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

November 13, 2024

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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

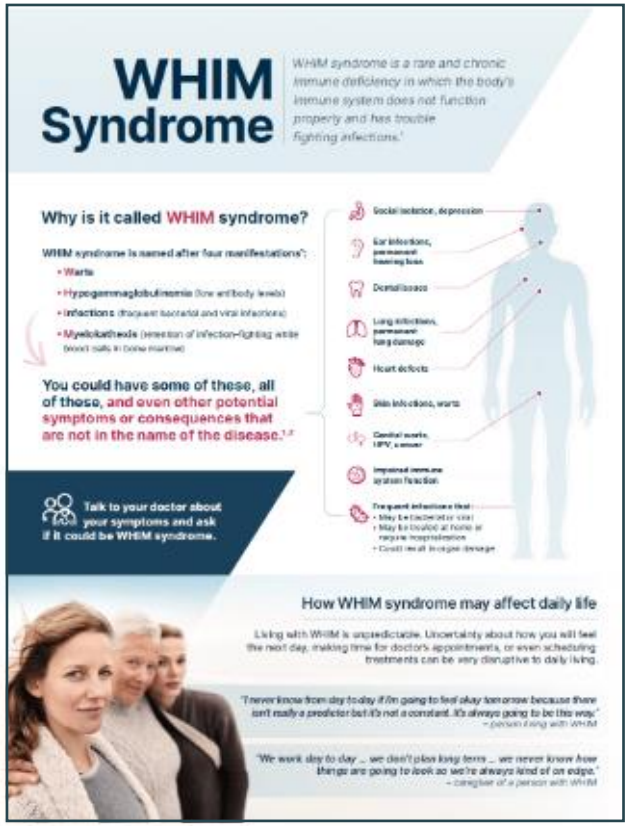
XOLREMDI® Launch and Phase 3 Chronic Neutropenia Clinical Trial Update

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

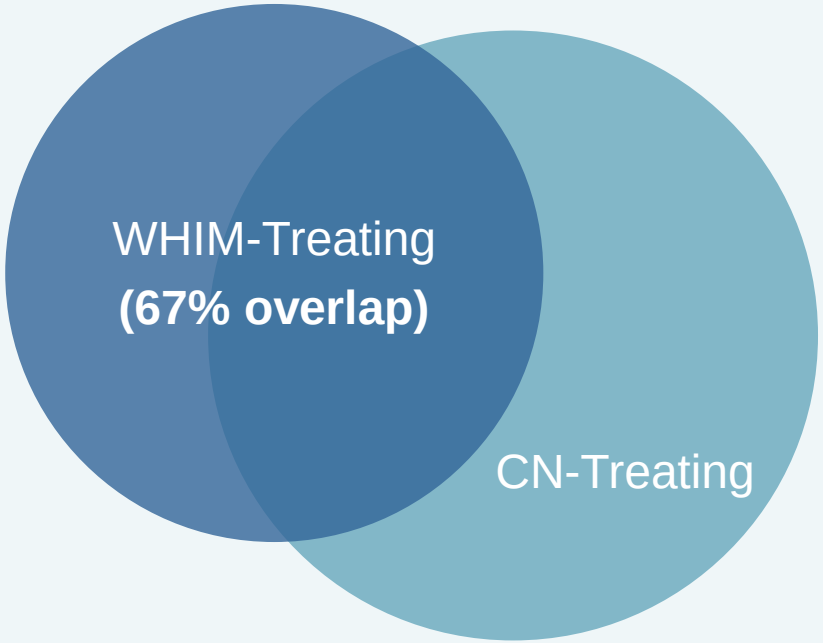
Recent Tracking Study of Likely XOLREMDI Prescribers[3]

