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 (Date of earliest event reported): **June 27, 2024**

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)

Not applicable
(Former name or former address, if changed since last report)

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 June 27, 2024, X4 Pharmaceuticals, Inc. (the "Company") issued a press release titled "X4 Pharmaceuticals Announces Positive Interim Clinical Data from Ongoing Six-Month Phase 2 Trial of Mavorixafor in Chronic Neutropenia (CN) and Initiation of Pivotal Phase 3 CN Trial". A copy of the press release is attached hereto as Exhibit 99.1.

On June 27, 2024, the Company posted a corporate presentation on the Company's website to provide updates and summaries of its business. A copy of the corporate presentation is attached as Exhibit 99.2 to this report.

The information in this Item 7.01, including Exhibits 99.1 and 99.2 to this report, shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section or Sections 11 and 12(a)(2) of the Securities Act of 1933, as amended (the "Securities Act"). The information contained in this Item 7.01 and in the accompanying Exhibit 99.1 and 99.2 shall not be incorporated by reference into any other filing under the Exchange Act or under the Securities Act, except as shall be expressly set forth by specific reference in such filing.

Item 8.01 Other Events.

On June 27, 2024, the Company announced positive new clinical data from its ongoing Phase 2 clinical trial evaluating the safety and efficacy of mavorixafor, an oral CXCR4 antagonist, in the treatment of people with chronic neutropenia (CN). An interim analysis of data from the ongoing six-month study showed that once-daily oral mavorixafor was generally well tolerated and durably increased participants' absolute neutrophil counts (ANC) both as a monotherapy and in combination with stable doses of injectable granulocyte colony-stimulating factor (G-CSF), the only therapy approved in the U.S. for severe chronic neutropenia.

The company also announced that it is currently screening patients for enrollment into its global, pivotal Phase 3 clinical trial, the 4WARD study, evaluating the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of G-CSF) in people with congenital, acquired primary autoimmune, or idiopathic CN who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll 150 participants.

Interim Analysis of Data from Phase 2 Clinical Study of Mavorixafor in CN

The Phase 2 study of mavorixafor is a six-month, open-label clinical trial that enrolled a total of 23 participants diagnosed with idiopathic, congenital, or cyclic CN. The interim analysis included results from the two treatment groups in the study (mavorixafor monotherapy and mavorixafor with stable-dose G-CSF) that most closely mirror the participant population of the newly initiated Phase 3 4WARD trial. Fifteen participants were enrolled across these two groups and, as of the May 14, 2024 interim analysis data cut-off date, seven had completed the study, and five remain ongoing. Data from a third treatment group of eight participants receiving mavorixafor and dose-adjusted G-CSF are expected to be presented later this year.

The mavorixafor monotherapy group included 10 participants and the mavorixafor with stable-dose G-CSF group included five participants. As of the data cut-off date, findings from the interim analysis show:

- 100% (6/6) of evaluable participants who had completed the six-month study achieved target ANC increase (rANC >500 cells/μL) at Months 3 and 6 on once-daily mavorixafor therapy with or without stable-dose G-CSF.
- Participants on mavorixafor monotherapy achieved mean ANC levels above the lower limit of normal for CN (≥1,500 cells/μL) at Month 3 (n=8) and Month 6 (n=3).

- Mavorixafor monotherapy also durably increased ANC in participants with severe CN (ANC<500 cells/μL at baseline), achieving mean ANC of ~800-1,000 cells/μL (ANC range targeted by experts) at Months 1, 3, and 6 (n=5, 3, and 2, respectively).
- Participants on mavorixafor in combination with stable-dose G-CSF experienced increases in mean ANC of >1,000 cells/μL at Months 1, 3, and 6 (n=4, 4, and 3, respectively) versus baseline.

Across the 23 participants enrolled in the study, mavorixafor was generally well tolerated as a monotherapy and in combination with G-CSF, with no drug-related serious adverse events reported, as of the interim analysis data cut-off date. Of the 23 participants, three discontinued due to non-serious adverse events. The overall safety profile remains consistent with previous clinical studies.

Forward-Looking Statements
This Form 8-K contains forward-looking statements within the meaning of applicable securities laws, including 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, implied or express statements regarding X4's expectations as to plans for commercial launch of XOLREMDI (mavorixafor), which is approved in the U.S. for use in patients 12 years of age and older with WHIM syndrome (the "Indication"); X4's belief in its readiness for commercial launch of XOLREMDI; the potential benefit of XOLREMDI in the Indication; the potential number of patients in the United States with WHIM syndrome and the potential market for XOLREMDI due to unmet potential patient needs; the initiation, timing, progress, and results of our current and future preclinical studies and clinical trials and related preparatory work and the period during which the results of the trials will become available, as well as our research and development programs; and the mission and goals for our business.

Any forward-looking statements in this Form 8-K are based on management's current expectations and beliefs. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond X4's control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including the risks that: X4's launch and commercialization efforts in the U.S. with respect to XOLREMDI may not be successful, and X4 may be unable to generate revenues at the levels or on the timing we expect or at levels or on the timing necessary to support our goals; the number of patients with WHIM syndrome, the unmet need for additional treatment options, and the potential market for XOLREMDI may be significantly smaller than we expect; XOLREMDI may not achieve the clinical benefit, clinical use or market acceptance we expect or we may encounter reimbursement-related or other market-related issues that impact the success of our commercialization efforts; we may encounter adverse events for XOLREMDI at any stage that negatively impact commercialization X4 may have difficulty establishing and maintaining an effective sales and marketing organization or suitable third-party alternatives for any approved products; X4 may not be able to obtain regulatory approval for, or successfully commercialize, mavorixafor or any other product candidate for other chronic neutropenic disorders or any other potential indication; the expected availability, content, and timing of clinical data from X4's ongoing clinical trials of mavorixafor may be delayed or unavailable, including its interim clinical results from its ongoing Phase 2 clinical trial; 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 design and rate of enrollment for clinical trials, including the current design of a potential Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders may not enable successful completion of the trial(s); the commercial opportunity for XOLREMDI in WHIM syndrome and other chronic neutropenic disorders may be smaller than we anticipate and X4's potential future revenue from XOLREMDI may be adversely affected; X4's use of capital and other financial results, including its financial runway; X4 may be unable to obtain and maintain regulatory approvals; uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; trials and studies may be delayed and may not have satisfactory outcomes; the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; 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, including assessing the ability of mavorixafor monotherapy to durably increase absolute neutrophil count in patients with chronic neutropenic; the potential adverse safety effects arising from the testing or use of our product and product candidates; general macroeconomic and geopolitical conditions which could impact X4's business; risks related to X4's ability to raise additional capital; risks related to the substantial doubt about X4's ability to continue as a going concern; there will be changes in expected or existing competition;

there will be changes in the regulatory environment; unexpected litigation or other disputes; the need to align with our collaborators may hamper or delay our development and commercialization efforts or increase our costs; our business may be adversely affected and our costs may increase if any of our key collaborators fails to perform its obligations or terminates our collaboration; the internal and external costs required for our ongoing and planned activities, and the resulting impact on expense and use of cash, may be higher than expected which may cause us to use cash more quickly than we expect or to change or curtail some of our plans or both; 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 May 7, 2024, 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.

