



X4 Pharmaceuticals Appoints Murray W. Stewart, M.D. to Board of Directors

April 2, 2019

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 2, 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 Murray Stewart, M.D., as an independent member of the X4 Board of Directors.

"We're very pleased to welcome Dr. Stewart to the X4 Board," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "His extensive global industry expertise, which spans research, clinical development, medical affairs, and regulatory strategy, combined with his strong biopharmaceutical leadership skills will be a tremendous asset as we transition to Phase 3 development with our lead product candidate, mavorixafor, and work to develop and commercialize innovative therapies for the treatment of rare diseases."

"X4 is at a critical juncture of growth and I am excited to join such a driven and dynamic team of leaders. As the company advances its pipeline of therapies for patients with rare genetic diseases and rare cancers, targeting the CXCR4 pathway, I look forward to contributing my experience and insights to help create long-term growth for a sustainable global rare disease business," commented Dr. Stewart.

Dr. Stewart currently serves as Chief Medical Officer of Rhythm Pharmaceuticals, Inc., a biopharmaceutical company focused on developing and commercializing therapies for the treatment of rare genetic disorders of obesity. Before joining Rhythm Pharmaceuticals in 2018, Dr. Stewart was Head of R&D for Novelon, where he oversaw global medical affairs for Juxtapid[®] and Myalept[®], two marketed products for rare metabolic diseases. Prior to that, Dr. Stewart was Chief Medical Officer at GlaxoSmithKline (GSK), with global responsibility for patient well-being across the vaccine, pharmaceutical, and consumer business units. During his 18-year career at GSK, Dr. Stewart held multiple research and development leadership roles, including Chief Medical Officer for the pharmaceutical business, Clinical Head of the Biopharma Unit, and Therapy Area Head for metabolic and cardiovascular diseases. Prior to his tenure with GSK, he worked as a consultant physician and honorary senior lecturer at the Diabetes Center in Newcastle upon Tyne in the U.K. Dr. Stewart is an M.D. from Southampton Medical School and is a Fellow of the Royal College of Physicians.

About X4 Pharmaceuticals

X4 Pharmaceuticals is developing novel therapeutics designed to improve immune cell trafficking to treat rare diseases, including primary immunodeficiencies and cancer. X4'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), will be commencing a global Phase 3 pivotal trial in patients with WHIM syndrome, a rare genetic, primary immunodeficiency disease, in the second quarter of 2019 and is currently also under investigation in a Phase 2a clinical trial in clear cell renal cell carcinoma. X4 was founded and is led by a team with extensive product development and commercialization expertise, including several former members of the Genzyme leadership team, and is located in Cambridge, Massachusetts. For more information, visit www.x4pharma.com.

Forward-Looking Statements

This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statement of historical facts, included in this press release regarding our strategy, future operations, and plans are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to plans for, or progress, scope, cost, duration or results or timing for the initiation, completion or availability of results of development of mavorixafor (X4P-001) or any of our other product candidates or programs, including regarding the Phase 3 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome, the target indication(s) for development, the size, design, population, location, conduct, objective, duration or endpoints of any clinical trial, or the timing for initiation or completion of or reporting of results from any clinical trial, the potential benefits of mavorixafor, or any other product candidate or program or the commercial opportunity in any target indication; or the potential benefits of orphan drug designation. Actual results or events could differ materially from the plans, intentions, expectations and projections disclosed in the forward-looking statements. Various important factors could cause actual results or events to differ materially from the forward-looking statements that X4 makes, including, but not limited to, 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 costs required to develop mavorixafor or other product candidates or to expand our operations will be higher than anticipated and other risks described in the "Risk Factors" section of the Registration Statement on Form S-4 filed by X4 Pharmaceuticals with the SEC and declared effective by the SEC on February 14, 2019. X4 does not assume any obligation to update any forward-looking statements, except as required by law.

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