Editas Medicine Announces Positive Initial EDIT-301 Safety and Efficacy Data from the First Four Patients Treated in the RUBY Trial and the First Patient Treated in the EdiTHAL Trial

June 9, 2023

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

First two RUBY patients achieved normal levels of total hemoglobin and fetal hemoglobin of >40% at 5 months and maintained these levels after 10 and 6 months of follow up

All five patients treated with EDIT-301 successfully engrafted and all four RUBY patients treated are free of vaso-occlusive events since infusion

RUBY clinical data to be presented at the European Hematology Association (EHA) Hybrid Congress on Saturday, June 10, at 5:30 p.m. CET/11:30 a.m. ET

Company to host a virtual event on the RUBY and EdiTHAL data on Monday, June 12 at 8:00 a.m. ET

CAMBRIDGE, Mass., June 09, 2023 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage genome editing company, today announced positive initial safety and efficacy data from the first four patients with sickle cell disease (SCD) treated with EDIT-301 in the RUBY trial and from the first transfusion-dependent beta thalassemia patient treated in the EdiTHAL trial.

The RUBY trial data will be presented in an oral presentation at European Hematology Association (EHA) Hybrid Congress in Frankfurt, Germany, and via live stream on Saturday, June 10, at 5:30 p.m. CEST/11:30 a.m. EDT. Editas Medicine will present the RUBY trial data and the EdiTHAL trial data on Monday, June 12, at 8 a.m. ET in a Company-sponsored webinar.

In the RUBY trial, Patients 1 and 2 reached normal hemoglobin levels five months post-treatment with EDIT-301, and both patients have maintained a normal hemoglobin level at ten- and six-month follow-up, respectively. Additionally, each of these patients has seen fetal hemoglobin levels of greater than 40% persist during the same time frame. Patients 3 and 4 in the RUBY trial saw increases in total hemoglobin and fetal hemoglobin at three and two months of follow up, respectively, that follow similar trajectories as those seen in the first two patients. All four treated RUBY patients are also free of vaso-occlusive events (VOEs) since infusion.

In the EdiTHAL trial, the first patient demonstrated successful neutrophil and platelet engraftment, and, at one and a half months post-infusion, the patient's response resembles that of the first four RUBY patients.

EDIT-301 was well-tolerated and demonstrated a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant by the four patients in the RUBY trial and the first patient in the EdiTHAL trial. After EDIT-301 infusion, no serious adverse events occurred, and no adverse events reported were related to treatment with EDIT-301.

RUBY Trial
Safety
All treated patients demonstrated successful neutrophil and platelet engraftment. No serious adverse events (SAEs) were reported after EDIT-301 infusion, and no adverse events (AEs) related to EDIT-301 treatment have been observed. All four treated patients in the RUBY trial are free from vaso-occlusive events since infusion with EDIT-301, with 2-10 months follow-up.

Efficacy
Patient 1 (male) has had 10 months of follow-up. Patient 1's total hemoglobin returned to normal physiological level of 16.4g/dL (male normal range: 13.6–18.0 g/dL) at five months after infusion of EDIT-301 and has been maintained at this level at the 10-month follow-up. In addition, Patient 1's fetal hemoglobin fraction increased from 5% at baseline to 45.4% five months after treatment with EDIT-301 and 43.4% at the 10-month follow-up.

Patient 2 (female) has had 6 months of follow-up. Patient 2's total hemoglobin reached normal physiological level of 12.7 g/dL (female normal range: 12.0–16.0 g/dL) at five months after infusion of EDIT-301 and fetal hemoglobin increased from 10.8% at baseline to 51.3% at the 6-month follow-up.

Patient 3 (female) and Patient 4 (male) have had three and two months of follow-up, respectively. Increases in total hemoglobin and fetal hemoglobin fractions for Patient's 3 and 4 are following similar trajectories as seen in Patients 1 and 2 at the same timepoints.

