Editas Medicine Reports Data Demonstrating Subretinal Injection of EDIT-101 Well-tolerated in Non-human Primates

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Presentation at the Annual Meeting of the American Society of Gene & Cell Therapy

CAMBRIDGE, Mass., May 18, 2018 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today announced results from a pre-clinical study demonstrating that EDIT-101 was well-tolerated when administered to non-human primates (NHP) via subretinal injection. EDIT-101 is the Company's experimental medicine for the treatment of Leber Congenital Amaurosis type 10 (LCA10). The Company reported these data today in an oral presentation at the 21st Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) in Chicago.

"In this study, we administered either EDIT-101 or a non-human primate surrogate vector using the procedure that we plan to use in the Phase 1/2 study, and we found that EDIT-101 was well-tolerated over the duration of the study based on a panel of clinical tests. Importantly, neither the presence of pre-existing nor induced immunity in non-human primates to either AAV5 or Staph. aureus Cas9 impacted productive editing," said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine. "To date, our comprehensive set of pharmacology, specificity, tolerability, and immunogenicity data gives us substantial confidence in EDIT-101 for the treatment of LCA10 as we make progress towards the clinic and towards our goal of making a CRISPR-based medicine for people suffering from this devastating eye disease."

In this study, non-human primates were given a subretinal injection of vehicle control, EDIT-101, or VIR-067, an NHP-surrogate vector, with or without prophylactic, transient steroid treatment, and followed for six to 13 weeks. Ocular tolerability was assessed by ophthalmic examination using the modified SUN, Hackett-McDonald and SPOTS uveitis scoring systems and intraocular pressure measurements. Surgery procedure-related responses were mild, transient, and comparable between vehicle- and EDIT-101 or VIR-067-treated eyes.

Both EDIT-101 and VIR-067 were well tolerated in animals treated with prophylactic steroids. Delayed, mild inflammation was observed infrequently in animals that were not treated with prophylactic steroids. There was no delayed inflammation observed in EDIT-101 treated eyes after discontinuation of prophylactic steroids. Comparable intraocular pressures were reported between vehicle- and EDIT-101- or VIR-067-treated eyes throughout the study. Additionally, low levels of pre-existing or induced immunity to *Staphylococcus aureus* Cas9 (SaCas9) did not correlate with ocular inflammation, whereas the anti-AAV5 capsid immune response in non-immunosuppressed animals may contribute to delayed ocular inflammation. Nevertheless, neither SaCas9- nor AAV5-specific immunity impacted the pharmacological activities of the study drug.

Editas Medicine aims to file an Investigational New Drug (IND) application for EDIT-101 by mid-2018. In March 2017, Editas Medicine and Allergan Pharmaceuticals International Limited (Allergan) entered into a strategic 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 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
As a leading genome editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas9 and CRISPR/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 a statement regarding the Company's goal of filing 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 because of new information, future events or otherwise.

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