Intellia Therapeutics Announces First Patient Dosed in the HAELO Phase 3 Study of NTLA-2002, an Investigational In Vivo CRISPR Gene Editing Treatment for Hereditary Angioedema

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CAMBRIDGE, Mass., Jan. 22, 2025 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies, today announced the first patient has been dosed in the global Phase 3 study of NTLA-2002 for the treatment of hereditary angioedema (HAE). NTLA-2002 is a wholly owned investigational *in vivo* CRISPR-based therapy in development as a single-dose treatment for this potentially life-threatening disease. Intellia expects to complete enrollment in the second half of 2025 and submit a biologics license application (BLA) in 2026 to support the Company's plans for a U.S. launch in 2027.

"We are pleased to have initiated dosing in the HAELO Phase 3 study as we are in our final lap of clinical development for NTLA-2002," said Intellia President and Chief Executive Officer John Leonard, M.D. "With the promising data we've presented thus far, we believe patients could achieve independence from both HAE attacks and medications required to treat this disease. We look forward to presenting longer-term data from the ongoing Phase 1/2 study later this year highlighting the durability of effect of NTLA-2002."

"We are excited to have treated the first patient in the U.S. with a new generation of therapy that could potentially provide patients with lifelong relief from the primary symptoms of HAE," commented Dr. Joshua Jacobs, Medical Director, Allergy and Asthma Clinical Research, Inc.

About the HAELO Study
The pivotal Phase 3 HAELO clinical trial is a randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of NTLA-2002 in 60 adults with Type I or Type II HAE. Patients will be randomized 2:1 to receive a single 50 mg infusion of NTLA-2002 or placebo. Patients randomized to the placebo arm will be eligible for optional crossover to NTLA-2002 at week 28. Key endpoints include the number of HAE attacks and the number of patients who achieve attack-free status from week 5 through week 28. For more information on HAELO (NCT06634420), please visit clinicaltrials.gov.

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, Inc. (NASDAQ:NTLA) is a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies. Since its inception, Intellia has focused on leveraging gene editing technology to develop novel, first-in-class medicines that address important unmet medical needs and advance the treatment paradigm for patients. 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.

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 our clinical program for NTLA-2002 for the treatment of hereditary angioedema (HAE), including its ability to be a single dose treatment for HAE and to offer patients independence from both HAE attacks and medications needed to treat HAE; near-term clinical milestones, including completing enrollment in the HAELO Phase 3 study in the second half of 2025; our interactions with regulatory authorities, including submitting a biologics license application for NTLA-2002 in 2026; our commercialization plans, including a U.S. launch of NTLA-2002 in 2027; and the expected timing and contents of future data releases, including presenting longer-term data from the ongoing Phase 1/2 study later this year highlighting the duration of effect of NTLA-2002.

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 any one or more of Intellia's product candidates, including NTLA-2002, will not be successfully developed and commercialized; the risk that the results of preclinical studies or clinical studies, such as the Phase 1/2 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; and risks related to Intellia's future financial condition and its ability to fund its operations. 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 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.

Intellia Contacts:

Investors:
Precision AQ for Intellia Therapeutics
intelliatx@precisionaq.com

Media:
Matt Crenson
Ten Bridge Communications
media@intelliatx.com
mcrenson@tenbridgecommunications.com

Source: Intellia Therapeutics, Inc.