



Prime Medicine Reports First Quarter 2025 Financial Results and Provides Business Updates

May 8, 2025

-- Initial data from Phase 1/2 trial of PM359 for p47^{phox} CGD expected in 2025 --

-- IND and/or CTA for PM577 for Wilson's Disease on-track for 1H 2026 --

-- Unveiled potentially best-in-class program for AATD; IND and/or CTA filing targeted for mid-2026 --

CAMBRIDGE, Mass., May 08, 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 quarter ended March 31, 2025 and provided a business update.

"We recently unveiled our AATD program, further demonstrating our commitment to building a liver franchise of Prime Editors designed to cure major genetic diseases," said Keith Gottesdiener, M.D., President and Chief Executive Officer of Prime Medicine. "Both our Wilson's Disease and AATD programs are advancing through preclinical development, and we look forward to initiating clinical trials in both indications in 2026. In addition, we remain on track to report initial data from our Phase 1/2 trial of PM359 in CGD this year, and continue to progress our efforts in CF."

Dr. Gottesdiener continued, "While PM359 leverages a different delivery mechanism from our programs in liver and lung diseases, we believe our forthcoming CGD data could provide important readthrough across our pipeline. If positive, these data would demonstrate the potential of Prime Editing as a powerful and differentiated technology, potentially capable of offering curative benefit to patients following a single treatment. We remain committed to executing with focus and discipline as we approach these first in-human data, which we hope will mark a key inflection point on our path to sustained growth."

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; lipid nanoparticle (LNP) Prime Editors for the treatment of Wilson's Disease and alpha-1 antitrypsin deficiency (AATD); 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.

Recent Business Updates

- In March 2025, Prime Medicine unveiled its preclinical program for the treatment of AATD. Prime Medicine's program leverages the Company's universal liver LNP to edit the E342K (Pi*Z) mutation in the SERPINA1 gene, the prevalent disease-causing mutation in AATD, with the potential to treat both lung- and liver-associated disease. In initial in vivo data, Prime Medicine observed high levels of editing at the target site, with full restoration of circulating wild-type AAT protein (M-AAT) to normal human range. Additionally, based on preclinical studies using unoptimized surrogate Prime Editors across Prime Medicine's liver franchise, the Company believes Prime Editing has the ability to correct disease-causing mutations without introducing off-target or bystander edits.

Anticipated Upcoming Milestones:

Chronic Granulomatous Disease (CGD):

- Announce initial clinical data from Cohort 1 in the 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, including 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 investigational new drug (IND) and/or clinical trial application (CTA) for PM577 in the first half of 2026.

AATD:

- Initiate IND-enabling studies for the treatment of AATD.
- File IND and/or CTA in mid-2026.

First Quarter 2025 Financial Results

- **Research and Development (R&D) Expenses:** R&D expenses were \$40.6 million for the three months ended March 31, 2025, as compared to \$37.8 million for the three months ended March 31, 2024. The increase in R&D expenses primarily due to the expansion and build out of our laboratory space at 60 First Street and 500 Arsenal Street.
- **General and Administrative (G&A) Expenses:** G&A expenses were \$13.3 million for the three months ended March 31, 2025, as compared to \$11.2 million for the three months ended March 31, 2024. The increase in G&A expenses was driven by personnel expenses.
- **Net Loss:** Net loss was \$51.9 million for the three months ended March 31, 2025, as compared to \$45.8 million for the three months ended March 31, 2024.
- **Cash Position:** As of March 31, 2025, cash, cash equivalents, investments, and restricted cash were \$158.3 million, as compared to \$204.5 million as of December 31, 2024.

Financial Guidance

Based on its current operating plans, Prime Medicine expects that its cash, cash equivalents and investments as of March 31, 2025 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 our 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.

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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, including the ongoing Phase 1/2 trial and the timing of the anticipated release of initial clinical data readout for p47^{Phox} CGD in 2025; the continued development and advancement of its AATD and Wilson's Disease programs, including the timing of the filing of IND and/or CTA applications in mid-2026 and 1H 2026, respectively; 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 significance of data from the Phase 1/2 trial of PM359 on other pipeline programs and Prime Editing; the potential of Prime Editing to correct the causative mutations of diseases, including of AATD, Wilson's Disease, CF and p47^{Phox} CGD; 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.

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.

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Condensed Consolidated Balance Sheet Data (unaudited)

(in thousands)	March 31, 2025	December 31, 2024
Cash, cash equivalents, and investments	\$ 144,256	\$ 190,442
Total assets	\$ 328,163	\$ 297,508
Total liabilities	\$ 221,239	\$ 144,359
Total stockholders' equity	\$ 106,924	\$ 153,149

Condensed Consolidated Statement of Operations (unaudited)

(in thousands, except share and per share amounts)	Three Months Ended March 31,	
	2025	2024
Revenue:		
Collaboration revenue — related party	\$ 1,454	\$ —
Collaboration revenue	—	591
Total revenue	1,454	591
Operating expenses:		
Research and development	\$ 40,562	\$ 37,774
General and administrative	13,284	11,158
Total operating expenses	53,846	48,932
Loss from operations	(52,392)	(48,341)
Other income:		
Interest income	1,182	676
Accretion (amortization) of investments	339	830
Change in fair value of short-term investment — related party	(1,056)	1,166
Other income, net	37	42
Total other income, net	502	2,714
Net loss before income taxes	(51,890)	(45,627)
Provision for income taxes	—	(134)
Net loss attributable to common stockholders	\$ (51,890)	\$ (45,761)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.40)	\$ (0.44)
Weighted-average common shares outstanding, basic and diluted	130,884,490	104,466,178