X4 Pharmaceuticals and Norgine Enter into Exclusive Licensing Agreement to Commercialize Mavorixafor in Europe, Australia, and New Zealand

January 13, 2025

X4 to receive a €28.5 million upfront payment and up to €226 million in potential regulatory and commercial milestone payments in addition to tiered, double-digit royalties up to the mid-twenties

Upfront non-dilutive funds strengthen X4's balance sheet as enrollment ramps up in the company's global Phase 3 clinical trial in chronic neutropenia

Agreement underscores Norgine's commitment to bring transformative therapies to patients in need in these key strategic territories

BOSTON and UXBRIDGE, United Kingdom, Jan. 13, 2025 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company driven to improve the lives of people with rare diseases of the immune system, and **Norgine**, a leading European specialist pharmaceutical company, today announced that they have entered into an exclusive licensing and supply agreement under which Norgine will commercialize mavorixafor in Europe, Australia, and New Zealand following regulatory approvals.

Mavorixafor is a selective CXCR4 receptor antagonist approved in the U.S. and marketed by X4 as XOLREMDI®, an oral, once-daily treatment for patients 12 years of age and older with WHIM syndrome, a rare primary immunodeficiency. X4 expects to announce shortly the submission of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for mavorixafor in the treatment of WHIM syndrome, for which it has been granted Orphan Drug Designation by both the EMA and the U.S. Food and Drug Administration. X4 is also developing mavorixafor to treat chronic neutropenia (CN) and is currently conducting a global, pivotal Phase 3 clinical trial in certain CN disorders.

"This strategic agreement is a significant milestone for X4 as we seek to maximize the global potential of mavorixafor and bring in funding for our ongoing global, Phase 3 trial in chronic neutropenia," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We believe Norgine to be the ideal partner due to their impressive infrastructure and successful commercialization track record in specialty pharmaceuticals, as well as a shared focus on putting patients first. We look forward to expanding access to mavorixafor and continuing to address the unmet needs of those with rare immune disorders."

Janneke van der Kamp, Chief Executive Officer of Norgine, commented on the announcement: "We are very pleased to partner with X4 in this underserved, rare disease space and expand access to mavorixafor to patients in Europe, Australia, and New Zealand. If approved by the respective regulatory bodies, mavorixafor would be the first treatment targeting a key underlying cause of WHIM syndrome, a disease characterized by low white blood cell counts and frequent and/or serious infections. Through this agreement, we continue to expand our innovative portfolio of products and our expertise across rare diseases and specialty markets. This important milestone for our company further underscores Norgine's position as a partner of choice across Europe and ANZ."

Under the terms of the license and supply agreement, X4 will receive €28.5 million in upfront consideration and up to €226 million contingent upon the achievement of certain regulatory and commercial milestones, in addition to escalating double-digit royalties of up to the mid-twenties on any future net sales in the licensed territories. X4 and Norgine will collaborate closely on regulatory filings, with X4 continuing to be responsible for the ongoing global, pivotal Phase 3 4WARD clinical trial evaluating mavorixafor in CN. Norgine will be responsible for all market access and commercialization activities and will eventually hold all marketing authorizations in the licensed territories. X4 will manufacture and supply mavorixafor to Norgine.

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 expertise in CXCR4 and immune system biology, X4 has successfully developed mavorixafor, an orally available CXCR4 antagonist that is currently being marketed in the U.S. as XOLREMDI® in its first indication. The company is also evaluating additional uses of mavorixafor and is conducting a global, pivotal Phase 3 clinical trial (4WARD) in people with certain chronic neutropenic disorders. X4 is headquartered in Boston, Massachusetts and operates a research center of excellence in Vienna, Austria. For more information, please visit www.x4pharma.com.

About Norgine
Norgine is a uniquely positioned, specialty pharmaceutical and consumer healthcare company, with more than €500 million of annual revenues and a 120-year track record of bringing life-changing products to patients and consumers across their core markets of Western Europe, Australia, and New Zealand. Today's Norgine is a nimble, innovative, and high-performing company that has been transformed by a relentless focus on operational excellence to do the right thing by patients, push boundaries, and take strides into new therapeutic areas. The company's integrated approach – strong commercial capabilities, deep medical, regulatory and clinical expertise, in-house manufacturing, robust supply networks, and best-in-class enabling functions – ensures delivery of high-quality, transformative medicines quickly and effectively to more than 25 million patients annually.

Forward Looking Statements
This press release contains forward-looking statements within the meaning of applicable securities laws, including 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, implied or express statements regarding the initiation, timing, progress, and results of X4’s current and future preclinical studies and clinical trials and related preparatory work and the period during which the results of trials will become available, as well as X4’s research and development programs; the timing and anticipated interactions with regulatory authorities and any related approvals for mavorixafor in Europe, Australia, and New Zealand; the potential market opportunity for mavorixafor; the anticipated strategic benefits of X4’s exclusive licensing agreement with Norgine and of any current or future collaborations; and the mission and goals for X4’s business. Any forward-looking statements in this press release are based on management's current expectations and beliefs. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond X4’s control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including the risks that: X4 may have difficulty establishing and maintaining an effective sales and marketing organization or suitable third-party alternatives for any approved products; X4 may not be able to obtain or maintain orphan drug designation or exclusivity for X4’s drug candidates, which could limit the potential profitability of X4’s product candidates; X4 may not be able to obtain regulatory approval for, or successfully commercialize, mavorixafor or any other product candidate for other chronic neutropenic disorders or any other potential indication; the expected availability, content, and timing of clinical data from X4's ongoing clinical trials of mavorixafor may be delayed or unavailable, including X4’s ongoing Phase 3 clinical trial; the design and rate of enrollment for clinical trials, including the current design of a Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders may not enable successful completion of the trial(s); the commercial opportunity for mavorixafor in chronic neutropenic disorders may be smaller than anticipated; X4 may be unable to obtain and maintain regulatory approvals; uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; the regulatory review and approval processes of the FDA and comparable foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable, and if X4 is ultimately unable to obtain regulatory approval for X4’s product candidates, including additional indications for mavorixafor, X4’s business will be substantially harmed; initial or interim results from a clinical trial may not be predictive of the final results of the trial or the results of future trials, including assessing the ability of mavorixafor monotherapy to durably increase absolute neutrophil count in patients with chronic neutropenia; adverse safety effects arise from the testing or use of X4’s product and product candidates; the need to align with X4’s collaborators may hamper or delay X4’s development and commercialization efforts or increase X4’s costs; X4’s business may be adversely affected and their costs may increase if any of X4’s key collaborators fails to perform its obligations or terminates the collaboration; the internal and external costs required for X4’s ongoing and planned activities, and the resulting impact on expense and use of cash, may be higher than expected, which may cause the company to use cash more quickly than expected or to change or curtail some of X4’s plans or both; and other risks and uncertainties, including those described in the section entitled “Risk Factors” in X4’s Quarterly Report on X4’s Form 10-Q filed with the Securities and Exchange Commission (SEC) on November 13, 2024, 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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