Item 9.01 **Financial Statements and Exhibits.**

Exhibit No.	Description
99.1	Press Release, dated June 27, 2024
99.2	Corporate Presentation, dated June 27, 2024
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: June 27, 2024

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

Exhibit 99.1

X4 Pharmaceuticals Announces Positive Interim Clinical Data from Ongoing Six-Month Phase 2 Trial of Mavorixafor in Chronic Neutropenia (CN) and Initiation of Pivotal Phase 3 CN Trial

100% of evaluable participants at Month 6 achieved target absolute neutrophil count (ANC) increase with once-daily, oral mavorixafor +/- stable-dose G-CSF as of the interim analysis data cut-off date

Durable mean ANC levels above the lower limit of normal for CN were achieved for participants on mavorixafor monotherapy at Months 3 and 6

Company webinar today at 8:00 am ET will detail the interim clinical results and feature clinical experts in the treatment of CN

BOSTON, June 27, 2024 – X4 Pharmaceuticals (Nasdaq: XFOR), a company driven to improve the lives of people with rare diseases of the immune system, today announced positive new clinical data from its ongoing Phase 2 clinical trial evaluating the safety and efficacy of mavorixafor, an oral CXCR4 antagonist, in the treatment of people with chronic neutropenia (CN). An interim analysis of data from the ongoing six-month study showed that once-daily oral mavorixafor was generally well tolerated and durably increased participants' absolute neutrophil counts (ANC) both as a monotherapy and in combination with stable doses of injectable granulocyte colony-stimulating factor (G-CSF), the only therapy approved in the U.S. for severe chronic neutropenia.

Today the company also announced that it is currently screening patients for enrollment into its global, pivotal Phase 3 clinical trial, the 4WARD study, evaluating the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of G-CSF) in people with congenital, acquired primary autoimmune, or idiopathic CN who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll 150 participants.

"For the first time, we have demonstrated the ability of mavorixafor monotherapy to durably and meaningfully increase ANC in people living with chronic neutropenia," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "In addition, we were pleased to see that once-daily mavorixafor used in combination with G-CSF also led to meaningful and sustained ANC increases and was generally well tolerated, further supporting the design of our newly initiated Phase 3 4WARD clinical trial, which will study the use of mavorixafor alone and with G-CSF in people with CN dealing with recurrent and/or serious infections. Today's exciting interim results build on the momentum we established in April with mavorixafor's first approval in the U.S., and we look forward to quickly advancing the 4WARD trial as we pursue a second indication to deliver for more patients in need."

Interim Analysis of Data from Phase 2 Clinical Study of Mavorixafor in CN

The Phase 2 study of mavorixafor is a six-month, open-label clinical trial that enrolled a total of 23 participants diagnosed with idiopathic, congenital, or cyclic CN. The interim analysis included results from the two treatment groups in the study (mavorixafor monotherapy and mavorixafor with stable-dose G-CSF) that most closely mirror the participant population of the newly initiated Phase 3 4WARD

trial. Fifteen participants were enrolled across these two groups and, as of the May 14, 2024 interim analysis data cut-off date, seven had completed the study, and five remain ongoing. Data from a third treatment group of eight participants receiving mavorixafor and dose-adjusted G-CSF are expected to be presented later this year.

The mavorixafor monotherapy group included 10 participants and the mavorixafor with stable-dose G-CSF group included five participants. As of the data cut-off date, findings from the interim analysis show:

- 100% (6/6) of evaluable participants who had completed the six-month study achieved target ANC increase (ΓANC >500 cells/µL) at Months 3 and 6 on once-daily mavorixafor therapy with or without stable-dose G-CSF.
- Participants on mavorixafor monotherapy achieved mean ANC levels above the lower limit of normal for CN (≥1,500 cells/µL) at Month 3 (n=8) and Month 6 (n=3).
    - Mavorixafor monotherapy also durably increased ANC in participants with severe CN (ANC<500 cells/µL at baseline), achieving mean ANC of ~800-1,000 cells/µL (ANC range targeted by experts) at Months 1, 3, and 6 (n=5, 3, and 2, respectively).
- Participants on mavorixafor in combination with stable-dose G-CSF experienced increases in mean ANC of >1,000 cells/µL at Months 1, 3, and 6 (n=4, 4, and 3, respectively) versus baseline.

Across the 23 participants enrolled in the study, mavorixafor was generally well tolerated as a monotherapy and in combination with G-CSF, with no drug-related serious adverse events reported, as of the interim analysis data cut-off date. Of the 23 participants, three discontinued due to non-serious adverse events. The overall safety profile remains consistent with previous clinical studies.

Jean Donadieu, M.D., Ph.D., pediatrician in the hemato-oncology department of Trousseau Hospital in Paris, coordinator of the French registry for chronic neutropenia, and coordinator of the French chronic neutropenia reference center, commented on the results: “I am pleased to see that these interim data are consistent with the previous results of the Phase 1b study, but now with durability of effect and a good tolerability profile out to six months of treatment. This patient group has only one currently approved treatment option – one that is injectable and that has dose-related, dose-limiting, and challenging side effects and risks. The results from this interim analysis offer a sound and compelling rationale for mavorixafor’s evaluation in a Phase 3 trial, which is very good news for my patients with chronic neutropenia who, I believe, would benefit from an oral therapy to help reduce recurring or severe infections.”

Investor Webinar Details:
X4 will host an investor webinar to present and discuss the new data today at 8:00 am ET. To register for the event, click here. A live Q&A will follow the formal presentation. Following the conclusion of the live webcast, a replay of the event and the presented slides will be available within the investors’ section of the X4 Pharmaceuticals website at www.x4pharma.com.

