Editas Medicine Announces Fourth Quarter and Full Year 2024 Results and Business Updates

March 5, 2025

On track to declare two in vivo *editing development candidates via gene upregulation, one in HSCs and one in liver, in mid-2025*
Company to present further in vivo *HSC preclinical data and further* in vivo *preclinical data in one liver indication by year-end*
On track to establish one additional target cell type/tissue by year-end
Strong cash position with operational runway into the second quarter of 2027

CAMBRIDGE, Mass., March 05, 2025 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company focused on developing transformative medicines for serious diseases, today reported financial results for the fourth quarter and full year 2024 and provided business updates.

"Our objective and strategy to become a leader in *in vivo* gene editing accelerated in the fourth quarter after we achieved *in vivo* preclinical proof of concept ahead of schedule and shared positive preclinical *in vivo* data demonstrating the potential of our platform technology to achieve gene upregulation, or amplifying the expression of an existing protein to achieve clinically relevant levels that could potentially drive cures across tissues with a single dose," said Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine. "We believe the ability to provide *in vivo* gene editing via gene upregulation holds the potential to significantly expand the addressable therapeutic possibilities for CRISPR-based gene editing, and we are poised to make meaningful progress towards the clinic in 2025."

"The data we have shared over the last several months demonstrate our ability to attain *in vivo* gene editing via gene upregulation to increase the level of a functioning protein to address diseases caused by loss of function or deleterious mutations. Notably, our progress highlights the potential of our gene upregulation strategy across multiple tissues with our 'plug 'n play' program," said Linda C. Burkly, Ph.D., Chief Scientific Officer, Editas Medicine. "I am proud of our team's progress, underscoring the therapeutic promise of our scientific work as a significant step towards the clinic as we develop our pipeline of potentially transformative *in vivo* gene editing medicines."

Recent Achievements and Outlook

***In Vivo* Medicines**

- Demonstrated preclinical proof of concept in non-human primates and humanized mice, highlighting the potential of Editas' gene upregulation strategy across tissues, presented in December 2024 and January 2025.

Hematopoietic Stem Cells

- Achieved effective delivery and meaningful levels of *in vivo* editing of the *HBG 1/2* promoter, our therapeutic target, in HSCs with Editas' proprietary targeted lipid nanoparticles (tLNPs) after a single dose of tLNP in non-human primates.
    - Ongoing evaluation of further optimized LNP formulations expected to achieve even higher therapeutic editing levels.
- The Company remains on track to declare an *in vivo* HSC development candidate in mid-2025 and to present additional preclinical *in vivo* HSC data by year-end.

Liver Cells

- Achieved proof of concept in non-human primates, validating high efficiency *in vivo* gene editing in the liver with first use of AsCas12a delivery by LNP.
- Demonstrated proof of upregulation strategy in mice by increasing clinically relevant target protein resulting in significant disease biomarker reduction for an undisclosed liver target.
- The Company remains on track to declare an *in vivo* liver development candidate in mid-2025 and to present additional preclinical *in vivo* liver data by year-end.

Other Cells/Tissues

- Demonstrated *in vivo* proof of concept for "plug 'n play" delivery to extrahepatic cell types using the Company's proprietary LNP targeting platform at high efficiency in humanized mice.
- The Company remains on track to establish and disclose one additional target cell type/tissue beyond HSCs and liver by year-end.

***Ex Vivo* Hemoglobinopathies**

- Reni-cel (renizgamglogene autogedtemcel, previously EDIT-301) for severe sickle cell disease and transfusion-dependent beta thalassemia.
    - In December 2024, the Company announced that it ended development of reni-cel after an extensive search failed

to yield a commercial partner.
- As a result of the decision to end development of reni-cel, the Company initiated cost savings measures, including an approximately 65% reduction in headcount to align workforce and resources to an *in vivo* pipeline.

Fourth Quarter and Full Year 2024 Financial Results
Cash, cash equivalents, and marketable securities as of December 31, 2024, were $269.9 million compared to $265.1 million as of September 30, 2024. The Company expects the existing cash, cash equivalents, and marketable securities, together with the retained portions of the payments payable under its license agreement with Vertex Pharmaceuticals, to fund operating expenses and capital expenditures into the second quarter of 2027. The Company's cash runway includes total estimated expenses of approximately $45.0 million to $55.0 million related to ending development of reni-cel and related employee exit costs.

Fourth Quarter 2024

- For the three months ended December 31, 2024, net loss attributable to common stockholders was $45.4 million, or $0.55 per share, compared to a net loss of $18.9 million, or $0.23 per share, for the same period in 2023.

