## Editas Medicine Announces Pre-Clinical Data for Program to Treat Sickle Cell Disease and Beta-Thalassemia

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CAMBRIDGE, Mass., May 11, 2017 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today announced results from its pre-clinical hematopoietic stem cell (HSC) program confirming the potential for CRISPR-based editing to treat sickle cell disease and beta-thalassemia. In an oral presentation today at the 20<sup>th</sup> Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) in Washington, D.C., the Company presented data demonstrating a substantial increase in fetal hemoglobin protein using a novel and potent genome editing strategy.

In pre-clinical experiments, human HSCs were edited *ex vivo* and infused into mice. Four months following transplant, the bone marrow was collected to evaluate engraftment and to assess HSC contribution to blood production. In this study, successful and durable repopulation of the blood system *in vivo* was observed. In addition, analysis of the relationship between editing frequency and the level of fetal hemoglobin production suggests this proprietary editing approach has the potential to be more potent than other approaches that have been previously reported. These data support the continued development of medicines for people with genetically-defined and genetically-treatable diseases of the blood.

"We are encouraged by these results demonstrating the potential for edited hematopoietic stem cells to provide a durable therapy for patients with  $\beta$ -hemoglobinopathies," said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine. "We believe durable repopulation of the blood system with edited stem cells is a critical component for developing genomic medicines for patients with genetic blood diseases. With this *in vivo* proof-of-concept, we are continuing to advance our program to develop differentiated medicines with our novel, potent editing strategy."

## **About Editas Medicine**

Editas Medicine is a leading genome editing company dedicated to treating patients with genetically-defined diseases by correcting their disease-causing genes. The Company was founded by world leaders in genome editing, and its mission is to translate the promise of genome editing science into a broad class of transformative genomic medicines to benefit the greatest number of patients.

## **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," 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 preclinical studies and clinical trials and clinical development of the Company's product candidates; availability and timing of results from preclinical 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 as a result of new information, future events or otherwise.

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