About Chronic Neutropenia and Mavorixafor
Chronic neutropenia is a rare blood condition lasting more than three months, persistently or intermittently, and characterized by increased risk of infections and reduced quality of life due to abnormally low levels of neutrophils circulating in the blood. Neutrophils are retained in the bone marrow by the CXCR4/CXCL12 axis, creating a reserve of cells. Downregulation of the CXCR4 receptor by mavorixafor, an orally active CXCR4 antagonist, has been shown to mobilize neutrophils from the bone marrow into the peripheral blood across multiple disease states. The level of circulating neutrophils is typically measured by drawing blood to determine the absolute neutrophil count (ANC).

About the Phase 1b/Phase 2 Chronic Neutropenia Trial
The Phase 1b/Phase 2 clinical trial (NCT04154488) is a proof-of-concept, open-label, multicenter study designed to assess the safety and tolerability of oral mavorixafor, with or without injectable G-CSF, in participants with chronic neutropenic disorders, including idiopathic, cyclic, and congenital neutropenia. In the Phase 1b portion of the study, participants received one dose of oral mavorixafor and were assessed for magnitude of absolute neutrophil count (ANC) response and tolerability. In this initial portion of the study, 100% of participants (n=25) responded to treatment and mavorixafor was generally well tolerated alone or dosed concurrently with G-CSF. The ongoing Phase 2 portion of the trial (n=23 fully enrolled) is assessing the safety, tolerability, and the impact on participants' neutropenia of oral, once-daily mavorixafor with and without concurrent injectable G-CSF therapy over a six-month period.

About the 4WARD Global, Pivotal, Phase 3 Clinical Trial
The 4WARD trial is a global, pivotal Phase 3 clinical trial (NCT06056297) evaluating the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without G-CSF) in people with congenital, acquired primary autoimmune, or idiopathic chronic neutropenia who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll 150 participants with confirmed trough ANC levels less than 1,500 cells per microliter at baseline screening and histories of two or more serious and/or recurrent infections in the prior year. The primary endpoint of the trial is based on two outcome measures: annualized infection rate and positive ANC response.

About X4 Pharmaceuticals
X4 is delivering progress for patients by developing and commercializing innovative therapies for those with rare diseases of the immune system and significant unmet needs. Leveraging our expertise in CXCR4 and immune system biology, we have successfully developed mavorixafor, which has received U.S. approval as XOLREMDI™ (mavorixafor) capsules in its first indication. We are also evaluating the use of mavorixafor in additional potential indications. X4 corporate headquarters are in Boston, Massachusetts and our research center of excellence is 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 applicable securities laws, including 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, implied or express statements regarding the potential therapeutic benefit of mavorixafor; the initiation, timing, progress, and results of our current and future studies and clinical trials, including the Phase 2 clinical trial in chronic neutropenia and the Phase 3 4WARD clinical trial and related preparatory work and the period during which the results of the trials will become available; and the mission and goals for our business. Any forward-looking statements in this press release are based on management's current expectations and beliefs. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond X4's control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including the risks that: we may encounter adverse events for mavorixafor at any stage that negatively impact development and/or commercialization; the expected availability, content, and timing of clinical data from our ongoing clinical trials of mavorixafor may be delayed or unavailable, including clinical results from our ongoing Phase 2 clinical trial and the announced Phase 3 4WARD trial; the trials and studies may not have satisfactory outcomes; the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the design and rate of enrollment for clinical trials, including the current design of our Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders may

not enable successful completion of the trial(s); we may be unable to obtain and maintain regulatory approvals; uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; 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 safety effects arising from the testing or use of our product and product candidates may negatively impact development and/or commercialization; there will be changes in expected or existing competition; there will be changes in the regulatory environment; our business may be adversely affected and our costs may increase if any of our key collaborators fails to perform its obligations or terminates our collaboration; the internal and external costs required for our ongoing and planned activities, and the resulting impact on expense and use of cash, may be higher than expected which may cause us to use cash more quickly than we expect or to change or curtail some of our plans or both; and other risks and uncertainties, including those described in the section entitled "Risk Factors" in X4's Annual Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 7, 2024, 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.

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Exhibit 99.2

Investor Event
June 27, 2024

Mavorixafor in Chronic Neutropenia

Interim data from ongoing Phase 2 clinical trial

Forward-Looking Statements

This presentation including any printed or electronic copy of these slides, the talks given by the presenters, the information communicated during any delivery of the presentation and any question and answer session and any documents or materials distributed at or in connection with the presentation, contains forward-looking statements within the meaning of applicable securities laws, including 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, business, plans, or intentions. Forward-looking statements include, without limitation, implied or express statements regarding X4's expectations as to plans for commercial launch of XOLREMDI (mavorixafor), which is approved in the U.S. for use in patients 12 years of age and older with WHIM syndrome (the "Indication"); X4's belief in its readiness for commercial launch of XOLREMDI; the potential benefit of XOLREMDI in the Indication; the potential number of patients in the United States with WHIM syndrome and the potential market for XOLREMDI due to unmet potential patient needs; the initiation, timing, progress, and results of our current and future preclinical studies and clinical trials and related preparatory work and the period during which the results of the trials will become available, as well as our research and development programs; and the mission and goals for our business.

Any forward-looking statements in this presentation are based on management's current expectations and beliefs. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond X4's control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including the risks that: X4's launch and commercialization efforts in the U.S. with respect to XOLREMDI may not be successful, and X4 may be unable to generate revenues at the levels or on the timing we expect or at levels or on the timing necessary to support our goals; the number of patients with WHIM syndrome, the unmet need for additional treatment options, and the potential market for XOLREMDI may be significantly smaller than we expect; XOLREMDI may not achieve the clinical benefit, clinical use, or market acceptance we expect or we may encounter reimbursement-related or other market-related issues that impact the success of our commercialization efforts; we may encounter adverse events for XOLREMDI at any stage that negatively impact commercialization; X4 may have difficulty establishing and maintaining an effective sales and marketing organization or suitable third-party alternatives for any approved products; X4 may not be able to obtain regulatory approval for, or successfully commercialize, mavorixafor or any other product candidate for other chronic neutropenic disorders or any other potential indication; the expected availability, content, and timing of clinical data from X4's ongoing clinical trials of mavorixafor may be delayed or unavailable, including its interim clinical results from its ongoing Phase 2 clinical trial; 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 design and rate of enrollment for clinical trials, including the current design of a potential Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders may not enable successful completion of the trial(s); the commercial opportunity for XOLREMDI in WHIM syndrome and other chronic neutropenic disorders may be smaller than we anticipate and X4's potential future revenue from XOLREMDI may be adversely affected; X4's use of capital and other financial results, including its financial runway; X4 may be unable to obtain and maintain regulatory approvals; uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; trials and studies may be delayed and may not have satisfactory outcomes; the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; 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, including assessing the ability of mavorixafor monotherapy to durably increase absolute neutrophil count in patients with chronic neutropenic; the potential adverse safety effects arising from the testing or use of our product and product candidates; general macroeconomic and geopolitical conditions which could impact X4's business; risks related to X4's ability to raise additional capital; risks related to the substantial doubt about X4's ability to continue as a going concern; there will be changes in expected or existing competition; there will be changes in the regulatory environment; unexpected litigation or other disputes; the need to align with our collaborators may hamper or delay our development and commercialization efforts or increase our costs; our business may be adversely affected and our costs may increase if any of our key collaborators fails to perform its obligations or terminates our collaboration; the internal and external costs required for our ongoing and planned activities, and the resulting impact on expense and use of cash, may be higher than expected which may cause us to use cash more quickly than we expect or to change or curtail some of our plans or both; 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 May 7, 2024, 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. X4 is the owner of various trademarks, trade names and service marks. Certain other trademarks, trade names and service marks appearing in this presentation are the property of third parties. Solely for convenience, the trademarks and trade names in this presentation are referred to without the ® and TM symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

