



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

January 13, 2020

*Provides updates on advancements in in vivo and engineered cell medicine programs*

*Details timeline to filing an IND for EDIT-301 for the treatment of sickle cell disease*

CAMBRIDGE, Mass., Jan. 13, 2020 (GLOBE NEWSWIRE) -- In a presentation to investors on Wednesday, January 15, 2020, at 10:30 a.m. PST at the 38th Annual J.P. Morgan Healthcare Conference, Editas Medicine, Inc. (Nasdaq: EDIT) President and CEO Cynthia Collins will discuss the Company's progress on developing *in vivo* and engineered cell medicines and building the leading genomic medicine company.

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

- Dose the first patient in the Brilliance clinical trial of EDIT-101 (AGN-151587) for the treatment of Leber congenital amaurosis 10 (LCA10) in the first quarter of 2020 and complete dosing the adult low- and mid-dose cohorts by the end of the year;
- File an Investigational New Drug (IND) application for EDIT-301 for the treatment of sickle cell disease by the end of 2020;
- Initiate IND-enabling studies for engineered natural killer (NK) cell medicine for the treatment of solid tumors;
- Advance alpha-beta T cell medicines in partnership with Bristol-Myers Squibb Company;
- Establish *in vivo* preclinical proof-of-concept for an engineered iPSC-derived NK (iNK) cell medicine; and
- Establish *in vivo* preclinical proof-of-concept for a neurological indication.

"We are entering 2020 with strong momentum and a strategic focus on driving our pipeline of *in vivo* CRISPR and engineered cell medicines forward with the ultimate vision of developing differentiated, transformational medicines for people living with serious diseases," said Collins. "Our team is making history with the first ever clinical trial of an *in vivo* CRISPR medicine, advancing our broader pipeline of *in vivo* CRISPR medicines, and progressing our engineered cell medicines for hemoglobinopathies and cancers. With our recent achievements, I expect our clinical pipeline to yield a robust and sustainable portfolio of differentiated, transformative medicines and ensure the Company's long-term growth."

In addition to sharing details on the Company's progress and timelines, Ms. Collins will also discuss recent achievements and outlook for 2020:

### ***Progress in In Vivo CRISPR Medicines***

- EDIT-101 is on track to be the first *in vivo* CRISPR-based genome editing medicine with first patient dosing expected in the first quarter of 2020.
- EDIT-102 development candidate declared for the treatment of Usher syndrome 2A (USH2A).
- Declare a development candidate for Autosomal Dominant Retinitis Pigmentosa Type 4 (adRP4) in 2020.
- Plans to establish *in vivo* preclinical proof-of-concept for a neurological indication in 2020 from collaboration with Asklepios BioPharmaceutical, Inc. (AskBio).

### ***Advancing Engineered Cell Medicines***

- File an IND for EDIT-301 for the treatment of sickle cell disease by the end of 2020.
- Progress on the Company's collaboration with Bristol-Myers Squibb to advance alpha-beta T cell medicines for the treatment of both solid and liquid tumors.
- Declare a development candidate and initiate IND-enabling activities this year for a gene edited healthy donor NK cell medicine.
- Progress towards establishing *in vivo* preclinical proof-of-concept for an engineered iNK cell medicine using technology from BlueRock Therapeutics.

### ***Advancing Organizational Excellence and Scaling for Growth***

- Advanced and strengthened pipeline through multiple collaborations and licensing agreements, including Bristol-Myers Squibb, BlueRock Therapeutics LP, AskBio, MaxCyte, Inc., and Sandhill Therapeutics, Inc.
- Strengthened the Company's executive team with hiring of a permanent Chief Executive Officer, Chief Financial Officer, Chief Medical Officer, and Senior Vice President, Operations (Manufacturing).

### **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 deliver the gene editing machinery directly to photoreceptor cells.

### **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 the Editas Medicine-Allergan Alliance**

In March 2017, Editas Medicine and Allergan Pharmaceuticals International Limited (Allergan) entered a strategic alliance and option agreement under which Allergan received exclusive access and the option to license up to five of Editas Medicine's genome editing programs for ocular diseases, including EDIT-101. Under the terms of the agreement, Allergan is responsible for development and commercialization of optioned products, subject to Editas Medicine's option to co-develop and share equally in the profits and losses of two optioned products in the United States. In August 2018, Allergan exercised its option to develop and commercialize EDIT-101 globally for the treatment of LCA10. Additionally, Editas Medicine exercised its option to co-develop and share equally in the profits and losses from EDIT-101 in the United States. Editas Medicine is also eligible to receive development and commercial milestones, as well as royalty payments on a per-program basis. 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 (also known as Cas12a).

#### **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](http://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 clinical trial timeline of EDIT-101, the timeline for filing an IND for EDIT-301 and the research timelines for the Company's other research programs. 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 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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