Intellia Therapeutics Receives Authorization to Initiate Phase 1/2 Clinical Trial of NTLA-2002 for the Treatment of Hereditary Angioedema

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- *NTLA-2002 is the first single-dose genome editing therapeutic candidate designed to prevent attacks in people living with HAE to enter clinical study*
- *NTLA-2002 is Intellia's second* in vivo *CRISPR genome editing therapeutic candidate; program to leverage platform insights gained from ongoing development of NTLA-2001 for transthyretin (ATTR) amyloidosis*
- *On track to initiate patient enrollment by year-end*

CAMBRIDGE, Mass., Oct. 06, 2021 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, today announced the authorization of its Clinical Trial Application (CTA) by the New Zealand Medicines and Medical Devices Safety Authority (MEDSAFE) to initiate a Phase 1/2 study evaluating NTLA-2002 for the treatment of adults with hereditary angioedema (HAE). HAE is a 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. NTLA-2002 is a systemically administered single-dose CRISPR/Cas9-based therapeutic candidate designed to inactivate the target gene *Kallikrein B1 (KLKB1)* to permanently reduce plasma kallikrein activity and thus prevent HAE attacks.

"We look forward to initiating this year our first-in-human study of NTLA-2002 for people living with HAE, a debilitating disorder that causes frequent, potentially life-threatening attacks," said Intellia President and Chief Executive Officer John Leonard, M.D. "We believe NTLA-2002 has the potential to be a curative therapy for patients with HAE by providing continuous suppression of plasma kallikrein activity following a single dose and eliminating the significant treatment burden associated with currently available HAE therapies. This study of NTLA-2002 leverages early insights from our ATTR amyloidosis program, where we established proof-of-concept for our modular *in vivo* genome editing platform with interim Phase 1 data earlier this year. The NTLA-2002 program represents the second systemic *in vivo* CRISPR genome editing therapy candidate to enter human clinical trials."

The Phase 1/2 study will evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of NTLA-2002 in adults with Type I or Type II HAE. This includes the measurement of kallikrein protein levels and activity as determined by HAE attack rate measures. The Phase 1 portion of the study is an open-label, single-ascending dose design used to identify up to two dose levels of NTLA-2002 that will be further evaluated in the randomized, placebo-controlled Phase 2 portion of the study. This Phase 1/2 study will identify the dose of NTLA-2002 for use in future studies. More information about the study will be available at clinicaltrials.gov.

Beyond New Zealand, Intellia is submitting additional regulatory applications in other countries as part of its ongoing, multi-national development approach for NTLA-2002.

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 NTLA-2002

Based on Nobel Prize-winning CRISPR/Cas9 technology, NTLA-2002 is potentially the first single-dose treatment 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 Intellia Therapeutics

Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics using CRISPR/Cas9 technology. To fully realize the transformative potential of CRISPR/Cas9, Intellia is pursuing two primary approaches. The company's *in vivo* programs use intravenously administered CRISPR as the therapy, leveraging proprietary delivery technology to enable 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 CRISPR/Cas9 to create new classes of genetic medicine. Learn more at intelliatx.com. Follow us on Twitter @intelliatweets.

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 its: being able to initiate clinical studies for NTLA-2002 for the treatment of hereditary angioedema (“HAE”) pursuant to its clinical trial applications (“CTA”), including enrollment of a first patient by the end of 2021, and submitting similar regulatory applications in other countries; advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, as well as its ability to maintain and expand its related intellectual property portfolio; ability to demonstrate its platform’s modularity and replicate or apply results achieved in preclinical studies, including those in its transthyretin amyloidosis and HAE programs, in any future studies, including human clinical trials; expectations of the potential impact of the coronavirus disease 2019 pandemic on strategy, future operations and timing of its clinical trials or IND submissions; statements regarding the timing of regulatory filings regarding its development programs; use of capital, expenses, future accumulated deficit and other 2021 financial results or in the future; and ability to fund operations beyond the next 24 months.

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 and conduct of studies and other development requirements for its product candidates; the risk that any one or more of Intellia’s product candidates will not be successfully developed and commercialized; the risk that the results of preclinical studies or clinical studies will not be predictive of future results in connection with future studies; and the risk that Intellia’s collaborations with Regeneron or its other collaborations will not continue or will not be successful. 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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