X4 Pharmaceuticals Receives Positive Opinion from the EMA CHMP Recommending Approval of Mavorixafor in the EU for WHIM Syndrome

February 27, 2026

- Potential First and Only Therapy in the EU for Ultra-Rare Immune Disorder -

BOSTON, Feb. 27, 2026 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company focused on improving the lives of people with rare hematology diseases, today announced the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion recommending the granting of marketing authorization, under exceptional circumstances, of mavorixafor for the treatment of WHIM syndrome in the European Union (EU). The positive opinion will now be reviewed by the European Commission (EC) with a final approval decision anticipated in the second quarter of 2026.

"This positive opinion from the CHMP for mavorixafor represents a key milestone toward making this treatment available to WHIM syndrome patients in the EU," said Adam Craig, M.D., Ph.D., Executive Chairman of X4 Pharmaceuticals. "WHIM syndrome is an ultra-rare disease with a significant unmet medical need. We look forward to the EC's decision and to working with Norgine, our commercial partner in Europe, Australia and New Zealand, to ensure patient access upon a potential approval to the first and only therapy in Europe to treat this devastating condition."

WHIM syndrome is an ultra-rare, inherited primary immunodeficiency named for its four classic manifestations: warts, hypogammaglobulinemia, infections, and myelokathexis. WHIM is caused by CXCR4 receptor dysfunction that results in impaired mobilization of white blood cells from the bone marrow into peripheral circulation resulting in serious and/or frequent infections.

Mavorixafor, a CXC chemokine receptor 4 antagonist, has been approved by the U.S. Food and Drug Administration (FDA) under the trade name **XOLREMDI®**, for use as an oral, once-daily therapy in patients 12 years of age and older with WHIM syndrome to increase the number of circulating mature neutrophils and lymphocytes. XOLREMDI is the first drug ever approved in the United States to treat WHIM syndrome.

The CHMP's positive opinion for mavorixafor is supported by results from the pivotal, Phase 3 clinical trial (4WHIM), a global, randomized, double-blind, placebo-controlled, 52-week multicenter study that evaluated the efficacy and safety of XOLREMDI in 31 people aged 12 years and older diagnosed with WHIM syndrome. The efficacy of XOLREMDI was determined by improvement in absolute neutrophil counts (ANC), improvement in absolute lymphocyte counts (ALC), and a reduction in infections. In the 4WHIM trial, XOLREMDI treatment demonstrated increased time above threshold (≥500 cells/microliter) for absolute neutrophil count (TAT-ANC) vs. placebo ($p<0.0001$) and increased time above threshold (≥1000 cells/microliter) for absolute lymphocyte count (TAT-ALC) v. placebo ($p<0.0001$). The efficacy of XOLREMDI was further assessed in a composite endpoint consisting of total infection score and total wart change score using a Win-Ratio method. Analyses of the individual components of this composite endpoint showed an approximate 40% reduction in total infection score, weighted by infection severity, in XOLREMDI-treated patients compared with placebo-treated patients. There was no difference in total wart change scores between the XOLREMDI and placebo treatment arms over the 52-week period. Treatment with XOLREMDI also resulted in a 60% reduction in the annualized infection rate compared with placebo-treated patients. The most common adverse reactions reported in the 4WHIM trial (≥10% and more frequently reported than placebo) were thrombocytopenia, pityriasis, rash, rhinitis, epistaxis, vomiting, and dizziness.

In January 2025, X4 entered into a licensing and supply agreement with Norgine , a European specialist pharmaceutical company, under which Norgine will commercialize mavorixafor in Europe, Australia and New Zealand following regulatory approvals. Under the terms of the license and supply agreement, X4 will receive up to €226 million contingent upon the achievement of certain regulatory and commercial milestones, in addition to escalating double-digit royalties of up to the mid-twenties on any future net sales in the licensed territories. All marketing authorizations in the licensed territories will be transferred to Norgine. Once completed, Norgine will be responsible for all market access and commercialization activities in the licensed territories. X4 will manufacture and supply mavorixafor to Norgine.

IMPORTANT SAFETY INFORMATION

CONTRAINDICATION

XOLREMDI® is contraindicated with drugs highly dependent on CYP2D6 for clearance.

