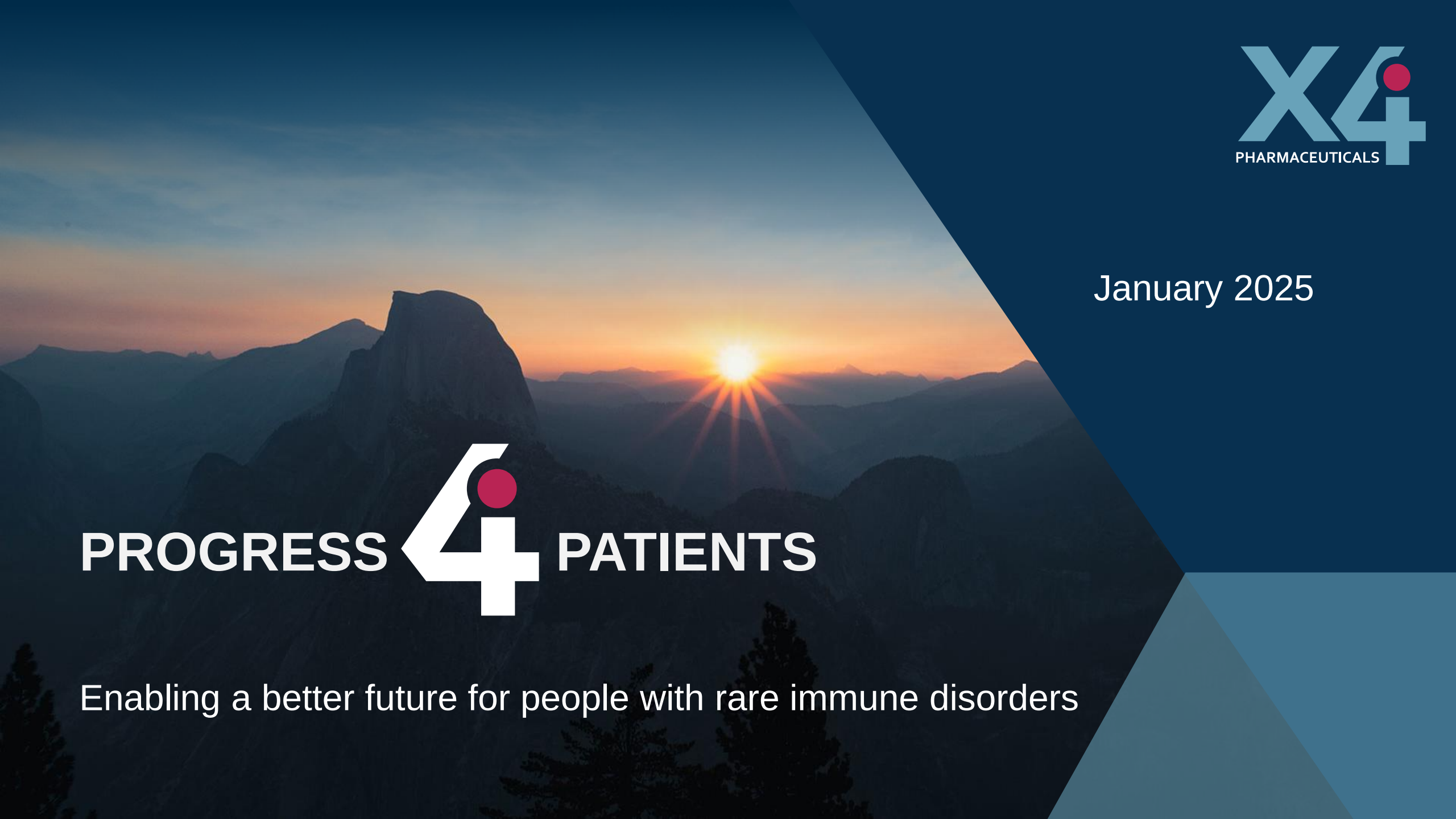

X4 PHARMACEUTICALS

January 2025

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 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 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 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; the timing and anticipated interactions with regulatory authorities and any related approvals for mavorixafor in Europe, Australia, and New Zealand; the potential market opportunity for mavorixafor; the anticipated strategic benefits of X4’s exclusive licensing agreement with Norgine and of any current or future collaborations; X4’s use of capital and other financial results; 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 or may not have satisfactory outcomes; 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, 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; the regulatory review and approval processes of the FDA and comparable foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable, and if X4 is ultimately unable to obtain regulatory approval for X4’s product candidates, including additional indications for mavorixafor, X4’s business will be substantially harmed; 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 may arise from the testing or use of our product and product candidates; general macroeconomic and geopolitical conditions that 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 may arise; 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 November 13, 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.

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X4's Momentum Addressing Unmet Needs in Rare Immune Disorders

Fully integrated company delivering on the promise of mavorixafor

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]

- U.S. launch ongoing with **patients on commercial product** and target physician engagement on track
- Disease awareness campaign bearing fruit, with **knowledge of and screening for WHIM increasing**

Partnership with Norgine to commercialize in Europe, Australia and New Zealand

- EU MAA submission for WHIM expected shortly

BALANCE SHEET SUPPORTS CONTINUED GROWTH

- Funds of $136 million as of 9/30/2024
  - Additional ~$30M (€28.5M) in non-dilutive cash from Norgine agreement
- Balance sheet expected to fund operations into late 2025[2]

NEXT VALUE DRIVER: MAVORIXAFOR IN CHRONIC NEUTROPENIA

- Successful Phase 2 results in CN derisk ongoing pivotal 4WARD Phase 3 clinical trial
- **4WARD expected to fully enroll in mid-2025**

1. WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis); 2. Projected runway excludes any potential U.S. sales of XOLREMDI.

