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): **September 27, 2022**

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 7.01 Regulation FD Disclosure.

On September 27, 2022, X4 Pharmaceuticals, Inc. (the "Company") issued a press release announcing data from its Phase 1b clinical trial evaluating its lead clinical candidate, mavorixafor, in people with idiopathic, cyclic or congenital chronic neutropenia ("CN"). The Company will host a conference call and webcast to discuss the results at 8:00 AM, Eastern Time, on September 27, 2022. A live audio webcast of the presentation will be available under "Events and Presentations" in the "Investors" section of the Company's website at www.x4pharma.com. The webcast will be archived on the Company's website for at least 30 days. The information contained in, or that can be accessed through, the Company's website is not a part of this filing.

A copy of the press release is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference. The information furnished under this Item 7.01, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or subject to the liabilities of that section. The information shall not be deemed incorporated by reference into any other filing with the Securities and Exchange Commission made by the Company, regardless of any general incorporation language in such filing.

Item 8.01 Other Events.

On September 27, 2022, the Company announced data from its Phase 1b clinical trial evaluating the ability of its lead clinical candidate, mavorixafor, to increase the absolute neutrophil count ("ANC") in people with idiopathic, cyclic, or congenital CN as monotherapy or concurrently with injectable granulocyte colony-stimulating factor ("G-CSF").

The Phase 1b clinical trial's three objectives included: (i) monotherapy with mavorixafor in neutropenic patients; (ii) combination therapy with mavorixafor and G-CSF in neutropenic patients; and (iii) combination therapy with mavorixafor and G-CSF in patients with ANCs above 1,500. The analyses included 25 patients from Phase 1b of the trial who were treated with 400 mg mavorixafor. Of the 25 patients in the trial, all 25 patients (100%) responded to treatment with a single dose of 400 mg of mavorixafor, alone or dosed concurrently with G-CSF. A mean ANC increase at peak of >3,000 cells/microliter was demonstrated. Consistent responses were seen across all of the CN disorders studied, including idiopathic, cyclic and congenital neutropenias.

All neutropenic participants (n=14, including patients with moderate to severe neutropenia at screening) reached normalized ANC levels (>1,500 cells/microliter). When assessed as a monotherapy in participants with severe chronic neutropenia who were not being treated with G-CSF (n=6), a single dose of mavorixafor led to normalized ANC levels in all participants within two hours, with a mean ANC increase at peak of approximately 2,500 cells/microliter. When assessed in participants with moderate or severe neutropenia despite being treated with G-CSF (n=8), 100% reached normalized ANC levels. The safety assessment of mavorixafor in the CN population was consistent with the prior clinical studies of chronic dosing of 400 mg mavorixafor once per day as a monotherapy in Warts, Hypogammaglobulinemia, Infections, and Myelokathexis (WHIM) syndrome.

Additionally, as noted above under Item 7.01, members of the management team of the Company will be holding a conference call and live webcast to discuss the data from the clinical trial. A copy of the slide presentation to be used by the Company during the conference call is filed as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 **Financial Statements and Exhibits.**

Exhibit No.	Description
99.1	Press Release, dated September 27, 2022
99.2	Conference Call Presentation, dated September 27, 2022
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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: September 27, 2022

By: /s/ Adam Mostafa
Adam Mostafa
Chief Financial Officer

Exhibit 99.1

X4 Pharmaceuticals Announces New Positive Phase 1b Data Supporting Mavorixafor's Broad Potential in Chronic Neutropenia (CN)

100% of study participants (n=25) achieved robust responses to oral mavorixafor

100% of neutropenic participants (n=14) achieved normalized neutrophil counts

Robust responses achieved across all CN disorders studied (idiopathic, cyclic, congenital); estimated diagnosed patient population ~ 50,000 in the U.S.

Results suggest mavorixafor could be the first oral treatment for CN disorders and has the potential to reduce or replace injectable G-CSF, the current standard of care

X4 to host investor webinar today, Tuesday, September 27, at 8:00 am ET, including live Q&A with clinical experts

BOSTON – Sept. 27, 2022 - X4 Pharmaceuticals, Inc. (Nasdaq: XFOR), a leader in the discovery and development of novel CXCR4-targeted small-molecule therapeutics to benefit people with diseases of the immune system, today announced new positive data from its Phase 1b clinical trial evaluating the ability of its lead clinical candidate, mavorixafor, to increase the absolute neutrophil count (ANC) in people with idiopathic, cyclic, or congenital chronic neutropenia (CN) as monotherapy or concurrently with injectable granulocyte colony-stimulating factor (G-CSF).

