



Nasdaq: XFOR

Virtual Investor Day

October 29, 2026

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Introduction & Corporate Overview

Adam Craig, MD, PhD
Executive Chairman

Meet the Speakers



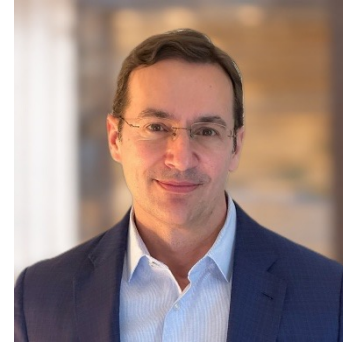
Adam Craig, MD PHD
Executive Chair



John Volpone
President and COO



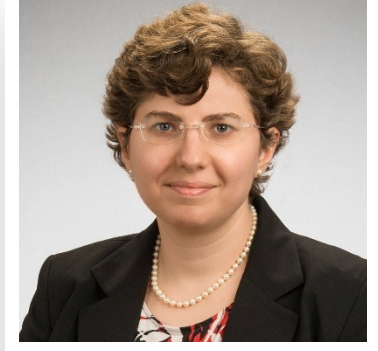
David Kirske
Chief Financial Officer



Michael Massello, MBA
SVP Market Strategy and
Development



Shreyans Gandhi,
MBBS, MD, FRCPATH



Julia Warren, MD, PhD



Agenda

Topic

Speaker

Introduction & Corporate Overview

Dr. Adam Craig

Mavorixafor Clinical Program

John Volpone

Market Opportunity in Primary Chronic Neutropenia

Michael Massello

KOL Fireside Chat

Dr. Adam Craig, Dr. Shreyans Gandhi &
Dr. Julia Warren

Question & Answer Session

Delivering on the Promise of CXCR4 Antagonist Mavorixafor

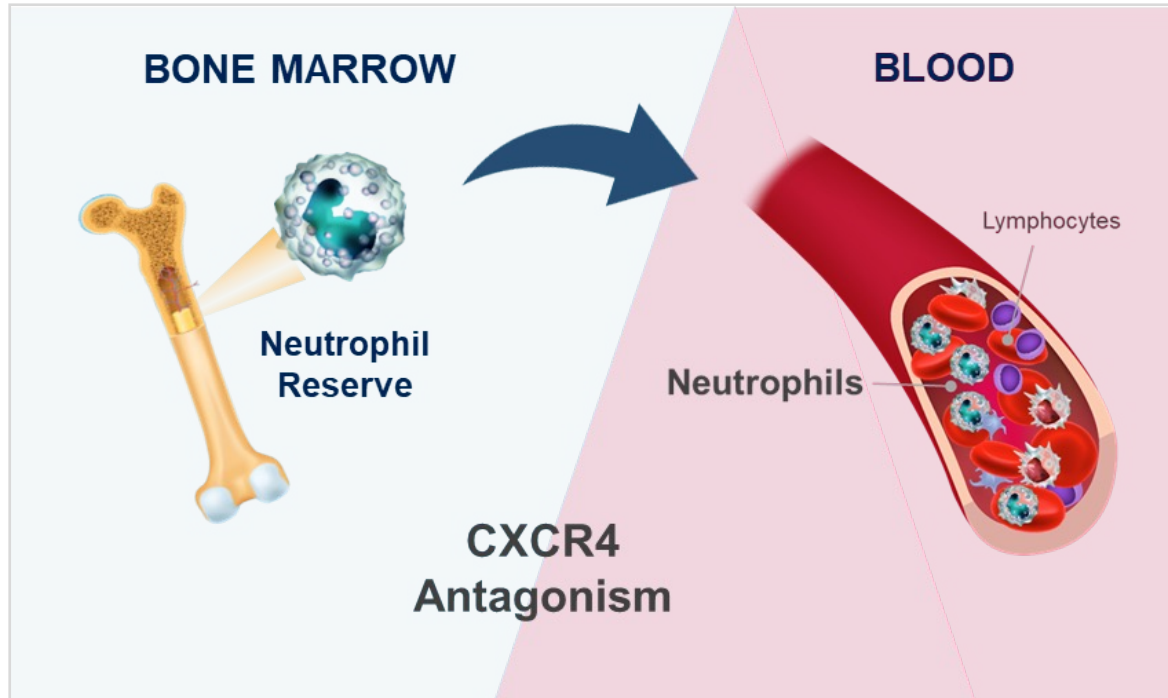
Focused on improving the lives of people with rare hematology diseases

