Investor Event
June 27, 2024

Mavorixafor in Chronic Neutropenia

Interim data from ongoing Phase 2 clinical trial

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Today's Agenda

01 Welcome

02 Overview of Chronic Neutropenia (CN)

03 Mavorixafor's Validated Mechanism of Action

04 Interim Phase 2 CN Trial Results

05 Phase 3 CN Trial & Market Opportunity

06 Conclusions and Q&A

Guest Speakers

Jean Donadieu, MD, PhD

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

Peter E. Newburger, MD

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

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

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

PROVEN SUCCESS IN RARE DISEASE DRUG DEVELOPMENT & COMMERCIALIZATION

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

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

STRONG BALANCE SHEET SUPPORTS CONTINUED GROWTH

Pro forma funds of $207 million[2]

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

NEXT VALUE DRIVER: MAVORIXAFOR IN CHRONIC NEUTROPENIA (CN)

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

Positive Interim Phase 2 Results Support Potential of Mavorixafor in CN

Summary of today's presentation

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

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

Pivotal Trial in CN initiated

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

Compelling Commercial Opportunity

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

Overview of Chronic Neutropenia

Chronic Neutropenia: Well Defined Market with Limited Treatment Options

~50,000[1]

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

1

Therapy approved for severe chronic neutropenia

Injectable Granulocyte Colony-Stimulating Factor (G-CSF)

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

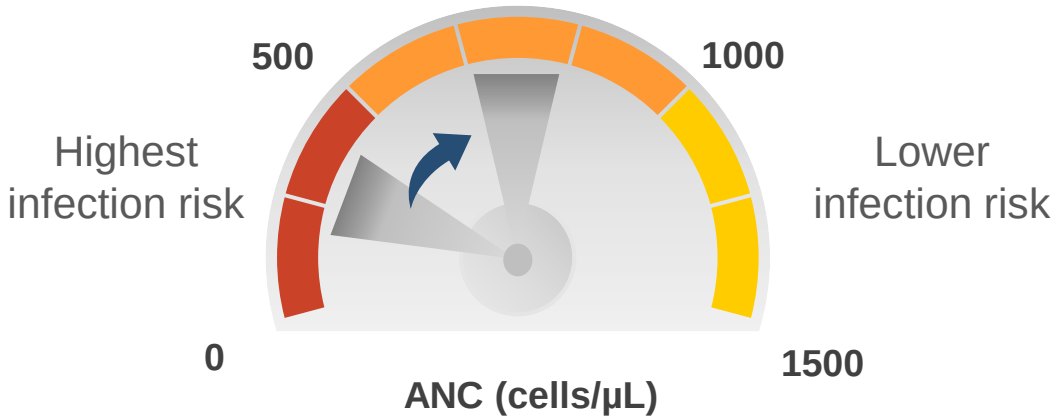
Innovation needed to address unmet patient needs

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

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

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

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



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

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

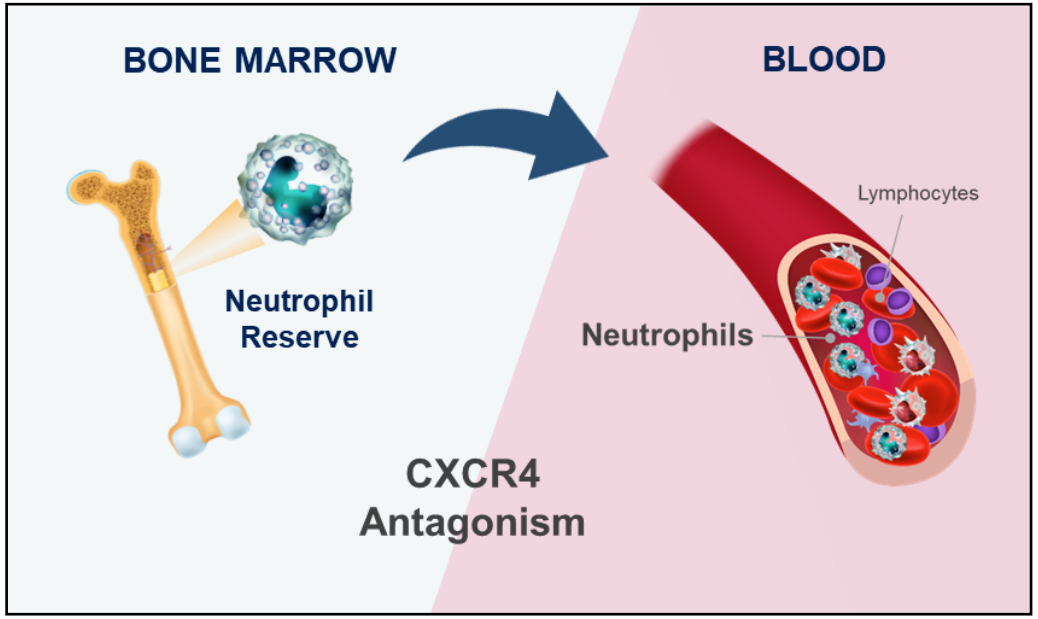
Mavorixafor’s Validated Mechanism of Action