X4 and Norgine Enter into Exclusive Licensing Agreement to Commercialize Mavorixafor in Europe, Australia, and New Zealand – January 2025

Maximizing the global potential of mavorixafor through strategic partnership

- Leverages Norgine's existing infrastructure and successful track record in commercializing specialty pharmaceuticals
- Companies will coordinate closely on regulatory filings in multiple geographies and indications
- X4 remains responsible for ongoing pivotal 4WARD Phase 3 clinical trial evaluating mavorixafor in CN
- Norgine responsible for all market access and commercialization activities
- X4 to manufacture and supply mavorixafor to Norgine

€28.5 million non-dilutive upfront payment

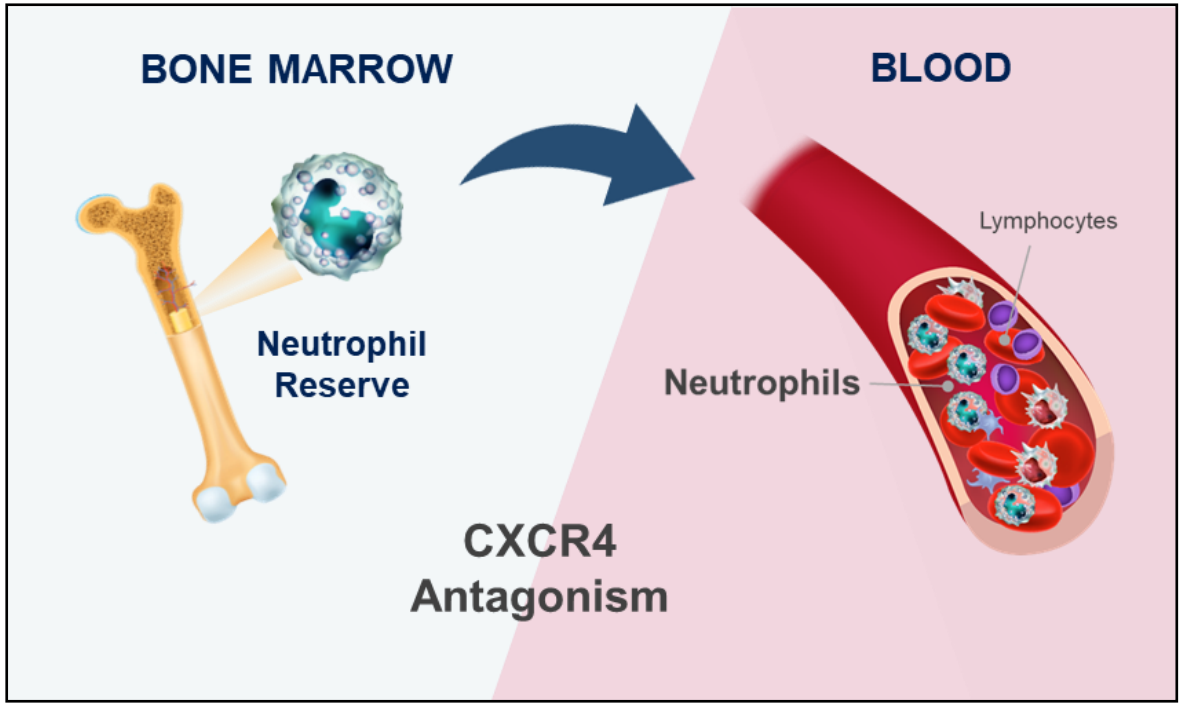
Up to €226 million in potential regulatory and commercial milestone payments

Tiered, double-digit royalties on net sales up to the mid-twenties

Breaking News!

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 at the 2022 annual meeting of the American Society of Hematology.

Advancing Innovation for Patients

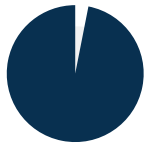
Only oral agent targeting rare immunodeficiencies

	Indication	Pre-clinical	Phase 1	Phase 2	Phase 3	FDA Approved	EXPECTED MILESTONES
XOLREMDI® (mavorixafor) capsules	**WHIM Syndrome** (Warts, Hypogammaglobulinemia, Infections, Myelokathexis)			Approved in U.S. April 2024			Progress on U.S. commercialization **EU MAA submission by early 2025**
Mavorixafor	**Chronic Neutropenia** (Congenital, Autoimmune, Idiopathic)			Phase 3 Trial Ongoing			**Full enrollment in global 4WARD trial expected in mid-2025**
X4P-003	TBD						

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

Heterogeneous presentation of symptoms caused by CXCR4 dysfunction[2]

Most frequently characterized by:



Neutropenia (98%)

Hypogammaglobulinemia (65%)

Recurrent infections (92%)

Warts (40%)

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.

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.

