X4 Pharmaceuticals Showcases Strategic Focus on WHIM Syndrome During 2020 Analyst Day Webinar

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–Market research confirms U.S. WHIM prevalence of 1,000 – 1,300 diagnosed patients, with an additional 800 – 2,400 potential undiagnosed patients–

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 7, 2020-- **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 hosted a virtual Analyst Day, *A Deep Dive into WHIM Syndrome*, the target disease of the company's lead Phase 3 candidate, mavorixafor. The replay of the full event, along with the slide presentation, will be available after 12 pm ET today on the company's website via this link.

The Analyst Day discussions provided details and background on WHIM (**W**arts, **H**ypogamma-globulinemia, **I**nfections, and **M**yelokathexis) syndrome, a rare, inherited immunodeficiency disease, and included a patient experience via video and an update on recently completed X4 market research on the prevalence of WHIM in the U.S. In addition, David C. Dale, M.D., a world expert in the translational research and clinical care of patients with WHIM syndrome, gave a formal presentation on his experiences with this rare form of immunodeficiency and with mavorixafor specifically, and participated in a virtual "fireside" chat. The event included a Q&A session with Dr. Dale and X4 senior management.

"It was our goal today to help the investment community visualize the 'face' of WHIM syndrome, a disease with severe and life-long medical complications, to better understand the challenges physicians face in treating WHIM, which we believe our late-stage Phase 3 candidate mavorxiafor could address, and to share our updated market research on the prevalence of the disease," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4. "In doing so, we also highlighted the scientific rationale and our Phase 2 trial results that support our increasing excitement as we advance mavorixafor towards being the first disease-modifying therapeutic available to treat the thousands of patients living with WHIM across the globe."

Dr. Dale also commented: "Since WHIM syndrome was first described in the 1960s, we've understood that patients experience decreased levels of both neutrophils and lymphocytes, resulting in significant immunodeficiency. As a result, patients experience what are often severe manifestations of disease, most often afflicted with bacterial infections such as sinusitis, otitis, and other upper respiratory infections, and viral infections, including the human papilloma virus, which typically cause debilitating warts to appear. Unfortunately, with no treatments currently available to treat the underlying cause of the syndrome, these patients experience critical and sometimes fatal complications – from chronic obstructive pulmonary disease, to hearing loss, to a higher risk of HPV-related cancers – highlighting a clear unmet medical need for disease-modifying therapeutics."

ANALYST DAY HIGHLIGHTS

Understanding the Severity of WHIM Syndrome

- The disease burden of WHIM is severe, with frequent respiratory infections that can lead to chronic lung disease, ear infections that may lead to hearing loss, and recurrent human papilloma virus(HPV) infections, often manifesting as warts that can increase the risk of anogenital and head and neck cancers.
- The complications and treatments associated with WHIM create a high demand for healthcare utilization. A study that examined insurance claims records of potential WHIM patients identified through an artificial intelligence algorithm demonstrated that 82% of these patients require respiratory services, 18% experience hearing loss, 51% had visited the emergency department and 44% had been hospitalized within the last year.

The WHIM Treatment Landscape: Clear Unmet Medical Need

- Current therapeutics used to treat WHIM are limited in efficacy, only treating symptoms and not addressing the underlying cause of the disease.
- X4 is developing mavorixafor, a first-in-class antagonist of CXCR4 that has demonstrated high potency and selectivity for inhibiting CXCR4 signaling and restoring the trafficking of neutrophils and lymphocytes to improve immunosurveillance.
- Informed by a successful Phase 2 trial, X4 has is conducting an ongoing global Phase 3 trial for mavorixafor in genetically confirmed WHIM patients; mavorixafor recently received Breakthrough Therapy Designation for WHIM in the U.S. suggesting recognition of the positive Phase 2 clinical data and the high unmet medical need in WHIM patients.

New Market Research Supports Heightened Market Opportunity

- During 2019, X4 conducted a broad, quantitative online survey of physicians, the results of which support the conclusion that there are between 1,000 and 1,300 *diagnosed* WHIM patients in the U.S. today.
- X4 also performed research using artificial intelligence, interrogating a database of more than 300 million anonymized patient records that spanned 10 years of insurance claims to help identify patient records that reflect the ‘face’ of WHIM. This robust algorithm then searched the database, identifying between 800 and 2,400 *additional* potential but unconfirmed and undiagnosed WHIM patients.
- Based on this research, X4 has provided updated guidance on its estimated range of WHIM prevalence to be between approximately 1,300 diagnosed patients and up to 3,700 in total, which includes potential undiagnosed WHIM patients, a significant increase from its prior estimate of approximately 1,000 WHIM patients in the U.S.

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 – as monotherapy in patients with Severe Congenital Neutropenia (SCN) and in combination with ibrutinib in patients with Waldenström’s macroglobulinemia. 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 X4’s clinical trials relating to WHIM, along with its prevalence. These statements are subject to various risks and uncertainties including, 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, and the risk that costs required to develop 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. 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 12, 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.

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