X4 PHARMACEUTICALS

Nasdaq: XFOR

Corporate Presentation

November 2026

Forward-Looking Statements

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Delivering on the Promise of CXCR4 Antagonist Mavorixafor

Focused on improving the lives of people with rare hematology diseases

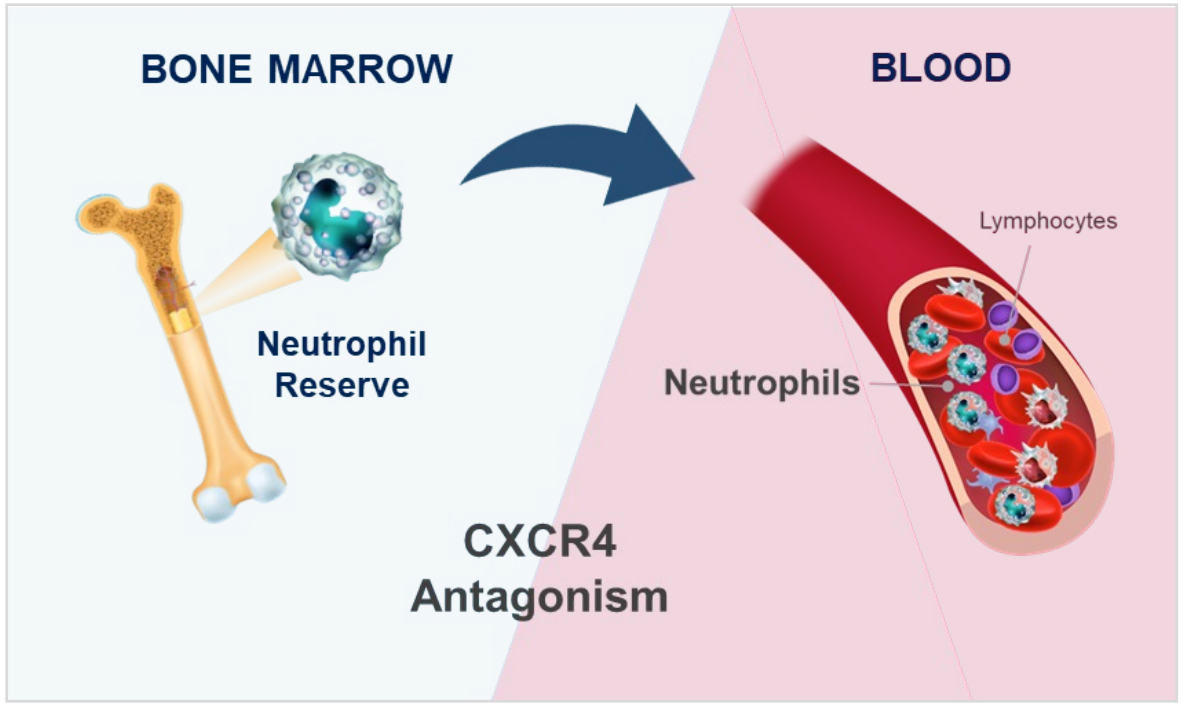
MAXIMIZING THE POTENTIAL OF MAVORIXAFOR IN PRIMARY CHRONIC NEUTROPENIA (PCN)

- Underwent corporate transformation in 2025/2026
- Focused on completing the **Pivotal Phase 3 4WARD Primary Chronic Neutropenia** trial
  - Full enrollment (N=126) expected by end of Q4 2026
  - Topline data expected in Q1 2028
  - Potential US FDA approval in late 2028 / early 2029
- Initiating Phase 2b clinical trial to demonstrate how to safely reduce a patient's G-CSF dosage following initiation of treatment with mavorixafor
- Targeting potential 15,000 to 30,000 U.S. patient market opportunity

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

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 Primary Chronic Neutropenia data expected in Q1 2028
- 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.

