



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

August 4, 2022

Highlights notable upcoming milestones including data from Phase 1b trial in chronic neutropenia, expected in late September, and results from global, pivotal Phase 3 trial in WHIM syndrome, expected in 4Q 2022

Announces additional positive data from Phase 1b trial in Waldenström's Macroglobulinemia supportive of a potential partnership to advance clinical development

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

BOSTON, Aug. 04, 2022 (GLOBE NEWSWIRE) -- [X4 Pharmaceuticals, Inc.](#) (Nasdaq: XFOR), a leader in the discovery and development of novel CXCR4-targeted small-molecule therapeutics to benefit people with diseases of the immune system, today reported financial results for the second quarter ended June 30, 2022. In addition, the company highlighted important upcoming milestones related to its lead clinical candidate, mavorixafor, including the presentation of data from its ongoing Phase 1b clinical trial in chronic neutropenic disorders and from the global, pivotal Phase 3 clinical trial of mavorixafor in WHIM (Warts, Hypogammaglobulinemia, Infections, & Myelokathexis) syndrome. The company also provided an update on its clinical oncology program following its recent strategic announcement prioritizing its use of resources to advance mavorixafor solely in chronic neutropenic disorders.

“With the completion of our recent financing and the sharpening of our strategic focus on chronic neutropenic disorders, we believe that we are well positioned to deliver significant future value to both patients and shareholders as we approach key milestones in the second half of this year,” said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. “While the data from our cancer programs continue to show promise, we are now pivoting our efforts towards unlocking the full value of our oncology portfolio through potential strategic partnerships, enabling us to focus on making the largest possible impact on the treatment of chronic neutropenic disorders, including WHIM syndrome. In late September, we expect to deliver on our next milestone – data from our fully enrolled proof-of-concept study in chronic neutropenic disorders; we expect these results to inform the regulatory path forward for mavorixafor across a broad range of chronic neutropenic patient populations. Finally, and notably, the unveiling of data from our pivotal Phase 3 4WHIM trial is on track for the fourth quarter; these results are expected to support our first regulatory filing in the U.S. early in the second half of 2023 and to bring us one large step closer to improving the lives of patients with chronic neutropenic disorders.”

Recent Highlights & Anticipated Upcoming Milestones

- Completed a private investment in public equity (PIPE) financing, raising gross proceeds of approximately \$56 million; the financing included participation from new and existing investors.
- Entered into an amendment to the company's loan and security agreement with Hercules Capital to

extend the interest-only period of its loan facility by up to twelve months (into 2024), subject to achieving certain financial and business milestones.

- Announced a strategic re-prioritization of resources towards advancing mavorixafor in chronic neutropenic disorder indications, including WHIM syndrome, while progressing oncology programs only upon completion of strategic partnership(s); the announced strategic update was also inclusive of cost-cutting initiatives estimated to support extension of X4's cash runway into the third quarter of 2023.
- Presented [research data](#) at the June 2022 meeting of the European Hematology Association (EHA) further supporting mechanism and market potential of mavorixafor across of range of clinical indications.
- Planned late September 2022 presentation of data from the fully enrolled Phase 1b trial evaluating mavorixafor for the treatment of chronic neutropenic disorders, including congenital, idiopathic, and cyclic neutropenia.
- Planned fourth quarter 2022 presentation of results from the global, pivotal Phase 3 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome; data anticipated to support a submission for U.S. regulatory approval early in the second half of 2023.

Positive Data Update from Phase 1b Trial in Waldenström's Macroglobulinemia (WM)

- The Phase 1b, open-label, multicenter, single-arm study is evaluating the safety and efficacy of mavorixafor in combination with the BTK inhibitor ibrutinib in adult patients (either treatment naïve or relapsed/refractory) with the rare B-cell lymphoma Waldenström's macroglobulinemia (WM) and confirmed MYD88 and CXCR4 genetic mutations.
- A total of 16 patients were enrolled in the study; all were dosed with oral, once-daily doses of ibrutinib (420 mg) and escalating doses (200 mg, 400 mg, 600 mg) of oral mavorixafor, also once daily.
- As of June 2022, 10 of 12 evaluable patients (83%) achieved a major response (MR) to therapy, or a greater than 50% reduction in serum IgM from baseline:
 - In relapsed/refractory patients, 8 of 9 (89%) achieved a MR
 - In treatment-naïve patients, MR was seen in all patients escalated to >200 mg of mavorixafor
- Adding mavorixafor to ibrutinib was associated with a higher MR rate at 9, 12, and 24 months compared to previously reported MR rates achieved with ibrutinib monotherapy.
- In addition, patients achieved elevations in absolute neutrophil count (ANC), with no neutropenic events reported; patients also experienced fewer infections over time with chronic dosing.
- No major safety signals had been identified in the trial as of the data cut-off; mavorixafor in combination with ibrutinib showed a safety profile similar to ibrutinib monotherapy (N=16, including 8 patients escalated to the 600 mg dose of mavorixafor).
- In June, mavorixafor was granted Orphan Drug Designation by the U.S. Food & Drug Administration for the treatment of patients with WM, regardless of CXCR4 mutation status.
- The Phase 1b clinical trial is expected to be completed in the fourth quarter of 2022; further clinical studies in WM will now be subject to completing a strategic partnership.