Today's Agenda

01 Welcome

02 Overview of Chronic Neutropenia (CN)

03 Mavorixafor's Validated Mechanism of Action

04 Interim Phase 2 CN Trial Results

05 Phase 3 CN Trial & Market Opportunity

06 Conclusions and Q&A

Guest Speakers

Jean Donadieu, MD, PhD
Pediatrician and epidemiologist, Hemato-Oncologic Department of Trousseau Hospital, Paris. Coordinator of both the French Chronic Neutropenia Registry and chronic neutropenia reference center.

Peter E. Newburger, MD
Physician-scientist, Professor and Vice Chair for Research, Department of Pediatrics/Division of Hematology-Oncology, UMass Chan Medical School. Editor-in-chief, *Pediatric Blood & Cancer*

X4's Growing Momentum Addressing Unmet Needs in Rare Immune Disorders

Strong foundation to deliver on the promise of mavorixafor in chronic neutropenia

PROVEN SUCCESS IN RARE DISEASE DRUG DEVELOPMENT & COMMERCIALIZATION

XOLREMDI™ (mavorixafor) approved by FDA in April 2024 - first therapy indicated for patients with WHIM syndrome[1]

- **First patients now on commercial product** with U.S. launch ongoing and field team fully deployed
- Clinical safety and efficacy data published online in ASH Journal *Blood*
- EU MAA submission expected late 2024/early 2025

STRONG BALANCE SHEET SUPPORTS CONTINUED GROWTH

Pro forma funds of $207 million[2]

Balance sheet expected to fund operations into late 2025[3]

NEXT VALUE DRIVER: MAVORIXAFOR IN CHRONIC NEUTROPENIA (CN)

1. WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis) See www.xolremdi.com for Prescribing Information; 2. Current funds include $82 million in cash and equivalents as of March 31, 2024 + $105 million in proceeds from PRV sale (May 2024) + $20 million from debt draw down from loan facility with Hercules Capital, Inc. (May 2024); 3. Projected runway excludes any potential U.S. sales of XOLREMDI.

Positive Interim Phase 2 Results Support Potential of Mavorixafor in CN

Summary of today's presentation

Overview of Key Results (as of May 14, 2024)

- Mavorixafor **durably increased absolute neutrophil counts (ANC)** across participants
- Mavorixafor **monotherapy durably increased ANC in severe CN** participants
- **Mavorixafor well tolerated** +/- stable-dose granulocyte colony-stimulating factor (G-CSF)

Pivotal Trial in CN initiated

Global, pivotal 4WARD Phase 3 clinical trial **now screening patients** across multiple international sites

Compelling Commercial Opportunity

Significant rare disease market opportunity in well defined patient population with high unmet needs and limited treatment options

Overview of Chronic Neutropenia

Chronic Neutropenia: Well Defined Market with Limited Treatment Options

~50,000[1]	~15,000[1]	1
U.S. Prevalence: total diagnosed with Chronic Neutropenia (CN)	Estimated subset with highest unmet need: minimum addressable market for mavorixafor in CN	Therapy approved for severe chronic neutropenia

Injectable Granulocyte Colony-Stimulating Factor (G-CSF)

- Approved to treat severe chronic neutropenia in 1995[2]
- Used as a chronic daily injection or as rescue during serious infection episodes
- Frequent treatment-related / treatment-limiting bone pain and other adverse events

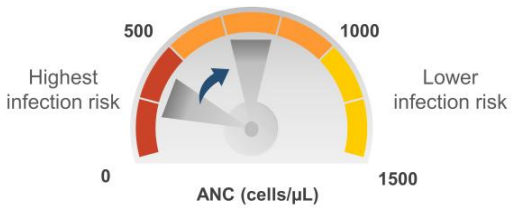
Innovation needed to address unmet patient needs

1. X4 Market Research, July 2023 – data on file; ICD-10 Code Research (2017-2023). 2. https://www.cancernetwork.com/view/fda-approves-new-indication-neupogen-chronic-neutropenia

Risk of Serious, Recurrent Infections Correlated to Severity of CN[1]

NIH Classification[2]	Absolute Neutrophil Count (ANC)
Severe (Grade 4)	<500 cells/µL
Moderate (Grade 3)	500 - 1,000 cells/µL
Mild (Grade 2)	1,000 - 1,500 cells/µL
Non-clinical (Grade 1)	1,500 = Lower Limit of Normal (LLN)

Increasing Neutrophil Counts >500 cells/µL Clinically Meaningful[6,7,8]



- Frequent and/or severe infections are the primary clinical consequence of chronic neutropenic disorders[3]
- Infections may lead to frequent hospitalizations or result in life-threatening complications, including death[4,5]

1. https://ctep.cancer.gov/protocoldevelopment/electronic_applications/docs/ctcae_v5_quick_reference_8.5x11.pdf. 2. Palmblad J, Dufour C, Papadaki HA. *Haematologica.* 2014 Jul;99(7):1130-1133. 3. Sicre de Fontbrune F, et al. *Blood.* 2015;126(14):1643-1650. 4. Donadieu J, et al. *Expert Rev Hematol.* 2021;14(10):945-960. 5. Salehi T, et al. *Iran J Allergy Asthma Immunol.* 2012;11(1):51-56. 6. Platzbecker, U, et al. *Blood.* 2019 Mar;133(10):1020-1030. 7. Donadieu J, et al. *Expert Rev Hematol.* 2021 Oct;14(10):945-960. 8. Newburger PE, et al. *Seminars in Hematology* 2013 Jul;50(3):198-206.