U.S. Launch in May 2024

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

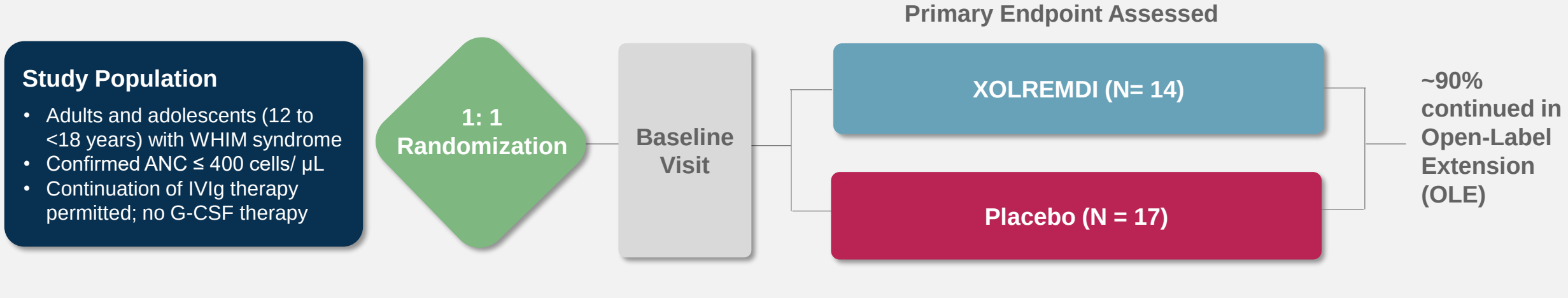
XOLREMDI®

(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

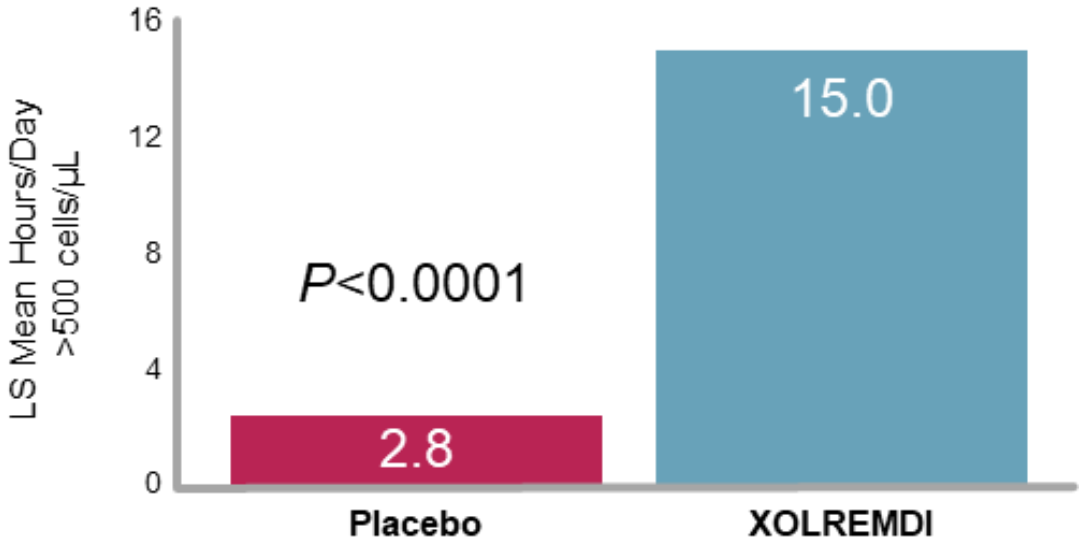
- Improvement in absolute lymphocyte count (ALC) as measured by the mean time above ALC threshold of 1000 cells/µL at 13, 26, 39, and 52 weeks
- Composite endpoint: Analysis of total infection score (rate, severity) and total wart change score

1. Badolato R, et al., A phase 3 randomized trial of mavorixafor, a CXCR4 antagonist, for WHIM syndrome, *Blood* (2024) 144 (1): 35–45 (https://doi.org/10.1182/blood.2023022658)

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

Primary endpoint

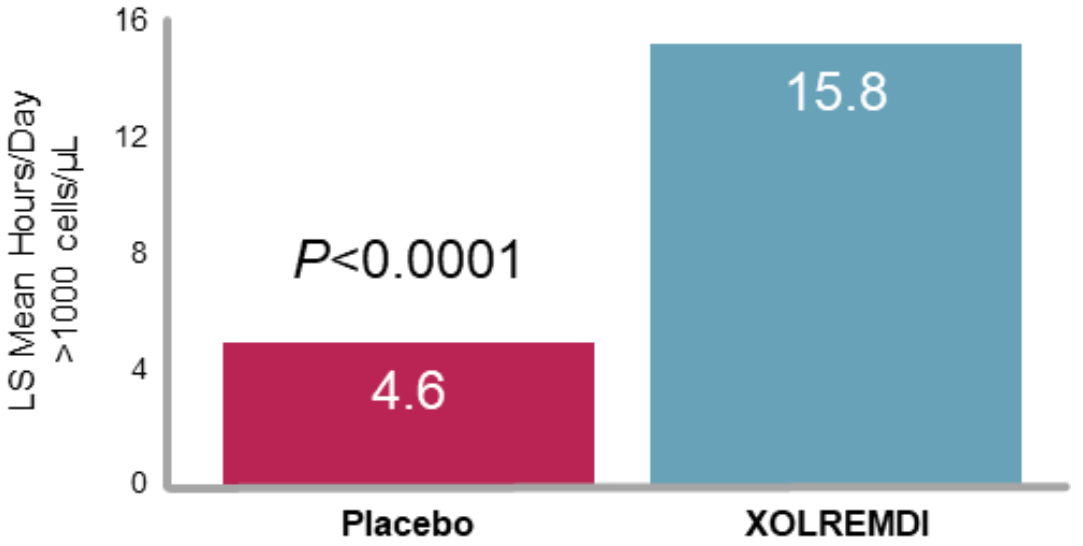
Significantly increased mean hours per day above the threshold for neutrophils



Severe neutropenia threshold = 500 cells/µL

Key secondary endpoint

Significantly increased mean hours per day above the threshold for lymphocytes



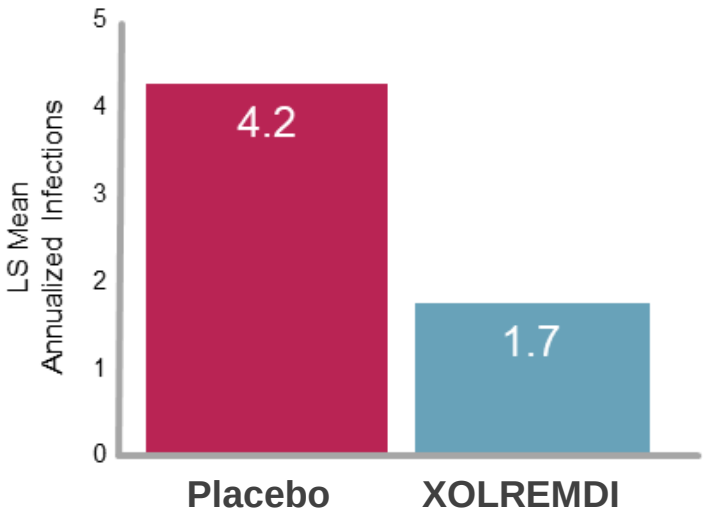
Severe lymphopenia threshold = 1000 cells/µL

4WHIM: ANC Increase Resulted in Clinical Infection Benefits[1,2]

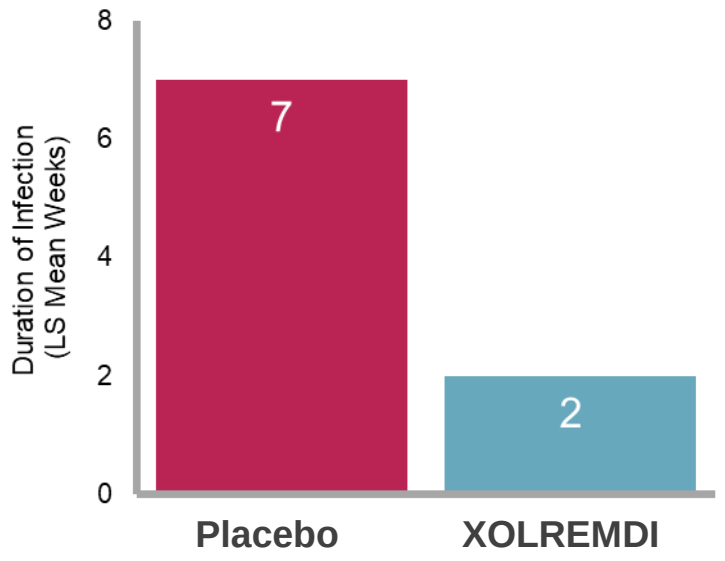
Mean ANC increases of >500 cells/µL reduced infection rate, duration, and severity

