Prime Medicine Reports Full Year 2024 Financial Results and Provides Business Updates

February 28, 2025

-- On track to report initial data from Phase 1/2 clinical trial of PM359 for p47^{phox} CGD in 2025 --

-- IND-enabling studies ongoing for PM577 for Wilson's Disease; expect to file IND and/or CTA in 1H 2026 --

-- Additional high-value programs advancing through preclinical development --

CAMBRIDGE, Mass., Feb. 28, 2025 (GLOBE NEWSWIRE) -- Prime Medicine, Inc. (Nasdaq: PRME), a biotechnology company committed to delivering a new class of differentiated one-time curative genetic therapies, today reported financial results for the full year ended December 31, 2024 and provided a business update.

"Entering 2025 marks the next chapter for Prime Medicine as we look to share initial data for our most advanced product candidate, PM359, in chronic granulomatous disease, the only Prime Editor currently in clinical development," said Keith Gottesdiener, M.D., President and Chief Executive Officer of Prime Medicine. "If positive, we believe this readout will be a watershed moment for our company, validating Prime Editing's differentiated safety profile and curative potential."

Dr. Gottesdiener added, "In parallel, we are advancing our Prime Editors for Wilson's Disease and Cystic Fibrosis. We are particularly encouraged by recent progress in Wilson's Disease, a program that we believe will be a fundamental driver of our long-term growth and remains on-track for clinical entry in 2026. We look forward to sharing new *in vivo* data from across our preclinical programs in 2025 as we continue to accelerate our pipeline through both internal efforts and additional strategic partnerships."

Prime Medicine's Pipeline:

Prime Medicine is advancing a set of high-value programs across its core areas of focus (hematology, immunology and oncology, liver, and lung). These include *ex vivo* hematopoietic stem cell (HSC) programs for the treatment of p47^{phox} chronic granulomatous disease (CGD) and X-linked CGD; a lipid nanoparticle (LNP) Prime Editor for the treatment of Wilson's Disease; a LNP or adeno-associated virus (AAV) Prime Editors for the treatment of cystic fibrosis (CF); and *ex vivo* T-cell therapies, which are being developed in collaboration with Bristol Myers Squibb.

Anticipated Upcoming Milestones:

Chronic Granulomatous Disease (CGD):

- Announce initial clinical data from Cohort 1 in Phase 1/2 trial of PM359 for p47^{phox} CGD in 2025.
- The initial readout will include safety and engraftment data, as well as key outcome measures: the rate of Prime Editing in hematopoietic stem cells and the reconstitution of NADPH oxidase activity as measured by the DHR assay.

Wilson's Disease:

- Advance PM577 through investigational new drug (IND)-enabling studies for the treatment of Wilson's Disease patients with the most prevalent Wilson's Disease mutation in the United States.
- File IND and/or clinical trial application for PM577 in the first half of 2026.

Full Year 2024 Financial Results

- **Research and Development (R&D) Expenses:** R&D expenses were $155.3 million for the year ended December 31, 2024, as compared to $147.9 million for the year ended December 31, 2023. The increase in R&D expenses was driven by increases in facilities, personnel and clinical expenses related to PM359.
- **General and Administrative (G&A) Expenses:** G&A expenses were $50.2 million for the year ended December 31, 2024, as compared to $43.4 million for the year ended December 31, 2023. The increase in G&A expenses was driven by increases in facilities and personnel-related expenses.
- **Net Loss:** Net loss was $195.9 million for the year ended December 31, 2024, as compared to $198.1 million for the year ended December 31, 2023.
- **Cash Position:** As of December 31, 2024, cash, cash equivalents, investments, and restricted cash were $204.5 million, as compared to $135.2 million as of December 31, 2023.

Financial Guidance

Based on its current operating plans, Prime Medicine expects that its cash, cash equivalents and investments as of December 31, 2024 will be

sufficient to fund its operating expenses and capital expenditure requirements into the first half of 2026.

About Prime Medicine

Prime Medicine is a leading biotechnology company dedicated to creating and delivering the next generation of gene editing therapies to patients. The Company is deploying its proprietary Prime Editing platform, a versatile, precise and efficient gene editing technology, to develop a new class of differentiated one-time curative genetic therapies. Designed to make only the right edit at the right position within a gene while minimizing unwanted DNA modifications, Prime Editors have the potential to repair almost all types of genetic mutations and work in many different tissues, organs and cell types. Taken together, Prime Editing's versatile gene editing capabilities could unlock opportunities across thousands of potential indications.

Prime Medicine is currently progressing a diversified portfolio of investigational therapeutic programs organized around its core areas of focus: hematology, immunology and oncology, liver and lung. Across each core area, Prime Medicine is focused initially on a set of high-value programs, each targeting a disease with well-understood biology and a clearly defined clinical development and regulatory path, and each expected to provide the foundation for expansion into additional opportunities. Over time, the Company intends to maximize Prime Editing's broad and versatile therapeutic potential, as well as the modularity of the Prime Editing platform, to rapidly and efficiently expand beyond the diseases in its current pipeline, potentially including additional genetic diseases, immunological diseases, cancers, infectious diseases, and targeting genetic risk factors in common diseases, which collectively impact millions of people. For more information, please visit www.primemedicine.com.

