Editas Medicine Announces First Quarter 2024 Results and Business Updates

May 8, 2024

Completed adult cohort enrollment and enrolled multiple patients in the adolescent cohort of the Phase 1/2/3 RUBY clinical trial of reni-cel for severe sickle cell disease

On track to present additional clinical data from the RUBY trial and the EdiTHAL trial of reni-cel for transfusion-dependent beta-thalassemia in mid-2024 and additional updates by year-end 2024

Presenting pre-clinical data at ASGCT on in vivo *capabilities to support development of transformative* in vivo *gene editing medicines*

Strong financial position with operational runway into 2026

CAMBRIDGE, Mass., May 08, 2024 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage gene editing company, today reported financial results for the first quarter 2024 and provided business updates.

"We made significant progress in all three pillars of our strategy this quarter, including reni-cel, *in vivo,* and business development, including intellectual property," commented Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine. "We entered 2024 with great momentum, and I am proud of the Editas team's significant progress toward becoming a commercial-stage company and on developing clinically differentiated, transformational medicines for people living with serious, previously untreatable diseases."

Recent Achievements and Outlook

***Ex Vivo* Hemoglobinopathies**

- **Reni-cel (renizgamglogene autogedtemcel, previously EDIT-301) for Severe Sickle Cell Disease (SCD)**
  - The Company has completed enrollment of the adult cohort of the Phase 1/2/3 RUBY trial for SCD and continues to dose patients.
  - The Company has enrolled multiple patients in the adolescent cohort in the RUBY trial.
  - The Company remains on-track to present a substantive clinical data set of sickle cell patients with considerable clinical follow-up in the RUBY trial in mid-2024 and further data by year-end 2024.

- **Reni-cel for Transfusion-dependent Beta Thalassemia (TDT)**
  - The Company continues to enroll and dose patients in the EdiTHAL trial for TDT.
  - The Company remains on-track to present additional clinical data from the EdiTHAL trial in mid-2024 and further data by year-end 2024.

***In Vivo* Medicines**

- The Company is presenting pre-clinical data later this week to support its development of transformative *in vivo* gene editing medicines at the American Society of Gene and Cell Therapy (ASGCT) 27th Annual Meeting on May 9 and 10. Presentations include:
  - Oral presentation of *in vivo* pre-clinical data from mouse studies using lipid nanoparticle (LNP)-mediated delivery of an optimized guide RNA (gRNA) and engineered AsCas12a messenger RNA (mRNA).
  - Pre-clinical data demonstrating AsCas12a gRNA modifications that enable high-potency gene editing in multiple cell types, including in the liver, and improve gene editing outcomes *in vivo,* enabling the development of *in vivo* gene editing medicines.
  - Research on identifying potent large serine recombinases (LSRs) as a foundation to develop novel *in vivo* gene editing technologies for whole gene knock-in, expanding potential *in vivo* gene editing targets for developing medicines.
- The Company is on track to establish *in vivo* preclinical proof-of-concept for an undisclosed indication by year-end.

Other Corporate Highlights

Business Development

- In March 2024, Editas Medicine and Bristol Myers Squibb (NYSE: BMY) signed a two-year extension to the collaboration under which the parties may research, develop, and commercialize autologous and allogeneic alpha-beta T cell medicines for the treatment of cancer and autoimmune diseases. The extension also has options to further extend the collaboration for up to an additional two years.

Intellectual Property

- *Oral Arguments held in CRISPR patent interference*
  On May 7, 2024, oral arguments were held before the U.S. Court of Appeals for the Federal Circuit (CAFC) regarding an appeal of the Patent Trial and Appeal Board's (PTAB's) previous decision favoring Broad Institute (Broad) in the U.S. patent interference involving specific patents for CRISPR/Cas9 editing in human cells between the University of California, the University of Vienna, and Emmanuelle Charpentier (collectively, CVC) and Broad. A decision on the case is expected in the second half of 2024.

Leadership

- *Gregory Whitehead named Executive Vice President and Chief Technical and Quality Officer*
  Mr. Whitehead joins the executive leadership team as Chief Technical and Quality Officer (CTQO), responsible for leading the Technical Development, Technical Operations, and Quality departments as the Company advances its gene editing medicines towards biologics license applications (BLAs). Mr. Whitehead has more than 25 years of experience in the biotech industry and extensive cell and gene therapy clinical and commercial development expertise. Prior to his current role, Mr. Whitehead served as Senior Vice President of Quality at Editas Medicine. Before joining Editas Medicine, Mr. Whitehead served as Chief Quality Officer at Rubius Therapeutics, establishing quality capabilities to support first-in-human clinical trials for genetically engineered red blood cells. Earlier in his career, he served as Vice President, Quality at bluebird bio. During his tenure at bluebird bio, Mr. Whitehead scaled the organization in support of multiple gene therapy clinical trials and commercial launch in the U.S. and Europe.