- Collaboration and other research and development revenues decreased to $30.6 million for the three months ended December 31, 2024, compared to $60.0 million for the same period in 2023. This decrease was primarily attributable to revenue recognized from the upfront payment under the Company's license agreement with Vertex executed in December 2023.

- Research and development expenses decreased by $21.0 million to $48.6 million for the three months ended December 31, 2024, compared to $69.6 million for the same period in 2023. The decrease was primarily attributable to sublicense payments made in connection with the Vertex license agreement in December 2023.

- General and administrative expenses increased by $1.9 million to $16.4 million for the three months ended December 31, 2024, compared to $14.5 million for the same period in 2023. The increase was primarily driven by increased professional service expenses for strategic business initiatives.

- Restructuring charges were $12.2 million for the three months ended December 31, 2024, compared to no restructuring charges for the same period in 2023. The restructuring charges were related to the discontinuation of the clinical development of the reni-cel program, initiated in December 2024, and the related workforce reduction.

Full Year 2024

- For the full year 2024, net loss attributable to common stockholders was $237.1 million, or $2.88 per share, compared to net loss of $153.2 million, or $2.02 per share, for the same period in 2023.

- Collaboration and other research and development revenues decreased to $32.3 million for 2024, compared to $78.1 million for the same period in 2023. The decrease was primarily attributable to revenue recognized from the upfront payment under the Company's license agreement with Vertex executed in December 2023.

- Research and development expenses increased by $21.5 million to $199.2 million for 2024, compared to $177.7 million for the same period in 2023. The increase was primarily related to clinical and manufacturing costs related to the progression of the Company's former reni-cel program as well as costs attributable to *in vivo* research and discovery.

- General and administrative expenses increased by $2.3 million to $72.0 million for 2024, compared to $69.7 million for the same period in 2023. The increase was primarily attributable to increased employee-related expenses related to increased headcount to support business operations.

- Restructuring charges were $12.2 million for 2024, compared to no restructuring charges for the same period in 2023. The restructuring charges were related to the discontinuation of the clinical development of our reni-cel program, initiated in December 2024, and the related workforce reduction.

Upcoming Events
Editas Medicine plans to participate in the following investor events:

- Leerink Partners Global Biopharma Conference
  March 10, 2024
  Miami Beach, FL

- Barclays 27th Annual Global Healthcare Conference
  March 11, 2024

Miami Beach, FL

No Conference Call
The Company is no longer hosting quarterly earnings conference calls.

About Editas Medicine
As a pioneering 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 transformative in vivo medicines for people living with serious diseases around the world. Editas Medicine aims to discover, develop, manufacture, and commercialize durable, precision in vivo gene editing 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 studies and its research and development programs, including the Company's expectation to declare two *in vivo* development candidates in mid-2025 and establish an additional *in vivo* target cell type/tissue beyond HSCs and the liver by the end of 2025; the timing for the Company's receipt and presentation of data from its preclinical studies, including presenting further *in vivo* HSC data and further *in vivo* data in one liver indication by the end of 2025; the potential of, and expectations for, the Company's *in vivo* product candidates; the timing or likelihood of regulatory filings and approvals; the amount of anticipated costs related to ending development of reni-cel and related employee exit costs; and the Company's expectations regarding cash runway into the second quarter of 2027. 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 preclinical studies; availability and timing of results from preclinical studies; expectations for regulatory approvals to conduct trials; and the 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 December 31,		Twelve Months Ended December 31,	
	2024	**2023**	**2024**	**2023**
Collaboration and other research and development revenues	30,604	60,049	32,314	78,123
Operating expenses:				
Research and development	48,611	69,556	199,247	177,651
General and administrative	16,354	14,455	71,987	69,653
Restructuring charges	12,232	—	12,232	—
Total operating expenses	77,197	84,011	283,466	247,304
Operating loss	(46,593)	(23,962)	(251,152)	(169,181)
Other income, net:				
Other expense, net	(3)	(14)	(3)	(1,604)
Interest income, net	1,201	5,102	14,062	17,566
Total other income, net	1,198	5,088	14,059	15,962
Net loss	$ (45,395)	$ (18,874)	$ (237,093)	$ (153,219)
Net loss per share, basic and diluted	$ (0.55)	$ (0.23)	$ (2.88)	$ (2.02)
Weighted-average common shares outstanding, basic and diluted	82,613,831	81,710,470	82,338,220	75,965,633

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

	December 31,	December 31,

	2024	2023
Cash, cash equivalents, and marketable securities	$ 269,913	$ 427,135
Working capital	212,090	277,612
Total assets	341,589	499,153
Deferred revenue, net of current portion	54,204	60,667
Total stockholders' equity	134,274	349,097

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Source: Editas Medicine, Inc.