Mavorixafor Pipeline

Indication	Phase 1	Phase 2	Phase 3	Approved	Key Milestones
PRIMARY CHRONIC NEUTROPENIA (Congenital, Autoimmune, Idiopathic)					
Phase 3 · Ongoing Pivotal trial in PCN patients experiencing recurrent and/or serious infections	**4WARD Global Pivotal**				**Full Enrollment End Q4 2026** **Topline Data Q1 2028**
Phase 2b · Initiating G-CSF dosage reduction in mavorixafor-treated participants	**4STEPS G-CSF Dose Reduction**				**First Participant Dosed Q1 2027** **Topline data Q1 2028**

WHIM SYNDROME
Warts, Hypogammaglobulinemia, Infections, Myelokathexis

U.S.
Launched as XOLREMDI® (2024)

EU
Approved by European Commission (2026)

MENA
Seeking approvals

Commercial partners

NORGINE
Europe, Australia, NZ

taiba access rare
MENA

Chronic Neutropenia Overview

High Disease Impact & Limited Treatment Options in Primary Chronic Neutropenia (PCN)

Rare, chronic blood disorder

- Neutrophil counts <1,500 cells/μL for >3 months
- Idiopathic, congenital, cyclic and autoimmune

Heterogeneous disease with a large underserved market that is often underdiagnosed and misdiagnosed

Limited treatment options via G-CSF injections or antibiotics; no approved therapies for moderate neutropenia

Significant unmet need remains despite available therapies

Recent market research indicates that mavorixafor may capture a significant share of the PCN market



No approved oral product indicated for PCN

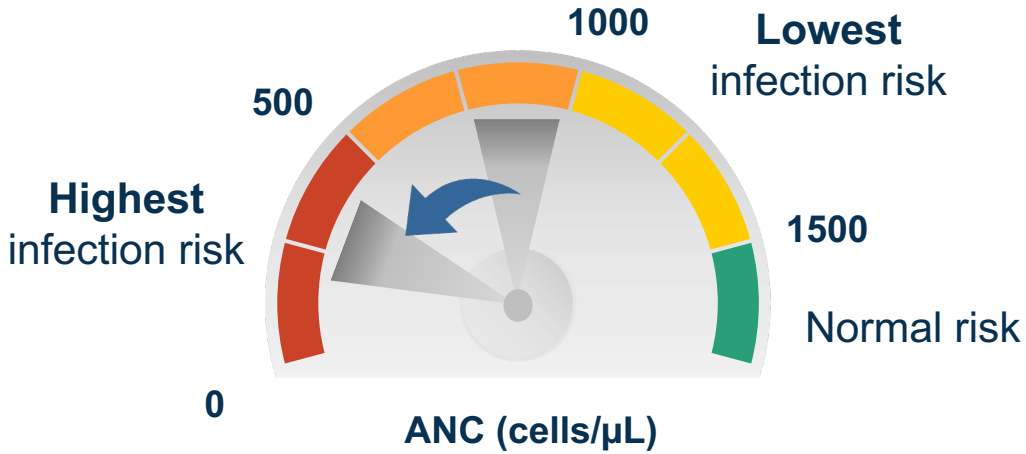
Patients are left with limited treatments from an existing assortment of off label, unproven, or difficult to use treatment options

1. Komodo Claims Analysis, 2026 (based on 2025 data), 2. Infection sites per Donadieu J, et al. Expert Rev Hematol. 2021; Newburger PE, et al. Semin Hematol. 2013; Dale DC, SCNIR publications.

PCN Can Lead to a High Risk of Infection

NIH Classification	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)

Decreasing Neutrophil Counts Increases Risk of Infection



Sources: 1. https://ctep.cancer.gov/protocoldevelopment/electronic_applications/docs/ctcae_v5_quick_reference_8.5x11.pdf. Palmblad J, Dufour C, Papadaki HA. Haematologica. 2014 Jul;99(7):1130-1133. Platzbecker, U, et al. Blood. 2019 Mar;133(10):1020-1030. Donadieu J, et al. Expert Rev Hematol. 2021 Oct;14(10):945-960. Newburger PE, et al. Seminars in Hematology 2013 Jul;50(3):198-206.

