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): **May 22, 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**

Attached as Exhibit 99.1 and furnished for purposes of Regulation FD is a presentation that X4 Pharmaceuticals, Inc. (the "Company") may use from time to time in presentations or discussions with investors, analysts, and other parties.

The information furnished under this Item 7.01 (including Exhibit 99.1) shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall such information be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01 **Financial Statements and Exhibits.**

Exhibit No.	Description
99.1	Corporate Presentation, dated May 22, 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: May 22, 2024

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


Exhibit 99.1
X4
PHARMACEUTICALS
May 2024
PROGRESS 4 PATIENTS
Enabling a better future for people with rare immune disorders

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"), including the success of its commercial launch in the U.S. through PANTHERx Rare; 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; 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; 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; 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.

X4 Pharmaceuticals Overview

Fully integrated company delivering innovation for patients with rare immune disorders

XOLREMDI™ (mavorixafor) capsules

- Approved by FDA in April 2024!
- First drug indicated for patients with WHIM syndrome[1]
- Launch underway targeting key immunologists and hematologists
- EU submission expected in late 2024 / early 2025
- Exploring additional global commercialization opportunities

ADVANCING MAVORIXAFOR IN ADDITIONAL INDICATIONS

- Clinical data from ongoing Phase 2 trial in chronic neutropenia (CN) expected in June 2024
- Global, pivotal Phase 3 clinical trial in CN initiation anticipated in 2Q 2024
- Exploring additional potential rare disease indications

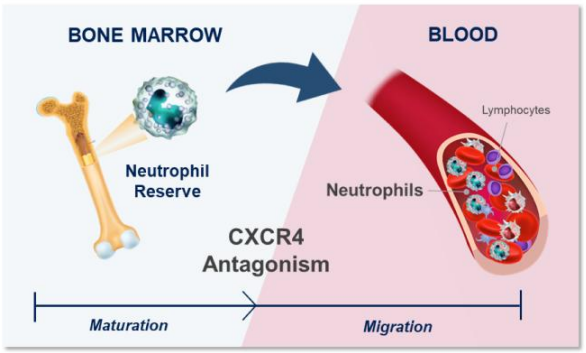
STRONG BALANCE SHEET SUPPORTS CONTINUED GROWTH

- Pro forma funds of $207 million[2]
- Balance sheet expected to fund operations into late 2025[3]

1. WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis); 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.

Mavorixafor: Pipeline in a Product via CXCR4 Antagonism

Validated mechanism shown to alleviate neutropenia and lymphopenia



Modified figure from reference 1

Targeted Mechanism

- CXCR4 regulates movement of white blood cells throughout the body[2]
- CXCR4 antagonism has been shown to increase the migration of cells from the bone marrow, increasing circulating levels of neutrophils and lymphocytes[3,4]

Orally active CXCR4 Antagonist

- Mavorixafor has been shown to raise circulating blood levels of neutrophils and lymphocytes[4,5,6]
- Clinical potential across multiple rare immunodeficiencies
- U.S. patent protection expected through 2038

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. Badolato R, et al. Blood. Published online April 21, 2024;blood.2023022658; 6. Warren, JT et al, Oral Presentation of results from Phase 1b trial of mavorixafor in patients with chronic neutropenic disorders at the 2022 annual meeting of the American Society of Hematology.

Advancing Mavorixafor in Chronic Neutropenic Disorders and WHIM Syndrome

Only oral candidate marketed / in clinical development across these indications

	Indication	Preclinical	Phase 1	Phase 2	Phase 3	FDA Approved	EXPECTED MILESTONES
XOLREMDI™ (mavorixafor)	WHIM Syndrome (Warts, Hypogammaglobulinemia, Infections and Myelokathexis)			FDA Approved in April 2024			U.S. launch underway EU submission in late 2024 / early 2025
Mavorixafor	Chronic Neutropenia (Congenital, Autoimmune, or Idiopathic)			Entering Phase 3			Interim Ph 2 data (n>15) in June 2024 Pivotal Phase 3 initiation 2Q 2024
X4P-003	TBD						

WHIM Syndrome: a Combined Primary Immunodeficiency and Chronic Neutropenic Disorder[1]

Heterogeneous presentation of symptoms caused by CXCR4 dysfunction[2]

Most frequently characterized by:

Fewer than 1 in 4 patients present with all 4 manifestations in the WHIM acronym (warts, hypogammaglobulinemia, infections, and myelokathexis)

Based on an international cohort of 66 patients with WHIM syndrome, which included pediatric (65%) and adult (35%) patients.