X4 PHARMACEUTICALS

Validated Mechanism Shown to Increase Circulating Neutrophils

Targeted Mechanism

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



Modified figure from reference 1

Mavorixafor: Orally Active CXCR4 Antagonist

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

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

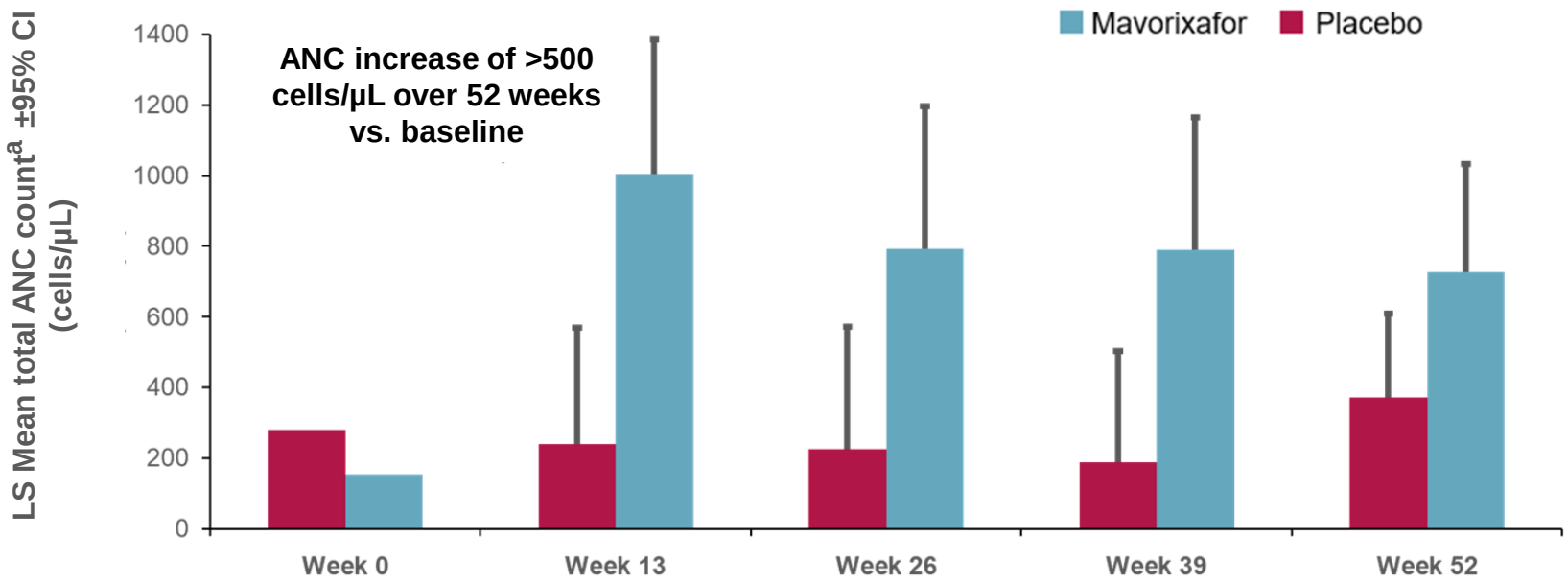
Mavorixafor Sustainably Raised ANC over 52 Weeks in Phase 3 4WHIM Trial

WHIM syndrome is a combined immunodeficiency and chronic neutropenia disorder

Primary Endpoint Met

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

Statistically Significant Increases in ANC Over Time*



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

All participants severely neutropenic at baseline

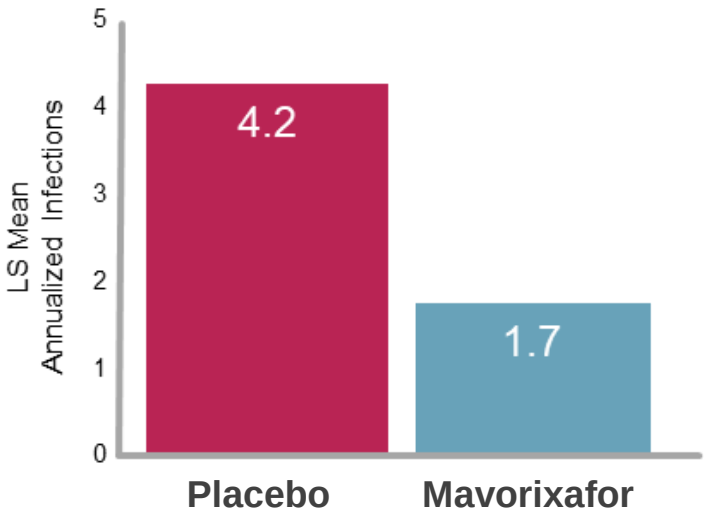
* Week 13 p=0.0049, Week 26 p=0.0397, Week 39 p=0.0196. a. Calculated as the mean of absolute cell counts over the 24-hour assessment period; b. At week 52, 3 of 17 placebo patients were given mavorixafor in advance of their TAT measurements as they entered the open-label portion of the study. All data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

