Intellia Therapeutics Receives U.S. FDA Orphan Drug Designation for NTLA-5001 for the Treatment of Acute Myeloid Leukemia

March 9, 2022

- *NTLA-5001, a novel T cell receptor (TCR)-T cell therapy, is currently being evaluated in a Phase 1/2a study in adults with persistent or recurrent acute myeloid leukemia*

CAMBRIDGE, Mass., March 09, 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 U.S. Food and Drug Administration (FDA) has granted orphan drug designation for Intellia's *ex vivo* investigational T cell receptor (TCR)-T cell therapy, NTLA-5001, for the treatment of acute myeloid leukemia (AML).

NTLA-5001 is an autologous TCR-T cell therapy designed to target the Wilms' Tumor (WT1) antigen, which is highly expressed in AML and many other hematologic and solid tumors. NTLA-5001 is currently being evaluated in a Phase 1/2a study in adults with persistent or recurrent AML who have previously received first-line therapy.

"The FDA's decision to grant orphan drug designation for NTLA-5001 reflects the serious need for novel treatment options for people living with AML, a disease with notably poor long-term survival," said Intellia President and Chief Executive Officer John Leonard, M.D. "As part of our full-spectrum genome editing strategy, we seek to leverage our proprietary CRISPR/Cas9-based platform to engineer differentiated cell therapies targeting cancers for which there are currently limited or no treatment options. We look forward to advancing our investigational TCR-T cell therapy, NTLA-5001, through the clinic in hopes of improving future treatment options for patients in need."

The FDA's Orphan Drug Designation program provides orphan status to drugs defined as those intended for the treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the United States. Orphan drug designation qualifies the sponsor of the drug for certain development incentives, including tax credits for qualified clinical testing, prescription drug user-fee exemptions and seven-year marketing exclusivity upon FDA approval.

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. NTLA-5001 utilizes a WT1-targeting TCR identified in collaboration with IRCCS Ospedale San Raffaele. 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: the safety, efficacy and advancement of its clinical programs for NTLA-5001 for the treatment of acute myeloid leukemia pursuant to its clinical trial applications (“CTA”) and IND submissions, including the expected timing of data releases, regulatory filings, and the initiation and completion of clinical trials; the expansion of its CRISPR/Cas9 technology and related technologies to advance additional development candidates, including to apply such technologies to engineer differentiated cell therapies, as well as its ability to maintain and expand its related intellectual property portfolio; and expectations of the potential impact of the coronavirus disease 2019 pandemic on strategy, and future operations and timing of its clinical trials.

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; risks related to the authorization, initiation and conduct of studies and other development requirements for its product candidates, such as NTLA-5001, including risks related to regulatory approvals to conduct clinical trials; the risk that any one or more of Intellia’s product candidates, such as NTLA-5001, will not be successfully developed and commercialized; and the risk that the results of preclinical studies or clinical studies will not be predictive of future results in connection with future studies for Intellia’s current and future product candidates, including current and future engineered cell therapy 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 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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Source: Intellia Therapeutics, Inc.