X4 Pharmaceuticals Reports Third Quarter 2020 Financial Results and Provides Corporate Update

November 5, 2020

WHIM Phase 2 data published in 'Blood' - the Official Journal of the American Society of Hematology

Granted Fast Track Designation by FDA for mavorixafor in WHIM syndrome

Key leadership appointments including CSO and Board Director

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

BOSTON, Nov. 05, 2020 (GLOBE NEWSWIRE) -- 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 reported financial results for the third quarter ended September 30, 2020. The company also provided an update on its lead investigational candidate, mavorixafor, a novel small molecule in a Phase 3 clinical trial for patients with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome and in two Phase 1b trials in patients with Waldenström's macroglobulinemia and Severe Congenital Neutropenia (SCN), respectively.

"The third quarter was highlighted by the publication of our positive Phase 2 safety and efficacy data for mavorixafor in WHIM syndrome in the prestigious journal, *Blood*, which, we believe, continues to demonstrate the significant potential of our lead candidate to treat this patient population," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Further, in early October, we announced that the U.S. Food and Drug Administration (FDA) granted mavorixafor Fast Track Designation for the treatment of WHIM, facilitating expedited review of mavorixafor as we proceed through clinical development. Both achievements enhance our confidence in mavorixafor's potential to deliver the first disease-modifying therapy for this undertreated patient population. We were also thrilled to enhance our leadership team and expertise through the additions of Art Taveras, Ph.D., as Chief Scientific Officer and new board member, Alison Lawton."

Dr. Ragan continued, "While the operating environment remains challenging due to the ongoing COVID-19 pandemic, we continue to advance our mavorixafor development programs and enroll patients into our clinical trials. We anticipate initial data from our Phase 1b Waldenström's macroglobulinemia trial in the first half of 2021, along with initial data from our Phase 1b trial in patients with SCN in 2021, and top-line results from our Phase 3 trial of mavorixafor in WHIM syndrome in 2022. As we continue to achieve important clinical progress," Dr. Ragan concluded, "we are supported by a strong balance sheet that we expect will drive our current strategic and development plans into early 2022."

Recent Highlights

- **Published comprehensive data from the Phase 2 and open label extension study of mavorixafor in WHIM syndrome in *Blood*, the official journal of the American Society of**

Hematology, expanding on previously presented data. New results included long-term patient-level data regarding the effect on neutrophils and lymphocytes and the effect of increasing doses of mavorixafor on total white blood cell counts and monocytes. In addition, the manuscript provided the most up-to-date pharmacokinetic data and presented a detailed analysis of the clinical improvements in infection rates and warts demonstrated with extended mavorixafor treatment.

- **Granted Fast Track Designation by the FDA for mavorixafor in WHIM syndrome.** Through the Fast Track program, X4 will be eligible for more frequent meetings with the FDA to discuss mavorixafor's development plan, protocols and clinical data that would support its potential approval for WHIM. Mavorixafor was previously granted Breakthrough Therapy Designation by the FDA, as well as Orphan Drug status by the FDA and the European Commission for the treatment of WHIM syndrome

- **Appointed Art Taveras, Ph.D., as Chief Scientific Officer.** Dr. Taveras is a seasoned Chief Scientific Officer with experience in the discovery of novel, next-generation CXCR2 antagonists for the treatment of cancer. Previously, Dr. Taveras was VP, Small Molecule Drug Discovery at Biogen, President and CSO of ShangPharma ChemPartners, and CSO of CoMET Therapeutics. With his expertise in chemokine-related chemistries, Dr. Taveras is well suited to lead X4's R&D initiatives and its continued evolution into a global rare disease company.

- **Expanded Board of Directors through the appointment of biopharmaceutical industry veteran, Alison Lawton**. Ms. Lawton strengthens the board's drug development, manufacturing and commercialization experience and brings a unique understanding of X4's core scientific and corporate goals, having previously served as a consulting Chief Operating Officer to the company and as a member of X4's corporate advisory board. Ms. Lawton had an over 20 year tenure at Genzyme Corporation (now Sanofi Genzyme) including Senior Vice President and General Manager of Sanofi Biosurgery, a $750 million business and led global functional organizations, including regulatory affairs and quality systems, public policy, health outcomes and strategic pricing, product safety and risk management. Ms. Lawton most recently served as CEO of Kaleido Biosciences and currently serves as an independent director on the Board of ProQR Therapeutics NV.

