



Intellia Therapeutics Awarded Innovation Passport in the United Kingdom for NTLA-2002, an Investigational Genome Editing Treatment for Hereditary Angioedema

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- *Innovation Passport provides entry to the U.K.'s Innovative Licensing and Access Pathway (ILAP), which aims to accelerate time to market and facilitate patient access to innovative medicines*
- *NTLA-2002 is a single-dose genome editing therapeutic candidate designed to prevent angioedema attacks in people with hereditary angioedema (HAE)*

CAMBRIDGE, Mass., Jan. 11, 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 U.K. Medicines and Healthcare products Regulatory Agency (MHRA) has awarded the Innovation Passport for NTLA-2002, an *in vivo* genome editing candidate being developed for the treatment of hereditary angioedema (HAE). The Innovation Passport is the point of entry into the U.K.'s Innovative Licensing and Access Pathway (ILAP), which is designed to accelerate time to market and facilitate patient access to innovative medicines.

"With the high treatment burden of currently available chronic therapies for hereditary angioedema, we are pleased to receive the ILAP designation, which will enable Intellia to further accelerate the clinical development of NTLA-2002," said Intellia President and Chief Executive Officer John Leonard, M.D. "We expect to begin the Phase 2 portion of the NTLA-2002 clinical study in the first half of this year, and we look forward to working with the U.K. and other regulatory agencies to bring this investigational single-dose genome editing treatment to patients as quickly as possible."

The Phase 1/2 study evaluating NTLA-2002 in adults with Type I or Type II HAE is currently ongoing, with the Phase 2 portion expected to initiate in the first half of 2023.

Delivered in partnership by the All Wales Therapeutics and Toxicology Centre (AWTTC), the Medicines and Healthcare products Regulatory Agency (MHRA), the National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC), the Innovation Passport prioritizes innovative medicines in early clinical development stages for the treatment of diseases with a significant patient or public health need. Benefits of the ILAP include access to a Target Development Profile, which defines key regulatory and development features and creates a roadmap for advancement through regulatory approval.

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 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. 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 intelliadx.com. Follow us on Twitter [@intelliadx](https://twitter.com/intelliadx).

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 ability to conduct, accelerate, and complete clinical studies for NTLA-2002 for the treatment of hereditary angioedema (HAE); its ability to begin the Phase 2 portion of the NTLA-2002 Phase 1/2 clinical study in the first half of 2023; its ability to generate data to demonstrate NTLA-2002 as a potential single-dose treatment for HAE; its ability to maintain and expand its related intellectual property portfolio, and avoid or acquire rights to valid intellectual property of third parties; its ability to replicate or apply results achieved in preclinical and clinical studies, including those in its NTLA-2002 program, in any future studies, including human clinical trials; its ability to develop other *in vivo* or *ex vivo* cell therapeutics of all types, and NTLA-2002 in particular, using CRISPR/Cas9 technology; and the timing of regulatory filings and clinical trial execution, including dosing of patients.

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 the successful enrollment of patients in the Phase 1/2 study for NTLA-2002 for the treatment of HAE; risks related to Intellia’s ability to protect and maintain its intellectual property position; risks related to the authorization, initiation and conduct of studies and other development requirements, including manufacturing, for 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, including for NTLA-2002, will not be predictive of future results in connection with future studies. 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 of 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 (SEC). 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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