

X4 Pharmaceuticals, Inc

(XFOR-NASDAQ)

**XFOR: Mavorixafor NDA Accepted by FDA;
PDUFA Date of April 30, 2024...**

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 (11/13/23) \$0.75
Valuation \$4.50

OUTLOOK

On November 9, 2023, X4 Pharmaceuticals, Inc. (XFOR) announced financial results for the third quarter of 2023 and provided a business update. The company recently announced that the New Drug Application (NDA) for mavorixafor in WHIM syndrome was accepted for Priority Review with a PDUFA action date of April 30, 2024. The company is also eligible for a Priority Review Voucher (PRV) if mavorixafor is approved. PRVs have sold for the past couple of years for approximately \$100 million each. The company will provide an update on the ongoing Phase 2 clinical trial of mavorixafor in chronic neutropenia (CN) at ASH in December 2023 and a pivotal Phase 3 trial in CN will be initiated in the first half of 2024.

SUMMARY DATA

52-Week High \$2.49
52-Week Low \$0.69
One-Year Return (%) -62.30
Beta 0.76
Average Daily Volume (sh) 2,117,184

Shares Outstanding (mil) 167
Market Capitalization (\$mil) \$126
Short Interest Ratio (days) N/A
Institutional Ownership (%) 79
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 -1.2
P/E using 2024 Estimate -1.4

Risk Level High
Type of Stock Small-Blend
Industry N/A

ZACKS ESTIMATES

Revenue (in millions of \$)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2022	0.0 A				
2023	0.0 A	0.0 A	0.0 A	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	-\$0.01 A	-\$0.16 E	-\$0.65 E
2024					-\$0.45 E
2025					-\$0.27 E

