Editas Medicine Announces Positive Safety and Efficacy Data from the First Two Patients Treated in the RUBY Trial of EDIT-301 for the Treatment of Severe Sickle Cell Disease

December 6, 2022

EDIT-301 was well-tolerated and demonstrated a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant

Both patients treated with EDIT-301 successfully engrafted and are free of vaso-occlusive events during the follow-up period

First patient treated has a total hemoglobin level of 16.4 g/dL and 45.4% fetal hemoglobin five months after treatment with EDIT-301

Company to host a webinar today at 8:00 a.m. ET

CAMBRIDGE, Mass., Dec. 06, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical stage genome editing company, today announced positive, initial clinical data from the first two patients with sickle cell disease (SCD) treated with EDIT-301 in the Phase 1/2 RUBY trial. EDIT-301 is under development for the treatment of severe sickle cell disease. The clinical data includes safety data from the first two patients and efficacy data from the first patient treated.

Both treated patients demonstrated successful neutrophil and platelet engraftment. Patient 1 achieved neutrophil engraftment at 23 days after EDIT-301 infusion and platelet engraftment at 19 days after EDIT-301 infusion. Patient 2 achieved neutrophil engraftment 29 days after EDIT-301 infusion and platelet engraftment 37 days after EDIT-301 infusion. Additionally, neither patient has experienced any vaso-occlusive events since treatment with EDIT-301, at five and 1.5 months follow up, respectively.

At five months post-treatment, the first patient treated with EDIT-301 has a total hemoglobin of 16.4 g/dL, a fetal hemoglobin (or HbF) of 45.4%, and a mean corpuscular HbF of 13.8 pg/red blood cell, exceeding the 10.0 pg/red blood cell threshold to suppress red blood cell sickling. Additionally, HbF increase in the first patient was highly pancellular, with F cells steadily increasing to reach greater than 95% of red blood cells.

EDIT-301 was well-tolerated in the two patients and demonstrated a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant. No serious adverse events occurred, and no adverse events reported were related to treatment with EDIT-301.

EDIT-301 uses AsCas12a, a novel, proprietary, highly efficient, and specific gene editing nuclease, to edit the promoter regions of gamma globin gene 1 and 2 to increase the expression of HbF to mimic the natural mechanism of hereditary persistence of fetal hemoglobin to treat SCD. The RUBY trial marks the first time AsCas12a was used to edit human cells in a clinical trial.

“These promising clinical results from the RUBY trial suggest clinical proof of concept for EDIT-301 and support our belief that EDIT-301 can be a clinically differentiated, one-time, durable medicine that can provide life changing clinical benefits to patients with severe sickle cell disease long term,” said Baisong Mei, MD, Ph.D., Senior Vice President and Chief Medical Officer, Editas Medicine. “I would like to thank the participants, their families, clinicians, and colleagues at collaborating institutions that contribute to the RUBY trial. We look forward to sharing further clinical updates in mid-2023.”

Webinar Information
Editas Medicine will host a webinar today, Tuesday, December 6, at 8:00 a.m. ET to present the data. The live and archived webcast of the presentation will be accessible through this webcast link, or through the Events & Presentations page of the “Investors” section of the Company’s website. The presentation will also be available for download shortly after the webinar.

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 (HbS). In sickle cell disease, the red blood cells are misshapen in a sickle shape instead of a typical disc shape. The abnormal shape causes the red blood cells to have shortened lifespan and 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) inhibit 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 Editas Medicine
As a clinical stage 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 Broad Institute and Harvard University'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, including statements regarding the Company's plans to continue development of EDIT-301 and share further clinical updates in mid-2023, and its belief about EDIT-301's potential for clinical differentiation. 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 important factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials, including the RUBY trial, and clinical development of the Company's product candidates, including EDIT-301; 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 Annual Report on Form 10-K, which is on file with the Securities and Exchange Commission, as updated by the Company'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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