PCN Drives Substantial Healthcare Utilization and Cost

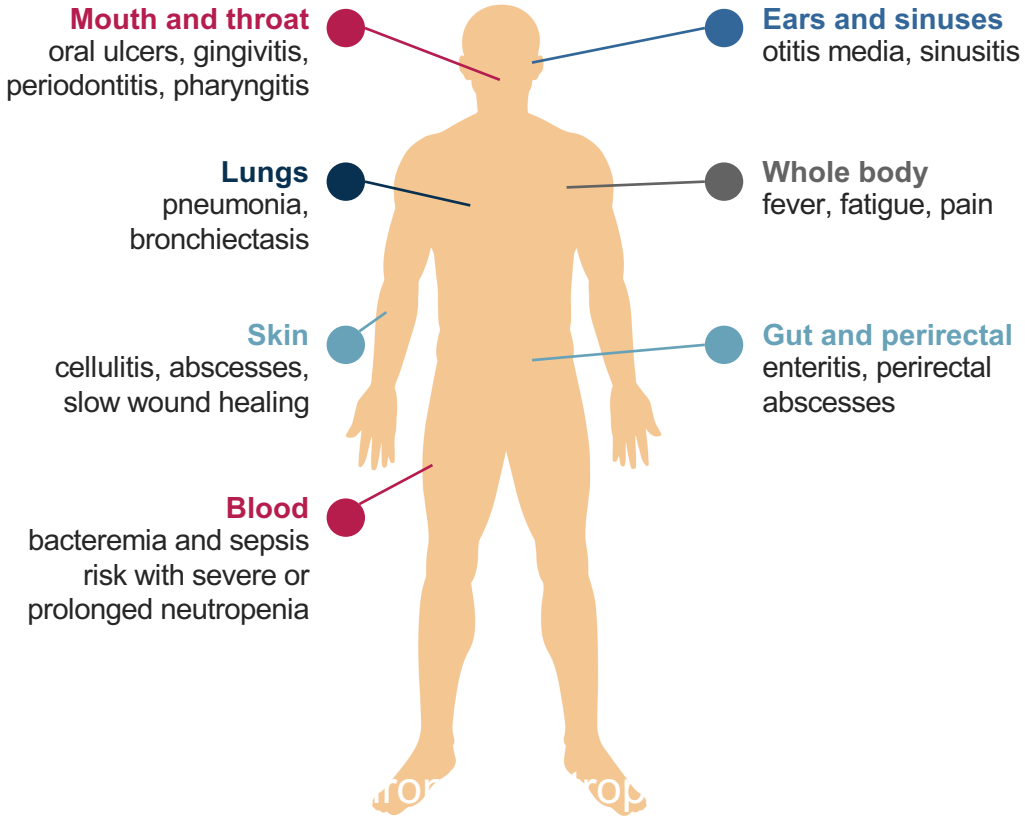
Recurrent and serious infections can lead to serious complications and hospitalization

- Pneumonia, bronchiectasis and sepsis, often result in ER visits and hospitalization

U.S. Hospitalizations in 2025

- ~6,300 primary CN patients were hospitalized with mean stay of ~4 days per infection[1]

PCN Disease Burden: Infections & Symptoms[2]



1. Komodo Claims Analysis, 2026 (based on 2025 data), 2. Infection sites per Donadieu J, et al. Expert Rev Hematol. 2021; Newburger PE, et al. Semin Hematol. 2013; Dale DC, SCNIR publications.

PCN is a High-Burden Disease with Significant Patient Desire for Better Treatment Options

Profound emotional impact	Social withdrawal and feelings of isolation from fear of the next infection
High motivation to manage disease	Patients want to control their disease given challenges with injections and antibiotics
High unmet need	Serious infections and hospitalizations are common in moderate-to-severe patients
Significant desire for oral option	An oral therapy that is simple, safe and effective with sustained disease control

X4 Burden of Illness survey, 2026 (n=110 primary CN patients; 80 adults).

Mavorixafor Clinical Program

Targeting the congenital, autoimmune, and idiopathic forms

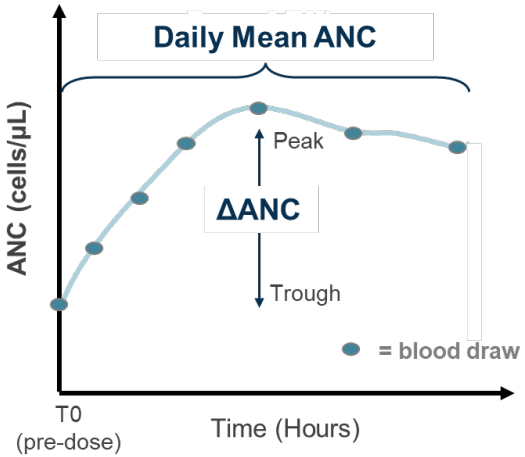
Phase 2 Proof of Concept Study of Mavorixafor in Chronic Neutropenia

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)