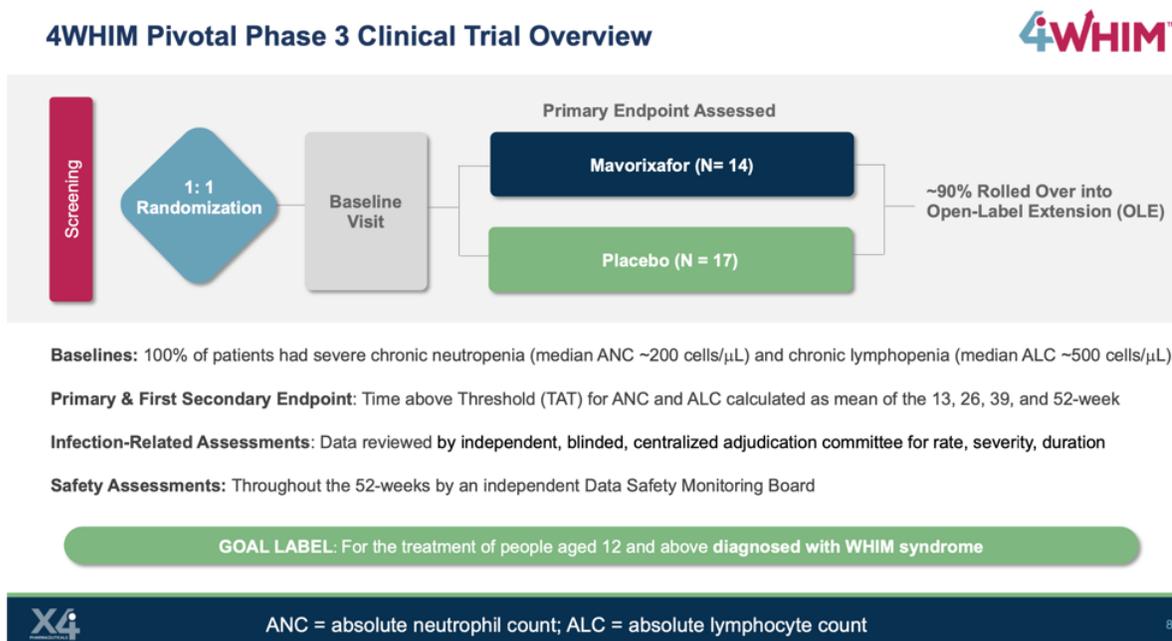
WHAT'S NEW

Business Update

NDA Accepted for Priority Review; PDUFA Action Date of April 30, 2024

On October 31, 2023, X4 Pharmaceuticals, Inc. (XFOR) announced that the U.S. Food and Drug Administration (FDA) has accepted for filing the company's New Drug Application (NDA) for mavorixafor for the treatment of patients 12 and older with WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency. The FDA granted Priority Review to the NDA and assigned a PDUFA action date of April 30, 2024. Due to mavorixafor's Rare Pediatric Disease designation for WHIM syndrome, X4 is eligible for a Priority Review Voucher (PRV) if mavorixafor is approved. PRV's are fully transferable and a number of them have sold in the past few years for approximately \$100 million each.

The NDA filing is supported in part by the results from the Phase 3 4WHIM trial of mavorixafor in patients with WHIM syndrome. An overview of that trial is provided below. It was a pivotal, global, randomized, double blind, placebo controlled, multicenter Phase 3 study designed to evaluate the efficacy and safety of mavorixafor in patients with genetically confirmed WHIM syndrome ([NCT03995108](https://clinicaltrials.gov/ct2/show/study/NCT03995108)).

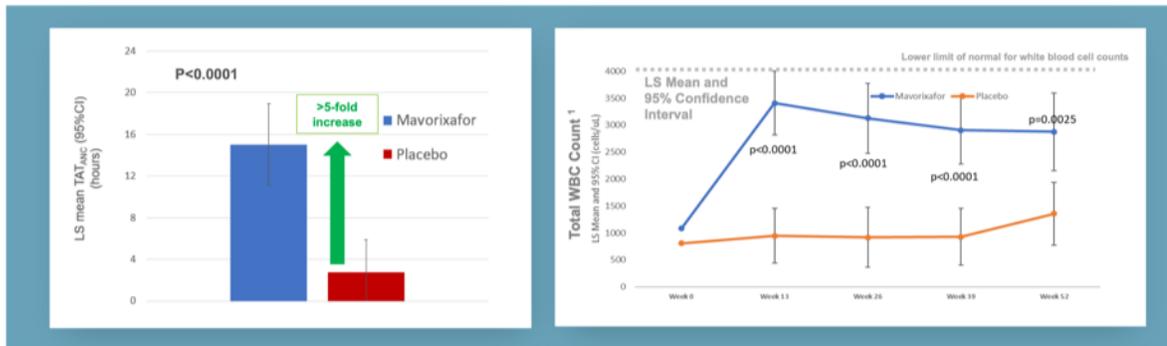


Source: X4 Pharmaceuticals, Inc.

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' 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, as shown in the following figure on the left. Mean TAT_{ANC} was 15.04 hours for mavorixafor compared to 2.75 hours for placebo, which represented a 5.5-fold improvement for mavorixafor-treated patients compared to placebo-treated. The following figure on the right shows that treatment with mavorixafor resulted in statistically significant increases in all white blood cells (WBCs) compared to placebo over 52 weeks.

4WHIM Primary Endpoint Met; Total WBC Counts Increased Over 52 Weeks

Intent-to-Treat (ITT) population analysis



Mavorixafor significantly improved the time above threshold of ANC over 52 weeks vs. placebo

Mean TAT_{ANC} was 15.04 hours for mavorixafor vs. 2.75 hours for placebo

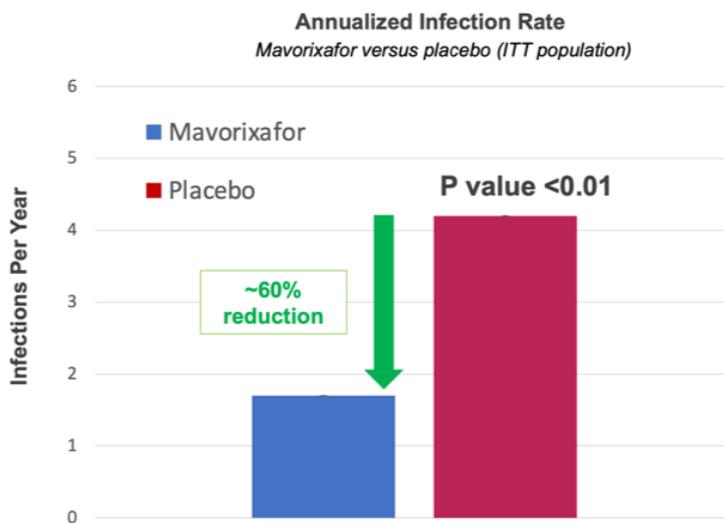
Statistically significant, durable increases in all WBC subtypes²

