Editas Medicine Announces Third Quarter 2024 Results and Business Updates

November 4, 2024

Achieved in vivo *preclinical proof of concept of HBG1/2 editing in hematopoietic stem and progenitor cells (HSPCs) using Editas Medicine's proprietary targeted LNP in a key step to developing a novel* in vivo *treatment for sickle cell disease and beta thalassemia*

On track to share additional clinical and patient reported outcomes data from 28 patients in the RUBY trial for sickle cell disease at the American Society of Hematology (ASH) Annual Meeting and Exposition in December

Company to provide an update on its in vivo *progress and pipeline development in 1Q 2025*

Company expects the existing cash, cash equivalents, and marketable securities, together with the upfront cash payment from DRI and the retained portions of the payments payable under the license agreement with Vertex, to fund operating expenses and capital expenditures into the second quarter of 2026

CAMBRIDGE, Mass., Nov. 04, 2024 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a clinical-stage gene editing company, today reported financial results for the third quarter 2024 and provided business updates.

"Achieving preclinical proof of concept of HBG1/2 editing in HSPCs using our proprietary targeted LNP, puts us on a clear path to develop a potentially first- and best-in-class *in vivo* gene edited medicine for the treatment of sickle cell disease and beta thalassemia," commented Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine. "As we continue our process to partner or out-license reni-cel, we also look forward to sharing a substantial clinical update from the RUBY trial of reni-cel, a potentially best-in-class cell therapy for the treatment of sickle cell disease, at ASH in December."

"I am proud of the Editas team's work and our advancement in 2024 as we move closer to achieving our vision of becoming a leader in *in vivo* programmable gene editing medicine," added Dr. O'Neill.

Recent Achievements and Outlook

***Ex Vivo* Hemoglobinopathies**

- **Reni-cel (renizgamglogene autogedtemcel, previously EDIT-301) Partnering/Out-licensing**
  - The Company has engaged Moelis & Company LLC, a leading global independent investment bank, to lead the global process to partner or out-license reni-cel.

- **Reni-cel for Severe Sickle Cell Disease (SCD)**
  - The Company will present a substantive RUBY clinical trial update of 28 patients with considerable clinical follow-up at the American Society of Hematology (ASH) Annual Meeting and Exposition, December 7-10, 2024.
    - Dataset will include safety data and efficacy data, including hematology parameters, vaso-occlusive events (VOEs), and patient reported outcomes (PROs).
  - The Company continues to dose adult patients in the RUBY trial and has dosed 28 patients to date.
  - The Company continues to manufacture drug product for the initial adolescent cohort patients and schedule dosing.
  - As previously announced, the Company has completed enrollment of the adult and adolescent cohorts of the Phase 1/2/3 RUBY trial for SCD.

- **Reni-cel for Transfusion-dependent Beta Thalassemia (TDT)**
  - The Company is on-track to present additional clinical data from the EdiTHAL trial by year-end 2024.
  - As previously announced, the Company completed enrollment of the adult cohort of the EdiTHAL trial for TDT and continues to dose patients.

***In Vivo* Medicines**

- ***In vivo* Preclinical Proof of Concept Achieved: Hematopoietic Stem and Progenitor Cell (HSPC) Editing**
  - As disclosed on October 22, the Company established *in vivo* preclinical proof of concept for the development of an *in vivo* medicine for sickle cell disease and beta thalassemia by demonstrating *in vivo* hematopoietic stem and progenitor cell (HSPC) editing of HBG1/2 promoter utilizing a novel, Editas-proprietary targeted lipid nanoparticle (tLNP) for extrahepatic tissue delivery in a humanized mouse model (mice engrafted with human hematopoietic stem cells).
  - The Company will provide an update on its *in vivo* progress and pipeline development in 1Q 2025.
  - The Company continues to pursue an *in vivo* strategy and approach aimed at driving functional upregulation of gene expression to address loss of function or deleterious mutations.

- **Additional *in vivo* Targets**
  - Editas Medicine and Genevant Sciences entered into a collaboration and nonexclusive license agreement to combine Editas' CRISPR Cas12a genome editing systems with Genevant's proprietary LNP technology in the development of *in vivo* gene editing medicines directed to functional upregulation of two liver targets.

