October 24, 2024

Monopar Therapeutics

Monopar Announces Agreement with Alexion, AstraZeneca Rare Disease For Late-Stage Wilson Disease Drug Candidate

WILMETTE, Ill., Oct. 24, 2024 (GLOBE NEWSWIRE) -- Monopar Therapeutics Inc. (Nasdaq: MNPR), a clinical-stage biotechnology company focused on developing innovative treatments for patients with unmet medical needs, today announced that it has entered into an agreement with Alexion, AstraZeneca Rare Disease for an exclusive worldwide license to ALXN-1840 (bis-choline tetrathiomolybdate), a drug candidate for Wilson disease that Alexion has progressed through a Phase 3 clinical trial that met its primary endpoint. Monopar will be responsible for all future global development and commercialization activities.

Chandler D. Robinson, MD, Co-Founder and Chief Executive Officer of Monopar previously researched tetrathiomolybdate at the laboratory bench, published his results in *Science*, and helped launch a company around what became known as ALXN-1840. The focus was on Wilson disease. Dr. Robinson came to know the Wilson disease community well through his efforts. At the Wilson Disease Association's request, he delivered the keynote address at their 2013 Annual Conference celebrating their 30th anniversary, sharing his experience helping advance ALXN-1840 from the laboratory bench to patients. In 2023, Alexion terminated the ALXN-1840 program in Wilson disease based on review of results from Phase II mechanistic trials and discussions with regulatory authorities.

Chris Starr, PhD, Co-Founder and Executive Chair of Monopar said, "Upon the 2023 announcement, Chandler, due to his long history with the program and the continued high level of unmet medical need, was contacted by Wilson disease patients, executives and board members of the Wilson Disease Association, as well as physicians regarding the potential for Monopar to obtain rights to ALXN-1840. Due in no small measure to the testimonials Chandler received from clinical trial patients who reported benefit while on the drug for years, we decided that this was an opportunity Monopar needed to pursue, and it fits well with my rare disease drug development and commercialization background as well as Chandler's background." Dr. Starr also previously co-founded the orphan drug companies BioMarin and Raptor Pharma (acquired by Horizon Pharma, now a part of Amgen).

Dr. Robinson commented, "Alexion has generated a substantial clinical data package on ALXN-1840, including a completed Pivotal Phase 3 clinical trial. The medical data gathered from Alexion's clinical trials furthers our understanding of Wilson disease and stands to benefit this community."

Under the terms of the license agreement, Monopar will pay Alexion an upfront in the form of a cash payment and equity in Monopar. Future payments are based on tiered royalties on net sales and pre-determined regulatory and sales milestones.

"We are excited to have Alexion and AstraZeneca as partners of Monopar as, in addition to

Alexion's work in Wilson disease, AstraZeneca maintains a significant presence in the radiopharma field, in which Monopar is committed to continue growing as Monopar recently announced positive human clinical data with our novel radiopharma program (link)," stated Andrew Cittadine, Chief Operating Officer of Monopar.

About Wilson Disease

Wilson disease is a rare and progressive genetic condition in which the body's pathway for removing excess copper is compromised. [1] It affects one in 30,000 live births in the US.[1] Over time this results in the build-up of toxic copper levels in the liver, brain, and other organs, leading to damage that greatly impacts a patient's life. [1] Patients can develop a wide range of symptoms, including liver disease and/or psychiatric or neurological symptoms, such as personality changes, tremors and difficulty walking, swallowing or talking. [1] In some cases, the damage and loss of function may be irreversible. [1,2,3]

About ALXN-1840

ALXN-1840 (bis-choline tetrathiomolybdate) is an investigational once-daily, oral medicine in development for the treatment of Wilson disease. This novel molecule is designed to selectively and tightly bind and remove copper from the body's tissues and blood. ALXN-1840 has been granted Orphan Drug Designation in the United States and orphan designation in the European Union for Wilson disease.

About the Phase 3 "FoCus" Clinical Trial

The FoCus trial was a pivotal Phase 3, randomized, rater-blinded, multi-center clinical trial designed to evaluate the efficacy and safety of ALXN-1840 versus standard-of-care (SoC) in patients with Wilson disease aged 12 years and older. The primary endpoint assessed copper mobilization over 48 weeks, defined as daily mean AUEC (Area Under the Effect Curve) for dNCC (directly measured non-ceruloplasmin-bound copper). In the trial, 214 patients were enrolled in one of two cohorts on a 3:1 basis (treatment-experienced:treatment-naïve). Each cohort was then randomized 2:1 (ALXN1840:SoC). The first cohort enrolled 161 patients who received SoC (chelation therapy with penicillamine or trientine, zinc therapy or a combination of both chelation and zinc therapy) for more than 28 days and the second cohort enrolled 53 patients who were treatment-naïve or had received SoC for 28 days or less. The FoCus trial met its primary endpoint demonstrating three-times greater copper mobilization from tissues compared to the SoC arm (Least Square Mean Difference [LSM Diff] 2.18 µmol/L; $p < 0.0001$), including in patients who had been treated previously for an average of 10 years. In the trial, people taking ALXN-1840 experienced rapid copper mobilization, with a response at four weeks and sustained through the 48 weeks. ALXN-1840 was generally well-tolerated with most reported adverse events considered mild to moderate, and no neurological worsening upon initiation of treatment was observed. In the ALXN-1840 treatment group, the most frequently reported adverse event was a reversible increase in transaminase levels.

About Monopar Therapeutics Inc.

Monopar Therapeutics is a clinical-stage biotechnology company with late-stage ALXN-1840 for Wilson disease, and radiopharma 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: the medical data gathered from Alexion’s clinical trials furthers our understanding of Wilson disease and stands to benefit this community; ALXN-1840 was generally well-tolerated with most reported adverse events considered mild to moderate, and no neurological worsening upon initiation of treatment; and the most frequently reported adverse event for the ALXN-1840 treatment group was a reversible increase in transaminase levels. The forward-looking statements involve risks and uncertainties including, but not limited to: our near term ability to raise sufficient funds in order for us to support continued clinical, regulatory and commercial development of our programs and to make contractual upfront and future milestone payments, as well as our 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; uncertainties related to the regulatory discussions we intend to initiate related to ALXN-1840 and the outcome thereof; the rate of market acceptance and competitiveness in terms of pricing, efficacy and safety, of any products for which we receive marketing approval, and our ability to competitively market any such products as compared to larger pharmaceutical firms; 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.

CONTACT:

Monopar Therapeutics Inc.
Investor Relations

Karthik Radhakrishnan
Chief Financial Officer
karthik@monopartx.com

Follow Monopar on social media for updates:
Twitter: @MonoparTx LinkedIn: Monopar Therapeutics

References:

1. Patil, M., et al. (2013) J Clin Exp Hepatol, 3, 321-336.

2. Roberts, E.A., Schilsky, M.L. American Association for the Study of Liver D. (2008). Diagnosis and treatment of Wilson disease: An update. Hepatology, 47(6), 2089-2111.

3. European Association for the Study of the Liver. (2012). EASL clinical practice guidelines: Wilson's disease. J Hepatol, 56(3), 671-685.

Monopar Therapeutics

Source: Monopar Therapeutics Inc.