X4 PHARMACEUTICALS

April 2024

PROGRESS 4 PATIENTS

Developing the first oral treatments for chronic neutropenic disorders

Forward-Looking Statements

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X4 Overview: A Compelling Investment Opportunity

Sharp focus on rare immunodeficiencies

Advancing innovative medicines to address significant unmet needs in people with rare immunodeficiencies and few to no treatment options

A pipeline in a product: mavorixafor

An orally available CXCR4 antagonist being developed as a once-daily therapy for a range of chronic neutropenia (CN) indications and WHIM[1] syndrome

Significant regulatory & clinical milestones expected in 2024:

✓ U.S. NDA accepted for priority review of mavorixafor in WHIM syndrome

- PDUFA date of April 30, 2024: PRV[2] eligible if approved for WHIM
- Pivotal, global Phase 3 CN trial to begin in 1H 2024
- Phase 2 CN data (n>15) in 1H 2024
- Global regulatory milestones
- Exploring further indications for mavorixafor

Unparalleled expertise in CXCR4[3] biology

Antagonizing the CXCR4/CXCL12 axis has been proven to increase the mobilization of white blood cells, including neutrophils and lymphocytes (B cells and T cells)

Cash runway[4] expected to fund operations into 2025

Strong balance sheet with possible upside from non-diluting financing opportunities, including potential monetization of Priority Review Voucher

1. WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis); 2. X4 eligible to receive a Priority Review Voucher (PRV) due to mavorixafor's Rare Pediatric Disease Designation in the U.S.; 3. C-X-C receptor type 4 (CXCR4) is a cell receptor that, with its ligand CXCL12, helps regulate the movement of immune cells in the body; 4. As of December 31, 2023.

Chronic Neutropenia: No Innovation in More Than 30 Years

~50,000[1]

U.S. Prevalence: total diagnosed with Chronic Neutropenia (CN)

~15,000[1]

Estimated subset with highest unmet need: minimal addressable market for mavorixafor in CN

Only One

Therapy Approved for Severe Chronic Neutropenia

Injectable Granulocyte Colony-Stimulating Factor (G-CSF)

- Inconvenient daily injections
- Frequent treatment-related/treatment-limiting bone pain and other adverse events

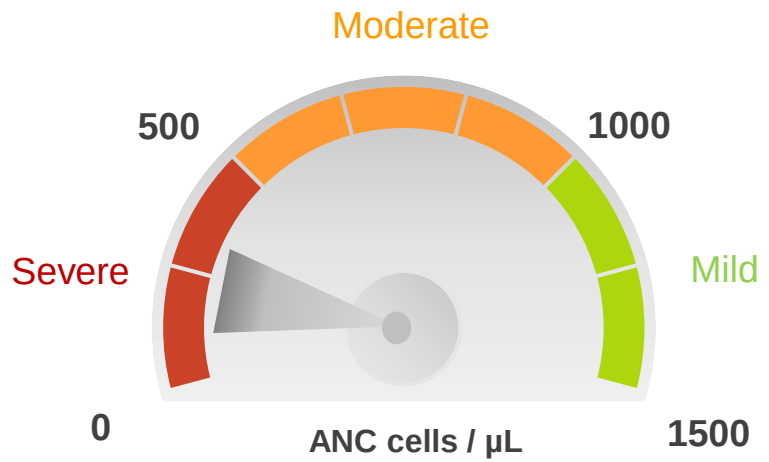
Clear Need for Increased Options for Patients: Efficacious, Oral, Well Tolerated Treatments

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

Living With Chronic Neutropenia (CN): Risk of Serious, Life-Threatening Infections[1]

NIH Classification[2]	ANC Levels (cells/µL)	Infection Risk with Immunodeficiency[1]
Severe (Grade 4)	<500	Moderate to severe
Moderate (Grade 3)	500-1,000	Moderate to severe
Mild (Grade 2)	1,000-1,500	Minimal to severe
Non-clinical (Grade 1)	1,500 - LLN	No clinical impact

Infection Risk and Low ANC ("Neutropenia")



ANC = Absolute neutrophil count

- Frequent and/or severe infections are the primary clinical consequence of chronic neutropenic disorders[3]
- Infection frequency, severity, and duration are correlated with magnitude and duration of decreased ANC levels[4]
- Infections may lead to frequent hospitalizations or result in life-threatening complications, including death[5,6]

1. Jan Palmblad, Carlo Dufour, Helen A. Papadaki, *Haematologica*, Vol. 99 No. 7 (2014): July, 2014; 2. https://ctep.cancer.gov/protocoldevelopment/electronic_applications/docs/ctcae_v5_quick_reference_8.5x11.pdf;
3. Sicre de Fontbrune F, et al. *Blood*. 2015;126(14):1643-1650; 4.Bodey, GP, *et al*, *Ann Intern Med*, 1966. 5. Donadieu J, et al. *Expert Rev Hematol*. 2021;14(10):945-960; 6. Salehi T, et al. Iran *J Allergy Asthma Immunol*. 2012;11(1):51-56.

