Intellia Therapeutics Announces U.S. FDA Acceptance of Investigational New Drug Application for NTLA-5001, its CRISPR/Cas9-Engineered TCR-T Cell Candidate for Acute Myeloid Leukemia

September 16, 2021

- *NTLA-5001 is Intellia's first* ex vivo *candidate using its proprietary cell engineering process for the treatment of cancer to enter clinical study*
- *NTLA-5001 targets Wilms' Tumor 1 (WT1), an overexpressed intracellular antigen on many hematologic malignancies and solid tumors*
- *Initiation of patient screening in Phase 1/2a study of NTLA-5001 expected by year-end*

CAMBRIDGE, Mass., Sept. 16, 2021 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, today announced that the U.S. Food and Drug Administration (FDA) has accepted the investigational new drug (IND) application for NTLA-5001, the company's first wholly-owned *ex vivo* CRISPR genome editing candidate for the treatment of cancer. NTLA-5001 is an autologous T cell receptor (TCR)-T cell therapy engineered to target the Wilms' Tumor (WT1) antigen for the treatment of all genetic subtypes of acute myeloid leukemia (AML). Intellia intends to initiate patient screening by year-end in a Phase 1/2a study evaluating NTLA-5001 in adults with persistent or recurrent AML who have previously received first-line therapy.

"The FDA's acceptance of our IND for NTLA-5001 is an important milestone in our pursuit of developing advanced cell therapies utilizing Intellia's proprietary engineering platform to treat patients with cancer. NTLA-5001 is our first wholly-owned *ex vivo* candidate to enter the clinic, and we expect to initiate this first-in-human study in adults with AML by year-end. Our treatment strategy is to leverage CRISPR/Cas9 genome editing technology to create next-generation engineered immune cells with the potential to attack cancer cells more effectively and safely than previously developed cell therapies," said Intellia President and Chief Executive Officer John Leonard, M.D. "Our study is an important first step toward improving treatment for people living with this aggressive form of cancer. AML is the most common type of acute leukemia in adults, that, despite currently available treatments, has a five-year survival rate of less than 30 percent."

The Phase 1/2a study will evaluate the safety, tolerability, cell kinetics and anti-tumor activity of a single dose of NTLA-5001 in adults who have detectable AML after having received standard first-line therapy. The study will contain a dose escalation and expansion phase, with up to 54 participants. The dose-escalation phase of the study will include two independent arms of up to three cohorts: Arm 1 will consist of adults with AML with lower disease burden, defined as those with less than 5% AML blasts in bone marrow, while Arm 2 will consist of adults with AML with higher disease burden, defined as those greater than or equal to 5% AML blasts in bone marrow. Once a dose is identified in each arm, two expansion cohorts will be opened for further safety assessment. More information about the study will be available at clinicaltrials.gov.

In addition to the U.S., Intellia has also submitted a regulatory application to the U.K. for NTLA-5001.

About Acute Myeloid Leukemia

Acute myeloid leukemia (AML) is a cancer of the blood and bone marrow that is rapidly fatal without immediate treatment. It is the most common type of acute leukemia in adults, with more than 20,000 estimated new cases in 2020. Despite currently available treatments for AML, the five-year overall survival rate for patients remains less than thirty percent. AML, along with other cancer types, is often characterized by overexpression of the Wilms' Tumor 1 (WT1) antigen.

About NTLA-5001

NTLA-5001 is a CRISPR/Cas9-engineered T cell receptor (TCR)-T cell therapy in development for the treatment of all genetic subtypes of acute myeloid leukemia (AML). This autologous cell therapy candidate is designed for AML patients with the HLA-A*02:01 allele whose tumors carry the Wilms' Tumor 1 (WT1) antigen, which is widely overexpressed in AML and other cancers. NTLA-5001 is Intellia's first wholly-owned *ex vivo* therapeutic candidate, developed using its proprietary cell engineering platform for the treatment of cancer. Based on preclinical results, Intellia believes its proprietary cell engineering platform will result in a pipeline of more efficacious and safer cell-based cancer therapies.

About Intellia Therapeutics

Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics using CRISPR/Cas9 technology. To fully realize the transformative potential of CRISPR/Cas9, 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 CRISPR/Cas9 to create new classes of genetic medicine. Learn more 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: acceptance of a clinical trial application ("CTA") or equivalent regulatory submission for NTLA-5001 for the treatment of acute myeloid leukemia ("AML") and ability to initiate a clinical trial by the end of 2021; ability to generate data to demonstrate NTLA-5001 as a potential best-in-class engineered T cell therapy designed to treat all genetic subtypes of AML; plans to evaluate in preclinical studies the potential use of NTLA-5001 to treat Wilms' Tumor 1 ("WT1")-positive solid tumors; plans to advance and complete preclinical studies for our research programs; development of our modular platform to advance our complex genome editing capabilities; further development of our proprietary genome editing tools for research and therapeutic development, including sequential editing; presentation of additional data at upcoming scientific conferences, and other preclinical data in 2021; advancement and expansion of our CRISPR/Cas9 technology to develop human therapeutic products; ability to maintain and expand our related intellectual property portfolio, and avoid or acquire rights to valid intellectual property of third parties; ability to demonstrate our platform's modularity and replicate or apply results achieved in preclinical studies, including those in our AML program, in any future studies, including human clinical trials; ability to develop other *in vivo* or *ex vivo* cell therapeutics of all types, and those targeting WT1 in AML in particular, using CRISPR/Cas9 technology; 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 our collaborations on our development programs, statements regarding the timing of regulatory filings and clinical trial execution, including dosing of patients, regarding our development programs; potential commercial opportunities, including value and market, for our product candidates; our expectations regarding our use of capital and other financial results during 2021; and our ability to fund operations beyond 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 ("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.

Intellia Contacts:

Investors:
Ian Karp
Senior Vice President, Investor Relations and Corporate Communications
+1-857-449-4175
ian.karp@intelliatx.com

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

Media:
Lisa Qu
Ten Bridge Communications
+1-678-662-9166
media@intelliatx.com
lqu@tenbridgecommunications.com

Source: Intellia Therapeutics, Inc.