Intellia Therapeutics Reports Additional Positive Phase 3 Results for Lonvoguran Ziclumeran (Ionvo-z) in Patients with Hereditary Angioedema

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- *Data from HAELO Phase 3 clinical trial presented today in a late-breaking oral session at European Academy of Allergy & Clinical Immunology Annual Congress 2026*
- *HAELO manuscript simultaneously published in the* New England Journal of Medicine

CAMBRIDGE, Mass., June 13, 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 presented additional positive results from the global Phase 3 HAELO clinical trial of lonvo-z (formerly NTLA-2002) for hereditary angioedema (HAE) in a late-breaking oral presentation at the European Academy of Allergy & Clinical Immunology (EAACI) Annual Congress 2026 in Istanbul, Türkiye. Results from the trial were simultaneously published in the *New England Journal of Medicine*. The presentation and publication can be accessed from the Scientific Publications and Presentations section of intelliatx.com.

As previously announced, HAELO met its primary endpoint with an 87% reduction (p<0.0001) in mean monthly attacks in the lonvo-z arm vs. the placebo arm during the efficacy evaluation period (weeks 5 to 28). In addition, 62% of patients in the lonvo-z arm were entirely attack free and therapy free for the six-month efficacy evaluation period, compared with 11% of patients in the placebo arm (p<0.0001), a key secondary endpoint. Today, Intellia reported data for the trial's other key secondary endpoints:

Key Secondary Endpoint	**Lonvo-z Arm (N=52)**	**Placebo Arm (N=28)**
Monthly rate of attacks requiring on-demand treatment Weeks 5-28, mean (95% CI)	0.19 (0.10, 0.36)	1.79 (1.27, 2.54)
	89% reduction (79%, 94%), p<0.0001	
Monthly rate of moderate/severe attacks Weeks 5-28, mean (95% CI)	0.11 (0.06, 0.23)	1.23 (0.84, 1.81)
	91% reduction (81%, 96%), p<0.0001	
Change from baseline to Week 28 in AE-QoL total score, mean (95% CI)	-23.51 (-27.64, -19.38)	-6.47 (-12.26, -0.68)
	-17.04 improvement (-24.15, -9.93), p<0.0001	

AE-QoL: Angioedema Quality of Life score, which is a validated, angioedema-specific patient-reported outcome measure with a lower score indicating improved quality of life. A 6-point reduction is considered to be a clinically important improvement in AE-QoL.

CI: Confidence interval

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) that were higher in the lonvo-z group compared to placebo were infusion-related reaction, headache, fatigue, back pain, and upper respiratory tract infection. All reported TEAEs were mild or moderate and there were no serious adverse events observed in the lonvo-z arm.

"These are the first Phase 3 results to deliver on the much-heralded promise of *in vivo* CRISPR gene editing," said John Leonard, M.D., Intellia President and Chief Executive Officer. "Regardless of age or prior use of long-term prophylaxis therapies, it was observed that a single lonvo-z treatment significantly reduced HAE attacks for all patients during the efficacy evaluation period, with all patients remaining LTP free as of the data cutoff. We thank the many patients, physicians and caregivers who participated in HAELO and are excited to be advancing this highly differentiated candidate toward a potential approval."

Danny Cohn, M.D., Ph.D., Internist, Department of Vascular Medicine, Amsterdam Cardiovascular Sciences, Amsterdam University Medical Center, and a HAELO principal investigator, added, "As a clinician who has witnessed patients struggle with the unpredictability and emotional toll of HAE, the prospect of offering lasting freedom from attacks and chronic medication with a one-time treatment is incredibly exciting. These results give me confidence that many patients will soon have the potential to enjoy a normal life."

Today's presentation and publication also included supplemental demographics, data and analyses, including:

- A time plot showing that the mean monthly attack rate for patients receiving lonvo-z through the data cutoff (February 10, 2026) was well below the reported rate in prescreening while patients were receiving standard-of-care therapy;
- Patient-level data demonstrating that all patients in the lonvo-z arm experienced attack-rate reductions from baseline during weeks 5 to 28;
- An analysis showing that meaningful attack-rate reductions were observed for all evaluated subgroups;
- A breakdown showing that 20% of the patients who enrolled in HAELO reported having complete disease control (no attacks) as their best response to prior long-term prophylaxis therapies; and

- A plasma kallikrein time plot showing that protein levels decreased substantially by the first measurement (day 15), reached a steady state by week 5 and remained stable through the data cutoff.

A rolling biologics license application (BLA) submission for lonvo-z was initiated in April with the U.S. Food and Drug Administration (FDA). The company continues to anticipate regulatory approval and a U.S. launch in the first half of 2027.

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" (formerly 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, 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 become the first one-time treatment for HAE and to permanently lower kallikrein by inactivating the *kallikrein B1* (*KLKB1*) gene with a single dose.

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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