EdiTHAL Trial
Safety
The first treated patient (male) in the EdiTHAL trial has had 1.5 months of follow-up. Patient 1 had successful neutrophil and platelet engraftment within 30 days of EDIT-301 infusion, and the safety profile has been consistent with that of myeloablative busulfan conditioning and autologous hematopoietic stem cell transplant. No serious adverse events occurred after EDIT-301 infusion, and no adverse events reported were related to treatment with EDIT-301.

Efficacy
The first EdiTHAL patient's experience with EDIT-301 resembles that of the first four RUBY patients, achieving a fetal hemoglobin fraction of 34.9% representing 4 g/dL of total hemoglobin at 1.5 months post-treatment.

"These promising data 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 sickle cell disease and beta thalassemia long term, specifically driving early and robust correction of anemia and

sustained increases in fetal hemoglobin," said Baisong Mei, MD, Ph.D., Senior Vice President and Chief Medical Officer, Editas Medicine. "I would like to thank the clinical trial participants, their families, clinicians, and colleagues at collaborating institutions that contribute to the RUBY and EdiTHAL trials. We remain on-track to dose 20 RUBY patients by year-end, and we look forward to sharing further RUBY and EdiTHAL clinical updates later this year."

"I am encouraged by the results from the RUBY trial, which indicate this investigational gene editing treatment has been well-tolerated and efficacious in treated trial participants thus far. While we need to let the trial finish and enroll many other patients to understand the overall benefits and risks of this treatment, I am pleased to see transformative results at this stage in the study," said Rabi Hanna, M.D., Department of Pediatric Hematology Oncology and Blood and Marrow Transplantation, Cleveland Clinic Children's.

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 naturally occurring mechanism of hereditary persistence of fetal hemoglobin to treat SCD. The RUBY trial marks the first time AsCas12a was used to successfully edit human cells in a clinical trial.

EHA Oral Presentation Details:
Title: EDIT-301 Shows Promising Preliminary Safety and Efficacy Results in the Phase I/II Clinical Trial (RUBY) of Patients with Severe Sickle Cell Disease Using Highly Specific and Efficient AsCas12a Enzyme
Presenting Author: Rabi Hanna, M.D., Department of Pediatric Hematology Oncology and Blood and Marrow Transplantation, Cleveland Clinic Children's, Cleveland, OH, United States
Date/Time: Saturday, June 10, 2023, 4:30 – 5:45 p.m. CEST/ 10:30 – 11:45 a.m. EDT
Location: Harmonie 1, Messe Frankfurt
Session: s437 Gene therapy and cellular immunotherapy – Clinical

Virtual Event Information
Editas Medicine will host a virtual event on Monday, June 12, at 8:00 a.m. ET to present the data for the RUBY and EdiTHAL trials. 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.

EDIT-301 is currently being investigated in clinical studies in patients with severe sickle cell disease (RUBY trial, NCT04853576) and transfusion-dependent beta thalassemia (EDITHAL trial, NCT05444894). In addition to the clinical data update from the RUBY trial at EHA and in a Company-sponsored webinar, the Company expects to present a further clinical update from the RUBY and EdiTHAL trials by year-end.

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 a typical disc shape. The abnormal shape causes the red blood cells (RBCs) 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 clinical manifestation of RBCs sickling.

About Beta Thalassemia
Beta thalassemia is an inherited blood disorder caused by mutations that reduce or abrogate beta globin gene expression. Insufficient beta globin production leads to ineffective red blood cell production, chronic hemolytic anemia, compensatory extramedullary hematopoiesis (creation of blood cells outside of the bone marrow), and requirement for regular blood transfusion support in patients with transfusion-dependent beta thalassemia (TDT). TDT is the most severe form of beta thalassemia, and chronic red blood cell transfusions are complicated by iron overload leading to organ dysfunction and failure. It is estimated that there are approximately 1,000 people in the United States currently living with transfusion-dependent beta thalassemia. Higher levels of fetal hemoglobin (HbF) ameliorate anemia thereby reducing the need for regular red blood cell transfusions.

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

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

About Editas Medicine
As a clinical-stage genome editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas12a and Cas9 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's Cas12a patent estate and Broad Institute and Harvard University's Cas9 patent estates 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 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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