Intellia Therapeutics Announces FDA Lift of Clinical Hold on MAGNITUDE-2 Phase 3 Clinical Trial in ATTRv-PN

January 27, 2026

- *Plan to resume MAGNITUDE-2 patient enrollment and dosing*
- *FDA engagement ongoing regarding clinical hold on MAGNITUDE Phase 3 clinical trial in ATTR-CM*

CAMBRIDGE, Mass., Jan. 27, 2026 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies, today announced that the U.S. Food and Drug Administration (FDA) has removed the clinical hold on the Investigational New Drug application (IND) for the MAGNITUDE-2 Phase 3 clinical trial of nexiguran ziclumeran (nex-z) for patients with hereditary transthyretin amyloidosis with polyneuropathy (ATTRv-PN).

"We appreciate the FDA's expeditious review of our submission and ongoing engagement and thank our study investigators and patients for their continued participation. With the clinical hold for MAGNITUDE-2 lifted, our team is focused on resuming patient enrollment as quickly as possible as we seek to advance this potential one-time treatment option for people living with ATTRv-PN," said Intellia President and Chief Executive Officer John Leonard, M.D.

Intellia's engagement with FDA is ongoing regarding the clinical hold on the IND for the MAGNITUDE Phase 3 clinical trial of nex-z for patients with transthyretin amyloidosis with cardiomyopathy (ATTR-CM). The company plans to provide an update once alignment has been achieved on the path forward for this program.

The clinical holds on the INDs for MAGNITUDE and MAGNITUDE-2 were imposed by the FDA on October 29, 2025, following the observation of Grade 4 liver transaminases and increased total bilirubin in a patient who was dosed with nex-z in MAGNITUDE that met the trial's protocol-defined pausing criteria. The company has aligned with the FDA on certain study modifications and mitigation measures related to MAGNITUDE-2 that include enhanced safety monitoring of liver laboratory tests. The company is engaged with clinical trial investigators, ethics committees, international regulatory authorities and other stakeholders to resume enrollment activities in MAGNITUDE-2 as quickly as possible.

MAGNITUDE-2 is a randomized, double-blind, placebo-controlled trial evaluating the efficacy and safety of nex-z in patients with ATTRv-PN. As part of the protocol amendment, Intellia has increased the trial's target enrollment from approximately 50 patients to approximately 60 patients with ATTRv-PN. The primary endpoints of the study are a change in modified neuropathy impairment score and a change in serum TTR levels. Adult patients with ATTRv-PN are randomized 1:1 to receive a single 55 mg infusion of nex-z or placebo. For more information on MAGNITUDE-2 (NCT06672237), please visit clinicaltrials.gov.

About Nex-z
Based on Nobel Prize-winning CRISPR/Cas9 gene editing technology, nex-z has the potential to become the first one-time treatment for transthyretin (ATTR) amyloidosis with cardiomyopathy (ATTR-CM) and/or hereditary ATTR with polyneuropathy (ATTRv-PN). Nex-z is designed to inactivate the TTR gene that encodes for the transthyretin (TTR) protein and is being investigated in MAGNITUDE and MAGNITUDE-2, Phase 3 clinical trials in ATTR-CM and ATTRv-PN, respectively. Interim Phase 1 clinical data showed the administration of nex-z led to consistent, deep and long-lasting TTR reduction. Nex-z has received an Orphan Drug and RMAT Designation from the U.S. Food and Drug Administration (FDA) and an Orphan Drug Designation (ODD) from the European Commission. Intellia leads development and commercialization of nex-z as part of a multi-target discovery, development and commercialization collaboration with Regeneron Pharmaceuticals, Inc.

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 regarding: the safety, tolerability, efficacy, success and advancement of its clinical programs for nexiguran ziclumeran or "nex-z" (also known as NTLA-2001), including the ability to engage with clinical trial investigators, ethics committees, international regulatory authorities and other stakeholders to resume enrollment activities in its MAGNITUDE-2 trial for nex-z in ATTRv-PN as quickly as possible, the ability to successfully complete the MAGNITUDE-2 trial, the ability to align with FDA and to resume and successfully complete its MAGNITUDE trial for nex-z in ATTR-CM, and the potential of nex-z to provide a one-time treatment option for people living with ATTRv-PN.
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 Intellia's ability to resume the MAGNITUDE and MAGNITUDE-2 trials; regulatory agencies' evaluation of regulatory filings and other information related to our product candidates, including nex-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; the risk that any one or more of Intellia's product candidates, including nex-z, will not be successfully developed and commercialized; 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; 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; and risks

related to Intellia's reliance on collaborations, including that its collaboration with Regeneron Pharmaceuticals, Inc. will not continue or will not be successful. 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 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.

Intellia Investor & Media Contact:
Jason Fredette
Vice President, Investor Relations and Corporate Communications
Intellia Therapeutics, Inc.
jason.fredette@intelliatx.com

Source: Intellia Therapeutics, Inc.