Lifelong impact[2]

Chronic, congenital disorder

Commonly presents in childhood, with median age of diagnosis of 5.5 years of age

Lower life expectancy vs. the general population[3,4] due to sepsis, irreversible organ damage, recurrent pneumonia, and certain cancers

Ultra-rare population[5]

Estimated to be at least 1,000 people in the U.S.

Based on X4 market research 2019, 2020.

References: 1. Dale DC, Firkin F, Bolyard AA, et al, Blood. 2020;136(26):2994-3003. 2. Geier CB, Ellison M, Cruz R, et al, J Clin Immunol. 2022;42(8):1748-1765; 3. Dotta L, Notarangelo L, Moratto D, et al. J Allergy Clin Immunol. 2019;7(5):1568-1577; 4. Beaussant Cohen S, Fenneteau O, Plouvier E, et al. Orphanet J Rare Dis. 2012;7:71; 5. Data on file. X4 Pharmaceuticals, Inc., 2024.

Until Now, WHIM Syndrome Managed with Treatments Not Addressing Underlying Cause

Symptomatic Treatments

- Not specifically indicated for WHIM syndrome
- No adequate and well controlled trials evaluating safety and efficacy in patients with WHIM syndrome[1,2]
- G-CSF and IVIg associated with burdensome administration
- Long-term use of antibiotics associated with risk of developing antimicrobial resistance (AMR) and cumulative risk of adverse events[3]
  - 73% of surveyed HCPs (n=74) are concerned about antibiotic resistance in WHIM syndrome patients[4]

G-CSF: granulocyte colony-stimulating factor; IVIg: intravenous immunoglobulin.

References: 1. Dale DC, Firkin F, Bolyard AA, et al, Blood. 2020;136(26):2994-3003; 2. Geier CB, Ellison M, Cruz R, et al, J Clin Immunol. 2022;42(8):1748-1765; 3. Kiss C, Connoley D, Connelly K, et al, Antibiotics, 2022 Jan 11(1): 62; 4. X4 March 2024 Research; 74 HCPs (44 Immunologists and 30 HEM/ONCs).

Now FDA approved!

For use in patients 12 years of age and older with WHIM syndrome (warts, hypogammaglobulinemia, infections and myelokathexis) to increase the number of circulating mature neutrophils and lymphocytes.

See full prescribing information at xolremdi.com

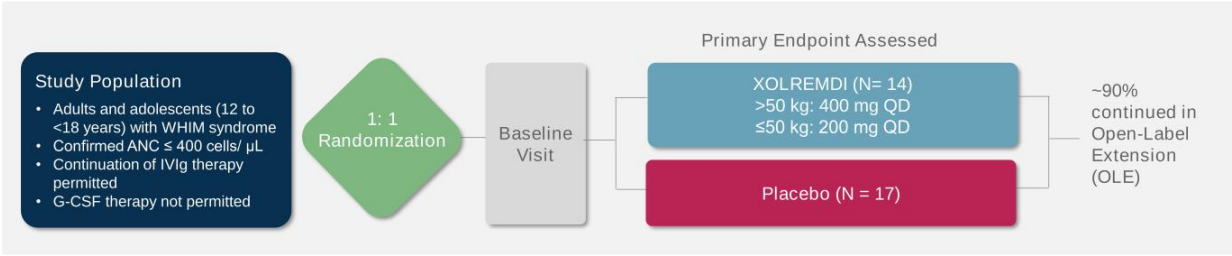
XOLREMDI™

(mavorixafor) capsules

(zōl-RĚM-dee)

4WHIM: the Largest Phase 3 Clinical Trial to Date in WHIM Syndrome

XOLREMDI was studied in a global, randomized, double-blind, placebo-controlled, Phase 3 trial conducted in 31 patients with WHIM syndrome



