Editas Medicine Announces Second Quarter 2022 Results and Business Updates

August 3, 2022

Appointed Baisong Mei, M.D., Ph.D., Chief Medical Officer, strengthening senior leadership team

Achieved successful engraftment of first patient treated with EDIT-301 for sickle cell disease; first clinical use of Editas-engineered AsCas12a enzyme

FDA removed partial clinical hold for RUBY trial of EDIT-301

Continued screening and enrolling new adult and pediatric patients for EDIT-101 Phase 1/2 BRILLIANCE study for LCA10, with clinical data update expected 2H 2022

On track to initiate IND-enabling studies of EDIT-103 for RHO-adRP by year-end; pre-IND FDA meeting feedback supports continued development

Entered research collaboration and licensing agreement with Immatics to develop cancer medicines, combining Immatics gamma-delta T cell adoptive cell therapies and Editas Medicine's gene editing technology

CAMBRIDGE, Mass., Aug. 03, 2022 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today reported business highlights and financial results for the second quarter of 2022.

"Since joining Editas Medicine two months ago, my review of the innovative technologies, strong CMC capabilities, and talented team has reinforced my enthusiasm about the company's potential. I see tremendous value in our technology and ability to develop novel medicines, and I intend to focus our efforts on transforming the business from a technology platform company into a therapeutics company," said Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine. "In addition, I'm excited to welcome our new Chief Medical Officer, Dr. Baisong Mei, to the leadership team, where he will utilize his deep experience in developing therapeutics from pre-clinical stages through global approvals to assist in this transformation."

Recent Achievements and Outlook

***Ex Vivo* Gene Edited Medicines**

- **EDIT-301 for Sickle Cell Disease**
  First SCD patient treated with EDIT-301 successfully engrafted and demonstrated positive initial safety profile

  - Editas Medicine dosed the first patient and confirmed successful neutrophil and platelet engraftment in the Phase 1/2 RUBY trial for the treatment of severe sickle cell disease (SCD).
  - This is the first time that the Company's engineered AsCas12a enzyme has been used to edit human cells in a clinical trial.
  - The Company is enrolling additional study participants, has successfully edited $CD34^+$ cells from patients in preparation for reinfusion, and remains on track to announce top-line clinical data by year-end.

  FDA removed partial clinical hold for RUBY trial of EDIT-301 for SCD

  - The Company also announced that in July the U.S. Food and Drug Administration (FDA) removed the previously disclosed partial clinical hold for EDIT-301 RUBY study for the treatment of SCD. This enables Editas Medicine to include patients' efficacy data in a marketing application for EDIT-301 in the future.

- **EDIT-301 for TDT**
  Received FDA Orphan Drug Designation; first patient dosing remains on track for 2022

  - In May, the FDA granted Orphan Drug Designation to EDIT-301 for the treatment of transfusion-dependent beta thalassemia (TDT).
  - Preparations to initiate the Phase 1/2 clinical trial designed to assess the safety, tolerability, and preliminary efficacy of EDIT-301 for the treatment of TDT are underway, and the Company remains on track to dose the first TDT patient in 2022.

***In Vivo* Gene Edited Medicines**

- **EDIT-101 for LCA10**
  Additional pediatric and adult patients dosed in BRILLIANCE trial; continued screening and enrollment of pediatric and adult patients; on track for clinical update in second half of 2022

  - Editas Medicine has completed dosing of the second patient in the pediatric mid-dose cohort and continues to screen and enroll new pediatric patients in the ongoing EDIT-101 BRILLIANCE trial for Leber Congenital Amaurosis 10 (LCA10), a

CEP290-related retinal degenerative disorder.
- The Company also continues to dose new patients in the study's expanded adult cohorts.
- Editas Medicine remains on track to provide a clinical update on the BRILLIANCE trial in the second half of 2022.

- **EDIT-103 for RHO-adRP**
  On track to initiate IND-enabling studies by year-end; pre-IND FDA meeting feedback supports continued development

- Editas Medicine remains on track to initiate IND-enabling studies for EDIT-103 by year-end. Following feedback from a pre-IND meeting with the FDA, the Company is continuing development of EDIT-103, which utilizes a unique *in vivo* knockout and replace mechanism, for the treatment of rhodopsin-associated autosomal dominant retinitis pigmentosa (RHO-adRP), a progressive form of retinal degeneration.
- In May, Editas Medicine presented preclinical data at the Association for Research in Vison and Ophthalmology (ARVO) Annual Meeting, demonstrating nearly 100% gene editing knockout of the endogenous RHO gene and the replacement RHO gene produced over 30% of normal RHO protein levels in the treated area of subretinal injection in non-human primates (NHPs) treated with EDIT-103. Furthermore, EDIT-103-injected eyes of NHPs showed restoration of RHO expression in the outer segments and retention of normal photoreceptor structure and function compared to the knockout-only-injected eye.

