



Editas Medicine Announces Third Quarter 2019 Results and Update

November 12, 2019

Amended Celgene collaboration to focus on engineered alpha-beta T cell medicines with a \$70 million payment to Editas Medicine

Appointed Judith R. Abrams, M.D., as Chief Medical Officer

EDIT-101 (AGN-151587) for LCA10 first patient dosing expected by early 2020

EDIT-301 for hemoglobinopathies in vivo pre-clinical data to be presented at ASH

CAMBRIDGE, Mass., Nov. 12, 2019 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the third quarter of 2019.

"Our momentum in 2019 remains strong in advancing our pipeline of *in vivo* CRISPR and engineered cell medicines," said Cynthia Collins, Chief Executive Officer of Editas Medicine. "We announced this morning an amended agreement with Celgene to further expand and accelerate our oncology pipeline. In hemoglobinopathies, we look forward to presenting *in vivo* pre-clinical data for EDIT-301 at ASH that supports its potential as a best-in-class medicine. Finally, we eagerly anticipate first patient dosing with EDIT-101 for LCA10 in the coming months."

Recent Achievements and Outlook

In Vivo CRISPR Medicines

- **EDIT-101 (AGN-151587) for LCA10**

First patient dosing expected by early 2020

Editas Medicine and its partner, Allergan, are conducting the Brilliance Phase 1/2 clinical trial to evaluate the safety, tolerability, and efficacy of EDIT-101 as a treatment for Leber congenital amaurosis 10 (LCA10). The first potential patient has been successfully screened and confirmation of the first surgery to administer EDIT-101 by early 2020 is pending.

- **Usher Syndrome 2A**

Expect to be ready for IND-enabling studies by YE19

Pre-clinical *in vitro* data were presented at the 27th Annual Congress of the European Society of Gene & Cell Therapy. The Company's lead candidate demonstrated successful knock-out of *USH2A* exon 13, producing up to 60% corrected gene expression with no off-target activity. These data supplement *in vivo* pre-clinical data presented earlier in the year supporting further development of the lead candidate to treat Usher syndrome 2A.

- **Gene Editing for Neurological Diseases**

Formed research collaboration with AskBio for AAV delivery of CRISPR medicines

Editas Medicine and Asklepios BioPharmaceutical, Inc. (AskBio) are working together to develop transformative medicines for neurological disorders by combining AskBio's adeno-associated virus (AAV) development and manufacturing expertise with Editas Medicine's leading CRISPR gene editing platform.

Engineered Cell Medicines

- **Oncology**

Amended Celgene collaboration to focus on engineered alpha-beta T cell medicines

Editas Medicine and Juno Therapeutics, Inc., a Celgene company (Celgene), have amended their collaboration, formed in 2015, to focus on developing autologous and allogeneic engineered alpha-beta T cell medicines for the treatment of cancer and autoimmune diseases. Under the amended collaboration, Editas Medicine is entitled to receive a \$70 million payment from Celgene and may be eligible for future milestone and royalty payments. Under the terms of the amended collaboration, Editas Medicine has expanded its wholly owned portfolio to treat cancer to include non-alpha-beta T cell medicines in addition to programs utilizing natural killer cells derived from both healthy donors and induced pluripotent stem cells.

- **EDIT-301 for Sickle Cell Disease and Beta-Thalassemia**

In vivo pre-clinical proof-of-concept data to be presented at ASH

Editas Medicine is developing EDIT-301 using CRISPR/Cas12a (Cpf1) as a potentially best-in-class medicine to treat sickle cell disease and beta-thalassemia. The Company will present *in vivo* pre-clinical data at the 61st American Society of Hematology Annual Meeting & Exposition (ASH) demonstrating that EDIT-301 induces predicted therapeutically relevant

levels of fetal hemoglobin with no off-target activity.

Corporate

• Leadership

In October, the Company announced the appointment of Judith R. Abrams, M.D., as Chief Medical Officer. Dr. Abrams is a leading drug development clinician who has brought multiple medicines from clinical stage development to regulatory approval. Dr. Abrams has more than 25 years of experience in leadership roles in the biopharmaceutical industry managing portfolios of products across all phases of global clinical development.

• Balance Sheet

The Company expects that its existing cash, cash equivalents and marketable securities of \$332.6 million at September 30, 2019, and anticipated interest income will enable it to fund its operating expenses and capital expenditures for at least 24 months from today. Cash, cash equivalents and marketable securities as of September 30, 2019 excludes the anticipated \$70 million payment from Celgene pursuant to the amended collaboration announced today.

