Editas Medicine to Present Clinical Data from the RUBY Trial of EDIT-301 for the Treatment of Severe Sickle Cell Disease at the EHA 2023 Congress

May 11, 2023

Company-sponsored webinar to be announced

CAMBRIDGE, Mass., May 11, 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 Phase 1/2 RUBY trial of EDIT-301 in patients with severe sickle cell disease has been accepted for an oral presentation at the European Hematology Association (EHA) Hybrid Congress being held June 8-11, 2023, in Frankfurt, Germany, and via live stream.

Key data from multiple patients will be shared in the oral presentation at EHA, confirming initial clinical data, include:

- Efficacy data, including total hemoglobin, fetal hemoglobin, percentage of F-cells, mean corpuscular fetal hemoglobin, and vaso-occlusive events (VOEs) post-infusion with EDIT-301.
- 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 that has been collected since the submission of the abstract, from the RUBY trial next month at the European Hematology Association Congress and in a Company-sponsored webinar. This data further supports our belief that EDIT-301 can be a potentially 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, clinicians, and colleagues at collaborating institutions that contribute to the RUBY trial."

The abstract can be accessed on the EHA website.

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

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). In addition to the clinical data update from the RUBY trial at EHA and in a Company-sponsored webinar next month, the Company will present a further clinical update from the RUBY trial by year-end. Additionally, the Company is on-track to dose 20 patients in the RUBY trial 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 (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 (NCT04853576).

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. 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 Company's expectation to provide a further clinical data update for the RUBY trial by year-end and to dose 20 total patients by year-end. 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, 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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