# Intellia Therapeutics Announces New, Robust Genome Editing Data for Sickle Cell Disease at the American Society of Hematology Meeting

December 11, 2017

- *Data on ex vivo CRISPR/Cas9 genome-edited human hematopoietic stem cells shows increased production of fetal hemoglobin*
- *Approximately 80-95 percent editing was achieved in CD34+ cells*
- *Increased fetal hemoglobin levels were sustained for at least 16 weeks following transplant in mice*

ATLANTA, Dec. 11, 2017 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on the development of potentially curative therapeutics using the CRISPR technology, and its collaborator, Novartis, presented initial data from their research collaboration on genome-edited human hematopoietic stem cells. These data showed successful *ex vivo* editing of the erythroid specific enhancer of *BCL11A*, a gene associated with prevention of sickle cell disease, and the ability of these cells to stably engraft in mice while maintaining their desired properties.

These data were presented today at the American Society of Hematology Annual Meeting in the platform presentation session: *Hemoglobinopathies, Excluding Thalassemia – Basic and Translational Science: Sickle Cell Disease – Hematopoiesis and Fetal Hemoglobin Augmentation.* In the presented studies, the companies:

- Achieved approximately 80-95 percent target site modification in human hematopoietic stem and progenitor CD34+ cells following electroporation of ribonucleoprotein (RNP) composed of Cas9 and a guide RNA (gRNA), selected for efficacy and potency;

- Demonstrated an approximately 40 percent reduction in *BCL11A* mRNA with a corresponding two-fold increase in γ-globin transcript and 30-40 percent more fetal hemoglobin-positive cells above background. Similar decreases in *BCL11A* mRNA and increases in γ-globin transcipt were observed when sickle cell disease-derived cells from patient donors were edited;

- Achieved engraftment over 16 weeks following transplantation of edited human bone marrow CD34+ cells into immune compromised mice, while maintaining editing levels in engrafted cells; and

- Observed no off-target events in CD34+ cells edited with the selected gRNA, as measured by targeted next generation sequencing of sites identified through *in silico* prediction and based on an unbiased, genome-wide, oligo-insertion detection method.

"We are pleased to be reporting data from studies generated through Intellia's collaboration with Novartis, demonstrating successful *ex vivo* CRISPR/Cas9 editing in hematopoietic stem cells," said John Leonard, M.D., executive vice president, Research & Development, Intellia Therapeutics. "These results are significant as we have shown high levels of editing as well as increased production of fetal hemoglobin to clinically relevant levels, which could potentially ameliorate sickle cell disease in affected patients. We are very encouraged to present this progress given that sickle cell disease is a serious condition that currently has limited treatment options."

**About Sickle Cell Disease**

Sickle cell disease is a life-threatening, hereditary disorder that impacts approximately 30 million people worldwide. The disease results from a single amino acid change in the β-globin gene, which causes polymerization of hemoglobin and the deformation of red blood cells, leading to vaso-occlusion, severe pain crisis and multi-organ dysfunction. The average life expectancy in the developed world is 40 to 60 years. About 80 percent of sickle cell disease cases are believed to occur in sub-Saharan Africa.

**About Intellia Therapeutics**

Intellia Therapeutics is a leading genome editing company focused on the development of 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. Our combination of deep scientific, technical and clinical development experience, along with our leading intellectual property portfolio, puts us 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; Follow us on Twitter @intelliatweets.

**Forward-Looking Statements**

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statement in this press release include, but are not limited to, statements regarding the successful development of CRISPR/Cas9-based therapies to

treat sickle cell disease or any other disease; the translation of the results from animal models to human therapies; the successful development of gRNAs with no off-target events; and the Company's or its collaborator's ability and intention to develop, seek regulatory approval for, and commercialize therapies to treat sickle cell disease using CRISPR/Cas9. Any forward-looking statements in this press release are uncertain, based on management's current expectations of future events, occurrences, actions and plans, and subject to various 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. The Company may not actually execute or obtain the results from the plans, goals, efforts or opportunities disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, goals, efforts or opportunities disclosed in these forward-looking statements as a result of various factors including: uncertainties inherent in the implementation and execution of preclinical studies and clinical trials, and preclinical and clinical development of the Company's or its collaborator's products candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a preclinical trial will be predictive of the final results of the preclinical or clinical trials or the results of future trials; expectations and requirements for regulatory approvals to conduct trials or to market products; and availability of funding sufficient for the Company's or its collaborator's foreseeable and unforeseeable operating expenses and capital expenditure requirements. For a discussion of risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in our most recent quarterly report on Form 10-Q and in our Prospectus filed with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in our other filings with the Securities and Exchange Commission. All information and forward-looking statements in this press release are as of the date of the release, and Intellia Therapeutics undertakes no duty to update this information, whether because of new information, future events or otherwise, unless required by law.


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