X4 Pharmaceuticals to Have Strong Presence at ASH 2021 with Seven Accepted Abstracts Highlighting New Clinical and Scientific Data; Company Reports Third Quarter Financial Results

November 4, 2021

Data continue to support mavorixafor's potential to benefit patients with WHIM syndrome, chronic neutropenia, and Waldenström's macroglobulinemia

Discovery of new prevalent WHIM variant further validates the company's U.S. WHIM prevalence projections

Company to host an Investor Day on December 16 with key opinion leaders leading discussions on four poster presentations and additional clinical and scientific data

Conference call today at 9:05 a.m. ET

BOSTON, Nov. 04, 2021 (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 third quarter and nine months ended September 30, 2021. The company also provided a summary of the data contained within the seven abstracts submitted and accepted for presentation and/or publication at the 63rd American Society of Hematology (ASH) Annual Meeting, taking place in Atlanta, Georgia and virtually December 11-14, 2021.

"This has been an amazing quarter of progress for all of us at X4, as we completed enrollment in the Phase 3 trial of mavorixafor in its first indication of WHIM syndrome and are now looking ahead to sharing a broad array of clinical and scientific data in the fourth quarter that further support the potential of mavorixafor across multiple additional therapeutic areas," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4. "As we continue to expand our pre-commercial activities for WHIM in anticipation of top-line data late next year, we are also working to build out our pipeline – further advancing clinical trials of mavorixafor in neutropenia and rare oncology indications, while also advancing our pre-clinical programs towards the clinic."

Key Takeaways from ASH Abstracts Published Today

WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome:

- Mavorixafor continues to show durable increases in neutrophils and lymphocytes, sustained improvements in infections and warts, and good tolerability in the ongoing Phase 2 open-label extension trial in WHIM syndrome.
- Patient interviews revealed that study participants experienced good tolerability and beneficial treatment effects when dosed with mavorixafor.
- Broader understanding of the clinical spectrum of WHIM syndrome and genotype/phenotype correlations have enabled assessment of novel CXCR4 variants for disease-correlation, including a

newly discovered missense mutation (p.D84H) that is relatively frequent in the general population.
    - The D84H mutation is the first mutation identified outside of the C-terminus of the CXCR4 receptor showing gain-of-function signaling and disease phenotype; the frequency of the D84H mutation derived from broad population genomic databases robustly supports current estimates of U.S. WHIM prevalence of 1,000 to 3,500 or more.
    - This and other related research will be shared at the Investor Day on December 16.
- Analyses of insurance claim databases using artificial intelligence indicate that the U.S. population of WHIM patients may be much larger than currently reported in the literature.
    - Results showed that there may be as many as 3,700 WHIM patients based on WHIM-like phenotypes described.
    - Data are consistent with novel genetic variant discoveries and genotype-phenotype correlations, building further confidence in estimates of potentially treatable WHIM patients in the U.S.
- The company's global Phase 3 clinical trial in WHIM syndrome (now fully enrolled) is the first double-blind, placebo-controlled, randomized trial in this patient population and the only one exploring an oral therapy. All enrolled patients had severe neutropenia and more than half are pediatric patients, illustrative of the severity and early onset of the disease. Top-line data from the trial are expected in the fourth quarter of 2022.

Chronic neutropenia and other indications:

- Mavorixafor alone or in combination with other therapies is the first oral treatment to acutely and chronically increase total peripheral white blood cells (WBCs) 1.5- to 3-fold across all disease populations examined (WHIM syndrome, Waldenström's macroglobulinemia, clear cell renal cell carcinoma, and healthy volunteers).
- Further, mavorixafor's ability to increase circulating WBCs (neutrophils, lymphocytes, and monocytes) across various disease states and in healthy individuals supports its potential utility in the treatment of patients with immunodeficiency, regardless of the presence or absence of CXCR4 mutations.
- A Phase 1b trial in chronic neutropenia populations is ongoing to assess the potential of mavorixafor to treat broader neutropenias by increasing neutrophil counts, as well as other white blood cell types; initial data from this trial are expected to be presented in an ASH poster presentation and at the Investor Day on December 16.