X4 1. At week 52, 3 of 17 placebo patients were given mavorixafor in advance of their TAT measurements as they entered the open-label portion of the study. All data are included in ITT analysis. 2. Data on file. 10

Source: X4 Pharmaceuticals, Inc.

Treatment with mavorixafor was shown to result in an approximately 60% reduction ($P < 0.01$) in the annualized infection rate, with deeper reduction in infection rate seen based on how long the patient was on mavorixafor treatment. The following figure shows an annual infection rate of less than two for patients treated with mavorixafor compared to a little greater than four for placebo-treated patients. The infection rate decreased to < 1.0 for mavorixafor compared to 4.5 for placebo ($P < 0.005$) during months 6-12.

Statistically Significant ~60% Reduction in Annualized Infection Rate



- **Deeper Reductions in Infection Rate with Time on Mavorixafor Treatment**
 - Infection rates decreased to < 1.0 on mavorixafor vs. 4.5 for placebo ($p < 0.005$) during months 6-12
- **Infection Rate Reductions With Mavorixafor Treatment Seen Across All Subgroups**

Source: X4 Pharmaceuticals, Inc.

Importantly, mavorixafor was well tolerated during the trial with no treatment-related serious adverse events, no discontinuations due to safety events, and no treatment-limiting toxicities. The safety and tolerability of mavorixafor is further supported by the fact that approximately 90% of the patients in the 4WHIM trial continued on to the open label extension study.

X4 is now turning its attention to a potential commercial launch in the first half of 2024 for WHIM syndrome. The company is focused on three areas: 1) Building support and awareness in the WHIM syndrome community – which will include education on WHIM syndrome, highlighting the unmet need, and supporting earlier diagnoses. The company recently launched the ‘What If It’s WHIM’ campaign to advance patient and physician education on the importance and benefits of early diagnosis; 2) Ensuring broad patient access – which will include engagement with payers to communicate the value proposition for mavorixafor to support rapid reimbursement along with implementing a distribution and supply chain for the drug; 3) Evolving X4 to a fully integrated biotech company – which will include building a rare disease commercial organization, establishing infrastructure, and coordinating cross-functional launch readiness.

Update on Chronic Neutropenia Program

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

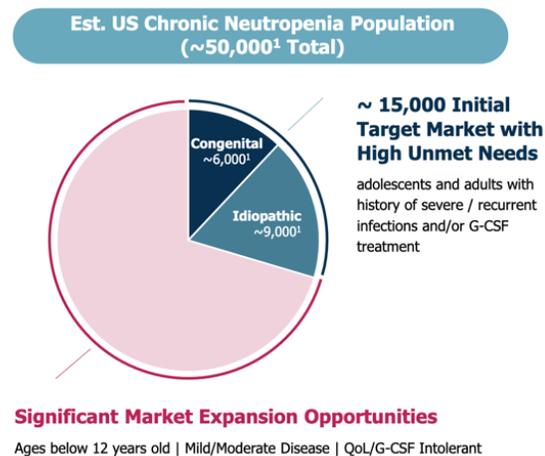
Significant Opportunity For Expansion

Methodology¹

- 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 age², CN disorders unlikely to respond to CXCR4 antagonism²
- **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



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 Results¹: Phase 2 Trial of Mavorixafor in Chronic Neutropenia
 Emerging Data Show Durability & Successful G-CSF Reduction

