X4 PHARMACEUTICALS

Nasdaq: XFOR

Corporate Presentation

April 2026

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Restructured Company Delivering on the Promise of CXCR4 antagonist Mavorixafor

Addressing unmet medical needs in hematology

MAXIMIZING THE POTENTIAL OF MAVORIXAFOR IN CHRONIC NEUTROPENIA

- Mavorixafor (XOLREMDI®) approved for the treatment of WHIM syndrome[1] in 2024
- Focused on completing the **Pivotal Phase 3 4WARD Chronic Neutropenia study**
- Full enrollment expected in Q3 2026
- Top-line data expected in 2H 2027
- Potential US FDA approval in 2028

CORPORATE RESTRUCTURING FOLLOWING RECAPITALIZATION

- $240M raised from blue chip investors including Fidelity, Counterpoint Global and Blackstone
- New C-Suite appointments from previous CTI BioPharma senior management team
- 50% reduction in workforce and on-going cost reductions to reduce monthly burn
- Cash runway through 2028

1. WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis), a rare primary immunodeficiency and chronic neutropenic disorder.

X4's Leadership Team – Turnaround, Drug Approval and Commercialization Experience

Former CTI BioPharma Senior Management sold company to SOBI for $1.7B in 2023

Adam Craig, MD PHD
Executive Chair

CTI
STRATUS Therapeutics
egenesis ENGINEERING HOPE

John Volpone
President and COO

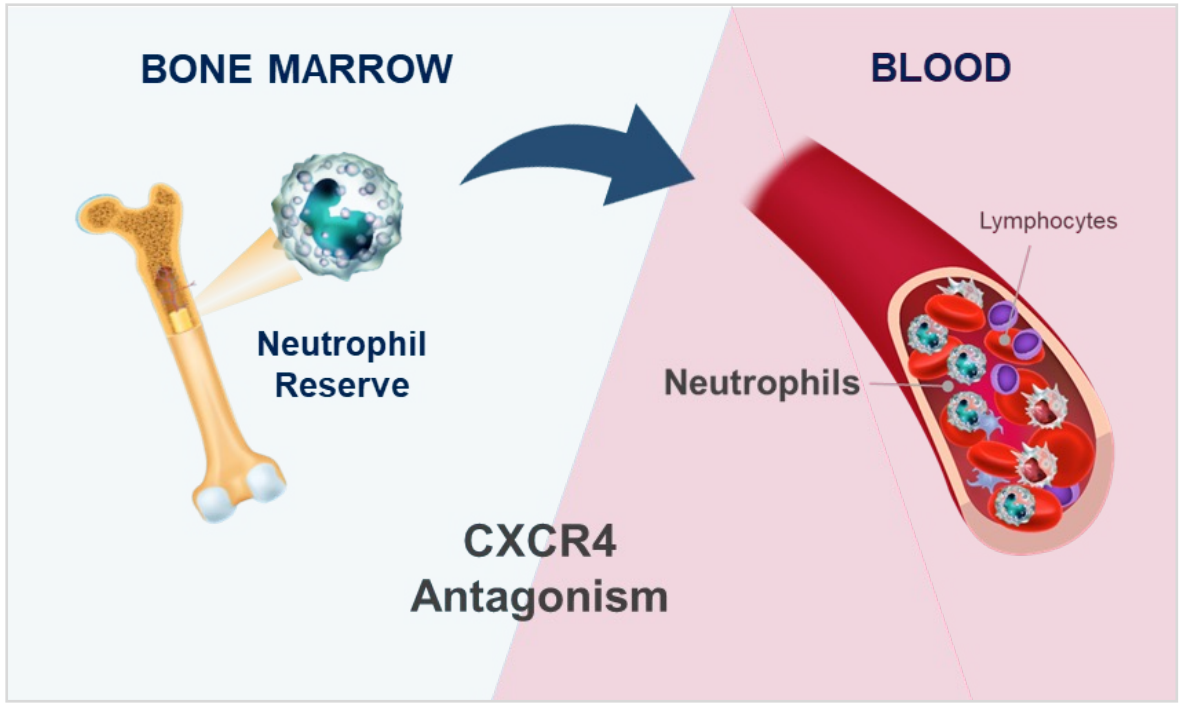
CTI
Bristol Myers Squibb
ZYMOGENETICS

David Kirske
Chief Financial Officer

CTI
STRATUS Therapeutics

Mavorixafor: An oral agent designed to alleviate neutropenia

Validated mechanism with the potential to increase neutrophil counts and decrease infection rates