- **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

For more on 4WARD trial: NCT06056297

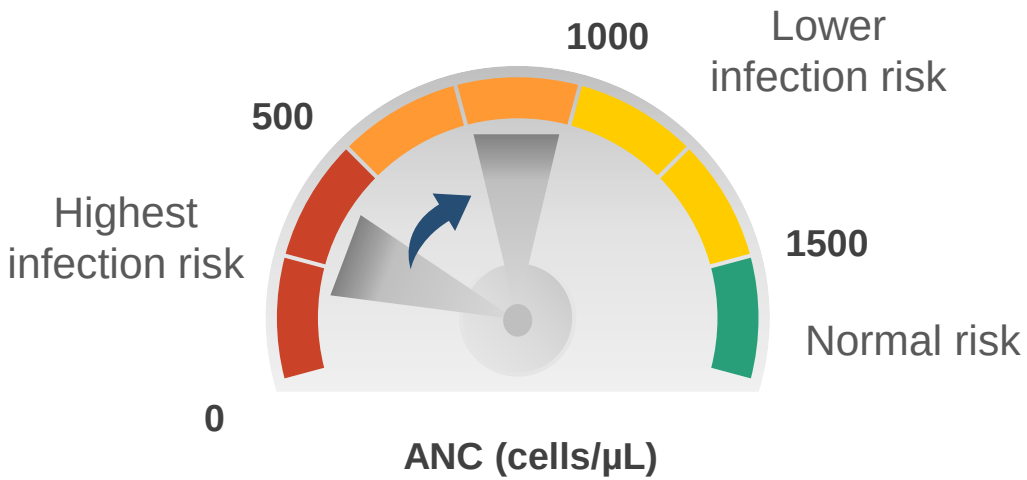
Phase 2 Chronic Neutropenia Study Results

X4 PHARMACEUTICALS

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)

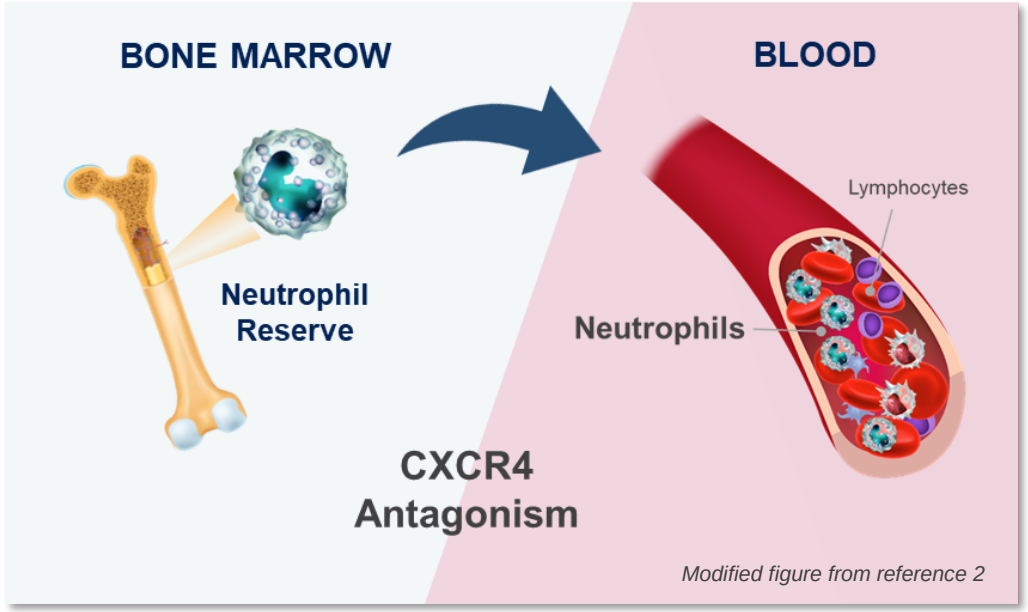
Increasing Neutrophil Counts >500 cells/µL Clinically Meaningful[6,7,8]



- 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

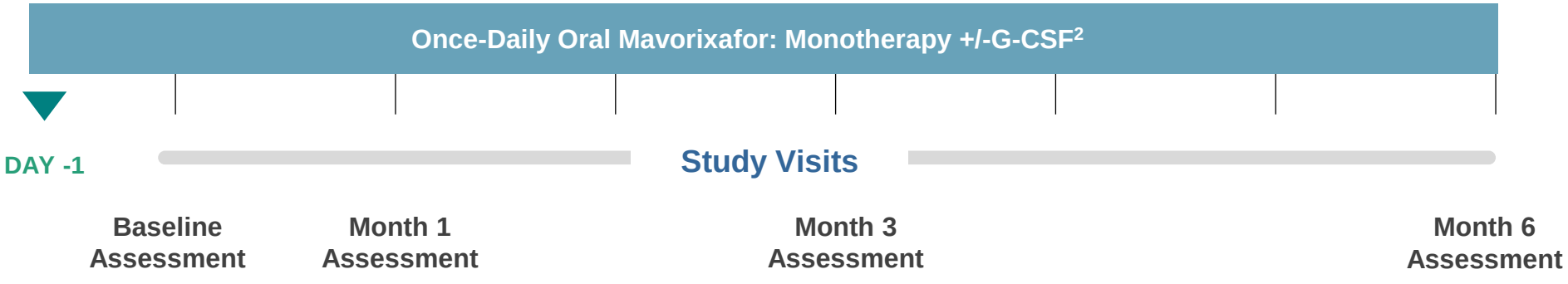
1. X4 Market Research, July 2023 – data on file; ICD-10 Code Research (2017-2023).