Primary endpoint

- Improvement in absolute neutrophil count (ANC) as measured by the mean time above ANC threshold of 500 cells/µL at 13, 26, 39, and 52 weeks

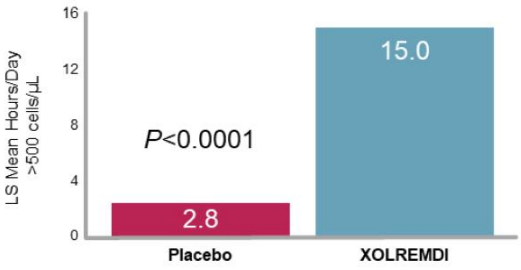
Secondary endpoints

- Improvement in absolute lymphocyte count (ALC) as measured by the mean time above ALC threshold of 1000 cells/µL at 13, 26, 39, and 52 weeks
- Composite endpoint: Analysis of total infection score (rate, severity) and total wart change score

4WHIM: XOLREMDI Significantly Increased Time Patients Stayed Above Key Immune Cell Count Thresholds over 52 Weeks versus Placebo

Primary endpoint

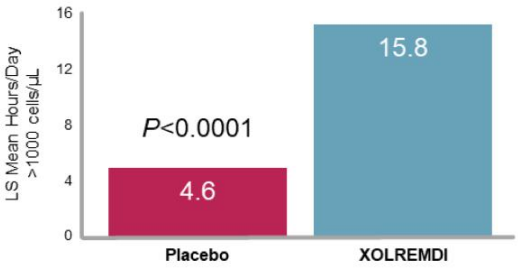
Significantly increased mean hours per day above the threshold for neutrophils



Severe neutropenia threshold = 500 cells/ µL

Key secondary endpoint

Significantly increased mean hours per day above the threshold for lymphocytes

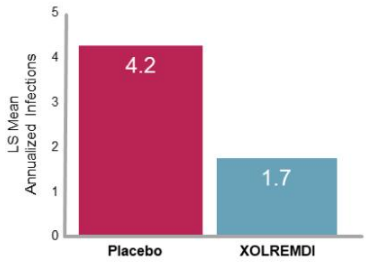


Severe lymphopenia threshold = 1000 cells/ µL

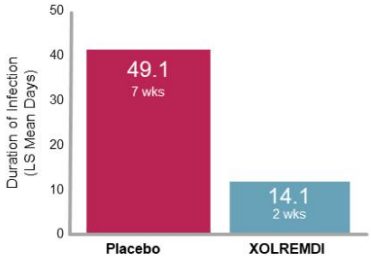
4WHIM: Patients Treated with XOLREMDI Experienced Improvements Across Infection Assessments over 52 Weeks versus Placebo[1]

Total infection score 40% lower for patients on XOLREMDI versus those on placebo

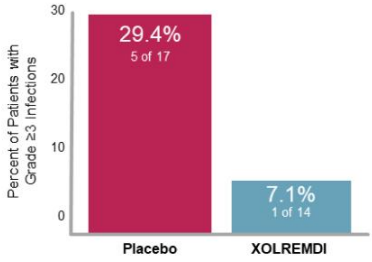
~60% reduction in annualized infection rate



On average, infection duration was 5 weeks shorter



Fewer patients with severe infection



No difference in wart change scores between XOLREMDI and placebo arms

1. Badolato R, et al. Blood. Published online April 21, 2024;blood.2023022658.

4WHIM: Treatment Generally Well Tolerated; Majority of Adverse Reactions Mild to Moderate in Severity

Adverse Reactions Section of Product Label[1]
(≥10% and at a frequency higher than placebo in 4WHIM)

Adverse Reaction	XOLREMDI (n=14)	Placebo (n=17)
Thrombocytopenia	3^	0
Pityriasis	2	0
Rash	2	0
Rhinitis	2	0
Epistaxis	2	1
Vomiting	2	1
Dizziness	2	1

^Serious adverse reactions of thrombocytopenia occurred in 3 of the 14 patients who received XOLREMDI, two of which occurred in the setting of infection or febrile neutropenia.

