



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

July 30, 2020

Presentation of positive clinical data at EHA 2020 supporting the ongoing Phase 3 trial in WHIM syndrome

Initial data expected later this year from Phase 1b clinical trial in Waldenström's macroglobulinemia

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

CAMBRIDGE, Mass., July 30, 2020 (GLOBE NEWSWIRE) -- [X4 Pharmaceuticals, Inc.](https://www.x4pharm.com) (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 second quarter ended June 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 (WM) and Severe Congenital Neutropenia (SCN).

"Despite the challenges posed by the ongoing COVID-19 pandemic, we achieved significant progress during the second quarter, benefiting from two key events related to the WHIM syndrome indication for mavorixafor, while continuing to advance our program in the Waldenström's indication," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "At our Analyst Day in early April, we presented market research data that supported significantly increasing our disease prevalence estimate for WHIM in the U.S., and during our presentation at the European Hematology Association (EHA) Annual Congress in June, we highlighted Phase 2 mavorixafor data that strengthen our confidence in mavorixafor's potential to be a disease-modifying therapy in patients with WHIM syndrome and in the Phase 3 trial design."

Dr. Ragan continued, "While our public presentations during the first half of 2020 focused primarily on WHIM syndrome, we expect that the second half of 2020 will see us increase exposure to our program in patients with Waldenström's macroglobulinemia, a rare form of lymphoma, where we look forward to announcing initial Phase 1b clinical results towards the end of the year." The ongoing Phase 1b clinical trial is expected to enroll between 12 and 18 patients with WM and is a multi-center, open-label, dose-escalation clinical trial assessing the safety and tolerability of mavorixafor in combination with ibrutinib. The trial is being conducted as part of a collaboration with The Leukemia & Lymphoma Society to accelerate the development of mavorixafor for the treatment of WM.

"As we await these important results," Dr. Ragan concluded, "we continue to expect top-line Phase 3 results of mavorixafor in WHIM syndrome in 2022 and initial data from our Phase 1b trial of mavorixafor in patients with SCN in 2021. In light of the continued uncertainties surrounding COVID-19, we intend to provide further clarity around these timelines as soon as is practicable."

Recent Highlights

- **Increased Guidance on Prevalence of WHIM at Virtual Analyst Day**, based on in-depth, internal market research indicating the range of diagnosed and undiagnosed WHIM patients in the United States to be greater than 3,500, a significant increase from the prior estimate of approximately 1,000 patients diagnosed with WHIM syndrome.
- **Presented Positive Data from the Phase 2 Open-Label Extension Study of Mavorixafor in WHIM Syndrome at EHA 2020**, supporting the selection of 400 mg once-daily and time above threshold for absolute neutrophil counts (TAT_{ANC}) as the dose and primary endpoint in the Phase 3 trial, respectively, and long-term favorable tolerability. At the median follow-up of 16.5 months, data revealed sustained, dose-dependent increases in WBC (white blood cells), ANC (absolute neutrophil count), and ALC (absolute lymphocyte count), with higher doses of mavorixafor shown to increase the TAT_{ANC} at least 4.5-fold versus lower doses. In patients treated for at least 6 months, mavorixafor also significantly decreased the yearly rate of infections versus the 12 months prior to treatment and effected a 75% reduction in cutaneous warts versus baseline.
- **Promoted Renato Skerlj, Ph.D., to the position of Chief Scientific Officer**. Dr. Skerlj, one of the scientific founders of X4 and co-inventor of mavorixafor, has more than 25 years of experience leading the discovery and development of disease-modifying small molecule drugs to treat genetically defined rare diseases. In this expanded role, Dr. Skerlj leads all research and non-clinical development functions at X4, overseeing operations at the company's Vienna, Austria research facility, as well as the company's efforts to advance and expand its pipeline targeting additional rare diseases.

