Intellia Therapeutics Announces FDA Regenerative Medicine Advanced Therapy (RMAT) Designation Granted to Nexiguran Ziclumeran (nex-z) for the Treatment of Transthyretin (ATTR) Amyloidosis with Cardiomyopathy

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CAMBRIDGE, Mass., March 26, 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 that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to nexiguran ziclumeran (nex-z, also known as NTLA-2001) for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy (ATTR-CM).

“This is a meaningful step forward in our efforts to accelerate the development of nex-z to bring this potentially transformative treatment to patients as quickly as possible,” said Intellia President and Chief Executive Officer John Leonard, M.D. “In both cardiomyopathy and polyneuropathy, nex-z has been shown, after a single dose, to lead to unprecedented rapid, durable and consistent reductions in serum TTR, which positively impacts markers of disease progression.”

The RMAT designation was established under the 21st Century Cures Act to expedite the development and review of promising therapeutic candidates, including genetic therapies, that are intended to treat, modify, reverse or cure a serious or life-threatening disease. RMAT designation includes benefits, such as early interactions with the FDA, including discussions on surrogate or intermediate endpoints that could potentially support accelerated approval and satisfy post-approval requirements, and potential priority review of a product’s biologics license application (BLA).

Nex-z has been granted Regenerative Medicine Advanced Therapy designations by the U.S. FDA for both cardiomyopathy and polyneuropathy. Nex-z has also been granted Orphan Drug Designation by the U.S. FDA and European Commission.

About nexiguran ziclumeran (nex-z, also known as NTLA-2001)
Based on Nobel Prize-winning CRISPR/Cas9 technology, nex-z has the potential to become the first one-time treatment for transthyretin (ATTR) amyloidosis. Nex-z is an investigational *in vivo* CRISPR-based therapy designed to inactivate the *TTR* gene that encodes for the transthyretin (TTR) protein. Interim Phase 1 clinical data showed the administration of nex-z led to consistent, deep and long-lasting TTR reduction. Intellia leads development and commercialization of nex-z as part of a multi-target discovery, development and commercialization collaboration with Regeneron.

About Transthyretin (ATTR) Amyloidosis
Transthyretin amyloidosis, or ATTR amyloidosis, is a rare, progressive and fatal disease. Hereditary ATTR (ATTRv) amyloidosis occurs when a person is born with mutations in the TTR gene, which causes the liver to produce structurally abnormal transthyretin (TTR) protein with a propensity to misfold. These damaged proteins build up as amyloid in the body, causing serious complications in multiple tissues, including the heart, nerves and digestive system. ATTRv amyloidosis predominantly manifests as polyneuropathy (ATTRv-PN), which can lead to nerve damage, or cardiomyopathy (ATTRv-CM), which can lead to heart failure. Some individuals without the genetic mutation produce non-mutated, or wild-type TTR proteins that become unstable over time, misfolding and aggregating in disease-causing amyloid deposits. This condition, called wild-type ATTR (ATTRwt) amyloidosis, primarily affects the heart. There are an estimated 50,000 people worldwide living with ATTRv amyloidosis and between 200,000 and 500,000 people with ATTRwt amyloidosis. There is no known cure for ATTR amyloidosis and currently available medications are limited to slowing accumulation of misfolded TTR protein.

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, efficacy, success and advancement of its clinical program for nexiguran ziclumeran (nex-z, also known as NTLA-2001) for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy (ATTR-CM) pursuant to its clinical trial applications and investigational new drug application, including the potential of NTLA-2001 to be a transformative treatment for patients with ATTR-CM; and the expected timing of regulatory filings, completion of clinical trials, submission of marketing authorizations, and commercialization.

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 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; and uncertainties related to the authorization, initiation, enrollment and conduct of studies and other development requirements for its product candidates, including nex-z; risks related to the results of preclinical or clinical studies, including that they may not be positive or predictive of future results; the risk that one or more of Intellia’s product candidates, including nex-z, will not be successfully developed and commercialized; and risks related to Intellia’s reliance on collaborations, including that its collaboration with Regeneron 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 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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