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.

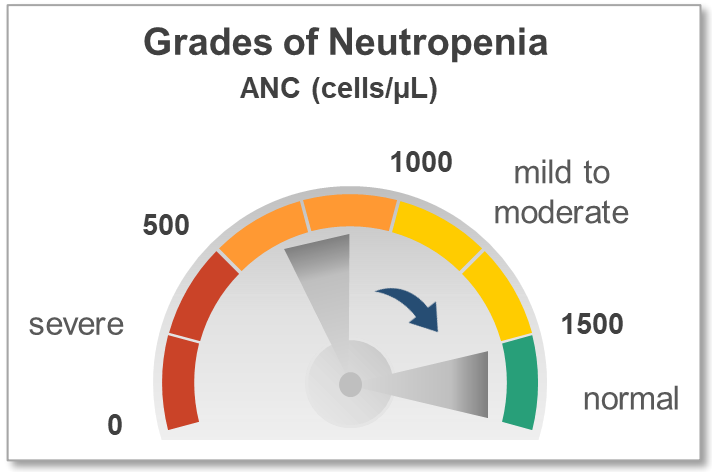
Questions Addressed Today from Phase 2 CN Study Results

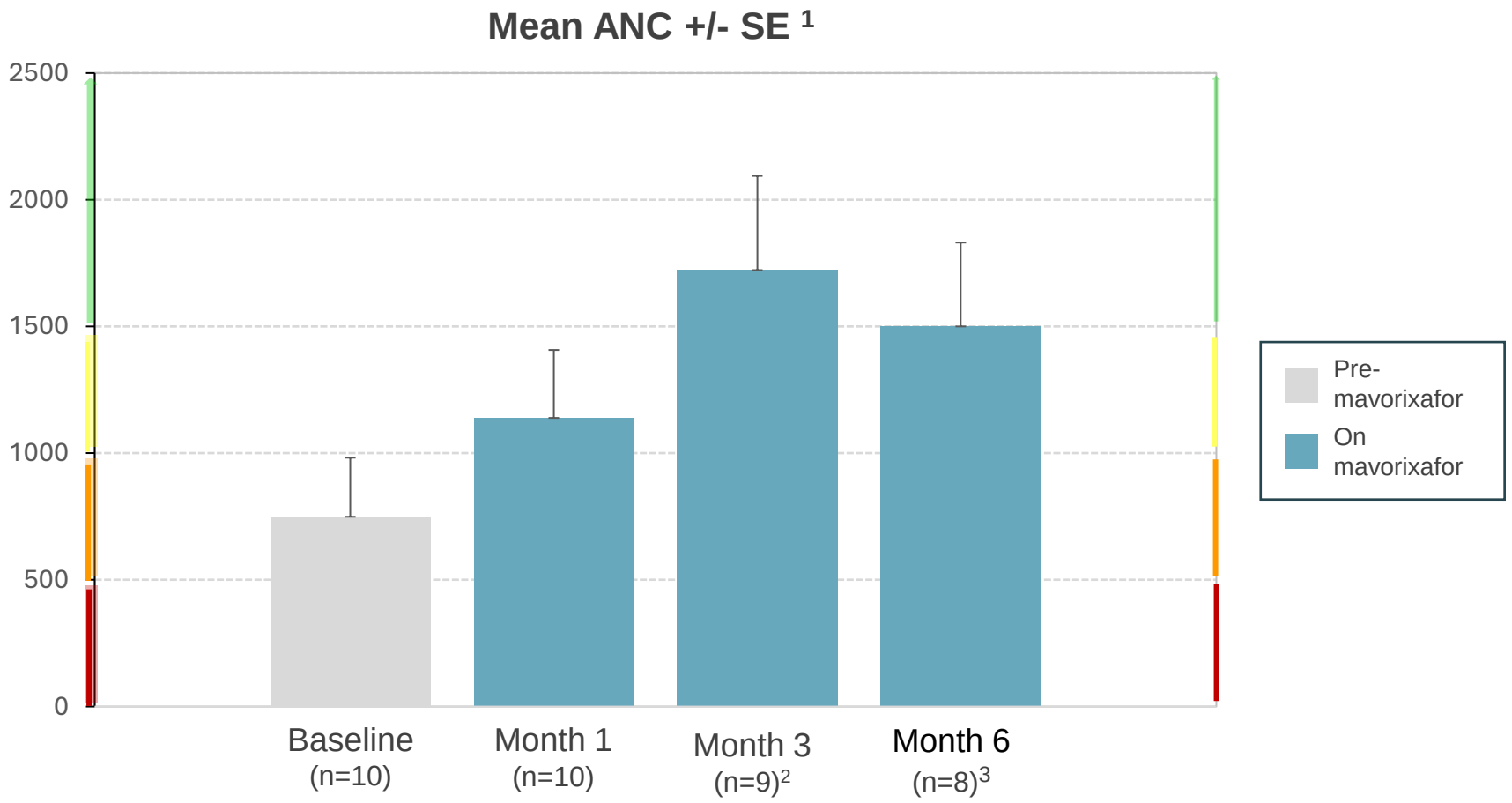
Phase 2 CN Study Population	Key Questions
Mavorixafor Monotherapy	• Does mavorixafor monotherapy durably sustain ANC at clinically meaningful levels?
NEW **Mavorixafor + Adjusted-Dose G-CSF**	• Are physicians and patients willing and able to adjust G-CSF with mavorixafor treatment? • Can G-CSF be reduced 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