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
- Plan to initiate Phase 2b clinical trial to demonstrate how to safely reduce a patient's G-CSF dosage following initiation of treatment with mavorixafor

Mavorixafor: An Oral Agent Designed to Alleviate Neutropenia

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



Modified figure from reference¹

Targeted Mechanism

- CXCR4 regulates movement of white blood cells throughout the body²
- **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

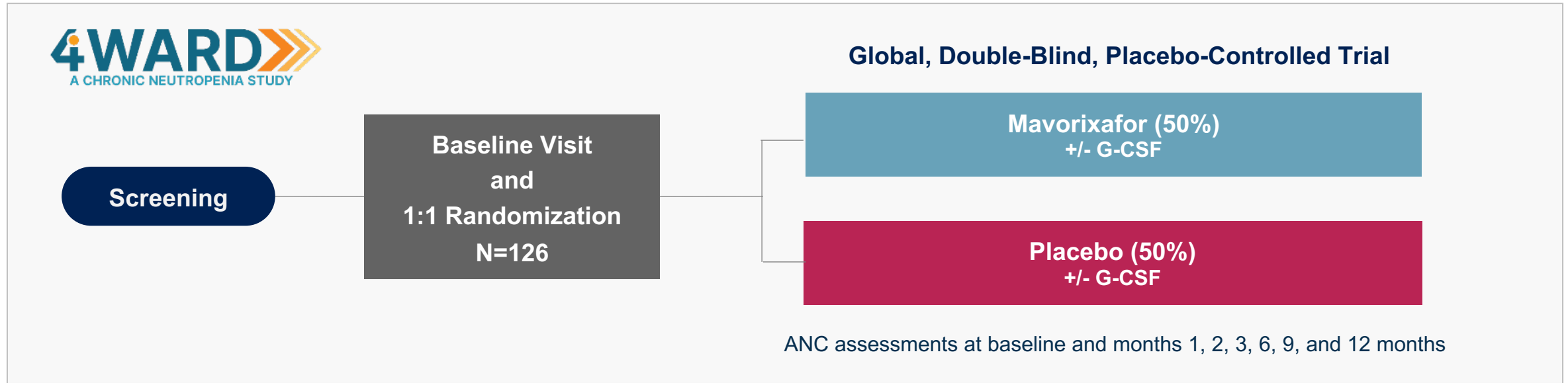


Mavorixafor Clinical Program

John Volpone
President & Chief Operating Officer

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/ μ L (moderate and severe disease)
- Infection History: 2 or more infections requiring intervention within last 12 months

Primary Endpoints: **ANC response (>500 cells/ μ L)** 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