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

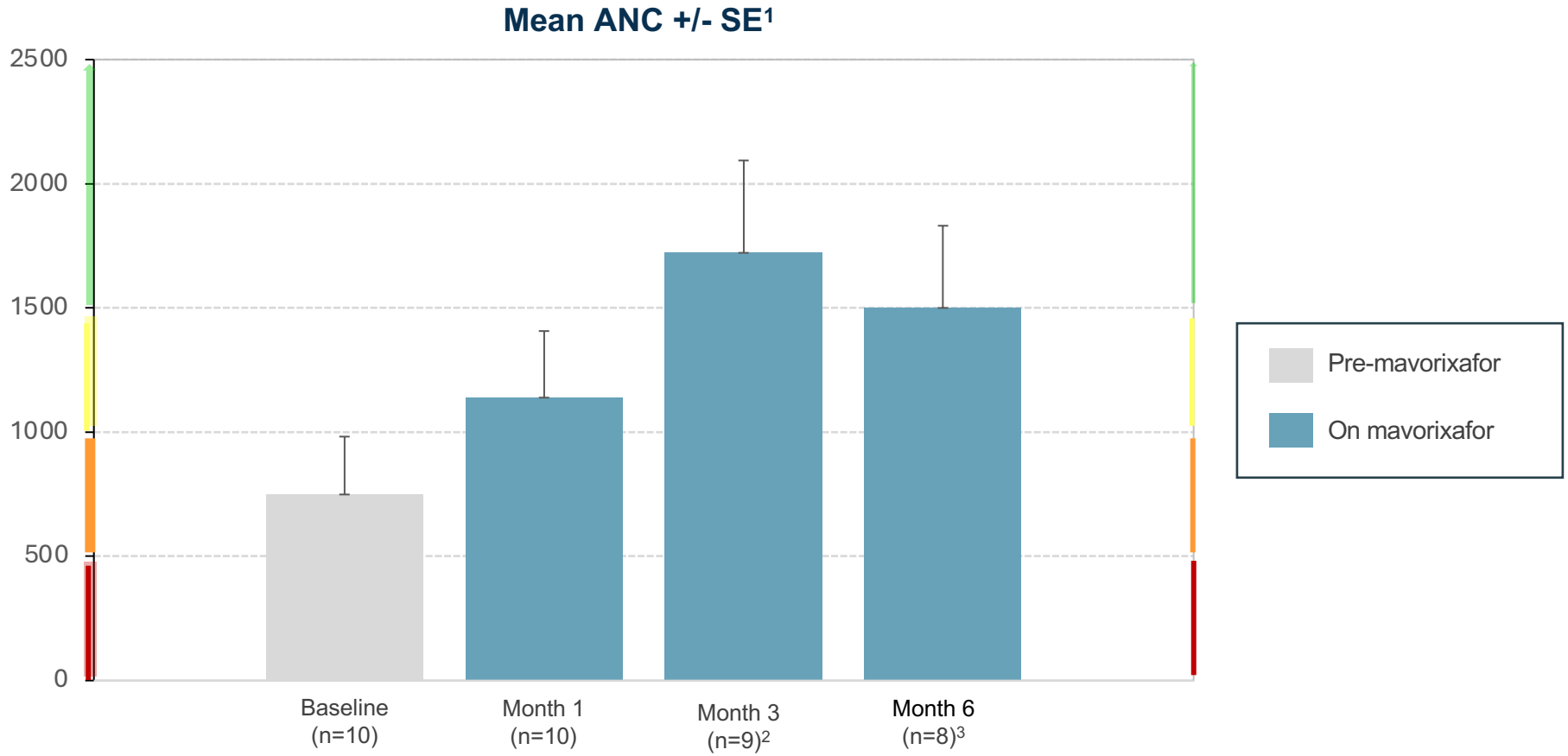
Mavorixafor + G-CSF
(mean baseline ANC 4034 cells/µL; range 470-11183 cells/µL)