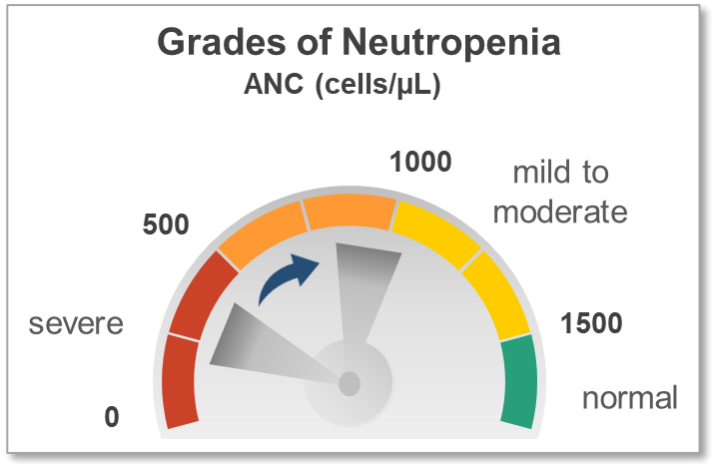


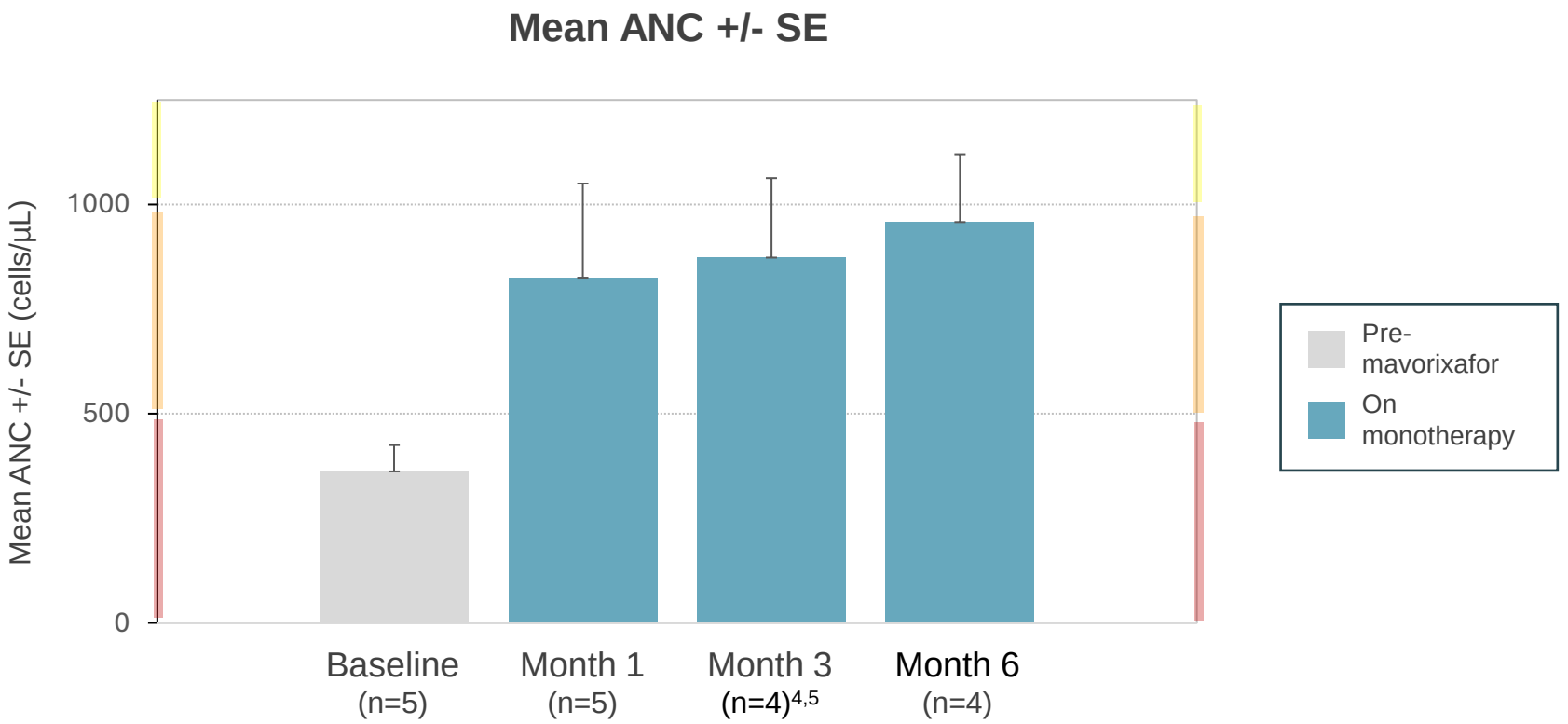
1. Data set contains two LOCF (last observation carried forward) values: one value missing at M3 assessment, one value missing at M6. 2. One patient discontinued prior to Month 3 assessment (no change from data set presented on June 27, 2024). 3. One patient discontinued prior to Month 6 assessment (no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