Waldenström's macroglobulinemia (WM):

- Additional preliminary clinical data are presented from the ongoing Phase 1b dose-escalation trial assessing the tolerability and efficacy, including clinical response rates, of mavorixafor in combination with ibrutinib in diagnosed WM patients with both MYD88 and CXCR4 mutations.
- As of the abstract cutoff date of June 15, 2021, the overall response rate (minor response or better) for evaluable patients was 100% (N=8), with 4 of 8 patients achieving a major response (corresponding to >50% reduction in serum IgM) and 1 of 8 patients achieving very good partial response (corresponding to >90% reduction in serum IgM).
- Additional clinical data are expected to be presented in an ASH poster presentation and at the Investor Day on December 16.

Abstracts Accepted for Presentation and Publication at ASH

Preliminary Clinical Response Data from a Phase 1b Study of Mavorixafor in Combination with Ibrutinib in Patients with Waldenström's Macroglobulinemia with MYD88 and CXCR4 Mutations	Poster Presentation on 12/11/2021 from 5:30 - 7:30 pm ET
Mavorixafor, an Oral CXCR4 Antagonist, for Treatment of Patients with WHIM Syndrome: Results from the Long-Term	Poster Presentation on 12/11/2021 from 5:30 - 7:30 pm ET

Extension of the Open-Label Phase 2 Study	
Oral Administration of Mavorixafor, a CXCR4 Antagonist, Increases Peripheral White Blood Cell Counts across Different Disease States	Poster Presentation on 12/12/2021 from 6:00 - 8:00 pm ET
Comprehensive in Vitro Characterization of CXCR4^{WHIM} variants to Decipher Genotype–Phenotype Correlations in WHIM Syndrome	Poster Presentation on 12/12/2021 from 6:00 - 8:00 pm ET
Characterization of a Novel Missense CXCR4 Mutation in a Patient With WHIM-like Syndrome	Abstract Publication only
Application of an Artificial Intelligence/Machine Learning Model for Estimating Potential US Prevalence of WHIM Syndrome, a Rare Immunodeficiency, From Insurance Claims Data	Abstract Publication only
Global Phase 3, Randomized, Placebo-Controlled Trial With Open-Label Extension Evaluating the Oral CXCR4 Antagonist Mavorixafor in Patients With WHIM Syndrome (4WHIM): Trial Design and Enrollment	Abstract Publication only

Following the ASH meeting, the company will be hosting an investor event on the morning of December 16. The call is expected to include commentary from prominent key opinion leaders (KOLs) as part of the program. Details will be forthcoming.

Third Quarter and Recent Highlights

- X4 achieved a major milestone in early October, completing enrollment in the ongoing pivotal Phase 3 clinical trial (4WHIM) of mavorixafor in the treatment of patients with WHIM syndrome. Thirty-one adult and pediatric patients have been enrolled in the 4WHIM trial, which was originally designed to enroll 18-28 patients.
- The company recently announced the appointment of Françoise de Craecker to the company's Board of Directors and the recent hiring of Karolyn Park to the newly created role of Vice President, U.S. Commercial, significantly strengthening the company's depth and breadth of commercial leadership experience in the strategic marketing of rare disease therapeutics.
- The company announced the promotion of Mary DiBiase, Ph.D. to the newly created position of Chief Operating Officer, reflecting her long-standing contributions to the company and the advancement of mavorixafor into global late-stage clinical development.