Modified figure from reference 1

Targeted Mechanism

- CXCR4 regulates movement of white blood cells throughout the body[2]
- **CXCR4 antagonism results in the** migration of **white blood cells** from the bone marrow[3,4]

Orally active CXCR4 Antagonist

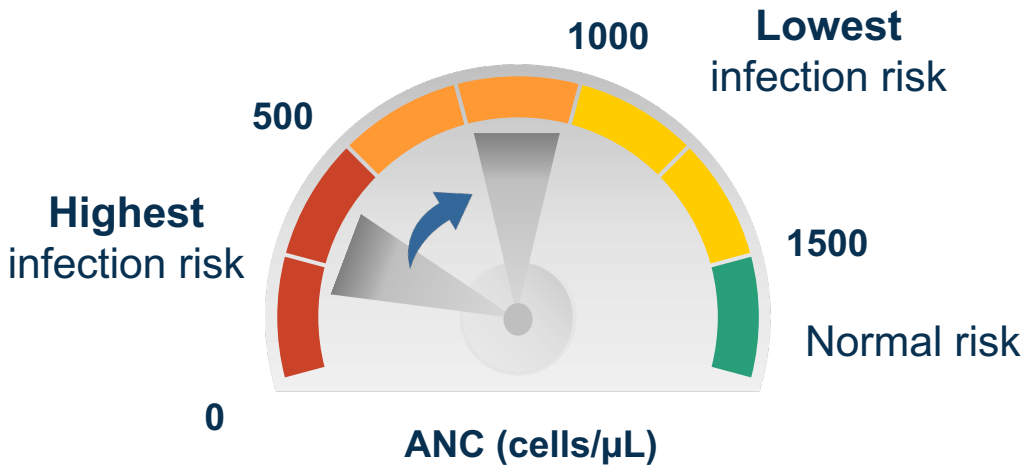
- Mavorixafor raises circulating blood levels of neutrophils and lymphocytes[4,5,6,7]
- Top-line pivotal Phase 3 study in Chronic Neutropenia data expected in 2H 2027
- U.S. patent protection expected through 2038

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 American Society of Hematology 2022.
7. US Product Label XOLREMDI 2024.

Chronic Neutropenia – Increasing Risk of Serious Infections and Hospitalization

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]



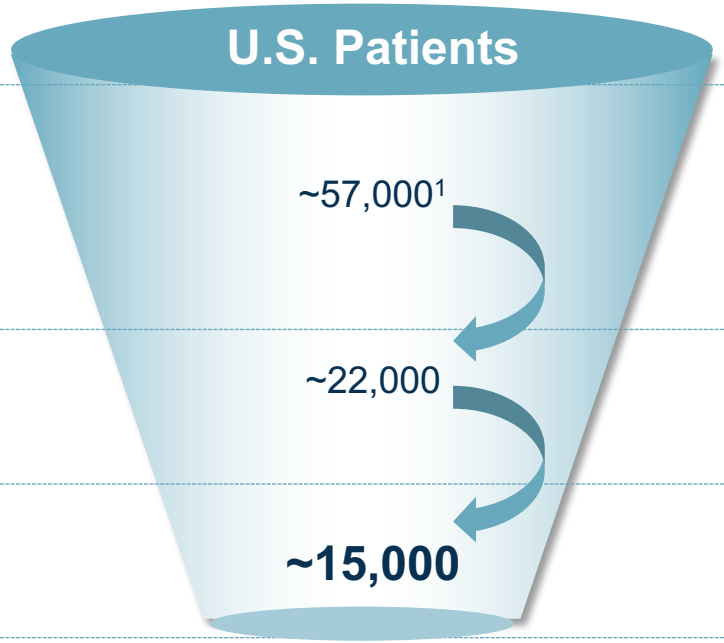
- Frequent and/or serious infections are the primary clinical consequence of chronic neutropenic disorders[3]
- Infections may lead to frequent hospitalizations or result in life-threatening complications, including death[4,5]

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.

