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): **November 13, 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 November 13, 2024, X4 Pharmaceuticals, Inc. (the "Company" or "X4") issued a press release titled "X4 Pharmaceuticals Announces Positive Results from Completed Six-Month Phase 2 Trial of Mavorixafor in Chronic Neutropenia (CN)". A copy of the press release is attached hereto as Exhibit 99.1.

On November 13, 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 November 13, 2024, the Company announced positive new clinical data from its now completed Phase 2 clinical trial evaluating mavorixafor, an oral CXCR4 antagonist, in the treatment of people with chronic neutropenia ("CN"). An analysis of final data from the six-month study showed that once-daily oral mavorixafor durably and meaningfully increased participants' mean absolute neutrophil counts ("ANC"). In addition, when tested in combination with injectable granulocyte colony-stimulating factor ("G-CSF"), mavorixafor treatment enabled clinicians to substantially reduce G-CSF dosing while maintaining normal mean ANC levels.

Results from Completed Phase 2 Study of Mavorixafor in CN

The Phase 2 study of mavorixafor was a six-month, open-label clinical trial that enrolled a total of 23 participants diagnosed with idiopathic, congenital, or cyclic chronic neutropenia. The analysis presented today includes results from the two study treatment groups: mavorixafor monotherapy (n = 10 at baseline) and mavorixafor in combination with G-CSF (n=13 at baseline).

Consistent with previously presented analyses, results from participants receiving mavorixafor monotherapy showed that mavorixafor durably increased mean ANC from baseline, with mean ANC reaching normal levels at Month 3 (n=9) and Month 6 (n=8). Further analysis showed that those with severe CN achieved greater than two-fold increases in mean ANC levels out to six months (n=4), reaching levels typically targeted by physicians for patients with severe CN.

New results presented today demonstrated that physicians chose to reduce G-CSF dosing in nine of 12 eligible participants. Of those nine, eight had G-CSF reduced at the earliest timepoint permitted and three were taken completely off of G-CSF prior to their Month 6 visit. Mean reductions in G-CSF were 52% at Month 3 (n=8) and 70% at Month 6 (n=9), while mean ANC levels remained in the normal range. The three participants receiving mavorixafor who remained on stable doses of G-CSF maintained mean ANC levels in the normal range at all timepoints.

The Company also announced new findings from a sub-study comparing the mean percentage of functional neutrophils in samples from healthy donors (n=5) to participants from the Phase 2 CN study (n=9) using two common study methods. These results demonstrated that the mean percentage of functional circulating neutrophils in CN participants in this sub-study was comparable to that of healthy donors after six months of mavorixafor dosing. The subset of participants with congenital neutropenia (n=4 of 9) also had mean percentage of functional neutrophils comparable to those of healthy donors. This is the first clinical demonstration of the functionality of

neutrophils mobilized by mavorixafor, and further increases the company’s confidence in achieving success in the ongoing pivotal Phase 3 4WARD clinical trial.

Overall, mavorixafor was generally well tolerated as a monotherapy and in combination with G-CSF, with no drug-related serious adverse events reported, 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 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 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 our ongoing Phase 3 clinical trial; the risk that trials and studies may be delayed and may not have satisfactory outcomes, including clinical results from our completed Phase 2 clinical trial; the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results, including clinical results from our completed Phase 2 clinical trial; 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 mavorixafor in chronic neutropenic disorders may be smaller than we anticipate; 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; 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 adverse safety effects arise from the testing or use of our product and product candidates; 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 August 8, 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 November 13, 2024.
99.2	Corporate Presentation, dated November 13, 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: November 13, 2024

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

Exhibit 99.1

X4 Pharmaceuticals Announces Positive Results from Completed Six-Month Phase 2 Trial of Mavorixafor in Chronic Neutropenia (CN)

Mavorixafor durably and meaningfully elevated participants' mean absolute neutrophil counts (ANC)

Mavorixafor enabled substantial reductions in G-CSF dosing while maintaining mean ANC at normal levels

Phase 2 study results and new analysis confirming functionality of neutrophils in sub-study participants bolster confidence in achieving success in Phase 3 4WARD trial

Company conference call and webcast today at 8:00 am ET

BOSTON, November 13, 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 now completed Phase 2 clinical trial evaluating mavorixafor, an oral CXCR4 antagonist, in the treatment of people with chronic neutropenia (CN). An analysis of final data from the six-month study showed that once-daily oral mavorixafor durably and meaningfully increased participants' mean absolute neutrophil counts (ANC). In addition, when tested in combination with injectable granulocyte colony-stimulating factor (G-CSF), mavorixafor treatment enabled clinicians to substantially reduce G-CSF dosing while maintaining normal mean ANC levels.

