



## Intellia Therapeutics Receives Grant to Develop Curative CRISPR/Cas9 In Vivo Sickle Cell Disease Treatments

November 11, 2020

*Funding from the Bill & Melinda Gates Foundation to develop safe, scalable and accessible non-viral treatments for sickle cell patients*

CAMBRIDGE, Mass., Nov. 11, 2020 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), announced that it has received a grant from the Bill & Melinda Gates Foundation to research in vivo sickle cell disease (SCD) treatments using its CRISPR/Cas9 genome editing technology. This pilot development program is part of the Gates Foundation's broader initiative to accelerate the advancement of safe, effective and durable gene-based cures in developing countries within the next seven to 10 years. The funding from the Gates Foundation will advance Intellia's preclinical validation of in vivo hematopoietic stem cells (HSCs) genome editing using the company's proprietary non-viral delivery systems and CRISPR/Cas9 technology to potentially cure SCD.

"We are excited to receive funding from the Gates Foundation to take the first steps toward development of a potential *in vivo* non-viral CRISPR/Cas9-based cure for SCD. Genome editing offers multiple opportunities to treat SCD as shown by encouraging emerging clinical data. Our goal is to deliver on the already demonstrated promise of CRISPR/Cas9, but avoid the severe complications of bone marrow transplantation that may limit the usefulness of current approaches," said Intellia's Chief Scientific Officer Laura Sepp-Lorenzino, Ph.D. "Intellia's ambition is to use our non-viral *in vivo* platform to create an innovative treatment for blood disorders, that is scalable and can overcome the challenges inherent to *ex vivo* cell-based therapies for global diseases."

Intellia's *in vivo* CRISPR/Cas9 delivery approach has the potential to develop HSC-based genome editing therapies that are more practical solutions to treat blood disorders, including SCD. Intellia's *in vivo* platform technology delivers CRISPR/Cas9 intravenously, potentially avoiding the need for bone marrow transplantation surgery.

### About Sickle Cell Disease

Sickle cell disease (SCD) affects millions of people throughout the world and is particularly common among those whose ancestors came from sub-Saharan Africa, certain regions in South and Central America, Saudi Arabia, India and some Mediterranean countries. SCD disproportionately affects populations in sub-Saharan Africa and India and leads to substantial morbidity, mortality and economic disadvantages. In the United States, approximately 100,000 people have been diagnosed, whereas in Nigeria, the country with the highest known incidence of SCD, there are approximately 150,000 babies born annually with sickle cell anemia.

### 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](https://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: 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; 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; use of capital, expenses, future accumulated deficit and other 2020 financial results or in the future; and ability to fund operations for at least 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 or grants 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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Source: Intellia Therapeutics, Inc.