Chronic Neutropenia Opportunity – High unmet need in ~15K patients in U.S.

Large number of primary CN patients experiencing severe/life threatening and recurrent infections

- Primary Chronic Neutropenia[1]
  - Excluding secondary causes[2]
- Serious recurring Infections[2]
  - Inadequately treated
- **Moderate and Severe Disease**[3-5]
  - ANC <1000 cells/μL



US Target Market for Mavorixafor in CN

Veeva Compass

January 2023 – April 2025

US Quantitative Survey of 95 Hematologists/Oncologists[3]
HCP self-reported estimates

Of the patients you have personally managed in the last 12 months what % of your primary CN patients fall into the following categories?

- Experiencing recurrent serious infections[4]
- Moderate (ANC 500 - 999 cells / μL) or Severe (<500 cells / μL) with recurrent serious infections

1. Analysis of claims data derived from Veeva Compass 2+ claims with ICD-10 codes: D70.0, D70.4, D70.8, D70.9 between January 2023 – April 2025.
2. Secondary Causes: drug/chemo induced neutropenia, chemotherapy, Transplant patients, ESRD, CKD, MDS, Antipsychotics/anticonvulsants, anemia, dialysis. Duffy Null mutation is a mild form of Neutropenia that was excluded.
3. US HCP Quantitative Market Research Survey, December 2024 (n=95 Hematologists/Oncologists)
4. Recurrent serious infections defined in quantitative survey[3] as: at least 2 infections in the past year, requiring the use of antibiotics (intravenous, oral, or topical) AND/OR requiring a visit to healthcare facility (including but not limited to emergency room visit, urgent care facility, primary care physician's office, or in-patient hospitalization).
5. Veeva Compass claims analysis from 1/2023-4/2025: ~15,000 patients (7% congenital, 93% idiopathic) with 1+ infection (congenital) or 6+ infections (idiopathic) to approximate moderate to severe disease

Significant Opportunity to Address Unmet Needs in CN Community

Broad opportunity for mavorixafor use as monotherapy or in combination with G-CSF

Mavorixafor Monotherapy

Treating patients who are:

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

Dose reductions of G-CSF to:

- To lessen pain and discomfort
- To reduce the long-term risk of malignancies

Mavorixafor Combination Therapy

Current use of G-CSF within primary CN patient populations[1]

- ~32% of patients on continuous G-CSF therapy
- ~26% of patients receiving sporadic/acute G-CSF
- ~42% of patients not receiving G-CSF therapy