"Since the U.S. approval of G-CSF to treat severe chronic neutropenia, there has remained a significant unmet need for an optimal treatment in terms of long-term efficacy, safety, and route of administration," said Teresa Tarrant, M.D., Associate Professor of Medicine, Rheumatology, and Immunology at Duke University School of Medicine. "I am encouraged by these Phase 2 results showing that mavorixafor not only increased circulating neutrophils across study participants, but also that these neutrophils were functional in a sub-study population. The larger Phase 3 mavorixafor CN trial will expand on these data, and I remain optimistic about this potential much-needed innovation for patients with chronic neutropenia."

"We could not be more pleased with the results from the six-month Phase 2 study of mavorixafor in CN, which are consistent with our earlier findings that mavorixafor durably and meaningfully increased ANC in this population," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Importantly, we gained insights into the potential real-world use of mavorixafor in CN, should we ultimately obtain approval. These data not only confirm our interim findings, but also increase our confidence in a positive outcome for our ongoing pivotal Phase 3 4WARD trial and the potential of mavorixafor to help people living with chronic neutropenia."

Results from Completed Phase 2 Study of Mavorixafor in CN

The Phase 2 study of mavorixafor was a six-month, open-label clinical trial that enrolled a total of 23 participants diagnosed with idiopathic, congenital, or cyclic chronic neutropenia. The analysis presented today includes results from the two study treatment groups: mavorixafor monotherapy (n = 10 at baseline) and mavorixafor in combination with G-CSF (n=13 at baseline).

Mavorixafor monotherapy:

- Consistent with previously presented analyses, results from participants receiving mavorixafor monotherapy showed that **mavorixafor durably increased mean ANC from baseline, with mean ANC reaching normal levels** at Month 3 (n=9) and Month 6 (n=8).
    - Further analysis showed that **those with severe CN achieved greater than two-fold increases in mean ANC levels** out to six months (n=4), reaching levels typically targeted by physicians for patients with severe CN.

Mavorixafor in combination with injectable G-CSF:

- **New results** presented today demonstrated that physicians chose to **reduce G-CSF dosing in nine of 12 eligible participants**. Of those nine, eight had G-CSF reduced at the earliest timepoint permitted and three were taken completely off of G-CSF prior to their Month 6 visit.
    - Mean **reductions in G-CSF were 52% at Month 3 (n=8) and 70% at Month 6 (n=9)**, while mean ANC levels remained in the normal range.
- The three participants receiving mavorixafor who remained on stable doses of G-CSF maintained mean ANC levels in the normal range at all timepoints.

Neutrophil functionality assessment sub-study

- X4 also announced **new findings** from a sub-study comparing the mean percentage of functional neutrophils in samples from healthy donors (n=5) to participants from the Phase 2 CN study (n=9) using two common study methods. These results demonstrated that the mean percentage of **functional circulating neutrophils in CN participants in this sub-study was comparable to that of healthy donors** after six months of mavorixafor dosing.
    - The subset of participants with congenital neutropenia (n=4 of 9) also had mean percentage of functional neutrophils comparable to those of healthy donors.
    - This is the first clinical demonstration of the functionality of neutrophils mobilized by mavorixafor, and further increases the company's confidence in achieving success in the ongoing pivotal Phase 3 4WARD clinical trial.

Safety summary

- Mavorixafor was generally well tolerated as a monotherapy and in combination with G-CSF, with no drug-related serious adverse events reported, consistent with previous clinical studies.

Investor Event Details:

The company will host a conference call and webcast today at 8:00 a.m. ET. The conference call can be accessed by dialing 1-800-267-6316 from the United States or 1-203-518-9783 internationally, followed by the conference ID: X4PHARMA. The live webcast and accompanying slide presentation will be accessible through the investor relations section of X4 Pharmaceuticals' website at www.x4pharma.com. A live Q&A will follow the formal presentation. Following the conclusion of the call and webcast, a replay will be available on the company's website.

