April 19, 2026

Monopar Therapeutics

Monopar Presents Phase 3 Data Showing Greater Neurologic Benefit with ALXN1840 vs SoC in Wilson Disease Patients with Neurologic Symptoms at AAN 2026

WILMETTE, Ill., April 19, 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, announced new analyses from the randomized controlled Phase 3 FoCus trial of ALXN1840 (tiomolibdate choline, TMC) showing greater neurologic benefit versus standard of care (SoC) in Wilson disease patients with neurologic symptoms at baseline. The data will be presented today at the American Academy of Neurology (AAN) Annual Meeting 2026, taking place April 18-22, 2026.

In a late-breaker oral and poster presentation titled "Greater clinical benefit with tiomolibdate choline versus standard-of-care in neurologic Wilson disease patients in the Phase 3 FoCus Trial," Dr. Peter Hedera, MD, PhD, Department of Neurology, University of Louisville School of Medicine, will present results showing that ALXN1840 provided greater neurologic improvement and significantly less worsening than standard of care through Week 48, with durable neurologic benefit observed over multiple years of treatment.

- In the randomized FoCus trial, analysis of patients with neurologic symptoms at baseline (TMC: n=77; SoC: n=35) demonstrated that treatment with ALXN1840 resulted in both higher rates of improvement and lower rates of worsening, addressing a critical unmet need in the neurologic management of Wilson disease.
    - Clinically meaningful neurologic worsening at Week 48 was observed in 25% of patients treated with standard of care vs 9% of ALXN1840-treated patients (p=0.038)
    - Clinically meaningful neurologic improvement at Week 48 was observed in 45% of ALXN1840-treated patients vs 32% on standard of care
    - CGI-S improvement from baseline to Week 48 was greater with ALXN1840 vs standard of care (61% vs 17%; p=0.008)
    - CGI-I improvement at Week 48 was greater with ALXN1840 vs standard of care (47% vs 19%; p=0.003)
- Durable neurologic benefit in the ALXN1840-treated group continued to increase during long-term follow-up on treatment and was sustained over approximately 3 years

- Neurologic benefit was consistent across both treatment-naïve and treatment-experienced patients with neurologic symptoms at baseline, supporting ALXN1840's potential as a novel treatment option for Wilson disease

- ALXN1840 has demonstrated a well-characterized and favorable safety profile across Phase 2 and Phase 3 studies (266 patients; median 2.58 years on treatment; max >8 years), with drug-related serious adverse events (SAEs) limited to 4.9% of patients — including neurologic SAEs in < 1% — and no treatment-related deaths

"These data highlight the potential of ALXN1840 to meaningfully change the treatment landscape for Wilson disease patients with neurologic symptoms by delivering both improved clinical outcomes and a lower likelihood of neurologic deterioration compared to standard of care," said Dr. Hedera.

The presentation and poster are available on Monopar's website.

These findings support the continued advancement of ALXN1840 toward the planned New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) in mid-2026.

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 demonstrated rapid and sustained copper mobilization (primary endpoint) that was 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.

About Monopar Therapeutics Inc.

Monopar Therapeutics is a clinical-stage biopharmaceutical company with late-stage ALXN1840 for Wilson disease, and radiopharmaceutical programs including Phase 1-stage MNPR-101-Zr for imaging advanced cancers, and Phase 1a-stage MNPR-101-Lu and late preclinical-stage MNPR-101-Ac225 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. Examples of these forward-looking statements include statements concerning: that the neurologic benefit supports ALXN1840’s potential as a novel treatment option for Wilson disease; that ALXN1840 has the potential to meaningfully change the treatment landscape for Wilson disease patients with neurologic symptoms by delivering both improved clinical outcomes and a lower likelihood of neurologic deterioration compared to standard of care; that these findings support the continued advancement of ALXN1840 toward the planned submission of an NDA to the FDA in mid-2026. The forward-looking statements involve risks and uncertainties including, but not limited to: 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, pre-commercial 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 therapeutics and imaging agents. 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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