"These compelling, positive results demonstrate, for the first time, the broad opportunity for mavorixafor in people with CN disorders beyond our most advanced investigational indication of WHIM syndrome," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Importantly, we believe mavorixafor's demonstrated ability in this trial to increase and normalize ANC levels in the three primary types of chronic neutropenia suggests an expanded market opportunity that could include up to an estimated 50,000 additional diagnosed patients in the U.S. With current injectable therapies associated with chronic, debilitating side effects, we also believe that mavorixafor carries the potential to address significant unmet patient needs if successfully developed as the first oral therapy for chronic neutropenic disorders."

Diego Cadavid, M.D., Chief Medical Officer of X4 Pharmaceuticals, added, "We are very pleased that this Phase 1b trial achieved all of its key objectives, and, most importantly, that all patients dosed with mavorixafor responded with meaningful increases in neutrophil counts. In addition, we are encouraged by the results of three exploratory sub-analyses we completed assessing the potential of mavorixafor to treat chronic neutropenia as monotherapy or concurrently with G-CSF. We believe these data support the further study of mavorixafor's potential to enable patients to reduce or even discontinue G-CSF treatment. In fact, the Phase 1b trial is currently being amended and expanded to assess the long-term

durability, safety, and tolerability of mavorixafor in a larger CN patient population. We anticipate this amended trial to begin generating additional clinical data in the first half of 2023."

Key highlights from the Phase 1b trial are as follows:

- 100% of study participants (n=25) responded to treatment with a single dose of 400 mg of mavorixafor, alone or dosed concurrently with G-CSF:
    - Participants achieved a mean ANC increase at peak of >3,000 cells/microliter.
    - Consistent responses were seen across all of the CN disorders studied – idiopathic, cyclic, and congenital neutropenias.
- All neutropenic participants (n=14) reached normalized ANC levels (>1,500 cells/microliter)
    - When assessed as a monotherapy **in participants with severe chronic neutropenia who were not being treated with G-CSF** (n=6), a single dose of mavorixafor led to normalized ANC levels in all participants within 2 hours, with a mean ANC increase at peak of ~2,500 cells/microliter.
    - When assessed **in participants with moderate or severe neutropenia despite being treated with G-CSF** (n=8), 100% reached normalized ANC levels, suggesting the potential of mavorixafor to both normalize the neutrophil counts in patients with partial response to G-CSF and also to potentially enable the reduction or elimination of G-CSF dosing.
- When assessed **in participants with CN with normalized ANC counts on chronic G-CSF** (n=11), all participants experienced a consistent and sustained increase in ANC, suggesting mavorixafor's potential to reduce or possibly eliminate G-CSF treatment in these patients.
- Mavorixafor was well tolerated in the study; all treatment-related adverse events were deemed to be low grade, consistent with previous clinical studies in WHIM syndrome, and no treatment-related serious adverse events were reported.

Investor Call Details:

X4 will host an investor webinar to present and discuss the new data **today from 8:00 - 9:15 am ET**. The event will include perspectives from patients and clinical experts on the unmet medical need. A live Q&A will follow the formal presentation with X4's management team and several expert clinicians available to answer questions. **To register for the event, click here.** Following the conclusion of the live webcast, a replay of the event will be available within the investors' section of the X4 Pharmaceuticals website at www.x4pharma.com.

About the Phase 1b Clinical Trial

The clinical trial (NCT04154488) is a proof-of-concept Phase 1b open-label, multicenter study designed to assess the safety and tolerability of oral mavorixafor, with or without G-CSF, in participants with chronic neutropenic disorders, including severe idiopathic, cyclic, and congenital neutropenia. Participants were dosed with a single dose of 400 mg oral mavorixafor to assess the magnitude of treatment response. An amendment to the Phase 1b clinical trial is currently being initiated and aims to evaluate the use of daily oral mavorixafor with or without G-CSF for 6 months in up to 50 participants with chronic neutropenic disorders. The study extension is also expected to assess the durability of ANC responses, the potential of mavorixafor to enable patients to taper down dosing with G-CSF, and to evaluate the tolerability of mavorixafor in combination with G-CSF in chronic use.

About Chronic Neutropenia

Chronic neutropenic disorders are rare blood conditions lasting more than three months, persistently or intermittently, and characterized by increased risk of infections and reduced quality of life due to persistent, abnormally low levels of neutrophils circulating in the blood. Chronic neutropenia can be

described by a number of etiologies, including idiopathic (of unknown origin), cyclic (typically a 21-day cycle), or congenital (of genetic causation). Patients are categorized as severely neutropenic when their ANC drops below 500 cells/microliter, moderately neutropenic when their ANC is between 500 and 999 cells/microliter, and mildly neutropenic when their ANC is between 1,000 and 1,499 cells/microliter. The lower limit of normal ANC is considered 1,500 cells/microliter. Neutrophils are retained in the bone marrow by the CXCL12/CXCR4 axis, creating a reserve of cells; downregulation of the CXCR4 receptor by G-CSF or inhibition of the receptor by a CXCR4 antagonist has been shown to mobilize neutrophils from the bone marrow into peripheral blood.

