X4 Pharmaceuticals Announces FDA Acceptance with Priority Review of U.S. NDA for Mavorixafor in WHIM Syndrome

October 31, 2023

FDA sets a Prescription Drug User Fee Act (PDUFA) target action date of April 30, 2024

If the NDA is approved, company eligible to receive a Priority Review Voucher (PRV) resulting from mavorixafor's rare pediatric designation in WHIM syndrome

NDA supported by positive results from global, pivotal 4WHIM Phase 3 clinical trial

BOSTON, Oct. 31, 2023 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company driven to improve the lives of people with rare diseases of the immune system, today announced that the United States Food and Drug Administration (FDA) has accepted for filing the company's New Drug Application (NDA) for once-daily, oral mavorixafor to treat individuals aged 12 and older with WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency. The FDA granted Priority Review of the mavorixafor NDA, establishing a goal of six months review from the date of acceptance and assigning a Prescription Drug User Fee Act (PDUFA) target action date of April 30, 2024.

"The FDA's acceptance of our mavorixafor NDA with priority review represents yet another significant step forward towards a potential treatment for those with WHIM syndrome, a rare disease for which there are currently no approved therapies," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We look forward to working closely with the FDA throughout the NDA review process with the goal of bringing mavorixafor to people with WHIM syndrome as quickly as possible."

Mavorixafor is an investigational small-molecule antagonist of the CXCR4 receptor being developed as a once-daily oral therapy for WHIM syndrome and certain chronic neutropenic disorders. For the WHIM syndrome indication, mavorixafor has been granted Breakthrough Therapy Designation, Fast Track Designation, and Rare Pediatric Disease (RPD) Designation in the U.S., and Orphan Drug Status in both the U.S. and European Union. Upon FDA approval of a product with RPD designation, the sponsor can receive a Priority Review Voucher that can be used to obtain priority review for a subsequent application or sold to another drug sponsor.

The NDA is supported by the results of the global, pivotal, 4WHIM Phase 3 clinical trial of once-daily, oral mavorixafor in individuals with WHIM syndrome. The 4WHIM trial met its primary endpoint of time above threshold for absolute neutrophil count (TAT-ANC) vs. placebo ($p<0.0001$), a key secondary endpoint, and was generally well tolerated in the trial, with no treatment-related serious adverse events reported and no discontinuations for safety events. The 4WHIM data also revealed that mavorixafor treatment resulted in reductions in the rate, severity, and duration of infections in trial participants versus placebo. These and additional 4WHIM Phase 3 data were published in oral presentations at the annual meetings of both the Clinical Immunology Society (CIS) and European Hematology Association (EHA).

About WHIM Syndrome
WHIM syndrome is a rare, inherited, combined immunodeficiency disease caused by reduced mobilization and trafficking of white blood cells from the bone marrow due to over-signaling of the CXCR4/CXCL12 pathway. WHIM syndrome is named for its four common clinical findings: Warts, Hypogammaglobulinemia, Infections, and Myelokathexis, although not all patients experience all symptoms, and not all symptoms are required for a diagnosis. People with WHIM syndrome characteristically have very low blood levels of neutrophils (neutropenia) and lymphocytes (lymphopenia), and as a result, experience frequent, recurrent infections with a high risk of lung disease and refractory warts from underlying human papillomavirus (HPV) infection. Those with WHIM syndrome may also have limited antibody production due to low levels of immunoglobulin and an increased risk of developing certain types of cancer.

About the 4WHIM Phase 3 Clinical Trial
The 4WHIM Phase 3 clinical trial was a global, randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the efficacy and safety of oral, once-daily mavorixafor in people with genetically confirmed WHIM syndrome. The trial enrolled 31 participants aged 12 and older who received either mavorixafor (n=14) or placebo (n=17) orally once daily for 52 weeks. An open-label extension phase of the clinical trial is ongoing (NCT03995108).

About X4 Pharmaceuticals
X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company driven to improve the lives of people with rare diseases of the immune system. Our lead clinical candidate is mavorixafor, a small molecule antagonist of chemokine receptor CXCR4 that is being developed as an oral, once-daily therapy across a variety of immunodeficiencies, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome and certain chronic neutropenic disorders. Following successful completion of a global, pivotal, Phase 3 clinical trial, we are seeking U.S. approval of oral, once-daily mavorixafor for the treatment of people aged 12 years and older with WHIM syndrome. We are also currently planning a Phase 3 clinical program evaluating mavorixafor in certain chronic neutropenic disorders. We continue to leverage our insights into CXCR4 and immune system biology at our corporate headquarters in Boston, Massachusetts and at our research center of excellence in Vienna, Austria. For more information, please visit our website at www.x4pharma.com.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of applicable securities laws, including the Private Securities Litigation Reform Act of 1995, as amended. These statements may be identified by the words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target,” or other similar terms or expressions that concern X4's expectations, strategy, plans, or intentions. Forward-looking statements include, without limitation, statements regarding the timing and potential impact of FDA acceptance and priority review of X4’s NDA for mavorixafor for the treatment of individuals with WHIM syndrome; and mavorixafor’s potential to be the first therapy for WHIM syndrome. Any forward-looking statements in this press release are based on management's current expectations and beliefs. Actual events or results may differ materially from those expressed or implied by any forward-looking statements contained herein, including, without limitation, risks of obtaining and maintaining regulatory approvals, including, but not limited to, potential regulatory delays or rejections or the risk that the FDA will require additional trials or data; the potential inability to raise sufficient capital to fund ongoing operations as currently planned or to obtain financings on terms similar to those arranged in the past; the ability to service indebtedness and otherwise comply with debt covenants; outcomes or trends from competitive studies; the timing and outcomes of clinical studies of mavorixafor; general economic conditions; and other risk and uncertainties, including those described in the section entitled “Risk Factors” in X4’s Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on August 10, 2023, and in other filings X4 makes with the SEC from time to time. X4 undertakes no obligation to update the information contained in this press release to reflect new events or circumstances, except as required by law.

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