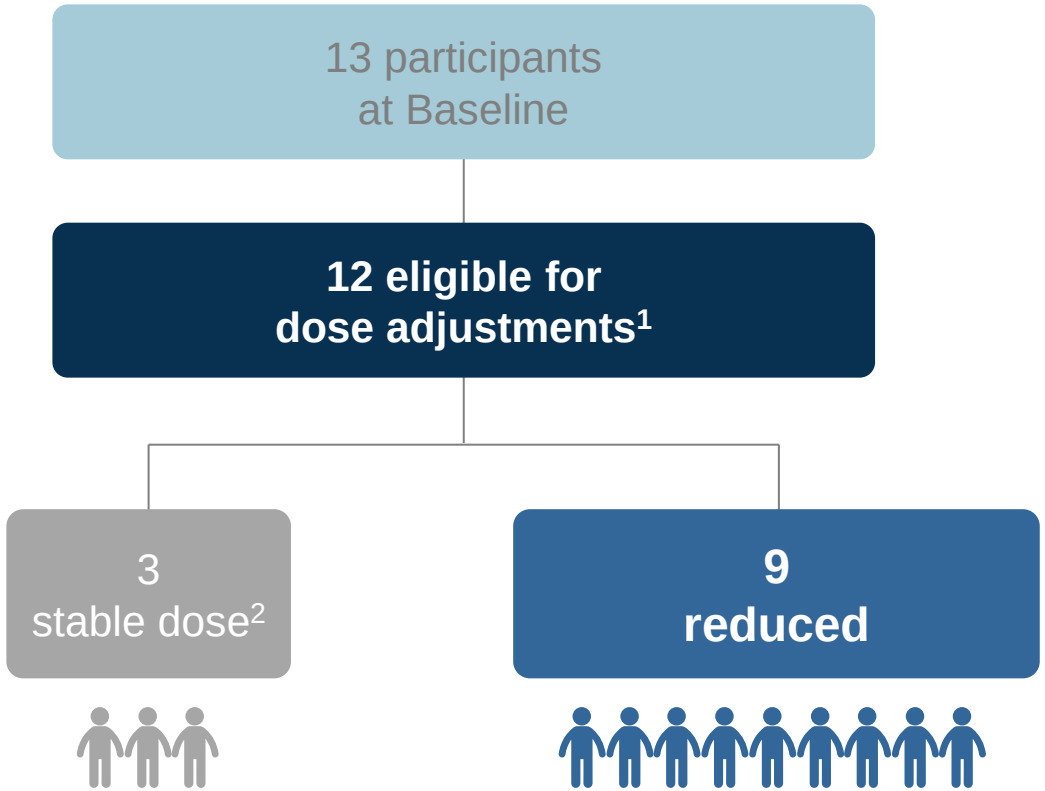




1. Platzbecker, U, et al. *Blood*. 2019 Mar;133(10):1020-1030. 2. Donadieu J, et al. *Expert Rev Hematol*. 2021 Oct;14(10):945-960. 3. Newburger PE, et al. *Seminars in Hematology* 2013 Jul;50(3):198-206. 4. Data set contains one LOCF (last observation carried forward) value, due to missing ANC at M3. 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

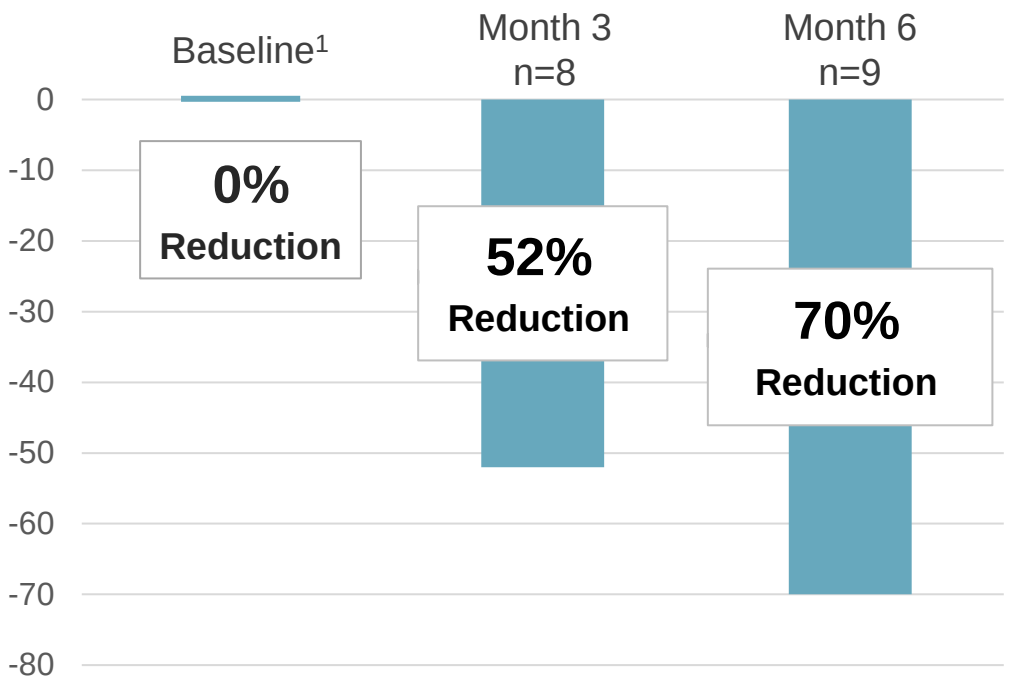
- 75% (9 of 12) eligible participants had G-CSF reduced
- **33% (3 of 9) of participants with dose adjustments taken completely off G-CSF prior to Month 6 visit**
- Perspective into physicians' possible real-world use of mavorixafor in CN

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

Mean G-CSF Reduction Over Time



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

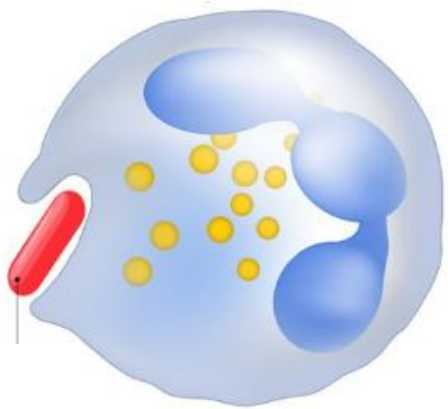
Purpose: **Demonstrate functionality of neutrophils in blood of individuals with CN, including those with congenital CN and genetic variants associated with neutrophil maturation arrest**

Neutrophil Functionality Assays[1]

Phagocytosis[2] (data to follow)

