Intellia Therapeutics' Investigational CRISPR Treatment NTLA-2001 Receives European Union Orphan Drug Designation for ATTR Amyloidosis

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CAMBRIDGE, Mass., March 30, 2021 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, announced today that the European Commission (EC) has granted orphan drug designation to NTLA-2001. This investigational medicinal product is being developed as a treatment for transthyretin amyloidosis (ATTR), a rare condition that can impact a number of organs and tissues within the body through the accumulation of misfolded transthyretin (TTR) protein deposits. NTLA-2001 is the first experimental CRISPR therapy – a Nobel Prize-winning technology – to be administered systemically via intravenous dosing to edit a gene, specifically the *TTR* gene, that encodes the production of TTR protein, inside the human body. In addition to being the first therapy of its kind, it also has the potential to be the first curative treatment and it may be able to halt and reverse ATTR progression.

"This news is a significant milestone for NTLA-2001 and the ATTR patient community," said Intellia President and Chief Executive Officer John Leonard, M.D. "We are pleased that the EC recognizes the potential significant benefit of NTLA-2001 in the treatment of patients with this debilitating disease where there is no cure. We look forward to advancing the global development of this genome editing product in collaboration with Regeneron."

This decision by the EC follows the initiation of Intellia's global Phase 1 study to evaluate NTLA-2001 for hereditary ATTR with polyneuropathy (hATTR-PN). The trial aims to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of NTLA-2001. Following safety assessment and dose optimization, Intellia intends to further evaluate NTLA-2001 in both polyneuropathy and cardiomyopathy patients. Orphan drug designation is granted to therapies that are intended for the treatment, prevention, or diagnosis of life threatening or chronically debilitating rare diseases where there are either no treatments or no satisfactory therapeutic options. The designation provides regulatory, financial and commercial incentives to develop therapies for rare diseases defined as having a prevalence of less than five in 10,000 people in the European Union.

About NTLA-2001
NTLA-2001 is the first experimental CRISPR therapy to be administered systemically, or through a vein, to edit a gene 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. Intellia's 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 dose of treatment. 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 Amyloidosis (ATTR)
Transthyretin amyloidosis, or ATTR, is a rare, progressive and fatal disease. Hereditary ATTR (hATTR) occurs when a person is born with a specific DNA mutation in the *TTR* gene, which causes the liver to produce a protein called transthyretin (TTR) in a misfolded form and build up in the body. hATTR can manifest as polyneuropathy (hATTR-PN), which can lead to nerve damage, or 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). 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 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 treatment course, 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" 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 its: being able to complete clinical studies for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR") pursuant to its clinical trial applications ("CTA"), including submitting additional regulatory applications in other countries; advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, as well as its ability to maintain and expand its related intellectual property portfolio; expectations of the potential impact of the coronavirus disease 2019 pandemic on strategy, future operations and timing of its clinical trials or IND submissions; ability to optimize the impact of its collaborations on its development programs, including but not limited to its collaborations with Regeneron, including its co-development programs for ATTR; and statements regarding the timing of regulatory filings regarding its development programs.

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; 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; 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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