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

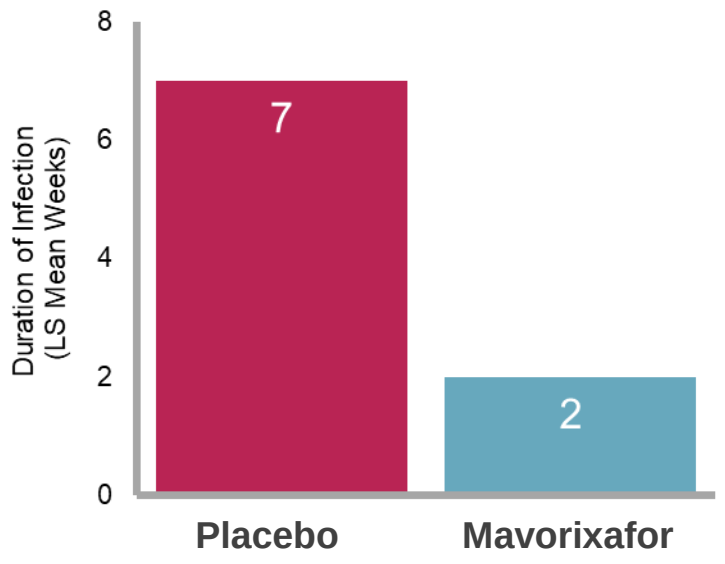
Mean ANC increases of >500 cells/µL reduced infection rate, duration, and severity

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

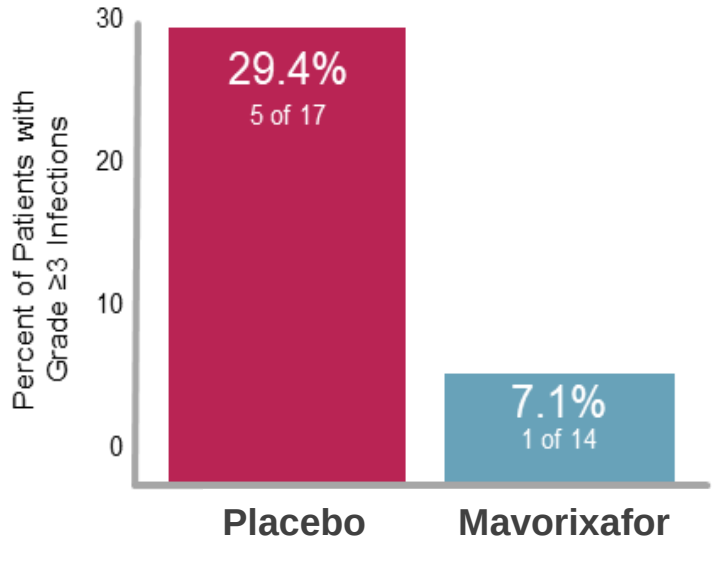
~60% reduction in annualized infection rate



Average infection duration was 5 weeks shorter



Fewer patients with severe infection



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

Interim Phase 2 CN Trial Results

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

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

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

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

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

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

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

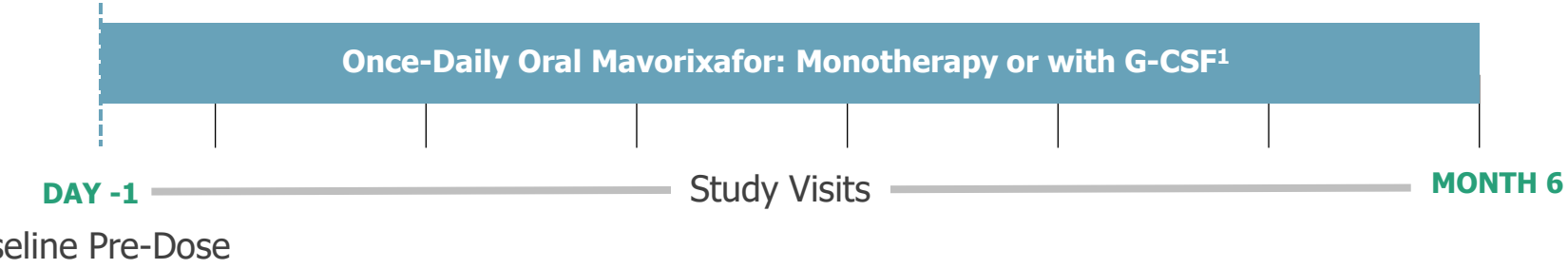
1. Warren JT, et al, oral presentation at ASH Annual Meeting December 2022. 2. X4 Advisory Board - expert opinion on file.

