



## X4 Pharmaceuticals Appoints Renato Skerlj, Ph.D. as Senior Vice President of Research and Development

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 18, 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 appointment of Renato Skerlj, Ph.D., as Senior Vice President, Research and Development. Dr. Skerlj has twenty-five years of experience leading the discovery and development of small molecule drugs to treat rare diseases, cancer, infection and neurodegenerative diseases. In addition, he was one of the original founders of X4 Pharmaceuticals.

"Renato's deep scientific expertise in the research and development of innovative, genetically-targeted treatments, combined with his foundational knowledge of X4 and our novel CXCR4 platform, will be invaluable as we advance our pre-clinical product candidates and further expand our rare disease pipeline," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We are thrilled to have Renato join our senior leadership team given his instrumental role in the founding of X4."

"I am very pleased to become a part of X4's dedicated team of experienced and thoughtful leaders, especially during this exciting period of X4's R&D expansion and corporate growth. I look forward to leading our Vienna-based research team and applying my decades of strategic scientific experience to advance and deliver novel therapeutic options to patients with rare diseases," commented Dr. Skerlj.

Most recently, Dr. Skerlj held drug discovery and development leadership roles at Cambridge-based Lysosomal Therapeutics, Inc. Prior to that, he was interim Head of Small Molecule Discovery at Genzyme, and was part of the executive team at AnorMED, a publicly-traded company that was acquired by Genzyme in 2006. Dr. Skerlj is an inventor of both plerixafor, a 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, and has been responsible for delivering multiple drug candidates into early clinical research. He has authored 65 publications and holds 50 patents. 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 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, mavoxixafor (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 the Phase 2a portion of an open-label Phase 1/2 clinical trial in clear cell renal cell carcinoma (ccRCC). X4 is also planning to commence clinical trials of mavoxixafor in Severe Congenital Neutropenia (SCN) and 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](http://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 business strategy, including its clinical development plans. 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 any one or more of X4's product candidates will not be successfully developed, approved or commercialized, the risk that X4's ongoing or planned clinical trials and studies may be delayed, 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 X4's product candidates or to expand its 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.

Source: X4 Pharmaceuticals, Inc.

**Investors:**

Stephanie Carrington  
Westwicke, an ICR company  
646-277-1282

[Stephanie.Carrington@icrinc.com](mailto:Stephanie.Carrington@icrinc.com)

**Media:**

Darcie Robinson  
Westwicke, an ICR company  
203-919-7905

[Darcie.robinson@icrinc.com](mailto:Darcie.robinson@icrinc.com)