About X4 Pharmaceuticals

X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company leading the discovery and development of novel therapies for people with diseases of the immune system. Our lead clinical candidate is mavorixafor, a first-in-class, small molecule antagonist of chemokine receptor CXCR4 that is being developed as a once-daily oral therapy. Due to mavorixafor's ability to antagonize CXCR4 and improve the mobilization of white blood cells, we believe that mavorixafor has the potential to provide therapeutic benefit across a wide variety of immune system diseases, including a range of chronic neutropenic disorders and certain types of cancer. The efficacy and safety of mavorixafor are being evaluated in a global Phase 3 clinical trial in patients with WHIM syndrome, a rare, primary immunodeficiency disease typically caused by genetic mutations in the CXCR4 receptor gene. We are also studying mavorixafor in two Phase 1b clinical trials – one in patients with chronic neutropenic disorders including congenital, idiopathic, and cyclic neutropenia, and one concurrently with ibrutinib in patients with Waldenström's macroglobulinemia (WM), a rare B-cell lymphoma. Further clinical development of mavorixafor in WM will now be subject to completing a strategic partnership as we focus our resources on advancing mavorixafor for the benefit of patients with chronic neutropenic disorders. We continue to leverage our insights into CXCR4 biology at our corporate headquarters in Boston, Massachusetts and at our research facility in Vienna, Austria. For more information, please visit our website at 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 and therapeutic potential of mavorixafor. 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, on account of uncertainties inherent in the initiation and completion of clinical trials and clinical development; the risk that trials and studies may not have satisfactory outcomes; the risk that the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the risk that initial or interim results from a clinical trial may not be predictive of the final results of the trial or the results of future trials; the potential adverse effects arising from the testing or use of mavorixafor or other product candidates; the risks related to X4's ability to raise additional capital, and other risks and uncertainties, including those 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 August 4, 2022, and in other filings X4 makes with the SEC from time to time.

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Exhibit 99.2

Chronic Neutropenia

Mavorixafor Beyond WHIM Syndrome

September 27, 2022

Forward-Looking Statements

This presentation 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 and therapeutic potential of mavorixafor for the treatment of WHIM syndrome, chronic and other neutropenias, and of X4’s other product candidates; X4’s possible exploration of additional opportunities for mavorixafor; the expected duration of patent protection; the expected availability, content and timing of clinical data from X4’s ongoing clinical trials of mavorixafor; anticipated regulatory filings; clinical trial design; patient prevalence; market opportunities; and X4’s cash runway.

Any forward-looking statements in this presentation 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, uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; the risk that trials and studies may be delayed and may not have satisfactory outcomes; the risk that the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the risk that initial or interim results from a clinical trial may not be predictive of the final results of the trial or the results of future trials; the potential adverse effects arising from the testing or use of mavorixafor or other product candidates; the risk that patient prevalence, market or opportunity estimates may be inaccurate; risks related to X4’s ability to raise additional capital; and other risks and uncertainties, including those 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 August 4, 2022, and in other filings X4 makes with the SEC from time to time. X4 undertakes no obligation to update the information contained in this presentation to reflect new events or circumstances, except as required by law.

Certain information contained in this presentation relates to or is based on studies, publications, surveys and other data obtained from third-party sources and X4’s own internal estimates and research. While X4 believes these third-party sources to be reliable as of the date of this presentation, it has not independently verified, and makes no representation as to the adequacy, fairness, accuracy, or completeness of, any information obtained from third-party sources. Finally, while X4 believes its own internal research is reliable, such research has not been verified or validated by any independent source.

Event Outline

01 Welcome

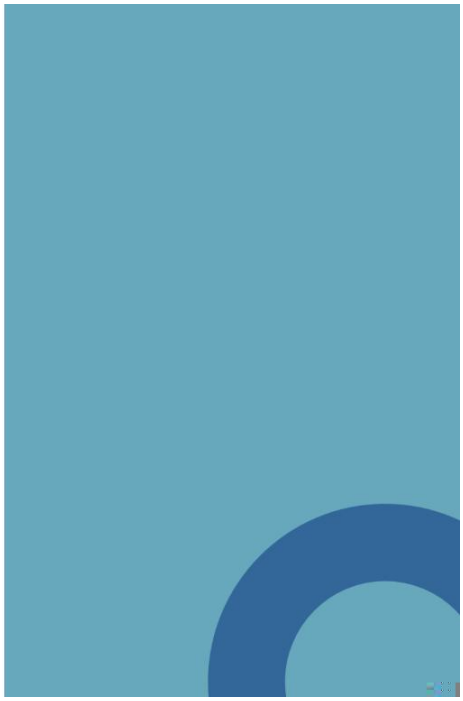
02 Overview of Chronic Neutropenia

03 Insights into Current Treatment Paradigm

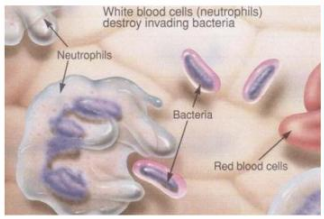
04 Phase 1b Clinical Trial Objectives and Results