Assessing Mavorixafor in 6-Month CN Phase 2 Clinical Trial

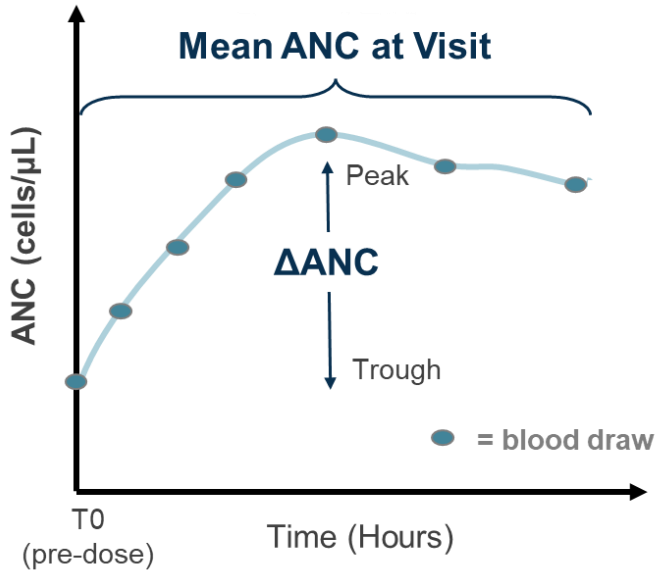
Mavorixafor dosed orally once-daily with or without background injectable G-CSF

Mavorixafor: Same Oral Dosing as 4WHIM Phase 3

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



Timepoint Efficacy Assessments – Per Participant



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

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

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

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

Ongoing study fully enrolled with 23 participants across three groups:

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

Participant Disposition as of May 14, 2024 Data Cut

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

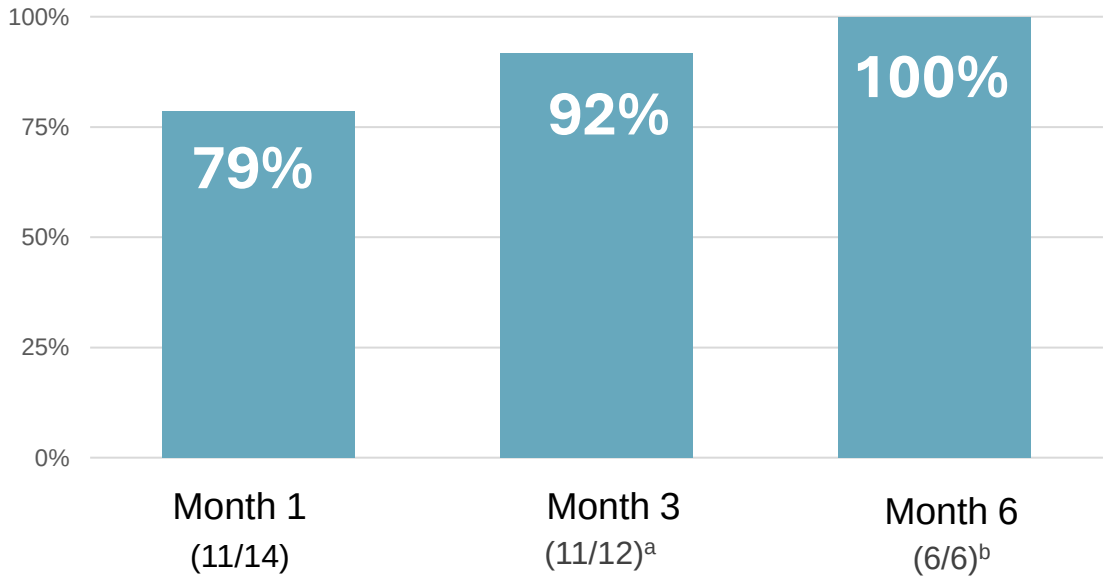
Types of chronic neutropenia studied: Idiopathic = 14; Congenital = 7; Cyclic = 2

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

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

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

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



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

TOTAL PARTICIPANTS (n=14)

- All had at least one timepoint to assess target ΔANC

Completed Participants (n=6)

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

Other Participants (n=8)