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 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 Phase 2 portion of the trial (n=23) assessed 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 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 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 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: 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 our ongoing Phase 3 clinical trial; the risk that trials and studies may be delayed and may not have satisfactory outcomes, including clinical results from our completed Phase 2 clinical trial; the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results, including clinical results from our completed Phase 2 clinical trial; the design and rate of enrollment for clinical trials, including the current design of a Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders may not enable successful completion of the trial(s); the commercial opportunity for mavorixafor in chronic neutropenic disorders may be smaller than we anticipate; 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; 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; adverse safety effects arise from the testing or use of our product and product candidates; 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 August 8, 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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(617) 430-7576

Exhibit 99.2

3Q 2024 Business Update & Phase 2 Chronic Neutropenia Study Results

November 13, 2024

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 sessions 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 the success of the 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 strategy for the commercial launch of XOLREMDI; the potential benefit of XOLREMDI in the Indication; the potential number of patients in the U.S. 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 trials will become available, as well as our research and development programs; X4's use of capital and other financial results, including its financial runway; 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 our ongoing Phase 3 clinical trial; the risk that trials and studies may be delayed and may not have satisfactory outcomes, including clinical results from our completed Phase 2 clinical trial; the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results, including clinical results from our completed Phase 2 clinical trial; the design and rate of enrollment for clinical trials, including the current design of a 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 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, including assessing the ability of mavorixafor monotherapy to durably increase absolute neutrophil count in patients with chronic neutropenic; adverse safety effects arise from the testing or use of our product and product candidates; general macroeconomic and geopolitical conditions could impact X4's business; X4 may be unable to raise additional capital; there is 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 August 8, 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	XOLREMDI Launch & Phase 3 Chronic Neutropenia Clinical Trial Update
03	Phase 2 Chronic Neutropenia Study Results • Mavorixafor monotherapy • Mavorixafor + adjusted-dose G-CSF • Neutrophil functionality subgroup analysis • Safety summary
04	Conclusions and Look Ahead to 2025
05	Q&A Session

Speakers

Paula Ragan, PhD
President & CEO

Christophe Arbet-Engels, MD, PhD
Chief Medical Officer

XOLREMDI® Launch and Phase 3 Chronic Neutropenia Clinical Trial Update

X4 PHARMACEUTICALS

U.S. Commercial Launch of XOLREMDI® in WHIM Syndrome[1]

XOLREMDI® (mavorixafor) capsules

Driving disease awareness to support patient identification and diagnosis across the U.S.

100% of launch targets reached: 3,400+ unique HCPs[2]

- **50+ conferences attended since launch** (national / regional / local)
- **Physician peer-to-peer speaker program** launched
- **Patient campaign** initiated
- **Favorable reimbursement** decisions and access:
  - Published policies represent >150 million covered lives

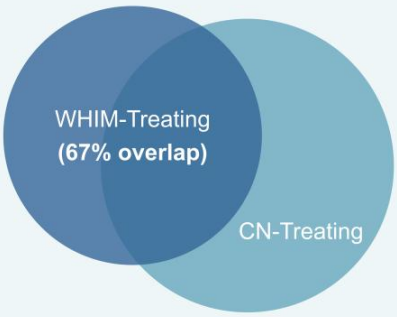
Recent Tracking Study of Likely XOLREMDI Prescribers[3]

- **Knowledge of WHIM syndrome** increased to >75%
- ~60% of HCPs report **increases in screening** for WHIM syndrome
- >80% of **HCPs considering prescribing XOLREMDI** for WHIM patients

1. WHIM (warts, hypogammaglobulinemia, infections, myelokathexis) - see full prescribing label at www.xolremdi.com; 2. HCPs (healthcare practitioners) reached through in-person and digital engagement; 3. X4 Market Research: HCP Tracking Study of immunologists and hematologists – Pre-launch (Mar 2024), Post-launch (October 2024)

WHIM Experience Builds Strong Foundation in Chronic Neutropenia (CN)

Hematologists* in United States



Significant Overlap Between WHIM and CN Treating Physicians; Similar Dynamic with U.S. Patient Advocacy Organizations

- 67% of targeted WHIM hematologists would also be potential prescribers for CN, if approved in U.S.
- X4 engaged with U.S. immunodeficiency and neutropenia patient advocacy groups that serve the WHIM and CN communities

Jeffrey Modell Foundation | Immune Deficiency Foundation | national neutropenia network

*Claims analysis, Oct 2024; XOLREMDI (mavorixafor) is being exclusively promoted in the U.S. for its approved indication of WHIM syndrome; mavorixafor is currently being studied as an investigational therapy for use in chronic neutropenia and has yet to be approved for the treatment for chronic neutropenia.