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

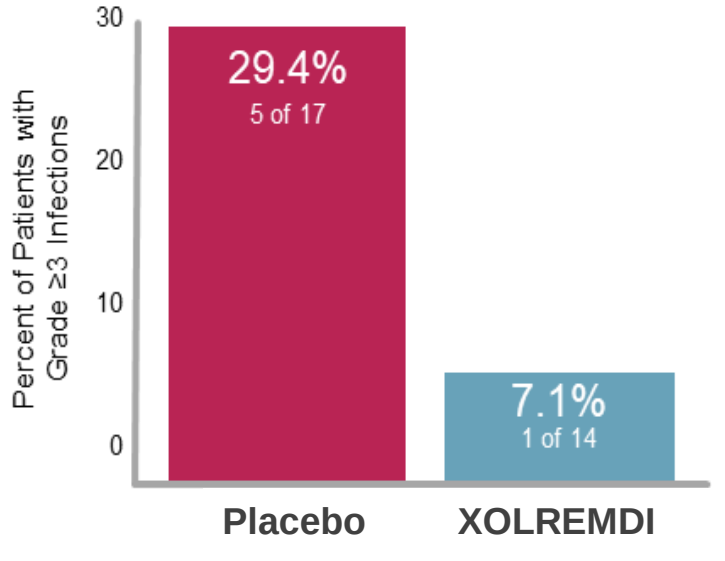
~60% reduction in annualized infection rate



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* (2024) 144 (1): 35–45. 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).

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

Addressing High Unmet Need with Targeted Innovation

XOLREMDI® (mavorixafor) capsules

- First and only FDA-approved therapy indicated for WHIM syndrome
- Targets the underlying cause of WHIM syndrome via CXCR4 antagonism
- Demonstrated efficacy & safety profile with oral formulation
- Potential to address high burden of disease and strengthen patients' immune function

WHIM syndrome diagnosis and support resources

Supporting Patient Diagnosis

- Educating on WHIM syndrome
- Providing diagnostic support
- Engaging at key medical conferences

Establishing XOLREMDI as Standard of Care in WHIM syndrome

- Targeting key hematologists & immunologists
- Communicating targeted MOA and clinical profile
- Driving adoption and uptake

Gaining Broad Access

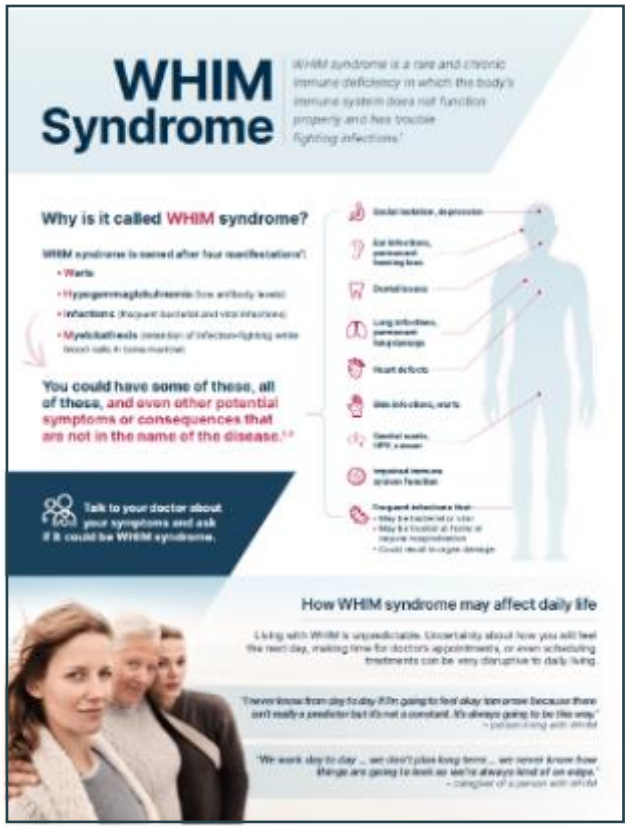
- Mitigating access barriers
- Providing full suite of patient support services

X4Connect™

XOLREMDI® U.S. Launch Update – November 2024

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[1]

- **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

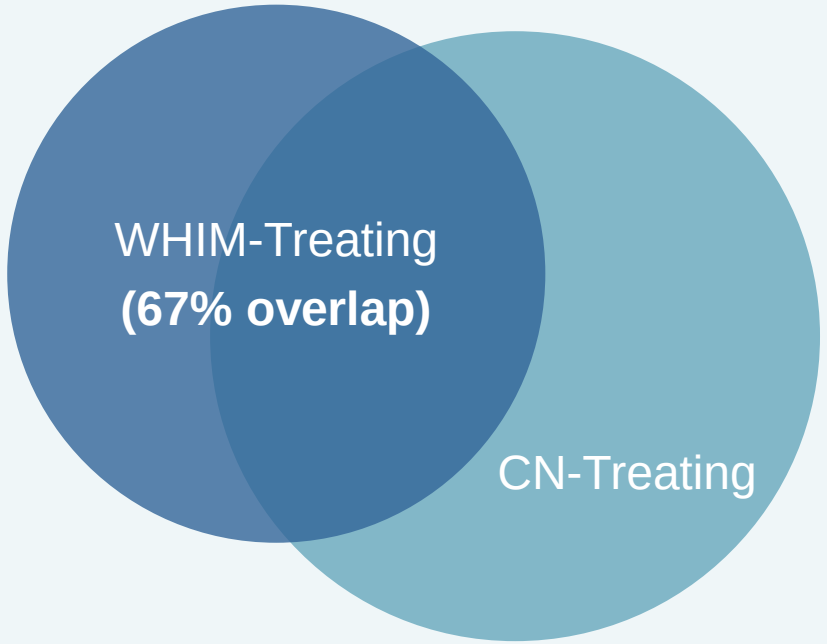
Recent Tracking Study of Likely XOLREMDI Prescribers[2]

- **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. HCPs (healthcare practitioners) reached through in-person and digital engagement; 2. 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.

