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

May 10, 2024

-- Announced FDA clearance of first-ever IND application for a Prime Editing product, PM359, for the treatment of CGD; initial data from planned Phase 1/2 clinical trial expected in 2025 --

-- Presented new preclinical data demonstrating broad potential of Prime Editing technology at LNP Formulation and Process Development Summit and ASGCT 2024 --

-- Appointed Tony Coles, M.D. as senior advisor --

CAMBRIDGE, Mass., May 10, 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 first quarter ended March 31, 2024, and provided a business update.

"In 2024, we expect to bring the first-ever Prime Editing-based product candidate to patients, while continuing to strengthen our modular Prime Editing platform and advance our next wave of programs across a range of target tissues," said Keith Gottesdiener, M.D., President and Chief Executive Officer of Prime Medicine. "In recent months, we made meaningful progress toward this goal. In April, the U.S. Food and Drug Administration (FDA) cleared our investigational new drug (IND) application for PM359, our Prime Editor for the treatment of chronic granulomatous disease (CGD), and the first-ever Prime Editor product candidate to advance to the clinic. This represents a watershed moment for gene editing and for Prime Medicine, and we are eager to initiate our Phase 1/2 trial as we work to establish the potential for PM359 to correct the disease-causing mutation of CGD and ameliorate this devastating disease."

Dr. Gottesdiener continued, "At recent scientific meetings, we also presented new preclinical data showcasing the safety and broad potential of our Prime Editing technology across our pipeline programs and highlighting our proprietary delivery capabilities. Together, these presentations reinforce efforts across our core areas of focus – hematology and immunology, liver, lung, ocular and neuromuscular disease – and support our plans to advance a diverse pipeline into the clinic. Finally, in March, we were fortunate to welcome Dr. Tony Coles as a senior advisor to Prime Medicine. Tony brings a wealth of strategic perspectives and company growth experience in innovative drug discovery and development, and we look forward to his many contributions as Prime Medicine enters its next phase of growth."

Recent Business Updates

Chronic Granulomatous Disease (CGD)

- In April 2024, Prime Medicine announced that the FDA had cleared the Company's IND application for PM359 for the treatment of CGD, enabling the Company to initiate its planned global Phase 1/2 clinical trial 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 efficacy of PM359 in adult and pediatric study participants. Prime Medicine expects to report initial clinical data from the Phase 1/2 trial in 2025.
- At the American Society of Cell & Gene Therapy (ASCGT) 7th Annual Meeting (May 7 – 11, 2024), Prime Medicine presented new preclinical data from its CGD program, demonstrating the ability of Prime Editing to correct the disease-causing mutation in CGD patient blood stem cells, leading to restoration of neutrophil function in an *in vivo* mouse model with no off-target edits detected. Read a summary of the data presented here.

Broader Pipeline and Prime Editing Platform

- Also at the ASGCT Meeting and at the 3rd Annual LNP Formulation and Process Development Summit (April 29 – May 2, 2024), Prime Medicine presented new preclinical data from across Prime Medicine's platform and initial pipeline showcasing the broad potential of its Prime Editing technology and supporting the advancement of its pipeline programs. Highlights included:
    - Details on Prime Medicine's proprietary end-to-end capabilities in lipid nanoparticle discovery, as well as its development of non-viral delivery technologies for planned use in liver programs and, potentially, in programs across hematology/immunology and lung.
    - Additional preclinical data from Prime Medicine's Rhodopsin (*RHO*)-mediated Retinitis Pigmentosa (*RHO*-RP) program, supporting Prime Medicine's ability to correct multiple mutations in the *RHO* gene and showing that the correction of pathogenic mutations in humanized mouse models resulted in preservation of photoreceptors.
    - A presentation on the development and characterization of Prime Medicine's off-target assays, which collectively have supported the observed specificity and minimal, if any, off-target activity of Prime Editing.

Corporate

- In March 2024, Prime Medicine appointed Tony Coles, M.D. as its senior advisor. Dr. Coles is a seasoned biopharmaceutical leader, with experience translating groundbreaking science into novel medicines. Dr. Coles currently serves as Chairperson of the board of directors at Cerevel Therapeutics Holdings, Inc.; he formerly also held the role of Cerevel's Chief Executive Officer (CEO). He previously co-founded and served as Chairperson and CEO of Yumanity Therapeutics, Inc. Earlier, Dr. Coles was the President, CEO and Chairperson of Onyx Pharmaceuticals, Inc. and, President, CEO and member of the board of directors of NPS Pharmaceuticals, Inc. He currently serves on the board of directors of Regeneron Pharmaceuticals, Inc. Dr. Coles previously served as a director of CRISPR Therapeutics AG, Laboratory Corporation of America Holdings, Campus Crest Communities, Inc., and McKesson Corporation.

