Intellia Therapeutics to Present Updated Interim Clinical Data from Ongoing Phase 1 Study of NTLA-2001 for the Treatment of Transthyretin (ATTR) Amyloidosis at the International Liver Congress™ 2022

June 8, 2022

- *Oral presentation to include additional durability data from patients treated with NTLA-2001, the first-ever systemically administered* in vivo *CRISPR investigational therapy*
- *Presentation to include data supporting fixed dose selection in the ongoing single-dose expansion cohort of polyneuropathy arm*

CAMBRIDGE, Mass., June 08, 2022 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing potentially curative therapies leveraging CRISPR-based technologies, today announced that interim clinical data from the Phase 1 study of NTLA-2001, an investigational therapy in development for the treatment of transthyretin (ATTR) amyloidosis, will be shared in an oral presentation at the European Association for the Study of the Liver's International Liver Congress™ 2022, taking placeJune 22 – 26 in London.

"We are pleased to be presenting longer-term, follow-up data from the first-in-human study of NTLA-2001, which is designed to potentially halt progression and reverse ATTR amyloidosis," said Intellia President and Chief Executive Officer John Leonard, M.D. "We look forward to sharing interim data we believe demonstrates the potential of our CRISPR-based investigational therapy to be a single-dose treatment that provides a deep and durable response."

The presentation will include new data from the ongoing Phase 1 study evaluating NTLA-2001 in people living with hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN). These include additional results from all four dose-escalation cohorts in Part 1, highlighting the durability of response after a single dose of NTLA-2001. Data supporting the fixed dose selection for Part 2, the single-dose expansion cohort of the polyneuropathy arm, will also be presented.

The presentation will build upon interim data presented in February, which showed significant reductions in serum TTR levels from baseline in a dose-dependent manner in 15 patients with ATTRv-PN. At the highest dose tested, 1.0 mg/kg, the mean and maximum serum TTR reductions were 93% and 98%, respectively, by day 28 across the six patients treated. The Company also previously announced it is now evaluating a fixed dose of 80 mg in Part 2 of the Phase 1 study, which is expected to deliver a similar exposure to the 1.0 mg/kg dose in the ATTRv-PN target population, and that the first patient has been dosed in the expansion cohort in the polyneuropathy arm. Intellia also continues to dose patients in the cardiomyopathy arm of the Phase 1 study, which is evaluating NTLA-2001 in dose-escalation cohorts of people with ATTR amyloidosis with cardiomyopathy (ATTR-CM).

Presentation Details

Title: *In vivo* CRISPR/Cas9 editing of the *TTR* gene with NTLA-2001 in patients with transthyretin amyloidosis – dose selection considerations

Session: Rare liver diseases (including paediatric and genetic)

Date and Time: Friday, June 24, 2022 from 10:00-11:30 a.m. BST

Presenter: Dr. Edward J. Gane, MBChB, MD, FRACP, MNZM, Professor of Medicine at the University of Auckland, New Zealand and Chief Hepatologist, Transplant Physician and Deputy Director of the New Zealand Liver Transplant Unit at Auckland City Hospital, the trial's national investigator in New Zealand

Intellia Therapeutics Investor Event and Webcast Information

Intellia will host a live webcast on Friday, June 24, 2022 at 8:00 a.m. ET to review the presented data. To join the webcast, please visit this link, or the Events and Presentations page of the Investors & Media section of the company's website at www.intelliatx.com. A replay of the webcast will be available on Intellia's website for at least 30 days following the call.

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.

About NTLA-2001
Based on Nobel Prize-winning CRISPR/Cas9 technology, NTLA-2001 could potentially be the first single-dose treatment for ATTR amyloidosis. NTLA-2001 is the first investigational CRISPR therapy candidate to be administered systemically, or through a vein, to edit genes inside the human

body. Intellia's proprietary non-viral platform deploys lipid nanoparticles to deliver to the liver a two-part genome editing system: guide RNA specific to the disease-causing gene and messenger RNA that encodes the Cas9 enzyme, which carries out the precision editing. Robust preclinical data, showing deep and long-lasting transthyretin (TTR) reduction following *in vivo* inactivation of the target gene, supports NTLA-2001's potential as a single-administration therapeutic. Intellia leads development and commercialization of NTLA-2001 as part of a multi-target discovery, development and commercialization collaboration with Regeneron. The global Phase 1 trial is an open-label, multi-center, two-part study of NTLA-2001 in adults with hereditary transthyretin amyloidosis with polyneuropathy (ATTRv-PN) or transthyretin amyloidosis with cardiomyopathy (ATTR-CM). Visit clinicaltrials.gov (NCT04601051) for more details.

About Intellia Therapeutics
Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics leveraging CRISPR-based technologies. To fully realize the transformative potential of CRISPR-based technologies, Intellia is pursuing two primary approaches. The company's *in vivo* programs use intravenously administered CRISPR as the therapy, in which proprietary delivery technology enables highly precise editing of disease-causing genes directly within specific target tissues. Intellia's *ex vivo* programs use CRISPR to create the therapy by using engineered human cells to treat cancer and autoimmune diseases. Intellia's deep scientific, technical and clinical development experience, along with its robust intellectual property portfolio, have enabled the company to take a leadership role in harnessing the full potential of genome editing to create new classes of genetic medicine. Learn more at intelliatx.com. Follow us on Twitter @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 concerning: its ability to successfully extend its leadership position and harness the full potential of genomic medicines to bolster its genome editing capabilities and pipeline; the safety, efficacy, success and advancement of its clinical programs for NTLA-2001 for the treatment of transthyretin amyloidosis pursuant to its clinical trial applications ("CTA"), including the expected timing of data releases, regulatory filings, and the initiation, enrollment, and completion of clinical trials; the expansion of its CRISPR/Cas9 technology and related technologies to advance additional development candidates and timing expectations of advancing such development candidates; its ability to maintain and expand its related intellectual property portfolio; expectations of the potential impact of the coronavirus disease pandemic, including the impact of any variants, on strategy, future operations and timing of its clinical trials; its ability to optimize the impact of its collaborations on its development programs, including, but not limited to, its collaboration with Regeneron Pharmaceuticals, Inc.

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 and conduct of studies and other development requirements for its product candidates, including uncertainties related to regulatory approvals to conduct clinical trials; the risk that any one or more of Intellia's product candidates will not be successfully developed and commercialized; the risk that the results of preclinical studies or clinical studies will not be predictive of future results in connection with future studies; the risk that clinical study results will not be positive; and the risk that Intellia's collaborations with Regeneron or its other collaborations 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 ("SEC"). 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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