



Editas Medicine Announces Second Quarter 2020 Results and Update

August 6, 2020

Regained full control of ocular medicines under new agreement with AbbVie

BRILLIANCE trial for EDIT-101 on track to dose at least three patients by end of 2020

Plan to file IND for EDIT-301 for sickle cell disease by end of 2020

Strengthened balance sheet through equity offering raising \$216 million in gross proceeds

Cash, cash equivalents, and marketable securities of \$599 million as of June 30, 2020

CAMBRIDGE, Mass., Aug. 06, 2020 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the second quarter of 2020.

"I am extremely proud of our recent progress, as we remain on track with previously shared guidance despite the unpredictable challenges presented by COVID-19," said Cynthia Collins, Chief Executive Officer of Editas Medicine. "Our lead program evaluating EDIT-101 for the treatment of Leber congenital amaurosis 10 has continued to advance with dosing resuming in the Phase 1/2 BRILLIANCE trial, further solidifying Editas as the leader in the field of *in vivo* gene editing. Substantial progress has also been made toward an IND filing for EDIT-301 this year, with recent data presented at EHA providing preclinical proof-of-concept for this potentially best-in-class cell medicine for sickle cell disease."

Ms. Collins continued, "Alongside the clinical and scientific advances made last quarter, we also executed on several corporate milestones to better position the Company as we expand our clinical-stage pipeline. To ensure the efficient advancement of our pioneering gene editing medicines, we strengthened our balance sheet with the closing of a common stock offering resulting in gross proceeds of approximately \$216 million. Importantly, we also secured dedicated cGMP-compliant manufacturing space to facilitate the completion of IND-enabling studies and early stage clinical manufacturing for our engineered cell medicines including EDIT-301 for sickle cell disease and EDIT-201, an allogeneic NK cell medicine for solid tumors."

Recent Achievements and Outlook

In Vivo CRISPR Medicines

- **Ocular Medicines**

Regained full control of ocular medicines

Editas Medicine (Company) has terminated its 2017 agreement with Allergan, now part of AbbVie, and entered a new agreement with AbbVie that returns development and commercialization rights for ocular medicines to the Company. The Company plans to continue to advance ocular medicines including EDIT-101 for Leber congenital amaurosis 10 (LCA10).

- **EDIT-101 for LCA10**

Continuation of dosing in BRILLIANCE Phase 1/2 clinical trial

Enrollment remains active and sites have reopened for dosing following a brief pause due to COVID-19. The study, which represents the first and only clinical trial to administer an *in vivo* CRISPR medicine, remains on track to complete dosing of the adult low-dose cohort (two patients) and dose at least one patient in the adult mid-dose cohort by the end of 2020.

Engineered Cell Medicines

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

Preclinical data presented at EHA reinforces best-in-class potential

Data presented at the 25th Congress of the European Hematology Association (EHA) showed that treatment with EDIT-301, which leverages the Company's proprietary Cas12a (Cpf1) enzyme to edit directly at the HBG1/2 promoter, resulted in long-term *in vivo* editing with elevated and pan-cellular fetal hemoglobin expression. The Company remains on track to file an investigational new drug application (IND) for EDIT-301 by the end of 2020.

- **EDIT-201 to Treat Solid Tumors**

Declared candidate and initiated IND-enabling studies for allogeneic NK cell medicine

EDIT-201 is an allogeneic healthy-donor NK cell medicine for the treatment of solid tumors. Editas Medicine plans to present preclinical data on EDIT-201 at a scientific conference in the second half of 2020. The Company plans to file an IND for EDIT-201 in the second half of 2021.

Corporate

- **Offering of Common Stock**

Strengthened balance sheet with gross proceeds of approximately \$216 million

Editas Medicine closed an underwritten offering of 6,900,000 shares of its common stock at a public offering price of \$31.25 per share, before deducting underwriter discounts and commissions and estimated offering expenses. This included 900,000 shares issued upon exercise in full by the underwriter of its option to purchase additional shares. All shares in the offering were sold by Editas Medicine.

- **Leadership**

Editas Medicine has appointed Gad Berdugo as Chief Business Officer. Mr. Berdugo will lead business and corporate development, alliance management, and strategic planning for the Company. He brings more than 25 years of business and corporate development experience, most recently serving as Chief Executive Officer of EpiVax Oncology, a precision cancer immunotherapy company that he co-founded in 2017.

- **Manufacturing**

Editas Medicine established manufacturing agreements with Azzur Group and Catalent to support preclinical and clinical development of the Company's portfolio of *in vivo* CRISPR and engineered cell medicines, including EDIT-101, EDIT-201, and EDIT-301. Following the termination of the 2017 agreement with Allergan, the Company has entered into a transition services agreement to transfer certain manufacturing material and processes for EDIT-101 and EDIT-102 from Allergan to Editas Medicine.

- **Intellectual Property**

On June 24, 2019, the U.S. Patent and Trademark Office declared an interference 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. Oral arguments in the interference took place on May 18, 2020. The Broad patents remain valid and in force. Foundational claims covering the use of CRISPR/Cas9 for gene editing in eukaryotic cells have issued and continue to issue to Broad as patents in the United States, Europe, Japan, and other jurisdictions.