Mavorixafor's Validated Mechanism of Action

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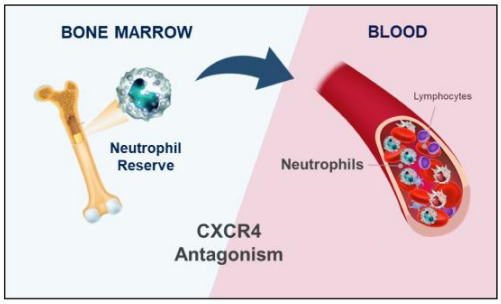
Validated Mechanism Shown to Increase Circulating Neutrophils

Targeted Mechanism

- CXCR4 regulates movement of white blood cells throughout the body[2]
- **CXCR4 antagonism** shown to increase migration of neutrophils from bone marrow to peripheral circulation[3,4]



Modified figure from reference 1

Mavorixafor: Orally Active CXCR4 Antagonist

- Single dose of oral mavorixafor shown to raise blood levels of neutrophils in patients with chronic neutropenia in Phase 1b clinical trial[5]
- Ongoing 6-month Phase 2 trial assessing chronic use of mavorixafor in patients with chronic neutropenia
- Approved for use in patients with WHIM syndrome, a rare primary immunodeficiency and chronic neutropenic disorder, “to increase the number of circulating mature neutrophils and lymphocytes”

1. Bainton DF (1980) *The Cell Biology of Inflammation*, vol 2, pp 1–25. Amsterdam: Elsevier/North-Holland. 2. Furze RC, et al, *Immunology*. 2008. 3. Mosi, RM, et al, *Biochem Pharmacol*, 2012. 4. Stone ND et al, *Antimicrob Agents Chemother*. 2007. 5. Warren JT, et al, oral presentation at ASH Annual Meeting December 2022.

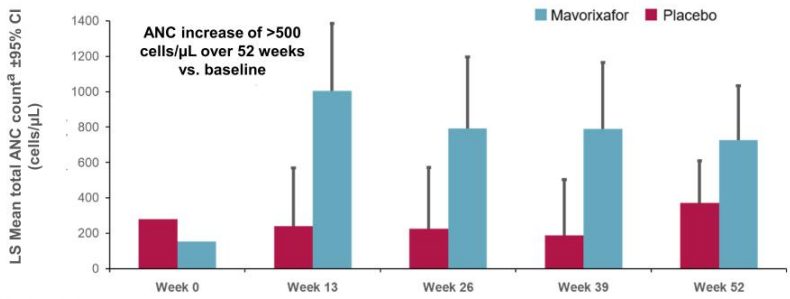
Mavorixafor Sustainably Raised ANC over 52 Weeks in Phase 3 4WHIM Trial

WHIM syndrome is a combined immunodeficiency and chronic neutropenia disorder

Primary Endpoint Met

- Significantly increased mean hours per day **above ANC threshold of 500 cells/µL**
- Mean time above threshold (TAT) for ANC was 15 hours for mavorixafor vs. 2.8 hours for placebo

Statistically Significant Increases in ANC Over Time*



	Week 0	Week 13	Week 26	Week 39	Week 52
Mavorixafor n	13	13	11	9	10
Placebo n	16	16	17	17	17[b]

All participants severely neutropenic at baseline

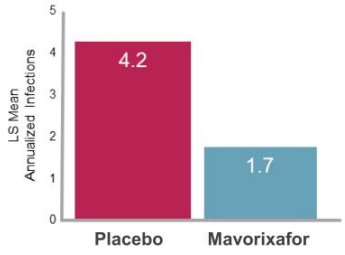
* Week 13 p=0.0049, Week 26 p=0.0397, Week 39 p=0.0196. a. Calculated as the mean of absolute cell counts over the 24-hour assessment period; b. At week 52, 3 of 17 placebo patients were given mavorixafor in advance of their TAT measurements as they entered the open-label portion of the study. All data are included in ITT analysis.

ANC Increase Resulted in Clinical Infection Benefits in Phase 3 4WHIM Trial[1,2]

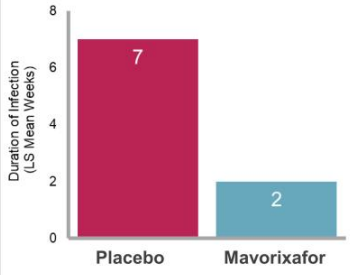
Mean ANC increases of >500 cells/µL reduced infection rate, duration, and severity

Total infection score[3] 40% lower for patients on mavorixafor versus those on placebo

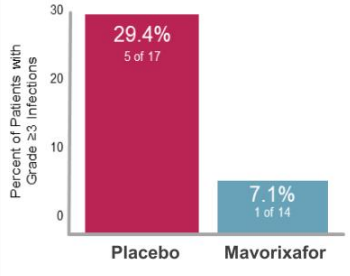
~60% reduction in annualized infection rate



Average infection duration was 5 weeks shorter



Fewer patients with severe infection



1. Badolato R, et al. *Blood.* Published online April 21, 2024;blood.2023022658. 2. Badolato R. et al. Oral Presentation at Annual Meeting of the Clinical Immunology Society, May 2023. 3.Total infection score calculated by summing the number of infection events weighted by severity and divided by the total exposure time (in years).

Interim Phase 2 CN Trial Results

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Objectives of Interim Analysis of Phase 2 Clinical Trial in Chronic Neutropenia

1 **Assess the ability of mavorixafor to increase ANC by at least 500 cells/µL as a monotherapy and in combination with stable-dose G-CSF over 6 months**

- Explore durability of Phase 1b study results showing 100% response (>500 cells/µL) to single dose of mavorixafor +/- G-CSF[1]

2 **Assess the ability of mavorixafor monotherapy to durably increase ANC in severe CN participants (with baseline ANC<500 cells/µL)**

- Severe CN seen as 'tougher-to-treat' population / similar to Phase 3 WHIM trial population
- Experts target ANC of ~800-1,000 cells/µL[2]

3 **Assess the safety of mavorixafor +/- G-CSF**

Success factors to confirm design of global, pivotal Phase 3 CN trial

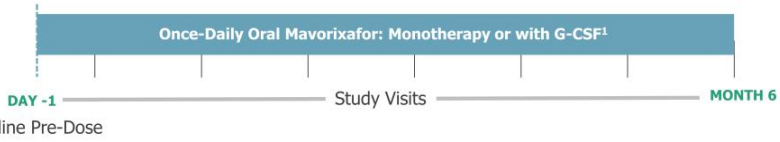
1. Warren JT, et al, oral presentation at ASH Annual Meeting December 2022. 2. X4 Advisory Board - expert opinion on file.