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: minimum addressable market for mavorixafor in CN

Only One

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 other adverse events, and long-term risk of myelodysplastic syndrome and/or leukemia**

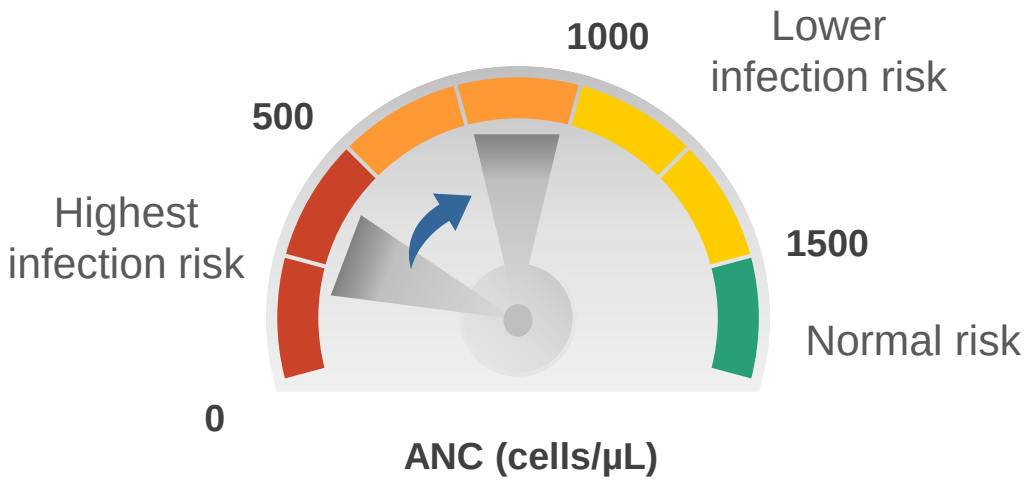
Innovation needed to address unmet patient needs

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 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.

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

"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

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).

Successful Phase 2 Study of Mavorixafor in Chronic Neutropenia

Phase 2 Study Enrolled a Total of 23 Participants

Assessed Safety and Durability of ANC Levels over 6-Month Period[1]

Participant Disposition (n=23)	
Type of CN	
Idiopathic	15
Congenital[3]	6
Cyclic	2
Sex	
Male	10
Female	13
Mean Age	34

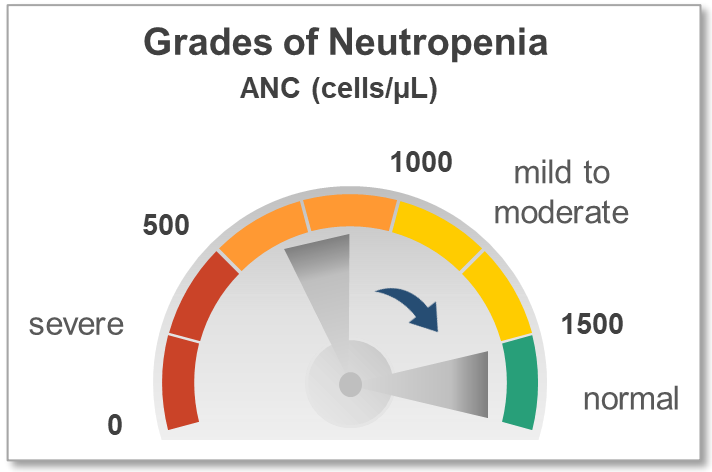
Mavorixafor Monotherapy	Baseline
Total	10

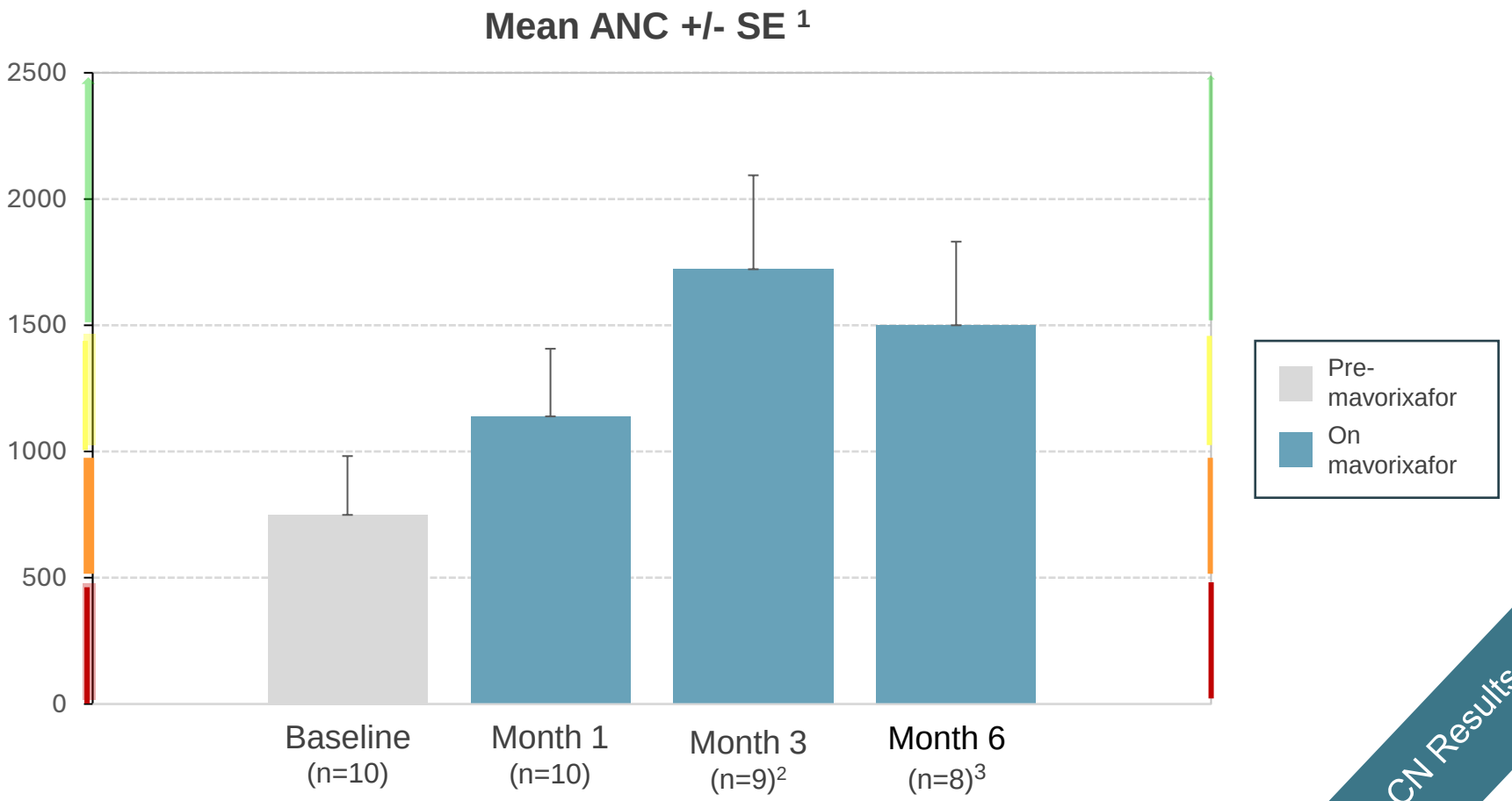
Mavorixafor + G-CSF	Baseline
Stable G-CSF	4
Adjusted G-CSF[4]	9

1. The neutrophil life-cycle is 10-14 days (https://doi.org/10.3389/fimmu.2021.766620); Phase 2 ANC measurements over 6 months assess bone marrow status and durability of neutrophil production.
2. ANC assessments (6 blood draws over 8 hours) at baseline and 1, 3, and 6 months; 3. 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); 4. Modifications to G-CSF dosing allowed after Month 2 at physician's discretion.

