



X4 Pharmaceuticals Provides Corporate Update, Including Expected Impact of COVID-19, and Reports First Quarter 2020 Financial Results

May 7, 2020

Data from Phase 2 open-label extension trial of mavorixafor in WHIM syndrome to be presented at the Virtual Edition of the 25th European Hematology Association (EHA) Annual Congress in June 2020

Company continues to expect data from its Phase 1b Waldenström's macroglobulinemia trial in 2H 2020

Company expects a delay into 2022 to report top-line data from its pivotal Phase 3 clinical trial in WHIM syndrome, and a delay into 2021 to report initial data from its Phase 1b trial in Severe Congenital Neutropenia (SCN) due to COVID-19 pandemic

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

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 7, 2020-- [X4 Pharmaceuticals, Inc.](#) (Nasdaq: XFOR), a leader in the discovery and development of novel therapies targeting diseases resulting from dysfunction of the CXCR4 pathway, today reported financial results for the first quarter ended March 31, 2020 and provided a corporate update, including commentary related to the impact of the COVID-19 pandemic on the company.

“During these challenging and unprecedented times, our thoughts are with everyone affected by the COVID-19 pandemic,” said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. “Here at X4, we continue our efforts to advance our lead product candidate, mavorixafor, through clinical development. As presented at our analyst day in April, we recently completed market research that enabled us to update and increase our estimates on the U.S. prevalence of Warts, Hypogammaglobulinemia, Infections, and Myelokathexis, or WHIM, syndrome, our lead indication for mavorixafor.”

Dr. Ragan continued, “Our first priority is the safety and wellbeing of our patients. During the pandemic, we have been regularly assessing the impact of COVID-19 on our current clinical development activities and timelines while taking measures to ensure the health and safety of our employees, patients, and healthcare providers. Our Phase 1b clinical trial of mavorixafor in patients with Waldenström's macroglobulinemia remains on track, with initial data expected in the second half of 2020. Based on our current assessment of the impact of COVID-19 on our clinical trial sites and the pace of enrollment at certain sites, we now expect top-line Phase 3 results from our clinical trial in patients with WHIM syndrome to be delayed into 2022. Based on a similar assessment, we now expect initial data from our Phase 1b clinical trial in patients with Severe Congenital Neutropenia, or SCN, to be delayed into 2021. We will continue to work on minimizing further impact of COVID-19 on our ongoing clinical trials. We plan to provide further updates, including narrowing our guidance regarding upcoming clinical milestones, as we gain greater clarity.”

Current Impact of COVID-19 on X4 Operations and Clinical Trials

In March 2020, X4 enacted a mandatory “work from home” policy for employees with non-laboratory based work and maintained a small number of lab-based employees, compliantly working in shifts, in its Vienna, Austria research facility in order to mitigate the risk of coronavirus spreading among its personnel.

- **Operations:** To date, the company believes that COVID-19 has had minimal effect on its ongoing business operations.
 - Since March 16, 2020, X4 has continued its business operations using technology and communications tools to effectively execute plans while working remotely and in virtual settings. X4 teams have continued to virtually attend meetings with investigators, regulatory authorities, partners, contractors, and investors.
 - Notably, X4 hosted a well-attended Virtual Analyst Day in early April, including a key opinion leader (KOL) presentation and a fireside chat. Additionally, representatives of X4 continue to attend virtual investor conferences and engage with the investment community.
- **Clinical Trials:** To date, the company believes that the COVID-19 pandemic has had the following impact on its clinical programs:
 - Phase 1b Waldenström’s Trial. X4’s Phase 1b clinical trial of mavorixafor in patients with Waldenström’s macroglobulinemia remains on track, with the initial data readout still expected in the second half of 2020. The Phase 1b multi-center, open-label, dose-escalation clinical trial is designed to assess the safety and tolerability of mavorixafor in combination with ibrutinib, as well as to obtain certain efficacy signals in patients with Waldenström’s macroglobulinemia, a rare form of non-Hodgkin’s lymphoma.
 - Phase 1b SCN Trial. X4 is updating its guidance regarding the timing of initial data from its Phase 1b clinical trial of mavorixafor in patients with SCN. The Phase 1b clinical trial is a 14-day, proof-of-concept trial designed to assess the safety and tolerability of daily, oral mavorixafor in up to 45 patients with SCN and other selected congenital neutropenia disorders. The company expects a delay into 2021 to report initial data, which were previously expected in the second half of 2020.
 - Phase 3 WHIM Trial. X4 is updating its guidance on the timing of top-line data from its pivotal Phase 3 clinical trial in patients with WHIM syndrome, a rare, inherited, primary immunodeficiency disease. The 4WHIM trial is a global, 52-week, randomized, double-blind, placebo-controlled clinical trial of mavorixafor for the treatment of WHIM. Following an assessment of and input from many of its clinical trial sites, the company expects a delay into 2022 to report top-line data, which were previously expected in the second half of 2021.

