X4 Pharmaceuticals Promotes Renato Skerlj, Ph.D., to Chief Scientific Officer

June 11, 2020

CAMBRIDGE, Mass., June 11, 2020 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals, Inc.** (Nasdaq: XFOR), a leader in the discovery and development of novel therapies targeting diseases resulting from dysfunction of the CXCR4 pathway, today announced the promotion of Renato Skerlj, Ph.D., to the position of Chief Scientific Officer. Dr. Skerlj is one of the scientific founders of X4 and co-inventor of X4's lead drug candidate, mavorixafor. He joined the company in September 2019 as Senior Vice President, Research and Development. As Chief Scientific Officer, Dr. Skerlj will lead all research and development functions and pre-clinical development activities.

"Dr. Skerlj's positive impact on X4 has been visible since day one," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "He has been integral to our success in the application and acceleration of our research and development capabilities and has effectively advanced our CXCR4-targeted candidates into the clinic. Dr. Skerlj's vision and proven track record in discovering and developing small molecule drugs for rare diseases significantly strengthens our senior leadership team. We look forward to his continued contributions as we progress mavorixafor through clinical development and expand our pipeline to support the long-term growth of the company."

Dr. Skerlj added, "This is an exciting time for X4 as we evaluate mavorixafor as a potential disease-modifying therapy for patients with WHIM syndrome and as a targeted treatment for genetically-defined blood cancers, such as Waldenström's macroglobulinemia. In my new role as Chief Scientific Officer, I look forward to our team continuing to leverage its robust in-house research and development capabilities as we advance our pipeline and expand into additional rare disease indications."

Dr. Skerlj has more than 25 years of experience leading the discovery and development of disease modifying small molecule drugs to treat genetically defined rare diseases. He has authored 67 publications, holds 52 patents and has advanced multiple drug candidates into clinical development.

Prior to joining X4, Dr. Skerlj held drug discovery and development leadership roles at Lysosomal Therapeutics. Previous to that, he was interim Head of Small Molecule Discovery at Genzyme and part of the executive team at AnorMED, a publicly traded company that was acquired by Genzyme in 2006. Dr. Skerlj is an accomplished drug developer, having contributed to the approval of two drugs; plerixafor, a CXCR4-targeted stem cell mobilizer approved by the U.S. Food and Drug Administration (FDA) in 2008, and ertapenem, an anti-bacterial approved by the FDA in 2001. Dr. Skerlj received his Ph.D. in synthetic organic chemistry from the University of British Columbia, and completed postdoctoral fellowships at the University of Oxford and Ohio State University.

About X4 Pharmaceuticals
X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company and a leader in the discovery and development of novel therapies for the treatment of diseases resulting from dysfunction of the CXCR4

pathway, with a focus on rare diseases and those with limited treatment options. The Company's lead candidate, mavorixafor, is a first-in-class, small molecule antagonist of chemokine receptor CXCR4 being developed as a once-daily oral therapy. X4 believes that inhibition of the CXCR4 receptor creates the potential for mavorixafor to provide therapeutic benefit across a wide variety of diseases, including primary immunodeficiencies and certain types of cancer. The efficacy and safety of mavorixafor, dosed once daily, is currently being evaluated in a global Phase 3 clinical trial in patients with WHIM syndrome, and in two Phase 1b clinical trials – in combination with ibrutinib in patients with Waldenström's macroglobulinemia, and as monotherapy in patients with severe congenital neutropenia (SCN). X4 is continuing to leverage its insights into CXCR4 biology at its corporate headquarters in Cambridge, Massachusetts and at its research facility in Vienna, Austria, and is discovering and developing 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 of mavorixafor in WHIM, Waldenström's macroglobulinemia and other indications. 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, the risks and uncertainties 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 7, 2020, 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.

Investors and Media:
Candice Ellis, 857-341-1043
Director, Corporate Communications & Investor Relations
Candice.Ellis@x4pharma.com

Source: X4 Pharmaceuticals