4WARD Phase 3 Trial On Track to Fully Enroll in Mid-2025

~40% of planned sites now initiated; participants being dosed across multiple countries

Recruitment, screening, and dosing ongoing

- Expect majority of sites to be initiated in early 2025

4WARD Plan	Status
20 – 25 countries	**On Track** Protocol authorizations in ~85% of targeted countries
90 – 110 sites	**On Track** ~40% of planned sites initiated

4WARD
A CHRONIC NEUTROPENIA STUDY

12-Month, Global, Double-Blind, Placebo-Controlled Phase 3 Trial

Oral, Once-Daily Mavorixafor (50%)
+/- G-CSF

Placebo (50%)
+/- G-CSF

- **150 participants** with congenital, acquired primary autoimmune, or idiopathic chronic neutropenia
- **Primary Endpoint:** ANC response and annualized infection rate

X4 PHARMACEUTICALS

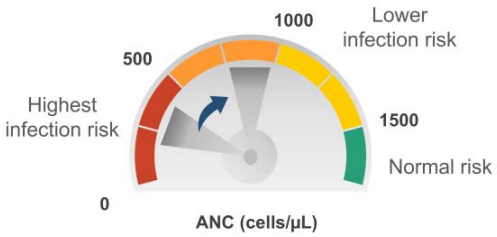
For more on 4WARD trial: NCT06056297

Phase 2 Chronic Neutropenia Study Results

Risk of Serious, Recurrent Infections Correlates with Neutrophil Counts in 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)

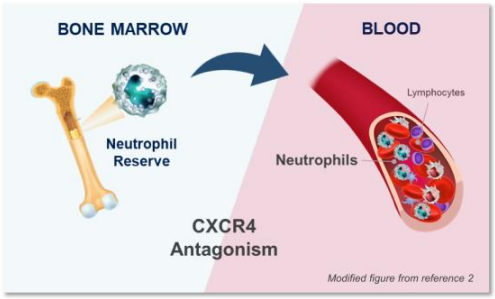
Increasing Neutrophil Counts >500 cells/µL Clinically Meaningful[6,7,8]



- Frequent and/or serious 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 Shown to Increase Circulating Neutrophils, Decrease Infections in WHIM Syndrome



Mavorixafor: Orally Active CXCR4 Antagonist

U.S. FDA Approved[1] 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”

Mean ANC increases of >500 cells/μL reduced infection rate, duration, and severity in pivotal Phase 3 WHIM trial

Mavorixafor Sustainably Raised ANC over 52 Weeks in 4WHIM Trial

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

1. See full prescribing label at www.xolremdi.com; 2. Bainton DF (1980) *The Cell Biology of Inflammation*, vol 2, pp 1–25. Amsterdam: Elsevier/North-Holland.

Unmet Needs in Chronic Neutropenia: Patients and Physicians Eager for Innovation

Only Currently Approved Therapy: Injectable Granulocyte Colony-Stimulating Factor (G-CSF)

- Approved to treat severe chronic neutropenia in 1995[1]
- Used as a chronic daily injection or as rescue during serious infection episodes
- **Frequent treatment-related / treatment-limiting bone pain other adverse events, and long-term risk of myelodysplastic syndrome and/or leukemia**

> "The administration [of G-CSF] is painful and also can have **long-term consequences**."
>
> **Jolan Walter, MD, PhD**

> "It is a medical need to improve the infection rate of the patient by a less aggressive or **less painful treatment**."
>
> **Jean Donadieu, MD, PhD**

> "Often, **the effective [G-CSF] dose is also a toxic dose**, so you have to slowly back down off the dose."
>
> **Peter Newburger, MD**

> "If I get the **extreme bone pain**, I am unable to sleep. It's unreal …**I dread injecting every day.** I dread it. It's the worst part of my day."
>
> **Vanessa, CN Patient**

> "**You're fighting a medicine** that's there to make you feel better or fend off infections …[but it] makes you feel like absolute crap."
>
> **Kevin, CN Patient**

1. https://www.cancernetwork.com/view/fda-approves-new-indication-neupogen-chronic-neutropenia

Significant Opportunity to Address Unmet Needs in CN Community

50,000[1] Diagnosed U.S. CN Population
~15,000 with High Unmet Needs

High unmet needs in ~15,000 patients in the U.S.[1]

- Patients diagnosed with idiopathic, autoimmune, or congenital CN (Phase 3 trial target population)
- Adolescents and adults with history of serious/recurrent infections and/or previous/ongoing treatment with G-CSF

Current use of G-CSF within these high unmet need patient populations

- ~51% of patients on chronic G-CSF therapy
- ~49% of patients not on chronic G-CSF therapy