"I have devoted my career to advancing new therapies to treat some of the most challenging, intractable diseases and I am now excited to work with Prime Medicine in its mission to develop next-generation gene-editing therapeutics," said Dr. Coles. "Now is a particularly exciting moment, as Prime Medicine is initiating the first clinical trial of a Prime Editor and continuing to generate encouraging preclinical data across its pipeline. I look forward to working with the company in support of the ultimate goal of developing one-time, curative genetic therapies for diseases that collectively impact millions of people."

Anticipated Upcoming Milestones

Prime Medicine expects the following activities and next steps to drive Prime Medicine forward and support the Company's maturation into a clinical-stage company:

Hematology and Immunology:

- Announce initial clinical data from the Phase 1/2 clinical trial of PM359 in CGD in 2025.
- Advance Shielded Hematopoietic Stem Cell (HSC) and Immunotherapy Pairs (SCIP) technology, establish proof-of-concept in HSC and immunotherapy and identify first clinical program(s) with this approach in 2024.
- Advance differentiated CAR-T program, using PASSIGE technology, into lead optimization.

Liver:

- Continue to advance preclinical studies for three liver programs and initiate IND-enabling activities for at least one in 2024, leading to an IND and/or clinical trial application (CTA) in the second half of 2025 or first half of 2026.

Ocular:

- Nominate development candidate for *RHO*-RP program and initiate IND-enabling activities in 2024.

Neuromuscular:

- Continue to advance Friedreich's Ataxia and advance one other program into lead optimization in 2024.
- In large animal studies, establish adeno-associated virus (AAV) delivery platform and route of administration for neuromuscular programs in 2024.

First Quarter 2024 Financial Results

- **Research and Development (R&D) Expenses**: R&D expenses were $37.8 million for the three months ended March 31, 2024, as compared to $30.9 million for the three months ended March 31, 2023. The increase in R&D expenses was driven by expenses related to the advancement of the Company's pipeline and platform.
- **General and Administrative (G&A) Expenses**: G&A expenses were $11.2 million for the three months ended March 31, 2024, as compared to $9.2 million for the three months ended March 31, 2023.
- **Net Loss**: Net loss was $45.8 million for the three months ended March 31, 2024, as compared to $39.4 million for the three months ended March 31, 2023.
- **Cash Position**: As of March 31, 2024, cash, cash equivalents, investments and restricted cash were $224.2 million, as compared to $135.2 million as of December 31, 2023.

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 core areas of focus: hematology and immunology, liver, lung, ocular and neuromuscular. Across each core area, Prime Medicine's initial focus is on genetic diseases with a fast, direct path to treating patients, and those with high unmet need not currently addressable using other gene editing approaches. Over time, the Company intends to maximize Prime Editing's broad and versatile therapeutic potential to expand beyond the genetic diseases in its initial pipeline, potentially including 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 potential of PM359 to correct the causative mutation of CGD; the anticipated maturation into a clinical-stage company by bringing PM359 into clinical development in 2024 with initial clinical data expected in 2025; the initiation, timing, progress, and results of its research and development programs, preclinical studies and future clinical trials, and the release of data related thereto; the potential for Prime Editors to repair genetic mutations and offer curative genetic therapies for a wide spectrum of diseases; the potential of Prime Editors to reproducibly correct disease-causing genetic mutations across different tissues, organs and cell types, and the capacity of its PASSIGE technology to edit CAR-T cells for the treatment of certain cancers and immune 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; certain activities and next steps to support the Company's maturation into a clinical-stage company, including opening IND and/or CTA applications, clinical data expectations, establishing proof of concept, advancing programs into lead optimization, advancing preclinical studies and initiating IND-enabling activities, nominating development candidates, and establishing delivery platform and rout of administration; 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; the modularity of the Prime Editing platform 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, including rising interest rates, inflation, and adverse developments affecting the financial services industry. 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, 2024	December 31, 2023
Cash, cash equivalents, and investments	$ 210,723	$ 121,665
Total assets	$ 311,383	$ 193,851
Total liabilities	$ 67,617	$ 60,780
Total stockholders' equity	$ 243,766	$ 133,071

Condensed Consolidated Statement of Operations
(unaudited)

	Three Months Ended March 31,	
(in thousands, except share and per share amounts)	2024	2023
Collaboration revenue	$ 591	$ —
Operating expenses:		

Research and development	37,774	30,880
General and administrative	11,158	9,153
Total operating expenses	48,932	40,033
Loss from operations	(48,341)	(40,033)
Other income:		
Change in fair value of short-term investment — related party	1,166	(1,701)
Other income, net	1,548	2,135
Total other income, net	2,714	434
Net loss before income taxes	(45,627)	(39,599)
(Provision for) benefit from income taxes	(134)	202
Net loss attributable to common stockholders	$ (45,761)	$ (39,397)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.44)	$ (0.44)
Weighted-average common shares outstanding, basic and diluted	104,466,178	89,064,895