Warnings and Precautions: Embryo-fetal toxicity and QTc interval prolongation.

Published Phase 3 trial data results[2] showed:

- XOLREMDI (mavorixafor) was generally well tolerated in participants with WHIM syndrome
- No discontinuations occurred due to treatment-emergent adverse events (TEAEs), and none were deemed related to treatment
- No treatment-related serious TEAEs were observed

1. XOLREMDI package insert. Please see Important Safety Information and full Prescribing Information at www.xolremdi.com.
2. Badolato R, et al. Blood. Published online April 21, 2024;blood.2023022658.

Commercial Strategy Overview: Targeted Education, Engagement, and Access

Support Patient Diagnosis

- Educate on WHIM syndrome
- Provide diagnostic support
- Engage at key medical conferences

Establish XOLREMDI as Standard of Care in WHIM syndrome

- Target key hematologists & immunologists
- Communicate targeted MOA and clinical profile
- Drive adoption and uptake in appropriate patients

XOLREMDI™ (mavorixafor) capsules

Gain Broad Access

- Mitigate access barriers
- Provide full suite of patient support services
- Help patients throughout their treatment journey

X4Connect™

Leveraging an agile commercial team to execute X4's first product launch

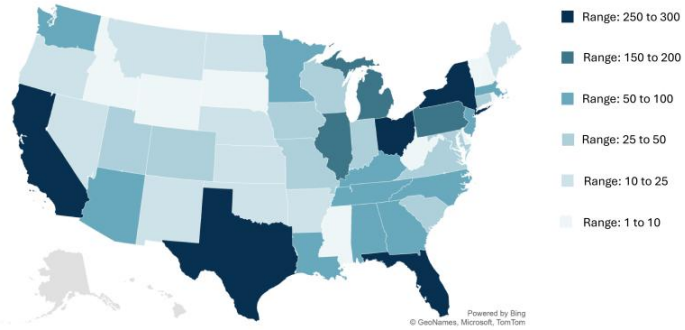
Targeted Approach to Covering the U.S. WHIM Market

Field team recruited from well known rare and ultra-rare organizations

Collectively more than 250 years of demonstrated success in commercial launches

Mission-driven, patient-centric: bringing a novel therapy to a historically underserved population

Refined Target List of ~3,500 HCPs
(primarily immunologists and hematologists)



- Focused engagement with ~20 top thought leaders
- Partnering with patient advocacy organizations

XOLREMDI Addressing High Unmet Need with Targeted Innovation

- Targeted Breakthrough therapy for ultra-rare patient population
- First and only FDA-approved therapy indicated for WHIM syndrome
- Demonstrated efficacy & safety profile
- Potential to address high burden of disease and strengthen relevant **aspects of patients' immune function**

XOLREMDI™ (mavorixafor) capsules

Annual Price* Reflects Value

- Patients >50 kg = 400 mg daily = $496,400 annually
- Patients ≤50 kg = 300 mg daily = $372,300 annually

Committed to Providing Innovative Solutions

Dedicated support and education available through X4Connect and PANTHERx Rare for all eligible patients

Helping unite the WHIM syndrome community through collaborations, targeted education, and support of earlier diagnosis

* Wholesale acquisition cost (WAC); assumes full compliance

Chronic Neutropenia: No Innovation in More Than 30 Years

~50,000[1]
U.S. Prevalence: total diagnosed with Chronic Neutropenia (CN)

~15,000[1]
Estimated subset with highest unmet need: minimal addressable market for mavorixafor in CN

Only One
Therapy Approved for Severe Chronic Neutropenia

Injectable Granulocyte Colony-Stimulating Factor (G-CSF)

- Inconvenient daily injections
- Frequent treatment-related/treatment-limiting bone pain and other adverse events

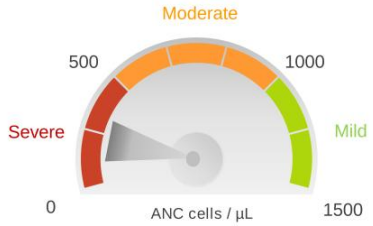
Clear Need for Increased Options for Patients: Efficacious, Oral, Well Tolerated Treatments