Publications

- *The New England Journal of Medicine* published the findings from the Phase 1/2 BRILLIANCE trial of EDIT-101, on May 6.
  - The BRILLIANCE trial was the first *in vivo* CRISPR gene editing study in humans, and the study demonstrated clinical proof of concept in treatment of a rare inherited blindness, Leber Congenital Amaurosis 10, with a favorable safety profile, and preliminary efficacy signals for patients.

First Quarter 2024 Financial Results

Cash, cash equivalents, and marketable securities as of March 31, 2024, were $376.8 million compared to $427.1 million as of December 31, 2023. The Company expects the existing cash, cash equivalents, and marketable securities together with the near-term annual license fees and the contingent upfront payment payable under its license agreement with Vertex Pharmaceuticals, Incorporated, to fund operating expenses and capital expenditures into 2026.

First Quarter 2024

- For the three months ended March 31, 2024, net loss attributable to common stockholders was $62.0 million, or $0.76 per share, compared to net loss of $49.0 million, or $0.71 per share, for the same period in 2023.

- Collaboration and other research and development revenues decreased to $1.1 million for the three months ended March 31, 2024, compared to $9.9 million for the same period in 2023. The decrease relates to the one-time sale in January 2023 of the Company's wholly owned oncology assets and related licenses.

- Research and development expenses increased by $11.0 million to $48.8 million for the three months ended March 31, 2024, compared to $37.8 million for the same period in 2023. The increase is primarily attributable to sublicense and license payments as well as clinical and manufacturing costs related to the continued progression of the Company's reni-cel program.

- General and administrative expenses decreased by $3.7 million to $19.3 million for the three months ended March 31, 2024, compared to $23.0 million for the same period in 2023. The decrease is primarily attributable to one-time professional service expenses related to 2023 strategic initiatives and business development activities as well as reduced legal and patent costs.

Upcoming Events

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

- American Society of Gene and Cell Therapy (ASGCT) 27th Annual Meeting
  May 7-11, 2024
  Baltimore, MD

Editas Medicine plans to participate in the following investor events:

- Bank of America Health Care Conference 2024
  May 14, 2024
  Las Vegas, NV

- 2024 RBC Capital Markets Global Healthcare Conference
  May 15, 2024
  New York, NY

- Stifel's 2nd Annual Genetic Medicines Forum
  May 28, 2024
  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 of 2024. To access the call, please dial 1-877-407-0989 (domestic) or 1-201-389-0921 (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 clinical-stage gene editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas12a and CRISPR/Cas9 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 Broad Institute's Cas12a patent estate and Broad Institute and Harvard University's Cas9 patent estates 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 establishing *in vivo* preclinical proof-of-concept for an undisclosed indication by year-end 2024, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including presenting additional clinical data from the RUBY and EdiTHAL trials in mid-2024 and additional updates by year-end 2024, potential of, and expectations for, the Company's product candidates, the timing or likelihood of regulatory filings and approvals, the Company's expectations regarding commercial readiness, 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 important factors, including: uncertainties inherent in the initiation and completion of pre-clinical studies and clinical trials, including the RUBY and EdiTHAL trials, and clinical development of the Company's product candidates, including reni-cel; 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 the 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 March 31,	
	2024	**2023**
Collaboration and other research and development revenues	$ 1,135	$ 9,851
Operating expenses:		
Research and development	48,787	37,804
General and administrative	19,339	23,008
Total operating expenses	68,126	60,812
Operating loss	(66,991)	(50,961)
Other income, net:		
Other income (expense), net	6	(1,584)
Interest income, net	5,035	3,509
Total other income, net	5,041	1,925
Net loss	$ (61,950)	$ (49,036)
Net loss per share, basic and diluted	(0.76)	(0.71)
Weighted-average common shares outstanding, basic and diluted	81,938,839	68,924,180

EDITAS MEDICINE, INC.

Selected Consolidated Balance Sheet Items
(amounts in thousands)
(Unaudited)

	March 31, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 376,776	$ 427,135
Working capital	239,068	277,612
Total assets	440,347	499,153
Deferred revenue, net of current portion	54,204	60,667
Total stockholders' equity	294,400	349,097

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