Prime Medicine Reports Third Quarter 2024 Financial Results and Provides Business Updates

November 12, 2024

-- Presented initial in vivo data for universal liver-targeted LNP and Wilson's Disease program at ESGCT; initiated IND-enabling activities for Wilson's Disease program in 4Q 2024 and remains on track to file IND and/or CTA in 1H 2026 --

-- Announced strategic research collaboration and license agreement with Bristol Myers Squibb; received $110 million upfront, with potential for more than $3.5 billion in milestone payments --

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

-- Unveiled strategically focused pipeline, prioritizing set of high value programs, each with a clearly defined path to value creation and the potential to accelerate development of follow-on programs --

-- Pro-forma cash, cash equivalents and investments as of September 30, 2024, expected to be sufficient to fund planned operations into 1H 2026 --

CAMBRIDGE, Mass., Nov. 12, 2024 (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 third quarter ended September 30, 2024 and provided a business update.

"In October, we presented the first *in vivo* preclinical data from our Wilson's Disease program, demonstrating that Prime Editors can efficiently correct pathogenic mutations, without introducing safety concerns or detectable off-target edits," said Keith Gottesdiener, M.D., President and Chief Executive Officer of Prime Medicine. "These data are meaningful both for our efforts in Wilson's Disease – a devasting condition that is believed to affect more than 20,000 people in the United States and Europe, and for which there are currently no approved disease-modifying therapies – and for our efforts in liver disease programs more broadly. Our Wilson's Disease program leverages our proprietary, universal LNP platform, and these interim results suggest that our LNP may successfully deliver increased potency, as well as an improved safety profile and biodistribution, compared to other commonly used LNPs in development. We look forward to advancing our Wilson's Disease program toward an IND and/or CTA filing in the first half of 2026."

Dr. Gottesdiener continued, "Also in recent months, we announced our decision to strategically prioritize a set of high value programs, each with a clear path to value inflection and the potential to unlock multiple follow-on opportunities. This includes our wholly owned efforts in CGD and Wilson's Disease, as well as our efforts in cell therapy and cystic fibrosis, which we are advancing with support from Bristol Myers Squibb and the Cystic Fibrosis Foundation, respectively. We believe partnerships like these will continue to play a critical role as we build Prime Medicine, allowing us access to best-in-class expertise and non-dilutive capital and enabling us to more rapidly realize the full potential of Prime Editing."

Recent Business Updates

Pipeline Updates

In September 2024, Prime Medicine unveiled its strategically focused pipeline and plans to prioritize a set of high value programs, each targeting a disease with well-understood biology and a clearly defined clinical development and regulatory path. Each prioritized program is also intended to serve as a beachhead, in that the modularity of the Prime Editing platform is expected to allow Prime Medicine to generate follow-on candidates rapidly and efficiently.

The prioritized programs sit across Prime Medicine's core areas of focus (hematology, immunology and oncology, liver, and lung), and include *ex vivo* hematopoietic stem cell (HSC) programs for the treatment of p47^{phox} chronic granulomatous disease (CGD) and X-linked CGD; *ex vivo* T-cell therapies; a lipid nanoparticle (LNP) Prime Editor for the treatment of Wilson's Disease; and LNP or adeno-associated virus (AAV) Prime Editors for the treatment of cystic fibrosis (CF). In order to pursue these prioritized high value programs as rapidly as possible, the Company is identifying partnership opportunities to advance its other programs, including those for neurological diseases, cell therapy, ocular diseases and hearing loss.

Hematology, Immunology and Oncology:

- In the third quarter of 2024, Prime Medicine initiated its global Phase 1/2 clinical trial of PM359 in the United States. The Phase 1/2 clinical trial is a multinational, first-in-human trial designed to assess the safety, biological activity and preliminary clinical activity of PM359 in adult and pediatric study participants. Prime Medicine expects to report initial clinical data from the Phase 1/2 trial in 2025.