	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

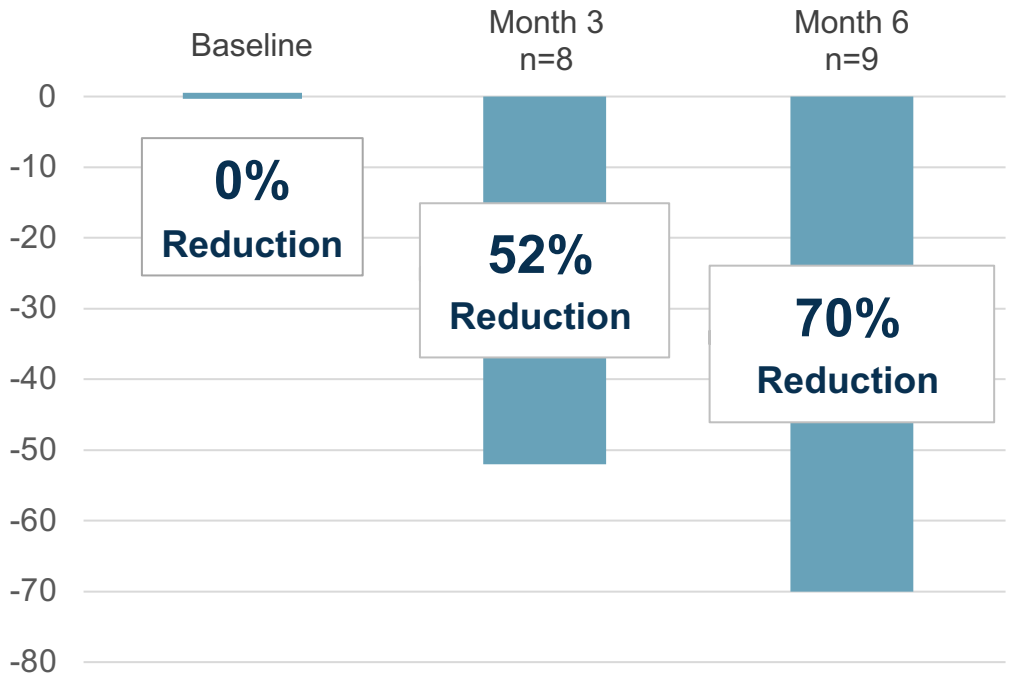
Mean ANC reached normal levels (ANC ≥ 1,500 cells/μL) at 3 and 6 months of treatment



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

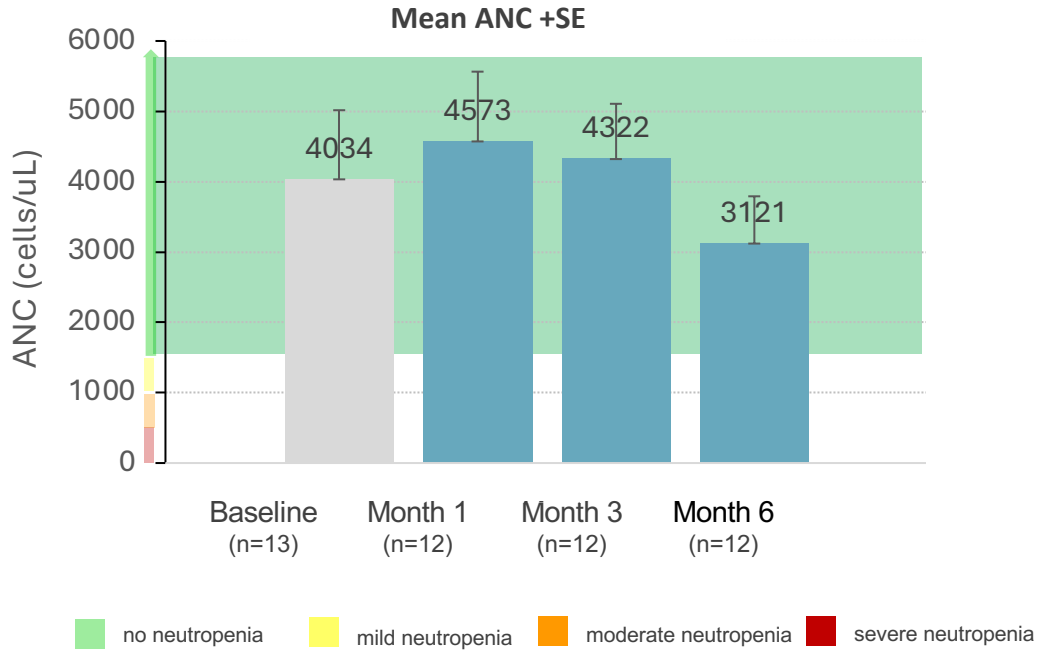
G-CSF:

- 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 maintained at normal levels (>1,500 cells/μL) through Month 6
- Data demonstrates the potential to reduce G-CSF usage with mavorixafor

