X4 Pharmaceuticals Announces European Commission Approval of XOLREMDI® (Mavorixafor), the First and Only Authorized Treatment for Patients with WHIM Syndrome in the European Union

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- European Commercialization will be Led by X4 Pharmaceuticals' Partner, Norgine -

- Global, Pivotal Phase 3 Clinical Trial Ongoing to Evaluate Mavorixafor in Chronic Neutropenia -

BOSTON, April 29, 2026 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company focused on improving the lives of people with rare hematology diseases, announced today that the European Commission (EC) has granted marketing authorization for XOLREMDI® (mavorixafor) capsules for the treatment of patients with WHIM syndrome in the European Union (EU). The approval follows a positive opinion from the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP). The marketing authorization was granted under exceptional circumstances, reflecting the ultra-rare nature of WHIM syndrome.

"The approval of mavorixafor in the European Union marks an important step in extending access to XOLREMDI beyond the United States and providing a new, targeted therapeutic option to patients living with WHIM syndrome, an ultra-rare hematologic disorder," said Adam Craig, M.D., Ph.D., Executive Chairman of X4 Pharmaceuticals. "We look forward to working with Norgine, our commercial partner in Europe, to enable patient access to mavorixafor to treat this devastating condition. In addition, we are now running a global, pivotal Phase 3 clinical trial (4WARD) evaluating mavorixafor in chronic neutropenia to further explore its potential benefit across multiple diseases."

XOLREMDI® (mavorixafor), a CXC chemokine receptor 4 (CXCR4) antagonist, is authorized for the treatment of patients 12 years and older with WHIM syndrome for use as an oral, once-daily therapy to increase the number of circulating mature neutrophils and lymphocytes. XOLREMDI is the first and only drug ever approved in the United States and the European Union to treat WHIM syndrome.

WHIM syndrome is an ultra-rare, inherited combined primary immunodeficiency and chronic neutropenic disorder named for its four classic manifestations: warts, hypogammaglobulinemia, infections, and myelokathexis. WHIM syndrome is caused by CXCR4 receptor dysfunction that results in impaired mobilization of white blood cells from the bone marrow into peripheral circulation. 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. As a CXCR4 antagonist, XOLREMDI targets CXCR4 dysfunction, the underlying cause of WHIM syndrome, enabling mobilization of white blood cells from the bone marrow into the bloodstream.

The EC approval is supported by results from the pivotal Phase 3 4WHIM trial, a global, randomized, double-blind, placebo-controlled, 52-week multicenter study that evaluated the efficacy and safety of mavorixafor in 31 people aged 12 years and older diagnosed with WHIM syndrome.

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 could 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.

Please see the U.S. Full Prescribing Information for XOLREMDI by clicking here.
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.

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 plans for the commercialization of XOLREMDI in the European Union by Norgine, the potential achievement of milestones and receipt of royalties under the

Company's licensing and supply agreement with Norgine 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 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 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.

X4 Investor Contact:
Remy Bernarda
Jenny Kobin
IR Advisory Solutions
ir@x4pharma.com

Source: X4 Pharmaceuticals, Inc.