Third Quarter 2020 Financial Results

- **Cash, Cash Equivalents & Restricted Cash:** X4 had $90.7 million in cash, cash equivalents and restricted cash, as of September 30, 2020. X4 continues to expect that its cash and cash equivalents will fund company operations into early 2022. Note this guidance does not include the $25 million in potential and contingent proceeds from our Hercules Debt Facility or any cash exercise proceeds from investors holding our outstanding warrants.

- **Research and Development Expenses** were $11.4 million for the third quarter ended September 30, 2020, as compared to $8.6 million for the comparable period in 2019. R&D expenses include $1.0 million of certain non-cash expenses for the quarter ended September 30, 2020.

- **General and Administrative Expenses** were $5.6 million for the third quarter ended September 30, 2020, as compared to $4.4 million for the comparable period in 2019. G&A expenses include $1.4 million of certain non-cash expenses for the quarter ended September 30, 2020.

- **Net Loss:** X4 reported a net loss of $17.4 million for the third quarter ended September 30, 2020, as compared to a net loss of $17.7 million for the comparable period in 2019. Net loss includes $2.5 million of certain non-cash expenses for the quarter ended September 30, 2020. Net loss in the third quarter of 2019 included a $4.0 million loss on the sale of non-financial assets.

Conference Call and Webcast
The Company will host a conference call and webcast today at 8:30 a.m. ET to discuss these financial results and business highlights. The conference call can be accessed by dialing (866) 721-7655 from the United States or (409) 216-0009 internationally, followed by the conference ID: 1096977. The live webcast can be accessed on the investor relations section of X4 Pharmaceuticals' website at www.x4pharma.com. Following the completion of the call, a webcast replay of the conference call will be available on the website.

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 – in combination with ibrutinib in patients with Waldenström's macroglobulinemia, and as monotherapy in patients with Severe Congenital Neutropenia. X4 is continuing to leverage its insights into CXCR4 biology at its corporate headquarters in Boston, 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, WHIM, Waldenström's macroglobulinemia, SCN or X4's other product candidates or programs, and fast track designation. 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on August 4, 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
License revenue	$ —	$ —	$3,000	$ —
Operating expenses:				
Research and development	11,381	8,589	29,634	23,098
General and administrative	5,599	4,383	15,585	13,726

Loss on transfer of non-financial assets	—	4,004	—	4,004
Total operating expenses	16,980	16,976	45,219	40,828
Loss from operations	(16,980)	(16,976)	(42,219)	(40,828)
Other expense, net	(469)	(738)	(1,364)	(1,142)
Loss before provision for income taxes	(17,449)	(17,714)	(43,583)	(41,970)
Provision for income taxes	—	—	148	—
Net loss	(17,449)	(17,714)	(43,731)	(41,970)
Adjustments related to convertible preferred stock	—	—	—	(592)
Net loss attributable to common stockholders	$(17,449)	$(17,714)	$(43,731)	$(42,562)
Net loss per share attributable to common stockholders- basic and diluted	$(0.87)	$(1.22)	$(2.18)	$(4.31)
Weighted average common shares outstanding-basic and diluted	20,085	14,562	20,035	9,866

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

	Nine months ended September 30,	
	2020	**2019**
Net loss	$(43,731)	$(41,970)
Adjustments to reconcile net loss to net cash used in operating activities	5,198	7,132
Changes in operating assets and liabilities	(2,781)	(3,976)
Net cash used in operating activities	(41,314)	(38,814)
Net cash (used in) provided by investing activities	(1,060)	27,211
Net cash provided by financing activities	4,765	80,377
Impact of foreign exchange on cash, cash equivalents and restricted cash	240	(266)
Net (decrease) increase in cash, cash equivalents and restricted cash	$(37,369)	$68,508
Cash, cash equivalents and restricted cash at beginning of period	$128,086	$8,498
Cash, cash equivalents and restricted cash at end of period	$90,717	$77,006

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

	September 30, 2020	**December 31, 2019**
Current assets:		
Cash and cash equivalents	$88,812	$126,184
Research and development incentive receivable	629	1,998

Prepaid expenses and other current assets	4,472	1,096
Total current assets	93,913	129,278
Property and equipment, net	1,334	403
Goodwill	27,109	27,109
Right-of-use assets	8,222	1,959
Other assets	2,548	1,949
Total assets	$133,126	$160,698
Current liabilities:		
Accounts payable	$3,891	$2,088
Accrued expenses	8,532	6,461
Current portion of lease liability	756	898
Total current liabilities	13,179	9,447
Long-term debt, including accretion, net of discount	25,537	20,097
Lease liabilities	4,677	1,918
Other liabilities	25	16
Total liabilities	43,418	31,478
Total stockholders' equity	89,708	129,220
Total liabilities and stockholders' equity	$133,126	$160,698

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