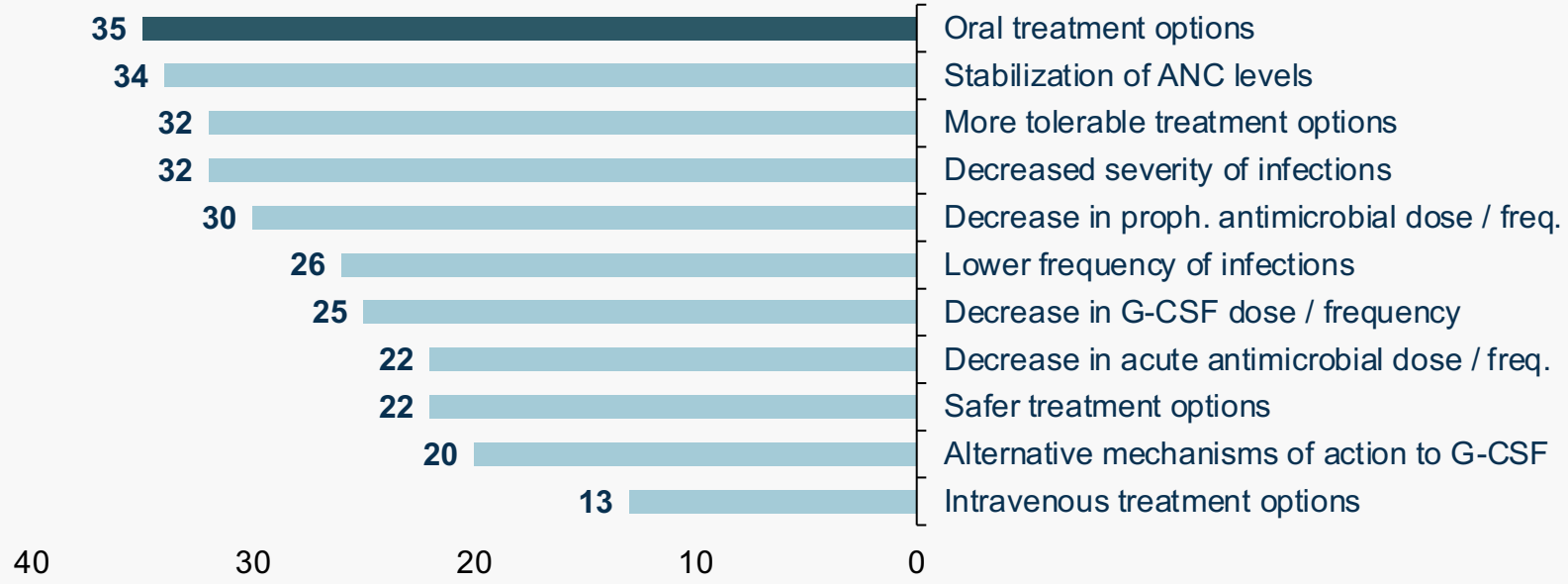
1. X4 Market Research, July 2023 – data on file; ICD-10 Code Research (2017-2023). 2. G-CSF – Granulocyte Colony Stimulating Factor.

Oral Treatment ranked as top unmet need by CN treating physicians

CN

Unmet Needs For CN Patients[1]

(Times Ranked in Top 3 by Respondents; N = 95 Physicians)



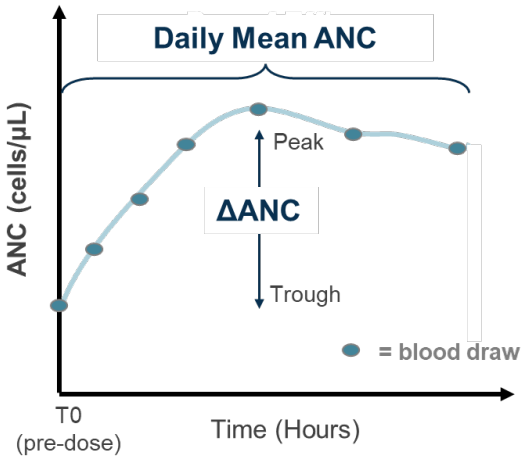
1. US HCP Quantitative Market Research, Sept 2024 (n=95 Hematologists with ~20 CN pts each).

Phase 2 Proof of Concept Study of Mavorixafor in Chronic Neutropenia (CN)

Phase 2 Study N=23 patients

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

Timepoint Efficacy Assessments



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

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

Phase 2 Clinical Study in Chronic Neutropenia: Participant Disposition

Study group representative of typical CN population with history of prior infections

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
(mean baseline ANC 750 cells/µL; range 180-2356 cells/µL)