Business Development

- On October 3, 2024, Editas Medicine announced the sale of certain future license fees and other payments owed to the Company under its Cas9 license agreement with Vertex Pharmaceuticals to a wholly owned subsidiary of DRI Healthcare Trust (DRI) for an upfront cash payment of $57 million. The upfront cash payment brings non-dilutive capital to Editas Medicine, helping enable further pipeline development and related strategic priorities.
- The Company ended the third quarter 2024 with approximately $265.1 million of cash, cash equivalents, and marketable securities, or approximately $322.1 million following receipt of the upfront cash payment from DRI.

Third Quarter 2024 Financial Results
Cash, cash equivalents, and marketable securities as of September 30, 2024, were $265.1 million compared to $318.3 million as of June 30, 2024. The Company expects the existing cash, cash equivalents, and marketable securities, together with the upfront cash payment from DRI and the retained portions of the payments payable under the license agreement with Vertex, to fund operating expenses and capital expenditures into the second quarter of 2026.

Third Quarter 2024

- For the three months ended September 30, 2024, net loss attributable to common stockholders was $62.1 million, or $0.75 per share, compared to net loss of $45.0 million, or $0.55 per share, for the same period in 2023.

- Collaboration and other research and development revenues decreased to $0.1 million for the three months ended September 30, 2024, compared to $5.3 million for the same period in 2023. The decrease is primarily attributable to the receipt in the 2023 period of the upfront payment for the non-exclusive license to Vor Bio.

- Research and development expenses increased by $7.1 million to $47.6 million for the three months ended September 30, 2024, compared to $40.5 million for the same period in 2023. The increase is primarily related to clinical and manufacturing costs related to the accelerated progression of the Company's reni-cel program as well as costs attributable to *in vivo* research and discovery.

- General and administrative expenses increased by $3.1 million to $18.1 million for the three months ended September 30, 2024, compared to $15.0 million for the same period in 2023. The increase is primarily attributable to increased employee-related expenses related to increased headcount to support business operations due to the progression of reni-cel program.

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

- American Society of Hematology (ASH) Annual Meeting and Exposition
  December 7-10, 2024
  San Diego, CA

Editas Medicine plans to participate in the following investor events:

- Guggenheim's Inaugural Healthcare Innovation Conference
  November 12, 2024
  Boston, MA

- Stifel 2024 Healthcare Conference
  November 19, 2024
  New York, NY

- 7th Annual Evercore ISI HealthCONx Conference
  December 3, 2024
  Coral Gables, FL

No 3Q Conference Call
The Company is not hosting a conference call this quarter given it recently held a Strategic Update Webinar on October 22, 2024. A replay of the webinar is available in the Investors section of the Editas Medicine website at https://ir.editasmedicine.com/events-and-presentations.

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 Company's intent to partner or out-license reni-cel and any benefits resulting therefrom, the initiation, timing, progress and results of the Company's preclinical and clinical studies and its research and development programs, the timing for the Company's receipt and presentation of data from its clinical trials and preclinical studies, including providing an update on its *in vivo* progress and pipeline development in the first quarter of 2025 and presenting additional clinical data from the RUBY trial at the ASH Annual Meeting and Exposition and from the EdiTHAL trial by year-end 2024, the potential of, and expectations for, the Company's product candidates, including any *in vivo* gene edited medicines the Company may develop, 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 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.

This press release contains hyperlinks to information that is not deemed to be incorporated by reference in this press release.

EDITAS MEDICINE, INC.
Consolidated Statement of Operations
(amounts in thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Collaboration and other research and development revenues	$ 61	$ 5,336	$ 1,710	$ 18,074
Operating expenses:				
Research and development	47,639	40,512	150,636	108,095
General and administrative	18,088	14,987	55,633	55,198
Total operating expenses	65,727	55,499	206,269	163,293
Operating loss	(65,666)	(50,163)	(204,559)	(145,219)
Other income, net:				
Other income (expense), net	(5)	—	—	(1,590)
Interest income, net	3,530	5,144	12,861	12,464
Total other income, net	3,525	5,144	12,861	10,874
Net loss	$ (62,141)	$ (45,019)	$ (191,698)	$ (134,345)
Net loss per share, basic and diluted	(0.75)	(0.55)	(2.33)	(1.81)
Weighted-average common shares outstanding, basic and diluted	82,485,199	81,648,250	82,245,679	74,029,645

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

	September 30, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 265,088	$ 427,135
Working capital	198,786	277,612
Total assets	327,567	499,153
Deferred revenue, net of current portion	54,204	60,667

Total stockholders' equity	175,634	349,097

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