May 25, 2018

Alexion Offer for Wilson Therapeutics Accepted

- Offer accepted by shareholders representing 97.7% of shares and votes in Wilson Therapeutics -

NEW HAVEN, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced that its offer, through a wholly owned subsidiary, for the shares in Wilson Therapeutics has been accepted by shareholders representing 97.7 percent of the total number of shares and votes in Wilson Therapeutics. The acquisition has also been approved by relevant regulatory authorities. As more than 90 percent of the total number of shares have been tendered and all conditions of the offer have been fulfilled, the offer has been declared unconditional and settlement of the tendered shares will occur on May 30, 2018.

"The acquisition of Wilson Therapeutics is a key first step in rebuilding our pipeline," said Ludwig Hantson, Ph.D., Chief Executive Officer of Alexion. "We look forward to using our significant experience in rare metabolic and neurological diseases to improve treatment for patients with Wilson disease through the development of WTX101, which has the potential to be the first new treatment in more than 20 years and become the new standard of care for Wilson disease."

In order to give Wilson Therapeutics' shareholders additional time to accept the offer, Alexion has extended the acceptance period until June 8, 2018. Settlement for shares tendered during this extended acceptance period is expected on June 15, 2018.

Alexion intends to initiate compulsory redemption proceedings regarding the remaining shares in Wilson Therapeutics as well as to promote a delisting of the shares from Nasdaq Stockholm.

For additional details on the transaction, please visit http://ir.alexion.com/acquisitions.cfm.

About Wilson Disease

Wilson disease is a rare, chronic, genetic, and potentially life-threatening liver disorder of impaired copper transport. Copper balance is normally maintained in the body by hepatic excretion of excessive copper in the bile. In patients with Wilson disease, a genetic mutation disables this biliary excretion pathway and excess copper accumulates over time in the liver cells. The accumulation of copper eventually overwhelms safe storage capacity and cellular injury occurs. When the liver's capacity for copper storage is exceeded, and when liver cells are injured, copper is released into the circulation and may accumulate in other organs, including the central nervous system. Untreated, Wilson disease leads to various combinations and severity of hepatic, neurologic, and psychiatric symptoms, and can be fatal.[1,2]

Wilson disease affects approximately one in every 30,000 people worldwide.[3] The average age of diagnosis is 15-20 years,[3] with the majority of patients presenting between the ages of 10 and 30.[4] Current standard of care includes metal chelators to remove serum copper, followed by maintenance with zinc to prevent re-accumulation.[1,2] There have been no new treatment options approved in over two decades and a significant unmet need still exists for patients.

About WTX101

WTX101 (bis-choline tetrathiomolybdate) is a first-in-class copper-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). WTX101 has a 10,000-fold higher affinity for copper than other chelators and addresses the underlying cause of the disease.

A Phase 2 study evaluating the efficacy and safety of WTX101 in patients with Wilson disease has been completed successfully.[5] 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. WTX101 has received Fast Track designation in the U.S. and Orphan Drug Designation for the treatment of Wilson disease in the U.S. and EU.

About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the innovation, development, and commercialization of life-changing therapies. Alexion is the global leader in complement inhibition and has developed and commercializes the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG). In addition, Alexion has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). As the leader in complement biology for over 20 years, Alexion focuses its research efforts on novel molecules and targets in the complement cascade, and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders. This press release and further information about Alexion can be found at: www.alexion.com.

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Forward-Looking Statement

This press release contains forward-looking statements, including statements related to the potential benefits of WTX101 for the treatment of Wilson disease and the potential benefits of the transaction. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, failure to satisfactorily address matters raised by the U.S. Food and Drug Administration and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations, the possibility that clinical trials of our product candidates could be delayed, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with Wilson disease are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended March 31, 2018 and in our other filings with the SEC. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

References

[1] Roberts, E. and M.L. Schilsky (2008). "Diagnosis and Treatment of Wilson Disease: An Update." *Hepatology* 47(6): 2089-2111.

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[3] Merle, U. et al., (2007). "Clinical presentation, diagnosis and long-term outcome of Wilson's disease: a cohort study." *Gut* 56: 115-120.

[4] Beinhardt, S. et al., (2014). "Long-term outcomes of patients with Wilson disease in a large Austrian cohort." *Clin Gastroenterol Hepatol* 12: 683-689.

[5] Weiss, K.H. et al., (2017). Bis-choline tetrathiomolybdate in patients with Wilson's disease: an open-label, multicentre, phase 2 study. *Lancet Gastroenterol Hepatol* 2(12): 869-876.

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