



Intellia Therapeutics Doses First Patient in Landmark CRISPR/Cas9 Clinical Trial of NTLA-2001 for the Treatment of Transthyretin Amyloidosis

November 9, 2020

NTLA-2001: First single-course therapy that potentially halts and reverses ATTR progression

First-ever in vivo CRISPR treatment delivered intravenously to a patient

CAMBRIDGE, Mass., Nov. 9, 2020 — Intellia Therapeutics, Inc. (NASDAQ:NTLA), announced that the first patient has been treated with NTLA-2001, which the company is developing as a single-course, potentially curative therapy for transthyretin amyloidosis (ATTR). Intellia's global Phase 1 study is to evaluate NTLA-2001 for hereditary ATTR with polyneuropathy (hATTR-PN). Following safety assessment and dose optimization, Intellia intends to further evaluate NTLA-2001 in a broader ATTR patient population of both polyneuropathy and cardiomyopathy patients.

"With today's news, we're entering a new era of potential genome editing cures for patients," said Intellia's President and Chief Executive Officer John Leonard, M.D. "Once we've assessed safety and established an optimal dose, we intend to rapidly initiate trials for the clinical manifestations of ATTR. NTLA-2001 may halt and reverse ATTR progression by producing a deeper, permanent TTR protein reduction for all patients – regardless of disease type – than the chronically administered treatments currently available."

"Only a few short years ago, there were no treatments available for this devastating disease," said Carlos Heras-Palou, M.D., founder and president of the United Kingdom ATTR Amyloidosis Patients Association (UKATPA), who also has hATTR. "Now, a cure for ATTR utilizing the groundbreaking CRISPR/Cas9 gene editing technology may be within reach."

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 consist 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 a clinical trial site in the U.K. Intellia is submitting additional regulatory applications in other countries as part of its ongoing, global development strategy. The company is planning to rapidly move to pivotal studies enrolling 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 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 course 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 DNA mutations 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 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). 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 Therapeutics and CRISPR/Cas9 at intelliatx.com. Follow us on Twitter [@intelliatweets](https://twitter.com/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") and submitting similar 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; ability to demonstrate its platform's modularity and replicate or apply results achieved in preclinical studies, including those in its ATTR, HAE, hemophilia A and hemophilia B programs, in any future studies, including human clinical trials; 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, hemophilia A and hemophilia B; statements regarding the timing of regulatory filings regarding its development programs; use of capital, expenses, future accumulated deficit and other 2020 financial results or in the future; and ability to fund operations at least through the next 24 months.

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. 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.

Intellia Contacts:

Media:

Jennifer Mound Smoter
Senior Vice President
External Affairs & Communications
+1 857-706-1071
jenn.smoter@intelliatx.com

Lynnea Olivarez
Director
External Affairs & Communications
+1 956-330-1917
lynnea.olivarez@intelliatx.com

Investors:

Lina Li
Associate Director
Investor Relations
+1 857-706-1612
lina.li@intelliatx.com

###