# Prime Medicine Announces U.S. FDA Clearance of Investigational New Drug Application for PM577a in H1069Q-mutated Wilson Disease

July 23, 2026

*-- FDA clearance of the IND, together with the previously cleared CTA, establishes a global Phase 1/2 clinical program for PM577a --*

*-- PM577a targets the H1069Q mutation in the ATP7B gene, the most prevalent WD-causing allele in North America and Europe --*

*-- Initial clinical data expected in 2027 --*

CAMBRIDGE, Mass., July 23, 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 cleared the Company's Investigational New Drug (IND) application for PM577a, an investigational *in vivo* Prime Editor for Wilson disease (WD). With the IND cleared, PM577a may proceed to clinical study in the United States. Together with the Company's previously announced New Zealand Clinical Trial Application (CTA) clearance, the IND clearance establishes a global Phase 1/2 program and opens participation to patients in the United States, where H1069Q is the single most common pathogenic variant causing WD.

"Wilson disease is a serious, progressive disorder with no approved curative option, and today's standard of care requires lifelong therapy burdened by significant side effects and low adherence," said Allan Reine, M.D., Chief Executive Officer of Prime Medicine. "With PM577a, we have the potential to offer a one-time therapy that corrects the disease at its genetic root. This clearance, alongside our recent New Zealand CTA, establishes a global Phase 1/2 program that reflects Prime's confidence in our *in vivo* Prime Editing approach. We expect to initiate the Phase 1/2 trial in the second half of 2026 with initial clinical data anticipated in 2027, and we are committed to advancing this program with the rigor patients deserve."

**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, biological activity, and efficacy of ascending doses of PM577a in adults and adolescents with WD. The study will initially enroll adults who are clinically stable on standard-of-care therapy. Biological activity and efficacy assessments may include copper efflux by $^{64}$Cu PET, serum ceruloplasmin, non-ceruloplasmin bound copper, 24-hour urinary copper excretion, and hepatic copper by biopsy.

**About PM577**

PM577 is a family of LNP-formulated Prime Editing products designed to correct pathogenic *ATP7B* mutations in hepatocytes via a single intravenous infusion. Prime Medicine's initial candidate, PM577a, targets the *ATP7B* H1069Q allele, which accounts for approximately 30–50% of WD-associated variants in the United States and Europe. The Company intends to develop additional products to address the majority of pathogenic variants on a global basis. Currently, a follow-on candidate is in pre-clinical development targeting R778L, the most common mutant allele in East Asian populations.

**About Wilson Disease**

Wilson disease is a rare, autosomal recessive disorder of hepatic copper transport caused by loss-of-function mutations in the *ATP7B* gene, affecting an estimated 1 in 30,000 individuals globally. Impaired biliary excretion drives progressive copper accumulation in the liver, followed by multi-organ involvement including the brain, kidneys, and cornea. Clinical manifestations range from asymptomatic hepatomegaly to decompensated cirrhosis and acute liver failure; neuropsychiatric complications affect approximately two-thirds of patients. Current pharmacologic therapies, copper chelators and zinc salts, are non-curative, require lifelong daily dosing under strict dietary conditions, carry tolerability challenges, and are associated with high non-adherence rates. Liver transplantation remains the sole curative option but is constrained by organ availability and carries substantial procedural risk.

**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. 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 PM577a to correct the causative mutations of, and to cure, WD; the global Phase 1/2 clinical trial of PM577a, including the anticipated timing of trial initiation in the second half of 2026 with initial clinical data anticipated in 2027; the continued development and advancement of the Company's WD program, including the development of follow-on candidates; 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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