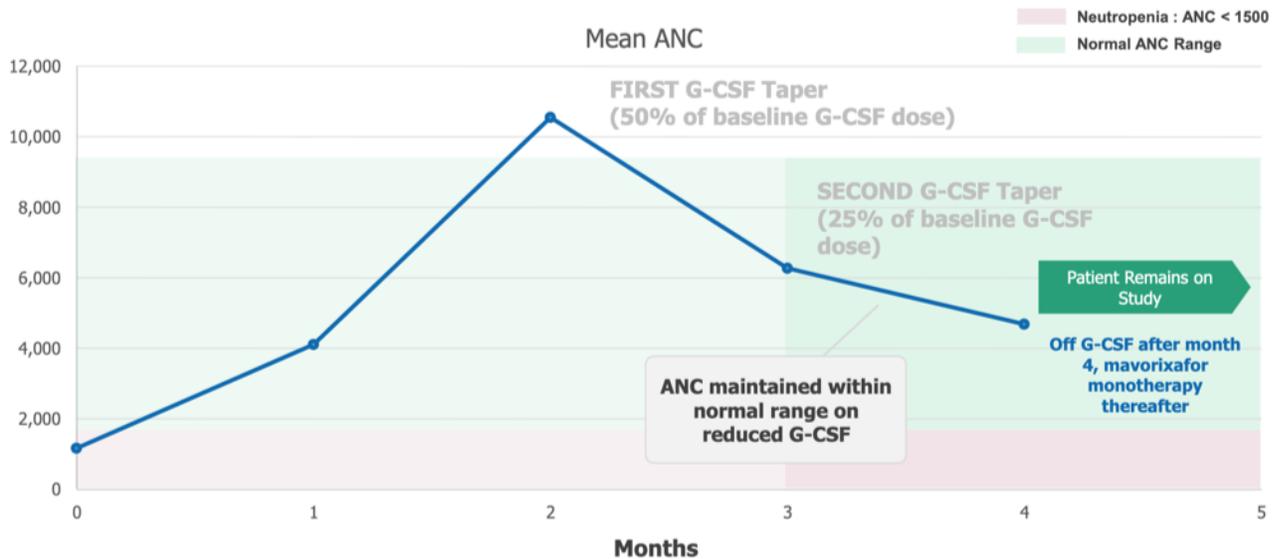
Participants		Baseline: Pre-Mav Dosing		After Mavorixafor Dosing		
	Diagnosis	G-CSF Use?	ANC Levels	Months Treated	ANC Levels	G-CSF Reduction
P1	CIN	Yes, chronic	Neutropenic with G-CSF	4	Increased response in ANC to normal ranges vs. BL	50% reduction at Month 2; off G-CSF after Month 4
P2	CIN	Yes, chronic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

Example (P1): Daily Mavorixafor Leads to Successful Reduction of G-CSF



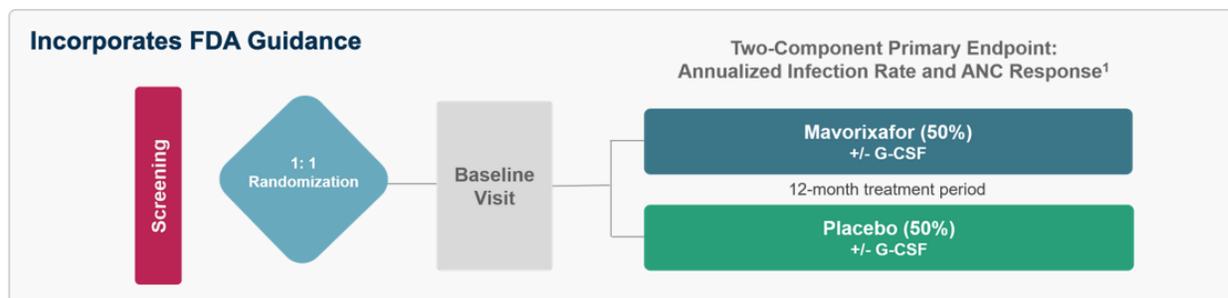
Data cut = July 14, 2023

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. The company will be presenting additional longer-term treatment data for those initial three patients at the upcoming American Society of Hematology (ASH) meeting in December 2023. In addition, efficacy and safety data will be presented from at least 15 of the currently enrolled participants in the Phase 2 study during the first half of 2024.

The company has now completed the study design for a pivotal Phase 3 trial in CN, which we anticipate initiating in the first half of 2024. The following figure gives an overview of the study design, which will include approximately 150 subjects, a 12-month treatment period, and a two-component primary endpoint: annualized infection rate and ANC response.

