Intellia Therapeutics Completes Enrollment in the Global Phase 3 HAELO Study of Lonvoguran Ziclumeran (lonvo-z) for Hereditary Angioedema

September 18, 2025

- *Completed patient enrollment within nine months with nearly half enrolled from the U.S.*
- *Expect to report Phase 3 topline data in the first half of 2026*
- *On track to submit BLA in the second half of 2026 for an anticipated U.S. launch in the first half of 2027*
- *Plan to present additional data from the Phase 1/2 study in the fourth quarter of 2025*

CAMBRIDGE, Mass., Sept. 18, 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 it has completed enrollment in the global Phase 3 HAELO study of lonvoguran ziclumeran (lonvo-z) for the treatment of hereditary angioedema (HAE). Topline data are expected in the first half of 2026. Intellia is on track to submit a biologics license application (BLA) in the second half of 2026 to support the company's plans for a U.S. launch in the first half of 2027.

"Completing HAELO enrollment within nine months since dosing the first patient marks a pivotal moment for the company and reflects the degree of unmet need we are hearing from people living with HAE," said Intellia President and Chief Executive Officer John Leonard, M.D. "We are deeply grateful to the patients, their families and the trial investigators for their enthusiasm and look forward to sharing topline results from the Phase 3 study next year. Our momentum remains strong for a planned BLA submission in 2026 as we seek to make this potentially life-changing therapy available to HAE patients starting in the U.S."

"Based on results from the Phase 1/2 study, lonvo-z shows great promise to positively transform the HAE treatment paradigm," said Aleena Banerji, M.D., Professor at Harvard Medical School, Clinical Director of the Allergy and Clinical Immunology Unit at Massachusetts General Hospital, and HAELO principal investigator. "We look forward to seeing the Phase 3 topline data next year."

Intellia dosed the first patient in the pivotal Phase 3 HAELO study in January 2025. The Company announced today it has completed enrollment, with nearly half of the patients enrolled from the U.S. HAELO is a randomized, double-blind, placebo-controlled trial designed to evaluate the efficacy and safety of lonvo-z in at least 60 adults and adolescents aged 16 years and older with Type I or Type II HAE. Patients are randomized 2:1 to receive a single 50 mg infusion of lonvo-z or placebo. Patients are eligible for optional blinded crossover 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), visit clinicaltrials.gov.
Intellia expects to present longer-term data from patients in the Phase 2 portion of the ongoing Phase 1/2 study in the fourth quarter of 2025.

About Lonvo-z
Based on Nobel Prize-winning CRISPR/Cas9 technology, lonvo-z has the potential to become the first one-time treatment for hereditary angioedema (HAE). Lonvo-z is an investigational *in vivo* CRISPR-based gene editing therapy designed to prevent HAE attacks by inactivating the *kallikrein B1* (*KLKB1*) gene, which encodes for prekallikrein, the kallikrein precursor protein. Interim Phase 1/2 clinical data showed dramatic reductions in attack rate, as well as consistent, deep and durable reductions in kallikrein levels. Lonvo-z has received five notable regulatory designations, including Orphan Drug 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), Priority Medicines (PRIME) Designation by the European Medicines Agency, as well as Orphan Drug Designation (ODD) 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. 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 lifelong 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 concerning: the safety, efficacy, success and advancement of its clinical program for lonvoguran ziclumeran or "lonvo-z" (also known as NTLA-2002) for the treatment of hereditary angioedema ("HAE") pursuant to its clinical trial applications and investigational new drug application submissions, including its plan to report Phase 3 topline data in the first half of 2026, its plan to submit a BLA in the second half of 2026, its expectation for a U.S. launch in first half of 2027, its plan to present additional data from the Phase 1/2 study in the fourth quarter of 2025, and the ability of lonvo-z to positively transform the HAE treatment paradigm and potentially be a life-changing therapy for patients 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 contract manufacturers, collaborators, licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; uncertainties related to the conduct of clinical studies and other development requirements for its product candidates, including risks related to the ability to develop and commercialize any one or more of Intellia's product candidates successfully; risks related to the results of preclinical studies or clinical studies not being predictive of future results in connection with future studies; the risk that clinical study results will not be positive; and risks related to the potential delay of planned clinical trials due to regulatory feedback or other developments. 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, including its quarterly report on Form 10-Q. 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:
Brittany Chaves
Senior Manager, Investor Relations
brittany.chaves@intelliatx.com

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

Source: Intellia Therapeutics, Inc.