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David Bautz, PhD
312-265-9471
dbautz@zacks.com

scr.zacks.com
10 S. Riverside Plaza, Chicago, IL 60606

X4 Pharmaceuticals, Inc

(XFOR-NASDAQ)

XFOR: NDA for Mavorixafor in WHIM Syndrome in Early 2H23…

Based on our probability adjusted DCF model that takes into account potential future revenues of mavorixafor and selling a PRV, XFOR is valued at $4.50 per share. This model is highly dependent upon the clinical and commercial success of mavorixafor and will be adjusted accordingly based on future results.

Current Price (08/15/23)	$1.25
Valuation	**$4.50**

OUTLOOK

On August 10, 2023, X4 Pharmaceuticals, Inc. (XFOR) announced financial results for the second quarter of 2023 and provided a business update. The company remains on track to file a New Drug Application (NDA) for mavorixafor in WHIM syndrome early in the second half of 2023. Initial data from the Phase 2 trial in chronic neutropenic disorders was also presented. The first three patients that have been on therapy for at least three months showed a positive response to therapy as exhibited by increased absolute neutrophil counts (ANC) and two of the patients have been taken off G-CSF therapy. An abstract has been submitted to the December ASH meeting, at which time we anticipate additional data being presented. A Phase 3 study in chronic neutropenia is anticipated to initiate in the first half of 2024.

SUMMARY DATA

52-Week High	**$2.49**
52-Week Low	**$0.69**
One-Year Return (%)	**-14.38**
Beta	**0.69**
Average Daily Volume (sh)	**3,296,766**
Shares Outstanding (mil)	**122**
Market Capitalization ($mil)	**$153**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**63**
Insider Ownership (%)	**1**
Annual Cash Dividend	**$0.00**
Dividend Yield (%)	**0.00**
5-Yr. Historical Growth Rates	
Sales (%)	**N/A**
Earnings Per Share (%)	**N/A**
Dividend (%)	**N/A**
P/E using TTM EPS	**N/A**
P/E using 2023 Estimate	**-1.9**
P/E using 2024 Estimate	**-2.5**

Risk Level	**Above Avg.**
Type of Stock	**Small-Growth**
Industry	**N/A**

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2022	0.0 A	0.0 A	0.0 A	0.0 A	0.0 A
2023	0.0 A	0.0 A	0.0 E	0.0 E	0.0 E
2024					13.0 E
2025					51.0 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2022	-$0.72 A	-$0.60 A	-$0.26 A	-$0.29 A	-$1.52 A
2023	-$0.16 A	-$0.33 A	-$0.19 E	-$0.19 E	-$0.88 E
2024					-$0.51 E
2025					-$0.32 E

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WHAT'S NEW

Business Update

NDA on Track for Mavorixafor for WHIM in Early 2H23

On August 10, 2023, X4 Pharmaceuticals, Inc. (XFOR) provided an update on mavorixafor, the company's lead development candidate that is a small molecule antagonist of chemokine receptor CXCR4. Mavorixafor is able to mobilize mature, functional white blood cells from the bone marrow into the bloodstream and thus may provide benefit for a wide range of chronic neutropenic disorders, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency.

- In November 2022, X4 announced positive topline results for the Phase 3 WHIM trial. The trial met its primary endpoint, with mavorixafor achieving clinical and statistical superiority over placebo (P<0.0001) when measuring the length of time that participants' absolute neutrophil count (ANC) remained above a clinically meaningful threshold of 500 cells per microliter over 24-hour periods at four time points throughout the 52-week trial.

- In April 2023, X4 announced that a late-breaker abstract had been accepted for oral presentation at the Annual Meeting of the Clinical Immunology Society (CIS). The presentation included new data related to clinical secondary endpoints from the 4WHIM Phase 3 trial. The company also hosted a virtual event to present and discuss the new data from the 4WHIM Phase 3 trial. There was a 58% reduction in the annualized infection rate for those treated with mavorixafor compared to placebo (P<0.01). In addition to reducing the number of infections, treatment with mavorixafor also reduced the severity of infections. Lastly, the duration of infections was also reduced by mavorixafor. The mean total time with infection for those treated with mavorixafor was approximately two weeks compared to approximately seven weeks for those treated with placebo.