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

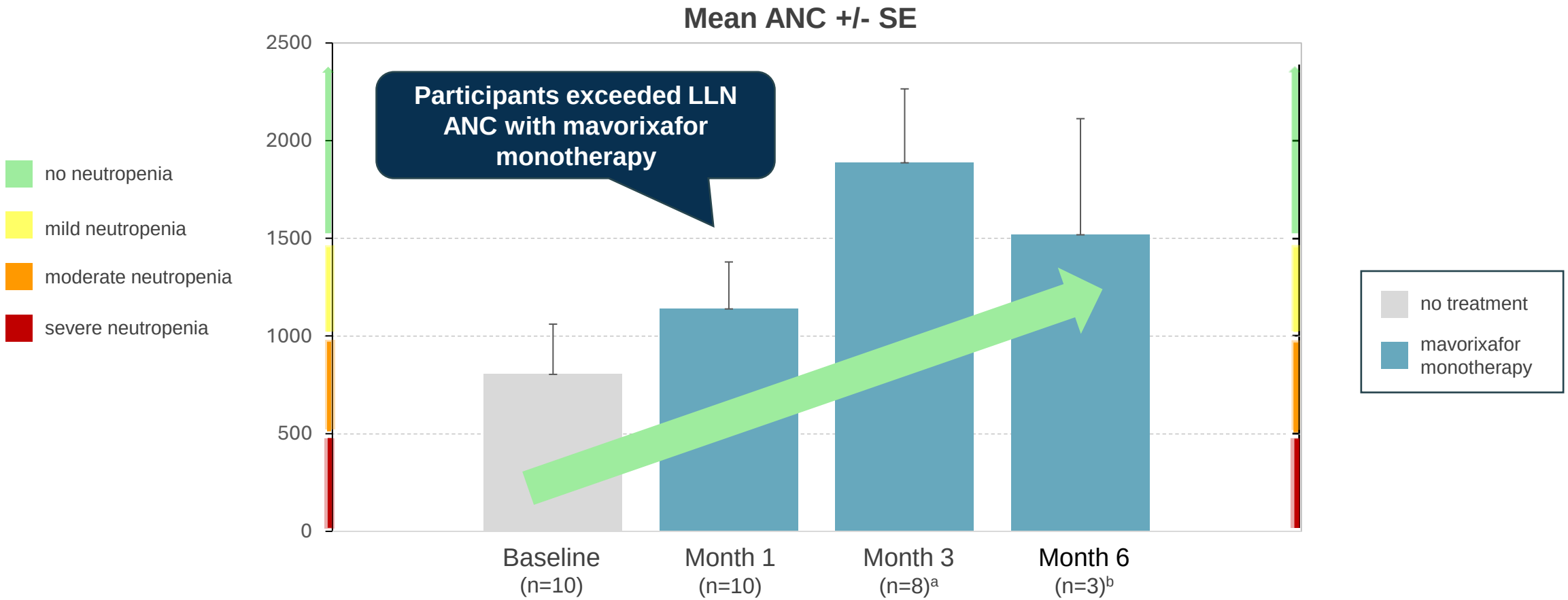
Demonstrated ability to maintain target ΔANC

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

Mavorixafor Monotherapy: Durable Increases in Mean ANC

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

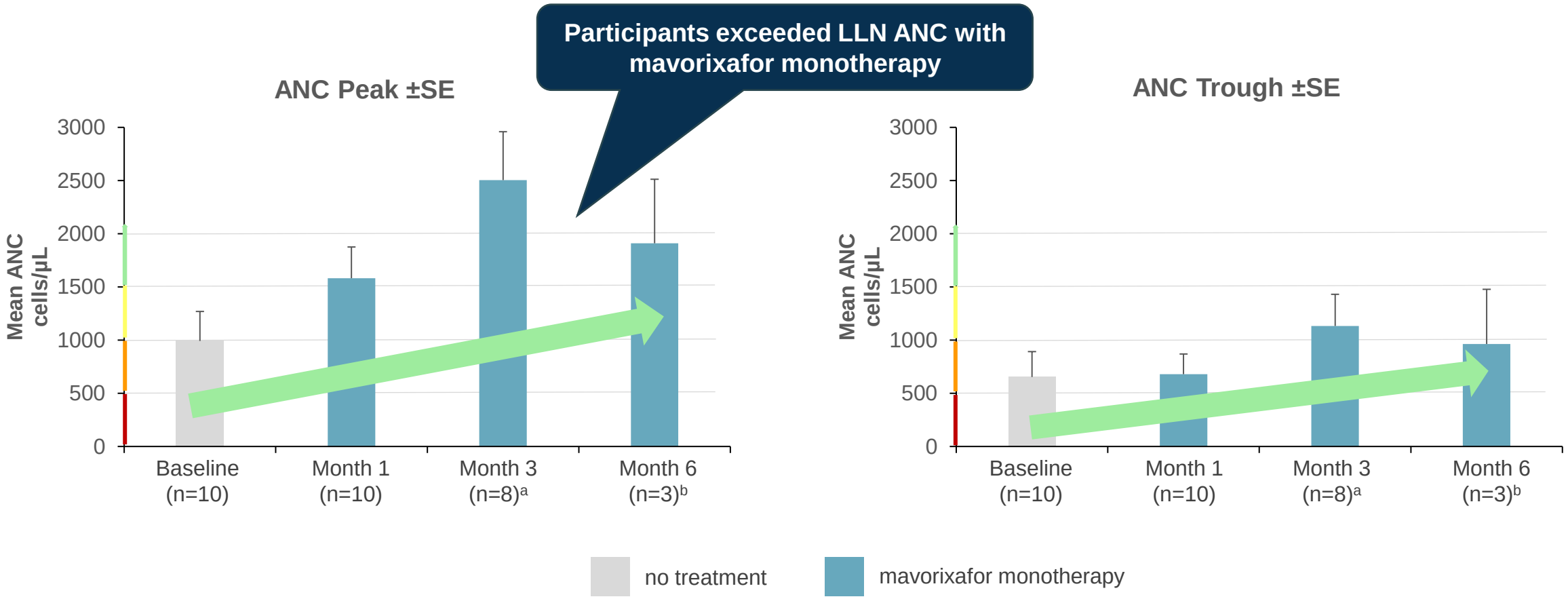
- Durable increases in mean ANC observed through Month 6



