



Prime Medicine Announces First Patient Dosed in Global Phase 1/2 Clinical Trial of PM577a for H1069Q-Mutated Wilson Disease

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-- Global, first-in-human study underway designed to evaluate safety, tolerability, and initial clinical efficacy of PM577a --

-- PM577a is designed as a one-time therapy to correct the H1069Q mutation in ATP7B, the most prevalent Wilson disease-causing mutation in North America and Europe --

-- PM577 granted Rare Pediatric Disease Designation by the U.S. FDA --

-- Initial clinical data expected in 2027 --

CAMBRIDGE, Mass., Oct. 05, 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 first patient has been dosed in the Company's global Phase 1/2 clinical trial of PM577a, an investigational *in vivo* Prime Editor for H1069Q-mutated Wilson disease. The milestone marks the initiation of the first clinical study of an *in vivo* Prime Editing therapy from Prime Medicine, as well as the first clinical evaluation of the company's liver franchise. The company also announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) designation to PM577 for Wilson disease.

"Dosing the first patient with PM577a is a meaningful step toward our goal of delivering a transformative, one-time treatment for people living with Wilson disease," said Allan Reine, M.D., Chief Executive Officer of Prime Medicine. "We've seen strong interest from patients and physicians that, together with our patient identification and prescreening efforts, positions us well for efficient enrollment. More broadly, PM577a reflects the modularity of our platform. While this initial candidate is designed to correct H1069Q, the most prevalent disease-causing mutation in the United States and Europe, the same shared LNP delivery approach provides a path to expand into additional Wilson disease patient populations globally, beginning with our follow-on candidate designed to correct the R778L mutation, the most common mutation in East Asian populations."

"Wilson disease is a serious, progressive genetic disorder with no approved curative therapy. Current pharmacologic treatments require lifelong management and can be limited by tolerability and adherence challenges," said Mohammed Asmal, M.D., Ph.D., Chief Medical Officer of Prime Medicine. "PM577a is designed to correct the H1069Q mutation at its genetic root through a single administration, with the potential to offer patients a one-time curative treatment, ideally before irreversible liver and neurological damage occur. Because Wilson disease frequently presents during childhood and adolescence, we are particularly encouraged by the FDA's Rare Pediatric Disease designation for PM577, which underscores the significant unmet need for therapies capable of altering the course of disease early in life."

Phase 1/2 Clinical Trial

The Phase 1/2 clinical trial is an open-label, global, first-in-human study designed to evaluate the safety, tolerability, and preliminary clinical efficacy of ascending doses of PM577a in adults and adolescents with Wilson disease containing at least one p.H1069Q allele in the ATP7B gene. The study will initially enroll adults who are clinically stable on standard-of-care therapy. Initial efficacy measures may include copper efflux by ⁶⁴Cu PET, serum ceruloplasmin, non-ceruloplasmin-bound copper, and 24-hour urinary copper excretion, among others. The trial will also evaluate discontinuation of baseline standard-of-care therapies.

Additional information about the trial is available at ClinicalTrials.gov under identifier [NCT07748403](https://clinicaltrials.gov/ct2/show/study/NCT07748403).

Rare Pediatric Disease Designation

FDA grants Rare Pediatric Disease designation to investigational therapies intended to treat serious or life-threatening diseases that primarily affect individuals from birth through 18 years of age and affect fewer than 200,000 people in the United States. Under the FDA's Rare Pediatric Disease Priority Review Voucher (PRV) program, a sponsor that receives an approval for a New Drug Application (NDA) or Biologics License Application (BLA) for a rare pediatric disease may qualify for a voucher that can be redeemed to receive a priority review for a subsequent marketing application, or it can be sold or transferred to another company.

About PM577

PM577 is a family of lipid nanoparticle-formulated Prime Editing products designed to correct pathogenic ATP7B mutations in hepatocytes through a single intravenous infusion. Prime Medicine's initial candidate, PM577a, targets the ATP7B H1069Q mutation, the most prevalent Wilson disease-causing allele in North America and Europe. PM577a has been granted Rare Pediatric Disease designation by the U.S. FDA for the treatment of Wilson disease. The Company intends to develop additional products to address the majority of pathogenic variants on a global basis. A follow-on candidate is in preclinical development targeting R778L, the most common mutant allele in East Asian populations.

About Wilson Disease

Wilson disease is a rare, serious and progressive genetic disorder of hepatic copper transport caused by loss-of-function mutations in the ATP7B gene. Impaired biliary excretion drives progressive copper accumulation in the liver, followed by multiorgan involvement, including the brain, kidneys and cornea. Current pharmacologic therapies, including copper chelators and zinc salts, are non-curative, require lifelong daily treatment and carry tolerability and adherence challenges. Liver transplantation remains the only curative option but is constrained by organ availability and carries substantial procedural risk. PM577a is designed to offer patients with H1069Q-mutated Wilson disease a one-time therapy that corrects the disease at its genetic root.

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 its 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. 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 potential of PM577a to correct the causative mutations of, and to cure, H1069Q-mutated Wilson disease; the global Phase 1/2 clinical trial of PM577a, including the efficiency of patient enrollment, trial design, and timing of initial clinical data in 2027; the continued development and advancement of the Company's Wilson disease program, including the development of follow-on candidates; the significance and potential benefits of the RPD designation for PM577a; the modularity of the Prime Editing platform and its LNP and the benefits thereof; 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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