DISEASE SEVERITY

Evenly divided between moderate and severe disease

AGE

36 years

Median age

SEX

70%

Female

BASELINE ANC

483 cells/ μ L

Median ANC

INFECTION HISTORY

68%

of participants with 3+
infections at baseline

G-CSF USE

37%

on concurrent G-CSF

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

DESIGN

Phase 2b

Single-arm, open-label,
multicenter study

ENROLLMENT CRITERIA

≥ 1000 cells/ μ L

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

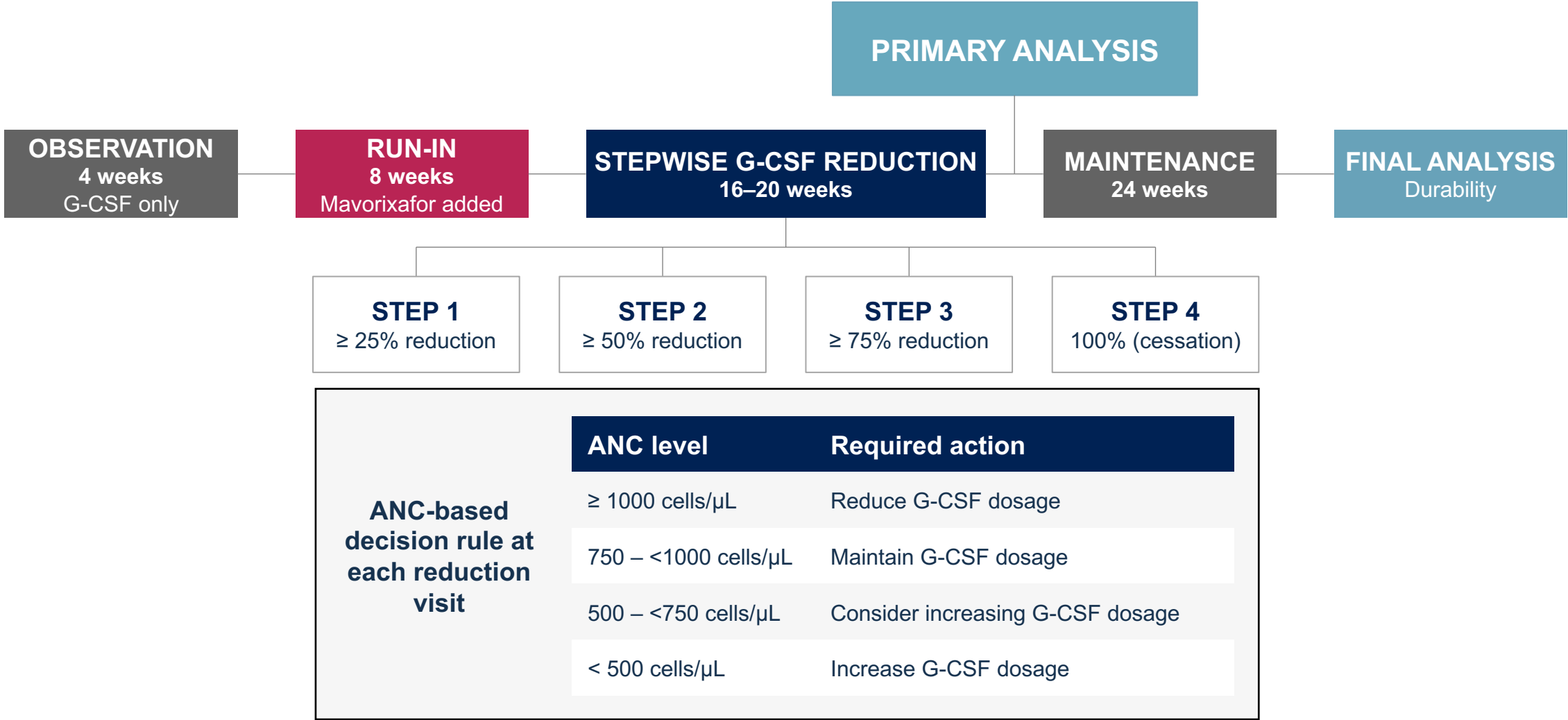
PRIMARY SAFETY ENDPOINT

Safety and tolerability of
mavorixafor

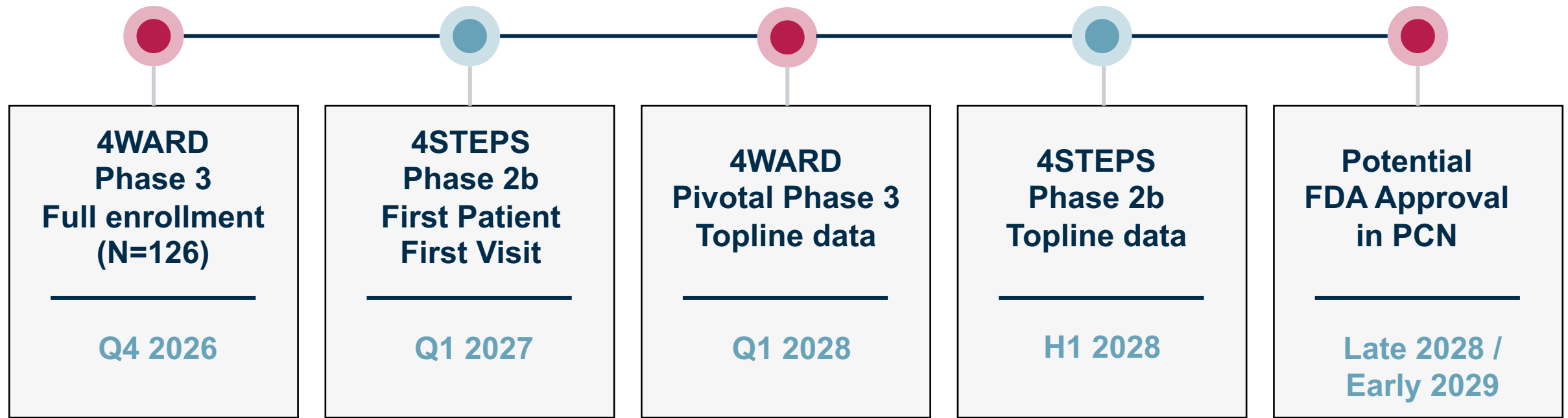
PRIMARY EFFICACY ENDPOINTS

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

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



Primary Chronic Neutropenia (PCN) - Development Timelines





Mavorixafor Commercial Opportunity

Michael Massello
SVP Market Strategy and Development

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

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

Market Research Establishing the PCN Commercial Opportunity



