# Editas Medicine Presents Preclinical Data Supporting the Initiation of the EDIT-301 Phase 1/2 RUBY Clinical Trial for the Treatment of Sickle Cell Disease at the European Hematology Association Congress

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*Preclinical data demonstrated robust fetal hemoglobin (HbF) induction in erythroid progeny cells with no detection of off-target editing; cells showed reduced sickling and improved rheological behavior*

*RUBY trial of EDIT-301 for sickle cell disease active and recruiting*

CAMBRIDGE, Mass., June 11, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced preclinical data supporting the initiation of the EDIT-301 Phase 1/2 RUBY clinical trial to evaluate EDIT-301, a one-time, durable, autologous cell therapy medicine to treat sickle cell disease. EDIT-301 is the first experimental medicine generated using CRISPR/Cas12a gene editing. The Company reported the data in an oral presentation at the 26th Congress of the European Hematology Association (EHA) being held virtually.

In these preclinical studies, CD34+ cells from normal donors and sickle cell patient donors were edited at the *HBG1* and *HBG2* promoters with Editas-engineered highly efficient and specific AsCas12a ribonucleoprotein (RNP) at research-scale and large-scale. The data demonstrated that high levels of editing were achieved, resulting in robust fetal hemoglobin (HbF) induction in erythroid cells with no detectable off-target editing. The red blood cells derived from edited sickle cell patient CD34+ cells showed significant reduction of sickling and improved rheological behavior. In addition, data from the Company's current Good Manufacturing Practices (cGMP) clinical scale process to support EDIT-301 manufacturing demonstrated successful scale-up production, with consistent and high-level editing of the *HBG1* and *HBG2* promoters while maintaining high specificity and robust HbF induction. Furthermore, stable and polyclonal long-term engraftment was observed when cells manufactured from the representative scale-up process were infused into immunodeficient mice.

"High levels of editing were achieved in CD34+ cells using the highly specific, Editas-engineered Cas12a RNP, leading to potentially therapeutically relevant levels of HbF expression. These findings further support our novel approach to developing EDIT-301 as a transformative, durable medicine for the potential treatment of sickle cell disease," said Kate Zhang, Ph.D., Vice President, Biological Development, Editas Medicine. "With the current non-clinical study results and early successful tests of manufacturability, we believe our approach may yield a safer and more effective medicine, with the possibility to change the lives of people living with sickle cell disease."

The RUBY clinical trial, a Phase 1/2 trial designed to assess the safety and efficacy of EDIT-301 for the treatment of sickle cell disease, is active and recruiting. The Company remains on track to begin patient dosing in the RUBY trial by the end of 2021.

**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 protein (HbS). In sickle cell disease, the red blood cells are misshapen, in a sickle shape instead of the disc shape. The abnormal shape causes the cells 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) inhibits HbS polymerization, thus reducing the manifestation of sickling.

**About EDIT-301**
EDIT-301 is an experimental, autologous cell therapy medicine under investigation for the treatment of sickle cell disease. EDIT-301 is comprised of sickle patient CD34+ cells genetically modified using a highly specific and efficient Editas-engineered CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP) that selectively targets the *HBG1* and *HBG2* promoters in the beta-globin locus where naturally occurring fetal hemoglobin (HbF) inducing mutations reside. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in HbF production, which has the potential to provide a one-time, durable treatment benefit for people living with sickle cell disease.

**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 people with severe sickle cell disease. Enrolled patients will receive a single administration of EDIT-301.

**About Editas Medicine**
As a leading genome editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas9 and CRISPR/Cas12a (also known as Cpf1) 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. 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 plans and expectations for EDIT-301, including beginning patient dosing in the RUBY trial by the end of 2021. 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 factors, including: uncertainties inherent in the initiation and completion of pre-clinical studies and clinical trials and clinical development of the

Company's product candidates; availability and timing of results from pre-clinical 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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