X4 Pharmaceuticals Announces Key Enrollment Milestone Achievements in Ongoing Mavorixafor Clinical Trials and Reports Second Quarter Financial Results

August 3, 2021

Twenty-three patients now enrolled in ongoing pivotal Phase 3 trial in WHIM syndrome, surpassing minimum needed for primary endpoint analyses; enrollment to complete in 3Q21 with top-line data expected in 4Q22

Minimum number of patients now enrolled to determine optimal dosing of mavorixafor in Waldenström's macroglobulinemia Phase 1b trial; continue to expect high-dose safety and efficacy data, as well as clinical response outcomes, in 4Q21

Initial data from Phase 1b trial in Severe Congenital Neutropenia (SCN) expected in 4Q21, as company explores expanded use of mavorixafor across broader chronic neutropenia populations

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

BOSTON, Aug. 03, 2021 (GLOBE NEWSWIRE) -- X4 Pharmaceuticals, Inc. (Nasdaq: XFOR), a leader in the discovery and development of novel therapies targeting diseases of the immune system resulting from dysfunction of the CXCR4 pathway, today reported financial results for the second quarter and six months ended June 30, 2021. The company also announced key enrollment milestone achievements for its lead product candidate, mavorixafor, a novel, oral 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 clinical trials for patients with Waldenström's macroglobulinemia and Severe Congenital Neutropenia (SCN) and chronic neutropenia disorders, respectively.

"We are very encouraged by the strong interest from both the physicians and patients participating in our mavorixafor clinical programs," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "With enrollment nearly complete in the 52-week placebo-controlled Phase 3 trial in WHIM syndrome, our initial indication for mavorixafor, and continued supportive data coming from our ongoing open-label Phase 2 trial in WHIM, we are starting to ramp up our pre-commercial planning, as we now look forward to Phase 3 top-line data in the fourth quarter of 2022. In addition, we are making strong progress in our ongoing Phase 1b clinical trial in Waldenström's and intend to announce preliminary high-dose data along with certain response measures from this trial in the fourth quarter of 2021 that we believe will build on the low- and mid-dose data we presented at EHA this past June. Lastly, as enrollment continues in our ongoing Phase 1b trial in SCN, from which we expect the first data in the fourth quarter of 2021, we are exploring the potential broader use of mavorixafor across the larger chronic neutropenia landscape. We look forward to reporting on our continued progress with mavorixafor, presenting additional clinical, pre-clinical and prevalence data, and providing a variety of company updates later this year."

Mavorixafor Clinical Trial Updates

- **Phase 3 Trial in WHIM Syndrome (4WHIM):**
  - The company today announced that it has surpassed the 18-patient minimum enrollment needed for primary endpoint analyses, determination of clinical benefit, and U.S. regulatory filing (if supported by the Phase 3 data), having enrolled 23 patients to date in its ongoing Phase 3 trial in WHIM syndrome. Enrollment will be completed in the third quarter of 2021, allowing the remaining identified patients to complete screening and potential enrollment. Top-line data are expected to be announced in the fourth quarter of 2022.
  - The 4WHIM Phase 3 trial is a global, randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the safety and efficacy of mavorixafor in 18-28 genetically confirmed WHIM patients over the course of a 52-week study with open-label extension. 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 over 24-hour periods. Secondary endpoints will assess infection rates, wart burden, markers of immune system function, and quality of life, among others.
  - The company is planning to announce new data from the open-label extension of its ongoing Phase 2 clinical trial, as well as an update on patient prevalence, and new data from research into the genetics of WHIM that will detail new insights into genotype/phenotype correlations and the identification of a new WHIM variant.
- **Phase 1b Trial in Waldenström's Macroglobulinemia (WM):**
  - The company also announced today that it has surpassed enrollment of the minimum 12 patients (Cohorts A and B) required to determine optimal dosing of mavorixafor in combination with ibrutinib in the ongoing Phase 1b clinical trial. The company is continuing enrollment in the optional Cohort C (up to an additional 6 patients).
  - This ongoing Phase 1b, open-label, multicenter, single-arm study examines intra-patient dose escalation, safety, pharmacokinetics (PK), and pharmacodynamics (PD) of mavorixafor (200 mg, 400 mg, and 600 mg) in combination with ibrutinib (420 mg), both delivered orally once daily, in patients with Waldenström's macroglobulinemia and confirmed *MYD88* and *CXCR4* mutations. Patients are followed for adverse events and change from baseline in IgM and hemoglobin, PK, and PD (including peripheral white blood cell counts), in addition to clinical response.
  - The company remains on track to announce additional dosing, efficacy, safety, and clinical response data from the ongoing trial in the fourth quarter of 2021, including patient data at the highest planned mavorixafor dose of 600 mg.
- **Phase 1b Trial in Severe Congenital Neutropenia (SCN):**
  - Enrollment continues in this clinical trial, with initial data anticipated in the fourth quarter of 2021. The company expects that the initial data from this trial, in combination with additional data emerging from prior and ongoing studies that show chronic, sustained white blood cell increases across a number of patient groups treated with mavorixafor, will support the company's exploration of opportunities for mavorixafor use across larger chronic neutropenic populations and more broadly in cellular immunodeficiencies.

