Editas Medicine to Present Clinical Data from the RUBY and EdiTHAL Trials of Reni-cel at the European Hematology Association 2024 Congress in June

May 14, 2024

CAMBRIDGE, Mass., May 14, 2024 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage gene editing company, today announced that two abstracts, including one oral presentation and one poster presentation, detailing clinical data from the RUBY and EdiTHAL trials of renizgamglogene autogedtemcel (reni-cel) have been accepted for presentation at the European Hematology Association (EHA) Hybrid Congress being held June 13-16, 2024, in Madrid, Spain, and via livestream.

Clinical data from patients treated in the Phase 1/2/3 RUBY trial of reni-cel in patients with severe sickle cell disease and in the Phase 1/2 EdiTHAL trial of reni-cel in patients with transfusion-dependent beta thalassemia will be shared, including:

- RUBY trial, Abstract #S285, oral presentation on Saturday, June 15:
  - Clinical data on at least 18 patients, with 2-21 months follow-up.
  - Efficacy data, including total hemoglobin, fetal hemoglobin, and vaso-occlusive events (VOEs).
  - Safety data, including neutrophil and platelet engraftment.

- EdiTHAL trial, Abstract #P1476, poster presentation on Friday, June 14:
  - Clinical data on seven patients, with 4-12 months follow-up.
  - Efficacy data, including total hemoglobin, fetal hemoglobin, and transfusion independence.
  - Safety data, including neutrophil and platelet engraftment.

“We are making significant progress with the continued development of reni-cel, and we look forward to sharing clinical data, including longer follow up of the dosed patients from the RUBY and EdiTHAL trials next month at the European Hematology Association Congress. These data further support our belief that reni-cel has the potential to be a 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. “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.”

The abstracts can be accessed on the EHA website.

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

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, 4:00 – 7:00 p.m. CEST / 10:00 a.m. – 1:00 p.m. EDT
Location: Hall 7, IFEMA MADRID Recinto Ferial (Fairgrounds)
Session: Poster Session

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 trial and the EdiTHAL trial at EHA next month, the Company will present a further clinical update from both trials by year-end.

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 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, 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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