	Baseline
Total	10

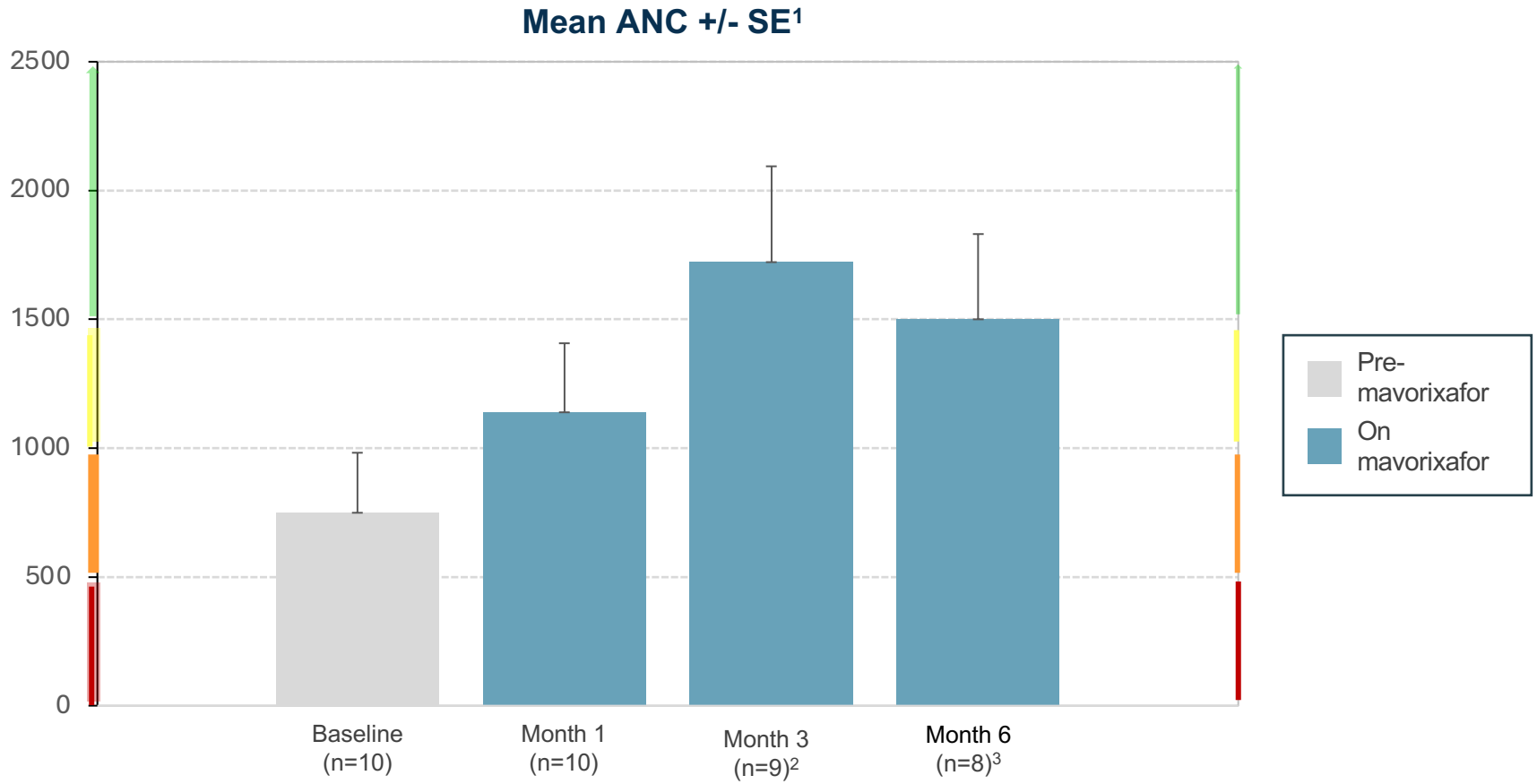
Mavorixafor + G-CSF
(mean baseline ANC 4034 cells/µL; range 470-11183 cells/µL)

	Baseline
Stable G-CSF Total	4
Adjusted G-CSF[2] Total	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 at physician's discretion

