Editas Medicine Highlights New In Vivo Preclinical Proof of Concept Data, Anticipated 2025 Key Milestones, and Three-year Strategic Priorities

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- *Achieved* in vivo *preclinical proof of concept of editing hematopoietic stem cells in non-human primates as a key step toward developing a novel* in vivo *treatment for sickle cell disease and beta thalassemia*
- *Achieved* in vivo *editing of liver cells in non-human primates and* in vivo *delivery to two additional cell types in humanized mice*
- *Anticipated 2025 milestones include: declare two* in vivo *development candidates, one in HSCs and one in liver; present further* in vivo *HSC data; present* in vivo *data in one liver indication; establish one additional target cell type/tissue; and continue to derive revenue through sublicensing foundational IP*
- *Strategic priorities through 2027 include: submit at least one IND/CTA; achieve human* in vivo *proof of concept in HSC editing for the treatment of sickle cell disease and beta thalassemia; and commence late-stage trial of at least one asset*
- *Strong financial position with operational runway into Q2 2027*
- *Company to present at the 43rd Annual J.P. Morgan Healthcare Conference on Wednesday, January 15 at 11:15 a.m. PST*

CAMBRIDGE, Mass., Jan. 13, 2025 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a pioneering gene editing company focused on developing transformative medicines for serious diseases, today announced its three-year strategic priorities, anticipated 2025 key milestones, and new *in vivo* preclinical proof of concept data in non-human primates editing hematopoietic stem cells (HSCs) and liver cells and *in vivo* delivery data in humanized mice to two additional target cell types.

"Two years ago, we detailed our objective and strategy to become a leader in *in vivo* programmable gene editing, and last month, supported by our scientific progress and multiple breakthroughs, we announced our transition to a fully *in vivo* company," said Gilmore O'Neill, M.B., M.M.Sc., President and Chief Executive Officer, Editas Medicine. "Today, we are also thrilled to share new *in vivo* preclinical data highlighting the potential of our gene upregulation strategy across multiple tissues with our 'plug 'n play' program. We believe the ability to provide *in vivo* gene editing that functions via gene upregulation across tissues holds the potential to significantly expand the addressable therapeutic possibilities for CRISPR-based gene editing and uniquely position Editas as a leader in the field moving forward. We are poised to make meaningful progress in 2025 towards the clinic as we develop our pipeline of potentially transformative *in vivo* medicines."

New *In Vivo* Proof of Concept Data in Non-human Primates and Humanized Mice Highlighting the Potential of Editas' Gene Upregulation Strategy Across Tissues

Hematopoietic Stem Cells

- Achieved effective delivery and meaningful levels of editing 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 therapeutic editing levels.

Liver Cells

- Achieved proof of concept in non-human primates validating high efficiency 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.

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.

Additional details of the data are contained in Editas Medicine's Corporate Presentation, available in the **Events and Presentations section** of the Company's website.

2025 Anticipated Milestones and *in vivo* Pipeline Advancement

- **Declare two *in vivo* development candidates by mid-2025,** one in HSCs for the treatment of sickle cell disease and

beta thalassemia and one in liver cells for an undisclosed indication;

- **Present additional *in vivo* preclinical editing data,** in both HSCs and liver cells in large animal models;
- **Establish an additional *in vivo* target cell type/tissue *beyond* HSCs and the liver by the end of 2025**; and
- **Derive additional value from the Company's foundational CRISPR IP,** building on the previously announced DRI Healthcare monetization financing and continuing to issue sublicenses.

2025-2027 Strategic Priorities

1. **Launch clinical trials for multiple *in vivo* programs,** including submitting at least one investigational new drug (IND) application/clinical trial application (CTA) by mid-2026, beginning human trials by the second half of 2026, and initiating at least one late-stage clinical trial in the second half of 2027;

2. **Achieve human *in vivo* proof of concept in at least one indication by the end of 2026,** validating the Company's *in vivo* upregulation strategy in humans; and

3. **Expand the range of diseases addressable by *in vivo* gene upregulation,** including announcing *in vivo* proof of concept in at least one additional tissue *beyond* HSCs and the liver by 2027**,** demonstrating the "plug 'n play" potential of Editas' proprietary extrahepatic LNP platform.

Financial Items
As of December 31, 2024, the Company had approximately $270 million of cash, cash equivalents, and marketable securities, and expects its cash runway to extend into the second quarter of 2027.

43rd Annual J.P. Morgan Healthcare Conference Presentation and Webcast
Dr. O'Neill will discuss the Company's new *in vivo* preclinical proof of concept data, anticipated 2025 key milestones, and three-year strategic priorities for its gene editing medicines and platform technology at the 43rd Annual J.P. Morgan Healthcare Conference on Wednesday, January 15, 2025 at 11:15 a.m. PST / 2:15 p.m. ET in San Francisco, CA. A **live webcast** of the presentation will be available on the "Investors" section of the Editas Medicine website at www.editasmedicine.com. An archived replay will be available on the website for approximately 30 days following the presentation.

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 development candidates for its *in vivo* programs by mid-2025, establish an additional *in vivo* target cell type/tissue beyond HSCs and the liver by the end of 2025 and achieve *in vivo* proof of concept by 2027; the timing for the Company's receipt and presentation of data from its preclinical studies, including presenting further *in vivo* HSC and liver data in 2025; the potential of, and expectations for, the Company's product candidates; the timing or likelihood of regulatory filings and approvals, including the timing of the Company's submission of any IND or CTA and ability to commence clinical trials for its *in vivo* programs; 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 speak only as of the date hereof, and the Company expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

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Source: Editas Medicine, Inc.