



Editas Medicine Reports on Recent Progress and Outlook at J.P. Morgan Healthcare Conference

January 11, 2021

Details progress on ocular programs, including EDIT-101 Phase 1/2 BRILLIANCE trial updates

Provides EDIT-301 updates, including timeline for dosing sickle cell disease patients in the Phase 1/2 RUBY trial and plans to file IND for the treatment of beta-thalassemia

CAMBRIDGE, Mass., Jan. 11, 2021 (GLOBE NEWSWIRE) -- In a presentation to investors on Wednesday, January 13 at 10:50 a.m. EST at the 39th Annual J.P. Morgan Healthcare Conference being held virtually, Editas Medicine, Inc. (Nasdaq: EDIT), President and Chief Executive Officer Cynthia Collins will discuss the Company's recent progress and outlook for its *in vivo* Gene EDITed medicines and *ex vivo* Gene EDITed cell medicines.

In her remarks, Ms. Collins will discuss several components of the Company's progress and detail timelines, including plans to:

- Accelerate enrollment in the Phase 1/2 BRILLIANCE clinical trial of EDIT-101 for the treatment of Leber congenital amaurosis 10 (LCA10) with a revised protocol, and dose first patient in the adult mid-dose cohort in Q1 2021;
- Share initial clinical data from the BRILLIANCE trial by the end of 2021;
- Declare a development candidate for the treatment of autosomal dominant retinitis pigmentosa 4 (RP4) by the end of 2021;
- Initiate dosing in the Phase 1/2 RUBY clinical trial of EDIT-301 for the treatment of sickle cell disease in 2021;
- File an Investigational New Drug (IND) application for EDIT-301 for the treatment of beta-thalassemia by the end of 2021; and
- Accelerate the development of engineered iPSC-derived NK (iNK) cell medicines for the treatment of solid tumor cancers.

"2020 was a momentous year towards our goal of developing differentiated, transformational medicines for people living with serious diseases. We began the year by making history with the first ever clinical trial of an *in vivo* Gene EDITed medicine and ended the year by filing an IND for our first *ex vivo* cell medicine, EDIT-301," said Collins. "Building on these successes, I am confident our strong momentum will continue in 2021 as we dose additional patients and share clinical data from the BRILLIANCE trial, bring EDIT-301 into the clinic, file our third IND, and advance our *in vivo* and *ex vivo* Gene EDITed medicine programs."

In addition to sharing details on the Company's progress and achievements, Ms. Collins will also discuss timelines and outlook for 2021:

In Vivo Medicines Outlook

- Advance multiple *in vivo* Gene EDITed medicines to solidify position as the global leader in *in vivo* gene editing.
- Progress ocular medicines pipeline following reacquisition of rights from AbbVie.
- Accelerate patient enrollment with a revised protocol in the BRILLIANCE clinical trial for EDIT-101.
- Dose first patient in the adult mid-dose cohort of BRILLIANCE trial in Q1 2021.
- Share EDIT-101 clinical data at a scientific conference by the end of 2021.
- Declare a development candidate medicine for the treatment of Retinitis Pigmentosa Type 4 (adRP4) by the end of 2021.
- Establish preclinical proof-of-concept in a neurological indication and conduct large animal studies.

Ex Vivo Cell Medicines Outlook

- Initiate the Phase 1/2 RUBY trial of EDIT-301 for the treatment of sickle cell disease.
- File an IND for EDIT-301 for the treatment of beta-thalassemia by the end of 2021.
- Leverage EDIT-201 preclinical efforts to enhance the development of iNK cell medicines for solid tumors and discontinue development of EDIT-201 (healthy donor cell medicine).
- Progress the Company's collaboration with Bristol-Myers Squibb to advance alpha-beta T cell medicines for the treatment of solid and liquid tumors.

Organizational Excellence and Scaling for Growth Outlook

- Advance pipeline through new and existing collaborations and partnerships with industry leaders Bristol-Myers Squibb, BlueRock Therapeutics LP, and AskBio.
- Continue to advance internal and external manufacturing capabilities for the Company's portfolio of *in vivo* gene EDITed medicines and *ex vivo* gene EDITed engineered cell medicines.
- Add additional expertise and talent in key areas including clinical, manufacturing, and research to continue advancing the Company's pipeline towards the clinic.

About EDIT-101

EDIT-101 is a CRISPR-based experimental medicine under investigation for the treatment of Leber congenital amaurosis 10 (LCA10). EDIT-101 is

administered via a subretinal injection to reach and deliver the gene editing machinery directly to photoreceptor cells.

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 up to 18 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. Additional details are available on www.clinicaltrials.gov (NCT#03872479).

About EDIT-301

EDIT-301 is an experimental, autologous cell therapy medicine under investigation for the treatment of sickle cell disease. EDIT-301 is comprised of sickle patient CD34+ cells genetically modified using a highly specific and efficient CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP) that targets the *HBG1* and *HBG2* promoters in the beta-globin locus where naturally occurring fetal hemoglobin (HbF) inducing mutations reside. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in HbF production, which has the potential to provide a durable treatment benefit for people living with sickle cell disease.

About RUBY

The RUBY Trial is a single-arm, open-label, multi-center Phase 1/2 study designed to assess the safety and efficacy of EDIT-301 in patients with severe sickle cell disease. Enrolled patients will receive a single administration of EDIT-301.

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 (also known as 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 statements regarding the initiation, timing, progress and results of the Company's preclinical and clinical studies and its research and development programs, including dosing the first patient in the adult mid-dose cohort in the BRILLIANCE trial in Q1 2021, the initiation of the RUBY trial and filing an IND for EDIT-301 for the treatment of beta-thalassemia by the end of 2021, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, and the timing or likelihood of regulatory filings and approvals. 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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