# Editas Medicine Announces Second Quarter 2019 Results and Update

August 6, 2019

*Cynthia (Cindy) Collins named President and Chief Executive Officer*

*Initiated Brilliance Phase 1/2 clinical trial of EDIT-101(AGN-151587) for LCA10*

*Patient dosing in Brilliance trial on track for 2H19*

*EDIT-301 for hemoglobinopathies pre-clinical* in vivo *data to be presented by YE19*

*Cash, cash equivalents, and marketable securities of $317.9 million as of June 30, 2019*

CAMBRIDGE, Mass., Aug. 06, 2019 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the second quarter of 2019.

"I am pleased and honored to be appointed CEO at this exciting time for the company," said Cynthia Collins, President and Chief Executive Officer of Editas Medicine. “Our team is making history with enrollment underway and patient dosing anticipated in the first ever clinical trial of an *in vivo* CRISPR medicine. As we enter this new phase in our company's development, we are also advancing our broader pipeline of *in vivo* CRISPR medicines, including our Usher syndrome program, as well as engineered cell medicines for hemoglobinopathies and cancers.”

**Recent Achievements and Outlook**

***In Vivo* CRISPR Medicines**

- **EDIT-101 for LCA10**
  *Patient screening initiated with dosing planned for 2H19*
  Editas Medicine and its partner, Allergan, expect to enroll approximately 18 patients, aged 3 years and above, in the Brilliance Phase 1/2 clinical trial. The Brilliance clinical trial is a multi-center, open label, dose escalation study to evaluate the safety, tolerability, and efficacy of EDIT-101 as a treatment for Leber congenital amaurosis 10 (LCA10). It is the first ever clinical trial of an *in vivo* CRISPR medicine.

- **Usher Syndrome 2A**
  *Ready for IND-enabling studies by YE19*
  *In vivo* proof-of-concept data was presented at the 2019 American Society of Gene & Cell Therapy Annual Meeting. The Company plans to present *in vitro* cellular data demonstrating predicted therapeutically relevant and specific editing with its lead molecule at a 2019 medical conference.

**Engineered Cell Medicines**

- **EDIT-301 for Sickle Cell Disease and Beta-thalassemia**
  *IND-enabling activities under way for a potentially best-in-class medicine*
  Editas Medicine is developing EDIT-301 to directly upregulate fetal hemoglobin by editing the *HBG1/2* promoter in the beta-globin locus. The Company plans to present pre-clinical *in vivo* data at a 2019 medical conference demonstrating robust and durable induction of fetal hemoglobin with EDIT-301.

- **Oncology**
  The Company is advancing engineered cell medicines for cancer including engineered T cells, in collaboration with Juno Therapeutics, Inc., a Celgene company (Celgene), as well as wholly owned engineered NK cell programs. Editas Medicine is developing universal allogeneic medicines derived from induced pluripotent stem cells (iPSCs) to create off-the-shelf oncology treatments that are highly engineered for areas of unmet need, such as solid tumors. Toward that end, the Company has successfully generated iPSCs from fibroblasts and peripheral blood cells, achieved efficient CRISPR gene editing of iPSCs, and differentiated iPSCs into functional NK cells.

**Corporate**

- **Leadership**
  The Company announced today the appointment of Cynthia Collins as President and Chief Executive Officer of Editas Medicine. Ms. Collins has served as a Director of the Company since December 2018, and as interim Chief Executive Officer since January 2019. Ms. Collins has more than 30 years of experience as an executive leading and growing gene and cell medicine companies.

- **Intellectual Property**
  On June 24, 2019, the U.S. Patent and Trademark Office declared an interference (#106115) between certain CRISPR/Cas9 patent applications submitted by the University of California, the University of Vienna, and Emmanuelle Charpentier and certain patents issued to the Broad Institute, Inc. (Broad) that have been licensed to Editas Medicine. The Broad patents continue to be valid and in force. Foundational claims covering the use of CRISPR/Cas9 for gene editing in eukaryotic cells have also issued to Broad as patents in each of the United States, Europe, Japan, and other jurisdictions.

