Intellia Therapeutics Receives European Union Orphan Drug Designation for NTLA-2002, an Investigational In Vivo CRISPR Genome Editing Treatment for Hereditary Angioedema

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CAMBRIDGE, Mass., Nov. 14, 2023 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing potentially curative single-dose therapeutics leveraging CRISPR-based technologies, today announced that the European Commission (EC) has granted orphan drug 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.

"The European Union orphan drug designation for NTLA-2002 represents another important milestone for Intellia as we continue to make rapid progress in the development of a novel, potential one-time treatment for people with hereditary angioedema," said Intellia President and Chief Executive Officer John Leonard, M.D. "We are on track to complete enrollment of the Phase 2 portion of the study in the coming weeks, which will bring us one step closer to our goal of delivering a potentially life-changing treatment for people who suffer from this serious and debilitating disease."

Orphan drug designation in the European Union (EU) is granted by the EC based on a positive opinion issued by the European Medicines Agency (EMA) Committee for Orphan Medicinal Products. To qualify for orphan drug designation, a candidate therapy must be intended for the treatment, prevention or diagnosis of a life-threatening or chronically debilitating disease that occurs in not more than five in 10,000 people in the EU. The designation provides regulatory, financial and commercial incentives to develop therapies for rare diseases where there are either no satisfactory treatment options or significant benefit to those affected by the disease.

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

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 intelliatx.com. Follow us on X (formerly known as Twitter) @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 its ability to complete enrollment of the Phase 2 portion of the study in the coming weeks, and its ability to deliver a potentially life-changing treatment for people with 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 Phase 1/2 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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