Editas Medicine Announces First Quarter 2021 Results and Update

May 5, 2021

Strengthening Leadership by Adding Mark S. Shearman, Ph.D., as Chief Scientific Officer

Advancing BRILLIANCE trial of EDIT-101 for LCA10; clinical data expected by year-end

RUBY trial of EDIT-301 for sickle cell disease active and recruiting

Preclinical ocular data presented at ARVO supports in vivo *gene editing*

Cash, cash equivalents, and marketable securities of $723 million as of March 31, 2021

CAMBRIDGE, Mass., May 05, 2021 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the first quarter of 2021.

"Our team is making tremendous progress towards discovering, developing, and manufacturing novel genome editing medicines, including excellent forward momentum this quarter for our two clinical-stage medicines," said James C. Mullen, Chairman, President and Chief Executive Officer, Editas Medicine. "We are pleased to announce Dr. Mark Shearman will be joining Editas as Chief Scientific Officer in June 2021. Mark has an outstanding track record of drug discovery and development, and we are confident that his expertise will help us achieve our near-term milestones and continue to realize the promise of bringing transformative medicines to patients."

Mr. Mullen continued, "In ocular, we advanced the BRILLIANCE trial of EDIT-101 for LCA10 and have line of sight for initiating the next cohort. We also presented preclinical data at ARVO that further validates our *in vivo* gene editing platform in USH2A and RP4. In *ex vivo,* we advanced the RUBY trial of EDIT-301 for sickle cell disease, including activating sites for recruitment and finalizing preparations to produce study drug material for dosing patients in the trial. We look forward to maintaining the momentum through the remainder of the year and expect to share Editas' first clinical data for EDIT-101 by year-end."

Recent Achievements and Outlook

***In Vivo* Gene Edited Medicines**

- **EDIT-101 for Leber Congenital Amaurosis 10 (LCA10)**
  Initial clinical data expected by year-end
  Editas Medicine is progressing the Phase 1/2 BRILLIANCE trial for the treatment of LCA10. The Company is continuing to dose patients in the second cohort, the adult mid-dose, and results of this cohort will inform the path to initiate dosing of pediatric patients. Clinical data from the first two cohorts is expected to be presented by the end of 2021.

- **Other Ocular Programs**
  Preclinical data for USH2A and RP4 presented at ARVO
  Editas Medicine presented preclinical data at the Association for Research in Vision and Ophthalmology (ARVO) 2021 Annual Meeting for Usher Syndrome 2A (USH2A) and retinitis pigmentosa 4 (RP4). For USH2A, the Company demonstrated that gene editing restored protein expression and rescued deficits in photoreceptor morphology in human retinal organoids. For RP4, the Company demonstrated that a dual adeno-associated virus editing system, which simultaneously knocks out and replaces the rhodopsin mutation of RP4, produced clinically relevant editing levels with no off-target editing. The Company expects to declare a development candidate for its RP4 program by the end of 2021.

***Ex Vivo* Gene Edited Medicines**

- **EDIT-301 for Sickle Cell Disease**
  On track to dose first patient in RUBY trial by year-end
  Editas Medicine is developing EDIT-301 using Cas12a (Cpf1), a proprietary enzyme, as a potentially best-in-class medicine to treat sickle cell disease. The Phase 1/2 RUBY trial for the treatment of sickle cell disease is active and recruitment of patients has started. The Company remains on track to begin patient dosing in the RUBY trial by the end of 2021.

- **EDIT-301 for Beta-Thalassemia**

On track to file IND by year-end
Editas Medicine is confirming the sustained effects of EDIT-301 on cells derived from beta-thalassemia patients. The Company remains on track to file an investigational new drug application for EDIT-301 for the treatment of beta-thalassemia by the end of 2021.

Cellular Therapy

- **Edited iPSC NK (iNK) Cell Medicines to Treat Solid Tumors**
  Preclinical data presented at AACR
  Editas Medicine presented preclinical data at the American Association for Cancer Research (AACR) 2021 Annual Meeting for its iNK program to treat solid tumors. The data showed that iPSC-derived natural killer (NK) cells, edited with CRISPR/Cas12a to knockout *CISH* and *TGFβR2*, demonstrated superior cytotoxicity and enhanced metabolic function in a tumor microenvironment, as well as improved serial killing capacity of certain tumors. These data confirm the expectation of the *CISH* and *TGFβR2* double knockout as a potential superior treatment for solid tumors.

- **Alpha-Beta T Cells for Oncology**
  Bristol Myers Squibb opting into additional program
  Editas Medicine and Bristol Myers Squibb continue to advance alpha-beta T cell medicines for the treatment of solid and liquid tumors. Bristol Myers Squibb recently opted into an additional gene editing program, further validating the Company's technology and expertise in engineered cell medicines.

Corporate

- **Leadership**
  Today, the Company announced Mark S. Shearman, Ph.D., will be joining Editas as Chief Scientific Officer, in June 2021. Dr. Shearman has more than 30 years of experience in drug discovery and development across multiple therapeutic modalities, and his expertise in neurology, ophthalmology, and immunology will strengthen the Company's existing pipeline. Dr. Shearman will join Editas Medicine from Applied Genetic Technologies Corporation (AGTC), where he is currently serving as Chief Scientific Officer and is responsible for leading the company's product candidate selection process, pre-clinical and translational research, and long-term research and development planning. Prior to AGTC, Dr. Shearman served as Senior Vice President of Research & Early Development at EMD Serono, Inc., the U.S. and Canadian subsidiary of Merck KGaA. Previously, Dr. Shearman was Executive Director of Merck & Co. Research Laboratories in Boston, and Senior Director at the Merck Sharp & Dohme Research Laboratories Neuroscience Research Centre in the United Kingdom. Dr. Shearman earned a B.Sc. from the University of Bristol, U.K., and a Ph.D. from the University of Nottingham, U.K. He also conducted academic research at institutes in Japan and Germany.

