Editas Medicine Announces Preclinical Data and Large-Scale Manufacturing Process 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 and manufacture a best-in-class, durable medicine for people living with hemoglobinopathies

IND filing for EDIT-301 planned by end of 2020

CAMBRIDGE, Mass., Dec. 05, 2020 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced preclinical data and successful development of a large-scale manufacturing process for EDIT-301, a potentially best-in-class, one-time, durable, autologous cell therapy medicine to treat sickle cell disease and beta-thalassemia. EDIT-301 is the first experimental medicine in development generated using CRISPR/Cas12a gene editing. The Company reported these data today at the 62nd Annual Meeting and Exposition of the American Society of Hematology (ASH) being held virtually.

The data showed that high levels of editing in CD34+ cells from normal donors and sickle cell patients were achieved with CRISPR/Cas12a at the *HBG1* and *HBG2* promoters, leading to robust fetal hemoglobin (HbF) induction in their erythroid progeny in a pan-cellular fashion. Red blood cells derived from edited sickle cell patient CD34+ cells showed remarkable correction of sickle cell disease phenotypes, including a reduction in sickling and improved rheological properties when deoxygenated.

In addition, the Company's large-scale manufacturing process was shown to be consistent and robust. When infused into immunodeficient mice, edited CD34+ cells from normal donors manufactured at large-scale led to long term multi-lineage hematopoietic reconstitution that was comparable to unedited control cells. The engraftment was stable and highly polyclonal with high levels of editing detected throughout the course of the study.

"These findings are very encouraging and further support our novel approach to developing and manufacturing EDIT-301 as a best-in-class and durable medicine for the potential treatment of sickle cell disease and beta-thalassemia," said Charles Albright, Ph.D., Executive Vice President and Chief Scientific Officer, Editas Medicine. "If these preclinical results translate to the clinic, we believe our editing approach may yield a safer and more effective medicine, addressing a significant unmet need for a transformative, durable treatment with the potential to transform the lives of people living with sickle cell disease and beta-thalassemia. "

Editas Medicine continues to prepare for a Phase 1/2 clinical trial evaluating EDIT-301 for the treatment of sickle cell disease. The Company has completed preclinical toxicology studies, identified a lead principal investigator, and engaged a contract research organization (CRO). Clinical trial materials are being manufactured by Editas Medicine. The Company remains on track to file an IND for the treatment of sickle cell disease by the end of 2020.

About Sickle Cell Disease
Sickle cell disease is an inherited blood disorder caused by a mutation in the beta-globin gene that leads to polymerization of the sickle hemoglobin protein (HbS). In sickle cell disease, the red blood cells are misshapen, in a sickle shape instead of the disc shape. The abnormal shape causes the cells to block blood flow causing anemia, pain crises, organ failure, and early death. There are an estimated 100,000 people in the United States currently living with sickle cell disease. Fetal hemoglobin (HbF) protects against sickle cell disease by inhibiting HbS polymerization.

About EDIT-301
EDIT-301 is an experimental, autologous cell therapy medicine under investigation for the treatment of sickle cell disease. EDIT-301 is comprised of sickle patient CD34+ cells genetically modified using a highly specific and efficient CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP) that targets the *HBG1* and *HBG2* promoters in the beta-globin locus where naturally occurring fetal hemoglobin (HbF) inducing mutations reside. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in HbF production, which has the potential to provide a durable treatment benefit for people living with sickle cell disease.

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/Cas12a (also known as Cpf1) 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 Company's plans and expectations for EDIT-301, including filing an IND for EDIT-301 by the end of 2020. 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 pre-clinical studies and clinical trials and clinical development of the Company's product candidates; availability and timing of results from pre-clinical 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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