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

Mavorixafor Monotherapy: Robust Daily Coverage in ANC

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



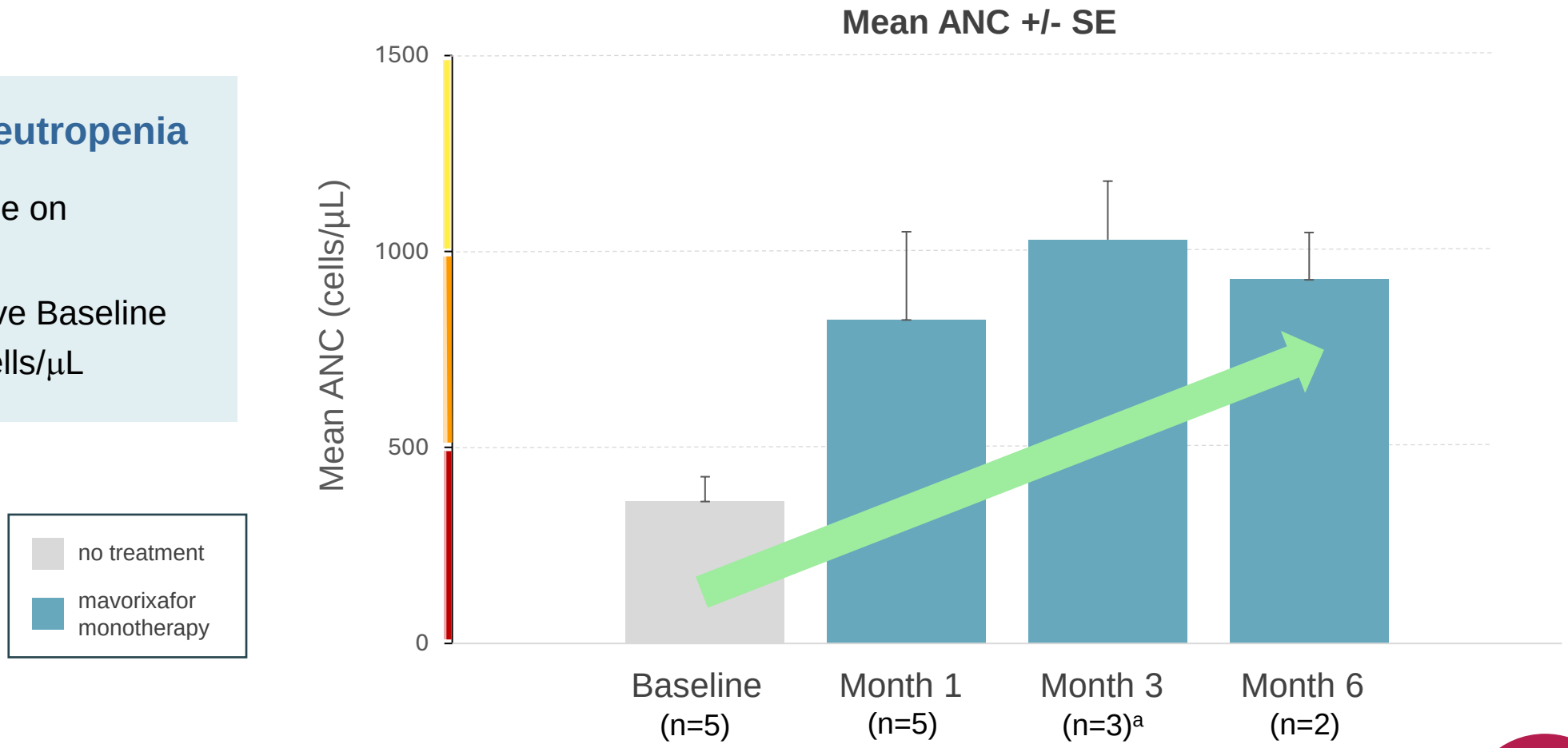
a. Samples from one (1) participant who completed the study were unevaluable at M3. b. Samples from one (1) participant who completed the study were unevaluable at M6.