First Quarter / Recent Highlights

- **Increased Guidance on Prevalence of WHIM at Virtual Analyst Day.** X4 conducted a broad online survey of U.S. physicians to validate current prevalence estimates, and conducted additional research using artificial intelligence (AI), interrogating a database of more than 300 million anonymized patient records that spanned 10 years of insurance claims, to help identify additional likely but unconfirmed WHIM patients.
 - Data from the quantitative market research support an estimate of between 1,000 and 1,300 diagnosed WHIM patients in the United States today.
 - The research that used AI identified between 800 and 2,400 additional potential WHIM patients in the United States, which the company believes are likely to be unconfirmed and undiagnosed WHIM patients.
 - Based on this research, X4 updated guidance on its estimated range of diagnosed and undiagnosed WHIM patients in the United States to be between 1,800 and 3,700, a significant increase from its prior estimate of approximately 1,000 WHIM patients.
- **Results of Open-Label Extension of Phase 2 WHIM Trial to Be Presented at EHA 2020.** The Phase 2 trial was an open-label, multi-center trial of mavorixafor in patients with WHIM syndrome. The trial had an initial 24-week treatment period followed by an extension phase. With its abstract accepted

as a poster presentation, X4 plans to present updated data from patients participating in the extension phase of the Phase 2 trial at the Virtual Edition of the [25th European Hematology Association \(EHA\)](#) Annual Congress, which will be held June 11-14, 2020.

- **Granted Two New U.S. Patents for Mavorixafor Composition of Matter and Method of Use.** In February 2020, X4 was issued patents that are expected to provide exclusivity through 2038 for mavorixafor, the company's lead therapeutic candidate, with additional protection for WHIM.
- **Expansion of Debt Facility.** In March 2020, X4 amended its credit agreement with Hercules Capital, Inc. to increase X4's potential borrowing capacity to \$50 million, of which \$25 million remains potentially available.
- **Milestone Payment from Abbisko Therapeutics.** In April 2020, X4 received its first milestone payment of \$3.0 million from Abbisko Therapeutics, X4's solid tumor oncology partner for the Greater China region. This payment, tied to the closing of a financing by Abbisko, represented the first milestone stemming from the companies' collaboration arrangement and was recognized by X4 as license revenue in the first quarter ended March 31, 2020.

First Quarter 2020 Financial Results

- **Cash, Cash Equivalents & Restricted Cash:** X4 had \$117.0 million in cash, cash equivalents and restricted cash, as of March 31, 2020. X4 continues to expect that its cash and cash equivalents will fund company operations into early 2022. In addition, X4 has \$25 million of potential borrowing capacity under its amended credit agreement with Hercules. X4 continues to monitor the impact of the COVID-19 pandemic and to manage cash and working capital accordingly in case of potential changes to its business plans.
- **License Revenues** were \$3.0 million for the first quarter of 2020 related to X4's strategic agreement with Abbisko. Cash related to this revenue was collected in April 2020 and is therefore not reflected in the \$117.0 million of cash, cash equivalents and restricted cash shown above. There were no license revenues recorded in the comparable period in 2019.
- **Research and Development Expenses** were \$8.9 million for the first quarter ended March 31, 2020, as compared to \$5.7 million for the comparable period in 2019.
- **General and Administrative Expenses** were \$4.7 million for the first quarter ended March 31, 2020, as compared to \$4.8 million for the comparable period in 2019.
- **Net Loss:** X4 reported a net loss of \$11.1 million for the first quarter ended March 31, 2020, as compared to a net loss of \$10.9 million for the comparable period in 2019.