Assessment of neutrophils' ability to engulf pathogens

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

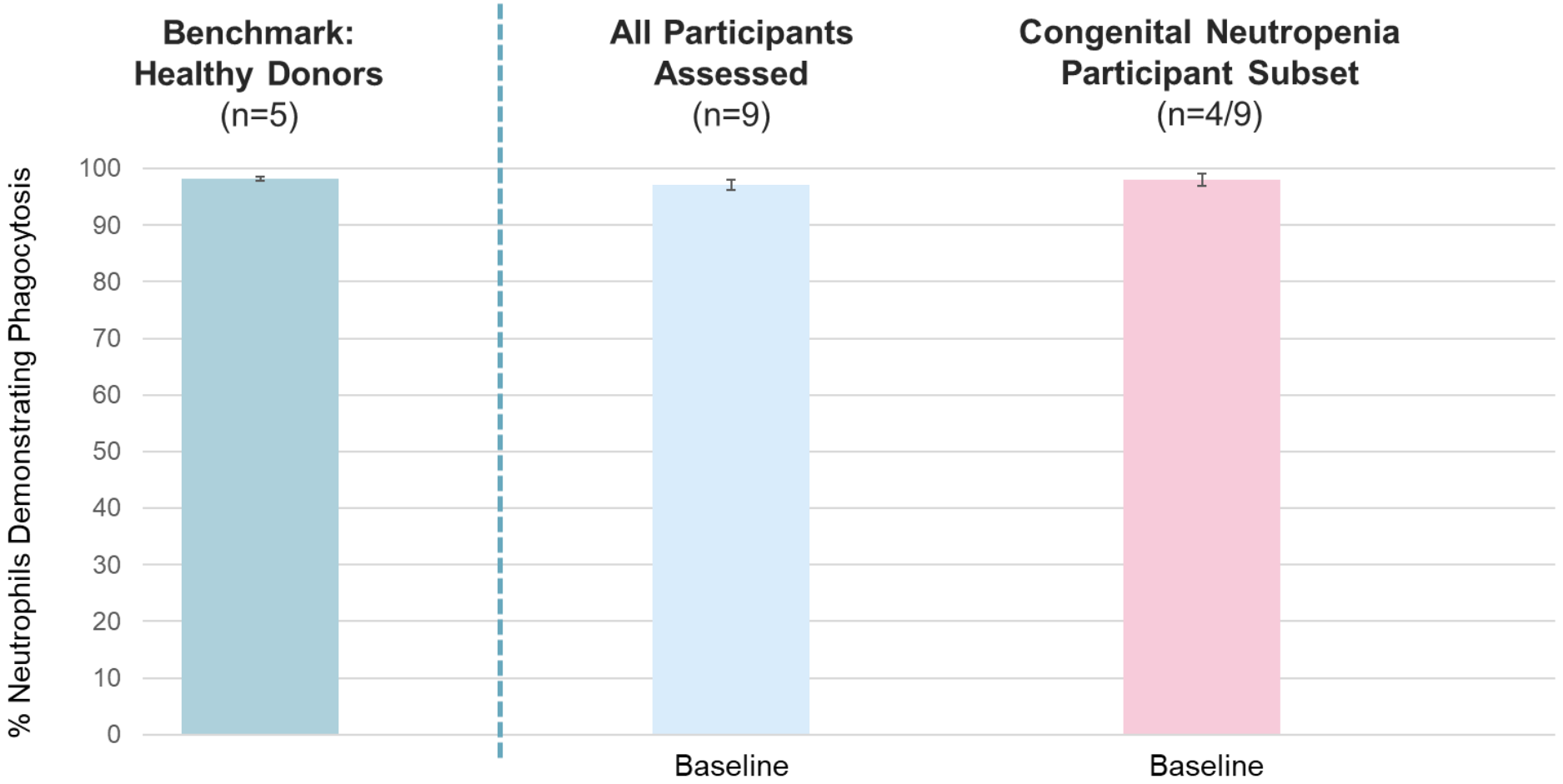
Phase 2 Sub-Study (n)[3]	**9**
Idiopathic / Congenital	5 / 4
Mav Mono / Mav + G-CSF	4 / 5
Healthy Donors (n)	**5**