Mavorixafor Monotherapy: Severe CN Participants Achieve Target ANC Increase

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

Severe Chronic Neutropenia

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



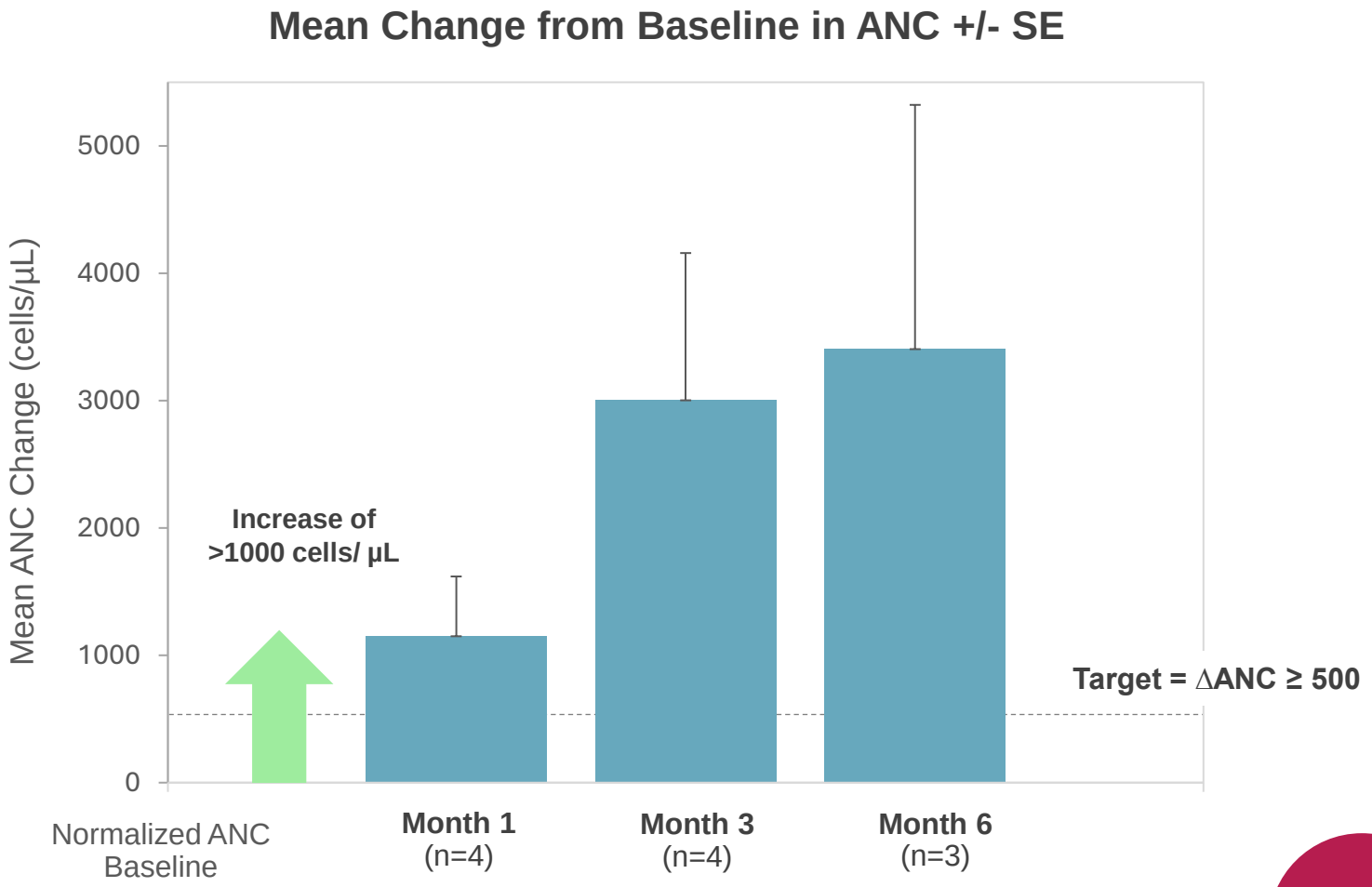
a. Samples from one (1) participant who completed the study were unevaluable at M3

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

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

Normalized to Baseline ANC

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



Phase 2 Chronic Neutropenia Study Safety Summary from Interim Analysis

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

→ Overall safety profile consistent with prior studies

→ No new safety issues when dosed in combination with G-CSF

→ No deaths and no drug-related serious adverse events (SAEs)

→ Most frequent adverse events GI related: nausea and diarrhea

- No discontinuations following education on possible GI effects that typically resolve over time