Conference Call and Webcast

The company will host a webcast and conference call to discuss its first quarter 2020 results and provide an update on recent corporate activities today at 8:30 a.m. Eastern Time. The webcast will be accessible under "Events & Presentations" on the Investors page of the company's website at www.x4pharma.com. 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: 5573325. 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 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 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 (SCN). X4 is continuing to leverage its insights into CXCR4 biology at its corporate headquarters in Cambridge, 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. Such forward-looking statements include, but are not limited to, statements regarding the anticipated and potential impact of the COVID-19 pandemic on X4’s business and operations, including the conduct of its ongoing clinical trials; X4’s expectations regarding its ability to adapt its business to the evolving COVID-19 pandemic, mitigate its impacts on the business and maintain business continuity, including its ability to protect the safety and wellbeing of its employees and patients; X4’s plans for clinical development of mavorixafor, including the timeline for patient enrollment, the timing of completion and results of its global Phase 3 clinical trial in patients with WHIM syndrome, its Phase 1b clinical trial in combination with ibrutinib in patients with Waldenström’s macroglobulinemia, and its Phase 1b clinical trial as monotherapy in patients with Severe Congenital Neutropenia (SCN); the expected timing of guidance and data disclosures on X4’s current clinical trials; and estimates regarding the WHIM patient population and potential market opportunity. Any forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties including, but are not limited to, the uncertainty related to the impact of the COVID-19 pandemic; whether X4’s clinical trials will be fully enrolled and completed when anticipated; the risk that the COVID-19 pandemic will impact clinical trial site initiation and patient enrollment or the ability of investigators and patients to adhere to clinical trial protocols or ability for follow-up visits with healthcare providers; the risk that X4’s ongoing trials and studies may be delayed for reasons in addition to the COVID-19 pandemic and may not have satisfactory outcomes; the risk that the supply chain for mavorixafor will be interrupted as a result of the COVID-19 pandemic; the impact on X4’s operations and financial results from the spread of COVID-19 in the geographies where X4 and its third-party partners operate; potential adverse effects arising from the testing or use of mavorixafor or other product candidates; the risk that costs required to develop product candidates or to expand X4’s operations will be higher than anticipated; whether preliminary or interim results from a clinical trial will be predictive of the final results of the trial or of other trials; whether mavorixafor will successfully advance through clinical development on a timely basis; whether mavorixafor will ultimately receive regulatory approval; as well as those risks and uncertainties described in the section titled “Risk Factors” in X4’s Annual Report on Form 10-K for the year ended December 31, 2019 filed with the Securities and Exchange Commission (SEC) on March 12, 2020, and in subsequent filings X4 makes with the SEC. All information in this press release speaks only as of the date hereof, and X4 undertakes no obligation to update the information to reflect new events or circumstances, except as required by law.

X4 PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS

(in thousands, except per share amounts)

**Three Months Ended
March 31,**

	2020	2019
License revenue	\$ 3,000	\$ -
Operating expenses:		
Research and development	8,911	5,655
General and administrative	4,670	4,783
Total operating expenses	<u>13,581</u>	<u>10,438</u>
Loss from operations	(10,581)	(10,438)
Other expense, net	(409)	(435)
Loss before provision for income taxes	<u>(10,990)</u>	<u>(10,873)</u>
Provision for income taxes	148	-
Net loss	<u>(11,138)</u>	<u>(10,873)</u>
Adjustments related to convertible preferred stock	-	(592)
Net loss attributable to common stockholders	\$ (11,138)	\$ (11,465)
Net loss per share attributable to common stockholders- basic and diluted	\$ (0.56)	\$ (6.67)
Weighted average common shares outstanding-basic and diluted	20,014	1,718

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

	Three months ended March 31,	
	2020	2019
Net loss	\$ (11,138)	\$ (10,873)
Adjustments to reconcile net loss to net cash used in operating activities	1,158	659
Changes in operating assets and liabilities	(5,547)	(1,536)
Net cash used in operating activities	<u>(15,527)</u>	<u>(11,750)</u>
Net cash (used in) provided by investing activities	(555)	26,406
Net cash provided by financing activities	4,984	113
Impact of foreign exchange on cash, cash equivalents and restricted cash	(34)	(21)
Net (decrease) increase in cash, cash equivalents and restricted cash	<u>\$ (11,132)</u>	<u>\$ 14,748</u>
Cash, cash equivalents and restricted cash at beginning of period	\$ 128,086	\$ 8,498
Cash, cash equivalents and restricted cash at end of period	<u>\$ 116,954</u>	<u>\$ 23,246</u>

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

	March 31,	December 31,
	2020	2019
Current assets:		
Cash and cash equivalents	\$ 115,054	\$ 126,184
Accounts receivable	3,000	-
Research and development incentive receivable	416	1,998

Prepaid expenses and other current assets	3,480	1,096
Total current assets	121,950	129,278
Property and equipment, net	475	403
Goodwill	27,109	27,109
Right-of-use assets	1,803	1,959
Other assets	2,380	1,949
Total assets	\$ 153,717	\$ 160,698
Current liabilities:		
Accounts payable	\$ 1,248	\$ 2,088
Accrued expenses	5,786	6,461
Current portion of lease liability	916	898
Total current liabilities	7,950	9,447
Long-term debt, including accretion, net of discount	25,266	20,097
Lease liabilities	1,684	1,918
Other liabilities	26	16
Total liabilities	34,926	31,478
Total stockholders' equity	118,791	129,220
Total liabilities and stockholders' equity	\$ 153,717	\$ 160,698

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