



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

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CAMBRIDGE, Mass., Jan. 07, 2019 (GLOBE NEWSWIRE) -- In a presentation to investors on Wednesday, January 9, 2019, at 9:00 a.m. PST at the 37th Annual J.P. Morgan Healthcare Conference, Editas Medicine, Inc. (Nasdaq: EDIT) President and CEO Katrine Bosley will discuss the Company's plans to initiate patient screening and patient dosing for EDIT-101. Additionally, she will outline progress in the Company's program for the treatment of sickle cell disease, including data that support opportunities to develop best-in-class, durable medicines for hemoglobinopathies. Ms. Bosley will also detail the Company's progress on "EM22," the Company's long-range goals through the year 2022 and vision for advancing Editas Medicine as a leading genome editing company, including sharing details on advancements in its ocular and engineered cell medicine programs.

EDIT-101 is an experimental CRISPR genome editing medicine being investigated for the treatment of Leber congenital amaurosis 10 (LCA10). It is set to be the first *in vivo*, or editing inside the body, CRISPR-based medicine administered to people anywhere in the world. In the Phase 1/2 clinical trial, Editas Medicine and Allergan plan to initiate patient screening mid-year and begin patient dosing in the second half of 2019, enrolling 10-20 patients in the U.S. and Europe.

"At Editas Medicine, we are pioneering the possible by harnessing the power of genome editing, engineered cell therapy and AAV gene delivery to develop a pipeline of genomic medicines for people living with serious diseases," said Bosley. "With our recent successes, including the FDA's acceptance of our IND for EDIT-101, we are entering 2019 with strong momentum towards achieving our EM22 goals. We look forward to entering the clinic later this year, and we hope to transform the lives of people living with LCA10."

Ms. Bosley will also provide an update on the Company's progress on EM22. By year-end 2022, Editas Medicine is driving to deliver medicines for people with serious diseases around the world by advancing at least three experimental medicines in early-stage clinical trials, at least two experimental medicines in or ready for late-stage clinical trials, a best-in-class platform and pipeline for developing genomic medicines, and building the company for the long term with a unique, "Inspiritas" culture. Recent achievements include:

### *Continued Commitment to Ocular Disorders*

- The LCA10 program is on track to be the first *in vivo* CRISPR-based genome editing medicine with patient dosing expected in the second half of 2019.
- The Company now has ocular programs in early research to treat Usher syndrome 2A (USH2A) and retinitis pigmentosa.

### *Important progress in engineered cell medicines*

- The Company made recent advances toward a durable medicine for sickle cell and beta-thalassemia. Editing at the *HBG1/2* site is a differentiated approach for development of a human therapeutic for the treatment of sickle cell disease and beta-thalassemia as compared to other medicines currently under development that edit at the *BCL11A* erythroid enhancer (*BCL11Ae*) site. Notably, editing *HBG1/2* promoters upregulated fetal hemoglobin with superior repopulation of red blood cell precursors as compared to editing the *BCL11Ae* site. The red blood cell precursors from bone marrow edited at the *BCL11Ae* site had lower productive editing rates compared to other lineages and showed increased level of apoptosis, or programmed cell death, in erythroid culture compared to *HBG1/2* promoter-edited cells.
- In the Company's collaboration with Juno Therapeutics, Inc., a Celgene company, CRISPR-edited product candidates are advancing in both solid and liquid tumors.

### *Advancing Organizational Excellence*

- The Company added key talent across hematology, oncology, ophthalmology, manufacturing, and *ex vivo* (editing outside the human body) research in 2018. This expertise is critical to the continued advancement of Editas Medicine's pipeline and platform.

### **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 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/Cpf1 (also known as 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](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 and the Company's EM22 goals. 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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