For a full overview of the data from the Phase 3 WHIM trial, please see our initiation report (here). Based on the positive data from the 4WHIM trial, X4 will be filing a new drug application (NDA) with the FDA early in the second half of 2023. Mavorixafor has been granted Rare Pediatric Designation by the FDA for the treatment of WHIM syndrome, thus approval of mavorixafor for WHIM syndrome could also include the issuance of a priority review voucher (PRV). PRVs are fully transferable and a number of them have recently sold for approximately $100 million each.

Mavorixafor for Chronic Neutropenic Disorders

Chronic neutropenia (CN) is a disease that is defined as having severe, chronic (> 3 months) low levels of circulating neutrophils. Its etiology can be idiopathic (unknown origin), cyclic (neutrophil levels rise and fall in a cycle), or congenital (genetic cause). The following slide shows the results of a market survey conducted by X4 to better define the target market for mavorixafor in CN. It is estimated there are a total of approximately 50,000 CN patient's in the U.S., with an initial target market of approximately 15,000 individuals with high unmet needs (adolescents and adults with severe/recurrent infections and/or G-CSF treatment).

Initial Target Market for Mavorixafor in CN: Those With High Unmet Need

Significant Opportunity For Expansion

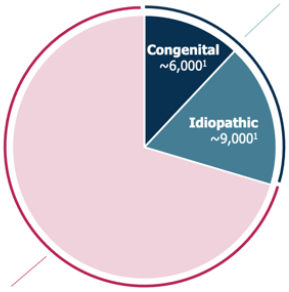
Methodology[1]

- Provides a real-world picture: U.S. CN landscape and needs
- Conducted interviews with and surveyed academic and community-based CN treaters (n=90)
- Reviewed detailed patient charts (n=300+)
- Triangulated using further claims (ICD-10 code) analysis using 6-year lookback

Defining Target Market & High Unmet Need

- **Excludes:** <12 years of age[2], CN disorders unlikely to respond to CXCR4 antagonism[2]
- **Includes:** only those with: history of severe/recurrent infections and/or history of G-CSF treatment
- **Finding:** ~90% of those with high unmet need (e.g., severe/recurrent infections) are also treated with G-CSF

Est. US Chronic Neutropenia Population (~50,000[1] Total)



~ 15,000 Initial Target Market with High Unmet Needs

adolescents and adults with history of severe / recurrent infections and/or G-CSF treatment

Significant Market Expansion Opportunities

Ages below 12 years old | Mild/Moderate Disease | QoL/G-CSF Intolerant

Source: X4 Pharmaceuticals, Inc.

X4 initially evaluated mavorixafor in a single-dose Phase 1b study in patients with CN. The rationale for testing mavorixafor in CN is due to neutrophil maturation, which occurs in the bone marrow, and mobilization out of the bone marrow being controlled by the CXCL12/CXCR4 signaling axis. The bone marrow contains approximately 20 times more neutrophils than are seen in circulation, and approximately 100 billion mature neutrophils are mobilized from the bone marrow each day (Furze *et al*., 2008). Maturation and mobilization of neutrophils results from downregulation of CXCR4, which decreases CXCR4 signaling, and antagonism of CXCR4 can also inhibit this signaling pathway (Mosi *et al*., 2012).

The Phase 1b CN trial was a success, as patients with all CN disorders (idiopathic, congenital, and cyclic) responded to mavorixafor treatment, with an increase in ANC of >2,000 cells/μL across all disorders. In severe neutropenia, all patients showed increased ANC to normal levels, which shows the potential for mavorixafor monotherapy. In addition, mavorixafor increased ANCs to normal levels in patients who were being treated with G-CSF, which supports further studies exploring the role of mavorixafor in replacing G-CSF therapy in these patients. Lastly, mavorixafor was well tolerated, all treatment-related adverse events were deemed to be low grade and consistent with what was seen in previous trials, and there were no treatment-related serious adverse events.