Broad Opportunity for Mavorixafor: Monotherapy or in Combination with G-CSF

Mavorixafor Monotherapy

To treat those:

- Naïve to G-CSF
- Intolerant or unresponsive to G-CSF
- Using G-CSF acutely, on demand

To enable a meaningful reduction in G-CSF dosing, lessening pain, discomfort, and long-term risk of malignancies

Mavorixafor + G-CSF

1. X4 Market Research, July 2023 – data on file; ICD-10 Code Research (2017-2023).

Phase 2 Clinical Trial in Chronic Neutropenia: Goals and Design

Main Phase 2 Study Goals

- ✓ Confirm durability of positive Phase 1b results
- ✓ Explore whether physicians will reduce G-CSF
- ✓ Assess long-term safety and tolerability
- ✓ Inform design of and derisk Phase 3 pivotal trial

Phase 2 Study: Assessing Safety, Durability of ANC Levels over 6-Month Period[1]

1. The neutrophil life-cycle is 10-14 days (https://doi.org/10.3389/fimmu.2021.766620); Phase 2 study's measurements of ANC over 6 months (at 30-day intervals) assess bone marrow status and durability of neutrophil production. 2. Modifications to G-CSF dosing allowed after Month 2 at physician's discretion.

Phase 2 Clinical Study in Chronic Neutropenia: Participant Disposition

Study group representative of typical CN population

Phase 2 Study Enrolled a Total of 23 Participants

Participant Disposition (n=23)	
Type of CN	
Idiopathic	15
Congenital[1]	6
Cyclic	2
Sex	
Male	10
Female	13
Mean Age	34

Mavorixafor Monotherapy	Baseline
Total	10

Mavorixafor + G-CSF	Baseline
Stable G-CSF Total	4
Adjusted G-CSF[2] Total	9

Neutrophil Functionality Sub-Study	Assessed
Total Evaluable Population[3]	9

1. Congenital CN participants included those with *ELANE* variant (n=2), VPS13B variant (Cohen syndrome), G6PC3 variant/ deficiency, SRP54 variant (SDS-like syndrome), WASp variant (Wiskott-Aldrich syndrome).

2. Modifications to G-CSF dosing allowed after Month 2 visit

3. Samples assessed for neutrophil functionality were limited by proximity to validated testing facility – complete data were available for 9 of the 23 enrolled participants.

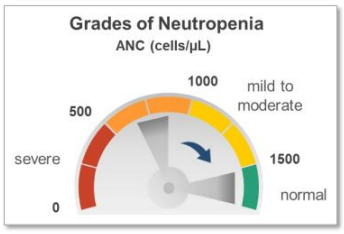
Questions Addressed Today from Phase 2 CN Study Results

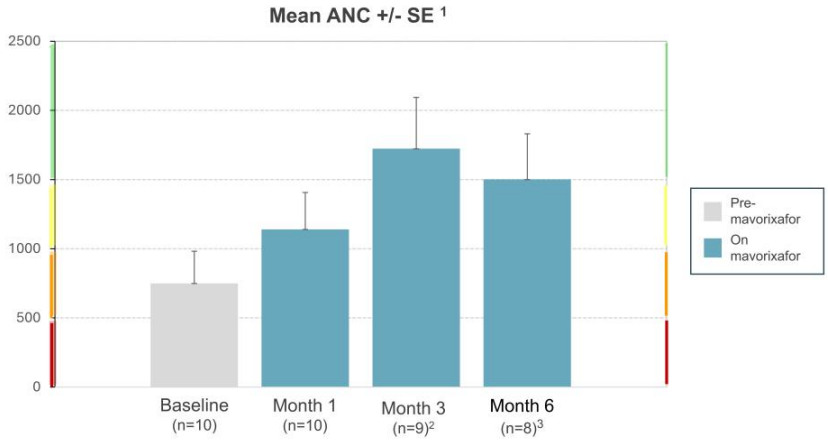
Phase 2 CN Study Population	Key Questions
Mavorixafor Monotherapy	• Does mavorixafor monotherapy durably sustain ANC at clinically meaningful levels?
NEW **Mavorixafor + Adjusted-Dose G-CSF**	• Are physicians and patients willing and able to adjust G-CSF with mavorixafor treatment? • Can G-CSF be reduced while maintaining clinically meaningful ANC levels?
NEW **Sub-Population Eligible for Neutrophil Functionality Study**	• Are neutrophils mobilized by mavorixafor functional?