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

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

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

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

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

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

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

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

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

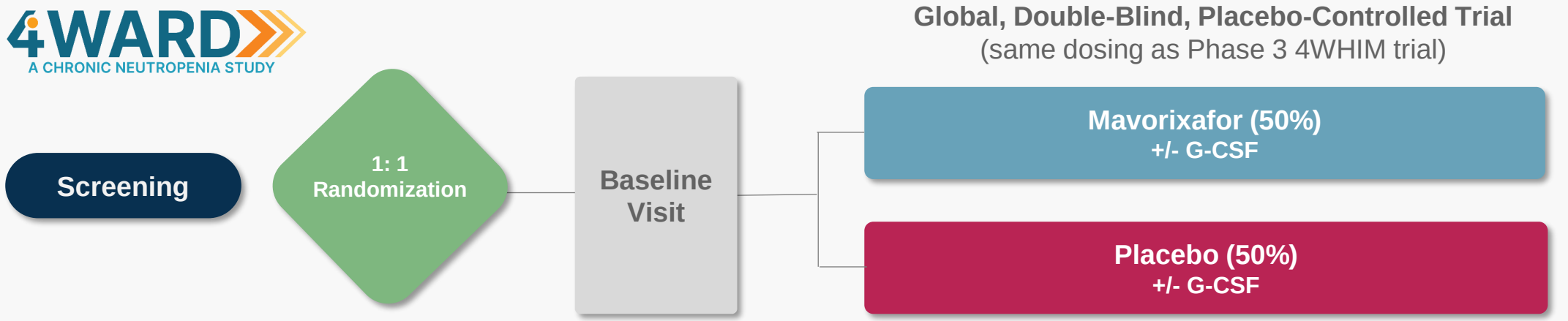
4WARD
A CHRONIC NEUTROPENIA STUDY

Phase 3 CN Trial & Market Opportunity

X4 PHARMACEUTICALS

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

Participants now in screening



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

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

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

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

Note: For those treated with G-CSF at baseline, G-CSF dose and frequency are required to remain constant throughout the trial unless adjustment is needed for safety reasons.

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

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

Phase 2 interim analysis:

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

Successful mavorixafor 4WHIM Phase 3 trial:

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

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

Significant Opportunity to Address Unmet Needs in the CN Community

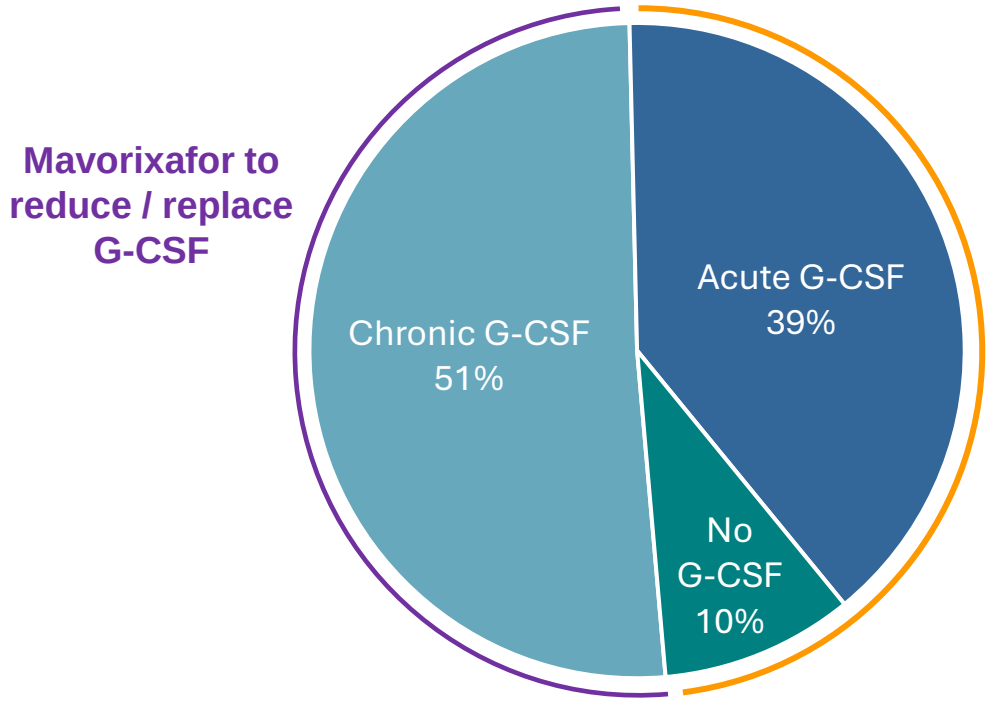
High unmet needs in ~15,000 patients in the U.S.

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

Current use of G-CSF within these patient populations

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

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



Potential role of mavorixafor

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

Conclusions

Mavorixafor Holds Potential to Address Needs of CN Community

Clear unmet need

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

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

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

Treatment success well defined

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

Clinical success = ability to reduce annualized rate of infections

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

Defined / identified U.S. CN patient population

Defined/identified U.S. CN patient population

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

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

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

X4 Well Positioned to Deliver on Promise of Mavorixafor

U.S. approval & launch for WHIM syndrome April 2024

Priority Review Voucher monetized

Laying a strong foundation for XOLREMDI U.S. sales & market growth

Positive interim data from Phase 2 study support and de-risk Phase 3 CN trial

Global, pivotal 4WARD Phase 3 CN trial initiated

Additional Phase 2 CN data expected end of 2024

Potential Market Opportunities

WHIM

>1,000 U.S. patients

Chronic Neutropenia

>15,000 U.S. patients

Q&A Session