Editas Medicine Presents Pre-Clinical Data for Treatment of Sickle Cell Disease and Beta-Thalassemia at the 24th Congress of the European Hematology Association

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IND-enabling activities initiated for EDIT-301, a potentially best-in-class experimental medicine for the treatment of sickle cell disease and beta-thalassemia

CAMBRIDGE, Mass., June 15, 2019 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced results from a follow-up study to assess two different CRISPR genome editing strategies, one targeting the *BCL11A* erythroid enhancer (*BC11Ae*) and one targeting the beta-globin locus, for the treatment of sickle cell disease and beta-thalassemia. The Company reported the data at the 24th Congress of the European Hematology Association in Amsterdam.

In this study, NBSGW mice received an infusion of human CD34+ cells which had been edited either at the *BCL11Ae* or at the beta-globin locus. *In vivo*-derived erythroid cells from *BCL11Ae*-edited CD34+ hematopoietic stem/progenitor cells had reduced total indels and increased non-productive indels as compared to other tested lineages, a phenomenon not observed with beta-globin locus editing. Additionally, further optimization of nuclease and guide RNA combinations led to fetal hemoglobin expression of approximately 40 percent in the beta-globin locus-edited erythroid cells. Based on the data, Editas Medicine has initiated IND-enabling activities for EDIT-301, an experimental CRISPR medicine designed to durably treat sickle cell disease and beta-thalassemia by editing the beta-globin locus.

“We are encouraged by these pre-clinical results demonstrating cells edited at the beta-globin locus repopulated all lineages of the blood system including, importantly, the red blood cell precursors and the high percentage of fetal hemoglobin expression. Editing at this site continues to meet our preclinical goals for making a medicine including robust, long-term induction of fetal hemoglobin and maintenance of normal hematopoietic stem/progenitor cell function,” said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine. “Our program is on track towards the clinic, and we have started our IND-enabling activities as we look to develop a best-in-class medicine for the treatment of sickle cell disease and beta-thalassemia.”

AboutEditas Medicine
As a leading genome editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas9 and CRISPR/Cpf1 (also known as 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. 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. 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 Quarterly Report on Form 10-Q, 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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