CN Pivotal, Global Phase 3 Trial: Delivering A Potential New Treatment Option



Key Inclusion Criteria:

- **Diagnosis:** congenital, autoimmune, or idiopathic neutropenia
- **Absolute Neutrophil Count (ANC):** <1500 cells/ μ l
- **Infection history:** 2 infections requiring intervention within last 12 months

Design: double-blinded, randomized, placebo-controlled on top of standard of care (+/- G-CSF²); same mavorixafor dosing as 4WHIM trial

Secondary Endpoints Include: severity and duration of infection, antibiotic use, fatigue, QoL, and safety

Endpoint and Power: 150 subjects, \geq 90% on primary endpoints of annualized infection rate and ANC response



1. ANC response: two of three ANC measurements in the first 3 months reach >1500 cells/ μ L (for participants with 500<ANC<1500 at BL) or 100% increase over baseline (for those ANC <500 at BL)
2. For those treated with G-CSF at baseline, G-CSF dose and frequency are required to remain constant throughout the trial unless adjustment is needed for safety reasons.

Source: X4 Pharmaceuticals, Inc.

Financial Update

On November 9, 2023, X4 announced financial results for the third quarter of 2023. As expected, the company did not report any revenues in the third quarter of 2023. R&D expenses for the third quarter of 2023 were \$19.1 million, compared to \$14.1 million for the third quarter of 2022. The increase in expenses was primarily due to higher regulatory costs associated with the preparation and submission of the NDA, higher contract manufacturing costs, and higher third-party costs associated with the Phase 3 4WHIM trial. SG&A expenses for the third quarter of 2023 were \$8.1 million, compared to \$6.0 million in the third quarter of 2022. The increase was primarily due to an increase in head count and third-party costs associated with the build out of commercial operations. Other income increased significantly compared to the same period in the prior year due to a decrease in the fair value of the company's Class C warrants, which were issued in the fourth quarter of 2022 and are accounted for as a liability at fair value.

As of September 30, 2023, X4 had cash, cash equivalents, marketable securities, and restricted cash of approximately \$141.7 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.

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 that the company should receive if mavorixafor is approved for the treatment of WHIM syndrome. As of November 7, 2023, X4 had

approximately 167.3 million shares outstanding and, when factoring in stock options and warrants, a fully diluted share count of 291.1 million.

