X4 Pharmaceuticals and taiba rare Announce Exclusive Agreement for the Distribution and Commercialization of XOLREMDI® (mavorixafor) in WHIM Syndrome in Select Middle East Countries

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Agreement covers Saudi Arabia, United Arab Emirates, Qatar, Oman, Kuwait, Bahrain, and Egypt

BOSTON and DUBAI, United Arab Emirates, Feb. 19, 2025 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company driven to improve the lives of people with rare diseases of the immune system, and **taiba rare,** a taiba Healthcare company with specialty & orphan drug marketing, sales, and distribution expertise in the MENA (Middle East and North Africa) region, today announced that they have entered into an exclusive agreement for the distribution and commercialization of XOLREMDI® (mavorixafor), an oral, once-daily treatment for WHIM syndrome (warts, hypogammaglobulinemia, infections and myelokathexis), in select Middle East countries (Saudi Arabia, United Arab Emirates, Qatar, Oman, Kuwait, Bahrain, and Egypt), following any approvals in the region.

XOLREMDI (mavorixafor) was approved by the U.S. Food and Drug Administration (FDA) in April 2024 for the treatment of patients 12 years of age and older with WHIM syndrome, a rare primary immunodeficiency, to increase the number of circulating mature neutrophils and lymphocytes. The Marketing Authorization Application (MAA) for mavorixafor for the treatment of WHIM syndrome is currently under review by the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP).

"We are very pleased to collaborate with taiba rare, an ideal partner for X4 due to their dedication to and experience providing access to innovative rare disease treatments throughout the region," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We look forward to working together to further expand the global reach of XOLREMDI for people with WHIM syndrome."

Saif Al Hasani, Chief Executive Officer of taiba Healthcare, commented on the announcement: "XOLREMDI is the first and only treatment approved for patients with WHIM syndrome. We are proud to partner with X4 to bring XOLREMDI to those with this rare primary immunodeficiency in the Middle East region. Having been granted Orphan Drug Designation in both the U.S. and the European Union, XOLREMDI fits well into our portfolio of innovative therapies for those with the highest unmet needs."

taiba will lead the distribution, promotion, marketing, and sales of XOLREMDI within the territory, working jointly with X4 on key strategic decisions. Local regulatory filings for XOLREMDI approval will be based on X4's registration dossier submitted to the U.S. FDA. Pending any regulatory approvals in the region, taiba is expected to be able to provide XOLREMDI to WHIM patients through a named-patient (compassionate use) program that allows physicians to prescribe medicines approved in other countries to local patients with no other treatment options.

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 a rare, 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 in the acronym. 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 XOLREMDI® (mavorixafor)
XOLREMDI (mavorixafor) is a selective CXCR4 receptor antagonist approved in the U.S. as a once-daily oral treatment for use in patients 12 years of age and older with WHIM syndrome to increase the number of circulating mature neutrophils and lymphocytes. XOLREMDI is the only treatment specifically approved for patients with WHIM syndrome in the U.S.

About X4 Pharmaceuticals
X4 is delivering progress for patients by developing and commercializing innovative therapies for those with rare diseases of the immune system and significant unmet needs. Leveraging expertise in CXCR4 and immune system biology, X4 has successfully developed mavorixafor, an orally available CXCR4 antagonist that is currently being marketed in the U.S. as XOLREMDI® in its first indication. The company is also evaluating additional uses of mavorixafor and is conducting a global, pivotal Phase 3 clinical trial (4WARD) in people with certain chronic neutropenic disorders. X4 is headquartered in Boston, Massachusetts. For more information, please visit www.x4pharma.com.

About taiba Healthcare
Established in 1980, taiba Healthcare is a leading specialty marketing, sales and distribution company in the MENA region providing innovative treatments to patients with orphan and rare diseases either through early access programs or through commercialization. taiba's vision is to cover the unmet medical need in its region providing breakthrough products and high level of service to healthcare organizations and commitment to patient treatment. Over the last 40 years, taiba has earned a reputation as a leading regional company in rare diseases by actively seeking opportunities to collaborate with healthcare professionals, organizations, and institutions, for the benefit of patients.

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 compiling and submitting regulatory submissions in the select Middle East countries and the potential for approval in the select Middle East countries; the initiation, timing, progress, and results of X4's current and future preclinical studies and clinical trials; the timing and anticipated interactions with regulatory authorities and any related approvals for mavorixafor in Saudi Arabia, United Arab Emirates, Qatar, Oman, Kuwait, Bahrain, and Egypt; the potential market opportunity for mavorixafor; the anticipated strategic benefits of X4's exclusive agreement for the distribution and commercialization with taiba and of any current or future collaborations; and the mission and goals for X4's business. 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: there are uncertainties inherent in the regulatory approval process in the select Middle East Countries including the timing, progress, and outcome of their review; potential delays or difficulties in commercializing mavorixafor in the Middle East if regulatory approval is granted; challenges in coordinating with external partners including taiba; X4 may have difficulty establishing and maintaining an effective sales and marketing organization or suitable third-party alternatives for any approved products; X4 may not be able to obtain or maintain orphan drug designation or exclusivity for X4's drug candidates, which could limit the potential profitability of X4's product candidates; X4 may not be able to obtain regulatory approval for, or successfully commercialize, mavorixafor or any other product candidate for any chronic neutropenic disorder or any other potential indication; the expected availability, content, and timing of clinical data from X4's ongoing clinical trials of mavorixafor may be delayed or unavailable, including X4's ongoing Phase 3 clinical trial; the design and rate of enrollment for clinical trials, including the current design of a Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders may not enable successful completion of the trial(s); X4's business could be substantially harmed due to the lengthy, time-consuming and inherently unpredictable regulatory review and approval processes of the FDA and comparable foreign regulatory authorities , and X4 may ultimately be unable to obtain regulatory approval for X4's product candidates, including additional indications for mavorixafor; the need to align with X4's collaborators may hamper or delay X4's development and commercialization efforts or increase X4's costs; X4's business may be adversely affected and their costs may increase if any of X4's key collaborators fails to perform its obligations or terminates the collaboration; the internal and external costs required for X4's ongoing and planned activities, and the resulting impact on expense and use of cash, may be higher than expected, which may cause the company to use cash more quickly than expected or to change or curtail some of X4's plans or both; and other risks and uncertainties, including those described in the section entitled "Risk Factors" in X4's Quarterly Report on X4's Form 10-Q filed with the Securities and Exchange Commission (SEC) on November 13, 2024, 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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