X4 Pharmaceuticals Reports First Quarter Financial Results and Provides Corporate Update

May 6, 2021

Company continues to make significant clinical progress with lead candidate mavorixafor across multiple rare disease indications

Enrollment update on Phase 3 WHIM trial expected in mid-2021

Initial data from ongoing Phase 1b trial in Waldenström's macroglobulinemia to be presented at EHA Meeting in June 2021

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

BOSTON, May 06, 2021 (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 first quarter ended March 31, 2021. The company also provided an update on its lead product candidate, mavorixafor, a novel small molecule currently being evaluated in a Phase 3 clinical trial (4WHIM) for patients with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome and in two Phase 1b trials for patients with Waldenström's macroglobulinemia and Severe Congenital Neutropenia (SCN), respectively.

"We are excited by the strong progress we have achieved to date in 2021 in the development of mavorixafor across multiple rare disease indications," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "In addition, we recently completed an at-the-market PIPE financing that provided $55 million in gross proceeds to the company. We believe that the significant level of new and existing investor interest demonstrates confidence in the clinical and commercial potential of mavorixafor while also extending our expected cash runway into late 2022, as well as supporting our additional pipeline programs. We look forward to continuing to report both clinical enrollment and data milestones throughout the rest of 2021 and beyond."

Recent Highlights

- **Mavorixafor Phase 1b Data in Waldenström's to be Presented at EHA 2021.** X4 has received notification of acceptance of an abstract for a poster presentation at this year's annual congress of the European Hematology Association (EHA), taking place virtually from June 9-17, 2021. The abstract and poster will include initial clinical safety and efficacy data from the ongoing Phase 1b clinical trial of mavorixafor in combination with ibrutinib in the treatment of a subset of Waldenström's macroglobulinemia patients with mutations to both the MYD88 and CXCR4 genes.
- **Enrollment Update in 4WHIM Clinical Trial Expected to be Announced in Mid-2021.** The 4WHIM Phase 3 trial is a randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the safety and efficacy of mavorixafor in approximately 18-28 genetically confirmed WHIM patients over the course of a 52-week study. The primary endpoint for the trial will compare the level of

circulating neutrophils relative to a clinically meaningful threshold in response to treatment with mavorixafor versus placebo. Secondary endpoints will assess infection rates, wart burden, and markers of immune system function and quality of life among others. Based on the current rate of enrollment, X4 expects to provide an important enrollment update on the trial in the second or third quarter of 2021.

- **Raised $55.0 Million in an At-the-Market PIPE Financing.** In March 2021, X4 priced a $55 million at-the-market private placement financing at $8.70 per common share. The financing transaction included participation from leading biotechnology investors that are both new and existing X4 shareholders. The financing extended the company's guidance on its expected cash runway into the fourth quarter of 2022.

First Quarter 2021 Financial Results

- **Cash, Cash Equivalents & Restricted Cash**: X4 had $116.7 million in cash, cash equivalents and restricted cash as of March 31, 2021. X4 expects that its cash and cash equivalents will fund company operations into the fourth quarter of 2022.
- **Research and Development Expenses** were $12.1 million for the first quarter ended March 31, 2021, as compared to $8.9 million for the comparable period in 2020. R&D expenses include $0.6 million and $0.2 million of certain non-cash expenses for the quarters ended March 31, 2021 and 2020, respectively.
- **General and Administrative Expenses** were $5.8 million for the first quarter ended March 31, 2021, as compared to $4.7 million for the comparable period in 2020. G&A expenses include $0.7 million and $0.4 million of certain non-cash expenses for the quarters ended March 31, 2021 and 2020, respectively.
- **Net Loss:** X4 reported a net loss of $18.7 million for the quarter ended March 31, 2021, as compared to a net loss of $11.1 million for the comparable period in 2020. Net losses include $1.3 million and $0.6 million of certain non-cash expenses for the quarters ended March 31, 2021 and 2020, respectively.

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: 5658628. 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 and X4’s other product candidates or programs and the company’s cash runway. 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 19, 2021, 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 March 31,	
	2021	**2020**
License revenue	$ —	$ 3,000
Operating expenses:		
Research and development	12,104	8,911
General and administrative	5,832	4,670
Total operating expenses	17,936	13,581
Loss from operations	(17,936)	(10,581)
Other expense, net	(734)	(409)
Loss before provision for income taxes	(18,670)	(10,990)
Provision for income taxes	6	148
Net loss	(18,676)	(11,138)
Deemed dividend due to Class B warrant price reset	(8,239)	—
Net loss attributable to common stockholders	$ (26,915)	$ (11,138)
Net loss per share attributable to common stockholders- basic and diluted	$ (1.30)	$ (0.56)
Weighted average common shares outstanding-basic and diluted	20,751	20,014

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

	Three months ended March 31,	
	2021	**2020**
Net loss	$ (18,676)	$ (11,138)

Adjustments to reconcile net loss to net cash used in operating activities	1,955	1,158
Changes in operating assets and liabilities	(1,723)	(5,547)
Net cash used in operating activities	(18,444)	(15,527)
Net cash used in provided by investing activities	(496)	(555)
Net cash provided by financing activities	55,041	4,984
Impact of foreign exchange on cash, cash equivalents and restricted cash	(143)	(34)
Net increase (decrease) in cash, cash equivalents and restricted cash	35,958	(11,132)
Cash, cash equivalents and restricted cash at beginning of period	80,702	128,086
Cash, cash equivalents and restricted cash at end of period	$ 116,660	$ 116,954

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

	March 31, 2021	December 31, 2020
Current assets:		
Cash and cash equivalents	$ 114,945	$ 78,708
Research and development incentive receivable	768	917
Prepaid expenses and other current assets	4,246	3,682
Total current assets	119,959	83,307
Property and equipment, net	1,788	1,237
Goodwill	27,109	27,109
Right-of-use assets	9,788	7,960
Other assets	2,391	3,258
Total assets	$ 161,035	$ 122,871
Current liabilities:		
Accounts payable	$ 3,091	$ 3,144
Accrued expenses	10,706	8,018
Current portion of lease liability	875	786
Total current liabilities	14,672	11,948
Long-term debt, including accretion, net of discount	33,356	33,178
Lease liabilities	5,579	4,484
Other liabilities	491	462
Total liabilities	54,098	50,072
Redeemable common shares	1,875	—
Total stockholders' equity	105,062	72,799
Total liabilities, redeemable common shares and stockholders' equity	$ 161,035	$ 122,871

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Source: X4 Pharmaceuticals