Preliminary long-term data for WTX101 in Wilson Disease accepted as a late-breaker presentation 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, has been accepted as a late-breaker poster presentation at The International Liver Congress™ 2018, the Annual Meeting of the European Association for the Study of the Liver (EASL) in Paris, France, 11-15 April, 2018.

An abstract of the poster is available today, highlighting that the initial improvements in free copper levels, hepatic status and neurological status observed at week 24 and 48 were preserved or further improved after once-daily treatment for 72 weeks with WTX101. As reported previously, mean levels of non-ceruloplasmin bound copper (NCCorrected) were significantly reduced in the Phase 2 study from 3.6 μM at baseline to 0.9 μM at week 24, and to 0.5 μM at week 48. The data that will be presented at EASL demonstrate that copper levels continued to remain controlled at week 72 at 0.5 μM. Continuous improvements in disability and neurological status were observed in most patients. Liver function tests (INR, ALT levels, albumin) and MELD score improved or remained unchanged between week 24 and week 72, indicating stabilization of liver function. The poster will also highlight the encouraging long-term safety profile of WTX101. Further details will be made available when the poster is presented.

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
The abstract is available on the conference website and can be accessed here: https://ilc-congress.eu/

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 tetraethiomolybdate-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 tetraethiomolybdate)

WTX101 (bis-choline tetraethiomolybdate) 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, tetraethiomolybdate, 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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