Mavorixafor Monotherapy Increased Mean ANC

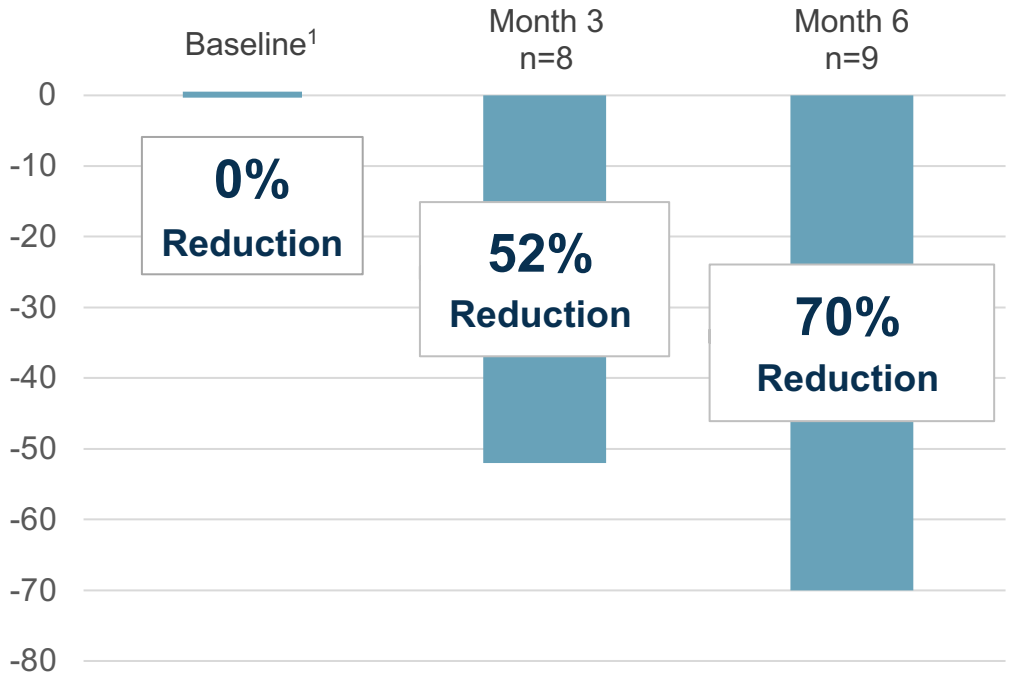
Mean ANC reached normal levels (ANC ≥ 1,500 cells/μL) at 3 and 6 months of treatment



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).

Substantial Reductions in G-CSF on Study

Mean G-CSF Reduction Over Time



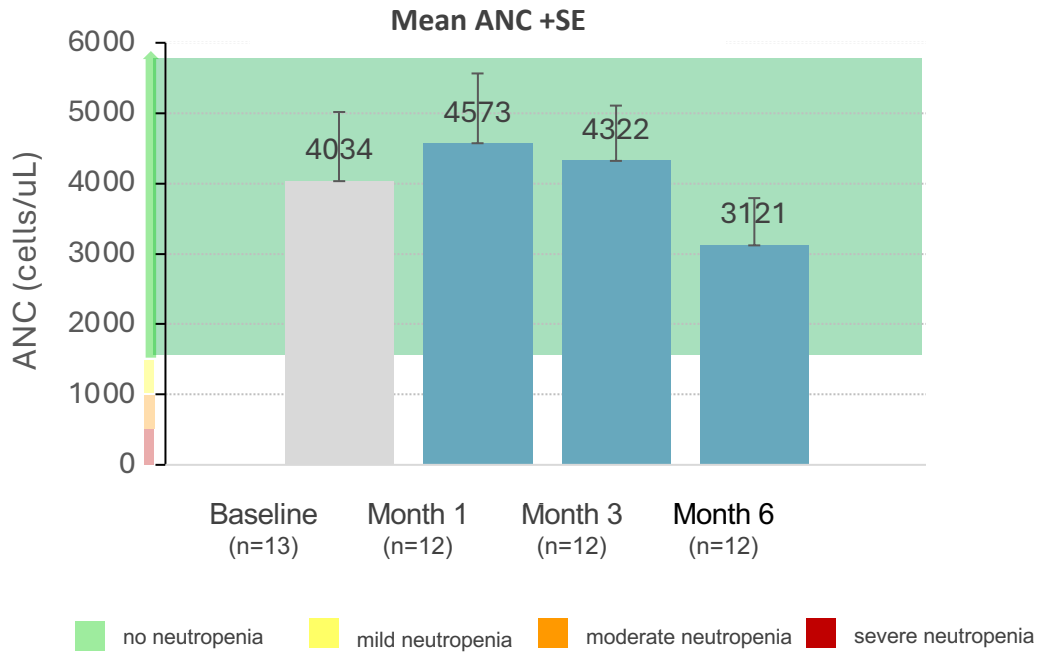
Key Takeaways

G-CSF:

- Treating physicians chose to reduce injectable G-CSF therapy in 9 of 12 (75%) eligible patients
- **Three patients with dose adjustments had G-CSF usage stopped by 6 months**
- Mean ANC maintain at normal levels (>1,500 cells/μL) through Month 6
- Data demonstrates the potential to reduce G-CSF usage with mavorixafor