Claims Analysis

Veeva claims analysis 2023-2025

Identified the symptomatic PCN population

Komodo claims analysis 2023-2025

Independently confirmed the symptomatic PCN population

Longitudinal claims analysis

Mapped the Patient Journey from diagnosis to treatment



Physicians

Quantitative demand study (n=100)

Qualitative disease state messaging (n=50)

Patient Journey qualitative research (n=30)

Advisory Boards



Patients

CONNECT PCN

Patient Burden of Illness/Burden of Treatment Survey (n=110)

Assess the impact of PCN on patient quality of life

Advisory Boards

PCN Drives Substantial Healthcare Utilization and Cost

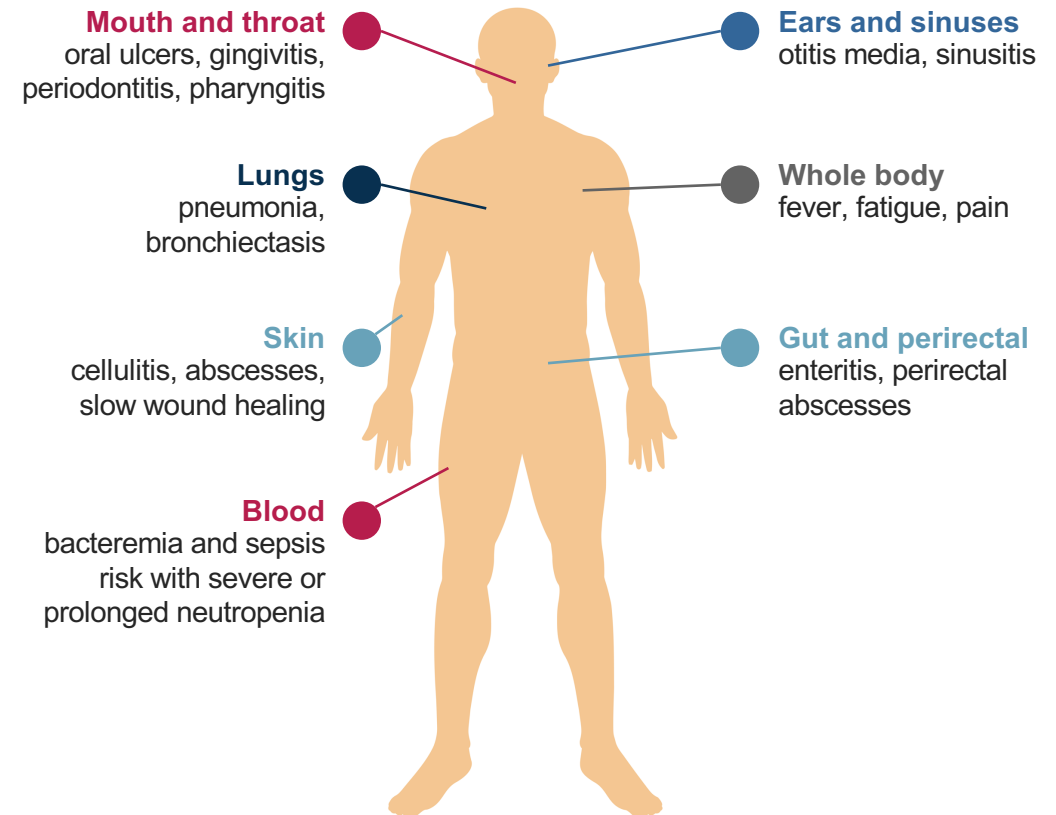
Recurrent and serious infections can lead to serious complications and hospitalization

- 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 infection¹

PCN Disease Burden: Infections & Symptoms²



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

Adults Living with PCN Restrict Everyday Life to Avoid Infection

Because of worry about infection, adults with primary CN...



