Editas Medicine Announces Clinical Achievements in the Development of EDIT-301 for Sickle Cell Disease

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Successful engraftment of first patient dosed with EDIT-301 for sickle cell disease

FDA removed partial clinical hold for the RUBY trial in EDIT-301

First clinical use of Editas-engineered AsCas12a enzyme

Initial clinical data from the RUBY trial expected by year-end

CAMBRIDGE, Mass., July 27, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced the dosing and confirmed successful neutrophil and platelet engraftment of the first patient in the Phase 1/2 RUBY trial of EDIT-301 for the treatment of severe sickle cell disease (SCD). This dosing is the first time that the Company's engineered AsCas12a enzyme, a proprietary, highly efficient, and specific gene editing nuclease, has been used to edit human cells in a clinical trial.

The trial is enrolling additional study participants at multiple centers in the U.S. and Canada. The Company has successfully edited $CD34^+$ cells from patients in preparation for reinfusion and remains on track to announce top-line clinical data by year-end.

Additionally, the Company announced that the U.S. Food and Drug Administration (FDA) removed the previously disclosed partial clinical hold on the RUBY trial, which enables the Company to include efficacy data from patients in a marketing application for EDIT-301 in the future.

"It is an exciting time at Editas as we continue to build momentum for our EDIT-301 program," said Gilmore O'Neill, M.B., M.M.Sc., President and CEO, Editas Medicine. "Dosing and successful engraftment of the first patient coupled with the FDA's removal of the partial clinical hold on the RUBY trial are important steps toward our goal of bringing this new and promising treatment to people living with sickle cell disease and thalassemia."

EDIT-301 is also being investigated in a clinical study in patients with transfusion-dependent beta thalassemia (TDT). Preparations to initiate the Phase 1/2 EDITHAL clinical trial designed to assess the safety, tolerability, and preliminary efficacy of EDIT-301 for the treatment of TDT are underway, and the Company remains on track to dose the first TDT patient in 2022.

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. Higher levels of fetal hemoglobin (HbF) inhibits HbS polymerization, thus reducing the manifestation of sickling.

About EDIT-301
EDIT-301 is an experimental cell therapy medicine under investigation for the treatment of severe sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT). EDIT-301 consists of patient-derived $CD34^+$ hematopoietic stem and progenitor cells edited at the gamma globin gene (HBG1 and HBG2) promoters, where naturally occurring fetal hemoglobin (HbF) inducing mutations reside, by a highly specific and efficient proprietary engineered AsCas12a nuclease. Red blood cells derived from EDIT-301 $CD34^+$ cells demonstrate a sustained increase in fetal hemoglobin production, which has the potential to provide a one-time, durable treatment benefit for people living with severe SCD and TDT.

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 patients with severe sickle cell disease. Enrolled patients will receive a single administration of EDIT-301. Additional details are available on www.clinicaltrials.gov (NCT#04853576).

About EDITHAL
The EDITHAL study is a single-arm, open label, multi-center Phase 1/2 study designed to assess the safety and efficacy of EDIT-301 in patients with transfusion-dependent beta thalassemia. Enrolled patients will receive a single administration of EDIT-301. Additional details are available on www.clinicaltrials.gov (NCT#05444894).

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 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. Editas Medicine is the exclusive licensee of Harvard and Broad Institute's Cas9 patent estates and Broad Institute's Cas12a patent estate for human medicines. 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 initiation, timing, progress and results of the Company's clinical studies, including dosing the first TDT patient in the Phase 1/2 EDITHAL study of EDIT-301 in 2022, and the timing for the

Company's receipt and presentation of data from its clinical trials, including the Company's announcement of initial clinical data from the RUBY trial by year-end 2022. 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, including the RUBY trial, 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 Annual Report on Form 10-K, which is on file with the Securities and Exchange Commission, as updated by Editas Medicine's subsequent filings 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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