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

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



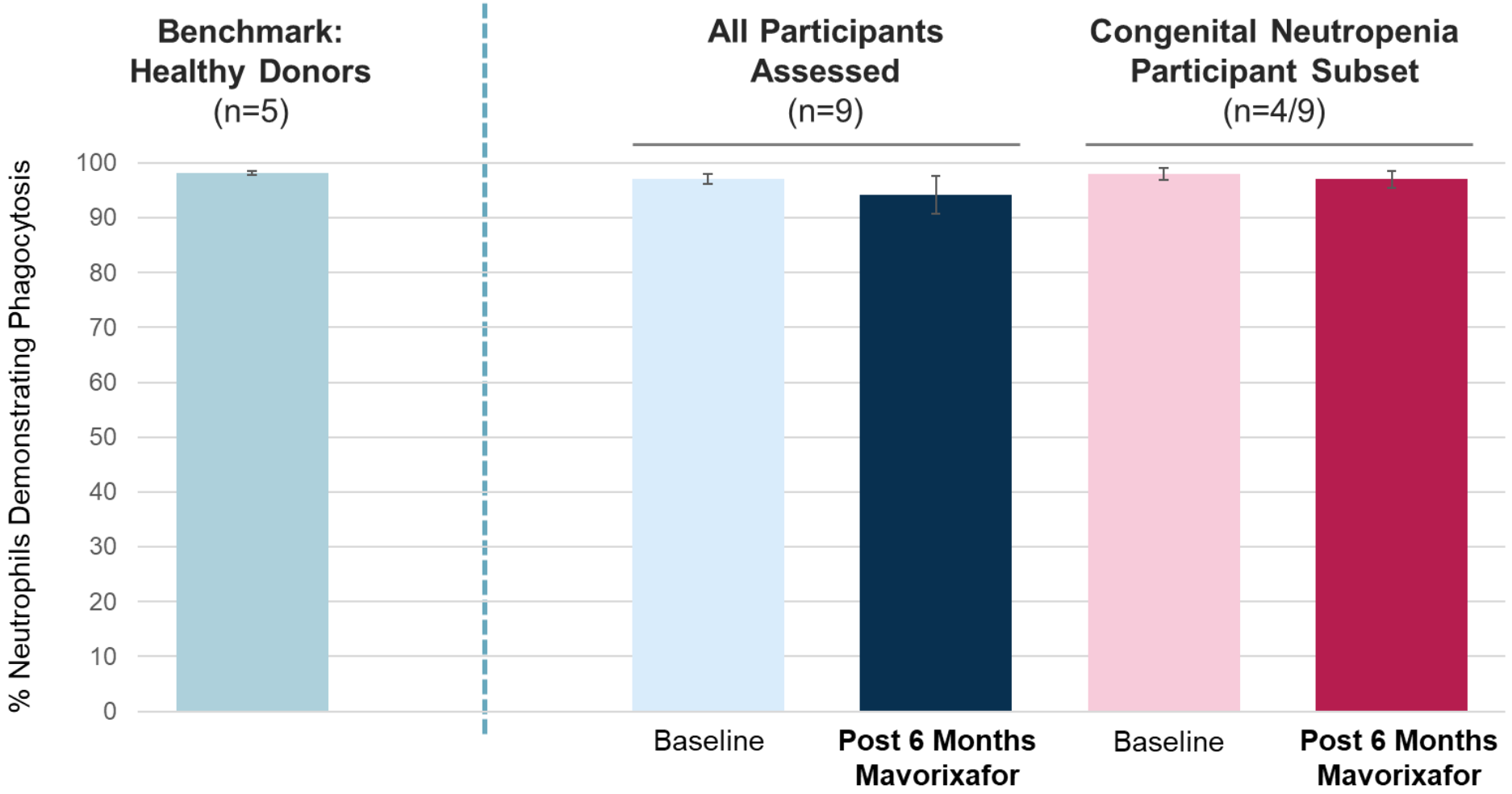
At baseline (pre-treatment): Mean percentage of functional neutrophils in study population were comparable to healthy volunteers

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

"Tapering off G-CSF could be a great alternative for many of our patients."

Jolan Walter, MD, PhD

"If you limit for this group of patients the number of injections of G-CSF … you will win."

Jean Donadieu, MD, PhD

"I believe that mavorixafor could be a life-changing therapy for patients with CN."

Peter Newburger, MD

"My ideal treatment short of a cure would be an oral medication."

Vanessa, CN Patient

"If I had to take a pill as opposed to giving a shot – I'd take that 100%."

Kevin, CN Patient

X4 PHARMACEUTICALS

Conclusions and Look Ahead to 2025

X4 PHARMACEUTICALS

Continuing to Deliver Progress for Patients

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

Expected Key 2025 Milestones

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

+

X4 PHARMACEUTICALS

Q&A Session