Data Demonstrates the Potential to Reduce or Stop G-CSF Usage with Mavorixafor

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

Mean ANC in patients taking Mavorixafor + G-CSF



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

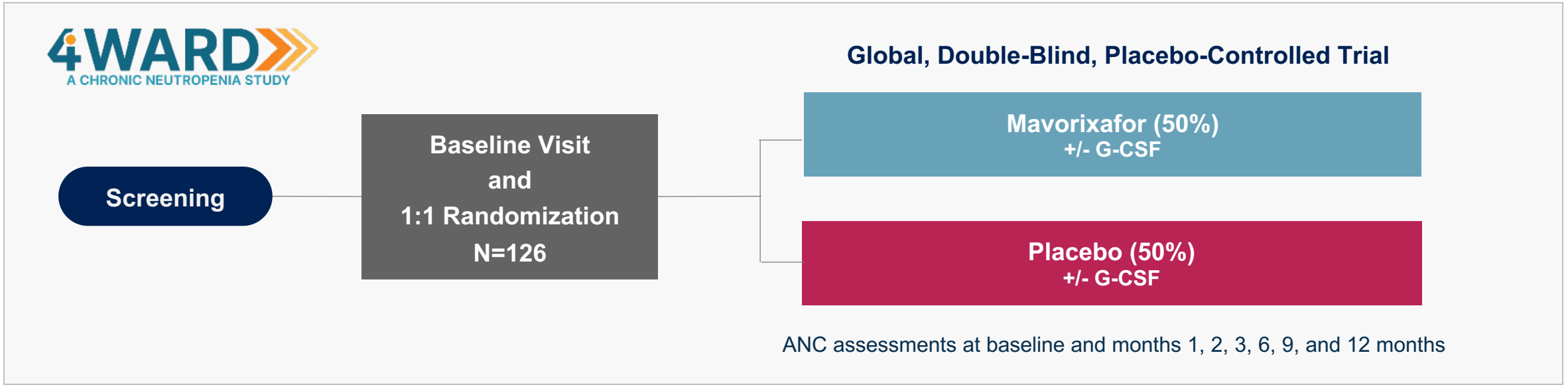
All mild to moderate

	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)

TEAE: treatment-emergent adverse event.

Pivotal Phase 3 4WARD Trial in Primary Chronic Neutropenia (PCN)

Full enrollment (N=126) expected Q4 2026 with top-line data expected Q1 2028



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)**

- Endpoints powered to 93% and 89%, respectively, for the ITT population

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

4WARD Baseline Characteristics

4WARD
A CHRONIC NEUTROPENIA STUDY

DISEASE SEVERITY

Evenly divided between moderate and severe disease

AGE

36 years

Median age

SEX

70%

Female

BASELINE ANC

483 cells/µL

Median ANC

INFECTION HISTORY

68%

of participants with 3+ infections at baseline

G-CSF USE

37%

on concurrent G-CSF

Blinded, pooled data as of September 2026.

4STEPS: A Phase 2b G-CSF Dose Reduction Trial with Mavorixafor

4STEPS
A CHRONIC NEUTROPENIA STUDY

DESIGN

Phase 2b

Single-arm, open-label, multicenter study

ENROLLMENT CRITERIA

≥ 1000 cells/µL

Baseline ANC

ESTIMATED ENROLLMENT

~25 patients

On stable G-CSF therapy at study entry

TOTAL SITES

~25

In the U.S. and UK

OBJECTIVE

To investigate the safety and tolerability of G-CSF dosage reduction in mavorixafor-treated participants with acquired primary autoimmune and idiopathic chronic neutropenia disorders

