X4 Pharmaceuticals Announces Strategic Prioritization of Programs and Resources Extending Cash Runway Through Expected Key Clinical and Regulatory Milestones

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Company now focused on advancing lead clinical candidate mavorixafor in chronic neutropenic disorders, including WHIM syndrome

Important mavorixafor clinical milestones anticipated in 2H 2022, including readout from pivotal Phase 3 4WHIM trial and Phase 1b results across a range of chronic neutropenic disorders

Recent capital raise, debt restructuring, revised company focus, and projected cost-reductions expected to extend cash runway into 3Q 2023 and accelerate mavorixafor WHIM NDA filing to early 2H 2023

BOSTON, July 20, 2022 (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 diseases of the immune system, today announced an update to its strategic priorities that includes streamlining resources to focus on advancing its lead clinical candidate, mavorixafor, in WHIM syndrome and other chronic neutropenic disorders, while progressing its clinical and pre-clinical oncology programs only via potential partnership(s).

Strategic Actions Summary:

- **Sharpening corporate focus and prioritizing resources** towards immunodeficiency-related clinical programs:
  - WHIM Syndrome: Data readout from the pivotal Phase 3 4WHIM trial of once-daily, oral mavorixafor in individuals 12 and older with WHIM (Warts, Hypogammaglobulinemia, Infections, & Myelokathexis) syndrome expected in the fourth quarter of 2022; U.S. New Drug Application (NDA) submission now anticipated early in the second half of 2023.
  - Chronic Neutropenic (CN) Disorders: Results from the Phase 1b study of mavorixafor in individuals with CN disorders anticipated in the third quarter of 2022; data expected to confirm broad potential of mavorixafor to treat CN disorders beyond WHIM, to support discussions with the U.S. Food & Drug Administration (FDA) on next steps, and to inform the regulatory path forward.
  - X4P-003: The candidate, a novel, small-molecule CXCR4 antagonist, has progressed to pre-clinical development and patent applications have been filed; further advancement dependent on the potential first approval of mavorixafor and lifecycle management of the company's product portfolio.

- **Progressing oncology efforts only via potential partnership(s)** for the company's clinical and pre-clinical programs:
  - Waldenström's macroglobulinemia (WM): Additional Phase 1b clinical data anticipated in early

August 2022 in patients with WM caused by both MYD88 and CXCR4 mutations; recently disclosed preclinical data highlight broad potential for treatment of leukemias and lymphomas in wild-type CXCR4; mavorixafor recently granted Orphan Drug Designation by the FDA for treatment of WM regardless of CXCR4 mutation status; new clinical trials in WM will now be subject to completing a strategic partnership.
    - X4P-002: IND-enabling toxicology studies are being finalized for the pre-clinical candidate, a novel, small-molecule CXCR4 antagonist that has demonstrated potential across a number of leukemias and lymphomas and that has been shown to cross the blood-brain barrier; IND filing will now be subject to completing a strategic partnership.

- **Reducing operating expenses** through discontinuation of further work on oncology programs and a workforce reduction of approximately 20%; measures are expected to result in an estimated $5 million reduction in expenditures in 2022 and a $20 million reduction in expenditures in 2023.

- **Extending X4's cash runway** into the third quarter of 2023 as a result of the company's recent $55 million capital raise, the recently completed amendment to X4's debt facility (which is expected to result in cash savings of up to $20 million over the interest-only period), and implementation of the announced cost reductions.

"The sharpening of our strategic focus towards developing treatments for those with chronic neutropenic disorders is intended to maximize the impact to all key stakeholders, including patients and shareholders," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We believe that commercializing mavorixafor and providing a new therapeutic option to individuals with life-threatening CN disorders has the potential to revolutionize the treatment landscape, which is currently only served by injectable therapies that have been associated with high-burden side effects and increased cancer risk in some patients. The robust data from our clinical trials continue to support the promise of mavorixafor to be a first-in-class, front-line, oral treatment for WHIM and other chronic neutropenic disorders. We now look forward to an exciting second half of 2022, with the expected presentations of clinical data from both our pivotal Phase 3 4WHIM trial and our Phase 1b study in chronic neutropenia."

Dr. Ragan concluded, "Parting with employees who have supported the company to this critical stage in our corporate evolution is a heavy decision; we would like to express our deepest gratitude to each of our X4 colleagues for their important contributions that have positioned X4 for long-term success."

About X4 Pharmaceuticals

X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company leading the discovery and development of novel therapies for people with diseases of the immune system. Our lead clinical 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 mobilization of white blood cells, we believe that mavorixafor has the potential to provide therapeutic benefit across a wide variety of diseases, including a range of chronic neutropenic disorders and certain types of cancer. The efficacy and safety of mavorixafor are being evaluated in a global Phase 3 clinical trial in patients with WHIM syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. We are also studying mavorixafor in two Phase 1b clinical trials – one as a monotherapy in patients with chronic neutropenia disorders including congenital, idiopathic, and cyclic neutropenia, and one in combination with ibrutinib in patients with Waldenström's macroglobulinemia (WM), a rare B-cell lymphoma. Further clinical development of mavorixafor in WM will now be subject to completing a strategic partnership as we focus our resources on advancing mavorixafor for the benefit of patients with chronic neutropenic disorders. We continue to leverage our insights into CXCR4 biology at our corporate headquarters in Boston, Massachusetts and at our research facility 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 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 and X4’s other product candidates or programs; X4’s possible exploration of additional opportunities for mavorixafor; the anticipated achievement of upcoming clinical milestones; the expected availability, content, and timing of clinical trial data; anticipated regulatory filings; clinical trial design, and the company’s cash runway and expenditures. 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 preclinical studies and clinical trials and clinical development; the risk that trials and studies may be delayed, including, but not limited to, as a result of the effect of the ongoing COVID-19 pandemic or delayed patient enrollment, 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 risk that initial or interim results from a clinical trial may not be predictive of the final results of the trial or the results of future trials; the potential adverse effects arising from the testing or use of mavorixafor or other product candidates; risks related to X4’s ability to raise additional capital or accurately estimate expenditures, 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 May 12, 2022, and in other filings X4 makes with the SEC from time to time.

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