...at least some of the time

Avoided crowded places such as restaurants or large events

88%

Felt especially alert or on guard around others

81%

Avoided public transportation

78%

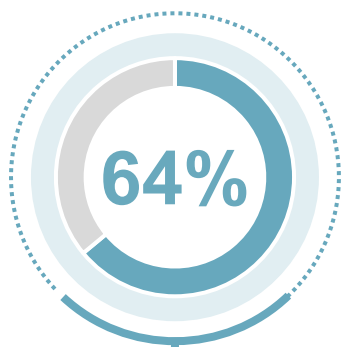
Avoided social gatherings

74%

Avoided traveling or vacations

69%

Adults with PCN Report a Significant Impact on Work



Missed Work

(n=51/80)

Mean 9.6 workdays missed
in Past Year



Reduced Work Hours

(n=43/80)



Missed a Promotion or Chose to Not Pursue a New Role

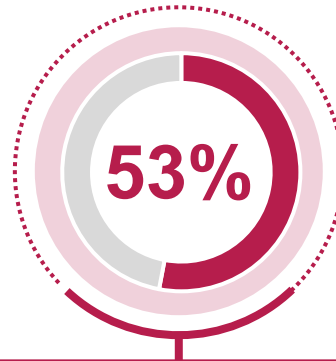
(n=16/80)



PCN has Profound Emotional Impact on Adolescents and Children



Missed school days
(n=18/30)



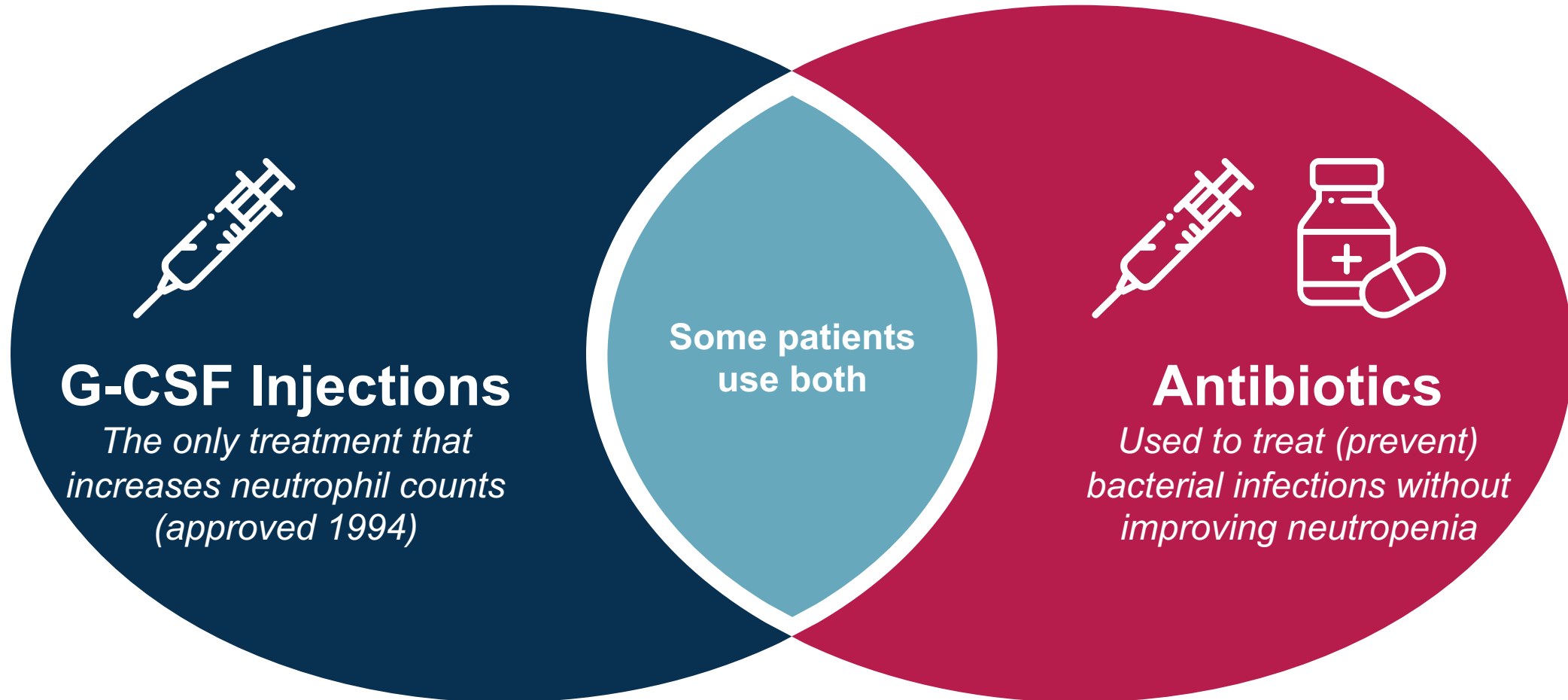
**Reduced participation
in afterschool
activities** (n=16/30)