Mavorixafor Monotherapy Durably and Meaningfully Increased Mean ANC

Results increase confidence in successful Phase 3 trial outcome

- Mean ANC reached normal levels (ANC ≥ 1,500 cells/µL) at 3 and 6 months of treatment



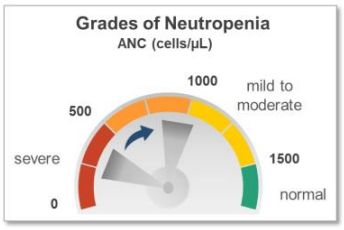


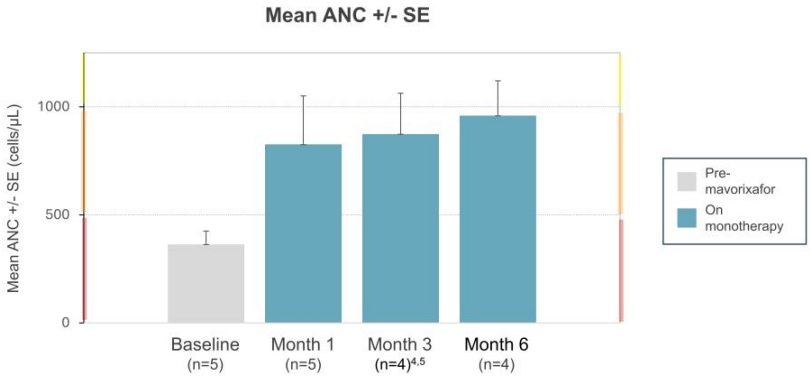
1. Data set contains two LOCF (last observation carried forward) values: one value missing at M3 assessment, one value missing at M6. 2. One patient discontinued prior to Month 3 assessment (no change from data set presented on June 27, 2024). 3. One patient discontinued prior to Month 6 assessment (no change from data set presented on June 27, 2024).

Mavorixafor Monotherapy Durably and Meaningfully Increased Mean ANC in Severe CN

Results increase confidence in successful Phase 3 trial outcome

- Physicians typically target ANC between 800 and 1,000 cells/µL in severe CN patients[1,2,3]
- Those with severe CN achieved >2x Baseline mean ANC through Month 6

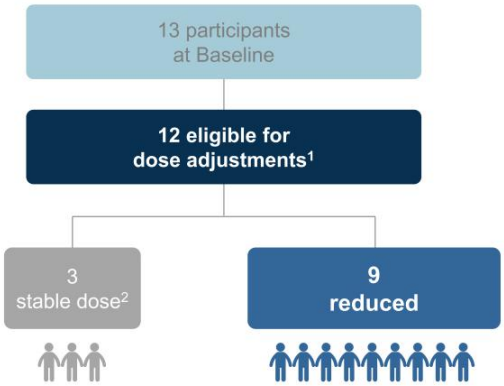




1. Platzbecker, U, et al. *Blood.* 2019 Mar;133(10):1020-1030. 2. Donadieu J, et al. *Expert Rev Hematol.* 2021 Oct;14(10):945-960. 3. Newburger PE, et al. *Seminars in Hematology* 2013 Jul;50(3):198-206. 4. Data set contains one LOCF (last observation carried forward) value, due to missing ANC at M3. 5. One patient discontinued prior to Month 3 assessment (no change from data set presented on June 27, 2024)

Physicians Chose to Reduce G-CSF in 75% of Eligible Participants

Mavorixafor + G-CSF Combination Group



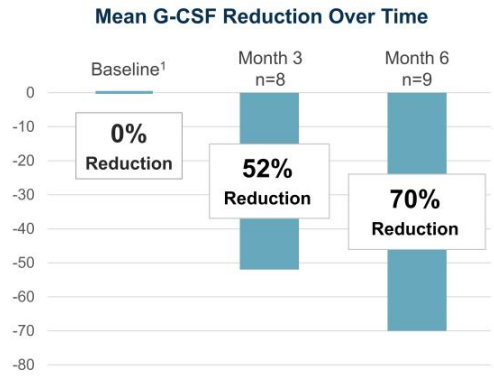
Clinicians given the option to reduce G-CSF following Month 2 visit

- 75% (9 of 12) eligible participants had G-CSF reduced
- **33% (3 of 9) of participants with dose adjustments taken completely off G-CSF prior to Month 6 visit**
- Perspective into physicians' possible real-world use of mavorixafor in CN

NEW

1. One participant discontinued prior to M1 assessment; 2. Results from 3 participants maintained on stable dose G-CSF similar to data set presented on June 27, 2024 (data on file).