Second Quarter Highlights and Upcoming Events

- **EHA 2021:** In June, the company announced the presentation of positive data from its ongoing Phase 1b clinical trial of mavorixafor in combination with ibrutinib in Waldenström's macroglobulinemia. Data showed robust decreases in serum IgM at low- and mid-doses of mavorixafor, suggesting best-in-class potential for this combination treatment; meaningful increases in hemoglobin levels suggested reduction in cancer burden in the bone marrow; and at 6 months, patients achieved median IgM level reductions of 60%-75%, with one patient achieving normal IgM; two of four patients (50%) had >50% reduction in serum IgM from baseline. The poster is available here; slides from the company's associated analyst event are available here.
- **X4 management will be participating in the following upcoming investor conferences:**

- **Canaccord Genuity Growth Conference –** taking place virtually August 10-12, 2021
- **Citi Annual BioPharma Conference–** taking place virtually September 8-10, 2021
- **Oppenheimer Fall Healthcare Life Science & Med Tech Summit** – taking place virtually September 20-23, 2021
- **Cantor Fitzgerald Healthcare Conference** – taking place virtually September 27-30, 2021

Second Quarter 2021 Financial Results

- **Cash, Cash Equivalents & Restricted Cash**: X4 had $96.5 million in cash, cash equivalents, and restricted cash as of June 30, 2021. The company expects 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 second quarter ended June 30, 2021, as compared to $9.3 million for the comparable period in 2020. R&D expenses include $0.8 million and $0.5 million of certain non-cash expenses for the quarters ended June 30, 2021 and 2020, respectively.
- **General and Administrative Expenses** were $5.8 million for the second quarter ended June 30, 2021, as compared to $5.3 million for the comparable period in 2020. G&A expenses include $1.0 million and $0.7 million of certain non-cash expenses for the quarters ended June 30, 2021 and 2020, respectively.
- **Net Loss:** X4 reported a net loss of $19.6 million for the quarter ended June 30, 2021, as compared to a net loss of $15.1 million for the comparable period in 2020. Net losses include $1.8 million and $1.2 million of certain non-cash expenses for the quarters ended June 30, 2021 and 2020, respectively.

Conference Call and Webcast
X4 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: 2236266. 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 and a leader in the discovery and development of novel therapies for the treatment of diseases of the immune system 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 number of clinical trials, including 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 and other chronic neutropenia disorders. 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 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; 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 May 6, 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 June 30,		Six Months Ended June 30,	
	2021	**2020**	**2021**	**2020**
License revenue	$ —	$ —	$ —	$ 3,000
Operating expenses:				
Research and development	13,193	9,342	25,297	18,253
General and administrative	5,804	5,316	11,636	9,986
Total operating expenses	18,997	14,658	36,933	28,239
Loss from operations	(18,997)	(14,658)	(36,933)	(25,239)
Other expense, net	(635)	(486)	(1,369)	(895)
Loss before provision for income taxes	(19,632)	(15,144)	(38,302)	(26,134)
Provision for income taxes	6	—	12	148
Net loss	(19,638)	(15,144)	(38,314)	(26,282)
Deemed dividend due to Class B warrant price reset	—	—	(8,239)	—
Net loss attributable to common stockholders	$ (19,638)	$ (15,144)	$ (46,553)	$ (26,282)
Net loss per share attributable to common stockholders- basic and diluted	$ (0.74)	$ (0.76)	$ (1.97)	$ (1.31)
Weighted average common shares outstanding-basic and diluted	26,527	20,032	23,655	20,016

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

	Six months ended June 30,	
	2021	**2020**
Net loss	$ (38,314)	$ (26,282)
Adjustments to reconcile net loss to net cash used in operating activities	4,345	2,591
Changes in operating assets and liabilities	(3,672)	(3,295)
Net cash used in operating activities	(37,641)	(26,986)
Net cash used in provided by investing activities	(582)	(564)
Net cash provided by financing activities	54,117	5,049
Impact of foreign exchange on cash, cash equivalents and restricted cash	(103)	60
Net increase (decrease) in cash, cash equivalents and restricted cash	15,791	(22,441)
Cash, cash equivalents and restricted cash at beginning of period	80,702	128,086
Cash, cash equivalents and restricted cash at end of period	$ 96,493	$ 105,645

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

	June 30, 2021	**December 31, 2020**
Current assets:		
Cash and cash equivalents	$ 95,161	$ 78,708
Research and development incentive receivable	1,053	917
Prepaid expenses and other current assets	5,157	3,682
Total current assets	101,371	83,307
Property and equipment, net	1,745	1,237
Goodwill	27,109	27,109
Right-of-use assets	9,430	7,960
Other assets	2,004	3,258
Total assets	$ 141,659	$ 122,871
Current liabilities:		
Accounts payable	$ 2,528	$ 3,144
Accrued expenses	9,607	8,018
Current portion of lease liability	985	786
Total current liabilities	13,120	11,948
Long-term debt, including accretion, net of discount	33,542	33,178
Lease liabilities	5,350	4,484
Other liabilities	434	462
Total liabilities	52,446	50,072

Redeemable common shares	1,875	—
Total stockholders' equity	87,338	72,799
Total liabilities, redeemable common shares and stockholders' equity	$ 141,659	$ 122,871

Contacts:
Daniel Ferry (Investors)
Managing Director, LifeSci Advisors
daniel@lifesciadvisors.com; (617) 430-7576

Mónica Rouco Molina (Media)
Senior Account Executive, LifeSci Communications
mroucomolina@lifescicomms.com

Source: X4 Pharmaceuticals