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

Living With Chronic Neutropenia (CN): Risk of Serious, Life-Threatening Infections

NIH Classification[1]	ANC Levels (cells/µL)	Infection Risk with Immunodeficiency[2]
Severe (Grade 4)	<500	Moderate to severe
Moderate (Grade 3)	500-1,000	Moderate to severe
Mild (Grade 2)	1,000-1,500	Minimal to severe
Non-clinical (Grade 1)	1,500 - LLN	No clinical impact

Infection Risk and Low ANC ("Neutropenia")



ANC = Absolute neutrophil count

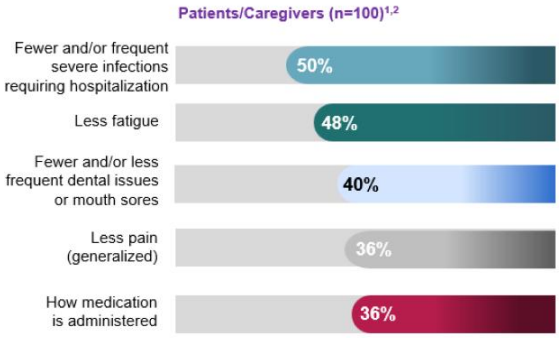
- Frequent and/or severe infections are the primary clinical consequence of chronic neutropenic disorders[3]
- Infection frequency, severity, and duration are correlated with magnitude and duration of decreased ANC levels[4]
- Infections may lead to frequent hospitalizations or result in life-threatening complications, including death[5,6]

1. https://ctep.cancer.gov/protocoldevelopment/electronic_applications/docs/ctcae_v5_quick_reference_8.5x11.pdf ; 2. Jan Palmblad, Carlo Dufour, Helen A. Papadaki, Haematologica, Vol. 99 No. 7 (2014): July, 2014; 3. Sicre de Fontbrune F, et al. Blood. 2015;126(14):1643-1650; 4.Bodey, GP, et al, Ann Intern Med, 1966. 5. Donadieu J, et al. Expert Rev Hematol. 2021;14(10):945-960; 6. Salehi T, et al. Iran J Allergy Asthma Immunol. 2012;11(1):51-56.

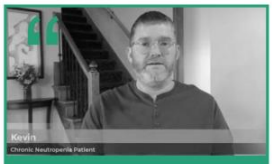
What Makes a Difference to Chronic Neutropenia Patients and Their Physicians?

Expanded treatment options, ideally:

- Reduced infection rates
- Oral formulation
- Good safety profile
- Alternate therapy to injectable G-CSF and/or
- Reduced G-CSF-dose & related toxicities



"There is a major diagnostic gap currently…, and also treatment options are extremely narrow"



"What I'd like to see with neutropenia would be a different way to administer the medicine… nobody likes needles."

"..an augmentation [of ANC] by 500 or 1000 would be adequate for clinical purposes of preventing infection."

".. I'm using Neupogen… I use it daily on a low dose… If I get the extreme bone pain…, I am unable to sleep…Yeah. It's unreal."

1. Ellis A, et al. poster presented at ASH Annual Meeting December 2022; 2. Other improvements included lower cost, fewer and/or less frequent short-term side effects from medication, fewer and/or less frequent gastrointestinal symptoms, fewer and/or less frequent long-term side effects from medication, and easier storage; respondents were allowed to select ≥1 options - total percentages may not add up to 100.

Assessing Mavorixafor in Six-Month CN Phase 2 Clinical Trial

Chronic daily dosing of mavorixafor +/- G-CSF

Mavorixafor: Same Oral Dosing as WHIM Phase 3

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

Primary Study Objectives:

Mavorixafor Monotherapy: Assess if mavorixafor raises ANC levels in neutropenic patients as a monotherapy

Mavorixafor + Stable-dose G-CSF: Assess safety in combination with G-CSF, increase and durability of ANC

Mavorixafor + G-CSF With Dose-Adjustments[1]: Assess safety in combination with G-CSF, increase and durability of ANC, and potential for G-CSF dose-reductions in selected patients

