Intellia Therapeutics Announces FDA Acceptance of Biologics License Application with Priority Review for Lonvoguran Ziclumeran (Lonvo-z) for Hereditary Angioedema (HAE)

September 8, 2026

- *FDA sets Prescription Drug User Fee Act (PDUFA) date of March 10, 2027*
- *Positions lonvo-z to be the world's first* in vivo *CRISPR-based therapy and the only one-time HAE treatment, if approved*

CAMBRIDGE, Mass., Sept. 08, 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 the U.S. Food and Drug Administration (FDA) has accepted the Biologics License Application (BLA) for lonvo-z and granted the BLA Priority Review with a PDUFA target action date of March 10, 2027. Additionally, FDA has advised the company that it is not currently planning to hold an advisory committee to discuss the application. If approved, lonvo-z would be the world's first *in vivo* CRISPR-based therapy and the only one-time treatment for HAE.

"Today marks an important milestone for the patients we are committed to serving and for Intellia's pioneering work in the field of *in vivo* gene editing," said John Leonard, M.D., Intellia President and Chief Executive Officer. "Backed by compelling Phase 3 data, we believe lonvo-z could fundamentally change the way HAE is treated and are excited by its potential to become the world's first approved *in vivo* CRISPR-based therapy. With the FDA's Priority Review underway, our team is well prepared to deliver this one-time treatment to patients who are waiting for new options."

Joshua Jacobs, M.D., Medical Director, Allergy and Asthma Clinical Research, Inc., and a HAELO trial investigator, added, "HAE is an unpredictable disease that can be responsible for profound disability and place patients at risk for fatal attacks. Today's announcement is exciting because it advances us one step closer to potentially having a one-time treatment option available for patients who continue to be burdened by this chronic disease."

The BLA is supported by positive data from Intellia's global Phase 3 HAELO clinical trial, which was fully enrolled with 80 patients in just nine months and was 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 1 or Type 2 HAE. HAELO met its primary and all key secondary endpoints, demonstrating an 87% reduction ($p<0.0001$) in mean monthly attacks for lonvo-z compared with placebo during the efficacy evaluation period (weeks 5 to 28). In addition, 62% of patients in the lonvo-z arm were entirely attack free and HAE therapy free for the six-month efficacy evaluation period, compared with 11% of patients in the placebo arm ($p<0.0001$). As of the February 10, 2026 data cutoff, all patients who received lonvo-z at baseline or in crossover after week 28 remained free from long-term prophylaxis therapy.

Favorable safety and tolerability data were observed for lonvo-z as of the data cutoff. The most common treatment emergent adverse events during the primary observation period (infusion through week 28) that were higher in the lonvo-z group compared to placebo were infusion-related reactions, headache, fatigue, back pain, and upper respiratory tract infection. All reported treatment emergent adverse events were mild or moderate and there were no serious adverse events observed in the lonvo-z arm.

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 that is administered in an outpatient setting. Lonvo-z has received five notable regulatory designations: Orphan Drug and Regenerative Medicine Advanced Therapy (RMAT) Designations 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
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 may 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 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 known as NTLA-2002) for the treatment of hereditary angioedema ("HAE"), including its expectations regarding review and approval of its biologics license application ("BLA") for lonvo-z, such as whether the FDA will hold an advisory committee to discuss the BLA and the timing of such review and approval based on the Prescription Drug User Fee Act ("PDUFA") target action date of March 10, 2027 for the BLA; its belief that lonvo-z could fundamentally change the way HAE is treated and has the potential to become the world's first approved *in vivo* CRISPR-based therapy; and its

expectations regarding its preparations for and the potential success of the commercial launch of lonvo-z, if approved.

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 review and approval of the BLA for lonvo-z and 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 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 recent 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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