



Editas Medicine Announces First Quarter 2019 Results and Update

May 7, 2019

- Building on focus in oncology through newly-formed partnership with BlueRock Therapeutics to develop universal allogeneic cell medicines for cancer -
- EDIT-101 (AGN-151587) for LCA10 on track for first patient dosing in second half of 2019 -
- Initiated IND-enabling activities for potentially best-in-class medicine for sickle cell disease and beta-thalassemia -
- \$342 million of cash, cash equivalents, and marketable securities as of March 31, 2019 -

CAMBRIDGE, Mass., May 07, 2019 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the first quarter of 2019.

"2019 is off to a strong start with tangible progress on multiple fronts," said Cynthia Collins, interim Chief Executive Officer of Editas Medicine. "We expanded and accelerated our focus in oncology through a newly formed collaboration with BlueRock Therapeutics. We made progress towards dosing patients in the second half with EDIT-101 for LCA10. And, we initiated IND-enabling activities for a potentially best-in-class medicine for sickle cell disease and beta-thalassemia. We look forward to maintaining the momentum through the remainder of the year and beyond."

Recent Achievements and Outlook

- **Advancing universal allogeneic cell medicines for cancer through partnership with BlueRock Therapeutics.** Editas Medicine and BlueRock Therapeutics will combine their respective CRISPR genome editing and induced pluripotent stem cell (iPSC) platforms to develop universal allogeneic cell medicines to treat cancer and other serious diseases. Gene-edited, iPSC-derived, allogeneic cell medicines represent universal, off-the-shelf treatments that may be mass produced with superior quality, greater scale, and lower cost than autologous or donor-derived therapies. The Company believes that the combination will enable the high level of multiplexed editing needed to realize the full potential of cell medicine, particularly in solid tumors where the greatest unmet need exists.
- **EDIT-101 on track for first patient dosing in second half of 2019.** Editas Medicine and Allergan Pharmaceuticals International Limited expect to enroll approximately 18 patients, aged 3 years and above, in a Phase 1/2 open label, dose escalation study to evaluate the safety, tolerability, and efficacy of EDIT-101 as a treatment for Leber congenital amaurosis 10 (LCA10). In April, initial data from the ongoing enLIGHTen natural history study of LCA10 patients were presented at the Retinal Cell and Gene Therapy Innovation Summit by Principal Investigator Eric A. Pierce, M.D., Ph.D., Director of the Ocular Genomics Institute and William F. Chatlos Professor of Ophthalmology at Massachusetts Eye and Ear and Harvard Medical School.
- **Initiated IND-enabling activities for a potentially best-in-class medicine for sickle cell disease and beta-thalassemia.** Editas Medicine is developing an experimental medicine to directly upregulate fetal hemoglobin by editing the beta-globin locus. The Company has previously presented data demonstrating potential advantages of its approach as compared to indirectly upregulating fetal hemoglobin by editing the BCL11A erythroid enhancer. Editas Medicine will present additional data next month at the 24th Congress of the European Hematology Association.
- **Progress towards an experimental medicine for Usher syndrome 2A (USH2A).** Pre-clinical *in vivo* proof-of-concept data presented at the 22nd Annual Meeting of the American Society of Gene & Cell Therapy demonstrated CRISPR/Cas9 gene editing of human *USH2A* exon 13 can rescue the retinal phenotype and restore auditory function in mice. Based on these data, the Company is optimizing a lead candidate to be ready for IND-enabling studies by the end of 2019.
- **Strong balance sheet to advance the business.** The Company held cash, cash equivalents, and marketable securities of \$342 million as of March 31, 2019, providing at least 24 months of funding for operating expenses and capital expenditures.

Upcoming Events

Editas Medicine will participate in the following investor events:

- 5th Annual SunTrust Robinson Humphrey Life Sciences Summit, May 8, New York City;
- RBC Capital Markets 2019 Global Healthcare Conference, May 21, 8:00 a.m. ET, New York City; and

- Raymond James 2019 Life Sciences and MedTech Conference, June 18-19, New York City.

Editas Medicine will participate in the following scientific and medical conferences:

- 24th Congress of the European Hematology Association, June 13-16, Amsterdam; and
- TIDES Oligonucleotide and Peptide Therapeutics Conference, May 20-23, San Diego.

First Quarter 2019 Financial Results

Cash, cash equivalents, and marketable securities at March 31, 2019, were \$342.1 million, compared to \$369.0 million at December 31, 2018.

For the three months ended March 31, 2019, net loss attributable to common stockholders was \$29.2 million, or \$0.60 per share, compared to \$30.9 million, or \$0.67 per share, for the same period in 2018.

- Collaboration and other research and development revenues were \$2.1 million for the three months ended March 31, 2019, compared to \$3.9 million for the same period in 2018. The \$1.9 million decrease was primarily attributable to a \$1.0 million decrease in revenue recognized pursuant to our collaboration agreement with Juno Therapeutics, a Celgene company that is a wholly-owned subsidiary of Celgene Corporation, and a \$0.9 million decrease in revenue recognized pursuant to our strategic alliance with Allergan Pharmaceuticals International Limited, partially offset by \$0.1 million in revenue recognized in the first quarter of 2019 pursuant to an out-license agreement.
- Research and development expenses decreased by \$5.5 million, to \$15.8 million for the three months ended March 31, 2019, from \$21.3 million for the same period in 2018. The \$5.5 million decrease was primarily attributable to \$5.8 million in decreased process and platform development expenses, mostly relating to the acquisition of certain non-capitalizable intangible assets during the first quarter of 2018, and \$0.5 million in decreased stock-based compensation expenses, partially offset by \$0.6 million in increased other expenses including facility-related expenses and \$0.3 million in increased employee related expenses.
- General and administrative expenses increased by \$3.3 million to \$17.5 million for the three months ended March 31, 2019, from \$14.2 million for the same period in 2018. The \$3.3 million increase was primarily attributable to \$1.9 million in increased stock-based compensation expenses, \$2.1 million in increased professional service expenses and \$0.7 million in increased employee related expenses, partially offset by \$1.4 million in decreased intellectual property and patent related fees.

Conference Call

The Editas Medicine management team will host a conference call and webcast today at 5:00 p.m. ET to provide and discuss a corporate update and financial results for the first quarter of 2019. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 8457858. 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 planned Phase 1/2 clinical trial for EDIT-101 (AGN-151587), including dosing patients in the second half of 2019. 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 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 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	
	March 31,	
	2019	2018
Collaboration and other research and development revenues	\$ 2,069	\$ 3,927
Operating expenses:		
Research and development	15,842	21,300
General and administrative	17,489	14,186
Total operating expenses	<u>33,331</u>	<u>35,486</u>
Operating loss	(31,262)	(31,559)
Other income, net:		
Other (expense) income, net	(44)	182
Interest income, net	2,057	438
Total other income, net	<u>2,013</u>	<u>620</u>
Net loss	<u>\$ (29,249)</u>	<u>\$ (30,939)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.60)</u>	<u>\$ (0.67)</u>
Weighted-average common shares outstanding, basic and diluted	<u>48,838,229</u>	<u>45,992,008</u>

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

	March 31,	December 31,
	2019	2018
Cash, cash equivalents, and marketable securities	\$ 342,065	\$ 368,955
Working capital	306,038	338,876
Total assets	378,916	420,386
Deferred revenue, net of current portion	105,865	115,614
Construction financing lease obligation, net of current portion	-	32,417
Total stockholders' equity	217,162	236,162

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Source: Editas Medicine, Inc.