FDA Accepts Investigational New Drug Application for CRISPR/Cas9-Based Sickle Cell Disease Therapeutic Candidate Developed Under Collaboration with Intellia Therapeutics

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CAMBRIDGE, Mass., March 31, 2020 (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 that the U.S. Food and Drug Administration (FDA) has accepted the Investigational New Drug (IND) application submitted by its collaborator, Novartis, for a CRISPR/Cas9-based engineered cell therapy for the treatment of sickle cell disease (SCD).

This Phase 1/2 clinical trial will begin investigating OTQ923 in adult patients with severe complications of SCD. OTQ923 is a SCD treatment based on genome editing of hematopoietic stem cells (HSCs), using CRISPR/Cas9 RNA guides identified through Intellia's cell therapy research collaboration with Novartis. This therapeutic approach results in highly targeted editing of the HSC's DNA to induce fetal hemoglobin (HbF) expression. The edited cells are returned to the patient, where the expression of HbF is expected to reduce the deleterious effects of sickle hemoglobin (HbS). Novartis' IND application triggered a milestone payment to Intellia, and the company is eligible to receive additional downstream success-based milestones and royalties.

"We are pleased to have worked alongside our colleagues at Novartis to achieve this important milestone, which moves this CRISPR/Cas9-based engineered cell therapy into the clinic, with the potential to significantly impact the lives of patients who suffer from sickle cell disease," said Intellia Chief Operating Officer and Executive Vice President Andrew Schiermeier, Ph.D. "Our research with Novartis over the past five years has laid the groundwork for the development of next-generation CRISPR/Cas9-based cell therapies for patients. Intellia looks forward to Novartis' efforts to advance other targets that were selected to develop as additional CRISPR/Cas9-based cell therapy products."

About Intellia's Engineered Cell Therapy Programs

From December 2014 through December 2019, Intellia and Novartis jointly researched CRISPR/Cas9-based cell therapies in various cell types, including certain stem cells and T cells. In parallel with its *ex vivo* collaboration with Novartis, Intellia has been advancing its wholly owned *ex vivo* pipeline of immuno-oncology and autoimmune cell therapies. Intellia's proprietary *ex vivo* programs include its acute myeloid leukemia (AML) program utilizing transgenic T cell receptors (TCRs) against Wilms' Tumor 1 (WT1), a target identified in collaboration with IRCCS Ospedale San Raffaele (OSR). Intellia plans to submit an IND application for NTLA-5001, the company's development candidate for the treatment of AML, in the first half of 2021. View Intellia's programs pipeline for more information.

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on developing proprietary, curative therapeutics using the CRISPR/Cas9 system. Intellia believes the CRISPR/Cas9 technology has the potential to transform medicine by permanently editing disease-associated genes in the human body with a single treatment course, and through improved cell therapies that can treat cancer and immunological diseases, or can replace patients' diseased cells. The combination of deep scientific, technical and clinical development experience, along with its leading intellectual property portfolio, puts Intellia in a unique position to unlock broad therapeutic applications of the CRISPR/Cas9 technology and create a new class of therapeutic products. Learn more about Intellia Therapeutics and CRISPR/Cas9 at intelliatx.com and 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: planned submission of an investigational new drug ("IND") application or similar clinical trial application for NTLA-5001, its first T cell receptor ("TCR")-directed engineered cell therapy development candidate for its acute myeloid leukemia ("AML") program in the first half of 2021; plans to advance and complete preclinical studies for its other in vivo and ex vivo programs; 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 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; ability to optimize the impact of its collaborations on its development programs, including but not limited to its collaborations with Novartis and OSR; statements regarding the timing of regulatory filings regarding its development programs; use of capital, expenses, future accumulated deficit and other 2019 financial results or in the future; and ability to fund operations through the end of 2021.

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 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 Novartis or its other *ex vivo* 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.

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