Intellia Therapeutics to Present New Clinical Data from the Phase 1 Study of nexiguran ziclumeran (nex-z) for the Treatment of Transthyretin (ATTR) Amyloidosis at the 2024 AHA Scientific Sessions

October 1, 2024

- *New data to be presented will include biomarkers of disease progression and functional capacity from the ongoing Phase 1 study of nex-z, an investigational* in vivo *CRISPR gene editing therapy for ATTR amyloidosis*

CAMBRIDGE, Mass., Oct. 01, 2024 (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 interim data from the ongoing Phase 1 study of nexiguran ziclumeran (nex-z, also known as NTLA-2001) will be presented in a late-breaking oral presentation at the 2024 American Heart Association (AHA) Scientific Sessions, taking place November 16 – 18 in Chicago, Illinois. Nex-z is an investigational *in vivo* CRISPR-based gene editing therapy designed to be a single-dose treatment for transthyretin (ATTR) amyloidosis. Intellia leads development and commercialization of nex-z as part of a multi-target discovery, development and commercialization collaboration with Regeneron. The presentation will include safety, reduction in serum TTR and biomarkers of disease progression, and functional capacity data in patients with ATTR amyloidosis with cardiomyopathy (ATTR-CM).

Presentation Details

Title: Nexiguran ziclumeran (nex-z, also known as NTLA-2001), an investigational *in vivo* CRISPR-based therapy for patients with transthyretin amyloidosis with cardiomyopathy (ATTR-CM): interim report of the Phase 1 study

Session: Late-Breaking Science 1: Celebrating a Century of Cardiovascular Science: From Prevention to Treatment, to Cure

Session Type: Late-Breaking Science

Date and Time: Saturday, November 16, 2024, from 8:30-9:45 a.m. CST

Presenter: Marianna Fontana, M.D., Ph.D., Professor of Cardiology and Honorary Consultant Cardiologist, University College London Centre for Amyloidosis, London, UK

Location: Main Event I

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 designed to inactivate the *TTR* gene that encodes for the transthyretin (TTR) protein. Nex-z is the first investigational CRISPR therapy to be administered systemically to edit genes inside the human body. 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. The company's *in vivo* programs use CRISPR to enable precise editing of disease-causing genes directly inside the human body. Intellia's *ex vivo* programs use CRISPR to engineer human cells outside the body for the treatment of cancer and autoimmune diseases. 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 transthyretin ("ATTR") amyloidosis pursuant to its clinical trial applications and investigational new drug submission, including its potential to become the first one-time treatment for ATTR amyloidosis; and the expected timing of data releases, including the oral presentation planned for November 16 at the 2024 American Heart Association (AHA) Scientific Sessions.

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 licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; uncertainties related to the authorization, initiation, enrollment and conduct of studies and other development requirements for its product candidates, including nex-z; the risk that any one or more of Intellia's product candidates, including nex-z, will not be successfully developed and commercialized; the risk that the results of preclinical studies or clinical studies, such as the Phase 1 clinical study of nex-z, 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 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.

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Source: Intellia Therapeutics, Inc.