Editas Medicine Announces Pre-Clinical Data Supporting Novel Approach for Treatment of Sickle Cell Disease and Beta-Thalassemia

December 3, 2018

Data support opportunities to develop best-in-class, durable medicines for hemoglobinopathies

CAMBRIDGE, Mass., Dec. 02, 2018 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ: EDIT), a leading genome editing company, today announced results from experiments to demonstrate expanded CRISPR genome editing strategies in hematopoietic stem cells for the treatment of sickle cell disease and beta-thalassemia. In these experiments, the Company demonstrated *HBG1/2* promoter-edited CD34+ cells robustly engrafted in mice without lineage skewing of red blood cell precursors. The Company reported these data today in an oral presentation at the 60th Annual Meeting of the American Society of Hematology (ASH) in San Diego.

In these experiments, NBSGW mice received an infusion of human CD34+ cells which had been edited either at the *BCL11A* erythroid enhancer *(BCL11Ae)* site or at the *HBG1/2* promoter site. Analysis of bone marrow collected eight to 16 weeks post-infusion demonstrated that robust fetal hemoglobin induction was achieved when targeting *HBG1/2* promoters. Notably, editing *HBG1/2* promoters upregulated fetal hemoglobin with superior repopulation of red blood cell precursors as compared to editing the *BCL11A*e site. The red blood cell precursors from bone marrow edited at the *BCL11A*e site had lower productive editing rates compared to other lineages and showed increased level of apoptosis, or programmed cell death, in erythroid culture compared to *HBG1/2* promoter-edited cells.

Increased production of fetal hemoglobin can be beneficial to patients with sickle cell disease or beta-thalassemia. Editing at the *HBG1/2* site is a differentiated approach for development of a human therapeutic for the treatment of sickle cell disease and beta-thalassemia as compared to other medicines currently under development that edit at the *BCL11Ae* site.

"We are encouraged by these preclinical results demonstrating cells edited at the *HBG1/2* promoters repopulated all lineages of the blood system including, importantly, the red blood cell precursors. Editing at this site met our preclinical goals including robust, long-term induction of fetal hemoglobin and maintenance of normal hematopoietic stem/progenitor cell function," said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine. "These findings further support our novel approach to developing a medicine for the potential treatment of sickle cell disease and beta-thalassemia. If these preclinical results translate to humans, we believe our editing approach for hemoglobinopathies may yield a safer and more effective medicine."

About Editas Medicine

As a leading genome editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas9 and CRISPR/Cpf1 (also known as Cas12a) genome editing systems into a robust pipeline of treatments for people living with serious diseases around the world. Editas Medicine aims to discover, develop, manufacture, and commercialize transformative, durable, precision genomic medicines for a broad class of diseases. For the latest information and scientific presentations, please visit www.editasmedicine.com.

Forward-Looking Statements

This press release contains forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "target," ''should,'' ''would,'' and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release include statements regarding the safety and efficacy of the Company's editing approach to treating hemoglobinopathies. The Company may not actually achieve the plans, intentions, or expectations 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, intentions and expectations disclosed in these forward-looking statements as a result of various factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development of the Company's product candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; expectations for regulatory approvals to conduct trials or to market products and availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements. These and other risks are described in greater detail under the caption "Risk Factors" included in the Company's most recent Quarterly Report on Form 10-Q, which is on file with the Securities and Exchange Commission, and in other filings that the Company may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

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