Prime Medicine Receives U.S. FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for PM359 for the Treatment of Chronic Granulomatous Disease (CGD)

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-- RMAT designation granted based on Phase 1/2 clinical data, including results previously published in The New England Journal of Medicine, demonstrating PM359's potential to address unmet need in p47 ^{phox}-deficient CGD --

-- Designation enables early and intensive FDA engagement, and provides eligibility for rolling and priority Biologics License Application review --

-- PM359 now holds RMAT, Fast Track, Orphan Drug, and Rare Pediatric Disease Designations --

CAMBRIDGE, Mass., June 22, 2026 (GLOBE NEWSWIRE) -- Prime Medicine, Inc. (Nasdaq: PRME), a biotechnology company committed to delivering a new class of differentiated one-time curative genetic therapies, today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to PM359, an investigational autologous Prime Edited hematopoietic stem cell therapy for the treatment of p47^{phox}-deficient chronic granulomatous disease (CGD). RMAT designation was granted based on Phase 1/2 clinical data, including data previously published in *The New England Journal of Medicine*, and will provide the benefits of intensive FDA guidance and expedited review through the program's development.

"FDA's decision to grant RMAT designation to PM359 reinforces the potential for this program to deliver a meaningful, disease-modifying impact in CGD, where patients face significant morbidity, lifelong complications, and limited treatment options," said Allan Reine, M.D., Chief Executive Officer of Prime Medicine. "The combination of RMAT, Fast Track, Orphan Drug, and Rare Pediatric Disease Designations underscores the seriousness of CGD and the need for transformative therapies that deliver durable benefit, while positioning us to engage with the FDA on the most efficient path to a Biologics License Application. We are working with urgency to advance PM359 toward potential approval, while building on this foundation across our broader pipeline, including programs in Wilson Disease and Alpha-1 Antitrypsin Deficiency."

RMAT designation was established under the 21st Century Cures Act to expedite the development and review of regenerative medicine therapies intended to treat serious or life-threatening diseases when preliminary clinical evidence indicates the therapy has the potential to address unmet medical need. RMAT designation provides all the benefits of Fast Track and Breakthrough Therapy Designations, including intensive FDA interaction, discussions on surrogate or intermediate endpoints that may support accelerated approval, and eligibility for rolling and priority review of a future Biologics License Application.

The initial clinical trial results were reported in the December 2025 publication in *The New England Journal of Medicine.* The data demonstrate that a single dose of PM359 drove rapid engraftment and a durable, clinically meaningful restoration of immune cell function in both treated patients, with neutrophil activity well above the level associated with clinical benefit and a safety profile consistent with busulfan-based conditioning alone. The findings support the potential of PM359 to deliver a one-time, disease-modifying treatment for patients with p47phox-deficient CGD.

About PM359

PM359 is an investigational autologous hematopoietic stem cell therapy designed to correct the delGT mutation in *NCF1*, the most prevalent disease-causing mutation in p47^{phox}-deficient CGD. PM359 is being evaluated in an ongoing Phase 1/2, multinational, first-in-human trial. In addition to RMAT designation, PM359 has received Fast Track, Orphan Drug, and Rare Pediatric Disease Designations from the FDA.

About Chronic Granulomatous Disease (CGD)

Chronic granulomatous disease (CGD) is a rare inherited hematologic disorder characterized by susceptibility to severe, difficult-to-treat infections and inflammatory and autoimmune complications. CGD is caused by mutations in any one of the subunits comprising the NADPH oxidase complex, which is required for phagocytic cells, in particular neutrophils, to destroy many invasive microorganisms. CGD causative mutations are estimated to occur in between one in 100,000 and one in 200,000 births in the United States, with the p47^{phox} form accounting for approximately 25% of cases. Current standard of care relies on lifelong antimicrobial prophylaxis and, in select cases, allogeneic hematopoietic stem cell transplantation, which carries significant morbidity and is not accessible to all patients. There are no approved gene editing therapies for CGD.

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: liver, lung, and immunology and oncology. 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 significance and potential benefits of RMAT designation; the significance and potential benefits of the combination of RMAT, Fast Track, Orphan Drug, and Rare Pediatric Disease designations; the ongoing regulatory interactions with the FDA based on the data from its Phase 1/2 trial of PM359 and the outcomes of any such interactions, including its plan to submit a Biologics License Application for PM359; the significance of the Phase 1/2 clinical data for PM359, including data published in *The New England Journal of Medicine*; the potential of PM359 to address the unmet medical need for patients with CGD; the potential of Prime Editing to correct the causative mutations of, and to cure, diseases, including CGD, Wilson Disease and Alpha-1 Antitrypsin Deficiency; 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.

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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