X4 Pharmaceuticals Reports First Quarter 2022 Financial Results and Provides Corporate Update

May 12, 2022

Top-line data from pivotal 4WHIM Phase 3 clinical trial in WHIM syndrome expected in 4Q22

Clinical and regulatory updates from ongoing chronic neutropenia Phase 1b study anticipated during 3Q 2022; Waldenström's macroglobulinemia Phase 1b study now fully enrolled with results expected in second half of 2022

Conference call to be hosted today at 8:30 a.m. EDT

BOSTON, May 12, 2022 (GLOBE NEWSWIRE) -- X4 Pharmaceuticals, (Nasdaq: XFOR), a leader in the discovery and development of novel oral CXCR4-targeted small molecule therapeutics to benefit people with rare immune system disorders, today reported financial results for the first quarter ended March 31, 2022.

"As we quickly approach top-line results from our 4WHIM pivotal Phase 3 trial for WHIM syndrome by the end of this year, the X4 team continues to identify and expand the breadth of individuals diagnosed with this rare disease. Initiatives aimed at supporting diagnosis and driving disease awareness, such as our PATH4WARD program offering free genetic sequencing to patients, and publishing peer-reviewed research to help define novel pathogenic genetic variants, are adding to the population of those known to be affected by WHIM and chronic neutropenia," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Specifically, to support those affected by chronic neutropenia, we are expanding the availability of genetic testing through PATH4WARD and evaluating patients with congenital, idiopathic, and cyclic chronic neutropenia in our ongoing Phase 1b study. We look forward to providing both clinical data and regulatory updates over the coming months on this important initiative."

Recent Highlights

- Presented posters at the CIS 2022 Annual Meeting that detailed the PATH4WARD genetic testing program to aid in the diagnosis of WHIM syndrome and chronic neutropenia, and the characterization of a novel pathogenic CXCR4 variant (S341Y).
- Presented preclinical data on mavorixafor's ability to significantly enhance the tumor-cell killing activity of the leading commercial and clinical stage Bruton Tyrosine Kinase Inhibitors (BTKi) including ibrutinib, zanubrutinib, pirtobrutinib (LOXO-305) and nemtabrutinib (ARQ-531), at the 2022 AACR Annual Meeting.
- Completed enrollment in the ongoing Phase 1b study of mavorixafor in combination with ibrutinib in patients with Waldenström's Macroglobulinemia (WM) whose tumors express mutations in MYD88 and CXCR4. A total of 16 patients were enrolled and dose escalation to the 600 mg dose of mavorixafor has been completed. Results from this study are expected to be reported during the second half of 2022.

First Quarter 2022 Financial Results

- **Cash, Cash Equivalents & Restricted Cash**: X4 had $67.7 million in cash, cash equivalents, and restricted cash as of March 31, 2022. The company expects that its cash and cash equivalents will fund company operations into the fourth quarter of 2022.
- **Research and Development (R&D) Expenses** were $14.1 million for the first quarter of 2022 as compared to $12.1 million for the comparable period in 2021. R&D expenses include $0.7 million and $0.6 million of certain non-cash expenses for the first quarter of 2022 and 2021, respectively.
- **Selling, General and Administrative Expenses (SG&A)** were $7.7 million for the first quarter of 2022 as compared to $5.8 million for the comparable period in 2021. SG&A expenses include $0.8 million and $0.7 million of certain non-cash expenses for the first quarter of 2022 and 2021, respectively.
- **Net Loss:** X4 reported a net loss of $22.0 million for the first quarter of 2022, as compared to $18.7 million for the comparable period in 2021. Net losses include $1.5 million and $1.3 million of certain non-cash expenses for the first quarter of 2022 and 2021, respectively.