- **Manufacturing**
  Editas Medicine continues to advance internal and external manufacturing capabilities for the Company's portfolio of *in vivo* gene edited medicines, *ex vivo* gene edited cell medicines, and cell therapy medicines. The Company is finalizing the preparation of internal manufacturing capabilities for EDIT-301. This capability will provide early phase product for the RUBY trial. Concurrently, the Company has initiated the technology transfer process of EDIT-301 to its strategic CDMO partner Catalent for the later stages of the RUBY trial.

- **Offering of Common Stock**
  Strengthened balance sheet with net proceeds of approximately $250 million
  Editas Medicine closed an underwritten offering of 4,025,000 shares of its common stock at a public offering price of $66.00 per share, before deducting underwriter discounts and commissions and estimated offering expenses. This included 525,000 shares issued upon exercise in full by the underwriters of their option to purchase additional shares. All shares in the offering were sold by Editas Medicine.

- **Balance Sheet**
  The Company expects that its existing cash, cash equivalents and marketable securities of $723.2 million as of March 31, 2021, and anticipated interest income will enable it to fund its operating expenses and capital expenditures well into 2023.

First Quarter for 2021 Financial Results

Cash, cash equivalents, and marketable securities as of March 31, 2021, were $723.2 million, compared to $511.8 million as of December 31, 2020.

For the three months ended March 31, 2021, net loss attributable to common stockholders was $56.7 million, or $0.86 per share, compared to $37.7 million, or $0.69 per share, for the same period in 2020.

- Collaboration and other research and development revenues were $6.5 million for the three months ended March 31, 2021, compared to $5.7 million for the same period in 2020. The majority of the revenue recognized this quarter was attributable to the Company's strategic alliance with Bristol Myers Squibb.
- Research and development expenses increased by $7.3 million, to $41.9 million for the three months ended March 31, 2021, from $34.6 million for the same period in 2020. The $7.3 million increase was primarily attributable to sublicense and license fees primarily related to success payments under certain of our license agreements, as well as increased manufacturing and clinical related costs, during the first quarter of 2021.
- General and administrative expenses increased by $3.6 million to $21.4 million for the three months ended March 31, 2021, from $17.8 million for the same period in 2020. The $3.6 million increase was primarily attributable to increased employee-related expenses.

Upcoming Events

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

- 24th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT), May 11-14, Virtual
- 2021 World Medical Innovation Forum, May 20, Virtual
- European Hematology Association (EHA) 2021 Virtual Congress, June 11, Virtual

Editas Medicine plans to participate in the following investor event:

- Raymond James Human Health Innovation Conference, June 22, Virtual

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 first quarter 2021. To access the call, please dial 844-348-3801 (domestic) or 213-358-0955 (international) and provide the passcode 7587147. 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/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 to reach and deliver the gene editing machinery directly to photoreceptor cells.

About BRILLIANCE
The BRILLIANCE Phase 1/2 clinical trial of EDIT-101 for the treatment of Leber congenital amaurosis 10 (LCA10) is designed to assess the safety, tolerability, and efficacy of EDIT-101 in up to 18 patients with this disorder. Clinical trial sites are enrolling up to five cohorts testing up to three dose levels in this open label, multi-center study. Both adult and pediatric patients (3 – 17 years old) with a range of baseline visual acuity assessments are eligible for enrollment. Patients receive a single administration of EDIT-101 via subretinal injection in one eye. Additional details are available on www.clinicaltrials.gov (NCT#03872479).

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) that targets the HBG1 and HBG2 promoters in the beta-globin locus where naturally occurring fetal hemoglobin (HbF) inducing mutations reside. Red blood cells derived from EDIT-301 CD34+ cells demonstrate a sustained increase in HbF production, which has the potential to provide a durable treatment benefit for people living with sickle cell disease.

About RUBY
The RUBY Trial is a single-arm, open-label, multi-center Phase 1/2 study designed to assess the safety and efficacy of EDIT-301 in patients with severe sickle cell disease. Enrolled patients will receive a single administration of EDIT-301. Additional details are available on www.clinicaltrials.gov (NCT#04853576).

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 initiation, timing, progress and results of the Company's preclinical and clinical studies and its research and development programs, including beginning patient dosing in the RUBY trial by the end of 2021, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including presenting data from the first two cohorts of the BRILLIANCE trial by the end of 2021, and the timing or likelihood of regulatory filings and approvals, including filing an IND for EDIT-301 for the treatment of beta-thalassemia by the end 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. 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 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 March 31,	
	2021	**2020**
Collaboration and other research and development revenues	$ 6,499	$ 5,723
Operating expenses:		
Research and development	41,937	34,570
General and administrative	21,445	17,769
Total operating expenses	63,382	52,339
Operating loss	(56,883)	(46,616)
Other income, net:		
Other income (expense), net	21	7,333
Interest income, net	134	1,559
Total other income, net	155	8,892
Net loss	$ (56,728)	$ (37,724)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.86)	$ (0.69)
Weighted-average common shares outstanding, basic and diluted	65,992,395	54,590,194

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

	March 31, 2021	**December 31, 2020**
Cash, cash equivalents, and marketable securities	$ 723,223	$ 511,774
Working capital	568,305	358,894
Total assets	780,294	572,602
Deferred revenue, net of current portion	56,667	73,984
Total stockholders' equity	637,996	393,586

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

Investors
Ron Moldaver

(617) 401-9052
ir@editasmed.com

Source: Editas Medicine, Inc.