Intellia Therapeutics to Present Interim Clinical Data from Ongoing Phase 1 Study of NTLA-2001 for the Treatment of Transthyretin (ATTR) Amyloidosis at the 2021 Peripheral Nerve Society Annual Meeting

June 4, 2021

- *Data to offer insight into safety and pharmacodynamics of NTLA-2001, the first-ever systemically administered* in vivo *CRISPR therapy candidate*
- *Late-breaking abstract selected for oral presentation on June 26*

CAMBRIDGE, Mass., June 04, 2021 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA) today announced that a late-breaking abstract featuring interim Phase 1 clinical data from NTLA-2001, the Company's lead CRISPR/Cas9 *in vivo* therapy in development as a single-dose, systemically administered treatment for transthyretin (ATTR) amyloidosis, has been selected for an oral presentation at the 2021 Peripheral Nerve Society (PNS) Annual Meeting, taking place this month.

The presentation will include interim data from the ongoing dose-escalation portion of Intellia's Phase 1 trial evaluating NTLA-2001 in people living with hereditary ATTR amyloidosis with polyneuropathy (hATTR-PN). Following safety assessment and dose optimization, Intellia intends to further evaluate NTLA-2001 in a broader population of people with ATTR amyloidosis, including those with cardiomyopathy.

"These findings represent Intellia's first clinical data readout and an important step forward in our commitment to develop breakthrough genome editing treatments for people living with severe diseases, such as transthyretin (ATTR) amyloidosis," said Intellia President and Chief Executive Officer John Leonard, M.D. "By knocking out the disease-causing gene, NTLA-2001 is designed to halt progression and potentially reverse the disease with a single dose, offering the potential of meaningful improvement over the standard of care, which requires chronic, lifelong administration. These interim data will provide a view of NTLA-2001's safety and activity profile as the dose-escalation portion of our study progresses, as well as insight into the promise of our modular platform to develop other systemically delivered *in vivo* CRISPR/Cas9 therapies across a range of diseases with unmet need."

Presentation Details
Title: "In vivo *CRISPR/Cas9 Editing of the TTR Gene by NTLA-2001 in Patients with Transthyretin Amyloidosis*"
Session: Platform Session II
Date and Time: Saturday, June 26, 2021 from 11:15-11:30 a.m. E.T.
Presenter: Dr. 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 national coordinating investigator

Intellia Therapeutics Investor Event and Webcast Information
Intellia will host a live webcast on Monday, June 28, 2021 at 8:00 a.m. E.T. 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 the NTLA-2001 Clinical Program
Intellia's global Phase 1 trial is an open-label, multi-center, two-part study of NTLA-2001 in adults with hereditary transthyretin amyloidosis with polyneuropathy (hATTR-PN). The trial's primary objectives are to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of NTLA-2001. Patients receive a single dose of NTLA-2001 via intravenous administration. The study will enroll up to 38 participants (ages 18-80 years) and consists of a single-ascending dose phase in Part 1 and, following the identification of an optimal dose, an expansion cohort in Part 2. Visit clinicaltrials.gov (NCT04601051) for more details.

Enrollment is ongoing at global clinical trial sites, as Intellia is submitting additional regulatory applications in other countries as part of its development strategy. After completion of the Phase 1 trial, the company is planning to rapidly move to pivotal studies for both polyneuropathy and cardiomyopathy patients.

About NTLA-2001
Based on Nobel Prize-winning CRISPR/Cas9 technology, NTLA-2001 could potentially be the first curative treatment for ATTR. NTLA-2001 is the first experimental CRISPR therapy candidate to be administered systemically, or through a vein, to edit genes inside the human body. Intellia's proprietary non-viral platform utilizes lipid nanoparticles designed to deliver to the liver a simple, two-part genome editing system: guide RNA specific to the disease-causing gene and messenger RNA that encodes the Cas9 protein. Our robust preclinical data showing deep and long-lasting transthyretin (TTR) reduction following knockout of the target gene *in vivo* support NTLA-2001's potential as a single-administration therapeutic. NTLA-2001 is part of a co-development/co-promotion agreement between Intellia, the lead development and commercialization party, and Regeneron Pharmaceuticals, Inc.

About Transthyretin (ATTR) Amyloidosis
Transthyretin amyloidosis, or ATTR, is a rare, progressive and fatal disease. Hereditary ATTR (hATTR) amyloidosis occurs when a person is born with DNA mutations in the *TTR* gene, which causes the liver to produce transthyretin (TTR) protein in a misfolded form and build up in the body. hATTR amyloidosis can predominantly manifest as polyneuropathy (hATTR-PN), which can lead to nerve damage, and cardiomyopathy (hATTR-CM), which involves heart muscle disease that can lead to heart failure. In addition, non-mutated, or wild-type TTR protein, can also accumulate in the body, leading to wild-type ATTR (wtATTR) amyloidosis. There are an estimated 50,000 hATTR patients worldwide and between 200,000 and 500,000 people with wtATTR.

About Intellia Therapeutics
Intellia Therapeutics is a leading clinical-stage genome editing company, focused on the development of proprietary, potentially curative therapeutics

using the CRISPR/Cas9 system. Intellia believes the CRISPR/Cas9 technology has the potential to transform medicine by both producing therapeutics that permanently edit and/or correct disease-associated genes in the human body with a single administration, and creating enhanced engineered cells that can treat oncological and immunological diseases. Intellia's combination of deep scientific, technical and clinical development experience, along with its leading intellectual property portfolio, puts it in a unique position to unlock broad therapeutic applications of the CRISPR/Cas9 technology and create new classes of therapeutic products. Learn more about Intellia and CRISPR/Cas9 at intelliatx.com. Follow us on Twitter @intelliatweets.

Forward-Looking Statements
This press release contains "forward-looking statements" of Intellia Therapeutics, Inc. ("Intellia", "we" or "our") 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 its: ability to enroll and dose the necessary subjects in the clinical studies for NTLA-2001 for the treatment of transthyretin ("ATTR") amyloidosis, provide timing on data readouts from the clinical studies, and successfully secure additional clinical studies authorizations, such as investigational new drug applications ("IND") and clinical trial applications ("CTA"), in other countries; ability to evaluate NTLA-2001 in a broader ATTR population; expectation that clinical results will support NTLA-2001's safety and activity profile; belief that NTLA-2001 can be approved as a single-dose therapy or that it can halt and reverse ATTR progression; plans to present data at upcoming scientific conferences; advancement and expansion of our CRISPR/Cas9 technology to develop breakthrough genome editing treatments for people living with severe diseases; ability to demonstrate our platform's modularity and replicate or apply results achieved in preclinical studies, including those in our ATTR program, in any future studies, including human clinical trials; ability to optimize the impact of our collaborations on our development programs, including but not limited to our collaboration with Regeneron Pharmaceuticals, Inc. ("Regeneron"); statements regarding the timing of regulatory filings and clinical trial execution, including dosing of patients, regarding our development programs; and potential commercial opportunities, including value and market, for our product candidates.

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 our ability to protect and maintain our intellectual property position; risks related to our relationship with third parties, including our licensors and licensees; risks related to the ability of our licensors to protect and maintain their intellectual property position; uncertainties related to regulatory agencies' evaluation of regulatory filings and other information related to our product candidates; uncertainties related to the authorization, initiation and conduct of studies and other development requirements for our product candidates; the risk that any one or more of our product candidates, including those that are co-developed, 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; and the risk that our collaborations with Regeneron or our other *ex vivo* 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 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 ("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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