Upcoming Events

Editas Medicine will participate in the following investor events:

- Piper Jaffray 31st Annual Healthcare Conference, Fireside Chat, December 3, 10:30 a.m. ET, New York City; and
- J.P. Morgan 38th Annual Healthcare Conference, January 13-16, 2020, San Francisco.

Editas Medicine will present pre-clinical data for EDIT-301 to address sickle cell disease and beta-thalassemia in at the 61st American Society of Hematology Annual Meeting & Exposition. Details are as follows:

Abstract Number: 4636

Title: *EDIT-301: An Experimental Autologous Cell Therapy Comprising Cas12a-RNP Modified mPB-CD34+ Cells for the Potential Treatment of SCD*

Presenter: Edouard De Dreuzy, Ph.D.

Session: 801. Gene Therapy and Transfer: Poster III

Time: Monday, December 9, 2019: 6:00 PM-8:00 PM

Location: Hall B, Orange County Convention Center, Orlando, FL

Third Quarter 2019 Financial Results

Cash, cash equivalents, and marketable securities at September 30, 2019, were \$332.6 million, compared to \$369.0 million at December 31, 2018. The \$36.4 million decrease was primarily attributable to operating and capital expenses related to our on-going preclinical and clinical activities, patent costs and license fees, and employee-related costs, partially offset by \$42.1 million in proceeds from financing activities.

For the three months ended September 30, 2019, net loss was \$32.9 million, or \$0.66 per share, compared to \$15.2 million, or \$0.32 per share, for the same period in 2018.

- Collaboration and other research and development revenues were \$3.8 million for the three months ended September 30, 2019, compared to \$14.5 million for the same period in 2018. The \$10.7 million decrease was primarily attributable to \$15.0 million in revenue recognized during the third quarter of 2018 related to the EDIT-101 option exercise payment pursuant to our strategic alliance with Allergan.
- Research and development expenses increased by \$5.3 million, to \$22.7 million for the three months ended September 30, 2019, from \$17.4 million for the same period in 2018. The \$5.3 million increase was primarily attributable to increased process and platform development expenses driven by increased manufacturing and clinical related costs, including costs under our profit-sharing arrangement with Allergan in the United States for EDIT-101.
- General and administrative expenses increased by \$2.4 million to \$15.7 million for the three months ended September 30, 2019, from \$13.3 million for the same period in 2018. The \$2.4 million increase was primarily attributable to increased professional service expenses.

Conference Call

The Editas Medicine management team will host a conference call and webcast today at 8:00 a.m. ET to provide and discuss a corporate update and financial results for the third quarter of 2019. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 6577216. A live webcast of the call will be available on the Investors & Media section of the Editas Medicine website at www.editasmedicine.com and a replay will be available approximately two hours after its completion.

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.

About EDIT-101 (AGN-151587)

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 (AGN-151587). 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).

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 Company's plans with respect to the Brilliance Phase 1/2 clinical trial for EDIT-101 (AGN-151587), including the Company's expectations regarding the timing of dosing a patient by early 2020. 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 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.

Editas Medicine, Inc.

Condensed Consolidated Statements of Operations (unaudited)

(amounts in thousands, except per share and share data)

	Three Months Ended September 30,	
	2019	2018
Collaboration and other research and development revenues	\$ 3,848	\$ 14,519
Operating expenses:		
Research and development	22,702	17,443
General and administrative	15,734	13,334
Total operating expenses	38,436	30,777
Operating loss	(34,588)	(16,258)
Other income, net:		
Other expense, net	(33)	(4)
Interest income, net	1,680	1,024
Total other income, net	1,647	1,020
Net loss	\$ (32,941)	\$ (15,238)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.66)	\$ (0.32)
Weighted-average common shares outstanding, basic and diluted	49,820,455	47,414,271

Editas Medicine, Inc.
Selected Condensed Consolidated Balance Sheet Items
(unaudited)
(amounts in thousands)

	September 30, 2019	December 31, 2018
Cash, cash equivalents, and marketable securities	\$ 332,616	\$ 368,955
Working capital	272,424	338,876
Total assets	372,359	420,386
Deferred revenue, net of current portion	82,379	115,614
Construction financing lease obligation, net of current portion	—	32,417
Total stockholders' equity	209,990	236,162

Investor Contact

Mark Mullikin
(617) 401-9083
mark.mullikin@editasmed.com

Media Contact

Cristi Barnett
(617) 401-0113
cristi.barnett@editasmed.com



Source: Editas Medicine, Inc.