PRESS RELEASE

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WTX101 granted Fast Track designation by the U.S. FDA for the treatment of Wilson Disease

Wilson Therapeutics AB (publ), today announced that the U.S. Food and Drug Administration (FDA) has granted WTX101 Fast Track designation for the treatment of Wilson Disease. The FDA's Fast Track program is designed to facilitate the development, and expedite the review of novel therapies to treat serious conditions and fill an unmet medical need. 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.

"We are extremely pleased to receive Fast Track designation from the FDA, which supports our view that WTX101 can address significant unmet medical needs in this rare and debilitating disease. The Fast Track designation also provides a number of regulatory advantages, including additional access to the FDA which can speed up the future review of WTX101 and help bring this drug to patients as quickly as possible", commented Wilson Therapeutics CEO Jonas Hansson.

The Fast Track Designation is supported by data from the 24-week open label multicenter Phase 2 trial in patients with Wilson Disease (WTX101-201), which was conducted by Wilson Therapeutics. The study showed that once-daily dosing of WTX101 has the potential to rapidly lower and control free copper, improve or stabilize neurological and liver status and improve patient-reported disability. Furthermore, no cases of initial drug-induced neurological worsening upon treatment initiation have been observed to date. An open-label extension study is ongoing, and the company expects to enroll the first patient in the Phase 3 FOCuS trial in early 2018.

About Fast Track designation
Fast Track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions that address an unmet medical need, by providing a therapy where none exists or providing a therapy which may be potentially better and shows some advantage over available therapy. Fast Track designation includes opportunities for more frequent meetings with the FDA to discuss trial design, development plans, data needed to support drug approval, submission of a New Drug Application (NDA) on a rolling basis and eligibility for accelerated approval and priority review, if relevant criteria are met.

Visit FDA's website for more information

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.

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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