X4 Pharmaceuticals Provides Corporate Update and Reports Fourth Quarter and Full Year 2019 Financial Results

March 12, 2020

– Pivotal Phase 3 clinical trial of lead candidate mavorixafor in WHIM syndrome ongoing with top-line results expected in the second half of 2021 –

– Initial Phase 1b results in ongoing Waldenström's macroglobulinemia (WM) and Severe Congenital Neutropenia (SCN) trials expected in the second half of 2020 –

– Conference call today at 8:30 a.m. ET –

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- 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 provided a corporate update and reported financial results for the fourth quarter and full year ended December 31, 2019.

"2019 was a remarkable year for X4, with significant achievements across the entire organization," said Paula Ragan, Ph.D., President and Chief Executive Officer of the Company. "We made important progress advancing our lead therapeutic candidate mavorixafor into pivotal Phase 3 development for patients with WHIM syndrome, while also initiating two proof-of-concept clinical trials in SCN and Waldenström's, and strengthening both our leadership team and Board of Directors. We're now well positioned for the years ahead as we focus on near-term clinical trial execution and prepare for key value creation events in our quest to bring new transformative therapies to patients with rare diseases."

Key 2019 Program Achievements and Upcoming Milestones

- **Initiated Pivotal Phase 3 Clinical Trial of Mavorixafor for the Treatment of WHIM Syndrome – June 2019**: The 4WHIM trial is a pivotal Phase 3 global clinical trial of mavorixafor for the treatment of WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, inherited, primary immunodeficiency disease.
  - Top-line data from the trial are expected in the second half of 2021.
  - Company to hold Analyst Day, which will also be webcast, on April 7, 2020 to discuss strategic focus on WHIM.
  - Phase 2 open-label extension study data update expected in mid-2020.
- **Received Orphan Drug Designation from the European Commission for Mavorixafor for the Treatment of WHIM Syndrome – July 2019**
  - Received Scientific Advice from the European Medicines Agency to align the Phase 3 registration trial globally.
- **Announced Positive Data from Phase 2a Trial of Mavorixafor in Clear Cell Renal Cell Carcinoma (ccRCC) Patients – September 2019**: Data presented at the European Society for Medical Oncology (ESMO) demonstrated that mavorixafor, in combination with axitinib, yielded clinically meaningful

improvements in median progression-free survival (mPFS) in a heavily pretreated advanced ccRCC patient population, and was generally well tolerated.

- **Initiated Phase 1b Clinical Trial of Mavorixafor in Patients with Severe Congenital Neutropenia (SCN) – November 2019**: The Phase 1b trial is a 14-day, proof-of-concept trial designed to assess the safety and tolerability of daily, oral mavorixafor in up to 45 patients with SCN and other selected congenital neutropenia disorders. The trial will evaluate the neutrophil response and genetic profiles in this patient population as an independent agent or in combination with granulocyte-colony stimulating factor (G-CSF).
    - Initial data from this trial are expected in the second half of 2020.
- **Received Breakthrough Therapy Designation from U.S. FDA for Mavorixafor for the Treatment of WHIM Syndrome – November 2019**: Highlighting the severity of the disease and the relevance of mavorixafor's Phase 2 clinical trial data to support the drug's role as a potential disease-modifying therapeutic option to this underserved patient population, Breakthrough Therapy Designation could expedite the development and regulatory review of mavorixafor.
- **Initiated Phase 1b Clinical Trial of Mavorixafor in Combination with Ibrutinib in Patients with Waldenström's Macroglobulinemia – December 2019**: The Phase 1b multi-center, open-label, dose-escalation clinical trial is designed to assess the safety and tolerability of mavorixafor in combination with ibrutinib as well as to obtain certain efficacy signals in patients with Waldenström's macroglobulinemia, a rare form of non-Hodgkin's lymphoma, who have acquired a "gain of function" mutation in CXCR4 in addition to the MYD88 mutation, which is a hallmark of Waldenström's.
    - Initial data from this trial are expected in the second half of 2020.
- **Granted New Composition of Matter Patent by U.S. PTO for Mavorixafor – February 2020**: Patent expected to provide exclusivity through 2038.