Cellular Therapy

- **Gamma-Delta T Cells for Oncology**
  Collaboration with Immatics to combine gamma-delta T cell adoptive cell therapies and gene editing to develop medicines for the treatment of cancer

- In June, 2022, Editas Medicine and Immatics N.V. announced a strategic research collaboration and licensing agreement to combine gamma-delta T cell adoptive cell therapies and gene editing to develop medicines for the treatment of cancer.
- As part of the licensing agreement, Immatics gains non-exclusive rights to Editas Medicine's CRISPR technology and intellectual property. By combining Editas Medicine's gene editing technology with Immatics' ACTallo® allogeneic, off-the-shelf adoptive cell therapy platform based on gamma-delta T cells, gamma-delta T cells may be redirected to cancer cell targets with the goal of creating cells with enhanced tumor recognition and destruction.
- Editas Medicine received an undisclosed upfront cash payment and is eligible to receive additional payments based on development, regulatory, and commercial milestones. In addition, Immatics will pay royalties on future net sales on any products that may result from this collaboration.

- **Alpha-Beta T Cells for Oncology**
  Bristol Myers Squibb opted into eighth genome editing program for alpha-beta T cell program

- Bristol Myers Squibb recently opted into an additional gene editing program, marking the eighth program opted into by Bristol Myers Squibb since the start of the collaboration, one of which has advanced to development candidate status.
- The ongoing collaboration between Editas Medicine and Bristol Myers Squibb continues to advance alpha-beta T cell investigational medicines for the treatment of solid and liquid tumors, leveraging Editas Medicine's unique platform technologies, including SpCas9 and AsCas12a.

Corporate Updates

- **Senior Leadership Strengthened with CMO Appointment**
  On July 18, 2022, the Company announced the appointment of Baisong Mei, M.D., Ph.D., as Chief Medical Officer. During his career, Dr. Mei has demonstrated a strong track record in bringing novel medicines through clinical development and approval. Dr. Mei joined Editas Medicine from Sanofi, where he served as Senior Global Project Head in Rare Disease and Rare Blood Disorders.

Second Quarter 2022 Financial Results

- Cash, cash equivalents, and marketable securities as of June 30, 2022, were $527.6 million, compared to $566.4 million as of March 31, 2022. The Company expects that its existing cash, cash equivalents and marketable securities will enable it to fund its operating expenses and capital expenditures into 2024.

- For the three months ended June 30, 2022, net loss attributable to common stockholders was $53.5 million, or $0.78 per share, compared to net loss of $55.3 million, or $0.81 per share, for the same period in 2021.

- Collaboration and other research and development revenues were $6.4 million for the three months ended June 30, 2022, compared to $0.4 million for the same period in 2021. The increase was primarily attributable to the additional program licensed by Bristol Myers Squibb in the second quarter of 2022.

- Research and development expenses increased by $9.9 million to $43.7 million for the three months ended June 30, 2022, from $33.8 million for the same period in 2021. The increase was primarily related to increased manufacturing and clinical-related expenses related to the Company's ongoing clinical trials, license obligations related to a clinical milestone achievement, employee-related expenses for workforce expansion, and increased expenses due to sublicense fees.

- General and administrative expenses decreased by $5.1 million to $16.9 million for the three months ended June 30, 2022, from $22.0 million for the same period in 2021. The decrease was primarily attributable to the performance awards granted in 2021 to our former Chief Executive Officer that were achieved or deemed probable in the second quarter of 2021, for which there was no similar expense during the three months ended June 30, 2022.

Upcoming Events

Editas Medicine plans to participate in the following investor events:

- Wells Fargo Healthcare Conference, September 7, Boston, MA
- Morgan Stanley Global Healthcare Conference, September 12, New York, NY
- Baird Global Healthcare Conference, September 13, New York, NY

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 second quarter of 2022. To access the call, please dial 877-407-0989 (domestic) or 201-389-0921 (international) and ask for the Editas Medicine earnings call. A live webcast of the call will also 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 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. Editas Medicine is the exclusive licensee of Harvard and Broad Institute's Cas9 patent estates and Broad Institute's Cas12a patent estate for human medicines. For the latest information and scientific presentations, please visit www.editasmedicine.com.

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 dosing the first TDT patient with EDIT-301 in 2022 and initiating IND-enabling studies of EDIT-103 for RHO-adRP by year-end 2022, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including top-line clinical data from the RUBY trial by year-end 2022 and a clinical update on the BRILLIANCE trial in the second half of 2022, the timing or likelihood of regulatory filings and approvals, and the Company's expectations regarding cash runway. 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, including the BRILLIANCE and RUBY 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, as updated by the Company's subsequent filings 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.
Consolidated Statement of Operations
(amounts in thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Collaboration and other research and development revenues	$ 6,362	$ 379	$ 13,134	$ 6,878
Operating expenses:				
Research and development	43,659	33,753	81,635	75,690
General and administrative	16,937	22,027	36,483	43,471
Total operating expenses	60,596	55,780	118,118	119,161
Operating loss	(54,234)	(55,401)	(104,984)	(112,283)

Other income, net:				
Other income(expense), net	235	(1)	1	19
Interest income, net	546	146	1,015	280
Total other income, net	781	145	1,016	299
Net loss	$ (53,453)	$ (55,256)	$ (103,968)	$ (111,984)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.78)	$ (0.81)	$ (1.52)	$ (1.67)
Weighted-average common shares outstanding, basic and diluted	68,640,858	67,877,126	68,563,348	66,939,967

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

	June 30, 2022	December 31, 2021
Cash, cash equivalents, and marketable securities	$ 527,620	$ 619,916
Working capital	424,956	460,426
Total assets	580,833	677,483
Deferred revenue, net of current portion	68,888	60,888
Total stockholders' equity	465,414	553,642

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Source: Editas Medicine, Inc.