Editas Medicine Announces In Vivo Proof-of-Concept Data for EDIT-301, in Development for the Treatment of Sickle Cell Disease and Beta-Thalassemia

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Data support novel approach to develop a best-in-class, durable medicine for people living with hemoglobinopathies

CAMBRIDGE, Mass., Dec. 09, 2019 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced *in vivo* proof-of-concept data supporting the development of EDIT-301 as a potentially best-in-class, durable medicine to treat sickle cell disease and beta-thalassemia. EDIT-301 is the first experimental medicine in development using Cas12a (formerly known as Cpf1). The Company reported these data today at the 61st Annual Meeting and Exposition of the American Society of Hematology (ASH) in Orlando, Fla.

Sickle cell disease is caused by a mutation in the beta-globin gene that leads to polymerization of the sickle hemoglobin protein (HbS). Fetal hemoglobin (HbF) protects against sickle cell disease by inhibiting HbS polymerization. Individuals with high levels of HbF are protected from sickle cell disease. EDIT-301 is an experimental, autologous cell therapy comprising CD34+ cells genetically modified using a Cas12a ribonucleoprotein (RNP) that targets the *HBG1/2* promoter in the beta-globin gene to stimulate HbF production.

In this study, when EDIT-301 was infused into NBSGW mice, HbF levels in human red blood cells were increased by approximately 50 percentage points above background at 16 weeks post-engraftment with pancellular distribution and no lineage skewing. These elevated HbF levels were observed after editing with Cas12a which created an editing profile that enriched genomic changes that favored high and persistent HbF levels.

"We are very encouraged by these *in vivo* findings as the data further support our novel approach to developing a best-in-class and durable medicine for the potential treatment of sickle cell disease and beta-thalassemia. IND-enabling activities were initiated earlier this year for and are ongoing for EDIT-301," said Charles Albright, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine. "If these preclinical results translate to humans, we believe our editing approach may yield a safer and more effective medicine, addressing a significant need for a transformative, durable treatment for people living with sickle cell disease and beta-thalassemia."

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 (formerly 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.

Editas Medicine 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 Company's expectations regarding EDIT-301. Editas Medicine 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 Editas Medicine'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 Editas Medicine'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 Editas Medicine's most recent Quarterly Report on Form 10-Q, which is on file with the Securities and Exchange Commission, and in other filings that Editas Medicine may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this press release represent Editas Medicine's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, Editas Medicine explicitly disclaims any obligation to update any forward-looking statements.

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