X4 is now conducting a Phase 2 trial to test the durability of ANC levels along with G-CSF reductions. The company recently presented results for the first three patients from the Phase 2 trial that have been on treatment for at least three months. These early positive results show that all three patients had ANC levels increase to the normal range and two of the three patients are now off G-CSF therapy. These results are summarized in the following slide.

Preliminary Results[1]: Phase 2 Trial of Mavorixafor in Chronic Neutropenia

Emerging Data Show Durability & Successful G-CSF Reduction

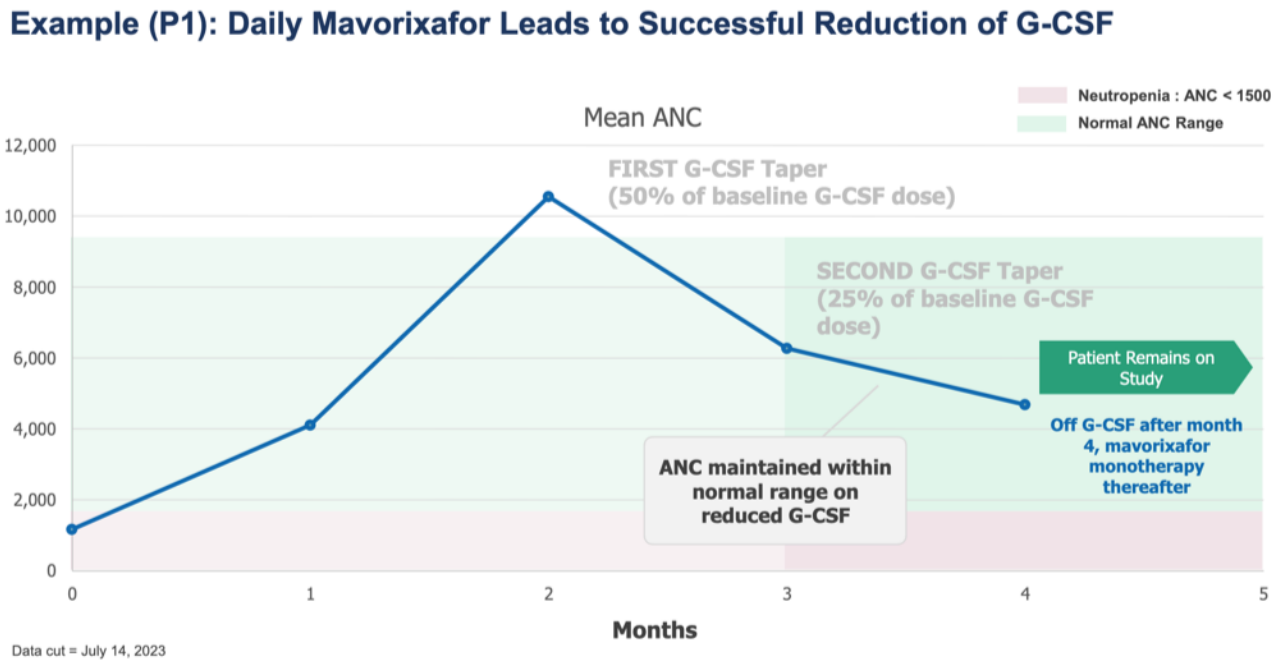
Participants		Baseline: Pre-Mav Dosing		After Mavorixafor Dosing		
	Diagnosis	G-CSF Use?	ANC Levels	Months Treated	ANC Levels	G-CSF Reduction
P1	CIN	Yes, chronic	Neutropenic with G-CSF	4	Increased response in ANC to normal ranges vs. BL	50% reduction at Month 2; off G-CSF after Month 4
P2	CIN	Yes, chronic	Normal with G-CSF	3	Increased response in ANC vs. BL	50% reduction at Month 2; off G-CSF after Month 3
P3	ELANE/Cyclic	Yes, chronic	Neutropenic with G-CSF	4	Increased response in ANC to normal ranges vs. BL	Recommended