- **Manufacturing**
  Editas Medicine is building a Good Manufacturing Practice facility in Boulder, Colorado, to supply guide RNA and ribonucleoprotein in support of its EDIT-301 experimental medicine for sickle cell disease and beta-thalassemia as well as engineered cell medicines to treat cancer. The Company expects the facility to be commissioned in 2020.

- **Balance Sheet**
  The Company held cash, cash equivalents, and marketable securities of $317.9 million as of June 30, 2019, providing at least 24 months of funding for operating expenses and capital expenditures.

**Upcoming Events**

Editas Medicine plans to participate in the following investor events:

- Citi 14th Annual Biotech Conference, September 4-5, Boston;
- Morgan Stanley 17th Annual Global Healthcare Conference, Fireside Chat, September 9, 2:15 p.m. ET, New York City; and
- Chardan 3rd Annual Genetic Medicines Conference, October 7-8, New York City.

Editas Medicine plans to participate in the following scientific and medical conferences:

- Cold Spring Harbor Genome Engineering, October 10-13, Cold Spring Harbor; and
- 27th Annual Congress of the European Society of Gene & Cell Therapy, October 22-25, Barcelona.

**Second Quarter 2019 Financial Results**

Cash, cash equivalents, and marketable securities at June 30, 2019, were $317.9 million, compared to $369.0 million at December 31, 2018.

For the three months ended June 30, 2019, net loss attributable to common stockholders was $33.8 million, or $0.69 per share, compared to $38.7 million, or $0.82 per share, for the same period in 2018.

- Collaboration and other research and development revenues were $2.3 million for the three months ended June 30, 2019, compared to $7.4 million for the same period in 2018. The $5.0 million decrease was primarily attributable to a decrease in revenue recognized pursuant to our collaboration agreement with Celgene and an out-license arrangement entered into during the second quarter of 2018, partially offset by an increase in revenue recognized pursuant to our strategic alliance with Allergan.

- Research and development expenses decreased by $9.2 million, to $23.6 million for the three months ended June 30, 2019, from $32.7 million for the same period in 2018. The decrease was primarily attributable to decreased sublicensing and success payment expenses due to additional expenses incurred during the second quarter of 2018.

- General and administrative expenses increased by $0.1 million to $14.4 million for the three months ended June 30, 2019, from $14.3 million for the same period in 2018.

**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 second quarter of 2019. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 8968723. 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 dosing patients in the second half of 2019, and its expectations of completing a manufacturing facility in Colorado. 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.

**Editas Medicine, Inc.**
**Condensed Consolidated Statements of Operations**
**(unaudited)**
**(amounts in thousands, except per share and share data)**

| | Three Months Ended June 30, | |
|---|---|---|
| | **2019** | **2018** |
| Collaboration and other research and development revenues | $ 2,330 | $ 7,372 |
| Operating expenses: | | |
| Research and development | 23,565 | 32,718 |
| General and administrative | 14,414 | 14,311 |
| Total operating expenses | 37,979 | 47,029 |
| Operating loss | (35,649) | (39,657) |
| Other income, net: | | |
| Other (expense) income, net | (68) | 154 |
| Interest income, net | 1,931 | 780 |
| Total other income, net | 1,863 | 934 |
| Net loss | $ (33,786) | $ (38,723) |
| Net loss per share attributable to common stockholders, basic and diluted | $ (0.69) | $ (0.82) |
| Weighted-average common shares outstanding, basic and diluted | 49,070,574 | 46,952,059 |

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

| | June 30, 2019 | December 31, 2018 |
|---|---|---|
| Cash, cash equivalents, and marketable securities | $ 317,930 | $ 368,955 |
| Working capital | 251,373 | 338,876 |
| Total assets | 355,625 | 420,386 |
| Deferred revenue, net of current portion | 75,911 | 115,614 |
| Construction financing lease obligation, net of current portion | — | 32,417 |
| Total stockholders' equity | 193,062 | 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.