X4 Pharmaceuticals Reports Fourth Quarter and Full Year 2021 Financial Results and Provides Corporate Update

March 17, 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 trial anticipated during 2Q/3Q 2022

Additional Waldenström's macroglobulinemia Phase 1b data expected in second half of 2022; supportive pre-clinical data to be presented at AACR 2022

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

BOSTON, March 17, 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 fourth quarter and twelve months ended December 31, 2021, and provided a corporate update.

"We are very pleased with our many accomplishments in 2021, particularly completing enrollment in the pivotal 4WHIM Phase 3 clinical trial in WHIM syndrome, the first indication we are pursuing for our oral, first-in-class CXCR4 inhibitor, mavorixafor," commented Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We also continued enrolling and treating patients in both our chronic neutropenia and Waldenström's macroglobulinemia Phase 1b clinical trials, which are designed to expand the potential market opportunities for mavorixafor, and we look forward to reporting key clinical and regulatory updates for these programs throughout the year."

Dr. Ragan continued, "Our clinical and research efforts into the potential of CXCR4 antagonism have also resulted in the discovery of several novel WHIM-causing CXCR4 mutations associated with expanded disease characteristics, which we believe further underscore WHIM syndrome as an underrecognized condition and strengthen our confidence in prevalence being potentially higher than the current estimates of WHIM patients in the U.S. We look ahead to the remainder of 2022 with great excitement, as we continue to anticipate top-line data from 4WHIM by the end of the year, and additional clinical and research data throughout the year, further supporting the broad clinical and commercial potential of mavorixafor across multiple indications."

Fourth Quarter 2021 Highlights

- Completed enrollment in the ongoing pivotal 4WHIM Phase 3 clinical trial of mavorixafor in patients with WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis) syndrome. Thirty-one adult and pediatric patients have been enrolled in the 4WHIM trial, which was originally designed to enroll 18 - 28 patients. Top-line data from the 4WHIM trial are expected in the fourth quarter of 2022.
- Presented a wide range of clinical and research data at the 2021 American Society of Hematology

(ASH) Annual Meeting supporting the potential use of mavorixafor across a number of immunodeficiencies and certain cancers:

- Ongoing studies across a wide variety of diseases, including Waldenström's macroglobulinemia (WM), clear cell renal cell carcinoma, WHIM syndrome, and chronic idiopathic neutropenia, showed that oral administration of mavorixafor increased blood neutrophils, lymphocytes, and monocytes regardless of the presence or absence of CXCR4 mutations.
- Mavorixafor efficacy has been clinically observed with short-term and long-term treatment both alone and in combination with other therapies, including axitinib, ibrutinib, and granulocyte-colony stimulating factor (G-CSF).
- Additional data from the Phase 2 open-label extension study of mavorixafor in WHIM patients continued to show durable increases in neutrophils, lymphocytes, and monocytes; decreased frequency, severity, and duration of infections; fewer hospital/doctor visits; and sustained improvements in warts. Chronic daily administration of mavorixafor continued to be well tolerated (median treatment duration = 148.4 weeks).
- Clinical and laboratory research resulted in the identification of a novel missense CXCR4 mutation, D84H, that further supports the company's WHIM prevalence estimate of at least 1,000 to 3,500 patients in the U.S.
- Initial results from the ongoing mavorixafor Phase 1b clinical trial in people with chronic neutropenia concurrently treated with granulocyte colony stimulating factor (g-CSF) and mavorixafor demonstrated elevations in white blood cells and absolute neutrophil, lymphocyte, and monocyte counts (n=4); enrollment continues, with additional data expected in the second or third quarter of 2022.
- In December 2021, interim results reported from the ongoing Phase 1b clinical trial in WM from the low- (200 mg) and mid-level (400 mg) dose groups (October 2021 data cut) showed a 100% overall response rate (n=10, median treatment duration of 272.5 days), sustained decreases in serum IgM (n=14), and trends towards normalization of hemoglobin levels (n=14 at baseline, n=3 at 12 months).
    - As of March 2022, cohort B evaluating 600 mg of mavorixafor in combination with ibrutinib met the safety requirements to allow for the dose escalation of patients enrolled in cohort A and previously treated at low- and mid-level doses. All eligible patients are being escalated to receive 600 mg of mavorixafor once daily in combination with ibrutinib. Additional data from this trial are expected in the second half of 2022.

Upcoming Presentations

Clinical Immunology Society (CIS)

X4 will present three posters at the CIS 2022 Annual Meeting: Immune Deficiency and Dysregulation North American Conference, March 31 - April 3, 2022:

- *PATH4WARD: A Genetic Testing Program to Aid in Molecular Diagnosis of Congenital Neutropenia and Other Primary Immunodeficiencies Including WHIM Syndrome.*
- *Characterization of CXCR4(S341Y) Variant of Uncertain Significance in the Setting of Infections, Hypogammaglobulinemia, and Warts.*
- *4WHIM: Evaluating the Oral CXCR4 Antagonist Mavorixafor in Patients With WHIM Syndrome via a Global Phase 3, Randomized, Placebo-Controlled Trial With Open-label Extension.*

American Association for Cancer Research (AACR)

X4 will present emerging preclinical data on mavorixafor's ability to significantly enhance the tumor cell killing activity of the leading commercial and clinical Bruton Tyrosine Kinase Inhibitors (BTKi) including ibrutinib, zanubrutinib, pirtobrutinib (LOXO-305) and nemtabrutinib (ARQ-531), at the 2022 AACR Annual

Meeting, taking place April 8 – 13, 2022.

