Editas Medicine to Present Clinical Data from the RUBY and EdiTHAL Trials of EDIT-301 at the ASH 2023 Annual Meeting and in a Company-sponsored Webinar

November 2, 2023

Company to host a webinar to discuss EDIT-301 clinical data on Monday, December 11, at 1:00 p.m. ET

Dr. Rabi Hanna from Cleveland Clinic Children's to present EDIT-301 clinical data at ASH on Monday, December 11

CAMBRIDGE, Mass., Nov. 02, 2023 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage genome editing company, today announced that a scientific abstract detailing safety and efficacy clinical data from the RUBY trial of EDIT-301 in patients with severe sickle cell disease and from the EdiTHAL trial of EDIT-301 in patients with transfusion-dependent beta thalassemia has been accepted for a poster presentation at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition being held December 9-12, 2023, in San Diego, CA, and online.

Editas Medicine will also host a Company-sponsored webinar on Monday, December 11, at 1:00 p.m. ET to discuss the RUBY and EdiTHAL clinical data.

Key data from patients treated in the RUBY trial and in the EdiTHAL trial will be shared, including:

- RUBY
  - Clinical data on 11 patients, including two patients with at least 12 months follow-up and an additional four patients with at least five months follow-up.
  - Efficacy data, including total hemoglobin, fetal hemoglobin, and vaso-occlusive events, or VOEs.
  - Safety data, including neutrophil and platelet engraftment.
- EdiTHAL
  - Clinical data on six patients, including at least five months data from the first two patients treated.
  - Efficacy data, including total hemoglobin and fetal hemoglobin.
  - Safety data, including neutrophil and platelet engraftment.

"We are making significant progress with EDIT-301, and we look forward to sharing clinical data, including additional data collected since the submission of the abstract, from the RUBY and EdiTHAL trials next month at the American Society of Hematology Annual Meeting and in a Company-sponsored webinar. This data further supports our belief that EDIT-301 has the potential to be a clinically differentiated, one-time, durable medicine that can provide life-changing clinical benefits to patients," Baisong Mei, M.D., Ph.D., Senior Vice President and Chief Medical Officer, Editas Medicine. "I would like to thank the participants, their families and caregivers, clinicians, and colleagues at collaborating institutions that contribute to the RUBY and EdiTHAL trials."

Webinar Presentation Details:
The live and archived webcast of the Company's webinar presentation will be accessible through this webcast link, or through the Events & Presentations page of the "Investors" section of the Company's website.

A replay of the webinar will be available upon conclusion of the webinar in the Investors section of the Editas Medicine website at https://www.editasmedicine.com/.

ASH Presentation Details:
Title: AsCas12a Gene Editing of *HBG1/2* Promoters with EDIT-301 Results in Rapid and Sustained Normalization of Hemoglobin and Increased Fetal Hemoglobin in Patients with Severe Sickle Cell Disease and Transfusion-Dependent Beta-Thalassemia
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: Monday, December 11, 2023, 6:00 – 8:00 p.m. PT/9:00 – 11:00 p.m. ET
Location: San Diego Convention Center, Halls G-H
Session: 801. Gene Therapies: Poster III
Publication Number: 4996

The abstract can be accessed on the ASH website.

EDIT-301 is currently being investigated in a clinical study in patients with severe sickle cell disease (RUBY trial, NCT04853576) and transfusion-dependent beta thalassemia (EDITHAL trial, NCT05444894).

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 gene editing 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 the RUBY Trial
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 the EdiTHAL Trial
The EdiTHAL 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 transfusion-dependent beta thalassemia. Patients will receive a single administration of EDIT-301. 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 CRISPR/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. 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. 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 and EdiTHAL trials, 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.

Media and Investor Contact:
Cristi Barnett
(617) 401-0113
cristi.barnett@editasmed.com

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