PRIMARY SAFETY ENDPOINT

Safety and tolerability of mavorixafor

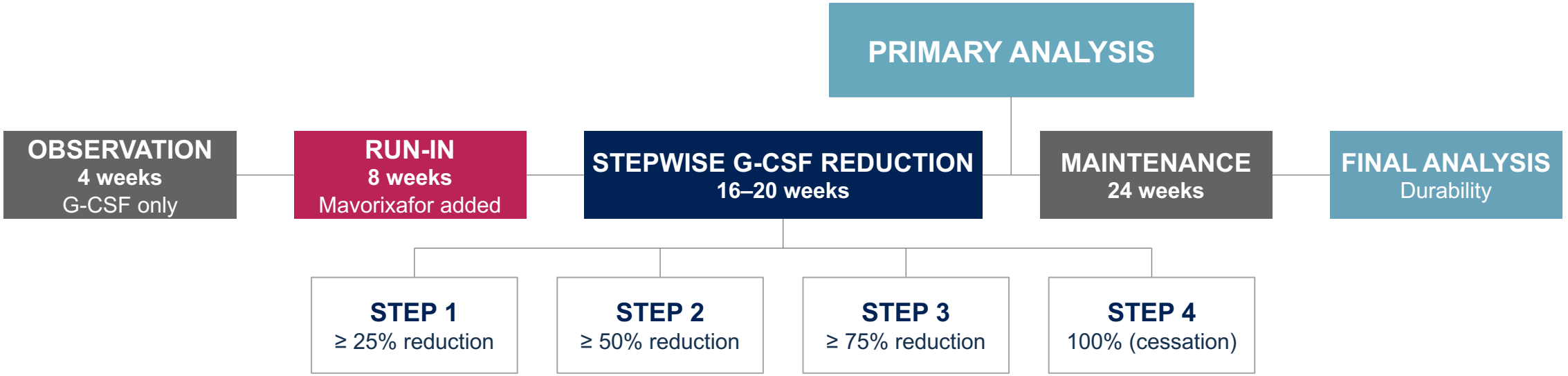
PRIMARY EFFICACY ENDPOINTS

- % participants achieving G-CSF cessation at 6 months*
- % reduction in average daily G-CSF dosage at 6 months*
- % participants achieving G-CSF dosage reduction ≥75% at 6 months*

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*W24 to W28 after starting study, depending on the timing of stepwise dose reductions.

4STEPS Trial Designed to Evaluate G-CSF Dose Reduction When Treating with Mavorixafor



ANC-based decision rule at each reduction visit

ANC level	Required action
≥ 1000 cells/μL	Reduce G-CSF dosage
750 – <1000 cells/μL	Maintain G-CSF dosage
500 – <750 cells/μL	Consider increasing G-CSF dosage
< 500 cells/μL	Increase G-CSF dosage

Mavorixafor
Commercial Opportunity

Hematologists View Mavorixafor as Differentiated From Current Standard of Care and Intend to Use It as the Dominant Agent for PCN

#1
Differentiator

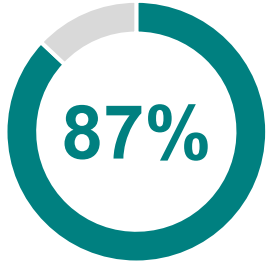
Oral route of administration[1,2]

#2
Differentiator

Greater infection control[1]



Report intent to prescribe mavorixafor[3]

