Intellia Therapeutics Reports Positive Phase 3 Results in Hereditary Angioedema, Marking a Global First for In Vivo Gene Editing

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- *Phase 3 HAELO trial of lonvoguran ziclumeran (lonvo-z) met primary and all key secondary endpoints; favorable safety and tolerability data observed*
- *Single dose of lonvo-z freed most patients from both attacks and ongoing therapy for six-month efficacy evaluation period, demonstrating its potential to be the first and only one-time HAE treatment*
- *Rolling biologics license application (BLA) submission initiated with the U.S. Food and Drug Administration (FDA); anticipate U.S. launch in the first half of 2027, if approved*
- *Intellia to host webcast today at 8:00 a.m. ET*

CAMBRIDGE, Mass., April 27, 2026 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (Nasdaq: NTLA), a leading biopharmaceutical company focused on revolutionizing medicine leveraging CRISPR gene editing and other core technologies, today announced positive topline results from the global Phase 3 HAELO clinical trial of lonvo-z (formerly known as NTLA-2002) in hereditary angioedema (HAE). HAE is a rare genetic condition in which patients experience recurrent and potentially life-threatening swelling (angioedema) attacks in various parts of their body, including the face, upper airway, abdomen and extremities due to an overproduction of bradykinin. Designed as a one-time treatment that is administered in an outpatient setting, lonvo-z is an *in vivo* CRISPR gene editing candidate that is intended to inactivate the *kallikrein B1* (*KLKB1*) gene to permanently lower kallikrein and bradykinin levels.

Intellia separately announced today that it has initiated a rolling BLA submission to the FDA to seek regulatory approval. The company is preparing for a potential U.S. launch of lonvo-z in the first half of 2027.

“As the first Phase 3 data reported for an *in vivo* gene editing therapy, today’s HAELO results represent a profound milestone for Intellia, the broader CRISPR and precision medicine fields and, most importantly, the HAE community,” said John Leonard, M.D., Intellia President and Chief Executive Officer. “For those patients who have spent years battling unpredictable breakthrough swelling attacks, anxiety about their next attack or the many burdens associated with chronic prophylactic treatment, lonvo-z represents a potential paradigm shift in treatment. These data affirm lonvo-z’s potential, with one dose, to offer prolonged freedom from both attacks and the need for ongoing therapy.”

“We extend our deep gratitude to the many patients, caregivers and clinicians who have helped advance gene editing science by participating in our clinical trials. It is because of their contribution that we are advancing toward our first potential approval, with the goal of making lonvo-z available to U.S. patients in the first half of 2027,” Dr. Leonard concluded.

HAELO Topline Results
HAELO is a randomized, double-blind, placebo-controlled Phase 3 trial designed to evaluate the efficacy and safety of a one-time 50 milligram dose of lonvo-z in adults and adolescents aged 16 years and older with Type I or Type II HAE. Key endpoints of the trial focused on the number of HAE attacks experienced by patients, quality of life, safety and tolerability.

A total of 80 patients were enrolled, with 52 receiving lonvo-z and 28 receiving placebo. Of the total population, 49% of patients were enrolled in the United States and 71% were on long-term prophylaxis (LTP) therapy at study entry. Patients on LTP were required to discontinue those therapies in the weeks prior to dosing.

Key findings from HAELO include:

- The trial met its primary endpoint. For the six-month efficacy evaluation period (weeks 5 to 28), a one-time infusion of lonvo-z reduced attacks by 87% versus placebo, with a mean monthly attack rate of 0.26 in the lonvo-z arm compared with 2.10 in the placebo arm ($p<0.0001$).
- The trial met all of its key secondary endpoints with statistical significance ($p<0.0001$). These included a 62% rate of patients who were entirely attack free and therapy free in the lonvo-z arm for the six-month efficacy evaluation period, compared with 11% of patients in the placebo arm.
- Favorable safety and tolerability data were observed for lonvo-z. The most common treatment emergent adverse events (TEAEs) during the primary observation period (infusion through week 28) were infusion-related reactions, headache and fatigue. All TEAEs reported as of the data cutoff (February 10, 2026) were mild or moderate and there were no serious adverse events observed in the lonvo-z arm.
- As of the data cutoff, all patients who received lonvo-z at baseline or in crossover after week 28 remained LTP free.

Additional clinical data from HAELO will be presented at the 2026 European Academy of Allergy and Clinical Immunology Congress (EAACI), taking place June 12-15 in Istanbul, Türkiye (abstract #100217).

“Despite the availability of several HAE treatments, many patients continue to experience significant burdens related to the disease, including breakthrough attacks and challenges associated with chronic treatment,” said Aleena Banerji, M.D., Professor at Harvard Medical School, Director of Clinical Care, Center for Drug and Vaccine Allergy at Massachusetts General Hospital, and a HAELO principal investigator. “The results we are seeing from lonvo-z demonstrate its potential to eliminate the need for chronic medication and related challenges. If approved as a one-time treatment, I would expect lonvo-z to be an appealing option for many patients.”

Webcast Information
The company will host a conference call and webcast today at 8:00 a.m. ET to discuss the topline results. To join the webcast, please visit the Events page of the Investors & Media section on Intellia's website at intelliatx.com. A replay of the webcast will be available for approximately 90 days.

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 *in vivo* CRISPR gene editing candidate that is intended to permanently lower kallikrein by inactivating the *kallikrein B1* (*KLKB1*) gene with a single dose. Lonvo-z has received five notable regulatory designations: 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 biopharmaceutical company focused on revolutionizing medicine leveraging CRISPR gene editing and other core technologies. The company's mission is to transform the lives of people with severe diseases by developing and commercializing potentially curative treatments. With deep scientific, technical and clinical development experience, Intellia aims to reset the standard for medicine by durably treating the root causes of disease. 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 success and advancement of its program for lonvoguran ziclumeran or "lonvo-z" (also known as NTLA-2002) for the treatment of hereditary angioedema ("HAE"), including its plan to complete the submission of a biologics license application ("BLA") for lonvo-z in the second half of 2026, its expectations regarding review and approval of that BLA, and its expectations regarding a potential U.S. launch of lonvo-z in the first half of 2027; and the potential of one dose of lonvo-z to offer prolonged freedom from both attacks and the need for ongoing therapy.

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: uncertainties related to the conduct of clinical studies and other development and commercialization requirements for its product candidates, including lonvo-z, including risks related to the ability to develop and successfully commercialize lonvo-z or any of Intellia's product candidates; 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; 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 or regulatory filings 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.

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