Cohort of patients on G-CSF at baseline, dosed for ≥ 3 months has shown:

- Tolerability: Well tolerated in combination with G-CSF: ***no serious adverse events (SAEs), chronic dosing supported***
- Durability: Large, sustained increases in ANC: ***into normal ranges***
- G-CSF: Reduction of G-CSF treatment: ***physicians elected to reduce G-CSF dose vs. mavorixafor***

Source: X4 Pharmaceuticals, Inc.

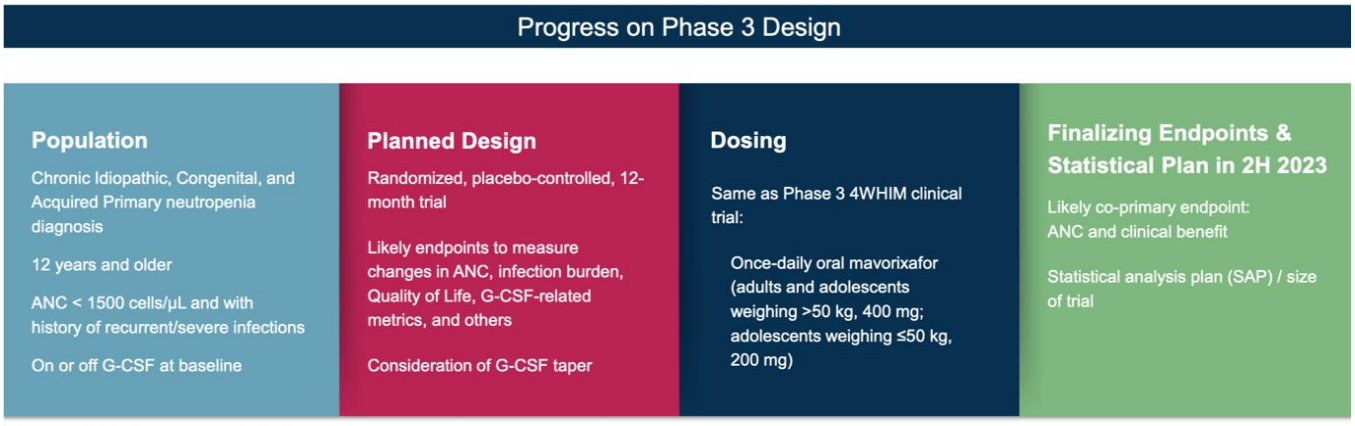
An example of the change in mean ANC is given below for patient P1. After achieving an ANC of >10,000, the patient's G-CSF level was reduced by 50% at month 2. Another 25% taper of G-CSF was performed in month 3 and the patient was off G-CSF therapy as of month 4. The ANC level has stayed in the normal range from month 2 through month 4.



Source: X4 Pharmaceuticals, Inc.

In summary, the early Phase 2 data is highly encouraging as mavorixafor combined with G-CSF has been well tolerated with no serious adverse events reported. In addition, all of the initial participants in the trial have demonstrated an increase in ANC compared to baseline and these increases have been sustained for months in the normal range. Physicians participating in the trial have been given the opportunity to reduce G-CSF dosing as they deem appropriate (e.g., with sustained ANC levels), which has occurred with two of the patients. We anticipate additional data being shared at the ASH meeting in December 2023.

The following slide gives an overview of some of the major points for the upcoming Phase 3 study, which we anticipate will initiate in the first half of 2024. The final study design will be contingent upon discussion with the FDA.



Source: X4 Pharmaceuticals, Inc.

