PRESS RELEASE

Stockholm May 30, 2016

Wilson Therapeutics presents encouraging clinical data on Decuprate® at the Congress of the European Academy of Neurology

Wilson Therapeutics AB (publ), announces that preliminary data from the company's ongoing Phase II clinical trial for Decuprate® (bis-choline tetrathiomolybdate; WTX101) in Wilson Disease were presented at the 2nd Congress of the European Academy of Neurology, taking place in Copenhagen, Denmark, May 28-31. The oral presentation of the data was given by Prof. Anna Czlonkowska during the Movement Disorders 2 session on May 29.

WTX101-201 is a Phase II clinical trial evaluating the efficacy and safety of Decuprate® monotherapy dosed once daily in 28 newly-diagnosed patients with Wilson Disease, aged 18 years and older, who are previously untreated or have received a standard of care agent for up to two years. The study is being conducted at 11 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.

The last patient was enrolled in the trial on May 18 and as of May 29, six patients have reached the end of the 24-week treatment period. All six patients have elected to continue Decuprate® treatment in the extension phase. The patients recruited had various degrees of hepatic impairment at the time of enrollment and the majority of enrolled patients also had neurological symptoms at study start.

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 20 out of 20 patients at the last observation compared to baseline. Neurological impairment was measured by a neurology scale specific for Wilson Disease (UWDRS 3). The neurological status has improved or remained stable in 18 out of the 19 evaluable patients when comparing the last UWDRS 3 assessment with baseline. Treatment with Decuprate® has generally been well tolerated with most reported adverse events being mild (grade 1) to moderate (grade 2). Importantly, no drug-induced neurological worsening was observed upon treatment initiation. Levels of free copper were reduced over the duration of treatment.

"I have cared for more than 800 Wilson Disease patients over the years and I would say that the preliminary results in this study clearly indicate that Decuprate® can become a very important treatment option for Wilson Disease," says Anna Czlonkowska, MD, Professor at the Second Department of Neurology at the Institute of Psychiatry and Neurology in Warsaw, and at the Department of Pharmacology, Medical University of Warsaw.

Carl Bjartmar, MD, PhD, Chief Medical Officer of Wilson Therapeutics, continues: "In clinical practice, treatment of Wilson Disease patients with neurological symptoms is a particular concern as approximately 25% of these patients experience a significant drug-induced worsening of neurological symptoms upon initiation of treatment. Long term these patients also have remaining symptoms in approximately 50% of the cases despite years of treatment. The trend in our ongoing trial is therefore very encouraging with a 95% neurological response rate and no cases of drug-induced worsening so far."

Jonas Hansson, Chief Executive Officer of Wilson Therapeutics adds: "We are very pleased with the progression of this study. The study is still ongoing so the data are preliminary but they do indicate that Decuprate® may address the unmet needs in Wilson Disease with potentially a

strong efficacy and benign safety profile. Combined with once daily dosing, this could lead to significant improvements for patients living with Wilson Disease so it will be exciting to follow the study until completion late this year or early next year."

About Decuprate® (bis-choline tetrathiomolybdate; WTX101)
Decuprate® is a first-in class de-coppering agent with a novel and unique mechanism of action. With its strong and selective binding to copper, Decuprate® has the potential to expand the body's own liver buffering capacity for copper, which is saturated 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 Decuprate® can rapidly lower and control toxic free copper levels and improve clinical symptoms in these patients. 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. Decuprate® is expected to have a once-daily dosing regimen which may potentially translate into improved compliance in Wilson Disease patients, leading to fewer treatment failures and ultimately improved outcomes as a result. Decuprate® has received orphan drug designation for the treatment of Wilson disease in the US and EU.

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. The therapies currently being used in Wilson Disease were introduced in the 1950's and 60's and since then there have been no new treatment options developed for patients with this disease.

About Wilson Therapeutics
Wilson Therapeutics is a biopharmaceutical company, based in Stockholm, Sweden, that develops novel therapies for patients with rare diseases such as Wilson Disease. Wilson Therapeutics' lead product, Decuprate®, is initially being developed as a novel treatment for Wilson Disease and is currently being evaluated in a Phase II clinical study. Wilson Therapeutics is listed in the Mid Cap segment on Nasdaq Stockholm with the stock ticker WTX.

Visit www.wilsontherapeutics.com for more information.

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The information in the press release is such that Wilson Therapeutics is required to disclose publicly in accordance with the Swedish Securities and Clearing Operations Act and/or the Swedish Financial Instruments Trading Act. The information was submitted for publication on May 30, 2016 at 8:00 a.m.