Editas Medicine Announces the FDA has Cleared Initiation of the EDIT-301 Clinical Trial

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EDIT-301 is in development as a best-in-class, durable medicine for people living with sickle cell disease

CAMBRIDGE, Mass., Jan. 11, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced the U.S. Food and Drug Administration (FDA) has cleared the initiation of the safety phase of the Company's EDIT-301 clinical trial, and the Company can begin dosing patients. The Company is required to develop and submit to the FDA an improved potency assay prior to enrolling the efficacy phase of the RUBY trial. EDIT-301 is an experimental, *ex vivo* gene editing cell medicine in development for the treatment of sickle cell disease. Previously, the Company received Rare Pediatric Disease designation from the FDA for EDIT-301. EDIT-301 is the first experimental medicine in development generated using CRISPR/Cas12a gene editing.

"The FDA's clearance for initiation for our EDIT-301 clinical trial is an exciting moment for us and the patients we hope to serve. We look forward to bringing this potentially best-in-class, one-time, durable medicine into the clinic and to patients," said Cindy Collins, President and Chief Executive Officer, Editas Medicine. "We know patients are counting on us, and we believe EDIT-301 has the potential to transform the lives of people living with sickle cell disease, addressing a significant unmet need."

Editas Medicine is preparing to initiate the RUBY clinical trial, a Phase 1/2 trial designed to assess the safety and efficacy of EDIT-301 for the treatment of sickle cell disease. The Company has identified a lead principal investigator and engaged a Clinical Research Organization (CRO). Clinical trial materials are being manufactured by Editas Medicine. The Company will need to resolve a partial clinical hold prior to commencement of the efficacy portion of the RUBY trial by developing an improved potency assay after the first patients are dosed in the safety portion of the trial before collection of data to support registration.

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 one-time, durable treatment benefit for people living with sickle cell disease.

About RUBY
The RUBY Trial is a single-arm, open-label, multi-center Phase 1/2 study designed to assess the safety and efficacy of EDIT-301 in people with severe sickle cell disease. Enrolled patients will receive a single administration of EDIT-301.

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, including statements regarding the expected initiation and conduct of the RUBY clinical trial. 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. 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.

Contacts:
Media
Cristi Barnett
(617) 401-0113

cristi.barnett@editasmed.com

Investors
Editas Medicine Investor Relations
(617) 401-9052
ir@editasmed.com

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