Financial Update

On August 10, 2023, X4 announced financial results for the second quarter of 2023. As expected, the company did not report any revenues in the second quarter of 2023. R&D expenses for the second quarter of 2023 were $15.6 million, compared to $13.8 million for the second quarter of 2022. The increase in expenses was primarily due to higher accrued in-license fees and third-party costs associated with the Phase 3 trial. SG&A expenses for the second quarter of 2023 were $10.2 million, compared to $6.7 million in the second quarter of 2022.

As of June 30, 2023, X4 had cash, cash equivalents, marketable securities, and restricted cash of approximately $142.3 million. On August 3, 2023, the company announced the closing of a $115 million loan facility with Hercules Capital, Inc. The company also announced it drew down $22.5 million upon closing of the transaction. The term loan provides for up to $115 million available to be funded in multiple tranches. In addition to its initial drawdown, X4 may draw an additional tranche of up to $20 million for a period of time following U.S. approval of mavorixafor for WHIM syndrome. An additional tranche will be available in the amount up to $7.5 million for a period of time following achievement of a certain clinical development-related milestone. The final tranche of up to $32.5 million is subject to the approval of the lenders. The facility refinanced $32.5 million in outstanding principal indebtedness and extends the initial interest-only period and maturity of future borrowings.

Including the $22.5 million drawn down from the Hercules loan, we estimate that X4 has sufficient capital to fund operations into 2025. This does not include any additional drawdowns from the debt facility or the potential sale of a Priority Review Voucher the company should receive upon approval of mavorixafor for the treatment of WHIM syndrome. As of August 7, 2023, X4 had approximately 164.4 million shares outstanding and, when factoring in stock options and warrants, a fully diluted share count of 286.5 million.

Conclusion

The initial data from the Phase 2 trial of mavorixafor is very encouraging and we look forward to additional data from the trial, which we expect at ASH in December 2023. We also look forward to updates regarding the design of the Phase 3 clinical trial in CN, which we anticipate initiating in the first half of 2024. The company is expected to file the NDA for mavorixafor for the treatment of WHIM with the FDA early in the second half of 2023. This could lead to approval in the first half of 2024. With no changes to our model our valuation stands at $4.50.

PROJECTED FINANCIALS

X4 Pharmaceuticals, Inc.	2022 A	Q1 A	Q2 A	Q3 E	Q4 E	2023 E	2024 E	2025 E
Mavorixafor	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$13.0	$51.0
License and other revenues	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Total Revenues	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$13.0**	**$51.0**
Cost of revenues	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$1.3	$5.1
Gross Margin	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*90%*	*90%*
Research & development	$61.1	$22.1	$15.6	$23.0	$24.0	$84.7	$64.0	$66.0
General & administrative	$27.0	$7.2	$10.2	$7.3	$7.4	$32.1	$40.0	$42.0
Goodwill impairment	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Gain on sale of nonfinancial assets	($0.5)	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Operating Income	($87.6)	($29.3)	($25.8)	($30.3)	($31.4)	($116.8)	($91.0)	($57.0)
Operating Margin	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*-700.0%*	*-111.8%*
Non-Operating Expenses (Net)	($6.3)	$5.3	($29.9)	($1.5)	($1.5)	($27.6)	($6.0)	($6.0)
Pre-Tax Income	($93.8)	($24.0)	($55.7)	($31.8)	($32.9)	($144.4)	($97.0)	($63.0)
Deemed Dividend on Class B Warrant price reset	$2.5	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Income Taxes	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	**($96.4)**	**($24.0)**	**($55.7)**	**($31.8)**	**($32.9)**	**($144.4)**	**($97.0)**	**($63.0)**
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($1.52)**	**($0.16)**	**($0.33)**	**($0.19)**	**($0.19)**	**($0.88)**	**($0.51)**	**($0.32)**
YOY Growth	-	-	-	-	-	-	-	-
Basic Shares Outstanding	63.5	146.0	168.7	170.0	170.0	163.7	190.0	200.0

