Editas Medicine Announces Clinical Data Demonstrating Proof of Concept of EDIT-101 from Phase 1/2 BRILLIANCE Trial

November 17, 2022

EDIT-101 demonstrates a favorable safety profile across all dose cohorts

Preliminary efficacy signals of consistent improvement in BCVA plus additional efficacy endpoints seen in homozygous patients

Achieved proof of concept and identified a responder population

In view of the small population, the Company will pause enrollment in the BRILLIANCE trial and seek to identify a collaboration partner to continue development of EDIT-101

Company to host a webinar today at 8:00 a.m. ET

CAMBRIDGE, Mass., Nov. 17, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical stage genome editing company, today announced clinical data from the Phase 1/2 BRILLIANCE trial of EDIT-101, an *in vivo* CRISPR/Cas9 genome editing medicine in a Company-sponsored webinar. EDIT-101 is under development for the treatment of blindness due to Leber congenital amaurosis 10 (LCA10, a *CEP290*-related retinal degenerative disorder) and is designed to repair the IVS26 CEP290 mutant allele that impacts approximately 1,500 LCA10 patients in the U.S. There is no effective treatment currently available for this serious, rare disease. The BRILLIANCE update includes safety and efficacy data from all 14 patients treated in the study to date, which includes 12 adult patients and two pediatric patients.

Three out of 14 treated subjects met a responder threshold having experienced clinically meaningful improvements in best corrected visual acuity (BCVA) (LogMAR >0.3) and demonstrated consistent improvements in two of the following three additional endpoints: full field sensitivity test (FST), visual function navigation course (VFN), or the visual function quality of life (VFQ).

An examination of baseline characteristics of the treatment responder patients revealed that two of the three responders were homozygous for IVS26 mutation (2/2; 100% of the homozygous patients treated). No other baseline characteristics that could pre-select a responder patient population were identified in the BRILLIANCE dataset.

EDIT-101 was tolerated with no ocular serious adverse events or dose-limiting toxicities observed. Most adverse events were mild and expected for subretinal delivery.

Since LCA10 patients homozygous for CEP290 IVS26 mutation represent an estimated population of approximately 300 in the U.S., the Company will not progress this program independently, and will seek to identify a collaboration partner to continue the development of EDIT-101. As a result, Editas Medicine is pausing further enrollment in the BRILLIANCE trial and will continue long term follow-up of all patients who have been treated to date.

“The results from the BRILLIANCE trial provide a proof of concept and important learnings for our inherited retinal disease programs. We’ve demonstrated that we can safely deliver a CRISPR-based gene editing therapeutic to the retina and have clinically meaningful outcomes,” said Gilmore O’Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine. “While we will not progress EDIT-101 on our own and have made the decision to pause enrollment, we have the patient community top of mind and are looking for a collaboration partner to advance this program.”

Webinar Information
Editas Medicine will host a webinar today, Thursday, November 17, at 8:00 a.m. ET to present the data. The live and archived webcast of the presentation will be accessible through this webcast link, or through the Events & Presentations page of the “Investors” section of the Company’s website. The presentation will also be available for download shortly after the webinar.

About EDIT-101
EDIT-101 is a CRISPR/Cas9-based experimental medicine under investigation for the treatment of Leber congenital amaurosis 10 (LCA10), by deleting the IVS26 CEP290 mutant allele. EDIT-101 is administered via a subretinal injection to reach and deliver the gene editing machinery directly to photoreceptor cells. EDIT-101 has been granted Rare Pediatric Disease and Orphan Drug designations from the U.S. Food and Drug Administration (FDA) and Orphan Medicinal Product designation from the European Medicines Agency (EMA).

About BRILLIANCE
The BRILLIANCE Phase 1/2 clinical trial of EDIT-101 for the treatment of Leber congenital amaurosis 10 (LCA10), a *CEP290*-related retinal degenerative disorder, is designed to assess the safety, tolerability, and efficacy of EDIT-101 in up to 34 patients with this disorder. Clinical trial sites are enrolling up to five cohorts testing up to three dose levels in this open label, multi-center study. Both adult and pediatric patients (3 – 17 years old) with a range of baseline visual acuity assessments are eligible for enrollment. Patients receive a single administration of EDIT-101 via subretinal injection in one eye. Patients are monitored every three months for a year after dosing and less frequently for an additional two years thereafter. Additional details are available on www.clinicaltrials.gov (NCT#03872479).

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 approximately three per 100,000 live births worldwide. Symptoms of LCA appear within the first years of life, resulting in significant vision loss and potentially blindness. The most common form of the disease, LCA10 or a *CEP290*-related retinal degenerative disorder, 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 clinical stage genome editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas9 and CRISPR/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. Editas Medicine is the exclusive licensee of Broad Institute and Harvard University's Cas9 patent estates and Broad Institute's Cas12a patent estate for human medicines. 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, including statements regarding the Company's plans to seek a collaboration partner to continue development of EDIT-101. 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 important factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials, including the BRILLIANCE trial, and clinical development of the Company's product candidates; the ability to establish and maintain a collaboration on favorable terms, if at all and the success of any such collaboration that the Company enters into; 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, as updated by the Company's subsequent filings 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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