**Avoided interacting
with others**
(n=18/30)

Management of Symptomatic PCN

Two main therapeutic options exist today with no new therapies in 30 years



G-CSF Injections Carry Significant Burden

The Burden of G-CSF injections



Injection burden

- Frequent, painful
- 66% of G-CSF patients have injection fatigue¹

Peaks and troughs: gaps in protection

Neutrophil counts rise and fall between doses²

Long-term safety

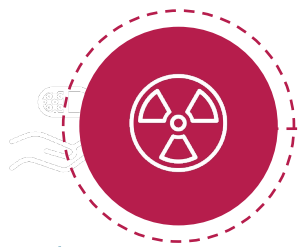
20%+ of secondary malignancies with high dose G-CSF³

Bone pain and quality-of-life impact

- 57% have Bone Pain¹
- 61% have Fatigue¹

Antibiotics Can Treat Infections But Do Not Address the Underlying Neutropenia

The Burden of Antibiotics



Disease not addressed

Treats infections but does not raise neutrophil counts¹

Toxicity

Cumulative exposure carries its own side effects and drug interactions²

Drug resistance

Long-term use can drive antimicrobial resistance³

The PCN Patient Journey and Where Care is Delivered

The Patient Journey

01

Initial Consultation

Patients typically first visit Primary Care Provider (PCP)

02

Diagnosis

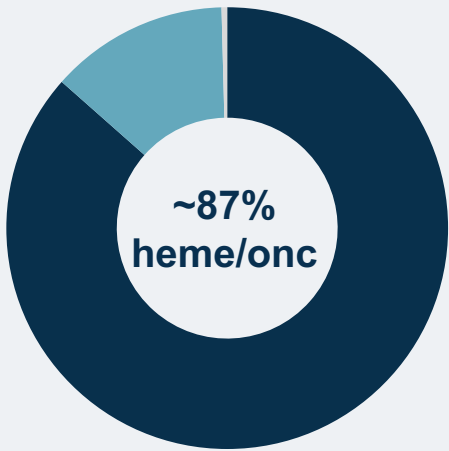
Confirmatory diagnosis by a hematologist/ oncologist (heme/onc)

03

Treatment

G-CSF is primarily prescribed by heme/oncs; antibiotics are managed by PCPs and heme/oncs

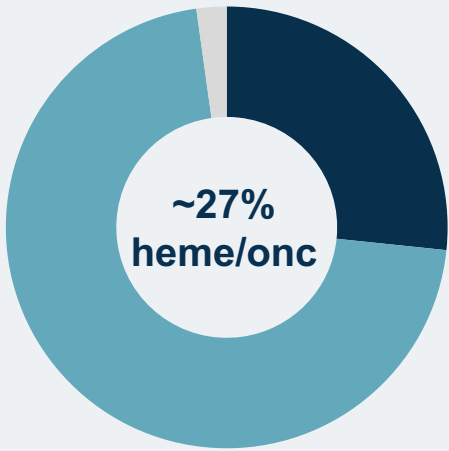
G-CSF Patients: Care Teams



■ Heme/onc involved
■ PCP / NP/PA, no hem/onc
■ Other

- G-CSF use, approximately 80% prefilled syringes and 20% vials
- Both formats require injections

Antibiotic Patients: Care Teams



■ Heme/onc involved
■ PCP / NP/PA, no hem/onc
■ Other

- 100% of patients requiring hospitalization or an ER visit received antibiotics
- Among these patients approximately 72% required IV/injectable administration

Hematologists and Patients are Aligned on the Key Drivers for a New Treatment

Health Care Provider (HCP) Treatment Goals

Fewer and less severe infections & hospitalizations

Increase and stabilize neutrophil counts

A safe and tolerable treatment option

Patient Treatment Goals

To stay injection-free and out of the hospital

Relief from fatigue, pain and constant vigilance

A treatment that fits daily life for the long term



"We are so frustrated for patients who have no other treatments than just a few modalities...ready to embrace anything new to treat this disease."

"Oral would be a game-changer in PCN"

**– Hematologist,
Advisory Board, Sept 2026¹**



"I'm scared to go out of my house and scared to go on vacations and won't leave the house without 3 antibiotics. I want control of my life back and to focus on the moments that matter!"