Mavorixafor Monotherapy Durably and Meaningfully Increased Mean ANC

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



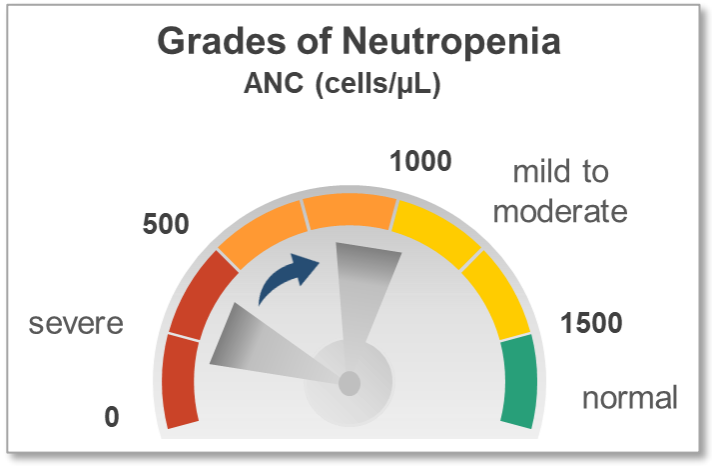


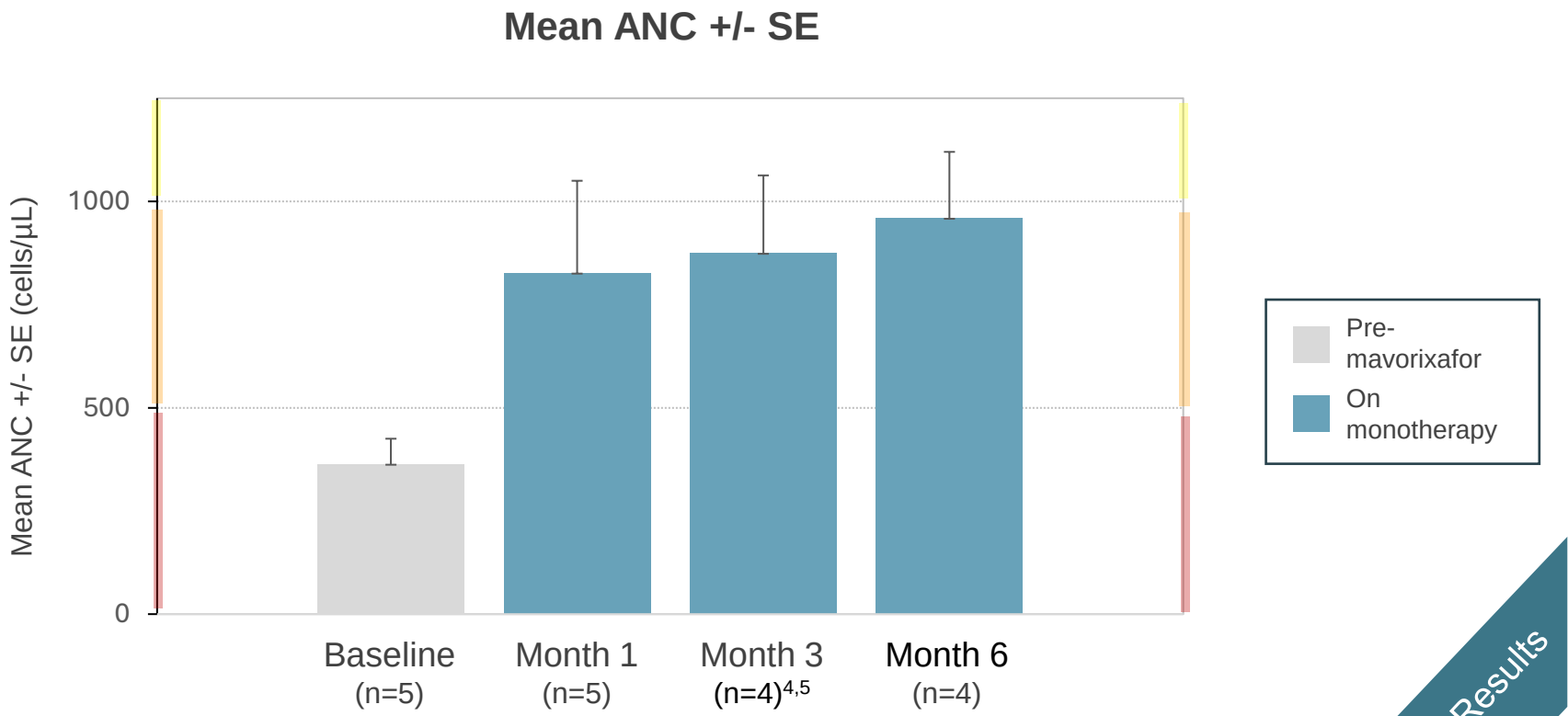
1. Data set contains two LOCF (last observation carried forward) values: one value missing at M3 assessment, one value missing at M6. 2. One patient discontinued prior to Month 3 assessment (no change from data set presented on 6/27/2024). 3. One patient discontinued prior to Month 6 assessment (no change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

