Intellia Therapeutics to Present the First-Ever Clinical Data From Patients Redosed with an Investigational In Vivo CRISPR Gene Editing Therapy at the Peripheral Nerve Society Annual Meeting 2024

June 17, 2024

- Data to offer insight into the safety and pharmacodynamics of redosing patients with a systemically delivered LNP-based CRISPR gene editing candidate

CAMBRIDGE, Mass., June 17, 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 the acceptance of an abstract featuring redosing data from the Phase 1 study of NTLA-2001 has been selected for an oral presentation at the Peripheral Nerve Society Annual Meeting, taking place June 22 – 25 in Montreal, Canada. NTLA-2001 is an investigational *in vivo* CRISPR-based gene editing therapy designed to be a single-dose treatment for transthyretin (ATTR) amyloidosis.

In the dose-escalation portion of the Phase 1 study, the initial three patients received the lowest dose of 0.1 mg/kg and subsequently received a follow-on dose of 55 mg. These data will be the first-ever clinical data from patients redosed with an *in vivo* CRISPR-based gene editing candidate and provide insight on the safety and pharmacodynamic effect. While repeat dosing is not planned for the NTLA-2001 program for ATTR amyloidosis, a redosing option could be an important advantage of Intellia's non-viral, lipid nanoparticle (LNP)-based delivery platform for future investigational therapies where a target additive effect is desired.

Presentation Details

Title: Activity of Follow-On Dosing for an Investigational *In Vivo* CRISPR-Based LNP Therapy in Transthyretin Amyloidosis

Session: Richard A.C. Hughes Symposium – Clinical Highlights – *Late Breaking Abstracts and Clinical Trials*

Date and Time: Tuesday, June 25, 2024, from 3:30 p.m. – 3:45 p.m. EST

Presenter: Jorg Taubel, M.D., FFPM, FESC, Visiting Professor at King's College London and Chief Executive Officer of Richmond Pharmacology

About NTLA-2001
Based on Nobel Prize-winning CRISPR/Cas9 technology, NTLA-2001 has the potential to become the first one-time treatment for transthyretin (ATTR) amyloidosis. NTLA-2001 is designed to inactivate the *TTR* gene that encodes for the transthyretin (TTR) protein. NTLA-2001 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 NTLA-2001 led to consistent, deep and long-lasting TTR reduction. Intellia leads development and commercialization of NTLA-2001 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 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 ability to redose Intellia's investigational therapies, including where a target additive effect is desired.

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 NTLA-2001; the risk that any one or more of Intellia's product candidates, including NTLA-2001, 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 NTLA-2001, 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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