Source: Zacks Investment Research, Inc. *David Bautz, PhD*

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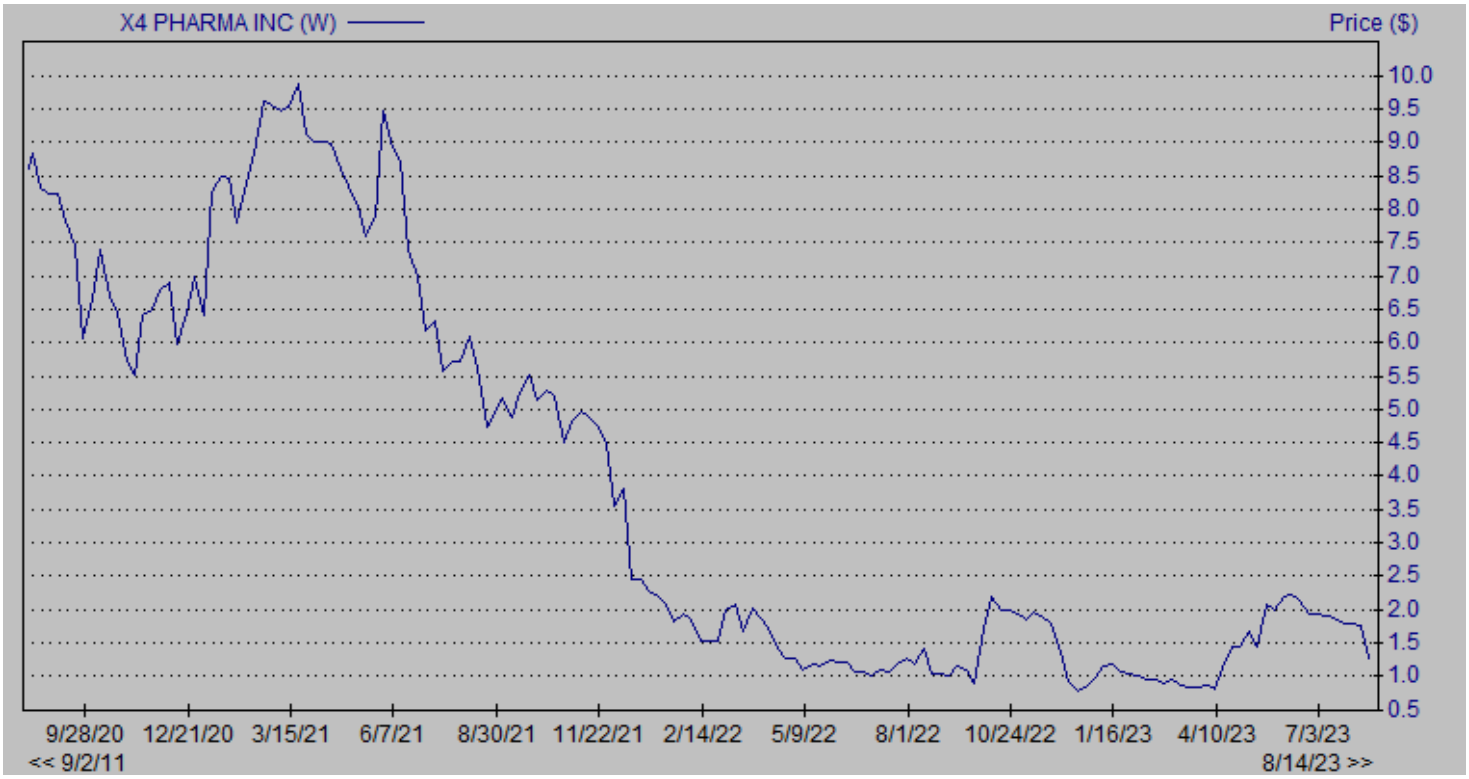
HISTORICAL STOCK PRICE



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