STOCKHOLM, Jan. 13, 2014 /PRNewswire/ -- Wilson Therapeutics , a privately-held biopharmaceutical company, today announced that it has successfully filed an Investigational New Drug (IND) application with U.S. Food and Drug Administration for the development of WTX101 (bis-choline tetrathiomolybdate), a novel de-coppering agent under evaluation as a treatment for Wilson Disease. The recent acceptance of the U.S. IND is a key initial step in the company's plan to advance WTX101 into pivotal clinical trials as a treatment for Wilson Disease, a rare genetic disorder that prevents the body from regulating copper and can lead to serious liver and brain damages.

"This IND enables us to proceed with a Phase 1 study that will provide additional pharmacokinetic and bioavailability data to support the overall development of WTX101 as it moves towards late stage, pivotal trials," said David Clark, M.D., chief medical officer of Wilson Therapeutics. "We expect to conduct this study quickly as we advance the WTX101 program forward this year."

WTX101 is the proprietary bis-choline formulation of tetrathiomolybdate (TTM), which has been evaluated in clinical studies in over 500 patients in various indications. TTM has been shown to rapidly lower copper levels. Previous data also suggest that TTM may stabilize neurological function and reduce the risk of neurological deterioration after initiation of treatment in Wilson Disease patients with neurological disease. WTX101 has also been tested in oncologic clinical trials and was shown to be safe and tolerable while efficiently lowering copper levels with once-daily dosing. WTX101 has received orphan drug designation in both the United States and the European Union.

"There remains a serious unmet need in the treatment of Wilson Disease patients, particularly those who present with neurological symptoms that are often suboptimally treated with currently approved medications," said Fred Askari, M.D., Ph.D., associate professor and director of the Wilson Disease Program and Wilson Disease Center of Excellence, Division of Gastroenterology in the Department of Internal Medicine at the University of Michigan. "We need to develop treatments that are more tolerable and convenient, and can be used at all phases of the disease."

About Wilson Therapeutics Wilson Therapeutics is a privately-held biopharmaceutical company focused on improving the lives of patients with Wilson Disease through development

of new treatment options and support for increased awareness and education about Wilson Disease. Wilson Therapeutics' lead product candidate, WTX101, is currently in clinical development as a treatment for Wilson Disease, a serious orphan disease that affects approximately 1 in 15,000 worldwide. Wilson Therapeutics was founded by Healthcap, one of the largest venture capital firms in Europe specialized in life sciences. Visit www.wilsontherapeutics.com for more information.

SOURCE Wilson Therapeutics

RELATED LINKS http://www.wilsontherapeutics.com