Second Quarter 2022 Financial Results

- **Cash, Cash Equivalents & Restricted Cash:** X4 had \$48.7 million in cash, cash equivalents, and restricted cash as of June 30, 2022. On June 30, 2022, the company announced a private equity financing (PIPE) of approximately \$56 million before deducting offering costs. The financing closed on July 6, 2022, and proceeds are not reflected in the cash on hand as of June 30, 2022. Including funds raised in this offering, the company's recently announced cost-cutting measures, and recent amendments to its loan and security agreement with Hercules, X4 believes that it has sufficient funds to support company operations into the third quarter of 2023.
- **Research and Development (R&D) Expenses** were \$13.8 million for the second quarter of 2022 as compared to \$13.2 million for the comparable period in 2021. R&D expenses include \$0.7 million and

\$0.8 million of certain non-cash expenses for the second quarter of 2022 and 2021, respectively.

- **Selling, General, and Administrative Expenses (SG&A)** were \$6.7 million for the second quarter of 2022 as compared to \$5.8 million for the comparable period in 2021. SG&A expenses include \$0.8 million and \$1.0 million of certain non-cash expenses for the second quarter of 2022 and 2021, respectively.
- **Net Loss:** X4 reported a net loss of \$21.2 million for the second quarter of 2022, as compared to \$19.6 million for the comparable period in 2021. Net losses include \$1.5 million and \$1.8 million of certain non-cash expenses for the second 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 (855) 327-6837 from the United States or (631) 891-4304 internationally, followed by the conference ID: 10019589. 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 diseases of the immune system. Our lead clinical 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 mobilization of white blood cells, we believe that mavorixafor has the potential to provide therapeutic benefit across a wide variety of diseases, including a range of chronic neutropenic disorders and certain types of cancer. The efficacy and safety of mavorixafor are being evaluated in a global Phase 3 clinical trial in patients with WHIM syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. We are also studying mavorixafor in two Phase 1b clinical trials – one as a monotherapy in patients with chronic neutropenic disorders including congenital, idiopathic, and cyclic neutropenia, and one in combination with ibrutinib in patients with Waldenström's macroglobulinemia (WM), a rare B-cell lymphoma. Further clinical development of mavorixafor in WM will now be subject to completing a strategic partnership as we focus our resources on advancing mavorixafor for the benefit of patients with chronic neutropenic disorders. We continue to leverage our insights into CXCR4 biology at our corporate headquarters in Boston, Massachusetts and at our research facility in Vienna, Austria. For more information, please visit our website at 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; the anticipated achievement of upcoming clinical milestones; the expected availability, content, and timing of clinical trial data; anticipated regulatory milestones; 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, 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 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 12, 2022, and in other filings X4 makes with the SEC from time to time.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	\$ 13,821	\$ 13,193	27,934	25,297
Selling, general and administrative	6,749	5,804	14,413	11,636
Gain on sale of non-financial asset	—	—	(509)	—
Total operating expenses	<u>20,570</u>	<u>18,997</u>	<u>41,838</u>	<u>36,933</u>
Loss from operations	(20,570)	(18,997)	(41,838)	(36,933)
Other expense, net	(638)	(635)	(1,312)	(1,369)
Loss before provision for income taxes	(21,208)	(19,632)	(43,150)	(38,302)
Provision for income taxes	4	6	27	12
Net loss	(21,212)	(19,638)	(43,177)	(38,314)
Deemed dividend due to Class B warrant price reset	—	—	(2,259)	(8,239)
Net loss attributable to common stockholders	<u>\$ (21,212)</u>	<u>\$ (19,638)</u>	<u>\$ (45,436)</u>	<u>\$ (46,553)</u>
Net loss per share attributable to common stockholders- basic and diluted	<u>\$ (0.60)</u>	<u>\$ (0.74)</u>	<u>\$ (1.31)</u>	<u>\$ (1.97)</u>
Weighted average common shares outstanding-basic and diluted	35,437	26,527	34,592	23,655

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

	Six months ended June 30,	
	2022	2021
Net loss	\$ (43,177)	\$ (38,314)
Adjustments to reconcile net loss to net cash used in operating activities	4,083	4,345
Changes in operating assets and liabilities	391	(3,672)
Net cash used in operating activities	<u>(38,703)</u>	<u>(37,641)</u>
Net cash used in investing activities	<u>(60)</u>	<u>(582)</u>

Net cash provided by financing activities	4,609	54,117
Impact of foreign exchange on cash, cash equivalents and restricted cash	(271)	(103)
Net (decrease) increase in cash, cash equivalents and restricted cash	(34,425)	15,791
Cash, cash equivalents and restricted cash at beginning of period	83,108	80,702
Cash, cash equivalents and restricted cash at end of period	\$ 48,683	\$ 96,493

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

	June 30, 2022	December 31, 2021
Current assets:		
Cash and cash equivalents	\$ 47,378	\$ 81,787
Research and development incentive receivable	843	747
Prepaid expenses and other current assets	4,364	5,344
Total current assets	52,585	87,878
Property and equipment, net	1,309	1,514
Goodwill	17,351	17,351
Right-of-use assets	7,976	8,710
Other assets	1,798	1,723
Total assets	\$ 81,019	\$ 117,176
Current liabilities:		
Accounts payable	\$ 3,677	\$ 4,283
Accrued expenses	8,582	7,870
Current portion of lease liability	1,136	1,075
Current portion of long-term debt	8,607	795
Total current liabilities	22,002	14,023
Long-term debt, including accretion, net of discount	24,490	33,139
Lease liabilities	4,140	4,776
Other liabilities	314	826
Total liabilities	50,946	52,764
Total stockholders' equity	30,073	64,412
Total liabilities and stockholders' equity	\$ 81,019	\$ 117,176

Contacts:

Daniel Ferry (investors)
Managing Director, LifeSci Advisors
daniel@lifesciadvisors.com
(617) 430-7576

Mónica Rouco Molina, Ph.D. (media)
Account Supervisor, LifeSci Communications
mroucomolina@lifescicomms.com



Source: X4 Pharmaceuticals