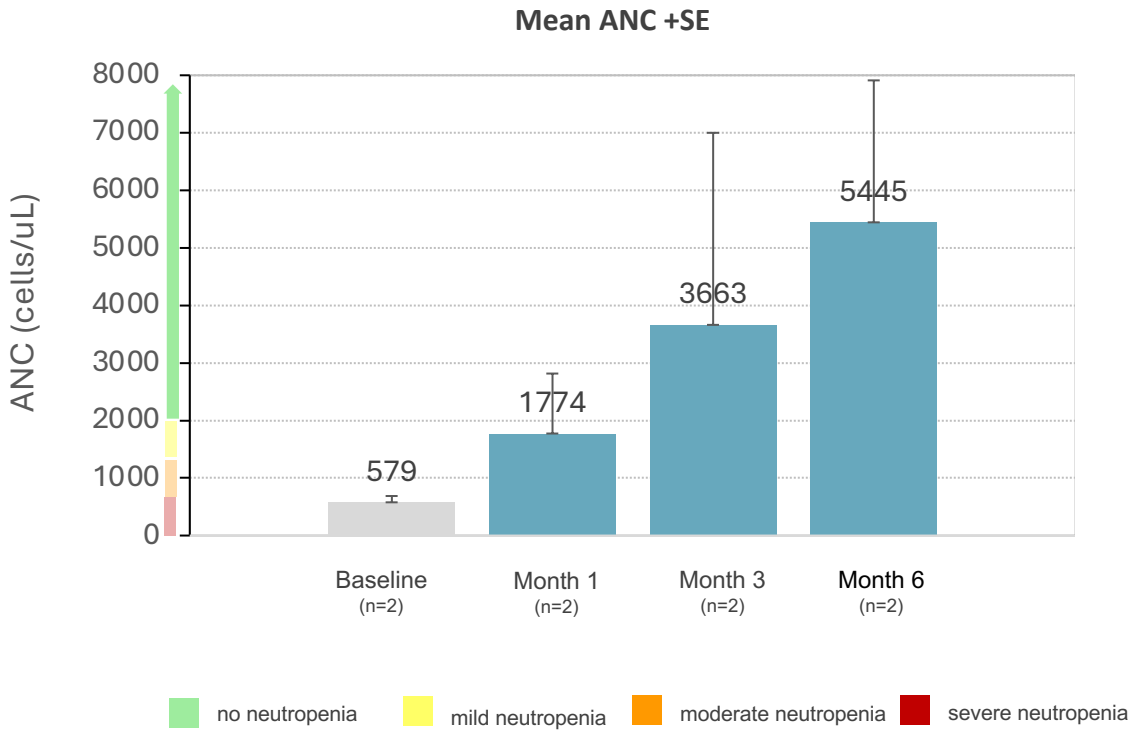
Mavorixafor maintains normal ANC counts with G-CSF stoppages and doses reductions

Mean ANC in patients taking Mavorixafor + G-CSF



Data demonstrates the potential to reduce or stop G-CSF usage with mavorixafor

Mean ANC for Moderate and Severe CN in G-CSF Cohort



- 2/13 G-CSF cohort subjects were moderate/severe (1 moderate/1 severe)
  - 001-007 was Cyclic CN on Stable G-CSF. Baseline ANC=687 cells/uL
  - 011-009 was Congenital CN whose G-CSF was reduced. Baseline ANC=470 cells/uL

Phase 2 Chronic Neutropenia Study Safety Summary

Mavorixafor is well tolerated as both 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
- Most frequent treatment-related TEAEs[1] were mild/moderate and GI related (nausea and diarrhea)
- No drug-related serious adverse events

