Editas Medicine Reports New Safety and Efficacy Data from the RUBY Trial of Reni-cel in 18 Patients with Sickle Cell Disease, Presented at the European Hematology Association (EHA) Annual Congress

June 14, 2024

All patients treated in the RUBY trial are free of vaso-occlusive events post-renizgamglogene autogedtemcel (reni-cel) infusion

Patients had early normalization of total hemoglobin with a mean within the normal range at >14 g/dL and rapid and sustained improvements in fetal hemoglobin well above levels of >40%.

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

EHA RUBY oral presentation on Saturday, June 15 at 11:30 a.m. CEST/5:30 a.m. EDT

CAMBRIDGE, Mass., June 14, 2024 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage genome editing company, today announced new safety and efficacy data in 18 patients living with sickle cell disease (SCD) treated with renizgamglogene autogedtemcel (reni-cel; formerly known as EDIT-301) in the Phase 1/2/3 RUBY clinical trial. Reni-cel, the first investigational AsCas12a gene-edited cell therapy medicine, is being studied in the RUBY trial as a potential one-time, durable medicine for people living with severe SCD. The data will be presented in an oral presentation at the European Hematology Association (EHA) Hybrid Congress in Madrid, Spain and via livestream, on Saturday, June 15 at 11:30 a.m. CEST (5:30 a.m. EDT).

In the RUBY trial to date, reni-cel was well-tolerated and continues to demonstrate a safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant by all patients (N=18). Since treatment with reni-cel, patients have been free of vaso-occlusive events (VOEs) (N=18) for up to 22.8 months of follow-up. Patients had early normalization of total hemoglobin (Hb) with a mean within the normal range at >14 g/dL and rapid and sustained improvements in fetal hemoglobin (HbF) well above levels of >40%. Patients in the RUBY trial underwent a median of 2.0 apheresis and mobilization cycles (min: 1.0, max: 4.0).

“These data confirm the observations from our prior clinical readouts and further support our belief that reni-cel has the potential to be a best-in-class and clinically differentiated, one-time, durable medicine that can provide life-changing clinical benefits to patients,” said Baisong Mei, M.D., Ph.D., Chief Medical Officer, Editas Medicine. “Importantly, we continue to make significant progress in the development of reni-cel. In the RUBY trial, we have now dosed more than 20 patients, completed adult cohort enrollment, and opened and enrolled patients in the adolescent cohort. I would like to thank the participants, their families and caregivers, clinicians, and colleagues at collaborating institutions that contribute to the RUBY trial.”

“I am encouraged by these results from the RUBY trial, demonstrating this investigational gene editing medicine has been well-tolerated and shows promising efficacy for people living with sickle cell disease. Treatment with reni-cel showed a favorable safety profile and promising preliminary efficacy, supporting further investigation as a differentiated gene-edited medicine for patients with SCD. We look forward to continuing to evaluate its effectiveness on this patient population in need of treatment options,” said Rabi Hanna, M.D., Chairman of the Division of Pediatric Hematology Oncology and Blood and Marrow Transplantation at Cleveland Clinic Children’s, and the RUBY presenting investigator.

Efficacy of reni-cel in Patients with Severe Sickle Cell Disease
All patients (N=18) are free of VOEs since reni-cel infusion with follow-up ranging from 2.4 to 22.8 months.

Reni-cel treatment drives early, robust increases and sustained levels of total Hb and HbF. Across patients with ≥6 months follow-up, at month 6, the mean (standard deviation; SD) total Hb was 14.3 g/dL (2.1 g/dL) (n=9) with a mean (SD) HbF of 48.5% (3.7%) (n=10).

The mean percentage of F-cells increased early and were sustained at >90% from month 4 through subsequent follow-ups for all patients with ≥4 months follow-up (n=12).

Mean corpuscular fetal hemoglobin (MCH-F) of HbF-containing red cells (F-cells) was sustained above the anti-sickling threshold of 10 pg/F-cell by month 3 after reni-cel infusion for all patients with ≥3 months follow-up (n=14).

All patients in the RUBY trial showed sustained high levels of editing in the *HBG1* and *HBG2* promoter regions.

Markers of hemolysis have been normalized or improved in patients treated with reni-cel.

Safety of reni-cel 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=18).

After reni-cel infusion, all patients (N=18) demonstrated successful neutrophil and platelet engraftment. Neutrophil engraftment occurred at a median of 23 days (min: 15 days, max: 29 days), and platelet engraftment occurred at a median of 24 days (min: 18 days, max: 51 days).

No serious adverse events (SAEs) related to reni-cel treatment in the RUBY trial have been reported.

EHA Presentations
In addition to the RUBY oral presentation, Editas will also present data from the EdiTHAL clinical trial of reni-cel for the treatment of transfusion-dependent beta thalassemia in a poster presentation on Friday, June 14.

RUBY Oral Presentation Details:
Title: Reni-cel, the first AsCas12a gene-edited cell therapy, led to hemoglobin normalization and increased fetal hemoglobin in severe sickle cell disease patients in an interim analysis of 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: Saturday, June 15, 2024, 11:30 a.m. – 12:45 p.m. CEST/ 5:30 – 6:45 a.m. EDT
Location: Hall Velasquez, IFEMA MADRID Recinto Ferial (Fairgrounds)
Session: s425 Gene therapy, cellular immunotherapy and vaccination – Clinical

EdiTHAL Poster Presentation Details:
Title: Reni-cel, the first AsCas12a gene-edited cell therapy, shows promising preliminary results in key clinical outcomes in transfusion-dependent beta thalassemia patients treated in the EdiThal trial
Presenting Author: Haydar Frangoul, M.D., M.S., Medical Director, Sarah Cannon Pediatric Hematology/Oncology & Cellular Therapy at TriStar Centennial, Nashville, TN, United States
Date/Time: Friday, June 14, 2024, 6:00 – 7:00 p.m. CEST / Noon – 1:00 p.m. EDT
Location: Hall 7, IFEMA MADRID Recinto Ferial (Fairgrounds)
Session: Poster Session

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

Reni-cel 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 and EdiTHAL trials at EHA, the Company will present a further clinical update from both trials by year-end 2024.

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. Enrolled patients will receive a single administration of reni-cel. The RUBY trial marks the first time AsCas12a was used to successfully edit human cells in a clinical trial. Additional details are available on www.clinicaltrials.gov (NCT04853576).

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 reni-cel in patients with transfusion-dependent beta thalassemia. Patients will receive a single administration of reni-cel. Additional details are available on www.clinicaltrials.gov (NCT05444894).

About Editas Medicine
As a clinical-stage 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 timing for the Company's receipt and presentation of data from its clinical trials, including presenting additional clinical data from the RUBY and EdiTHAL trials by year-end 2024, and 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 and EdiTHAL trials, and clinical development of the Company's product candidates, including reni-cel; availability and timing of results from 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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