05 Future Potential of Mavorixafor in CN Disorders

06 Conclusion and Q&A



Chronic Neutropenia (CN): a Severe Condition with Significant Unmet Needs



Neutrophils circulate in blood & are critical for fighting infections

Risk of Infections Increases with Lower Neutrophil Counts (Neutropenia)

Neutropenia Category	Blood Neutrophil Levels (ANC) – cells/uL
Lower Limit of Normal	>1,500
Mild	1,000 to 1,499
Moderate	500 to 999
Severe	Below 500

Image source: "Understanding Severe Chronic Neutropenia: A handbook for patients and their families," SCNIR 2017; Table data and information on infection risk: https://neutropenianet.org/what-is-neutropenia/

Disease Definition

Persistent, low levels of circulating neutrophils

- Severe, chronic (>3 months) neutropenia carries mortality risk due to infections
- Various types/etiologies of CN disorders: idiopathic, cyclic, congenital, autoimmune

Unmet Need

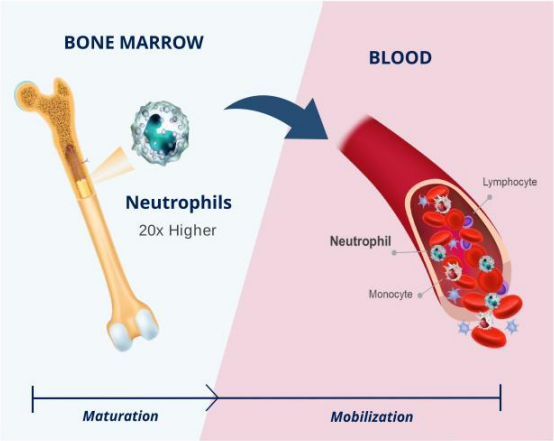
Only one Rx option: daily injectable recombinant granulocyte colony stimulating factor (G-CSF)

- Infection risks remain, although improved
- High likelihood of debilitating side effects
- Increased cancer risk in certain populations
- Burden of administration

SIGNIFICANT OPPORTUNITY FOR AN ORAL EFFICACIOUS THERAPY WITH GOOD TOLERABILITY

CXCR4 Plays A Key Role in Maturation & Mobilization of Neutrophils

Mature neutrophil pool in bone marrow is 20 times higher than in circulation[1]



Neutrophils are retained in the bone marrow (BM) by the CXCL12/CXCR4 **axis creating a "reserve"**

- More than 100 billion neutrophils exit the BM per day[2]
- Vast majority are held in reserve

Downregulation of CXCR4 leads to maturation of neutrophils and mobilization into the blood

- G-CSF down-regulates CXCR4 expression[3]
- CXCR4 antagonism inhibits signaling[4]

1. Bainton DF (1980) The cells of inflammation: a general view. In Weissmann G (ed) The Cell Biology of Inflammation, vol 2, pp 1–25. Amsterdam: Elsevier/North-Holland 2. Furze RC, et al, Immunology. 2008 3. Kim HK, et al, Blood. 2006. 4. Mosi, RM, et al, Biochem Pharmacol, 2012

Mavorixafor

Realizing the Potential of CXCR4 Antagonism in an Oral Capsule

Mavorixafor: the only oral CXCR4 antagonist in development

- A small molecule with high potency and selectivity
- Demonstrated ability to increase the mobilization of white blood cells, including neutrophils, from bone marrow to bloodstream
- Durable half-life supporting once-daily dosing
- Potential utility across broad range of chronic neutropenic disorders with high unmet needs

Safety Profile To Date Supports Chronic Use

- >200 patients/subjects treated to date
- Prior Phase 2 trial in WHIM syndrome:
  - Median treatment duration at 400 mg = 184 weeks
  - Low-grade adverse events reported (most commonly reported adverse events include: dyspepsia, nausea, dry mouth, conjunctivitis)

Patent Protection Expected Through 2038 and Beyond

Injectable G-CSF is the Only Approved Therapy for CN Disorders

Treatment Has Significant Challenges, Limitations, and Risks

Injections of G-CSF

- Approved for idiopathic, cyclic, and congenital severe CN
- Up to twice-daily to increase neutrophil counts and reduce severe neutropenia, infection risk
- Recommended starting dose 5-6 mcg/kg twice daily
- Often dosed by “down-titration” and at reduced frequencies
- Multiple visits to optimize dose & side effects

Challenges for patients on G-CSF

- Increased risk of myelodysplastic syndromes (MDS)[1]
- 70% with bone-pain impacting compliance and quality of life[2]

