Editas Medicine Presents Pre-Clinical Data from a Study of EDIT-301 with Sickle Patient Cells for the Potential Treatment of Sickle Cell Disease

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IND filing for novel genome editing medicine by end of 2020

CAMBRIDGE, Mass., June 12, 2020 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced results from a pre-clinical, proof-of-concept study of EDIT-301. EDIT-301 is being developed as a potentially best-in-class, durable medicine to treat sickle cell disease. EDIT-301 contains CD34+ hematopoietic stem cells from sickle patients that are edited at the *HBG1/2* promoter in the beta-globin locus using Cas12a (also known as Cpf1) to induce fetal hemoglobin (HbF) where HbF-inducing mutations occur naturally. The Company reported the data at the 25th Congress of the European Hematology Association being held virtually.

In vitro studies with EDIT-301 revealed several desirable properties. In particular, editing was highly efficient and reproducible, with approximately 90 percent editing in multiple sickle patient donors. Further, EDIT-301 derived red blood cells had more than 50 percent HbF expression. Finally, EDIT-301 derived red blood cells had a significant improvement in deformability, which could aid red blood cell transit without sickling, and a four-fold decrease in sickling, when subjected to reduced oxygen levels compared to unedited control cells. These data suggest EDIT-301 can provide potential clinical benefit for sickle patients.

In vivo studies with EDIT-301 revealed desirable properties. In particular, editing was highly efficient with greater than 90 percent editing in bone marrow cells from mice infused with edited CD34+ cells 16 weeks post infusion. Further, HbF expression was increased by approximately 50 percent in the red blood cells derived from these edited cells. Finally, approximately 90 percent of these cells were HbF positive, demonstrating that HbF expression was pan-cellular, a likely critical property for potential clinical benefit.

"By editing sickle patient cells at the *HBG1/2* promoter region, a region where fetal hemoglobin inducing mutations naturally occur, we showed efficient and reproducible editing that created red blood cells with improved deformability at low oxygen levels. By editing CD34+ cells, we showed efficient long-term editing *in vivo* with elevated and pan-cellular HbF. All of these properties are likely essential for clinical benefit and support our novel approach to develop a best-in-class, durable medicine for the potential treatment of sickle cell disease," said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine. "IND-enabling activities are ongoing, and we remain on-track to file an IND for EDIT-301 by the end of the year."

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) to edit the *HBG1/2* promoter region in the beta-globin locus. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in fetal hemoglobin (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/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 Company's plans with respect to 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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