PRESS RELEASE
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Promising preliminary long-term data for WTX101 in Wilson Disease presented at EASL Annual Meeting

Wilson Therapeutics AB (publ), announced today that preliminary long-term efficacy and safety data from the ongoing extension phase of the company's Phase 2 trial of WTX101 (bis-choline tetrathiomolybdate), an investigational first-in-class copper-protein-binding agent under investigation as a novel therapy for Wilson Disease, will be presented today during a Late Breaker poster session at The International Liver Congress™ 2018, the Annual Meeting of the European Association for the Study of the Liver (EASL), in Paris.

Carl Bjartmar, Chief Medical Officer, Wilson Therapeutics AB commented: "Data from the Phase 2 study have previously demonstrated that once-daily treatment with WTX101 significantly reduces free copper levels in patients with Wilson Disease. The 72-week extension data indicate that the control of copper is maintained long term. Importantly, this translates into further improvements or stabilization of liver status, as well as continuous improvements in neurological status and patient reported disability over the whole 72-week study period. This is very promising, especially for patients with persistent neurological problems despite years of therapy with currently available drugs. WTX101 also appears to be generally well tolerated with few adverse events reported during the extension period. All in all, these long-term data are highly encouraging and we look forward to further evaluating the profile of WTX101 in our ongoing Phase 3 FOCuS study".

All 22 patients who completed the 24-week open-label, single-arm, Phase 2 study opted to continue once-daily WTX101 treatment in the extension phase. 20 patients completed week 72; one patient discontinued treatment due to her wish to conceive and one patient was unable to comply with study procedures due to a progressive disease course despite ongoing treatment.

As previously announced when the abstract was published on March 28, 2018, the initial improvements in free copper levels, hepatic status, neurological status and disability observed at week 24 and 48 were preserved or further improved after once-daily treatment for 72 weeks with WTX101.

WTX101 was generally well tolerated over 72 weeks of treatment. Reversible ALT elevations requiring dose adjustment (observed in about one-third of patients in the core period of the Phase 2 trial) have not been observed in the extension phase. No cases of drug-induced neurological worsening have been observed during the study.

Late Breaker poster presentation summary details:

Abstract identifier: LBP-001

Title: "Long-term efficacy and safety of WTX101 in Wilson disease: Data from an ongoing extension of a phase 2 study (WTX101-201)"

Presenter: Karl Heinz Weiss, MD, Professor, University of Heidelberg, Germany

Session: Late Breaker Posters

Date: 12 April 2018

Time: from 09:00 CET

Location: Poster Area, Paris expo Porte de Versailles – Pavillon 7

About the Phase 2 study

WTX101-201 was a 24-week open-label Phase 2 study evaluating the efficacy and safety of WTX101 monotherapy in 28 newly diagnosed patients with Wilson Disease, aged 18 years and older, who had received either no prior treatment for Wilson Disease or a standard of care agent for up to two years. Patients recruited in the study had various degrees of hepatic impairment at the time of enrollment and the majority also had neurological symptoms at study start. The study was conducted at 11 sites in the US and Europe. Patients received WTX101 at individualized doses between 15 and 120 mg/day. The primary endpoint was defined as achieving or maintaining normalized levels of less than 2.3 µM of free blood copper, or reaching a reduction of at least 25% in free copper in blood from baseline, after 24 weeks of treatment. Free copper in blood was measured as non-ceruloplasmin-bound copper, corrected for the amount of copper bound to tripartite tetrathiomolybdate-copper-albumin complexes formed during WTX101 treatment ($NCC_{corrected}$). Secondary endpoints included reduction of serum free copper from baseline, neurological disability and status measured as Unified Wilson Disease Rating Scale (UWDRS) part II and III respectively, liver status measured with the Modified Nazer Score and quality of life measured with the EuroQOL 5 Dimensions Visual Analogue Scale (EQ VAS). A 36-month extension phase of the study is ongoing.

About WTX101 (bis-choline tetrathiomolybdate)

WTX101 (bis-choline tetrathiomolybdate) is a first-in-class copper-protein-binding agent with a unique mechanism of action, under investigation as a novel therapy for Wilson Disease. In contrast to current treatments, WTX101 provides an alternative copper-protein binding mechanism by forming a tripartite complex with copper and albumin. WTX101 thereby detoxifies excess copper in both liver and blood, and promotes copper clearance through biliary excretion (the body's natural route of elimination).

A Phase 2 study evaluating the efficacy and safety of WTX101 in patients with Wilson Disease has successfully been completed. In addition, the active moiety of WTX101, tetrathiomolybdate, has been tested in several previous clinical studies in Wilson Disease patients. The data from these studies suggest that WTX101 can reduce and control free copper levels and improve symptoms and associated disabilities.

The data also suggest that WTX101 is generally well tolerated with a low risk of drug-induced neurological worsening. The tolerability profile and the expected once-daily dosing regimen have the potential to improve compliance in Wilson Disease patients, leading to fewer treatment failures and ultimately improved outcomes. WTX101 has received Fast Track designation in the US and orphan drug designation for the treatment of Wilson Disease in the US and EU.

In addition, WTX101 has shown potential as a treatment for several other medical conditions including Amyotrophic Lateral Sclerosis (ALS). WTX101 has received US orphan drug designation for the treatment of ALS.

About Wilson Disease

Copper is an essential trace element that plays a critical role in key physiological cellular processes. Due to its toxic potential, copper is normally tightly bound to copper-carrying proteins inside the liver, and excess copper is eliminated from the body via biliary excretion. Wilson Disease is a rare genetic disorder of impaired copper transport and excretion, caused by loss of function of the ATP7B copper-binding protein. This leads to copper overload in the liver, release of free copper into the blood, and damaging accumulation of copper in the brain and other organs. Untreated Wilson Disease inevitably leads to various combinations and severity of hepatic, neurologic and psychiatric symptoms, and is ultimately fatal.

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 1950s and 60s. Since then, no new treatment options have been developed and considerable unmet medical needs still exist.

About Wilson Therapeutics

Wilson Therapeutics is a biopharmaceutical company, based in Stockholm, Sweden, that develops novel therapies for patients with rare copper-mediated disorders. Wilson Therapeutics' lead product, WTX101, is in Phase 3 development as a novel treatment for Wilson Disease. 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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