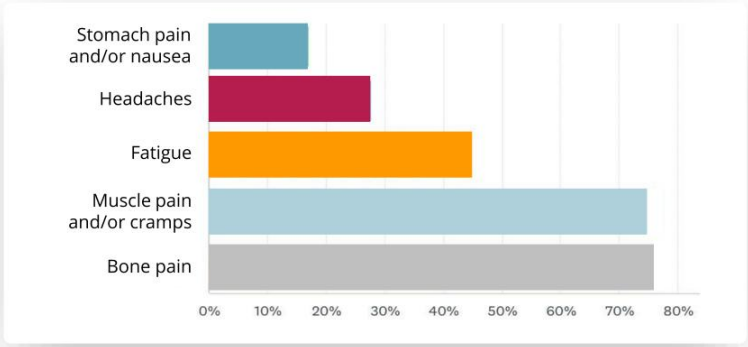
G-CSF For Injection
Indicated for Idiopathic, Cyclic and Congenital Severe Neutropenia

No alternative therapies except for bone marrow transplantation

1. Dale et al Support Cancer Ther 2006 Jul 1;3(4):220-31: 2. Michniacki et al, Blood (2019) 134 (Supplement_1): 3449; Neupogen label

100-Patient Survey[1] Results:

Significant Challenges with G-CSF



The Patient Experience

91 experienced with G-CSF

- 51 surveyed could tolerate continuing G-CSF treatment:
  - 76% (39) of them noted bone pain as a side effect
  - 75% (38) patients noted muscle pain and/or cramps as a side effect

1. August 2022 Savvy Cooperative Survey Results: Neutropenia Patient Perspective

Significant Potential Across CN Disorders Studied in Phase 1b Trial

~50,000 Estimated Chronic Neutropenia Patients in the U.S.[1]

Idiopathic	Cyclic	Congenital
~40,000	~5,000	~2,000
Most commonly diagnosed chronic neutropenia	Typically, a 21-day cycle	Rare hematological genetic diseases
Not attributable to drugs or specific infectious, inflammatory, autoimmune or malignant causes	Autosomal-dominant disorder	Can be caused by ELANE and other mutations
	Can be caused by ELANE mutations	Often resistant to G-CSF

1. U.S. Prevalence Based on ICD-10 Code Research, Average Across 3 Years (2018, 2019, & 2021); >90% greater than 18 years of age, ~2/3 female, mixed G-CSF use

Objectives of X4's Phase 1b Study in Chronic Neutropenic Disorders

PRIMARY STUDY OBJECTIVES

Assess mavorixafor treatment for safety and ability to increase absolute neutrophil count (ANC) across a range of chronic neutropenic disorders (idiopathic, cyclic, congenital)

Three Exploratory Sub-Analyses:

Mavorixafor Monotherapy in Those with Neutropenia

Assess if mavorixafor normalizes ANC levels in neutropenic patients as a monotherapy (in those off G-CSF)

Mavorixafor + G-CSF in Those with Neutropenia

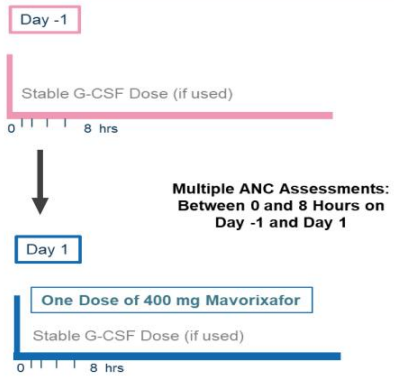
Assess if mavorixafor normalizes ANC levels in neutropenic patients in combination with G-CSF (in those on G-CSF)

Mavorixafor + G-CSF in Those with ANC >1,500 to Assess Potential for G-CSF Taper

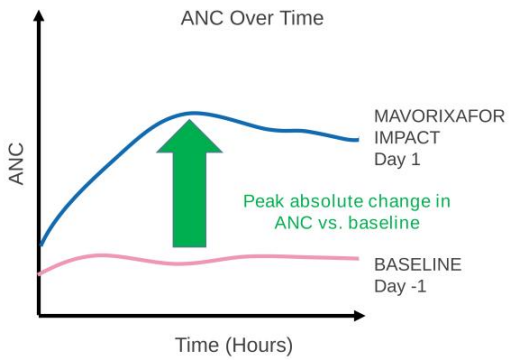
Assess if mavorixafor increases ANC levels in those treated with G-CSF and ANCs >1,500 at baseline to support future study of reducing or discontinuing G-CSF treatment

Measuring Maximum ANC Increase After Single Oral Dose of Mavorixafor

Trial Design



Trial Measurements



Baseline value defined as average ANC over Day-1

Phase 1b CN Trial Demographics & Safety Summary

	Overall	Idiopathic	Congenital	Cyclic
# Patients	25	16	6	3
Mean Age	36	37	32	41
Male/Female	10/15	6/10	1/5	3/0
On/Off G-CSF	18/7	10/6	5/1	3/0
Baseline G-CSF Dose for Those Treated Median (mcg/kg/day)*	1.09	0.56	1.09	1.09