**– Patient,
Advisory Board, Sept 2026²**

Mavorixafor is a Highly Differentiated Therapy in Development for PCN



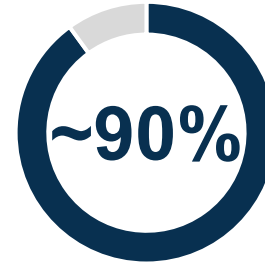
Hematologists View Mavorixafor as Differentiated From Current Standard of Care and Intend to Use It as the Dominant Agent for PCN



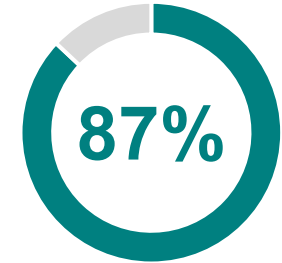
Oral route of administration^{1,2}



Greater infection control¹



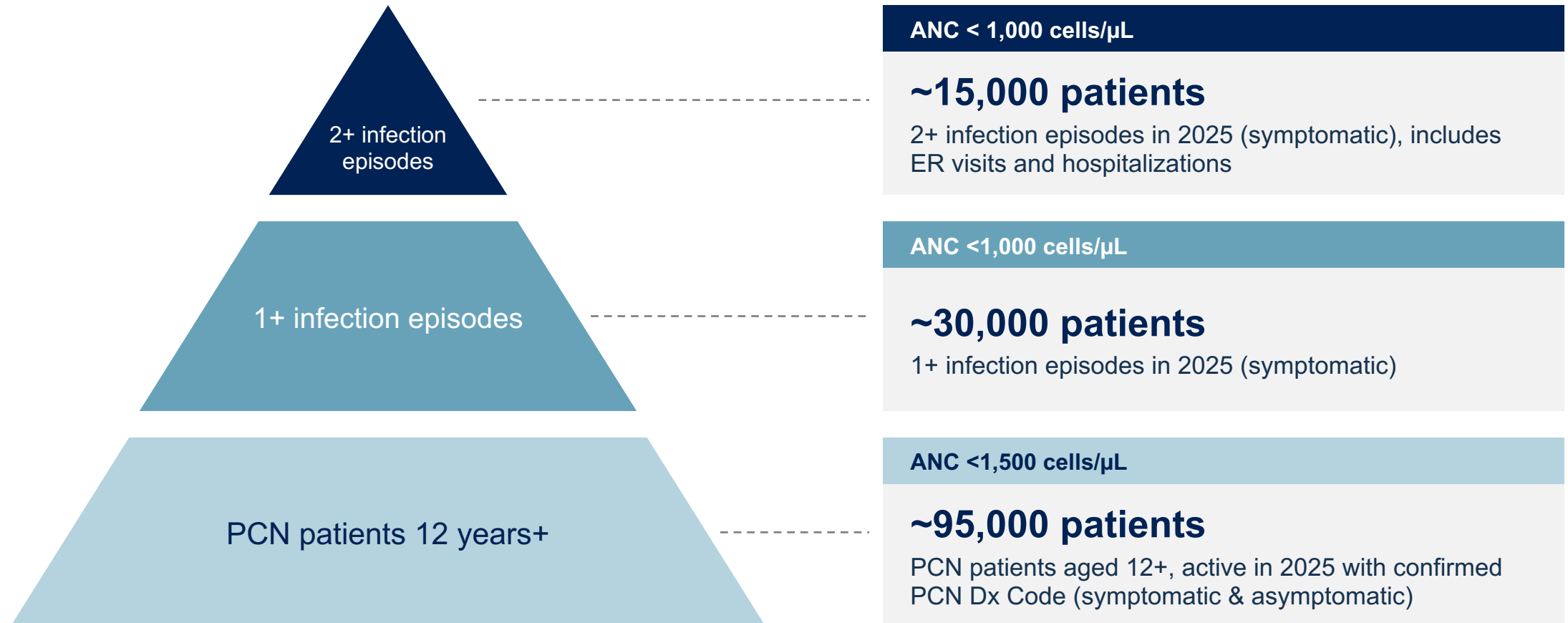
Report intent to prescribe mavorixafor³



Rated mavorixafor favorably (>5/7)³