Assessing Mavorixafor in 6-Month CN Phase 2 Clinical Trial

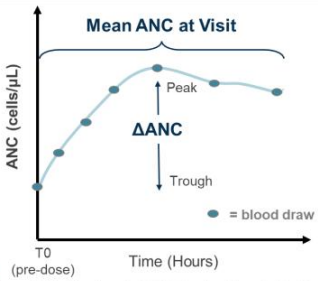
Mavorixafor dosed orally once-daily with or without background injectable G-CSF

Mavorixafor: Same Oral Dosing as 4WHIM Phase 3

Phase 2 Trial: Safety, Durability of ANC Levels over 6-Month Period



Timepoint Efficacy Assessments – Per Participant



Assessments at Baseline, Month 1, Month 3, and Month 6

- **At Each Visit:** up to 7 blood samples drawn over 8 hours
- **Mean ANC at Visit:** mean of absolute neutrophil counts from blood draws over the 8-hour period
- **ΔANC:** ANC at Peak minus ANC at Trough (T0)[2]

1. Modifications to G-CSF dosing allowed after Month 2 at doctor's discretion; additional data expected in late 2024. 2. For one participant, trough = first available sample

Interim Analysis of Six-Month Phase 2 Clinical Trial in Chronic Neutropenia

Ongoing study fully enrolled with 23 participants across three groups:

- Mavorixafor Monotherapy
- Mavorixafor + Stable-dose G-CSF
- Mavorixafor + G-CSF with Dose-Adjustments

Participant Disposition as of May 14, 2024 Data Cut

Phase 2 Treatment Groups	Participants	Month 1	Month 3	Month 6 (Complete)	Ongoing	Discontinued[1]
Mavorixafor Monotherapy	10	10	9	4	4	2
Mavorixafor + Stable-dose G-CSF	5	4	4	3	1	1
Mavorixafor + G-CSF w/ Dose-Adjustments	8 → dose-adjustments ongoing					

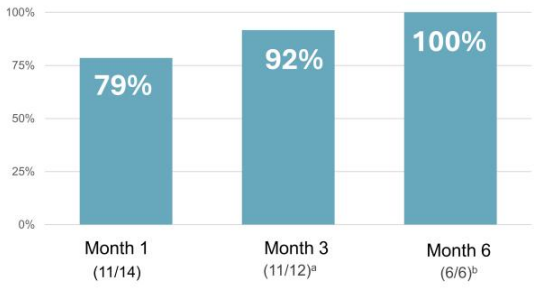
Types of chronic neutropenia studied: Idiopathic = 14; Congenital = 7; Cyclic = 2

Today's presentation is focused on monotherapy and stable-dose G-CSF groups

1. Three discontinuations due to Grade 1/2 GI tolerability following Day 1, Month 1, and Month 3 dosing, respectively

100% of Evaluated Study Participants Achieved Target ΔANC at Month 6

Percentage of Participants Achieving Target ANC Increase of >500 cells/µL at Each Timepoint



Interim data demonstrate durability of ANC response seen in single-dose Phase 1b trial of mavorixafor

TOTAL PARTICIPANTS (n=14)

- All had at least one timepoint to assess target ΔANC

Completed Participants (n=6)

- 6 (out of 14) completed and evaluable at M6
  - 100% achieved target ΔANC at M3 and sustained through M6

Other Participants (n=8)

- 6 (out of 14) evaluable through M3
  - 5/6 reached target ΔANC at M3
- 2 (out of 14) discontinued post M1 and M3
  - 2/2 achieved target ΔANC at all timepoints

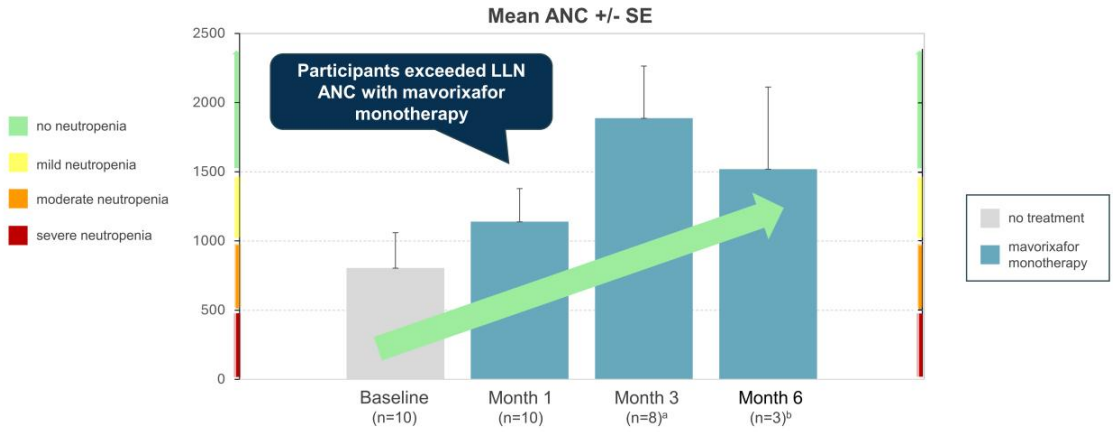
Demonstrated ability to maintain target ΔANC

a. Samples from one (1) participant who completed the study at M6, were unevaluable at M3. b. Samples from one (1) participant who completed the study were unevaluable at M6 (excluded at M6); participant data included in M1 and M3 assessments.

