in vivo* gene editing candidate to address an unmet medical need for people living with hereditary angioedema," said Intellia President and Chief Executive Officer John Leonard, M.D. "We look forward to continuing to work closely with the EMA and regulatory agencies around the world to bring this innovative therapy to patients as quickly as possible."

PRIME designation is granted by the EMA to drug candidates that may offer a major therapeutic advantage over existing treatments or that benefit patients without treatment options. The PRIME designation was created by the EMA to provide early and proactive support to developers of promising medicines to optimize their development plans and speed up evaluation. The goal is to help patients benefit as early as possible from innovative new therapies that have demonstrated potential to significantly address unmet medical need.

PRIME designation was granted based on positive interim data from the Phase 1 portion of the ongoing Phase 1/2 clinical trial of NTLA-2002 in patients with HAE. In June, Intellia [announced](#) that across all 10 patients dosed in the Phase 1, a 95% mean reduction in monthly attack rate was observed after a single dose of NTLA-2002 through the latest follow-up. The median duration of follow-up was 9.0 months (range of 5.6 – 14.1 months). At all three dose levels evaluated, NTLA-2002 has been well tolerated, and the majority of adverse events were mild in severity.

PRIME is the fourth specialty regulatory designation Intellia has received for NTLA-2002. NTLA-2002 was also granted Orphan Drug Designation and RMAT Designation by the U.S. Food and Drug Administration (FDA), as well as the Innovation Passport by the U.K. Medicines and Healthcare products Regulatory Agency (MHRA).

About the NTLA-2002 Clinical Program

Intellia's global Phase 1/2 study is evaluating the safety, tolerability, activity, 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. 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 intelliathx.com. Follow us on X (formerly known as Twitter) [@intelliathx](https://twitter.com/intelliathx).

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, including the expected timing of regulatory filings, the initiation and completion of clinical trials, marketing authorization, and commercialization.

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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