Editas Medicine Announces Dosing of First Pediatric Patient in the BRILLIANCE Clinical Trial of EDIT-101 for LCA10

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Marks the first-ever in vivo delivery of an experimental CRISPR gene editing medicine to a pediatric patient

Company on track to complete dosing of the pediatric mid-dose cohort in the first half of 2022 and expects to initiate dosing of the pediatric high-dose cohort this year

CAMBRIDGE, Mass., April 11, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced the administration of EDIT-101, an experimental CRISPR gene editing medicine, to the first pediatric patient enrolled in the BRILLIANCE clinical trial, which is designed to test the safety of EDIT-101 for the treatment of Leber congenital amaurosis 10 (LCA10), a *CEP290*-related retinal degenerative disorder. This marks the world's first *in vivo,* or inside the body, dosing of a pediatric patient with a CRISPR gene editing experimental medicine.

"Administering the experimental medicine to the first pediatric patient in the BRILLIANCE trial marks a significant milestone toward delivering on the potential of CRISPR gene editing medicines being safe and effective in treating LCA10, which often results in significant vision loss and blindness early in life," said James C. Mullen, Chairman, President, and CEO, Editas Medicine. "Currently, there are no approved treatments for LCA10, and we look forward to sharing future updates from the BRILLIANCE trial, including sharing additional clinical data, later this year."

"Enrolling this first pediatric patient in the BRILLIANCE trial is an important step toward bringing potentially life-changing treatments to children with genetic retinal diseases. We are excited to be involved in research focused on testing potential new treatments for untreatable diseases like LCA10," said trial principal investigator for the site, Tomas S. Aleman, MD, the Irene Heinz-Given and John LaPorte Research Associate Professor at the Scheie Eye Institute of the Perelman School of Medicine at the University of Pennsylvania, and a retinal degeneration specialist with the Division of Pediatric Ophthalmology at Children's Hospital of Philadelphia (CHOP).

Albert M. Maguire, MD, the F.M. Kirby Professor of Molecular Ophthalmology at Penn and a member of the Center for Advanced Retinal and Ocular Therapeutics, is the surgeon in the trial, in collaboration with Children's Hospital of Philadelphia (CHOP), the nation's first hospital devoted exclusively to the care of children and the source of many breakthroughs and firsts in pediatric medicine. CHOP's Clinical In Vivo Gene Therapy (CIGT) program provided the clinical operations support to conduct the work at CHOP.

Editas Medicine initiated enrollment in the pediatric mid-dose cohort in the BRILLIANCE trial following the Independent Data Monitoring Committee (IDMC) endorsement based on an analysis of safety data from a clinical trial in adult patients that tested low-dose and mid-dose levels of the experimental medicine. The Company remains on track to complete testing of the pediatric mid-dose in the first half of 2022 and expects to initiate testing of the pediatric high-dose this year.

Previously, Editas Medicine completed dosing of all adult cohorts in its BRILLIANCE study and announced preliminary EDIT-101 clinical results demonstrated a favorable safety profile and encouraging signals of clinical benefit. The Company expects to provide a clinical update on the BRILLIANCE trial in the second half of 2022. The update is expected to provide safety and efficacy assessments on all adult patients who have had at least six months of follow-up evaluations, which will include at least 12 months of data on the adult mid-dose cohort, and at least six months of data on the adult high-dose cohort. Additionally, the Company is expanding enrollment in one or more of the previously completed adult cohorts to explore dose response and support establishment of registrational trial endpoints, which are anticipated by year-end.

About EDIT-101
EDIT-101 is a CRISPR/Cas9-based experimental medicine under investigation for the treatment of Leber congenital amaurosis 10 (LCA10), a CEP290-related retinal degenerative disorder. 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 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) is designed to assess the safety, tolerability, and efficacy of EDIT-101 in 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 two to 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, 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/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. Forward-looking statements in this press release include statements regarding the initiation, timing, progress and results of the Company's preclinical and clinical studies and its research and development programs, including completing dosing of the pediatric mid-dose cohort in the first half of 2022, initiating dosing of the pediatric high-dose cohort in the BRILLIANCE trial in 2022, and establishing registrational trial criteria by year-end 2022, and the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including a clinical update on the BRILLIANCE trial in the second half of 2022. 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 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 represent the Company's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, the Company explicitly disclaims any obligation to update any forward-looking statements.

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