Intellia Therapeutics Initiates Rolling Submission of Biologics License Application to FDA for Lonvoguran Ziclumeran (lonvo-z) as a One-Time Treatment for Hereditary Angioedema

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Expect to complete BLA submission in second half of 2026; anticipate launch in first half of 2027, if approved

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 it has initiated a rolling submission of a biologics license application (BLA) to the U.S. Food and Drug Administration (FDA) seeking approval of lonvo-z (formerly known as NTLA-2002) for hereditary angioedema (HAE). 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 also separately announced positive topline data today from the Phase 3 HAELO clinical trial of lonvo-z in HAE. The trial met its primary and all key secondary endpoints, demonstrating that a one-time dose of lonvo-z led to freedom from both HAE attacks and the use of ongoing therapy for most patients during the six-month primary observation period.

"If approved, lonvo-z will become the world's first *in vivo* CRISPR-based gene editing therapy," said John Leonard, M.D., Intellia President and Chief Executive Officer. "The promising results from HAELO reinforce our conviction that lonvo-z could revolutionize how HAE is treated for many patients, with the potential to free most of them from both attacks and the need for ongoing therapy with just one dose. We look forward to our continued engagement with the FDA as we seek to ease many of the burdens for people living with HAE."

Pursuant to the Regenerative Medicine Advanced Therapy (RMAT) designation that the FDA granted to lonvo-z for the treatment of HAE, a rolling BLA allows Intellia to submit portions of the BLA on an ongoing basis and provides the FDA with an opportunity to expedite its review. In addition to the RMAT program, Intellia participated in the FDA's Chemistry, Manufacturing, and Controls (CMC) Development and Readiness Pilot. This program allows sponsors to discuss their CMC product development strategies and goals with FDA review staff and address their questions. The increased communication is intended to help sponsors complete CMC activities to expedite their drug development programs to support application submission and earlier patient access.

Intellia anticipates completing its BLA submission in the second half of 2026. If the filing is accepted by the FDA, the agency is expected to determine if it will grant a priority review and provide a target action date to complete its evaluation. If approved, Intellia plans to launch lonvo-z commercially 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" (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, including the potential for expedited review, its expectations regarding a planned U.S. launch of lonvo-z in first half of 2027, and the possibility that lonvo-z to revolutionize how HAE is treated for many patients with the potential to free most from both attacks and the need for ongoing therapy with just one 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 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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