Intellia Therapeutics Announces Positive Three-Year Data from Phase 1 Trial of Lonvoguran Ziclumeran (lonvo-z) in Patients with Hereditary Angioedema (HAE) at the European Academy of Allergy and Clinical Immunology Congress

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- *With up to three years of follow-up, a single dose of lonvo-z led to a 98% mean reduction in monthly HAE attack rate in all 10 patients*
- *All 10 patients were attack-free and treatment-free for a median of 23 months through the latest follow-up, demonstrating the potential of lonvo-z to become the first one-time therapy for most HAE patients*
- *Lonvo-z was well tolerated and continues to demonstrate a favorable safety profile*
- *The global Phase 3 HAELO trial of lonvo-z has concluded screening ahead of schedule with more than half screened from U.S. sites; Intellia to provide an update on enrollment in the future*

CAMBRIDGE, Mass., June 15, 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 three-year follow-up data from the Phase 1 portion of the ongoing Phase 1/2 study in patients with HAE after receiving a single dose of lonvoguran ziclumeran (lonvo-z, also known as NTLA-2002). Results were shared in an oral presentation at the European Academy of Allergy and Clinical Immunology (EAACI) Congress 2025, held June 13-16 in Glasgow, United Kingdom.

“Today’s results underscore the promising potential of Intellia’s approach to gene editing therapy – a one-time treatment that was well tolerated and offered a highly differentiated, durable effect for patients suffering from a serious disease,” said Intellia President and Chief Executive Officer John Leonard, M.D. “Seeing all 10 patients in the Phase 1 portion of this study free from both HAE attacks and chronic therapy at nearly two years of median follow-up is incredibly encouraging. These data fuel our optimism for the outcomes of our ongoing Phase 3 HAELO study, which we expect to report in the first half of 2026, and highlight the strong value we believe it will offer patients, physicians and payers.”

“People living with HAE often report a reduced quality of life because they worry about the likelihood of their next attack, either because they still experience attacks or are reminded of it by their use of chronic therapy,” said Dr. Joshua Jacobs, Medical Director, Allergy and Asthma Clinical Research, Inc. “Based on the data, it is reasonable to expect lonvo-z could offer patients the potential to be free from both physical HAE attacks and the burden of managing chronic HAE treatment.”

In the Phase 1 portion of the study, a one-time dose of 25 mg (N=3), 50 mg (N=4) or 75 mg (N=3) of lonvo-z was administered via intravenous infusion and plasma kallikrein protein levels were measured along with HAE attacks. At the time of the February 12 data cutoff, patients were attack-free and treatment-free for a median of nearly two years. With up to three years of follow-up, a single dose of lonvo-z led to a mean reduction in monthly HAE attack rate of 98% over the study period, compared to pre-treatment baseline. For all 10 patients, deep, dose-dependent and durable reductions in plasma kallikrein protein continued to be observed through the latest assessment.

Safety
Across all three dose levels, lonvo-z has been well tolerated and continues to demonstrate a favorable safety profile consistent with earlier data presented at EAACI in 2024. The most frequent adverse events during the study period were infusion-related reactions (IRRs). IRRs were mostly Grade 1 and resolved with all patients receiving the full dose. With up to 3 years of follow-up, no treatment-emergent serious adverse events were observed, and no treatment-related adverse events were observed during the period following 28 days after dosing.

Clinical Development Plans
Intellia’s global Phase 3, randomized, double-blind, placebo-controlled HAELO trial is ongoing to assess the safety and efficacy of lonvo-z at the 50 mg dosage. The Company announced today the HAELO trial has successfully completed screening ahead of schedule, with over half of the patients being screened in the United States. The study is no longer recruiting and Intellia will provide an update on enrollment in the future. New and longer-term data from the Phase 2 portion of the ongoing Phase 1/2 study is planned to be presented in the second half of 2025. Intellia expects to submit a biologics license application (BLA) in 2026 to support the Company’s plans for a U.S. launch in 2027. For more information on HAELO (NCT06634420), please visit clinicaltrials.gov.

About the Lonvoguran Ziclumeran (lonvo-z, also known as NTLA-2002) Clinical Program
Intellia’s ongoing Phase 1/2 study is evaluating the safety and efficacy of lonvo-z in adults with Type I or Type II hereditary angioedema (HAE). The Phase 1 portion of the study is an international, open-label study designed to identify the dose level of lonvo-z selected for further evaluation in the Phase 2 portion of the study. Enrollment in both portions of the Phase 1/2 study is complete. Intellia dosed the first patient in the global Phase 3, randomized, double-blind, placebo-controlled HAELO trial in January of 2025. Visit clinicaltrials.gov (NCT05120830) for more details.

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 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 programs for lonvoguran ziclumeran or "lonvo-z" (also known as NTLA-2002) for hereditary angioedema ("HAE"), including the ability to successfully complete its global Phase 3 HAELO study; its expectation to present additional data regarding lonvo-z, including reporting outcomes of the Phase 3 HAELO study in the first half of 2026 and presenting new and longer-term data from the Phase 2 portion of the ongoing Phase 1/2 study of lonvo-z in the second half of 2025; and its expectation to be able to support a biologics license application for lonvo-z for the treatment of HAE by 2026 for a U.S. launch in 2027.

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, licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; risks related to Intellia's ability to protect and maintain its intellectual property position; risks related to valid third party intellectual property; 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 regulatory agencies' evaluation of regulatory filings and other information related to our product candidates, including lonvo-z; uncertainties related to the authorization, initiation and conduct of studies and other development requirements for our product candidates, including uncertainties related to regulatory approvals to conduct clinical trials, including our ability to complete the Phase 3 HAELO study for HAE; the risk that any one or more of Intellia's product candidates, including lonvo-z, will not be successfully developed and commercialized; and the risk that the results of preclinical studies or clinical studies will not be predictive of future results in connection with future studies for the same product candidate or Intellia's other product candidates. 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 of Form 10-K and 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.

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