Liver:

- At the European Society for Gene and Cell Therapy (ESGCT) 31st Annual Congress (October 22-25, 2024), Prime Medicine presented data from multiple studies showcasing the potential of its proprietary, universal LNP platform to precisely deliver Prime Editors to correct disease-causing mutations in the liver, including in Wilson's Disease. Prime Medicine's universal LNP contains a GalNAc-targeting ligand (GalNAc-LNP), a validated mechanism for liver-specific delivery of gene editors. Highlights included:
    - Preclinical data demonstrated that the delivery of Prime Editors using a GalNAc-LNP (GalNAc-LNP Prime Editor)

resulted in increased potency, as well as improved safety profile and biodistribution, compared to traditional LNPs that have gone into the clinic.
    - *In vivo* proof-of-concept data from Prime Medicine's GalNAc-LNP Prime Editor for the treatment of Wilson's Disease, demonstrated up to 80% precise correction of the H1069Q mutation and restoration of ATP7B mRNA to wild-type levels in a humanized mouse model, as well as precise editing of liver cells in non-human primates (NHPs), with up to 51% precise editing with a surrogate H1069Q Prime Editor. In preclinical studies to-date, Prime Medicine observed significant reductions of copper accumulation in the livers of humanized mice. In both mouse and NHP studies, no detectable off-target edits or unintended edits at the target site were observed.
- Prime Medicine is completing the final stages of lead optimization and recently initiated investigational new drug (IND)-enabling activities for its Wilson's Disease program, with an IND and/or clinical trial application (CTA) filing expected in the first half of 2026. Prime Medicine will present on its Wilson's Disease program at the American Association for the Study of Liver Diseases (AASLD), to be held on November 15-19, 2024. Longer term, the Company expects to use its universal LNP across all liver disease programs, allowing more rapid and cost-efficient expansion into follow-on rare and non-rare liver indications.

Corporate Updates

In September 2024, Prime Medicine entered into a strategic research collaboration and license agreement with Bristol Myers Squibb to develop and commercialize multiple Prime Edited *ex vivo* T-cell therapies. Under the terms of the agreement, Prime Medicine received a $55 million upfront payment and a $55 million equity investment from Bristol Myers Squibb. Prime Medicine is also eligible to receive more than $3.5 billion in milestones, including up to $1.4 billion in development milestones and more than $2.1 billion in commercialization milestones, along with royalties on net sales.

Third Quarter 2024 Financial Results

- **Research and Development (R&D) Expenses:** R&D expenses were $40.3 million for the three months ended September 30, 2024, as compared to $41.0 million for the three months ended September 30, 2023.
- **General and Administrative (G&A) Expenses:** G&A expenses were $14.1 million for the three months ended September 30, 2024, as compared to $10.5 million for the three months ended September 30, 2023. The increase in G&A expenses was driven by personnel expenses, primarily related to an increase in non-cash stock-based compensation expense.
- **Net Loss:** Net loss was $52.5 million for the three months ended September 30, 2024, as compared to $50.7 million for the three months ended September 30, 2023.
- **Cash Position:** As of September 30, 2024, pro-forma cash, cash equivalents, investments and restricted cash were $244.6 million, which includes the $55 million equity investment from Bristol Myers Squibb received in September 2024 and the $55 million up-front consideration received in October 2024. Cash, cash equivalents, investments, and restricted cash were $189.6 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 pro-forma cash, cash equivalents and investments as of September 30, 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 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.

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 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 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, GSD1b, and other rare and non-rare liver indications; the modular universal LNP platform's ability to be used repeatedly to generate candidates that offer an improved safety profile and biodistribution compared to other LNPs in development; the anticipated maturation into a clinical-stage company by bringing PM359 into clinical development in 2024 with initial clinical data from the ongoing Phase 1/2 clinical trial of PM359 expected in 2025; 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 safety profile, tolerability, and durability of

its universal LNP; the initiation, timing, progress, and results of its research and development programs, preclinical studies and future clinical trials; 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; its ability to demonstrate superior off-target profiles for Prime Editing programs; 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; and the potential of Prime Editing to unlock opportunities across thousands of potential indications. 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; 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)	September 30, 2024	December 31, 2023
Cash, cash equivalents, and investments	175,527	121,665
Total assets	332,784	193,851
Total liabilities	143,280	60,780
Total stockholders' equity	189,504	133,071

Condensed Consolidated Statement of Operations
(unaudited)

(in thousands, except share and per share amounts)	Three Months Ended September 30, 2024	2023
Collaboration revenue	$ 209	$ —
Operating expenses:		
Research and development	$ 40,340	$ 40,967
General and administrative	14,101	10,492
Total operating expenses	54,441	51,459

Loss from operations	(54,232)	(51,459)
Other income:		
Accretion (amortization) of investments	885	1,769
Interest income	697	410
Change in fair value of short-term investment — related party	215	(1,579)
Other income, net	(83)	43
Total other income, net	1,714	643
Net loss before income taxes	(52,518)	(50,816)
Benefit from income taxes	—	108
Net loss attributable to common stockholders	$ (52,518)	$ (50,708)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.44)	$ (0.55)
Weighted-average common shares outstanding, basic and diluted	119,764,270	91,846,835

Source: Prime Medicine, Inc.