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): **March 4, 2021**

X4 PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-38295**	**27-3181608**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

61 North Beacon Street, 4th Floor	
Boston, Massachusetts	**02134**
(Address of principal executive offices)	(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 March 4, 2021, X4 Pharmaceuticals, Inc. (the “Company”) issued a press release announcing its financial results and other business highlights for the fourth quarter and full year ended December 31, 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.

(d) Exhibits.

Exhibit No.	**Description**
99.1	Press release of X4 Pharmaceuticals Inc. dated March 4, 2021.

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.

X4 PHARMACEUTICALS, INC.

Date: March 4, 2021

By: /s/ Derek Meisner
Derek Meisner
General Counsel

Exhibit 99.1

X4 Pharmaceuticals Reports Fourth Quarter and Full Year 2020 Financial Results and Provides Corporate Update

2021 expected to be a year of key value-driving catalysts as company advances multiple clinical programs with lead candidate mavorixafor

Company anticipates completing full enrollment in WHIM Phase 3 trial in 2021 and presenting clinical data from the ongoing Phase 1b Waldenström's trial throughout the year

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

BOSTON, Mass. – March 4, 2021 -- 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 fourth quarter and full year ended December 31, 2020. The company also provided an update on its lead product candidate, mavorixafor, a novel 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 trials for patients with Waldenström's macroglobulinemia (Waldenström's) and Severe Congenital Neutropenia (SCN), respectively.

"As many companies did, we encountered a number of unexpected challenges in 2020, most notably the COVID-19 pandemic. However, I could not be prouder of our accomplishments, as we remained steadfast in our commitment to advance our lead candidate, mavorixafor, in a number of rare disease indications," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We believe that we have much to look forward to this year, as we have overcome these obstacles, have made significant progress in advancing our clinical and research programs, and assembled an exceptional team to execute on our 2021 goals and beyond.

"On the clinical front, we continue to expect top-line data from our Phase 3 WHIM trial in 2022 and, as our Phase 1b Waldenström's trial is progressing well, we continue to expect initial data from that study in the first half of 2021, with a more fulsome dataset expected towards the end of 2021. We also anticipate initial data from our SCN Phase 1b trial in 2021."

Recent Highlights

- **Received Fast Track and Rare Pediatric Designations (RPD) from the FDA for Mavorixafor for the treatment of WHIM Syndrome**: These FDA designations further recognize WHIM as a serious condition with a clear unmet need in both adult and pediatric populations.
    - Through the Fast Track program, X4 will be eligible for more frequent meetings with the FDA to discuss the drug's development plan, protocols and clinical data that would support mavorixafor's potential approval for WHIM.
    - Under the RPD program, a sponsor who receives an approval for a drug for a "rare pediatric disease" and a Fast Track designation may qualify for a voucher that can be

redeemed to receive a priority review by the FDA for any subsequent marketing application for a different product. Such a voucher is transferable and may be sold.
    - Mavorixafor was previously granted Breakthrough Therapy Designation by the FDA, as well as Orphan Drug status by the FDA and the European Commission for the treatment of WHIM syndrome.
- **Strengthened Leadership Team**: During the Fourth Quarter of 2020:
    - Diego Cadavid, M.D., joined as Chief Medical Officer, bringing more than 20 years of industry experience and having led multiple programs through all phases of clinical development, including small molecules and biologics for the treatment of rare and immunological diseases.
    - Art Taveras, Ph.D., joined as Chief Scientific Officer with more than 30 years of experience leading small molecule research and development programs focused on cancer, dysregulated immune disorders, neurodegeneration, and metabolic diseases.
    - Alison Lawton was appointed to the company's Board of Directors, bringing more than 30 years of experience across a full spectrum of drug development and commercial roles and having previously served on X4's corporate advisory board and as consulting Chief Operating Officer.

Fourth Quarter 2020 Financial Results

- **Cash, Cash Equivalents & Restricted Cash**: X4 had $80.7 million in cash, cash equivalents and restricted cash as of December 31, 2020. X4 continues to expect that its cash and cash equivalents will fund company operations into 2022.