Key 2019 Corporate Highlights

- **Shares of X4 Pharmaceuticals Began Trading on the Nasdaq Capital Market Under the Symbol "XFOR" – March 2019**
- **Completed Two Public Stock Offerings that Raised Gross Proceeds of $150.8 Million:** The first offering was completed in April and the second offering was completed in November.
- **Announced Multiple Development and Collaboration Agreements:**
    - **Announced Collaboration with The Leukemia & Lymphoma Society (LLS) – May 2019:** Mavorixafor selected for LLS' Therapy Acceleration Program® (TAP), a strategic initiative creating an alliance to develop mavorixafor for patients with Waldenström's macroglobulinemia.
    - **Announced Partnership with Invitae to Provide No-Cost Genetic Testing to Patients – June 2019**: The collaborative PATH4WARD program provides greater access to faster and earlier diagnosis for individuals who may carry genetic mutations known to be associated with WHIM syndrome and SCN.
    - **Entered into Oncology Development and Commercialization Agreement with Abbisko for Mavorixafor in Greater China – July 2019**: Provided Abbisko Therapeutics with the exclusive rights in China, Taiwan, Hong Kong, and Macau to develop and commercialize mavorixafor in combination with checkpoint inhibitors or other agents in solid tumor oncology indications.
- **Strengthened Leadership Team and Board of Directors Throughout the Year**: Appointed E. Lynne Kelley, M.D., as Chief Medical Officer and Murray W. Stewart, M.D., to Board of Directors in April 2019; appointed Renato Skerlj, Ph.D., as Senior Vice President of Research and Development and William E. Aliski to Board of Directors in September 2019; appointed Derek Meisner, J.D., as General Counsel in November 2019.

Financial Results

- **Cash, Cash Equivalents & Restricted Cash:** X4 had $128.1 million in cash, cash equivalents and restricted cash, as of December 31, 2019. We expect that our cash and cash equivalents will fund our

operations into early 2022.

- **Research and Development Expenses** were $7.1 million for the fourth quarter of 2019, and $30.2 million for the year ended December 31, 2019, as compared to $4.7 million and $20.3 million for the comparable periods in 2018, respectively.
- **General and Administrative Expenses** were $3.9 million for the fourth quarter of 2019, and $17.6 million for the year ended December 31, 2019, as compared to $3.4 million and $8.7 million for the comparable periods in 2018, respectively.
- **Net Loss:** X4 reported a net loss of $10.8 million for the fourth quarter of 2019, and a net loss of $52.8 million for the year ended December 31, 2019, as compared to a net loss of $11.3 million and a net loss of $33.3 million for the comparable periods in 2018, respectively. Net loss of $52.8 million for the year ended December 31, 2019 includes $3.9 million of non-cash losses related to the sale of in-process research and development intangible assets.

Conference Call and Webcast

The Company will host a webcast and conference call to discuss its fourth quarter and full year 2019 results and provide an update on recent corporate activities today at 8:30 a.m. Eastern Time. The webcast will be accessible under “Events & Presentations” in the Investors page of the Company’s website at www.x4pharma.com. Individuals can participate in the conference call by dialing (866) 721-7655 (domestic) or (409) 216-0009 (international), followed by the conference ID: 3816258.

