Editas Medicine Announces First Quarter 2017 Results and Update

May 15, 2017 4:01 PM ET

Entered strategic R&D alliance with ophthalmology leader Allergan to develop and commercialize ground-breaking medicines for patients with serious eye diseases

Completed successful follow-on offering of common stock raising $104 million in gross proceeds

Presented new data at the American Society of Gene & Cell Therapy Annual Meeting demonstrating advancements in translating CRISPR technology into medicines for eye and blood diseases

CAMBRIDGE, Mass., May 15, 2017 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (NASDAQ:EDIT), a leading genome editing company, today reported financial results for the first quarter ended March 31, 2017, and provided an update on recent achievements and upcoming events.

“We made outstanding progress this quarter building our business for the long term, including forming a strategic alliance with Allergan in ocular diseases, cementing our unmatched intellectual property position with a favorable USPTO decision, and doubling our cash runway,” said Katrine Bosley, President and Chief Executive Officer of Editas Medicine. “These achievements represent significant strides forward in positioning Editas Medicine to execute our vision to build a company that pioneers a broad new category of genomic medicines.”

Recent Achievements

Building the business for the long term by assembling the capabilities to fully develop and commercialize important medicines

- **Established strategic alliance with Allergan plc**. Allergan Pharmaceuticals International Limited (Allergan), a wholly-owned subsidiary of Allergan plc, and Editas Medicine formed a strategic alliance to research, develop, and commercialize CRISPR genome editing medicines for patients suffering from serious ocular diseases. Under the terms of the agreement, Editas Medicine received an upfront payment of $90 million and granted Allergan an option to license up to five candidate programs. Allergan will be responsible for development and commercialization of any optioned products, subject to Editas Medicine's option to co-develop and co-promote up to two products in the United States, including the program for Leber Congenital Amaurosis type 10 (LCA10). Editas Medicine is also eligible to receive more than $200 million in milestone payments per optioned product, with more than half of this value for development, approval, and launch milestones, as well as high-single digit royalties on net sales of optioned products, subject to the co-development and co-promotion arrangement.

- **Updated timelines for LCA10 Investigational New Drug (IND) application submission.** The target date for filing the IND application for the LCA10 program is being moved to the middle of 2018 to accommodate certain delays in third-party manufacturing of our LCA10 product candidate and to take full advantage of our newly formed alliance with Allergan.

- **Successfully completed common stock offering** resulting in $104 million in gross proceeds including full exercise of the underwriters' option to purchase additional shares.

- **Strengthened Editas Medicine’s unmatched intellectual property position.** The U.S. Patent and Trademark Office (USPTO) issued a highly favorable decision in the patent interference between the University of California, the University of Vienna, Emmanuelle Charpentier and the Broad Institute, Inc. (Broad) regarding certain CRISPR/Cas9 patents the Company exclusively licenses from Broad. The USPTO granted Broad’s Motion for No Interference in Fact, ending the interference before the USPTO and upholding Broad's issued patents.

The European Patent Office also issued a notice of intent to grant the first Cpf1 patent, which is exclusively licensed to Editas Medicine. Cpf1 offers several potential advantages including expanding the range of genomic sites that can be edited, simplifying manufacturing and delivery, and increasing efficiency and accuracy of some forms of gene insertion or correction. The patent is expected to grant on May 31, 2017.

As the only company with rights to both Cas9 and Cpf1, the two proven CRISPR systems for human medicines, Editas Medicine believes it is in an unparalleled position to continue building a broad and differentiated product pipeline.

Advancing a pipeline strategy to enable successful product development and expand Editas Medicine's platform in the years ahead

- **Presented six posters and oral presentations at the recent American Society of Gene & Cell Therapy (ASGCT) meeting demonstrating progress in translating the promise of CRISPR technology into a broad class of genomic medicines.**

  — Demonstrated therapeutically relevant editing of photoreceptors in non-human primates in the Company's LCA10 program. In this study, two guide RNAs and *S. aureus* Cas9 expressed under the control of a photoreceptor-specific promoter were delivered in a single, subretinally-injected adeno-associated virus (AAV) at two different doses. Gene editing was shown to be dose and time dependent. For animals treated with the higher dose, the projected productive editing rate may be as high as 50% of alleles in photoreceptor cells, based on directly measured editing of 15% in total genomic DNA and an estimate for the proportion of cells represented by photoreceptors. We believe this is well above the editing rate needed to have a therapeutic effect in patients.

  — Demonstrated the induction of therapeutically relevant levels of fetal hemoglobin protein and showed that edited hematopoietic stem cells durably repopulate the bone marrow and blood *in vivo* in program to treat sickle cell disease and beta-thalassemia. Also, presented data that suggests this proprietary editing approach has the potential to be more potent than other approaches that have been previously reported.

  — Released data on new proprietary technologies to potentially enable unprecedented medicines. Showed rigorous new empirical methods for assessing specificity referred to as Uni-directional Targeted Sequencing (UDITAS™), novel compositions and methods for making covalently-coupled dual guide RNAs, the potential for self-inactivating Cas9 to control expression while maintaining efficient gene editing, and targeted insertion of DNA for efficient and specific gene correction.

