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Monopar Therapeutics Receives FDA Rare Pediatric Disease Designation for ALXN1840 for the Treatment of Wilson Disease

WILMETTE, Ill., June 30, 2026 (GLOBE NEWSWIRE) -- Monopar Therapeutics Inc. ("Monopar" or the "Company") (Nasdaq: MNPR), a clinical-stage biopharmaceutical company developing innovative treatments for patients with unmet medical needs, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) designation to ALXN1840 (tiomolibdate choline, TMC), the Company's late-stage candidate for the treatment of Wilson disease.

The FDA grants RPD designation to therapies intended to treat serious or life-threatening diseases that primarily affect children from birth to 18 years of age. The designation provides the Company with the potential at the time of NDA approval to receive a pediatric Priority Review Voucher (PRV), which can be used to obtain priority review of a subsequent marketing application or sold or transferred to another sponsor. Priority review can reduce the FDA's target review time by several months.

"Receiving Rare Pediatric Disease designation for ALXN1840 underscores the serious impact that Wilson disease has on patients and reinforces the urgency of bringing forward a new treatment option," said Chandler Robinson, M.D., Chief Executive Officer of Monopar.

About Wilson Disease

Wilson disease is a rare genetic disorder that affects approximately 1 in 30,000 people worldwide. It is caused by mutations in the ATP7B gene, which impairs the body's ability to excrete copper. It is characterized by toxic accumulation of copper in the liver, brain, and other organs, leading to progressive and potentially fatal outcomes if untreated.

About ALXN1840

ALXN1840 (tiomolibdate choline, TMC) is a novel first-in-class albumin tripartite complex (ATC) activator under investigation for the treatment of Wilson disease. ALXN1840 rapidly mobilizes and tightly sequesters excess copper in ATCs, suppressing its redox reactivity, limiting oxidative damage, and blocking transport across the blood–brain barrier. Clinical data demonstrate that ALXN1840 improves copper balance by increasing fecal copper excretion. In the Phase 3 pivotal trial, ALXN1840 met the primary endpoint by demonstrating rapid and sustained copper mobilization significantly greater than standard of care over 48 weeks in both previously treated and untreated patients. Durable clinical improvement and a favorable safety and tolerability profile were observed across 645 patient-years of follow-up in 266 patients. ALXN1840 is an oral tablet with a once-a-day dosing regimen.

About Monopar Therapeutics Inc.

Monopar Therapeutics is a clinical-stage biopharmaceutical company with late-stage ALXN1840 for Wilson disease, and radiopharmaceutical programs including MNPR-101-Zr (Phase 1) for imaging advanced cancers along with MNPR-101-Lu (Phase 1a) and MNPR-101-Ac (late preclinical) for the treatment of advanced cancers. For more information, visit: www.monopartx.com.

Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. An example of a forward-looking statement includes the statement concerning: that the designation (RPD) provides the Company with the potential at the time of (ALXN1840) NDA approval to receive a pediatric Priority Review Voucher (PRV). The forward-looking statements involve risks and uncertainties including, but not limited to: uncertainties related to whether the ALXN1840 marketing application will receive marketing approval and, if approved, whether Monopar will be awarded a Priority Review Voucher; whether, if awarded, the Priority Review Voucher can be used to obtain priority review of a subsequent marketing application or sold or transferred to another sponsor; the continued authorization and availability of the Rare Pediatric Disease Priority Review Voucher program; uncertainties related to the regulatory process that Monopar intends to initiate related to ALXN1840 and the outcome thereof; the rate of market acceptance and competitiveness in terms of pricing, efficacy and safety, of any products for which Monopar receives marketing approval, and Monopar's ability to competitively market any such products as compared to larger pharmaceutical firms; Monopar's ability to raise sufficient funds in order for the Company to support continued preclinical, clinical, regulatory, precommercial and commercial development of its programs and to make contractual milestone payments, as well as its ability to further raise additional funds in the future to support any existing or future product candidate programs through completion of clinical trials, the approval processes and, if applicable, commercialization; and the significant general risks and uncertainties surrounding the research, development, regulatory approval, and commercialization of imaging agents and therapeutics. Actual results may differ materially from those expressed or implied by such forward-looking statements. Risks are described more fully in Monopar's filings with the Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made. Monopar undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made. Any forward-looking statements contained in this press release represent Monopar's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date.

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