About Mavorixafor

Mavorixafor is an investigational, oral, targeted therapy that antagonizes the chemokine receptor CXCR4 via allosteric inhibition. The CXCR4 receptor plays a key role in enabling the trafficking of immune cells and effective immunosurveillance. Over-stimulation of CXCR4 results in various immune-system deficiencies. X4 Pharmaceuticals is currently investigating mavorixafor as a once-daily treatment in a global Phase 3 pivotal trial in patients with WHIM syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. The FDA has granted Breakthrough Therapy Designation to mavorixafor for the treatment of WHIM syndrome. Mavorixafor is being developed in two proof-of-concept Phase 1b clinical trials – as monotherapy in patients with Severe Congenital Neutropenia (SCN), a group of rare blood disorders characterized by abnormally low levels of white blood cells, and in combination with ibrutinib in patients with Waldenström’s macroglobulinemia, a form of non-Hodgkin’s lymphoma, who have acquired mutations in the CXCR4 receptor.

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, oral, small molecule antagonist of chemokine receptor CXCR4. 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 the clinical development of mavorixafor, Waldenström’s, SCN or X4's other product candidates or programs, as well as statements regarding intellectual property protection and cash flows. 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 11, 2019, 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 new events or circumstances, except as required by law.

X4 PHARMACEUTICALS INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except per share amounts)

	Year Ended December 31,		Three Months Ended December 31,	
	2019	**2018**	**2019**	**2018**
Operating expenses:				
Research and development	$ 30,163	$ 20,346	$ 7,065	$ 4,689
General and administrative	17,640	8,739	3,914	3,365
Loss on transfer of nonfinancial assets	3,900	-	(104)	-
Total operating expenses	51,703	29,085	10,875	8,054
Loss from operations	(51,703)	(29,085)	(10,875)	(8,054)
Other income (expense), net	(1,104)	(4,200)	38	(3,228)
Net loss	(52,807)	(33,285)	(10,837)	(11,282)
Adjustments related to convertible preferred stock	(592)	(3,022)	-	(756)
Net loss attributable to common stockholders	$ (53,399)	$ (36,307)	$ (10,837)	$ (12,038)
Net loss per share attributable to common stockholders- basic and diluted	$ (4.63)	$ (79.15)	$ (0.66)	$ (25.33)
Weighted average common shares outstanding-basic and diluted	11,530	459	16,466	475

X4 PHARMACEUTICALS INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,	Three Months Ended

	2019	December 31, 2019
Net loss	$ (52,807)	$ (10,837)
Adjustments to reconcile net loss to net cash used in operating activities	7,988	856
Changes in operating assets and liabilities	(3,236)	740
Net cash used in operating activities	(48,055)	(9,241)
Net cash provided by investing activities	27,232	21
Net cash provided by financing activities	140,661	60,284
Impact of foreign exchange on cash, cash equivalents and restricted cash	(250)	16
Net increase in cash, cash equivalents and restricted cash	$ 119,588	$ 51,080
Cash, cash equivalents and restricted cash at beginning of period	$ 8,498	$ 77,006
Cash, cash equivalents and restricted cash at end of period	$ 128,086	$ 128,086

X4 PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands)

	December 31, 2019	September 30, 2019
Current assets:		
Cash and cash equivalents	$ 126,184	$ 76,251
Research and development incentive receivable	1,998	1,730
Prepaid expenses and other current assets	1,096	1,234
Total current assets	129,278	79,215
Property and equipment, net	403	348
Goodwill	27,109	27,109
Right-of-use assets	1,959	2,110
Other assets	1,949	755
Total assets	$ 160,698	$ 109,537
Current liabilities:		
Accounts payable	$ 2,088	$ 1,704
Accrued expenses	6,461	5,697
Current portion of lease liability	898	878
Total current liabilities	9,447	8,279
Long-term debt, including accretion, net of discount and current portion	20,097	19,986
Lease liabilities	1,918	2,147
Other liabilities	16	18

Total liabilities	31,478	30,430
Total stockholders' equity	129,220	79,107
Total liabilities and stockholders' equity	$ 160,698	$ 109,537

View source version on businesswire.com: https://www.businesswire.com/news/home/20200312005210/en/

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

Source: X4 Pharmaceuticals, Inc.