WARNINGS AND PRECAUTIONS

- **Embryo-Fetal Toxicity:** Based on its mechanism of action, XOLREMDI is expected to cause fetal harm when administered to a pregnant woman. Verify pregnancy status of female patients of reproductive potential prior to starting XOLREMDI. Advise females of reproductive potential to use effective contraception during treatment with XOLREMDI and for three weeks after the final dose.
- **QTc Interval Prolongation:** XOLREMDI causes concentration-dependent QTc prolongation. QTc prolongation may occur when XOLREMDI is taken with concomitant medications that increase XOLREMDI exposure and/or drug products with a known potential to prolong QTc. Correct any modifiable risk factors for QTc prolongation, assess QTc at baseline, and monitor QTc during treatment as clinically indicated in patients with risk factors for QTc prolongation or receiving concomitant medications that increase XOLREMDI exposure and/or drugs with a

known potential to prolong the QTc interval. Dose reduction or discontinuation of XOLREMDI may be required.

ADVERSE REACTIONS
The most common adverse reactions (in ≥10% patients and more frequently reported than placebo) were thrombocytopenia, pityriasis, rash, rhinitis, epistaxis, vomiting, and dizziness.

DRUG-DRUG INTERACTIONS
Avoid co-administration of XOLREMDI and strong CYP3A4 inducers. Reduce XOLREMDI daily dosage when administered with strong CYP3A4 inhibitors. Monitor more frequently for adverse reactions associated with an increase in exposure of XOLREMDI when used concomitantly with moderate CYP3A4 inhibitors or P-gp inhibitors and reduce XOLREMDI daily dosage if necessary.

USE IN SPECIFIC POPULATIONS

- Advise females that breastfeeding is not recommended during treatment with XOLREMDI and for three weeks after the final dose.
- The safety and effectiveness of XOLREMDI have not been established in pediatric patients younger than 12 years of age.
- XOLREMDI is not recommended in patients with severe renal impairment, end-stage renal disease, or moderate to severe hepatic impairment.

To report suspected adverse reactions, contact X4 Pharmaceuticals at 1-866-MED-X4MI (1-866-633-9464) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.
Please see the full Prescribing Information for XOLREMDI.

About WHIM Syndrome

WHIM syndrome is an ultra-rare, inherited, combined primary immunodeficiency and chronic neutropenic disorder caused by CXCR4 receptor dysfunction that results in impaired mobilization of white blood cells from the bone marrow into peripheral circulation. WHIM syndrome is named for its four classic manifestations: warts, hypogammaglobulinemia, infections, and myelokathexis, although only a minority of patients experience all four manifestations. People with WHIM syndrome characteristically have low blood levels of neutrophils (neutropenia) and lymphocytes (lymphopenia), and as a result, experience serious and/or frequent infections.

About X4 Pharmaceuticals

X4 Pharmaceuticals is a company focused on improving the lives of people with rare hematology diseases by developing and commercializing innovative therapies in areas with significant unmet needs. Leveraging expertise in diseases of the immune system and CXCR4 biology, X4 has successfully developed mavorixafor, an orally available CXCR4 antagonist that is commercially available in the U.S. as XOLREMDI® in its first indication. The Company is currently conducting a global, pivotal Phase 3 clinical trial (4WARD) evaluating mavorixafor in chronic neutropenic disorders. The U.S. FDA has granted Fast Track designation to mavorixafor for the treatment of chronic neutropenia. X4 is headquartered in Boston, Massachusetts. For more information, please visit www.x4pharma.com.

X4 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, implied or express statements regarding the potential for and the timing of a positive final approval decision from the European Commission (EC) regarding mavorixafor in WHIM syndrome, the potential achievement of milestones and receipt of royalties under the Company’s licensing and supply agreement with Norgine Pharma and other future plans for the Company. Any forward-looking statements in this press release are based on management’s current expectations and beliefs. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond X4’s control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including the risks that the EC does not provide a positive final approval decision with regard to mavorixafor in WHIM syndrome; that even if approved, mavorixafor may not ultimately be commercially successful; ; and other risks and uncertainties, including those described in the section entitled “Risk Factors” in X4’s most recent Annual Report on X4’s Form 10-K, as well as in other filings X4 makes with the Securities and Exchange Commission, including its quarterly reports on Form 10-Q, 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.

Source: X4 Pharmaceuticals, Inc.