Phase 2 CN Results

Mavorixafor Monotherapy Durably and Meaningfully Increased Mean ANC in Severe CN

- 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



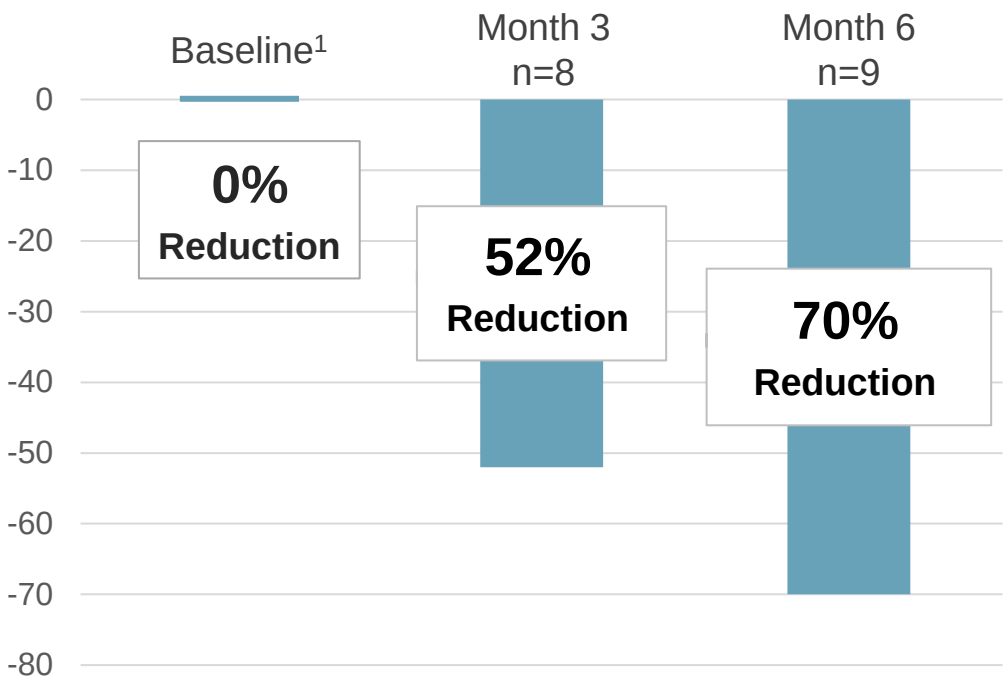


Phase 2 CN Results

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 Substantially Reduced G-CSF, Maintaining Normal Mean ANC

Mean G-CSF Reduction Over Time



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

Key Takeaways

G-CSF:

- Given the option, physicians chose to substantially reduce injectable G-CSF therapy in 9 of 12 (75%) eligible[1] patients
- 89% (8 of the 9) had G-CSF adjusted at earliest possible timepoint (following Month 2 visit)
- **33% (3 of 9) of participants with dose adjustments taken completely off G-CSF prior to Month 6 visit**
- Potential to improve patients' quality of life and lower long-term risk of malignancy from chronic G-CSF use

ANC:

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

Phase 2 CN Results

1. One participant discontinued prior to M1 assessment.

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

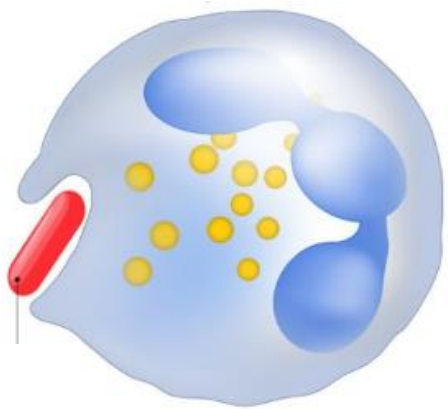
Purpose: **Demonstrate functionality of neutrophils in blood of individuals with CN, including those with congenital CN and genetic variants associated with neutrophil maturation arrest**

Neutrophil Functionality Assays[1]

Phagocytosis[2] (data to follow)

Assessment of neutrophils' ability to engulf pathogens

Pathogen such as *E. coli*



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**

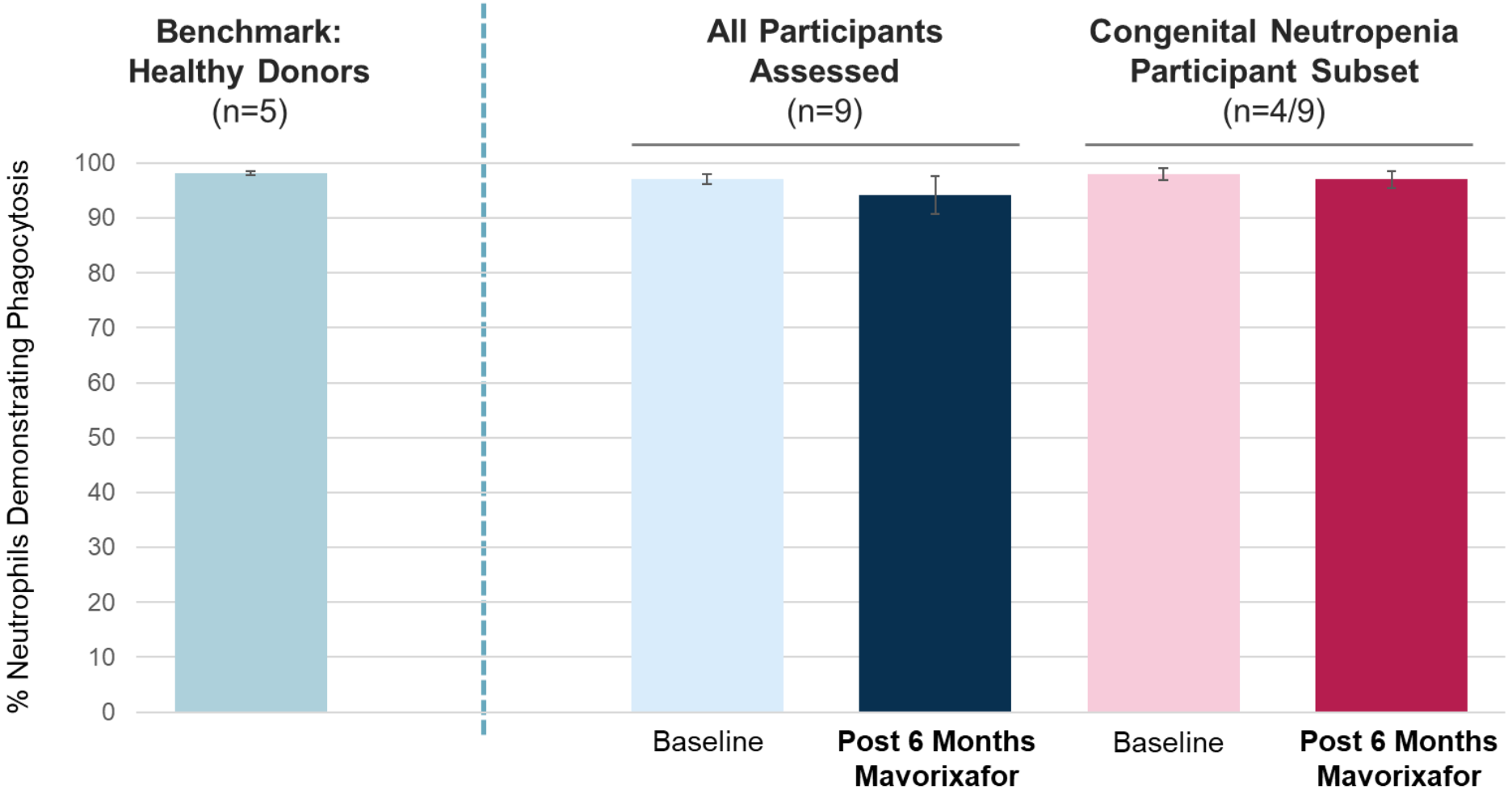
Neutrophil function studies assessed *ex vivo* blood neutrophil responses to bacterial challenge (opsonized *E. coli*) from clinical samples drawn from participants during the study.