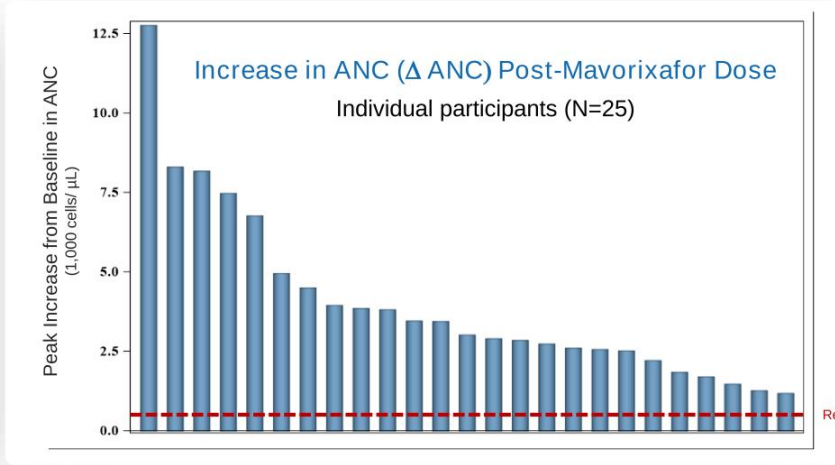
Phase 1b Trial in CN Disorders Safety Profile

- Single dose treatment mavorixafor 400 mg
- All low-grade treatment-emergent adverse events
- No treatment-related serious adverse events reported

**Among 7 patients not on G-CSF, one had baseline ANC value above 1,500 cells/uL*

Primary Objective: 100% of Patients Responded

Response defined as increase in ANC >500 cells/ µL[1]

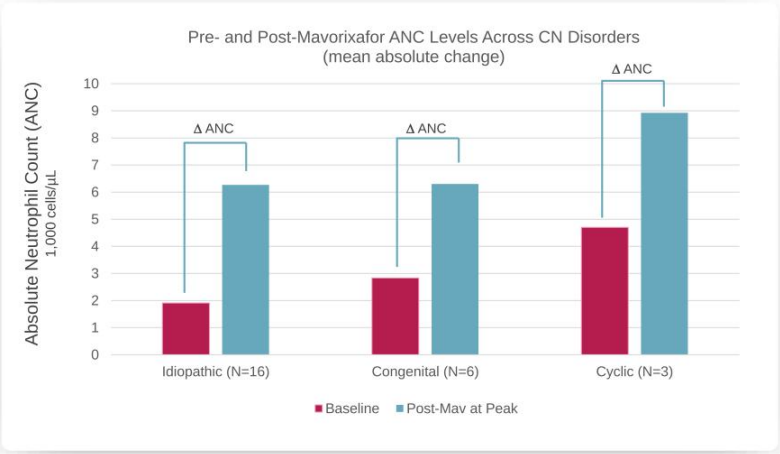


- All participants responded
  - Suggests bone marrow reserve of neutrophils can be accessed
- Responses exceeded 500 cells/µL for every individual participant

1. Increase of at least 500 cells/µL corresponds to improvement in at least one grade (e.g. severe neutropenia improves to moderate neutropenia).

Primary Objective Results: Oral Mavorixafor Increased ANC By >3,000 cells/µL

Consistent, large increases seen across all CN disorders studied (idiopathic, congenital, cyclic)



Idiopathic
Δ ANC of ~4,200 cells/µL

Congenital
Δ ANC of ~3,400 cells/µL

Cyclic
Δ ANC of ~4,200 cells/µL

Baseline value defined as average ANC over Day-1

Exploratory Sub-Analyses: Patient Numbers and Baseline Profiles

25 Participants: 3 Sub-Groups (no overlap between groups)

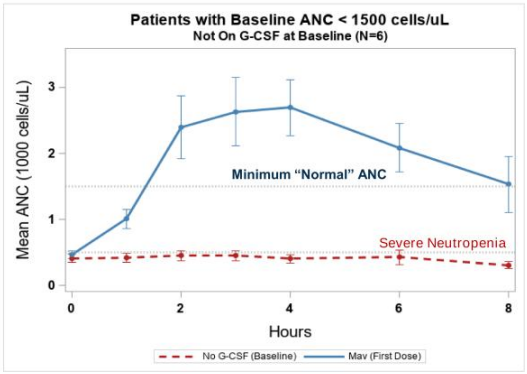
N = 6	N = 8	N = 11*
Assess impact of mavorixafor monotherapy in neutropenic patients	Assess impact of mavorixafor + G-CSF in neutropenic patients	Assess impact of mavorixafor + G-CSF in patients with ANC >1,500 to support G-CSF taper
Severe Neutropenia at Screening Mean ANC = 420 cells/µL No G-CSF	Moderate Neutropenia at Screening Mean ANC = 1,000 cells/µL G-CSF: 0.91 mcg/kg/day	Normal ANC at Screening Mean ANC = 4,300 cells/µL G-CSF: 1.09 mcg/kg/day