- **Balance Sheet**

The Company expects that its existing cash, cash equivalents and marketable securities of \$598.7 million at June 30, 2020, and anticipated interest income will enable it to fund its operating expenses and capital expenditures into 2023.

Second Quarter for 2020 Financial Results

Cash, cash equivalents, and marketable securities at June 30, 2020, were \$598.7 million, compared to \$415.0 million at March 31, 2020. The increase was largely due to the \$203.7 million in net proceeds raised from the company's recent equity offering.

For the three months ended June 30, 2020, net loss attributable to common stockholders was \$23.6 million, or \$0.43 per share, compared to \$33.8 million, or \$0.69 per share, for the same period in 2019.

- Collaboration and other research and development revenues were \$10.7 million for the three months ended June 30, 2020, compared to \$2.3 million for the same period in 2019. The \$8.4 million increase was primarily attributable to \$7.6 million in cash revenues received in connection with an out-license agreement and \$0.8 million in non-cash revenue earned under our ongoing collaborations.
- Research and development expenses increased by \$4.4 million, to \$28.0 million for the three months ended June 30, 2020, from \$23.6 million for the same period in 2019. The \$4.4 million increase was primarily attributable to fees incurred related to licensing and sublicensing activities, research personnel growth to support our programs as well as our expansion of the development organization and facilities. These increases were partially offset by a decrease in process and platform expenses and share-based compensation.
- General and administrative expenses decreased by \$0.3 million to \$14.1 million for the three months ended June 30, 2020, from \$14.4 million for the same period in 2019. The \$0.3 million decrease was primarily attributable to a decrease in the expense for professional service expenses and patent related fees which was partially offset by an increase in costs related to the hiring of key executives in the second half of 2019 and into 2020.

Upcoming Events

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

- Society for Immunotherapy of Cancer 35th Annual Meeting, November 10-15, Virtual.

Editas Medicine plans to participate in the following investor events:

- Citi's 15th Annual BioPharma Conference, September 9-10, Virtual;
- 2020 Wells Fargo Healthcare Conference, September 9-10, Virtual; and
- Morgan Stanley 18th Annual Global Healthcare Conference, September 14-18, Virtual.

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 2020. To access the call, please dial (844) 348-3801 (domestic) or (213) 358-0955 (international) and provide the passcode 9185596. A live webcast of the call will be available on the Investors 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/Cas12a (also known as Cpf1) 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

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 using the proprietary *Staphylococcus aureus* Cas9 (SaCas9) enzyme, which can be packaged in a single adeno-associated virus (AAV) to deliver the gene editing machinery to photoreceptor cells. EDIT-101 is the first *in vivo* CRISPR medicine administered to humans.

About EDIT-201

EDIT-201 is an experimental, allogeneic natural killer (NK) cell medicine under investigation for the treatment of solid tumor cancers. EDIT-201 is comprised of NK cells derived from pooled healthy donor blood and genetically modified using a highly specific and efficient CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP).

About EDIT-301

EDIT-301 is an experimental, autologous cell therapy medicine under investigation for the treatment of sickle cell disease. EDIT-301 is comprised of sickle patient CD34+ cells genetically modified using a highly specific and efficient CRISPR/Cas12a (also known as Cpf1) ribonucleoprotein (RNP) to edit the HBG1/2 promoter region in the beta-globin locus. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in fetal hemoglobin (HbF) production, which has the potential to provide a durable treatment benefit for people living with sickle cell disease.

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 timing of dosing in and updates related to the Phase 1/2 BRILLIANCE clinical trial for EDIT-101, filing an IND for EDIT-301 by the end of 2020, and filing an IND for EDIT-201 in the second half of 2021. 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. Those risks and uncertainties include, among other things, that the Company's expectations regarding the effects of COVID-19 may be incorrect, that data from the Company's development programs may not support registration or further development of its potential medicines due to safety, efficacy or other reasons, and other risks listed under "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 represent Company's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, the Company explicitly disclaims any obligation to update any forward-looking statements.

EDITAS MEDICINE, INC.

Condensed Consolidated Statements of Operations

(unaudited)

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

	Three Months Ended June 30,	
	2020	2019
Collaboration and other research and development revenues	\$ 10,749	\$ 2,330
Operating expenses:		
Research and development	28,007	23,565
General and administrative	14,081	14,414

Total operating expenses	42,088	37,979
Operating loss	(31,339)	(35,649)
Other income, net:		
Other income (expense), net	7,175	(68)
Interest income, net	592	1,931
Total other income, net	7,767	1,863
Net loss	<u>\$ (23,572)</u>	<u>\$ (33,786)</u>
Net loss per share basic and diluted	<u>\$ (0.43)</u>	<u>\$ (0.69)</u>
Weighted-average common shares outstanding, basic and diluted	<u>55,346,052</u>	<u>49,070,574</u>

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

	<u>June 30,</u> <u>2020</u>	<u>December 31,</u> <u>2019</u>
Cash, cash equivalents, and marketable securities	\$ 598,720	\$ 457,140
Working capital	541,976	403,881
Total assets	655,481	508,885
Deferred revenue, net of current portion	138,406	163,207
Total stockholders' equity	426,772	262,437

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Source: Editas Medicine, Inc.