- e-Poster #6093: *Mavorixafor Enhances Efficacy of Bruton Tyrosine Kinase Inhibitors by Overcoming the Protective Effect of Bone Marrow Stroma on Tumor Cells in Waldenström's Macroglobulinemia* .
  Session: OPO.TB06.01 - Tumor Microenvironment
  E-posters are expected to be released at 12:00 PM CT on Friday, April 8.

Fourth Quarter and Full Year 2021 Financial Results

- **Cash, Cash Equivalents & Restricted Cash**: X4 had $83.1 million in cash, cash equivalents, and restricted cash as of December 31, 2021. 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 $12.2 million and $50.6 million for the fourth quarter and full year ended December 31, 2021, as compared to $12.3 million and $41.9 million for the comparable periods in 2020. R&D expenses include $0.7 million and $2.7 million of certain non-cash expenses for the quarter and full year ended December 31, 2021, respectively.
- **General and Administrative Expenses (G&A)** were $7.1 million and $24.7 million for the fourth quarter and full year ended December 31, 2021, as compared to $5.4 million and $20.9 million for the comparable periods in 2020. G&A expenses include $0.9 million and $3.5 million of certain non-cash expenses for the quarter and full year ended December 31, 2021, respectively.
- **Net Loss:** X4 reported a net loss of $30.2 million and $88.7 million for the quarter and full year ended December 31, 2021, as compared to a net loss of $18.4 million and $62.1 million for the comparable periods in 2020. Net loss for the current quarter and full year period includes a non-cash goodwill impairment charge of $9.8 million. There was no goodwill impairment charge in the prior year periods. Net losses include $1.6 million and $6.2 million of stock-based compensation expense for the quarter and full year ended December 31, 2021, respectively. Net losses included $1.4 million and $5.4 million of stock-based compensation expense for the quarter and full year ended December 31, 2020, 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: 9772687. 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. 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. 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; anticipated updates on regulatory interactions and commercial plans; clinical trial design, 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, 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 potential adverse effects arising from the testing or use of mavorixafor or other product candidates; 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 November 4, 2021, 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 December 31,		Twelve Months Ended December 31,	
	2021	2020	2021	2020
License revenue	$ —	$ —		$ 3,000
Operating expenses:				
Research and development	12,162	12,298	50,647	41,932
General and administrative	7,135	5,357	24,702	20,942
Goodwill impairment	9,758	—	9,758	—
Total operating expenses	29,055	17,655	85,107	62,874
Loss from operations	(29,055)	(17,655)	(85,107)	(59,874)
Other expense, net	(1,149)	(745)	(3,572)	(2,109)
Loss before provision for income taxes	(30,204)	(18,400)	(88,679)	(61,983)

Provision for income taxes	3	—	17	148
Net loss	(30,207)	(18,400)	(88,696)	(62,131)
Deemed dividend due to Class B warrant price reset	(5,704)	—	(13,943)	—
Net loss attributable to common stockholders	$ (35,911)	$ (18,400)	$ (102,639)	$ (62,131)
Net loss per share attributable to common stockholders-basic and diluted	$ (1.24)	$ (0.91)	$ (3.99)	$ (3.09)
Weighted average common shares outstanding-basic and diluted	29,011	20,174	25,749	20,077

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

	Twelve months ended December 31,	
	2021	**2020**
Net loss	$ (88,696)	$ (62,131)
Adjustments to reconcile net loss to net cash used in operating activities	19,289	7,376
Changes in operating assets and liabilities	(1,498)	(4,063)
Net cash used in operating activities	(70,905)	(58,818)
Net cash used in investing activities	(615)	(1,362)
Net cash provided by financing activities	74,245	12,394
Impact of foreign exchange on cash, cash equivalents and restricted cash	(319)	402
Net increase (decrease) in cash, cash equivalents and restricted cash	2,406	(47,384)
Cash, cash equivalents and restricted cash at beginning of period	80,702	128,086
Cash, cash equivalents and restricted cash at end of period	$ 83,108	$ 80,702

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

	December 31, 2021	**December 31, 2020**
Current assets:		

Cash and cash equivalents	$ 81,787	$ 78,708
Research and development incentive receivable	747	917
Prepaid expenses and other current assets	5,344	3,682
Total current assets	87,878	83,307
Property and equipment, net	1,514	1,237
Goodwill	17,351	27,109
Right-of-use assets	8,710	7,960
Other assets	1,723	3,258
Total assets	$ 117,176	$ 122,871
Current liabilities:		
Accounts payable	$ 4,283	$ 3,144
Accrued expenses	7,870	8,018
Current portion of lease liability	1,075	786
Current portion of long-term debt	795	—
Total current liabilities	14,023	11,948
Long-term debt, including accretion, net of discount	33,139	33,178
Lease liabilities	4,776	4,484
Other liabilities	826	462
Total liabilities	52,764	50,072
Total stockholders' equity	64,412	72,799
Total liabilities and stockholders' equity	$ 117,176	$ 122,871

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