Mavorixafor Monotherapy: Durable Increases in Mean ANC

Mean ANC reached normal levels (ANC≥1500 cells/µL) after 3 months

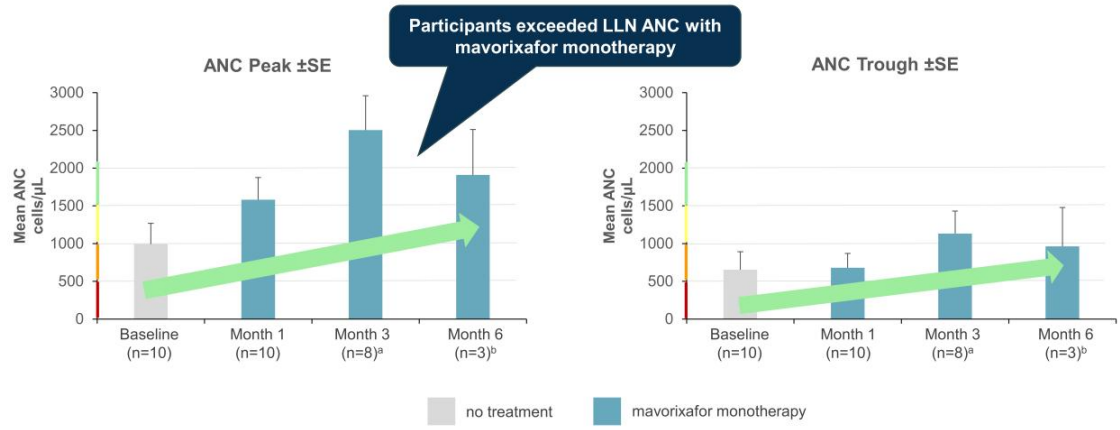
- Durable increases in mean ANC observed through Month 6



a. Samples from one (1) participant who completed the study were unevaluable at M3. b. Samples from one (1) participant who completed the study were unevaluable at M6.

Mavorixafor Monotherapy: Robust Daily Coverage in ANC

- Mean ANC Peak exceeded 1500 cells/μL Lower Limit of Normal (LLN) through 6 months
- Mean ANC Trough increased through 6 months



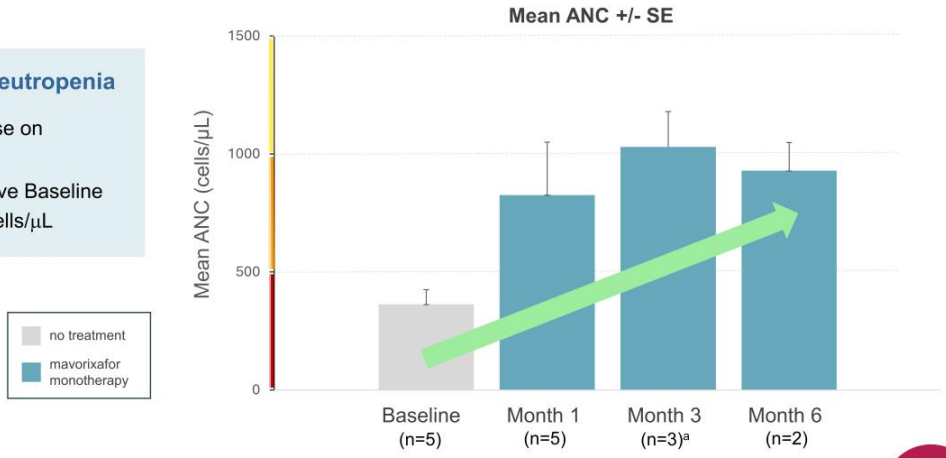
a. Samples from one (1) participant who completed the study were unevaluable at M3. b. Samples from one (1) participant who completed the study were unevaluable at M6.

Mavorixafor Monotherapy: Severe CN Participants Achieve Target ANC Increase

- Mean ANC increases of >500 cells/µL observed at Month 3 and Month 6 versus baseline

Severe Chronic Neutropenia

- 5/10 (50%) of those on monotherapy
- All participants have Baseline ANC below 500 cells/µL



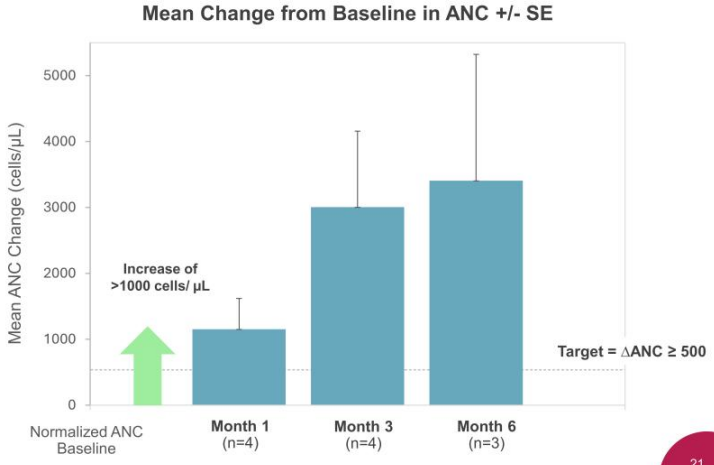
a. Samples from one (1) participant who completed the study were unevaluable at M3

Mavorixafor + Stable-Dose G-CSF: Robust Increases in ANC from Baseline

- Mean increases in ANC of >1000 cells/μL from baseline at all timepoints
- Supports potential for decreasing G-CSF dose

Normalized to Baseline ANC

- Due to G-CSF dose/ANC variability
- ANCs at baseline ranged from ~700 cells/μL to >1500 cells/μL



Phase 2 Chronic Neutropenia Study Safety Summary from Interim Analysis

Chronic mavorixafor well tolerated as monotherapy and in combination with stable-dose G-CSF

- → Overall safety profile consistent with prior studies
- → No new safety issues when dosed in combination with G-CSF
- → No deaths and no drug-related serious adverse events (SAEs)
- → Most frequent adverse events GI related: nausea and diarrhea
  - No discontinuations following education on possible GI effects that typically resolve over time

Interim Phase 2 Results Support Advancing to Pivotal Phase 3 CN Clinical Trial

✓ **Mavorixafor durably increased ANC by >500 cells/µL as a monotherapy**

- Raised participants' mean ANC above the lower limit of normal at Months 3 and 6
- Lowered potential infection risk by improving grade of neutropenia

✓ **Mavorixafor monotherapy durably increased ANC in severe CN participants (baseline ANC<500 cells/µL)**

- Achieved target ANC of ~800-1,000 cells/µL in this 'tougher-to-treat' population

✓ **Mavorixafor durably increased ANC by >1000 cells/µL in combination with stable-dose G-CSF**

- Supports potential for mavorixafor use to reduce G-CSF therapy

✓ **Mavorixafor well tolerated +/- stable-dose G-CSF**

- Safety profile consistent with prior studies of mavorixafor; supports chronic dosing +/- G-CSF