Phase 2 CN Results

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.

Neutrophil Functionality Comparable to Heathy Donors Pre- and Post-Mavorixafor

Meaningful increases in circulating functional neutrophils expected to reduce infection risk



Mean percentage of functional neutrophils remained comparable to healthy donor controls prior to and following 6 months of mavorixafor treatment

Notes: Samples assessed for neutrophil functionality were limited by proximity to validated testing facility – complete data were available for 9 of the 23 enrolled Phase 2 study participants; ROS results for all subjects demonstrate similar profiles to phagocytosis and idiopathic CN subjects had similar results as those with congenital neutropenia (data on file)

Phase 2 CN Results

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 Ongoing Phase 3 4WARD 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

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

Phase 2 CN Results

4WARD Phase 3 Trial On Track to Fully Enroll in Mid-2025 – November 2024 Update

~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[1] and annualized infection rate

1. Discussions ongoing with U.S. FDA on finalization of ANC response endpoint. For more on the 4WARD trial: NCT06056297

Continuing to Deliver Progress for Patients

U.S. launch of XOLREMDI ongoing

XOLREMDI® (mavorixafor) capsules

Global, pivotal 4WARD Phase 3 CN trial initiated

4WARD A CHRONIC NEUTROPENIA STUDY

Positive Phase 2 CN data derisk ongoing 4WARD trial

2025 Expected Milestones

Ex-U.S. partner secured (Europe and ANZ)

EU MAA WHIM submission expected shortly

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

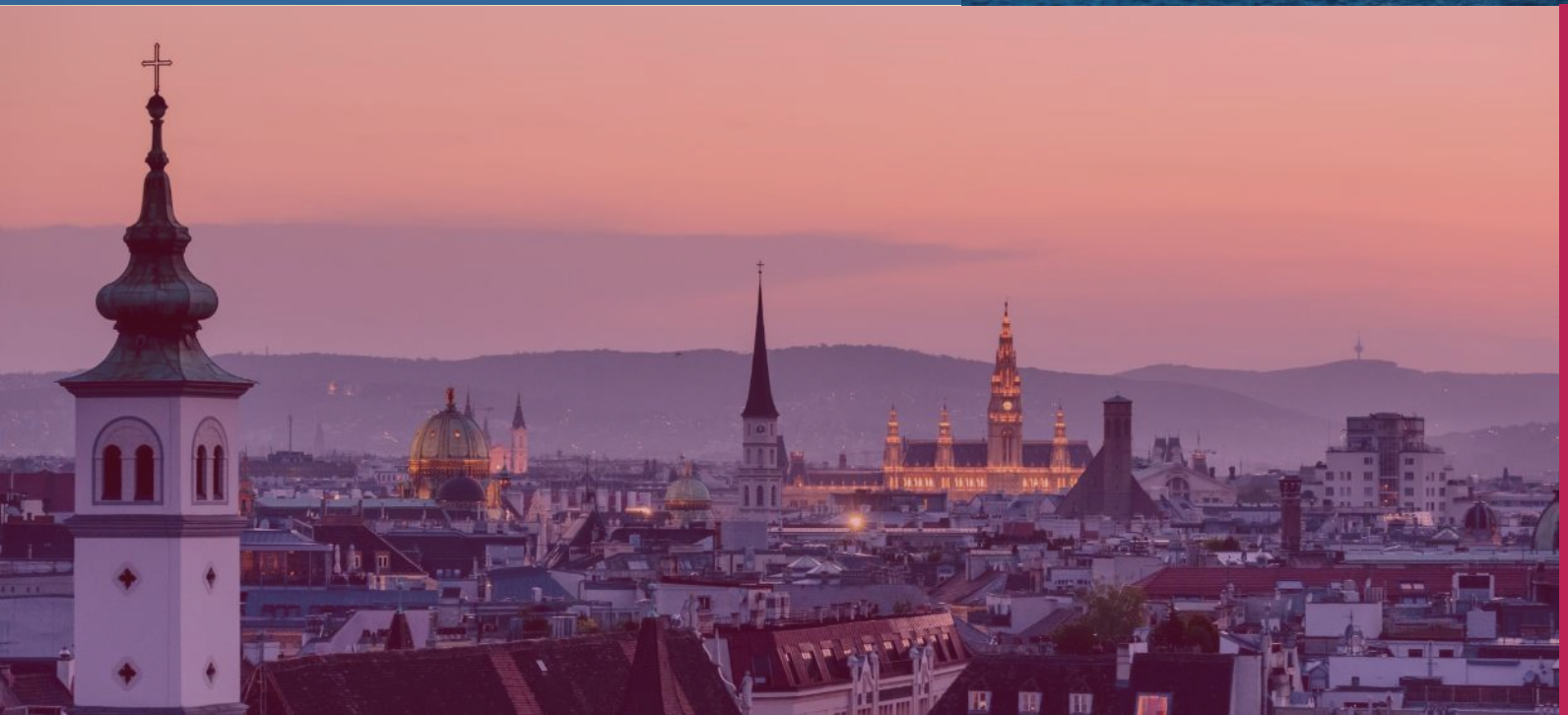
+

U.S. Headquarters

61 North Beacon Street, 4th Floor

Boston, MA 02134

NASDAQ: XFOR



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Balance Sheet Supports Expected Upcoming Milestones

$136 million[1]

Funds expected to support operations into late 2025[2]

Additional ~$30 million in non-dilutive funds received in January 2025 from ex-U.S. partnership[3]

Top-tier Life Science-Focused Institutional Shareholder Base

Analyst Coverage

BROOKLINE CAPITAL MARKETS

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1. Funds as of September 30, 2024; 2. Projected runway excludes any potential U.S. sales of XOLREMDI; 3. Exclusive licensing and supply agreement completed with Norgine to commercialize mavorixafor in Europe, Australia, and New Zealand (announced January 13, 2025).