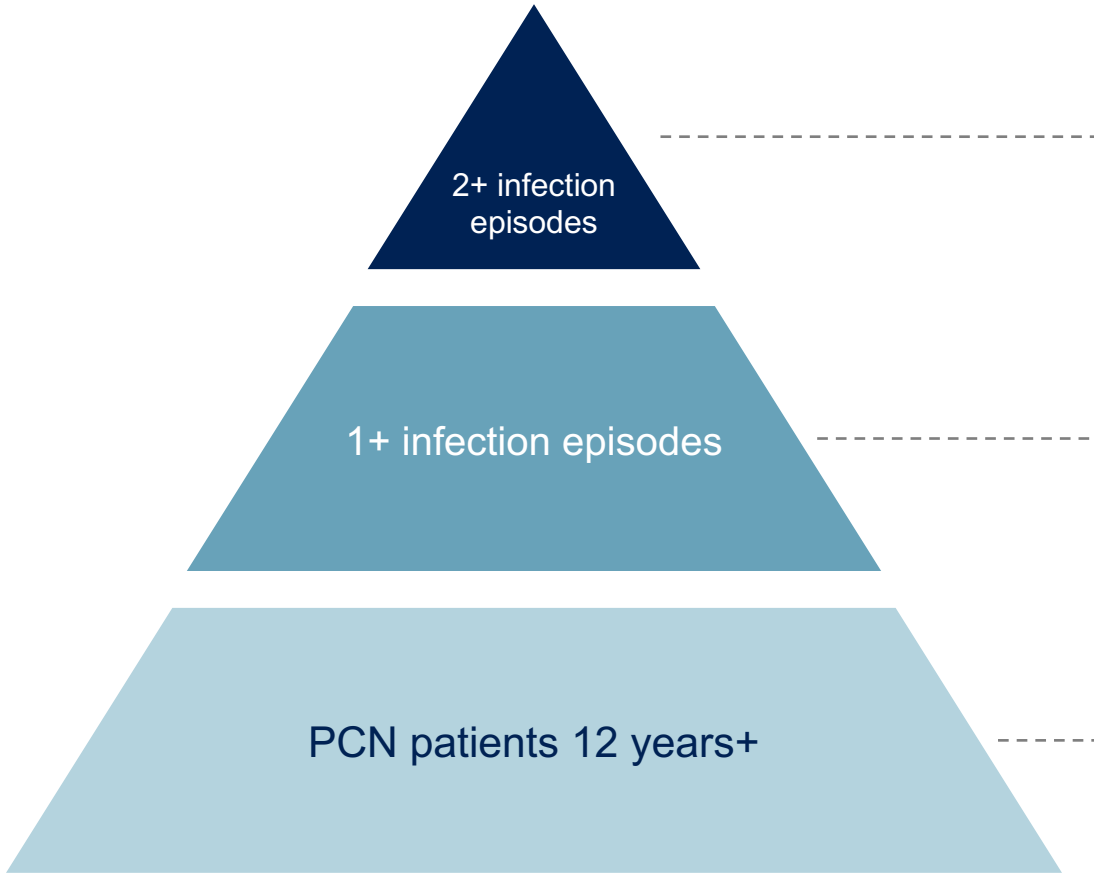


Rated mavorixafor favorably (>5/7)[3]

1. Clarivate qualitative market research 2026 n=20 60-minute interviews (n=3 PCP; n=6 community hematologist; n=11 academic hematologist/oncologist). 2. US hematology survey, 2024 (n=95). 3. X4 quantitative survey, 2025 (n=100).

U.S. Prevalence of PCN

Consistent and validated across multiple platforms



ANC < 1,000 cells/µL

~15,000 patients

2+ infection episodes in 2025 (symptomatic), includes ER visits and hospitalizations

ANC <1,000 cells/µL

~30,000 patients

1+ infection episodes in 2025 (symptomatic)

ANC <1,500 cells/µL

~95,000 patients

PCN patients aged 12+, active in 2025 with confirmed PCN Dx Code (symptomatic & asymptomatic)

Source: Komodo Healthcare longitudinal claims analysis, Jan 2018 to Dec 2025.

X4 is Well Positioned to Capture the ~15,000 Patient Base Case

~15K
Patients



Patient Profile

- **ANC < 1,000 cells/µL**
- **2+ Infections**
- **Symptomatic**

1. **4WARD includes patients spanning the base case:** Enrolls patients age 12+ with recurrent infections, on or off G-CSF

2. **Patients live with recurrent infections:** Every patient in the base case has two or more infections a year.

3. **Infections are often serious:** About 1 in 3 had an infection-related ER visit or hospital admission in the past year for an average of 4 days.

Source: Komodo Healthcare longitudinal claims analysis, Jan 2018 to Dec 2025.

Mavorixafor Represents a Significant Initial Market Opportunity with Potential Upside

~15K

Patients

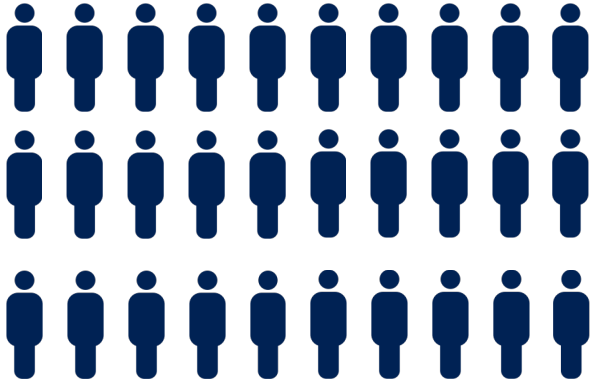


Patient Profile

- **ANC < 1,000 cells/µL**
- **2+ Infections**
- **Symptomatic**

~30K

Patients



Patient Profile

- **ANC < 1,000 cells/µL**
- **1+ Infections**
- **Symptomatic**

Source: Komodo Healthcare longitudinal claims analysis, Jan 2018 to Dec 2025.

Corporate Overview

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

Financial Position

Cash Runway Expected into Q1 2029

September 30, 2026	
Cash	**Fully Diluted Common Stock Outstanding**
$187.7 M	144.4 M

Fully diluted common stock outstanding includes pre-funded warrants

X4 on Track to Deliver Pivotal Phase 3 Data in 2028

Potential FDA approval late 2028/early 2029



- C-Suite team experienced in hematology drug approvals and product launches
- Blue chip investor base established after PIPE/CMPO
- Cash runway expected into 2029



- Proof of concept for mavorixafor in PCN from Phase 2 study
- Topline 4WARD Phase 3 data expected in Q1 2028; approval anticipated late 2028/early 2029
- Phase 2 4STEPS G-CSF dose reduction data expected in 1H 2028



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

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