Intellia Therapeutics to Present Longer-Term Data from the Ongoing Phase 1 Clinical Trial of Nexiguran Ziclumeran (nex-z) for the Treatment of Hereditary Transthyretin (ATTR) Amyloidosis with Polyneuropathy

September 22, 2025

Longer-term data to be presented at the 5th International ATTR Amyloidosis Meeting for Patients and Doctors on September 25 highlighting up to three years of patient follow-up and additional insight into ATTRv-PN disease-relevant measures

CAMBRIDGE, Mass., Sept. 22, 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 longer-term data from the ongoing Phase 1 trial of investigational nex-z for the treatment of hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN) will be presented at the 5th International ATTR Amyloidosis Meeting for Patients and Doctors, taking place September 25-26 in Baveno, Italy. This presentation will include ATTRv-PN disease-relevant measures, including up to three years of patient follow-up.

Presentation Details:

Title: Efficacy and Safety of Nexiguran Ziclumeran, an Investigational CRISPR/Cas9 Gene Editing Treatment: 24-Month Follow-Up from a Phase 1 Study in Patients with Hereditary ATTR with Polyneuropathy
Session: New Perspectives in ATTR Amyloidosis Treatment
Date and Time: Thursday, September 25, 2025, from 3:52 - 4:04 p.m. CEST
Presenter: Julian Gillmore, M.D, Ph.D., FRCP, FRCPath, Professor of Medicine, National Amyloidosis Centre, UCL Division of Medicine, Royal Free Hospital, U.K., the trial's U.K. national coordinating investigator

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. Nex-z is 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 Pharmaceuticals, Inc.

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.

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