X4 Pharmaceuticals Presents New Clinical and Scientific Data at 2021 ASH Annual Meeting Supporting Broader Potential for Mavorixafor in the Treatment of Primary Immunodeficiencies

December 13, 2021

- Presented data highlight mavorixafor's ability to raise the spectrum of white blood cell counts across a broad range of diseases with or without CXCR4 mutations including Chronic Neutropenia -

- Mavorixafor continues to show sustained improvements in infections and has been well tolerated in the ongoing Phase 2 open-label extension trial in WHIM syndrome -

- X4 to host virtual seminar webcast on primary immunodeficiencies and the potential role for mavorixafor in the treatment landscape on Thursday, December 16, 2021 at 8:30 a.m. EST -

BOSTON, Dec. 13, 2021 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals, Inc.** (Nasdaq: XFOR), a leader in the discovery and development of novel CXCR4-targeted small molecule therapeutics to benefit people with immune system dysfunction, today highlighted key takeaways from three clinical and scientific posters presented over the weekend at the American Society of Hematology (ASH) Annual Meeting, taking place both virtually and in person in Atlanta, GA from December 11-14, 2021.

As **previously announced**, X4 will be hosting a **virtual seminar** on Thursday, December 16, 2021 at 8:30 a.m. EST to review the following ASH posters as well as additional scientific data from the company. All posters are now available on the **X4 corporate website.** Conference attendees can access accompanying audio presentations of the posters on the **ASH 2021 website.**

Poster #2186: *"Oral Administration of Mavorixafor, a CXCR4 Antagonist, Increases Peripheral White Blood Cell Counts Across Different Disease States"*

Key Poster Takeaways:

- Ongoing studies across a wide variety of diseases, including Waldenström's macroglobulinemia (WM), clear cell renal cell carcinoma (ccRCC), WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis) syndrome, and chronic idiopathic neutropenia (CN), show that oral administration of mavorixafor increases blood neutrophils, lymphocytes, and monocytes.
- Mavorixafor efficacy has been observed with short-term and long-term treatment both alone and in combination with other therapies, including axitinib, ibrutinib, and granulocyte-colony stimulating factor (G-CSF).
- These results suggest that mavorixafor, through its mechanism of CXCR4 antagonism, has the potential to reduce the prevalence and/or severity of a broader array of immunodeficiencies than previously recognized, regardless of the presence or absence of CXCR4 mutations.

Poster #1121: *"Mavorixafor, an Oral CXCR4 Antagonist, for Treatment of Patients with WHIM Syndrome: Results from the Long-Term Extension of the Open-Label Phase 2 Study"*

Key Poster Takeaways:

- Mavorixafor continues to show durable increases in neutrophils, lymphocytes, and monocytes, sustained improvements in infections and warts, and has been well tolerated in the ongoing Phase 2 open-label extension trial in WHIM syndrome (median treatment duration = 148.4 weeks).
- Infection rates decreased with increased doses of mavorixafor.
- Decreases in mean annualized infection rates correlate well with time above threshold for absolute neutrophil count (TAT-ANC), the primary endpoint in X4's ongoing, fully enrolled, global, Phase 3 registrational trial in WHIM syndrome.
- Standardized patient interviews revealed that long-term treatment with mavorixafor has been well tolerated and continues to demonstrate beneficial treatment effects, including decreased frequency, severity, and duration of infections and fewer hospital/doctor visits.
- Data continue to support the potential of mavorixafor to be a safe, effective, and long-term oral therapy targeting the underlying cause of WHIM syndrome.
- Phase 3 top-line clinical results from the 52-week trial are expected in the fourth quarter of 2022.

Poster #2063: *"Comprehensive* In Vitro *Characterization of CXCR4^{WHIM} Variants to Decipher Genotype–Phenotype Correlations in WHIM Syndrome"*

Key Poster Takeaways:

- In this study, detailed functional analyses of 14 published CXCR4^{WHIM} mutations were performed.
- Data suggest that CXCR4 internalization and AKT activation may be used as key assays for the prediction of CXCR4 variant pathogenicity *in vitro* and potentially as *in vitro* WHIM-related disease biomarkers.
- All tested CXCR4 variant cell lines were sensitive to mavorixafor at clinically relevant concentrations.

About X4 Pharmaceuticals
X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company leading the discovery and development of novel therapies for people with immune system dysfunction. The company's lead candidate is mavorixafor, a first-in-class, small molecule antagonist of chemokine receptor CXCR4 that is being developed as a once-daily oral therapy. Due to mavorixafor's ability to antagonize CXCR4 and improve the healthy maturation and trafficking of white blood cells, X4 believes that mavorixafor has the potential to provide therapeutic benefit across a wide variety of diseases, including primary immunodeficiencies (PIDs) and certain types of cancer. Mavorixafor has already demonstrated clinical potential in a Phase 2 trial in people with WHIM syndrome, a rare PID. Its efficacy and safety continue to be evaluated in a global Phase 3 clinical trial in WHIM (fully enrolled) and in two Phase 1b clinical trials – one, as monotherapy in people with Severe Congenital Neutropenia (SCN) and other chronic neutropenia conditions, and another in combination with ibrutinib in people with Waldenström's macroglobulinemia, a rare B-cell lymphoma. X4 is continuing to leverage its insights into CXCR4 biology at its corporate headquarters in Boston, Massachusetts and at its research facility in Vienna, Austria, to discover and develop additional product candidates. For more information, please visit www.x4pharma.com.

Forward Looking Statements:
This press release contains forward-looking statements within the meaning of 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 clinical development and therapeutic potential of mavorixafor. 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, uncertainties inherent in the initiation and completion of clinical trials and clinical development; the risk that trials and studies may be delayed, including, but not limited to, as a result of the effects of the ongoing COVID-19 pandemic, and may not have satisfactory outcomes; the risk that the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the potential adverse effects arising from the testing or use of mavorixafor; and other risks 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 November 4, 2021, 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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