- **Research and Development Expenses** were $12.3 million and $41.9 million for the fourth quarter and full year ended December 31, 2020, respectively, as compared to $7.1 million and $30.2 million for the comparable periods in 2019, respectively. R&D expenses include $0.6 million and $2.3 million of certain non-cash expenses for the fourth quarter and fully year ended December 31, 2020, respectively.

- **General and Administrative Expenses** were $5.4 million and $20.9 million for the fourth quarter and full year ended December 31, 2020, respectively, as compared to $3.9 million and $17.6 million for the comparable periods in 2019, respectively. G&A expenses include $0.8 million and $3.1 million of certain non-cash expenses for the fourth quarter and full year ended December 31, 2020, respectively.

- **Net Loss:** X4 reported net losses of $18.4 million and $62.1 million for the fourth quarter and full year ended December 31, 2020 as compared to net losses of $10.8 million and $52.8 million for the comparable periods in 2019, respectively. Net losses include $1.4 million and $5.4 million of certain non-cash expenses for the fourth quarter and full year ended December 31, 2020, respectively. Net losses in the full year of 2019 included a $3.9 million loss on the sale of non-financial assets.

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: 1053189. 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 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 of mavorixafor and X4's other product candidates or programs, the potential benefits resulting from Fast Track and Rare Pediatric Disease designations, 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, 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 November 5, 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.

(Tables Follow)

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

	Three Months Ended December 31,		Year Ended December 31,	
	2020	2019	2020	2019
License revenue	$ —	$ —	$ 3,000	$ —
Operating expenses:				
Research and development	12,298	7,065	41,932	30,163
General and administrative	5,357	3,914	20,942	17,640
Loss on transfer of non-financial assets	—	(104)	—	3,900
Total operating expenses	17,655	10,875	62,874	51,703
Loss from operations	(17,655)	(10,875)	(59,874)	(51,703)
Other income (expense), net	(745)	38	(2,109)	(1,104)
Loss before provision for income taxes	(18,400)	(10,837)	(61,983)	(52,807)
Provision for income taxes	—	—	148	—
Net loss	(18,400)	(10,837)	(62,131)	(52,807)
Adjustments related to convertible preferred stock	—	—	—	(592)
Net loss attributable to common stockholders	$ (18,400)	$ (10,837)	$ (62,131)	$ (53,399)
Net loss per share attributable to common stockholders- basic and diluted	$ (0.91)	$ (0.66)	$ (3.09)	$ (4.63)
Weighted average common shares outstanding-basic and diluted	20,174	16,466	20,077	11,530

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

	Year ended December 31,	
	2020	2019
Net loss	$ (62,131)	$ (52,807)
Adjustments to reconcile net loss to net cash used in operating activities	7,376	7,988
Changes in operating assets and liabilities	(4,063)	(3,236)
Net cash used in operating activities	(58,818)	(48,055)
Net cash (used in) provided by investing activities	(1,362)	27,232
Net cash provided by financing activities	12,394	140,661
Impact of foreign exchange on cash, cash equivalents and restricted cash	402	(250)
Net (decrease) increase in cash, cash equivalents and restricted cash	(47,384)	119,588
Cash, cash equivalents and restricted cash at beginning of period	128,086	8,498
Cash, cash equivalents and restricted cash at end of period	$ 80,702	$ 128,086

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

	December 31, 2020	December 31, 2019
Current assets:		
Cash and cash equivalents	$ 78,708	$ 126,184
Research and development incentive receivable	917	1,998
Prepaid expenses and other current assets	3,682	1,096
Total current assets	83,307	129,278
Property and equipment, net	1,237	403
Goodwill	27,109	27,109
Right-of-use assets	7,960	1,959
Other assets	3,258	1,949
Total assets	$ 122,871	$ 160,698
Current liabilities:		
Accounts payable	$ 3,144	$ 2,088
Accrued expenses	8,018	6,461
Current portion of lease liability	786	898
Total current liabilities	11,948	9,447
Long-term debt, including accretion, net of discount	33,178	20,097
Lease liabilities	4,484	1,918
Other liabilities	462	16
Total liabilities	50,072	31,478
Total stockholders' equity	72,799	129,220
Total liabilities and stockholders' equity	$ 122,871	$ 160,698

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