Third Quarter 2021 Financial Results

- **Cash, Cash Equivalents & Restricted Cash**: X4 had $77.7 million in cash, cash equivalents, and restricted cash as of September 30, 2021. The company continues to expect that its cash and cash equivalents will fund company operations into the fourth quarter of 2022.
- **Research and Development Expenses** were $13.2 million for the third quarter ended September 30, 2021, as compared to $11.4 million for the comparable period in 2020. R&D expenses include $0.6 million and $1.0 million of certain non-cash expenses for the quarters ended September 30, 2021 and 2020, respectively.
- **General and Administrative Expenses** were $5.9 million for the third quarter ended September 30, 2021, as compared to $5.6 million for the comparable period in 2020. G&A expenses include $0.9 million and $1.2 million of certain non-cash expenses for the quarters ended September 30, 2021 and 2020, respectively.
- **Net Loss:** X4 reported a net loss of $20.2 million for the quarter ended September 30, 2021, as compared to a net loss of $17.4 million for the comparable period in 2020. Net losses include $1.5

million and $2.2 million of certain non-cash expenses for the quarters ended September 30, 2021 and 2020, respectively.

Conference Call and Webcast

X4 will host a conference call and webcast today at 9:05 a.m. ET to discuss the financial results and business highlights, as well as the abstracts accepted to this year's ASH Annual Meeting. 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: 7582968. 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 company 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 Severe Congenital Neutropenia (SCN) and other chronic neutropenia conditions, 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 regulatory filings 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 August 3, 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.

(Tables Follow)

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
License revenue	$ —	$ —	$ —	$ 3,000
Operating expenses:				
Research and development	13,188	11,381	38,485	29,634
General and administrative	5,931	5,599	17,567	15,585
Total operating expenses	19,119	16,980	56,052	45,219
Loss from operations	(19,119)	(16,980)	(56,052)	(42,219)
Other expense, net	(1,054)	(469)	(2,423)	(1,364)
Loss before provision for income taxes	(20,173)	(17,449)	(58,475)	(43,583)
Provision for income taxes	2	—	14	148
Net loss	(20,175)	(17,449)	(58,489)	(43,731)
Deemed dividend due to Class B warrant price reset	—	—	(8,239)	—
Net loss attributable to common stockholders	$ (20,175)	$ (17,449)	$ (66,728)	$ (43,731)
Net loss per share attributable to common stockholders- basic and diluted	$ (0.76)	$ (0.87)	$ (2.71)	$ (2.18)
Weighted average common shares outstanding-basic and diluted	26,609	20,085	24,667	20,035

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

	Nine months ended June 30,	
	2021	**2020**
Net loss	$ (58,489)	$ (43,731)
Adjustments to reconcile net loss to net cash used in operating activities	6,809	5,198
Changes in operating assets and liabilities	(172)	(2,781)
Net cash used in operating activities	(51,852)	(41,314)
Net cash used in investing activities	(602)	(1,060)
Net cash provided by financing activities	49,675	4,765
Impact of foreign exchange on cash, cash equivalents and restricted cash	(203)	240

Net decrease in cash, cash equivalents and restricted cash	(2,982)	(37,369)
Cash, cash equivalents and restricted cash at beginning of period	80,702	128,086
Cash, cash equivalents and restricted cash at end of period	$ 77,720	$ 90,717

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

	September 30, 2021	December 31, 2020
Current assets:		
Cash and cash equivalents	$ 76,393	$ 78,708
Research and development incentive receivable	646	917
Prepaid expenses and other current assets	3,661	3,682
Total current assets	80,700	83,307
Property and equipment, net	1,632	1,237
Goodwill	27,109	27,109
Right-of-use assets	9,071	7,960
Other assets	2,005	3,258
Total assets	$ 120,517	$ 122,871
Current liabilities:		
Accounts payable	$ 2,424	$ 3,144
Accrued expenses	9,074	8,018
Current portion of lease liability	1,041	786
Current portion of long-term debt	693	—
Total current liabilities	13,232	11,948
Long-term debt, including accretion, net of discount	33,043	33,178
Lease liabilities	5,067	4,484
Other liabilities	496	462
Total liabilities	51,838	50,072
Total stockholders' equity	68,679	72,799
Total liabilities and stockholders' equity	$ 120,517	$ 122,871

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Source: X4 Pharmaceuticals