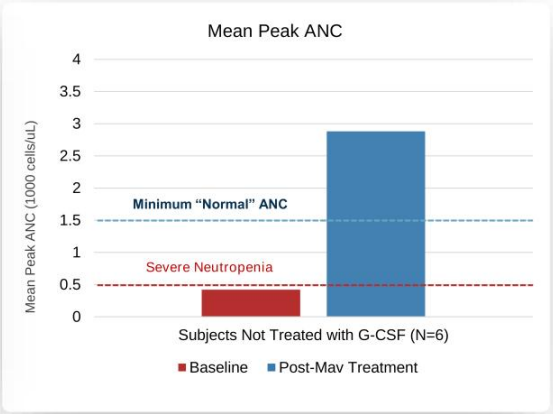
* Includes one participant with congenital neutropenia who was not on G-CSF but had baseline ANC > 1,500 cells/µL.

Sub-Analysis 1: Mavorixafor Monotherapy Normalized ANC in Neutropenic Patients

Kinetics and Mean Increase in ANC

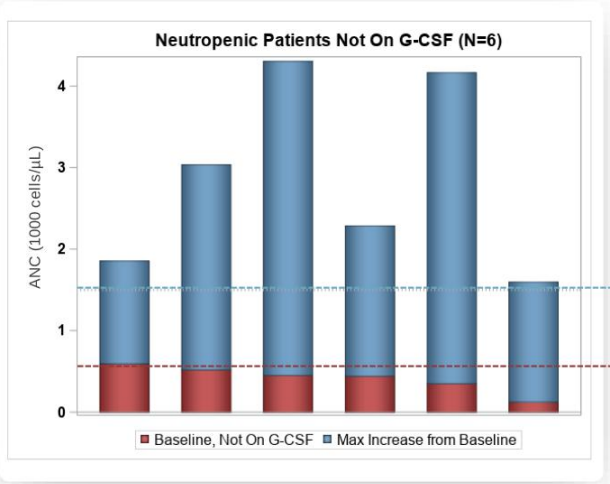
- Mavorixafor normalized ANC levels within 2 hours
- Mean ANC increase of ~2,500 cells/µL across all participants





Sub-Analysis 1: Mavorixafor Monotherapy Normalized ANC in Neutropenic Participants

Individual Participant Data



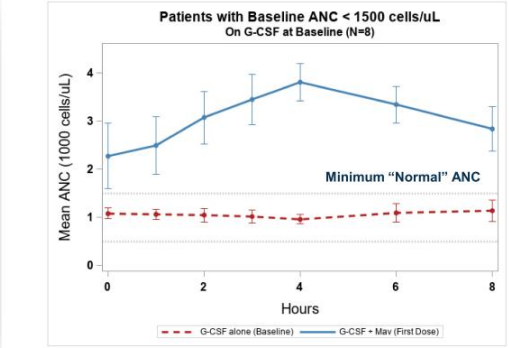
All patients had severe neutropenia pre-treatment

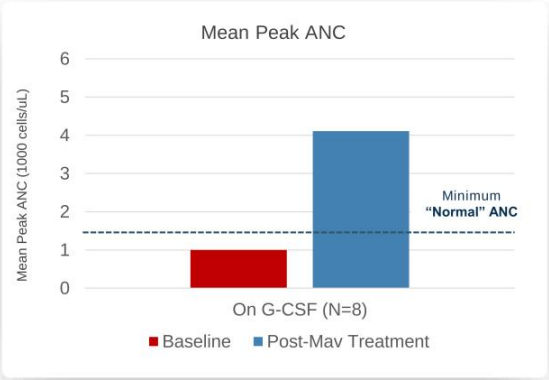
- All (100%) participants responded
- All (100%) achieved normalized ANC levels

Sub-Analysis 2: Mavorixafor + G-CSF Normalized ANC in Neutropenic Participants

Kinetics and Mean Increase in ANC

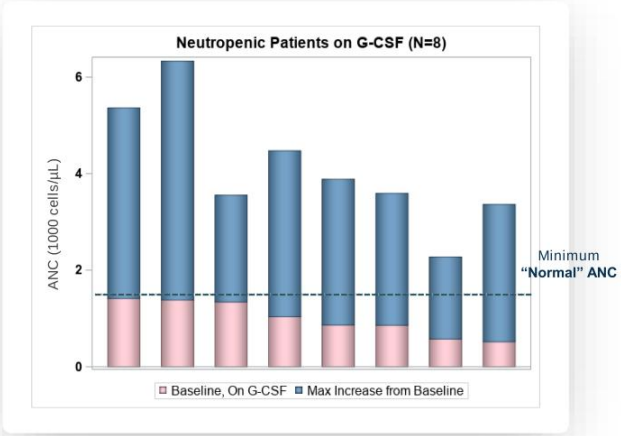
- ANC reached normal levels throughout dosing cycle
- Mean ANC increase of >3,000 cells/µL in neutropenic patients





