Prime Medicine Announces The New England Journal of Medicine Publication of PM359 Clinical Data for the Treatment of Chronic Granulomatous Disease

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CAMBRIDGE, Mass., Dec. 07, 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 announced the publication of Phase 1/2 clinical data with PM359, the Company's investigational autologous hematopoietic stem cell product for p47 ^{phox} chronic granulomatous disease (CGD) in the *New England Journal of Medicine (NEJM).* The data will also be presented in a poster session at the 67th American Society of Hematology (ASH) Annual Meeting, December 6-9, 2025 in Orlando, Florida.

The publication, titled "Prime Editing for p47 ^{phox}-Deficient Chronic Granulomatous Disease," reports initial data for two patients treated in the Phase 1/2 trial of PM359, which was designed to assess safety, biological activity and preliminary efficacy in adult and pediatric study participants. Both patients experienced rapid neutrophil and platelet engraftment, as well as durable restoration of NADPH oxidase activity and early clinical benefit, without any safety concerns. Together, these results provide the first-in-human demonstration of the safety and efficacy of Prime Editing, and support the potential for PM359 as a precise therapeutic strategy for CGD:

- Both patients enrolled in the study had a history of prior CGD-defining complications, including CGD-associated colitis (CAC), and skin and soft tissue infections, and both were maintained on long-term prophylactic therapy.
- Both patients experienced rapid neutrophil engraftment, achieving 69% and 83% dihydrorhodamine-positive (DHR+) neutrophils by Day 30, respectively, far in excess of the 20% projected minimum threshold for clinical benefit. DHR activity remained stable over time in both patients, suggesting that gene correction occurred in the long-term repopulating hematopoietic stem cells (HSCs) of the bone marrow.
- Both patients remain free of new CGD-related complications or significant intercurrent illnesses post-infusion; additionally, Patient 1 stopped his mesalamine treatment and has not experienced a flare of CAC, and Patient 2's levels of fecal calprotectin have decreased substantially, and his chronic CAC symptoms have abated.
- No clinically significant adverse events attributable to PM359 occurred in either patient, and all observed toxicities were consistent with busulfan-based conditioning.

"Publication of these first-in-human data highlights Prime Editing's promise as a next-generation therapeutic platform, which is capable of delivering meaningful benefits to patients and which can be manufactured and delivered at clinical scale," said Mohammed Asmal, M.D., Ph.D., Chief Medical Officer of Prime Medicine. "Beyond demonstrating early clinical efficacy, these results offer important insights into Prime Editing's safety profile and potential advantages over other gene editing technologies. As described in the *NEJM* publication, we observed high recovery rates of viable corrected cells after a single mobilization cycle, as well as the rapid reconstitution of the hematopoietic system after infusion. Both support our belief that the mechanism of Prime Editing, which does not induce double-strand breaks, may be better tolerated by HSCs and other cell types – and therefore safer for patients – than other approaches."

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.

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 of data from its Phase 1/2 trial of PM359; the potential for PM359 to be a precise and safe therapeutic strategy for CGD;; the safety and efficacy of Prime Editing, including in comparison to other approaches; the potential of Prime Editing to correct the causative mutations of, and to cure, diseases; 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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