PRESS RELEASE

Stockholm April 14, 2016

Wilson Therapeutics presents data on Decuprate™ (WTX101) from ongoing Phase 2 study at The International Liver Congress™ 2016

Wilson Therapeutics AB, announces that data from the company's ongoing Phase 2 clinical trial for Decuprate™ (bis-choline tetrathiomolybdate; WTX101) in Wilson Disease will be presented today at The International Liver Congress™ 2016, taking place in Barcelona, Spain April 13-17. The oral presentation of the data will be given by Prof. Karl Heinz Weiss during the General Hepatology session on April 14.

WTX101-201 is a Phase 2 clinical trial evaluating the efficacy and safety of Decuprate™ dosed once daily in up to 30 newly-diagnosed patients with Wilson Disease aged 18 years and older. The study is being conducted at 10 sites in the U.S. and Europe, and will follow patients on Decuprate™ for 24 weeks. Patients completing the 24 weeks can elect to stay on Decuprate™ in an extension phase of the study.

As of April 1st, 20 patients have been enrolled in the study and five patients have reached the end of the 24-week treatment period. All five patients have chosen to enter the extension phase. The patients recruited thus far have demonstrated various degrees of hepatic impairment at the time of enrollment and with few exceptions all patients enrolled have demonstrated neurological symptoms at baseline.

Preliminary data show that during the study, liver status measured using the Revised King's Score (Modified Nazer Score) has improved or remained stable in 14 out of 15 patients at the last observation compared to baseline. Improvement of neurological disease was observed early after the start of treatment in most patients. Neurological impairment was measured by a neurology assessment specific for Wilson Disease (UWDRS part 3). Improvements in average UWDRS part 3 scores were seen in the treatment group after 12 weeks and the score continued to improve with time on treatment. Treatment with Decuprate™ has generally been well tolerated with most reported adverse events being mild (grade 1) to moderate (grade 2). Importantly, no paradoxical neurological worsening was observed upon treatment initiation. Elevated baseline free copper on average has normalized within approximately three months, with further reductions in the relatively few patients followed longer on treatment.

"We are very excited to be involved in the WTX101-201 study, the first clinical trial to evaluate a new therapy for Wilson Disease for close to two decades," says Karl Heinz Weiss, MD, Professor, University of Heidelberg. "Although the study is ongoing, the preliminary data is very encouraging as they seem to support the promise that Decuprate™ could offer a treatment option with a significantly simplified dosing regimen combined with a lowered risk of neurological worsening in patients with Wilson Disease."

Jonas Hansson, Chief Executive Officer of Wilson Therapeutics continues: "We are very pleased with the progression of this study. Although treatments for Wilson Disease have been available since the 1950s, there is a clear need for novel therapies that can address the significant unmet medical needs that still remain. Acknowledging that the study is ongoing and the data are still preliminary, they do indicate that Decuprate™ has a strong effect with a high response rate and a benign safety profile. Combined with once daily dosing, this could lead to significant improvements for patients living with Wilson Disease."

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About Decuprate™ (bis-choline tetrathiomolybdate; WTX101)
Decuprate™ is a first-in class de-coppering agent dosed once-daily with a novel and unique mechanism of action. With a strong and selective binding to copper, Decuprate™ has the potential to expand the body's own buffer capacity for copper, which is filled up in patients with Wilson Disease. The active ingredient of Decuprate™, tetrathiomolybdate, has been tested in several clinical studies in Wilson Disease patients and the data from these studies, as well as preliminary data from the Company's ongoing Phase II study, suggest that tetrathiomolybdate can rapidly lower and control the toxic free copper levels and improve clinical symptoms in patients with Wilson Disease. The data also suggest that Decuprate™ is well-tolerated with the potential for a reduced risk of neurological worsening after initiation of therapy compared to existing therapies. The once-daily dosing regimen is expected to translate into improved patient compliance in Wilson Disease patients with fewer treatment failures as a result.

About Wilson Disease
Wilson Disease is a rare genetic disease that causes serious copper poisoning. The genetic defect causes excessive copper accumulation, primarily in the liver and/or the central nervous system and the disease results in life-threatening damage to the liver and brain if left untreated. Wilson Disease affects approximately one in every 30,000 people worldwide, corresponding to a prevalence of approximately 10,000 patients in the US and 15,000 patients in the EU.

About Wilson Therapeutics
Wilson Therapeutics is a biopharmaceutical company, based in Stockholm, Sweden, that develops novel therapies for patients with rare diseases. Wilson Therapeutics' lead product, Decuprate™, is initially developed as a new treatment for Wilson Disease and is currently being evaluated in a Phase II clinical study in Wilson Disease patients. Investors in Wilson Therapeutics include Abingworth, HealthCap, MVM Life Science Partners and Neomed.

Visit www.wilsontherapeutics.com for more information.

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