Sub-Analysis 2: Mavorixafor + G-CSF Normalized ANC in Neutropenic Participants

Individual Participant Data



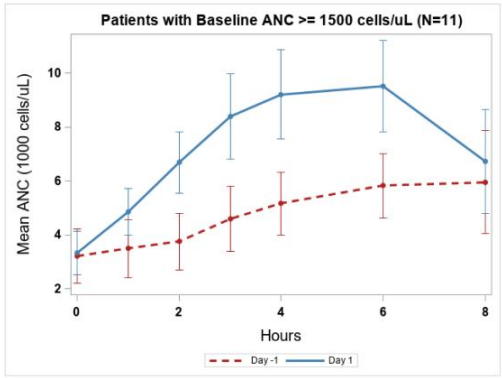
All are neutropenic; 5/8 (~60%) participants continued to have moderate or severe neutropenia although on G-CSF

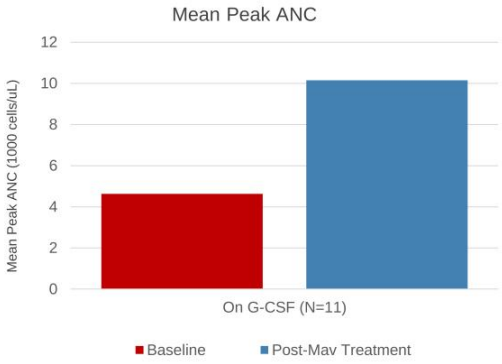
- All (100%) participants responded
- All (100%) achieved normalized ANC levels

Supports exploring potential of mavorixafor to reduce use of G-CSF

Sub-Analysis 3: Mavorixafor + G-CSF Increased ANC in Non-Neutropenic Participants

To Assess Potential for G-CSF Taper: Kinetics and Mean Increase in ANC





Includes one participant with congenital neutropenia who was not on G-CSF but had baseline ANC > 1,500 cells/µL.

Further supports exploring potential of mavorixafor to reduce use of G-CSF

Study Conclusions: Favorable Results Support Path Forward in CN

Primary Objectives Met All Participants Responded	• All CN Disorders – Idiopathic, Cyclic and Congenital – responded to mavorixafor • Increase in ANC of >2,000 cells/µL across all disorders
Potential for Mavorixafor Monotherapy	• Mavorixafor monotherapy increased ANC to normal levels in severe neutropenia • All (100%) individuals responded and achieved normalized ANC
Data Support Exploration of Mav as G-CSF Replacement	• Mav increased ANCs to normal levels in neutropenic patients on G-CSF • Mav increased ANCs robustly (2,000–5,000 cells/µL) in all patients treated with G-CSF
Well Tolerated Safety Profile Consistent with previous studies	• Low-grade treatment-related adverse events were observed • Safety data from prior studies support chronic dosing over years

Potential New, Oral Treatment for Chronic Neutropenic Disorders, including WHIM

Mavorixafor: potential to **become "standard of care"** across multiple CN disorders	Previous mavorixafor results suggest high likelihood of success	Significant unmet needs
ANC elevations and corresponding reduction in infection burden being assessed in Phase 3 4WHIM trial – Q4 readout Good tolerability and reduction in treatment burden vs. G-CSF	Oral, once daily treatment with well tolerated safety profile[1] Durable increase in ANC levels when treated for up to four years[2] Decreased frequency of infections in chronic studies in WHIM[2] and WM[3]	Larger target market than previously anticipated ~50,000 patients in the U.S. with chronic neutropenia, including idiopathic, cyclical, and congenital

1. Dale, Blood, 2019. 2. Dale, ASH, 2021. 3. X4 WM = Waldenstrom's Macroglobulinemia; Data released in X4 Q3 Earnings Presentation, August 2022.

Significant Near-Term Milestones / Meaningful Growth Potential

1. Phase 1b protocol amendment near completion: 6-month dosing in up to 50 patients to assess ANC durability, G-CSF taper, tolerability vs. G-CSF and other.

Sharp focus on chronic neutropenic disorders

Unparalleled expertise in immune system dysfunction and CXCR4 biology

Mavorixafor – a late-stage clinical CXCR4 antagonist candidate with broad commercial potential

Key mavorixafor clinical milestones expected over next 3 to 6 months

Strong balance sheet, with cash runway expected to fund operations into 3Q 2023