- **Joined Duke-Margolis Value-Based Payment Consortium, a multi-disciplinary initiative to define and develop more feasible paths to value-based payment arrangements that support better care and outcomes for patients. One of the focuses of the initiative is in Genomic Medicine and it is led by Mark McClellan, M.D., Ph.D., Director of the Duke-Margolis Center for Health Policy.** Dr. McClellan is the former Administrator of the Centers for Medicare and Medicaid Services and former Commissioner of the Food and Drug Administration. The advisory group brings together a select team of leaders in the biopharmaceutical and medical device industries, including patient advocates, payers, manufacturers, and providers, as well as experts on regulatory affairs, law, and policy makers to address common legal and regulatory issues as well as to innovate payment models that create value for the healthcare system and for patients.

Upcoming Events

Editas Medicine will participate in the following upcoming investor conferences:

- Bank of America Merrill Lynch 2017 Health Care Conference, May 16-18, Las Vegas;

- UBS Global Healthcare Conference, May 22-24, New York City;
- JMP Securities 2017 Life Sciences Conference, June 20-21, New York City; and
- Goldman Sachs Third Annual Innovation Symposium, June 27, New York City.

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

- CRISPR 2017, June 8-10, Big Sky;
- 2017 Synthetic Biology: Engineering, Evolution & Design (SEED), June 20-23, Vancouver; and
- Cold Spring Harbor Genome Engineering, July 21-24, Cold Spring Harbor.

First Quarter 2017 Financial Results

Cash and cash equivalents at March 31, 2017, were $351.6 million, compared to $185.3 million at December 31, 2016.

For the quarter ended March 31, 2017, net loss attributable to common stockholders was $31.1 million, or $0.85 per share, compared to $17.8 million, or $0.80 per share, for the same period in 2016.

- Collaboration and other research and development revenues were $0.7 million for the quarter ended March 31, 2017, compared to $0.8 million for the same period in 2016. The $0.1 million decrease was due to a decrease in revenue recognized pursuant to our collaboration with Juno Therapeutics, Inc.

- Research and development expenses were $19.0 million for the quarter ended March 31, 2017, compared to $8.9 million for the same period in 2016. The $10.1 million increase is attributable to the issuance of $5.0 million notes payable to the Broad Institute and Wageningen University that became issuable under one of our licensing agreements during the first quarter of 2017, an increase of $2.3 million that became due to certain of our licensors in connection with receiving the Allergan upfront payment during the first quarter of 2017, a $1.4 million increase in employee related expenses, excluding stock-based compensation, a $1.1 million increase in process and platform development costs, as well as a $0.2 million increase in stock-based compensation expense.

- General and administrative expenses were $12.3 million for the quarter ended March 31, 2017, compared to $9.8 million for the same period in 2016. The $2.5 million increase is attributable to an increase of $1.4 million in stock-based compensation expense, an increase of $1.0 million in employee related expenses, an increase of $0.5 million in contractor consulting fees, and an increase of $0.3 million in other facility-related expenses, partially offset by a decrease of $0.7 million in external IP legal and patent-related fees, including expenses associated with the prosecution and maintenance of our patents and patent applications owned by us or licensed to us.

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 ended March 31, 2017. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 17355274. 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

Editas Medicine is a leading genome editing company dedicated to treating patients with genetically-defined diseases by correcting their disease-causing genes. The Company was founded by world leaders in genome editing, and its mission is to translate the promise of genome editing science into a broad class of transformative genomic medicines to benefit the greatest number of patients.

Forward-Looking Statements

This press release contains forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995, including statements regarding the Company's goals of submitting of an IND for the LCA10 program by the middle of 2018 and achieving any milestones under the Company's alliance with Allergan. 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. 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 as a result of new information, future events or otherwise.

Editas Medicine, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(unaudited)

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

	Three Months Ended March 31,	
	2017	2016
Collaboration and other research and development revenues	$ 682	$ 805
Operating expenses:		
Research and development	19,021	8,882
General and administrative	12,288	9,762
Total operating expenses	31,309	18,644
Operating loss	(30,627)	(17,839)
Other income (expense), net:		
Other income (expense), net	140	(30)
Interest income (expense), net	(610)	124
Total other income (expense), net	(470)	94
Net loss and comprehensive loss	$ (31,097)	$ (17,745)
Reconciliation of net loss to net loss attributable to common stockholders:		
Net loss	$ (31,097)	$ (17,745)
Accretion of redeemable convertible preferred stock to redemption value	—	(47)
Net loss attributable to common stockholders	$ (31,097)	$ (17,792)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.85)	$ (0.80)

Weighted-average common shares outstanding, basic and diluted	36,485,421	22,280,797

EDITAS MEDICINE, INC.
Selected Condensed Consolidated Balance Sheet Items
(amounts in thousands)
(unaudited)

	March 31, 2017	December 31, 2016
Cash and cash equivalents	$ 351,552	$ 185,323
Working capital	303,499	154,100
Total assets	395,041	229,182
Deferred revenue, net of current portion	104,033	26,000
Construction financing lease obligation, net of current portion	33,929	35,096
Total stockholders' equity	206,461	134,607

Media Contact
Cristi Barnett
Editas Medicine, Inc.
(617) 401-0113
cristi.barnett@editasmed.com

Investor Contact
Mark Mullikin
Editas Medicine, Inc.
(617) 401-9083
mark.mullikin@editasmed.com

Editas Medicine