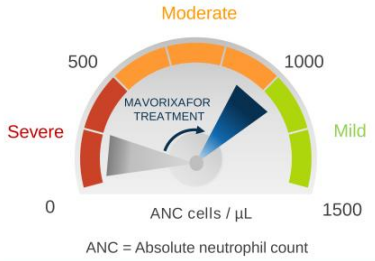
Safety of chronic mavorixafor therapy +/- G-CSF

1. Modifications in G-CSF allowed after month 2 at doctor's discretion.

Mavorixafor: Potential to Deliver First Innovation in CN in 30+ Years

Successful WHIM Phase 3 Clinical Trial Results Guide Success Factors in CN

Significant Increase in ANC Over 52 Weeks = ~60% Reduction in Annualized Infection Rate



ANC = Absolute neutrophil count

Positive Phase 1 & 2 CN Data to Date Support Advancement to Phase 3

CN Phase 1b Results
- ANC increased by >500 cells/µL in 100% (n=25) after single dose

Early CN Phase 2 Results
- Durable increases in ANC[1] in all subjects reported to date (n=3)
  - Clinically meaningful (Δ ANC >500 cells/µL)
- Infection rates
  - No infections in all patients after month 2
  - No increase in infections despite reduction/withdrawal of G-CSF (n=2)

1. All subjects increased absolute neutrophil count (ANC) over first 3 months of dosing (when G-CSF maintained at fixed, baseline dose)

CN Clinical Data to Date Support Advancing Mavorixafor into Phase 3 Trial

Overall learnings

- ✓ First supporting evidence that mavorixafor treatment durably increases ANC
- ✓ Levels of observed ANC increase (Δ ANC ≥ 500) correlate with infection risk reduction
- ✓ Safety profile to date supports chronic treatment with mavorixafor
- ✓ Preliminary data support responder criteria used as primary endpoint in planned CN Phase 3

Mavorixafor delivered on patient needs

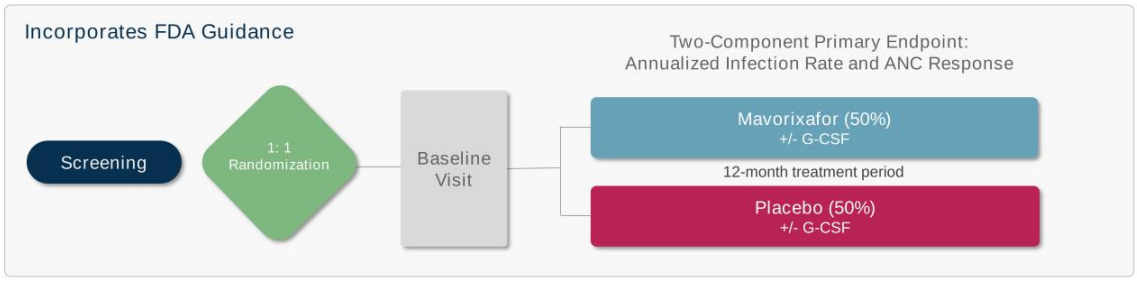
- ✓ Neutropenic participants achieved normalized ANCs
- ✓ G-CSF could be reduced meaningfully (50% or more)
- ✓ No additional adverse effects seen to date in combination with G-CSF

Phase 2 CN Trial Ongoing

- >20 participants enrolled in trial – 40% on mavorixafor monotherapy
- Interim Phase 2 data expected in late June 2024 (n>15)
- Full data set expected in late 2024

Data Across Multiple Studies to Date + Input from FDA Informed Pivotal, Global Phase 3 Trial Design

CN Pivotal, Global Phase 3 Trial Expected to Initiate in 2Q 2024



Key Inclusion Criteria:

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

Design: double-blinded, randomized, placebo-controlled on top of standard of care (+/- G-CSF[1]); same mavorixafor dosing as 4WHIM trial

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

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

1. 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.

