Intellia Therapeutics Announces First Patient Dosed in Phase 1/2a Clinical Trial of NTLA-5001 for the Treatment of Acute Myeloid Leukemia

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- *NTLA-5001 is a novel investigational T cell receptor (TCR)-T cell therapy which leverages Intellia's proprietary cell engineering platform*
- *NTLA-5001 targets Wilms' Tumor 1 (WT1), an overexpressed intracellular antigen frequently found in acute myeloid leukemia (AML) and other hematologic and solid tumors*

CAMBRIDGE, Mass., March 01, 2022 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing potentially curative therapeutics leveraging CRISPR-based technologies, today announced that the first patient has been dosed with NTLA-5001, the company's *ex vivo* CRISPR/Cas9 genome editing candidate for the treatment of acute myeloid leukemia (AML). NTLA-5001 is an autologous T cell receptor (TCR)-T cell therapy designed to target the Wilms' Tumor (WT1) antigen, which is found in AML and many other hematologic and solid tumors. It is the company's first *ex vivo* candidate developed using Intellia's advanced lipid nanoparticle cell engineering platform, designed to improve cell performance as compared to traditional *ex vivo* delivery technologies.

"As Intellia's first wholly-owned *ex vivo* candidate to be dosed in a patient, this NTLA-5001 milestone represents a significant step forward in our full-spectrum approach to genome editing," said Intellia President and Chief Executive Officer John Leonard, M.D. "AML is the most common type of acute leukemia in adults, where despite recent advancements, a significant therapeutic need still exists. We look forward to advancing this investigational engineered cell therapy as a treatment for people living with this aggressive cancer of the blood and bone marrow."

About the NTLA-5001 Clinical Program

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 includes a dose escalation and expansion phase, with up to 54 total participants. The dose-escalation phase of the study includes two independent arms of up to three cohorts each: Arm 1 consists of adults with AML with lower disease burden, defined as those with less than 5% blasts in bone marrow, while Arm 2 consists of adults with AML with higher disease burden, defined as those with greater than or equal to 5% blasts in bone marrow. Once a dose is identified in each arm, two expansion cohorts will be opened for further safety assessment. Visit clinicaltrials.gov (NCT05066165) for more details.

About NTLA-5001

NTLA-5001 is an investigational 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 and 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 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 in the U.S., with more than 20,000 estimated new cases in 2021. Despite currently available treatments for AML, the five-year overall survival rate for patients remains less than 30%. AML, along with other cancer types, is often characterized by overexpression of the Wilms' Tumor 1 (WT1) antigen.

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 regarding: its ability to conduct and complete clinical studies for NTLA-5001 for the treatment of AML, including manufacturing of NTLA-5001 to support such clinical trials; the safety, efficacy and advancement of our clinical program for NTLA-5001 for the treatment of AML; its ability to generate data to demonstrate NTLA-5001 as a potential engineered T cell therapy designed to treat all genetic subtypes

of AML; the ability to enroll patients in both arms of the Phase 1/2a study; the ability to enroll patients in expansion cohorts upon the identification of a preferred dosage in each arm of the Phase 1/2a study; its ability to develop its modular platform and full-spectrum approach to advance its complex genome editing capabilities, including to apply its proprietary cell engineering platform to additional product candidates; the advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products; its ability to maintain and expand its related intellectual property portfolio, and avoid or acquire rights to valid intellectual property of third parties; its ability to demonstrate its 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; its 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; its expectations of the potential impact of the coronavirus disease 2019 pandemic on strategy, future operations, manufacturing and timing of its clinical trials or IND submissions; the timing of regulatory filings and clinical trial execution, including dosing of patients; and potential commercial opportunities, including value and market, for its product candidates, including NTLA-5001 for AML; and its 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 the successful enrollment of patients in the Phase 1/2a study for NTLA-5001 for the treatment of AML; risks related to the successful manufacturing of NTLA-5001 to support the Phase 1/2a study for NTLA-5001 for the treatment of AML; 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, including manufacturing, for its *in vivo* and *ex vivo* product candidates, including NTLA-5001; the risk that any one or more of Intellia's product candidates, including NTLA-5001, will not be successfully developed and commercialized; the risk that the results of preclinical studies or clinical studies, including for NTLA-5001, will not be predictive of future results in connection with future studies; and the risk that Intellia's will not be able to demonstrate its platform's modularity and replicate or apply results achieved in preclinical studies to develop additional product candidates, including to apply its proprietary cell engineering platform successfully to additional product candidates. 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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