X4 Pharmaceuticals to Present Trial-in-Progress Poster on Phase 3 Study of Mavorixafor in Chronic Neutropenia at 66th ASH Annual Meeting & Exposition

December 4, 2024

Pivotal Phase 3 4WARD clinical trial is evaluating use of oral mavorixafor as monotherapy and in combination with injectable G-CSF

BOSTON, Dec. 04, 2024 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company driven to improve the lives of people with rare diseases of the immune system, today announced its upcoming presentations at the 66th American Society of Hematology (ASH) Annual Meeting and Exposition, including a trial-in-progress poster on the ongoing pivotal Phase 3 clinical trial exploring the use of mavorixafor, an oral CXCR4 antagonist, for the treatment of people with chronic neutropenia (CN). The meeting will take place December 7-10, 2024, in San Diego, CA.

"We're pleased to be sharing details of our ongoing Phase 3 clinical trial at ASH as we continue to recruit patients and activate study sites across the globe. The initiation of this trial was a significant milestone for the chronic neutropenia community that – unfortunately – hasn't seen any treatment innovation in nearly 30 years," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We look forward to connecting with clinicians at the ASH meeting and sharing more about our vision for mavorixafor as an oral option for the treatment of CN that could potentially reduce or replace the use of injectable G-CSF."

The Phase 3 4WARD trial is evaluating the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of injectable G-CSF) in people with congenital, acquired primary autoimmune or acquired primary idiopathic CN who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll 150 participants across 90 to 110 sites in 20 to 25 countries.

X4 will also be hosting a booth (#2106) at the conference to share information about the ongoing 4WARD clinical trial.

Additionally, in an oral presentation at the meeting, the company will present preclinical data on the potential for CXCR4 antagonism to correct peripheral blood neutropenia and bone marrow neutrophil accumulation caused by a loss-of-function in the CXCR2 pathway.

Poster Presentation Details:

Title: "Trials in Progress: A Phase 3 Study to Investigate Efficacy, Safety, and Tolerability of Mavorixafor in Participants with Congenital and Acquired Primary Autoimmune and Idiopathic Chronic Neutropenic Disorders"

Number: 3924.1

Date and Time: Monday, December 9, 2024: 6:00-8:00 p.m. PST

Location: Manchester Grand Hyatt San Diego, Halls G-H

Oral Presentation Details:

Title: "CXCR4 Antagonism Corrects Peripheral Neutropenia and Mature Neutrophil Accumulation in Bone Marrow in a Pharmacological Mouse Model of CXCR2 Loss-of-Function"

Number: 418

Date and Time: Sunday, December 8, 2024: 10:15 a.m. PST

Location: Manchester Grand Hyatt San Diego, Grand Hall D

About the 4WARD Global, Pivotal, Phase 3 Clinical Trial

The 4WARD trial is a global, pivotal Phase 3 clinical trial (NCT06056297) evaluating the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without G-CSF) in people with congenital, acquired primary autoimmune or acquired primary idiopathic chronic neutropenia who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll 150 participants with confirmed trough ANC levels less than 1,500 cells per microliter at baseline screening and histories of two or more serious and/or recurrent infections in the prior year. The primary endpoint of the trial is based on two outcome measures: annualized infection rate and positive ANC response.

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 our expertise in CXCR4 and immune system biology, we have successfully developed mavorixafor, which has received U.S. approval as XOLREMDI® (mavorixafor) capsules in its first indication. We are also evaluating the use of mavorixafor in additional potential indications. X4 corporate headquarters are in Boston, Massachusetts and our research center of excellence is in Vienna, Austria. For more information, please visit our website at www.x4pharma.com.

Company Contact:
José Juves
Head of Corporate & Patient Affairs
jose.juves@x4pharma.com

Investor Contact:
Daniel Ferry
Managing Director, LifeSci Advisors
daniel@lifesciadvisors.com
(617) 430-7576

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