Treatment-related TEAEs Occurring in >20% of Participants

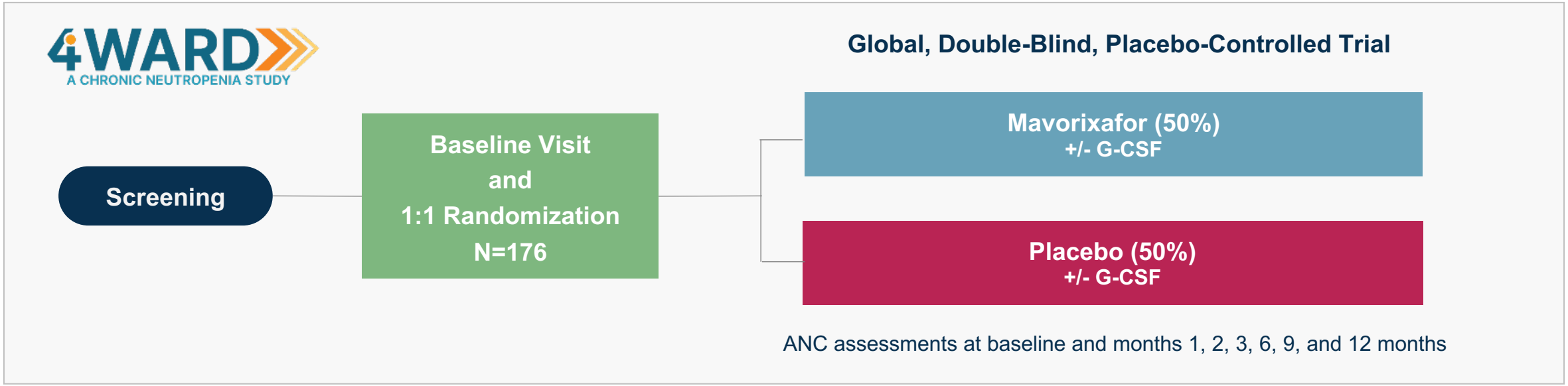
All mild to moderate

	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.

Pivotal Phase 3 4WARD Study in Chronic Neutropenia

Full enrollment expected Q3 2026 with top-line data expected 2H 2027



Key Inclusion Criteria for **congenital, autoimmune, or idiopathic chronic neutropenia**

- Absolute Neutrophil Count (ANC): <1,000 cells/μL (moderate and severe disease)
- Infection History: 2 or more infections requiring intervention within last 12 months

Primary Endpoints: **ANC response (>500 cells/μL)** and **annualized infection rate (AIR)**

- Both endpoints powered to >96% for ITT population

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

Emphasis on 4WARD Enrollment

Corporate resources dedicated to enrollment enhancing activities

- Increase in the number of U.S. sites
- Enhanced engagement with U.S. sites
- Consolidating CROs to improve efficiencies
- Investment in database mining to identify patients
- Global MSL activities solely focused on site interaction and enrollment support

Strong Financial Position

Fully Funded to launch

Cash (12.31.25)	Fully Diluted Common Stock Outstanding
$253 M	141 M

Fully diluted common stock outstanding includes pre-funded warrants

X4 on Track to Deliver Pivotal Phase 3 Data in 2H 2027

Potential FDA approval in 2028

- New C-Suite team experienced in hematology drug approvals and product launches
- Blue chip investor base established after recent PIPE/CMPO
- Cash runway through anticipated CN indication launch in 2028

- Clear proof of concept for mavorixafor in CN from Phase 2 study
- 4WARD study revamped with additional resources
- Topline data expected in 2H 2027 with sNDA submission to FDA anticipated late 2027

- Large market opportunity for mavorixafor in CN in the US
- ~15,000 patients with moderate and severe disease
- Clear medical need for well tolerated oral agent - monotherapy or in combination with G-CSF

Appendix

Mavorixafor: Pipeline and Partners

	Indication	Pre-clinical	Phase 1	Phase 2	Phase 3	Approved	Partners	Expected Milestones
Mavorixafor	**Chronic Neutropenia** (Congenital, Autoimmune, Idiopathic)	Global Pivotal Phase 3 Study Ongoing						Full enrollment 3Q 2026 Top-line data 2H 2027
	WHIM Syndrome (Warts, Hypogammaglobulinemia, Infections, Myelokathexis)	U.S. FDA Approved MAA Under Review in EU Seeking Approvals in MENA Region					NORGINE taiba access rare	Launched as XOLREMDI® in U.S. 2024 EU approval expected in Q2 2026