
**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Data of earliest event reported): **July 30, 2020**

X4 PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of incorporation)

001-38295
(Commission File Number)

27-3181608
(IRS Employer Identification No.)

955 Massachusetts Avenue, 4th Floor
Cambridge, Massachusetts
(Address of principal executive offices)

02139
(Zip Code)

(857) 529-8300
(Registrant's telephone number, including area code)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

| Title of each class | Trading Symbol(s) | Name of each exchange on which registered |
|---|-------------------|---|
| Common Stock, par value \$0.001 per share | XFOR | The Nasdaq Stock Market LLC |

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (240.12b-2 of this chapter). Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act

Item 2.02 Results of Operations and Financial Condition.

On July 30, 2020, X4 Pharmaceuticals, Inc. (the "Company") issued a press release announcing its financial results and other business highlights for the quarter ended June 30, 2020. A copy of the press release is being furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information contained in this Item 2.02 in the Current Report on Form 8-K (including Exhibit 99.1) shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934 or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such a filing.

Item 9.01 Financial Statements and Exhibits.**Exhibit
Number Description**

99.1 [Press Release of X4 Pharmaceuticals, Inc. dated July 30, 2020.](#)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934 the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: July 30, 2020

X4 PHARMACEUTICALS, INC.

By: /s/ Derek Meisner

Derek Meisner

General Counsel



Exhibit 99.1

X4 Pharmaceuticals Reports Second Quarter 2020 Financial Results and Provides Corporate Update

Presentation of positive clinical data at EHA 2020 supporting the ongoing Phase 3 trial in WHIM syndrome

Initial data expected later this year from Phase 1b clinical trial in Waldenström's macroglobulinemia

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

CAMBRIDGE, Mass. — July 30, 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 second quarter ended June 30, 2020. The company also provided an update on its lead investigational candidate mavorixafor, a novel small molecule in a Phase 3 clinical trial for patients with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome and in two Phase 1b trials in patients with Waldenström's macroglobulinemia (WM) and Severe Congenital Neutropenia (SCN).

"Despite the challenges posed by the ongoing COVID-19 pandemic, we achieved significant progress during the second quarter, benefiting from two key events related to the WHIM syndrome indication for mavorixafor, while continuing to advance our program in the Waldenström's indication," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "At our Analyst Day in early April, we presented market research data that supported significantly increasing our disease prevalence estimate for WHIM in the U.S., and during our presentation at the European Hematology Association (EHA) Annual Congress in June, we highlighted Phase 2 mavorixafor data that strengthen our confidence in mavorixafor's potential to be a disease-modifying therapy in patients with WHIM syndrome and in the Phase 3 trial design."

Dr. Ragan continued, "While our public presentations during the first half of 2020 focused primarily on WHIM syndrome, we expect that the second half of 2020 will see us increase exposure to our program in patients with Waldenström's macroglobulinemia, a rare form of lymphoma, where we look forward to announcing initial Phase 1b clinical results towards the end of the year." The ongoing Phase 1b clinical trial is expected to enroll between 12 and 18 patients with WM and is a multi-center, open-label, dose-escalation clinical trial assessing the safety and tolerability of mavorixafor in combination with ibrutinib. The trial is being conducted as part of a collaboration with The Leukemia & Lymphoma Society to accelerate the development of mavorixafor for the treatment of WM.

"As we await these important results," Dr. Ragan concluded, "we continue to expect top-line Phase 3 results of mavorixafor in WHIM syndrome in 2022 and initial data from our Phase 1b trial of mavorixafor in patients with SCN in 2021. In light of the continued uncertainties surrounding COVID-19, we intend to provide further clarity around these timelines as soon as is practicable."

Recent Highlights

- **Increased Guidance on Prevalence of WHIM at Virtual Analyst Day**, based on in-depth, internal market research indicating the range of diagnosed and undiagnosed WHIM patients in the United States to be greater than 3,500, a significant increase from the prior estimate of approximately 1,000 patients diagnosed with WHIM syndrome.
- **Presented Positive Data from the Phase 2 Open-Label Extension Study of Mavorixafor in WHIM Syndrome at EHA 2020**, supporting the selection of 400 mg once-daily and time above threshold for absolute neutrophil counts (TAT_{ANC}) as the dose and primary endpoint in the Phase 3 trial, respectively, and long-term favorable tolerability. At the median follow-up of 16.5 months, data revealed sustained, dose-dependent increases in WBC (white blood cells), ANC (absolute neutrophil count), and ALC (absolute lymphocyte count), with higher doses of mavorixafor shown to increase the TAT_{ANC} at least 4.5-fold versus lower doses. In patients treated for at least 6 months, mavorixafor also significantly decreased the yearly rate of infections versus the 12 months prior to treatment and effected a 75% reduction in cutaneous warts versus baseline.
- **Promoted Renato Skerlj, Ph.D., to the position of Chief Scientific Officer**. Dr. Skerlj, one of the scientific founders of X4 and co-inventor of mavorixafor, has more than 25 years of experience leading the discovery and development of disease-modifying small molecule drugs to treat genetically defined rare diseases. In this expanded role, Dr. Skerlj leads all research and non-clinical development functions at X4, overseeing operations at the company's Vienna, Austria research facility as well as the company's efforts to advance and expand its pipeline targeting additional rare diseases.

