X4 Pharmaceuticals Initiates Phase 1b Clinical Trial of Mavorixafor for the Treatment of Severe Congenital Neutropenia

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 5, 2019-- X4 Pharmaceuticals, Inc. (Nasdaq: XFOR), a clinical-stage biopharmaceutical company focused on the development of novel therapeutics for the treatment of rare diseases, today announced the initiation of a Phase 1b clinical trial of mavorixafor (X4P-001) for the treatment of Severe Congenital Neutropenia (SCN), a group of rare blood disorders characterized by abnormally low levels of neutrophils.

The Phase 1b trial is a 14-day, proof-of-concept trial designed to assess the safety and tolerability of daily, oral mavorixafor in participants with SCN and other selected congenital neutropenia disorders. In addition, the trial will evaluate the neutrophil response in this patient population as an independent agent or in combination with granulocyte-colony stimulating factor (G-CSF). The trial will enroll up to 45 patients in total.

“SCN often presents as a diagnostic challenge for physicians, and there is an unmet clinical need for targeted treatment options that may be better tolerated than existing therapies. Current treatments are limited to non-specific stimulation of the bone marrow with daily injections of G-CSF and antibiotics for infections, although G-CSF can cause bone pain as well as injection site reactions. Additionally, in rare instances, high chronic doses of G-CSF have been associated with myelodysplasia and acute myeloid leukemia in this patient population,” said Lynne Kelley, M.D., FACS, Chief Medical Officer of X4 Pharmaceuticals. “We are interested in exploring whether mavorixafor may provide a safe and well-tolerated treatment alternative for these patients.”

“We’re thrilled to initiate this clinical trial with mavorixafor for patients with SCN as we continue to validate the therapeutic potential of this potentially first-in-class drug candidate for the treatment of rare diseases,” said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. “We believe mavorixafor’s unique mechanism of action may have application across a variety of primary immunodeficiency diseases in which CXCR4-associated immune cell trafficking is disrupted. We look forward to advancing the clinical development of mavorixafor to potentially benefit SCN patients who currently have limited treatment options.”

About Severe Congenital Neutropenia

Severe Congenital Neutropenia (SCN) comprises a group of rare hematological diseases that may result from mutations in many different genes. These genes play a role in the maturation and function of neutrophils. In these patients, neutropenia may result from impaired maturation of neutrophils, increased cell death, or impaired function.[1] Affecting an estimated 2,000 to 3,000 people in the US and Europe, patients with SCN are prone to recurrent, often life-threatening infections beginning in their first months of life.[2,3] Patients with SCN may be treated with daily sub-cutaneous injections of G-CSF, which causes bone pain in a subset of patients and may increase the risk of myelodysplasia and acute myeloid leukemia.[1,4]

About Mavorixafor

X4 Pharmaceuticals’ lead product candidate, mavorixafor (X4P-001), is a potential first-in-class, once-daily, oral inhibitor of CXCR4, currently in a Phase 3 clinical trial for the treatment of WHIM syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. Mavorixafor has demonstrated proof-of-concept in WHIM syndrome in a Phase 2 clinical trial, including clinically meaningful increases in neutrophil and lymphocyte biomarker counts, as well as a trend of reduction in infection rates and wart burden, and a favorable safety profile. Mavorixafor was designated orphan drug status by the U.S. Food and Drug Administration in 2018 and by the European Commission in 2019 for the treatment of WHIM syndrome, and is also in development for Severe Congenital Neutropenia (SCN), Waldenström’s macroglobulinemia (WM), and clear cell renal cell carcinoma (ccRCC).

About X4 Pharmaceuticals

X4 Pharmaceuticals is developing novel therapeutics designed to improve immune cell trafficking to treat rare diseases, including primary immunodeficiencies and certain cancers. The company’s oral small molecule drug candidates antagonize the CXCR4 pathway, which plays a central role in immune surveillance. X4’s most advanced product candidate, mavorixafor (X4P-001), is in a global Phase 3 pivotal trial in patients with WHIM syndrome, a rare, inherited, primary immunodeficiency disease, and is currently also under investigation in combination with axitinib in an open-label Phase 1/2 clinical trial in clear cell renal cell carcinoma (ccRCC), with eight (12%) patients remaining on therapy over 12 months beyond the primary endpoint. X4 is further investigating mavorixafor in a Phase 1b clinical trial for the treatment of Severe Congenital Neutropenia (SCN). X4 is also planning to commence a clinical trial of mavorixafor with ibrutinib for the treatment of Waldenström’s macroglobulinemia (WM) in 2019. X4 was founded and is led by a team with extensive biopharmaceutical product development and commercialization expertise and is committed to advancing the development of innovative medicines on behalf of patients with limited treatment options. X4 is a global company that is headquartered in Cambridge, Massachusetts with research offices based in Vienna, Austria. 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. The

words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements include, but are not limited to, statements regarding X4’s plans for the development of mavorixafor (X4P-001), including regarding the Phase 1b clinical trial of mavorixafor alone or in combination with granulocyte-colony stimulating factor (G-CSF) in patients with Severe Congenital Neutropenia (SCN); the potential benefits of mavorixafor; the safety or efficacy of mavorixafor or its commercial opportunity in any target indication. These statements are subject to various risks and uncertainties, actual results could differ materially from those projected and X4 cautions investors not to place undue reliance on the forward-looking statements in this press release. These risks and uncertainties include, without limitation, the risk that trials and studies may be delayed and may not have satisfactory outcomes, potential adverse effects arising from the testing or use of mavorixafor or other product candidates, the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical studies or clinical trials will not be replicated or will not continue in ongoing or future studies or trials involving X4’s product candidates, and the risk that costs required to develop mavorixafor or other product candidates or to expand X4’s operations will be higher than anticipated. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, the risks and uncertainties described in the section entitled “Risk Factors” in X4’s most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as updated by X4’s Current Report on Form 8-K filed with the SEC on April 11, 2019, 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 subsequently occurring events or circumstances.

[1] Donadieu J, Fenneteau O, et al. Congenital neutropenia: diagnosis, molecular bases and patient management. Orphanet J Rare Dis. 2011 May 19;6:26. doi: 10.1186/1750-1172-6-26.

[2] Orphanet. Rare Diseases: Severe Congenital Neutropenia. ORPHA:42738. https://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=en&Expert=42738.

[3] Welte et al., Nature reviews, 2017.

[4] Freedman MH, Bonilla MA, et al. Myelodysplasia syndrome and acute myeloid leukemia in patients with congenital neutropenia receiving G-CSF therapy. Blood. 2000 Jul 15;96(2):429-36.

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