Continuing to Deliver Progress for Patients

XOLREMDI™ (mavorixafor) capsules

- **U.S. Approval & Launch for WHIM syndrome** April 2024
  - Priority Review Voucher monetized
- Laying a strong foundation for XOLREMDI U.S. sales & market growth
- Additional Phase 2 CN trial data expected in late June 2024
- Initiation of pivotal Phase 3 CN trial anticipated in 2Q 2024
- Potential pipeline and geographic expansion opportunities

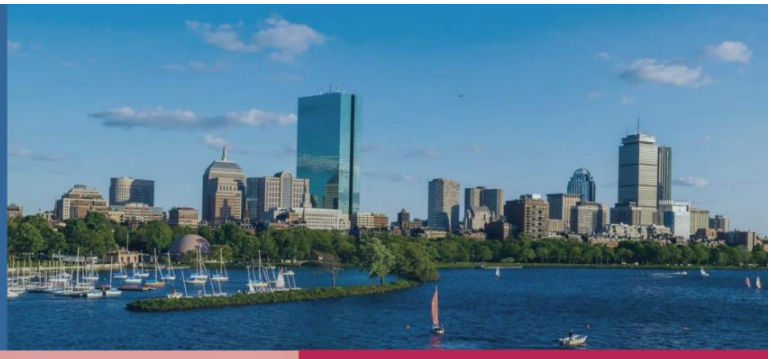
Potential Market Opportunities

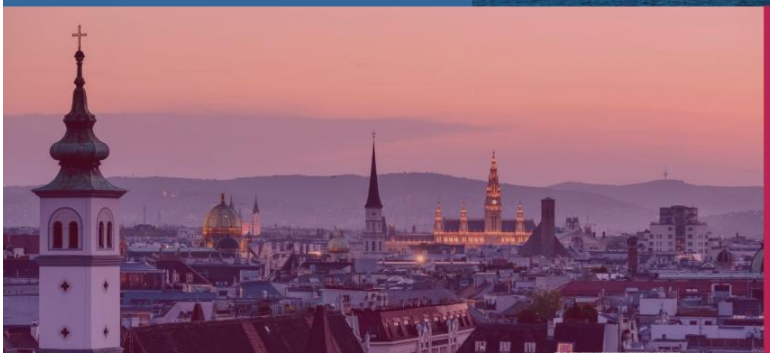
WHIM
~1,000 U.S. patients

Chronic Neutropenia
>15,000 U.S. patients

U.S. Headquarters
61 North Beacon Street, 4th Floor
Boston, MA 02134

NASDAQ: XFOR





Research Center of Excellence
Helmut-Qualtinger-Gasse 2
A-1030 Vienna, Austria

www.x4pharma.com

Seasoned Executive Leadership Team

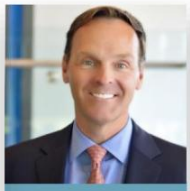
Experienced in research, development, & commercialization of first-in-class, innovative therapies

PAULA RAGAN, Ph.D.
President & CEO

SANOFI
genzyme

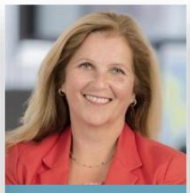
CHRISTOPHE ARBET-ENGELS, M.D., Ph.D.
Chief Medical Officer

MILLENDO THERAPEUTICS
Biogen



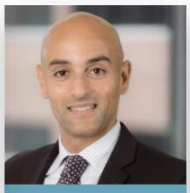
MARK BALDRY
Chief Commercial Officer

Amicus Therapeutics
Biogen



MARY DIBIASE, Ph.D.
Chief Operating Officer

Biogen
EPIRUS BIOPHARMACEUTICALS



ADAM MOSTAFA
Chief Financial Officer

abpro
CANTOR Fitzgerald

ART TAVERAS, Ph.D.
Chief Scientific Officer

Schering-Plough
Biogen

Strong Balance Sheet Supports Expected Upcoming Milestones

~$207 million[1]

Funds expected to support operations into late 2025[2]

Top-tier Life Science-Focused Institutional Shareholder Base

Analyst Coverage

B. RILEY FBR | BROOKLINE CAPITAL MARKETS | CANTOR Fitzgerald | PIPER SANDLER | STIFEL | HCW H.C. WAINWRIGHT & CO.

1. 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); 2. Projected runway excludes any potential U.S. sales of XOLREMDI.