Physicians Substantially Reduced G-CSF, Maintaining Normal Mean ANC



Key Takeaways:

G-CSF:

- Given the option, physicians chose to substantially reduce injectable G-CSF therapy in 9 of 12 eligible patients
- 89% (8 of the 9) had G-CSF adjusted at earliest possible timepoint (following Month 2 visit)
- Potential to improve patients' quality of life and lower long-term risk of malignancy from chronic G-CSF use

	Baseline	Month 3 (8 adjusted)	Month 6 (9 adjusted)
Mean ANC (cells/µL)	>1,500	>1,500	>1,500

ANC:

- Mean ANC maintained at normal levels (>1,500 cells/µL) through Month 6

NEW

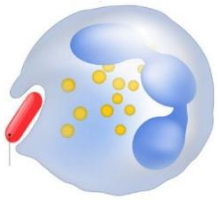
Neutrophil Functionality Assessed in Participants Enrolled in Phase 2 Sub-Study

Purpose: Demonstrate functionality of neutrophils in blood of individuals with CN, including those with congenital CN and genetic variants associated with neutrophil maturation arrest

Neutrophil Functionality Assays[1]

Phagocytosis[2] (data to follow)

Assessment of neutrophils' ability to engulf pathogens



ROS production (data on file)

Assessment of neutrophils' ability to produce ROS (reactive oxygen species) to damage/kill pathogens

Participant Disposition Well Balanced

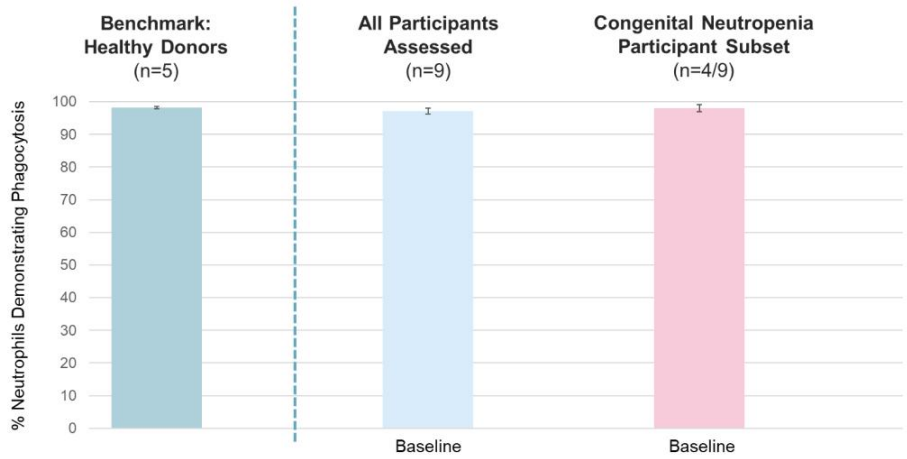
Phase 2 Sub-Study (n)[3]	**9**
Idiopathic / Congenital	5 / 4
Mav Mono / Mav + G-CSF	4 / 5
Healthy Donors (n)	**5**

Neutrophil function studies assessed *ex vivo* blood neutrophil responses to bacterial challenge (opsonized *E. coli*) from clinical samples drawn from participants during the study.

NEW

1. Ashley N. Connelly, et. al., Optimization of methods for the accurate characterization of whole blood neutrophils, *Scientific Reports*, 12:3667 (2022); 2. Ankur Gupta-Wright, et. al., Functional Analysis of Phagocyte Activity in Whole Blood from HIV/Tuberculosis-Infected Individuals Using a Novel Flow Cytometry-Based Assay, *Frontiers in Immunology*, Vol 8, Article 1222, (2017); 3. Three trial sites were eligible to participate in the neutrophil functionality sub-study; eligibility requirements included ability to ship clinical samples for analysis at validated testing facility within 24-hour window.