Second Quarter 2020 Financial Results

- **Cash, Cash Equivalents & Restricted Cash:** X4 had \$105.6 million in cash, cash equivalents and restricted cash, as of June 30, 2020. X4 continues to expect that its cash and cash equivalents will fund company operations into early 2022. In addition, X4 continues to have \$25 million of potential borrowing capacity under its amended credit agreement with Hercules.
- **Research and Development Expenses** were \$9.3 million for the second quarter ended June 30, 2020, as compared to \$8.9 million for the comparable period in 2019.
- **General and Administrative Expenses** were \$5.3 million for the second quarter ended June 30, 2020, as compared to \$4.6 million for the comparable period in 2019.
- **Net Loss:** X4 reported a net loss of \$15.1 million for the second quarter ended June 30, 2020, as compared to a net loss of \$13.4 million for the comparable period in 2019.

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: 6091009. 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 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 anticipated and potential impact of the COVID-19 pandemic on X4’s business and operations, including the timing of its ongoing clinical trials; X4’s borrowing capacity under credit agreements; X4’s plans for clinical development of mavorixafor, including the timing of completion and results of its global Phase 3 clinical trial in patients with WHIM syndrome, its Phase 1b clinical trial in combination with ibrutinib in patients with Waldenström’s macroglobulinemia, and its Phase 1b clinical trial as monotherapy in patients with Severe Congenital Neutropenia; the expected timing of guidance and data disclosures on X4’s current clinical trials; and estimates regarding the WHIM patient population and potential market opportunity. 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 May 7, 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)

	Three Months Ended		Six Months Ended	
	June 30, 2020	2019	June 30, 2020	2019
License revenue	\$-	\$-	\$3,000	\$-
Operating expenses:				
Research and development	9,342	8,854	18,253	14,509
General and administrative	5,316	4,560	9,986	9,343
Total operating expenses	14,658	13,414	28,239	23,852
Loss from operations	(14,658)	(13,414)	(25,239)	(23,852)
Other income (expense), net	(486)	31	(895)	(404)
Loss before provision for income taxes	(15,144)	(13,383)	(26,134)	(24,256)
Provision for income taxes	-	-	148	-
Net loss	(15,144)	(13,383)	(26,282)	(24,256)
Adjustments related to convertible preferred stock	-	-	-	(592)
Net loss attributable to common stockholders	\$(15,144)	\$(13,383)	\$26,282	\$(24,848)
Net loss per share attributable to common stockholders- basic and diluted	\$(0.76)	\$(1.02)	\$(1.31)	\$(3.32)
Weighted average common shares outstanding-basic and diluted	20,032	13,177	20,016	7,479

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

	Six months ended	
	June 30,	
	2020	2019
Net loss	\$(26,282)	\$(24,256)
Adjustments to reconcile net loss to net cash used in operating activities	2,591	1,457
Changes in operating assets and liabilities	(3,295)	(3,243)
Net cash used in operating activities	(26,986)	(26,042)
Net cash (used in) provided by investing activities	(564)	26,396
Net cash provided by financing activities	5,049	86,791
Impact of foreign exchange on cash, cash equivalents and restricted cash	60	(2)
Net (decrease) increase in cash, cash equivalents and restricted cash	\$(22,441)	\$87,143
Cash, cash equivalents and restricted cash at beginning of period	\$128,086	\$8,498
Cash, cash equivalents and restricted cash at end of period	\$105,645	\$95,641

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

	June	December
	30, 2020	31, 2019
Current assets:		
Cash and cash equivalents	\$103,744	\$126,184
Research and development incentive receivable	509	1,998
Prepaid expenses and other current assets	5,634	1,096
Total current assets	109,887	129,278
Property and equipment, net	421	403
Goodwill	27,109	27,109
Right-of-use assets	1,644	1,959
Other assets	3,473	1,949
Total assets	\$142,534	\$160,698
Current liabilities:		
Accounts payable	\$1,838	\$2,088
Accrued expenses	8,003	6,461
Current portion of lease liabilities	940	898
Total current liabilities	10,781	9,447
Long-term debt, including accretion, net of discount	25,398	20,097
Lease liabilities	1,445	1,918

Other liabilities	26	16
Total liabilities	37,650	31,478
Total stockholders' equity	104,884	129,220
Total liabilities and stockholders' equity	\$ 142,534	\$ 160,698

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Source: X4 Pharmaceuticals