Second Quarter 2020 Financial Results

- **Cash, Cash Equivalents & Restricted Cash**: X4 had \$105.6 million in cash, cash equivalents and restricted cash, as of June 30, 2020. X4 continues to expect that its cash and cash equivalents will fund company operations into early 2022. In addition, X4 continues to have \$25 million of potential borrowing capacity under its amended credit agreement with Hercules.
- **Research and Development Expenses** were \$9.3 million for the second quarter ended June 30, 2020, as compared to \$8.9 million for the comparable period in 2019.
- **General and Administrative Expenses** were \$5.3 million for the second quarter ended June 30, 2020, as compared to \$4.6 million for the comparable period in 2019.
- **Net Loss**: X4 reported a net loss of \$15.1 million for the second quarter ended June 30, 2020 as compared to a net loss of \$13.4 million for the comparable period in 2019.

Conference Call and Webcast

The Company 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: 6091009. 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 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. 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 that concern X4’s expectations, strategy, plans or intentions. Forward-looking statements include, without limitation, statements regarding the anticipated and potential impact of the COVID-19 pandemic on X4’s business and operations, including the timing of its ongoing clinical trials; X4’s borrowing capacity under credit agreements; X4’s plans for clinical development of mavorixafor, including 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; 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 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, the risks and uncertainties 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 7, 2020, 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.

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

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|--------------------------------|-------------|------------------------------|-------------|
| | 2020 | 2019 | 2020 | 2019 |
| License revenue | \$ — | \$ — | \$ 3,000 | \$ — |
| Operating expenses: | | | | |
| Research and development | 9,342 | 8,854 | 18,253 | 14,509 |
| General and administrative | 5,316 | 4,560 | 9,986 | 9,343 |
| Total operating expenses | 14,658 | 13,414 | 28,239 | 23,852 |
| Loss from operations | (14,658) | (13,414) | (25,239) | (23,852) |
| Other income (expense), net | (486) | 31 | (895) | (404) |
| Loss before provision for income taxes | (15,144) | (13,383) | (26,134) | (24,256) |
| Provision for income taxes | — | — | 148 | — |
| Net loss | (15,144) | (13,383) | (26,282) | (24,256) |
| Adjustments related to convertible preferred stock | — | — | — | (592) |
| Net loss attributable to common stockholders | \$ (15,144) | \$ (13,383) | \$ (26,282) | \$ (24,848) |
| Net loss per share attributable to common stockholders- basic and diluted | \$ (0.76) | \$ (1.02) | \$ (1.31) | \$ (3.32) |
| Weighted average common shares outstanding-basic and diluted | 20,032 | 13,177 | 20,016 | 7,479 |

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

| | Six months ended June 30, | |
|--|---------------------------|-------------|
| | 2020 | 2019 |
| Net loss | \$ (26,282) | \$ (24,256) |
| Adjustments to reconcile net loss to net cash used in operating activities | 2,591 | 1,457 |
| Changes in operating assets and liabilities | (3,295) | (3,243) |
| Net cash used in operating activities | (26,986) | (26,042) |
| Net cash (used in) provided by investing activities | (564) | 26,396 |
| Net cash provided by financing activities | 5,049 | 86,791 |
| Impact of foreign exchange on cash, cash equivalents and restricted cash | 60 | (2) |
| Net (decrease) increase in cash, cash equivalents and restricted cash | \$ (22,441) | \$ 87,143 |
| Cash, cash equivalents and restricted cash at beginning of period | \$ 128,086 | \$ 8,498 |
| Cash, cash equivalents and restricted cash at end of period | \$ 105,645 | \$ 95,641 |

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

| | June 30, 2020 | December 31, 2019 |
|--|----------------------|--------------------------|
| Current assets: | | |
| Cash and cash equivalents | \$ 103,744 | \$ 126,184 |
| Research and development incentive receivable | 509 | 1,998 |
| Prepaid expenses and other current assets | 5,634 | 1,096 |
| Total current assets | 109,887 | 129,278 |
| Property and equipment, net | 421 | 403 |
| Goodwill | 27,109 | 27,109 |
| Right-of-use assets | 1,644 | 1,959 |
| Other assets | 3,473 | 1,949 |
| Total assets | \$ 142,534 | \$ 160,698 |
| Current liabilities: | | |
| Accounts payable | \$ 1,838 | \$ 2,088 |
| Accrued expenses | 8,003 | 6,461 |
| Current portion of lease liability | 940 | 898 |
| Total current liabilities | 10,781 | 9,447 |
| Long-term debt, including accretion, net of discount | 25,398 | 20,097 |
| Lease liabilities | 1,445 | 1,918 |
| Other liabilities | 26 | 16 |
| Total liabilities | 37,650 | 31,478 |
| Total stockholders' equity | 104,884 | 129,220 |
| Total liabilities and stockholders' equity | \$ 142,534 | \$ 160,698 |

Investors and Media:

Candice Ellis, 857-341-1043
Director, Corporate Communications & Investor Relations
Candice.Ellis@x4pharma.com