Editas Medicine Reports Updated Clinical Data from the RUBY Trial of Reni-cel in Patients with Severe Sickle Cell Disease at the American Society of Hematology (ASH) Annual Meeting

December 9, 2024

Poster presentation at ASH on Monday, December 9 at 6:00 p.m. PT / 9:00 p.m. ET

CAMBRIDGE, Mass., Dec. 09, 2024 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading gene editing company, will present updated safety and efficacy data in 28 patients living with severe sickle cell disease (SCD) treated with renizgamglogene autogedtemcel (reni-cel; formerly known as EDIT-301) in the Phase 1/2/3 RUBY clinical trial. The data will be presented by Dr. Rabi Hanna, M.D., Department of Pediatric Hematology Oncology and Blood and Marrow Transplantation, Cleveland Clinic Children's, during a poster presentation at the American Society of Hematology (ASH) Annual Meeting in San Diego, CA, today at 6:00 p.m. PT (9:00 p.m. ET).

In the RUBY trial as of the data cutoff date (October 29, 2024), reni-cel was well-tolerated and continued to demonstrate a safety profile consistent with myeloablative busulfan conditioning and autologous hematopoietic stem cell transplant by all patients (N=28). Patients were at a median of 9.5 months post-reni-cel infusion, with 11 patients having >1 year follow-up. Since reni-cel treatment, 27 of the 28 patients were free of vaso-occlusive events (VOEs). Patients were observed to have early normalization of total hemoglobin, with a mean total hemoglobin increasing from 9.8 g/dL at baseline to 13.8 g/dL at Month 6 (n=18). Patients were also observed to have rapid and sustained improvements in fetal hemoglobin (HbF) ≥40% and mean corpuscular concentration (MCH-F) of HbF per F-cells, well above the anti-sickling threshold. In addition, sustained clinically meaningful improvements were observed in patient-reported outcome domains for pain, physical function, and social roles and activities.

Efficacy data in Patients with Severe Sickle Cell Disease
Patients were a median (range) of 9.5 (0.7–25.2) months post-reni-cel infusion, with 11 patients having >1 year follow-up. Of 28 patients, 27 were VOE-free post-reni-cel infusion. Reni-cel administration led to early, robust increases and sustained levels of total Hb and HbF. At month 6, the mean total Hb was 13.8 g/dL with a mean HbF percentage of 48.1% (n=18).

The mean percentage of F-cells increased early and was sustained at >90% from month 4 through last follow-up (n=20). MCH-F of HbF-containing red cells (F-cells) increased early, with mean value of 16.3 pg/F-cells at month 4 visit and sustained above the anti-sickling threshold of 10 pg/F-cell through last follow-up. Markers of hemolysis, including absolute reticulocyte count, indirect bilirubin, lactate dehydrogenase, and haptoglobin, improved or normalized by Month 6 and were generally maintained or improved as of last follow-up.

Sustained clinically meaningful improvements were observed in pain, physical, and social patient-reported outcome domains following treatment with reni-cel.

Safety data in Patients with Severe Sickle Cell Disease
Reni-cel was well-tolerated and demonstrated a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant by all evaluated RUBY trial patients (N=28). After reni-cel infusion, all evaluable patients (n=27) achieved successful engraftment; with median time to neutrophil engraftment of 23 days and median time to platelet engraftment of 25 days, which is important for limiting infection and bleeding risk. Two serious adverse events (SAEs) assessed by the investigators as possibly related to reni-cel treatment have been reported in the RUBY trial.

RUBY Poster Presentation Details:
Title: Reni-Cel, an Investigational AsCas12a Gene-Edited Cell Medicine, Led to Sustained Hemoglobin Normalization and Increased Fetal Hemoglobin in Patients with Severe Sickle Cell Disease Treated in the RUBY Trial
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 9, 2024; 6:00 p.m. – 8:00 p.m. PT / 9:00 p.m. – 11:00 p.m. ET
Location: San Diego Convention Center, Halls G-H
Session: 801. Gene Therapies: Poster III

The poster can be accessed on the Editas Medicine website in the posters and presentations section.

About renizgamglogene autogedtemcel (reni-cel)
Reni-cel, formerly known as 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). Reni-cel 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 AsCas12a, a novel, proprietary, highly efficient, and specific gene editing nuclease. Red blood cells derived from reni-cel $CD34^+$ 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 1/2/3 study designed to assess the safety and efficacy of reni-cel in patients with severe sickle cell disease. Additional details are available on www.clinicaltrials.gov (NCT04853576).

About Editas Medicine
As a leading gene 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. Forward-looking statements in this press release include statements regarding the potential of, and expectations for, the Company's product candidates. 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 clinical trials, including the RUBY trial, and clinical development of the Company's product candidates, including reni-cel; 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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