From time to time Prime Medicine may use its website, our X, formerly Twitter, account (@PrimeMedicine) or its LinkedIn profile at https://www.linkedin.com/company/prime-medicine to distribute material information. Its financial and other material information is routinely posted to and accessible on the Investors section of its website, available at www.primemedicine.com. Investors are encouraged to review the Investors section of its website because the Company may post material information on that site that is not otherwise disseminated by the Company. Information that is contained in and can be accessed through the Company's website or its social media is not incorporated into, and does not form a part of, this press release.

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Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, implied and express statements about Prime Medicine's beliefs and expectations regarding: the continued development and advancement of PM359 and PM577, including the ongoing Phase 1/2 trial of PM359 and the timing of the anticipated release of initial clinical data readout for $p47^{phox}$ CGD in 2025, and the timing for completion of IND-enabling studies for PM577; the timing, progress, and results of its Wilson's Disease program, including the timing of the release of updated data and filing of an IND and/or CTA application in the first half of 2026; the potential of PM359 to correct the causative mutation of CGD; the potential of Prime Editing to correct the causative mutations of Wilson's Disease and CF; the potential for its modular universal LNP platform to precisely deliver Prime Editors, correct disease-causing mutations in the liver, and deliver transformative treatments for Wilson's Disease, and other rare and non-rare liver indications; the ability to demonstrate, and the timing of, preclinical proof-of-concept in vivo for multiple programs; the further advancement of Prime Editors to maximize their versatility, precision and efficiency; the collaboration with Bristol Myers Squibb and the intended and potential benefits thereof, including the receipt of potential milestone and royalty payments from commercial product sales, if any; the initiation, timing, progress, and results of its research and development programs, preclinical studies and future clinical trials, including the release of data related thereto; the modularity of the Prime Editing platform and the benefits thereof; the potential for Prime Editors to more precisely and effectively achieve genetic modification; the potential for Prime Editors to repair genetic mutations and offer curative genetic therapies for a wide spectrum of diseases; its continued development and optimization of various non-viral and viral delivery systems; the expansion of Prime Editing's therapeutic potential and the creation of value through strategic business development to extend the reach and impact of Prime Editing to areas beyond Prime Medicine's current core areas of focus; exploring business development opportunities that could accelerate existing work and the benefits thereof; its expectations regarding the breadth of Prime Editing technology and the implementation of its strategic plans for its business, programs, and technology; the potential of Prime Editing to unlock opportunities across thousands of potential indications; and its expected cash runway . The words "may," "might," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "expect," "estimate," "seek," "predict," "future," "project," "potential," "continue," "target" and similar words or expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks associated with: uncertainties related to Prime Medicine's product candidates entering clinical trials; the authorization, initiation, and conduct of preclinical and IND-enabling studies and other development requirements for potential product candidates, including uncertainties related to opening INDs and obtaining regulatory approvals; risks related to the development and optimization of new technologies, the results of preclinical studies, or clinical studies not being predictive of future results in connection with future studies; the scope of protection Prime Medicine is able to establish and maintain for intellectual property rights covering its Prime Editing technology; Prime Medicine's ability to identify and enter into future license agreements and collaborations; Prime Medicine's expectations regarding the anticipated timeline of its cash runway and future financial performance; and general economic, industry and market conditions. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in Prime Medicine's most recent Annual Report on Form 10-K, as well as any subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent Prime Medicine's views only as of today and should not be relied upon as representing its views as of any subsequent date. Prime Medicine explicitly disclaims any obligation to update any forward-looking statements subject to any obligations under applicable law. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

Investor Contacts

Gregory Dearborn
Prime Medicine
713-503-3364
gdearborn@primemedicine.com

Hannah Deresiewicz
Precision AQ
212-362-1200
hannah.deresiewicz@precisionaq.com

Media Contact

Dan Budwick, 1AB
dan@1ABmedia.com

Condensed Consolidated Balance Sheet Data
(unaudited)

(in thousands)	December 31, 2024	December 31, 2023
Cash, cash equivalents, and investments	$ 190,442	$ 121,665
Total assets	297,508	193,851
Total liabilities	144,359	60,780
Total stockholders' equity	153,149	133,071

Condensed Consolidated Statement of Operations
(unaudited)

(in thousands, except share and per share amounts)	Year Ended December 31, 2024	Year Ended December 31, 2023
Revenue:		
Collaboration revenue — related party	$ 1,609	$ —
Collaboration revenue	1,374	—
Total revenue	2,983	—
Operating expenses:		
Research and development	155,289	147,905
Settlement payment — related party	—	13,500
General and administrative	50,161	43,387
Total operating expenses	205,450	204,792
Loss from operations	(202,467)	(204,792)
Other income:		
Interest income	3,522	2,811
Accretion (amortization) of investments	3,507	5,677
Change in fair value of short-term investment — related party	(485)	(2,382)
Other income, net	41	274
Total other income, net	6,585	6,380
Net loss before income taxes	(195,882)	(198,412)
Benefit from income taxes	—	279
Net loss attributable to common stockholders	$ (195,882)	$ (198,133)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.65)	$ (2.18)
Weighted-average common shares outstanding, basic and diluted	118,600,381	90,969,327