U.S. Prevalence of PCN

Consistent and validated across multiple platforms



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



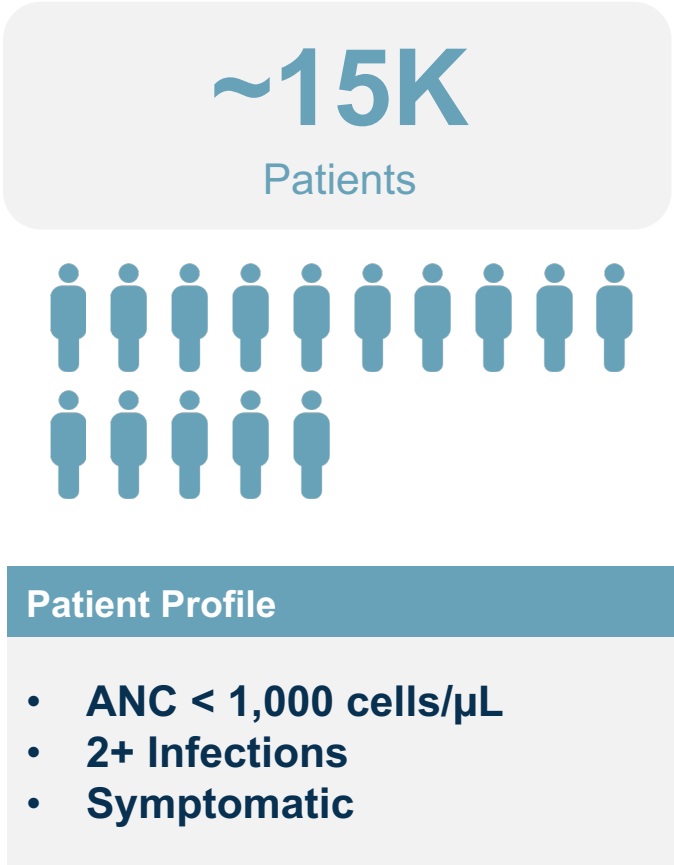
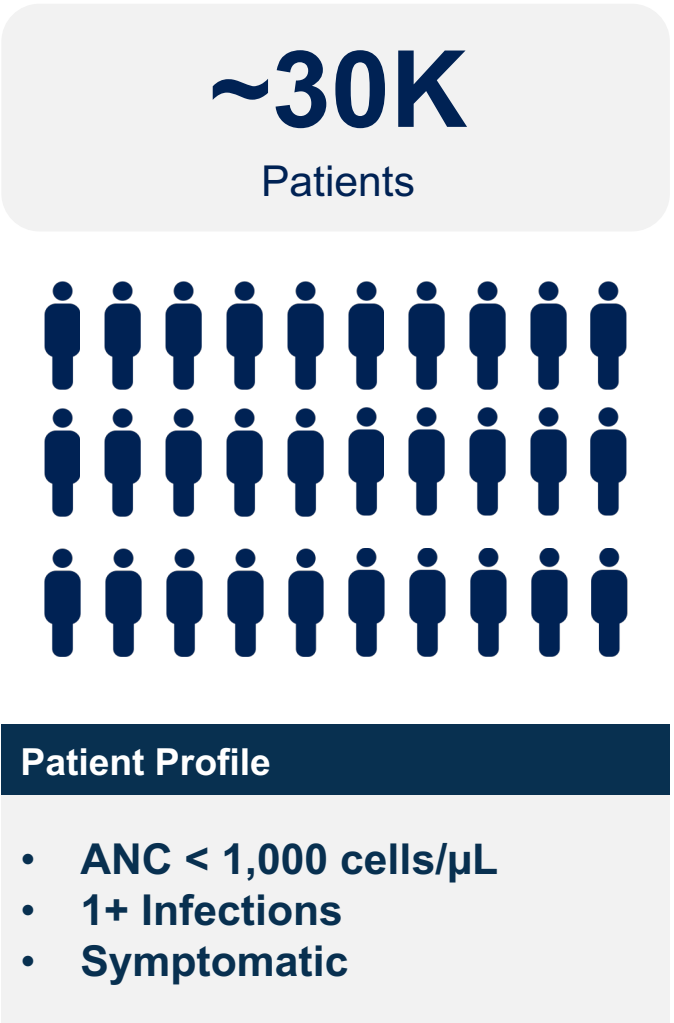
2

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.

Mavorixafor Represents a Significant Initial Market Opportunity with Potential Upside





Fireside Chat

Dr. Adam Craig

Dr. Shreyans Gandhi

Dr. Julia Warren

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