PRESS RELEASE
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First patient enrolled in pivotal Phase 3 FOCuS trial evaluating WTX101 for the treatment of Wilson Disease

Wilson Therapeutics (publ) today announced that the first patient has been enrolled in the pivotal Phase 3 FOCuS clinical trial evaluating WTX101 (bis-choline tetrathiomolybdate), an investigational first-in-class copper-protein-binding agent with a unique mechanism of action, for the treatment of Wilson Disease. FOCuS is a randomized, controlled, rater-blinded, multi-center study that will enroll approximately 100 Wilson Disease patients, aged 18 years or over, to receive once-daily WTX101 or standard of care. The primary endpoint will be copper control assessed as the percentage change in free copper levels in blood from baseline to 48 weeks. Top-line data from the study is expected to be released H2 2019.

Carl Bjartmar, MD, PhD, Chief Medical Officer, Wilson Therapeutics commented: "The start of the FOCuS study represents an important milestone for Wilson Therapeutics as well as for patients and families affected by Wilson Disease, as WTX101 has the potential to become the first new medicine for this serious disorder in several decades. Through its novel mode of action with its high affinity and specificity to copper, WTX101 detoxifies excess copper in the liver and in the blood by forming stable tripartite complexes with copper and albumin that are then cleared through bile, the natural elimination route of copper. This unique approach to copper control has the potential to improve symptoms and associated disabilities in Wilson Disease patients, which was demonstrated in our successful Phase 2 trial. The Phase 3 FOCuS study is the first randomized controlled trial ever conducted to support approval of a new treatment option for Wilson Disease and we look forward to further evaluating the differentiated profile of WTX101 in this head-to-head study versus standard of care."

The first patient was enrolled at the University of Michigan. Frederick K. Askari, MD, PhD, Associate Professor and Director of the Wilson Disease program at the University of Michigan, added: "WTX101 has shown great promise and I am excited to be part of the team advancing it through the clinic. The profile of this investigational drug is very encouraging, particularly the rapid control of clinical symptoms in combination with the simple once-daily dosing regimen and its promising side effect profile which could help improve compliance to therapy and treatment outcomes as a result. WTX101's unique potential to remove copper from the saturated copper stores in the liver is also very encouraging. If the results of the Phase 2 study are replicated in this Phase 3 trial and the product gains regulatory approval, I am confident WTX101 will have the potential to make a significant difference for the Wilson Disease community."

About the FOCuS study

The Phase 3 FOCuS clinical trial is 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 copper levels in blood from baseline (day 1) to 48 weeks. If non-inferiority is met superiority will be assessed. 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, disease related disability, psychiatry) 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. Further information about the study, including eligibility requirements, is available at www.clinicaltrials.gov [study ID: WTX101-301; clinicaltrials.gov identifier NCT03403205].

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.

For further information please contact:

Jonas Hansson, CEO, Wilson Therapeutics AB
Phone: +46 8 796 00 00
E-mail: jonas.hansson@wtx.se

Lauren Williams, Head of Investor Relations, Wilson Therapeutics AB
Phone: +44 7958 669 896
E-mail: lauren.williams@wtx.se

Wilson Therapeutics AB (publ)
Corp. Reg. No.556893-0357
Kungsgatan 3
SE-111 43 Stockholm

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