Editas Medicine Initiates Clinical Natural History Study to Evaluate Patients with Leber Congenital Amaurosis Type 10 (LCA10)

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Massachusetts Eye and Ear named as first site for the study

CAMBRIDGE, Mass., Sept. 12, 2017 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today announced that the Company initiated a clinical natural history study of Leber Congenital Amaurosis type 10 (LCA10). LCA10 is caused by mutations in the *CEP290* gene. The study will prospectively evaluate patients to assess the course of the disease and to pilot potential clinical trial endpoints and designs. This knowledge will inform the interventional clinical trial design for EDIT-101, Editas Medicine's pre-clinical product candidate to treat LCA10.

"We are very excited with the progress we are making in our LCA10 program. The initiation of this natural history study brings us one step closer to our goal of making a CRISPR-based medicine available to people with significant vision loss caused by LCA10," said Gerry Cox, M.D., Ph.D., Chief Medical Officer, Editas Medicine. "The data generated in this study will increase our knowledge of the disease and its impact on vision-related activities. It is a key step towards interventional clinical trials, and we are pleased to be working with Massachusetts Eye and Ear."

Editas Medicine plans to enroll approximately 40 patients, ages three and above, at multiple sites in the U.S. and Europe in this study. The study will evaluate and follow patients for at least one year. Massachusetts Eye and Ear, an international center for treatment and research and a teaching hospital of Harvard Medical School, is the first site enrolling patients for this study.

"We are delighted to be working at the forefront of this research with Editas Medicine to better understand the course of disease for patients with *CEP290*-associated retinal degeneration, and to be working towards the interventional study," said Eric A. Pierce, M.D., Ph.D., Director of the Ocular Genomics Institute and Solman and Libe Friedman Professor of Ophthalmology at Massachusetts Eye and Ear and Harvard Medical School, and Principal Investigator for the study.

Editas Medicine plans to submit an Investigational New Drug (IND) application for EDIT-101 in mid-2018. In March, Editas Medicine and Allergan Pharmaceuticals International Limited ("Allergan") entered into a strategic research and development alliance under which Allergan received an exclusive option to license up to five of Editas Medicine's genome editing ocular programs, including Editas Medicine's lead program for LCA10. The agreement covers a range of first-in-class ocular programs targeting serious, vision-threatening diseases based on Editas Medicine's unparalleled CRISPR genome editing platform, including CRISPR/Cas9 and CRISPR/Cpf1.

About Leber Congenital Amaurosis
Leber Congenital Amaurosis, or LCA, is a group of inherited retinal degenerative disorders caused by mutations in at least 18 different genes. It is the most common cause of inherited childhood blindness, with an incidence of two to three per 100,000 live births worldwide. Symptoms of LCA appear within the first year of life, resulting in significant vision loss and blindness. The most common form of the disease, LCA10, is a monogenic disorder caused by mutations in the CEP290 gene and is the cause of disease in approximately 20-30 percent of all LCA patients.

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," 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 goals for the Clinical Natural History study and goal of submitting of an IND for the LCA10 program by mid-2018. 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 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 as a result of new information, future events or otherwise.

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