Conclusion

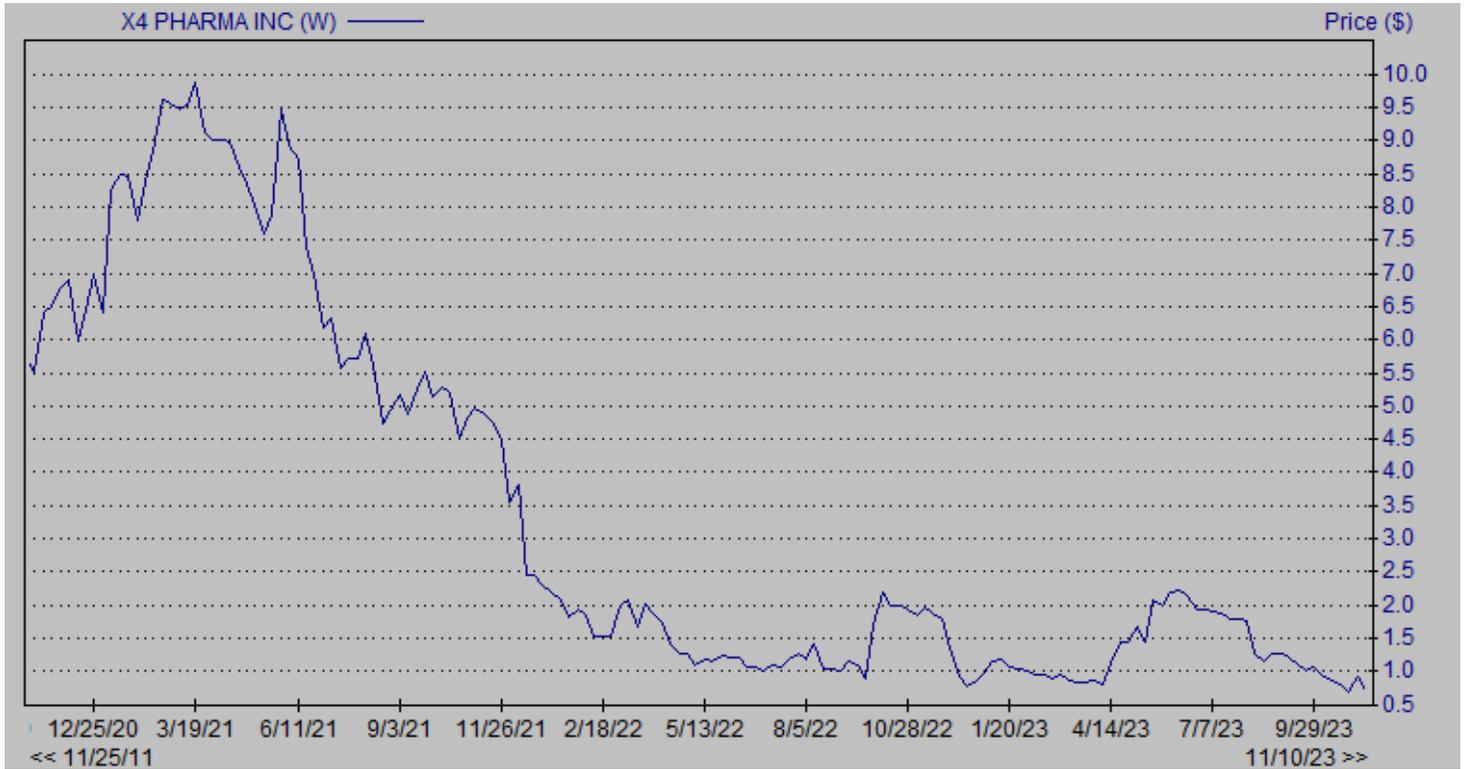
The acceptance of the NDA is a major milestone for the company and we look forward to the FDA's decision on the approval of mavorixafor for WHIM syndrome on or before April 30, 2024. PRV's continue to be sold for approximately \$100 million each, and we see no reason why the company would not be able to monetize a PRV that would be issued should mavorixafor be approved. It is good to hear that the company is fully focused on getting commercial operations up and running in anticipation of launching the drug in the first half of 2024, if approved. Upcoming milestones in CN include updated data from the first three participants in the Phase 2 trial at ASH in December 2023, an update on at least 15 participants in the Phase 2 trial in the first half of 2024, and initiation of the Phase 3 clinical trial 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 A	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
<i>Gross Margin</i>	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	90%	90%
Research & development	\$61.1	\$22.1	\$15.6	\$19.1	\$24.0	\$80.7	\$64.0	\$66.0
General & administrative	\$27.0	\$7.2	\$10.2	\$8.1	\$7.4	\$33.0	\$50.0	\$60.0
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)	(\$27.2)	(\$31.4)	(\$113.7)	(\$101.0)	(\$75.0)
<i>Operating Margin</i>	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	-776.9%	-147.1%
Non-Operating Expenses (Net)	(\$6.3)	\$5.3	(\$29.9)	\$24.9	(\$1.5)	(\$1.2)	(\$6.0)	(\$6.0)
Pre-Tax Income	(\$93.8)	(\$24.0)	(\$55.7)	(\$2.3)	(\$32.9)	(\$114.9)	(\$107.0)	(\$81.0)
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
<i>Tax Rate</i>	0%	0%	0%	0%	0%	0%	0%	0%
Net Income	(\$96.4)	(\$24.0)	(\$55.7)	(\$2.3)	(\$32.9)	(\$115.0)	(\$107.0)	(\$81.0)
<i>Net Margin</i>	-	-	-	-	-	-	-	-
Reported EPS	(\$1.52)	(\$0.16)	(\$0.33)	(\$0.01)	(\$0.16)	(\$0.65)	(\$0.45)	(\$0.27)
<i>YOY Growth</i>	-	-	-	-	-	-	-	-
Basic and Diluted Shares Outstanding	63.5	146.0	168.7	197.0	200.0	177.9	240.0	300.0

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

HISTORICAL STOCK PRICE



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