Neutrophils Functional in Healthy Donors and Pre-Treatment Phase 2 Participants



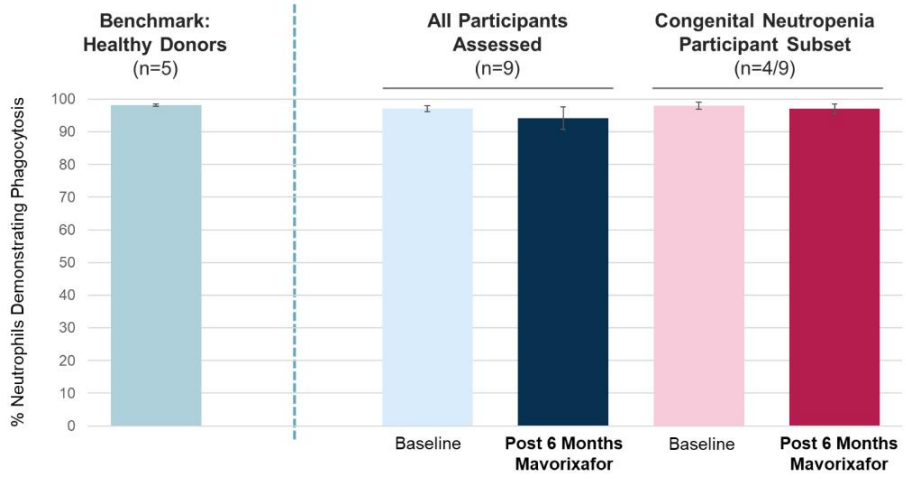
At baseline (pre-treatment): Mean percentage of functional neutrophils in study population were comparable to healthy volunteers

Notes: ROS results for all subjects demonstrate similar profiles to phagocytosis; Idiopathic CN subjects had similar results as those with congenital neutropenia (all data on file)

NEW

Neutrophil Functionality Maintained After 6 Months of Mavorixafor Therapy

Meaningful increases in circulating functional neutrophils expected to reduce infection risk



Mean percentage of functional neutrophils remained comparable to healthy donor controls after 6 months of treatment

Mean percentage of neutrophils performing ROS functions[1,2] were also comparable to healthy donors

NEW

Notes: ROS results for all subjects demonstrate similar profiles to phagocytosis; Idiopathic CN subjects had similar results as those with congenital neutropenia (all data on file)

Phase 2 Chronic Neutropenia Study Safety Summary

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

- Overall safety profile consistent with prior studies
- No new safety issues observed when dosed in combination with G-CSF
- No deaths and no drug-related serious adverse events (SAEs)
- Most frequent treatment-related TEAEs[1] were GI related (nausea and diarrhea); 3 discontinuations in total (all early in study execution)[2]

Treatment-related TEAEs Occurring in >20% of Participants

All mild to moderate

	Combination (n=13), n (%)	Monotherapy (n=10) n (%)	Overall (n=23) n (%)
Any Related AE	10 (76.9)	7 (70.0)	17 (73.9)
Nausea	4 (30.8)	5 (50.0)	9 (39.1)
Diarrhea	4 (30.8)	3 (30.0)	7 (30.4)

1. TEAE: treatment-emergent adverse event; 2. No further discontinuations once education on GI symptoms and support were implemented.

Phase 2 Results Support Mavorixafor Potential in CN and Raise Confidence in Success of Pivotal, Phase 3 Trial

Key Questions	Phase 2 Findings
• Does mavorixafor monotherapy durably sustain ANC at clinically meaningful levels?	**Yes**, mavorixafor durably and meaningfully increased mean ANC
• Are physicians and patients willing and able to adjust G-CSF with mavorixafor treatment?	**Yes,** physicians chose to reduce G-CSF dosing in the majority of eligible participants
• Can G-CSF be reduced while maintaining clinically meaningful ANC levels?	**Yes,** mavorixafor enabled reductions in G-CSF dosing while maintaining mean ANC at normal levels
• Are neutrophils mobilized by mavorixafor functional?	**Yes,** neutrophils mobilized by mavorixafor were durably functional in idiopathic and congenital CN participants

Mavorixafor generally well tolerated +/- G-CSF

Meaningful increases in circulating functional neutrophils expected to reduce infection risk in CN Phase 3 population

Physicians and Patients Eager for an Innovation like Mavorixafor

Conclusions and Look Ahead to 2025

X4 PHARMACEUTICALS

Continuing to Deliver Progress for Patients

Expected Key 2025 Milestones

- **U.S. Launch of XOLREMDI ongoing** — XOLREMDI (mavorixafor) capsules
- **Positive Phase 2 CN data support and derisk Phase 3 CN trial**
- **Global, pivotal 4WARD Phase 3 CN trial ongoing** — 4WARD A CHRONIC NEUTROPENIA STUDY
- **EU MAA WHIM submission by early 2025**
- **XOLREMDI commercial uptake**
- **4WARD Trial fully enrolled in mid-2025**

Potential Market Opportunities

WHIM
>1,000 U.S. patients

Chronic Neutropenia
>15,000 U.S. patients

Q&A Session