4WARD
A CHRONIC NEUTROPENIA STUDY

Phase 3 CN Trial & Market Opportunity

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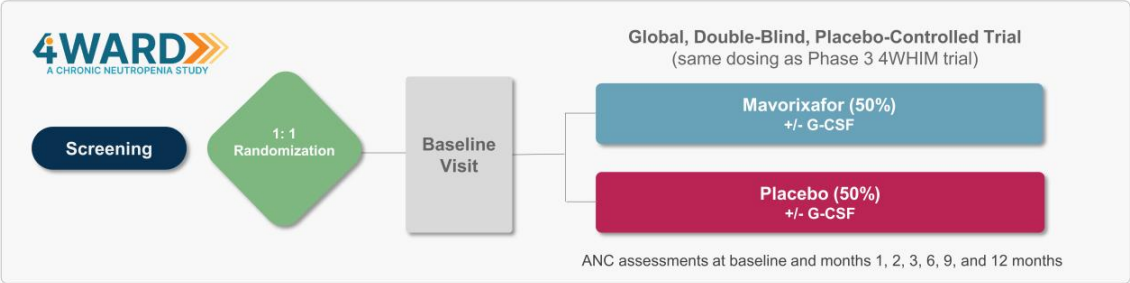
4WARD Pivotal, Global Phase 3 Trial in Most Common CN Indications

Participants now in screening



Key Inclusion Criteria for 150 participants with congenital, autoimmune, or idiopathic chronic neutropenia

- **Absolute Neutrophil Count (ANC):** <1500 cells/µL
- **Infection History:** 2 or more infections requiring intervention within last 12 months

Primary Endpoint: Two-component endpoint: positive ANC response and annualized infection rate

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

Note: 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.

Data to Date Support 4WARD Phase 3 CN Trial Primary Endpoint

Two-component Phase 3 endpoint: ANC response and annualized infection rate

Phase 2 interim analysis:

- 10 evaluated Phase 2 participants met Phase 3 inclusion criteria of baseline ANC<1500 cells/µL
  - **80%** (4/5) with baseline ANC<500 cells/µL demonstrated ≥2-fold ANC increase from baseline for at least 1 visit[1]
  - **80%** (4/5) with 500≤baseline ANC<1500 cells/µL achieved ANC≥1500 for at least 1 visit[1]
- Results exceed ~90% power currently proposed for positive ANC response endpoint in 150-participant Phase 3 trial

Successful mavorixafor for 4WHIM Phase 3 trial:

- Mavorixafor significantly increased participants' mean hours per day **above ANC threshold of 500 cells/µL**
- **ANC elevation >500 cells/ µL** resulted in:
  - ~60% reduction in annualized rate of infection
  - 5 weeks shorter duration of infection
  - Fewer patients with severe infections

1. Plan for durability analysis of ANC response is being finalized with U.S. FDA

Significant Opportunity to Address Unmet Needs in the CN Community

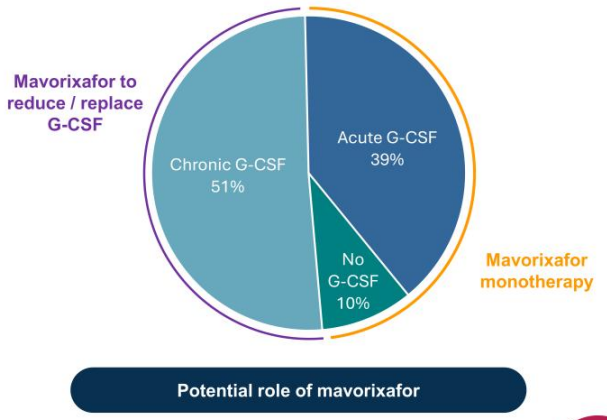
High unmet needs in ~15,000 patients in the U.S.

- Patients diagnosed with idiopathic, autoimmune, or congenital CN
- Adolescents and adults with history of severe/recurrent infections and/or previous/ongoing treatment with G-CSF

Current use of G-CSF within these patient populations

- ~51% of patients on chronic G-CSF therapy
- ~49% of patients not using G-CSF or on rescue use only

Current Use of G-CSF in ~15,000 U.S. CN Population with High Unmet Needs



Potential role of mavorixafor

X4 Market Research, July 2023 – data on file; ICD-19 Code Research (2017-2023).

Conclusions

Mavorixafor Holds Potential to Address Needs of CN Community

Clear unmet need

Only approved option has significant and treatment-limiting side effects and risks[1]

CN patients continue to experience frequent / severe infections despite G-CSF use

- **Mavorixafor: potential for well tolerated, chronic, oral, once-daily treatment to reduce / replace injectable G-CSF**

Treatment success well defined

Biomarker success = increase of at least 500 cells/μL in ANC shown to be clinically meaningful

Clinical success = ability to reduce annualized rate of infections

- **Mavorixafor: previous clinical benefits demonstrated in severely neutropenic WHIM patient population**

Defined / identified U.S. CN patient population

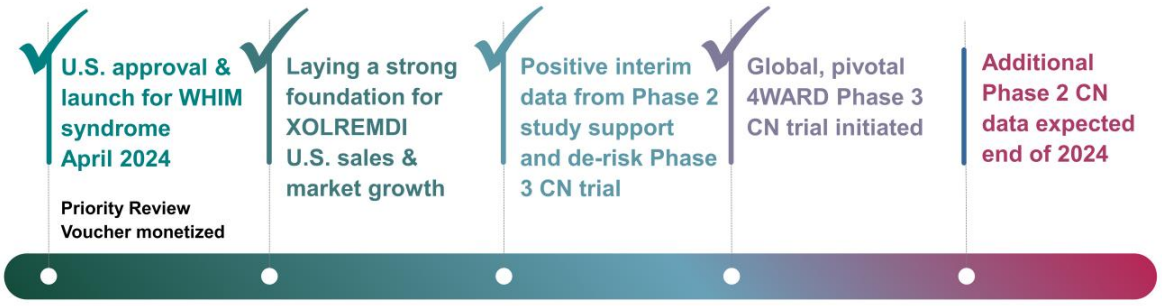
Defined/identified U.S. CN patient population

CN offers a large, well defined minimum U.S. market opportunity: ~15,000 already identified with high unmet needs

- **Mavorixafor: global, pivotal, 4WARD Phase 3 clinical trial aiming to address those with greatest unmet needs**

1. *Curr Opin Hematol*. 2017 January ; 24(1): 46–53.

X4 Well Positioned to Deliver on Promise of Mavorixafor



Potential Market Opportunities

Q&A Session