PRESS RELEASE

Stockholm October 23, 2017

Wilson Therapeutics reaches agreement with the FDA and EMA to initiate pivotal Phase 3 FOCuS study with WTX101 in Wilson Disease

Wilson Therapeutics (publ) today announced that the discussions with the U.S Food and Drug Administration (FDA) and the European Medicines Agency (EMA), regarding the study protocol for the single pivotal Phase 3 FOCuS trial for WTX101 in Wilson Disease, have been concluded successfully. Wilson Therapeutics will now complete the preparatory work to start the trial and expects to enroll the first patient in early 2018. 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.

The Phase 3 FOCuS trial will be a randomized, controlled, rater-blinded, multi-center study assessing the efficacy and safety of WTX101 monotherapy administered once daily for 48 weeks, compared to standard of care (SoC), in patients with Wilson Disease aged 18 years and older. The study will enroll approximately 100 patients with hepatic and/or neurological symptoms, who are treatment naïve or have previously received SoC therapy. The primary endpoint will be copper control, assessed as the percentage change in free blood copper levels from baseline to 48 weeks. Consistent with the successful Phase 2 study, copper control will be assessed using $NCC_{corrected}$. Additional endpoints include clinical (hepatology, neurology, psychiatry, disability) and quality of life related endpoints, and safety of WTX101. The study will be conducted at approximately 30 sites in the US, EU and Israel, including sites involved in the completed Phase 2 study. Patients completing the FOCuS study through 48 weeks will be offered continued WTX101 treatment in an extension phase.

In line with Wilson Therapeutics' commitment to improve the life of patients with Wilson Disease, the company will continue to conduct exploratory research in parallel with the FOCuS study to further deepen the understanding of the disease, the mode of action of WTX101 and its impact on copper control.

"We are very excited to move WTX101 into the pivotal phase of development and initiate the first global, randomized, controlled, multi-center study ever conducted in Wilson Disease. The FDA and the EMA have been very supportive and collaborative and have provided valuable discussions and input on the design of the Phase 3 study. We are confident that we have a robust trial design with the potential to repeat and confirm the excellent data observed in our Phase 2 trial, and support approval", said Jonas Hansson, CEO of Wilson Therapeutics.

"Having cared for patients with Wilson Disease for more than 30 years, I am extremely excited to be part of the WTX101 development program. Today, people with Wilson Disease are routinely treated with drugs that were first introduced in the 1950s and 1960s and there is a significant need for improved therapies. We are excited to offer WTX101 in the context of a clinical trial. The data generated in the Phase 2 study were very promising, particularly the rapid and clinically meaningful improvement of disease-related symptoms achieved with once-daily dosing of WTX101. In addition, the possibility of using WTX101 to avoid the early paradoxical neurological worsening, unfortunately seen in some patients started on the currently approved treatments penicillamine and trientine, is encouraging. Also, the once-daily dosing of WTX101 has the potential to minimize the treatment burden, leading to better long-term compliance and treatment outcomes. I am very hopeful we will be able to replicate the positive Phase 2 results in Phase 3", said Frederick K. Askari, MD, PhD, Associate Professor and Director of the Wilson Disease program at the University of Michigan.

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About the Phase 3 FOCuS study
The Phase 3 FOCuS clinical trial will be a randomized, controlled, rater-blinded, multi-center study assessing the efficacy and safety of WTX101 monotherapy administered once daily for 48 weeks, compared to standard of care (SoC), in patients with Wilson Disease aged 18 years and older. The study will enroll approximately 100 patients with hepatic and/or neurological symptoms, who are treatment naïve, or have previously received SoC therapy. Approximately 25% of the patients enrolled are expected to be treatment naïve, or to have received SoC therapy for <28 days. Patients will be randomized in a 2:1 ratio to receive treatment with WTX101 or SoC. The study is designed to show non-inferiority versus SoC and the primary endpoint will be copper control assessed as the percentage change in free blood copper levels from baseline to 48 weeks. Free copper in blood will be measured as non-ceruloplasmin-bound copper, corrected for the amount of copper bound in tripartite tetrathiomolybdate-copper-albumin complexes ($NCC_{corrected}$). Additional endpoints will include clinical (hepatology, neurology, psychiatry, disability) and quality of life related endpoints, and safety of WTX101. The study will be conducted at approximately 30 sites in the US, EU and Israel. Patients completing the Phase 3 study through 48 weeks will be offered continued WTX101 treatment in an extension phase.

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 lower 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 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 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.

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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 development as a novel treatment for Wilson Disease. A Phase 2 clinical study has been successfully completed and preparations for a pivotal Phase 3 study are ongoing. 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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