Conference Call and Webcast

X4 will host a conference call and webcast today at 8:30 am EDT to discuss 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: 4382059. 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 leading the discovery and development of novel therapies for people with rare diseases of the immune system. The company's lead candidate is mavorixafor, a first-in-class, small molecule antagonist of chemokine receptor CXCR4 that is being developed as a once-daily oral therapy. Due to mavorixafor's ability to antagonize CXCR4 and improve the healthy maturation and trafficking of white blood cells, X4 believes that mavorixafor has the potential to provide therapeutic benefit across a wide variety of diseases, including primary immunodeficiencies (PIDs) and certain types of cancer. Mavorixafor has already demonstrated clinical potential in a Phase 2 trial in people with WHIM syndrome, a rare PID. Its efficacy and safety continue to be evaluated in a global Phase 3 clinical trial in WHIM (fully enrolled) and in two Phase 1b clinical trials – one, as monotherapy in people with chronic neutropenia, including Severe Congenital Neutropenia (SCN), and another in combination with ibrutinib in people with Waldenström's macroglobulinemia (also fully enrolled). 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, to discover and develop 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 and therapeutic potential of mavorixafor and X4's other product candidates or programs; X4's possible exploration of additional opportunities for mavorixafor; the anticipated achievement of upcoming clinical milestones; the expected availability, content, and timing of clinical trial data; clinical trial design, the prevalence of and market opportunity associated with WHIM and chronic neutropenia, 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, on account of uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; the risk that trials and studies may be delayed, including, but not limited to, as a result of the effects of the ongoing COVID-19 pandemic or delayed patient enrollment, and may not have satisfactory outcomes; the risk that the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the risk that 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; the risk that market or prevalence estimates for WHIM and chronic neutropenia may be inaccurate and higher than the actual number of treatable patients; the potential adverse effects arising from the testing or use of mavorixafor or other product candidates; the risks related to X4's ability to raise additional capital, and other risks and uncertainties, including those 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 17, 2022, and in other filings X4 makes with the SEC from time to time.

(Tables Follow)

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

	Three Months Ended March 31,	
	2022	**2021**
Operating expenses:		
Research and development	14,113	12,104
Selling, general and administrative	7,664	5,832
Gain on sale of non-financial asset	(509)	—
Total operating expenses	21,268	17,936
Loss from operations	(21,268)	(17,936)
Other expense, net	(674)	(734)
Loss before provision for income taxes	(21,942)	(18,670)
Provision for income taxes	23	6
Net loss	(21,965)	(18,676)
Deemed dividend due to Class B warrant price reset	(2,259)	(8,239)
Net loss attributable to common stockholders	$ (24,224)	$ (26,915)
Net loss per share attributable to common stockholders- basic and diluted	$ (0.72)	$ (1.30)
Weighted average common shares outstanding-basic and diluted	33,737	20,751

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

	Three months ended March 31,	
	2022	**2021**

Net loss	$	(21,965)	$	(18,676)
Adjustments to reconcile net loss to net cash used in operating activities		2,036		1,955
Changes in operating assets and liabilities		(300)		(1,723)
Net cash used in operating activities		(20,229)		(18,444)
Net cash used in investing activities		(22)		(496)
Net cash provided by financing activities		4,956		55,041
Impact of foreign exchange on cash, cash equivalents and restricted cash		(69)		(143)
Net (decrease) increase in cash, cash equivalents and restricted cash		(15,364)		35,958
Cash, cash equivalents and restricted cash at beginning of period		83,108		80,702
Cash, cash equivalents and restricted cash at end of period	$	67,744	$	116,660

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

	March 31, 2022	December 31, 2021
Current assets:		
Cash and cash equivalents	$ 66,427	$ 81,787
Research and development incentive receivable	868	747
Prepaid expenses and other current assets	3,586	5,344
Total current assets	70,881	87,878
Property and equipment, net	1,403	1,514
Goodwill	17,351	17,351
Right-of-use assets	8,344	8,710
Other assets	1,646	1,723
Total assets	$ 99,625	$ 117,176
Current liabilities:		
Accounts payable	$ 3,569	$ 4,283
Accrued expenses	6,877	7,870
Current portion of lease liability	1,109	1,075
Current portion of long-term debt	3,415	795
Total current liabilities	14,970	14,023
Long-term debt, including accretion, net of discount	29,809	33,139
Lease liabilities	4,482	4,776
Other liabilities	650	826
Total liabilities	49,911	52,764
Total stockholders' equity	49,714	64,412
Total liabilities and stockholders' equity	$ 99,625	$ 117,176

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Source: X4 Pharmaceuticals