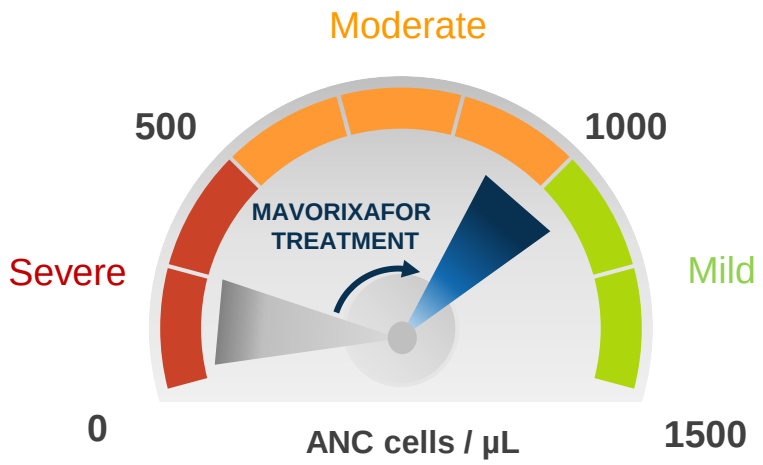
New Treatment Needed: A Well Tolerated Oral Option that Reduces Infection Risk

Mavorixafor: Potential to Deliver First Innovation in 30+ Years

Successful WHIM Phase 3 with NDA Accepted

PDUFA Date: April 30, 2024

Significant Increase in ANC Over 52 Weeks
~60% Reduction in Infection Rates



ANC = Absolute neutrophil count

Positive Phase 1 & 2 CN Data to Date Support Advancement to Phase 3

CN Phase 1b Results

- ANC increased by >500 cells/µL in 100% (n=25) after single-dose

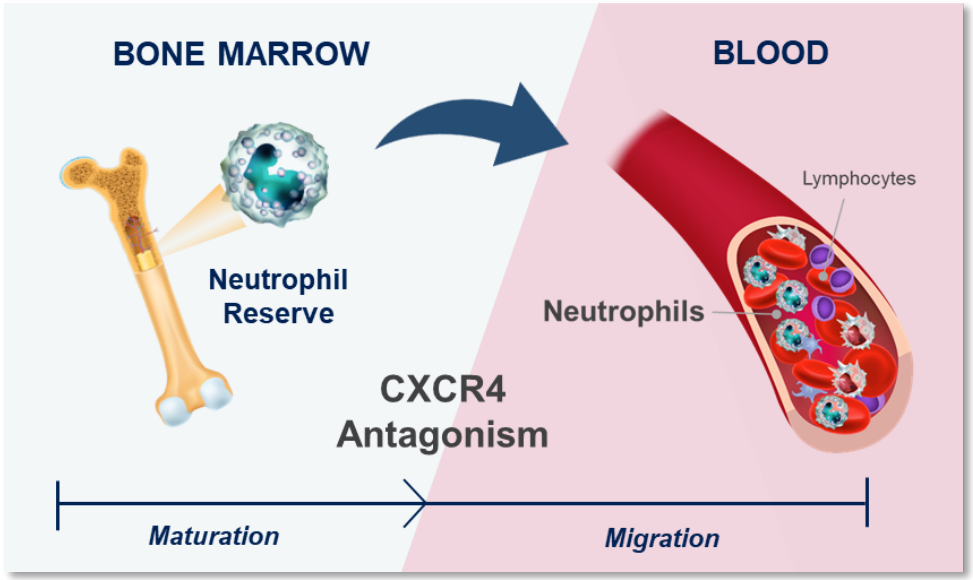
***Preliminary* CN Phase 2 Results**

- Durable increases in ANC[1] in all subjects reported to date (n=3)
  - Clinically meaningful (Δ ANC >500 cells/µL)
- Infection rates
  - No infections in all patients after month 2
  - No increase in infections despite reduction/withdrawal of G-CSF (n=2)

1. All subjects increased absolute neutrophil count (ANC) over first 3 months of dosing (when G-CSF maintained at fixed, baseline dose)

Mavorixafor: a Pipeline in a Product via CXCR4 Antagonism

Validated mechanism shown to alleviate neutropenia and lymphopenia



Modified figure from reference 1

Targeted Mechanism

- Neutrophils retained in the bone marrow by the CXCR4/CXCL12 axis, creating a “reserve”[2]
- **CXCR4 antagonism** results in migration of cells from the bone marrow, increasing circulating levels of neutrophils and lymphocytes[3,4]

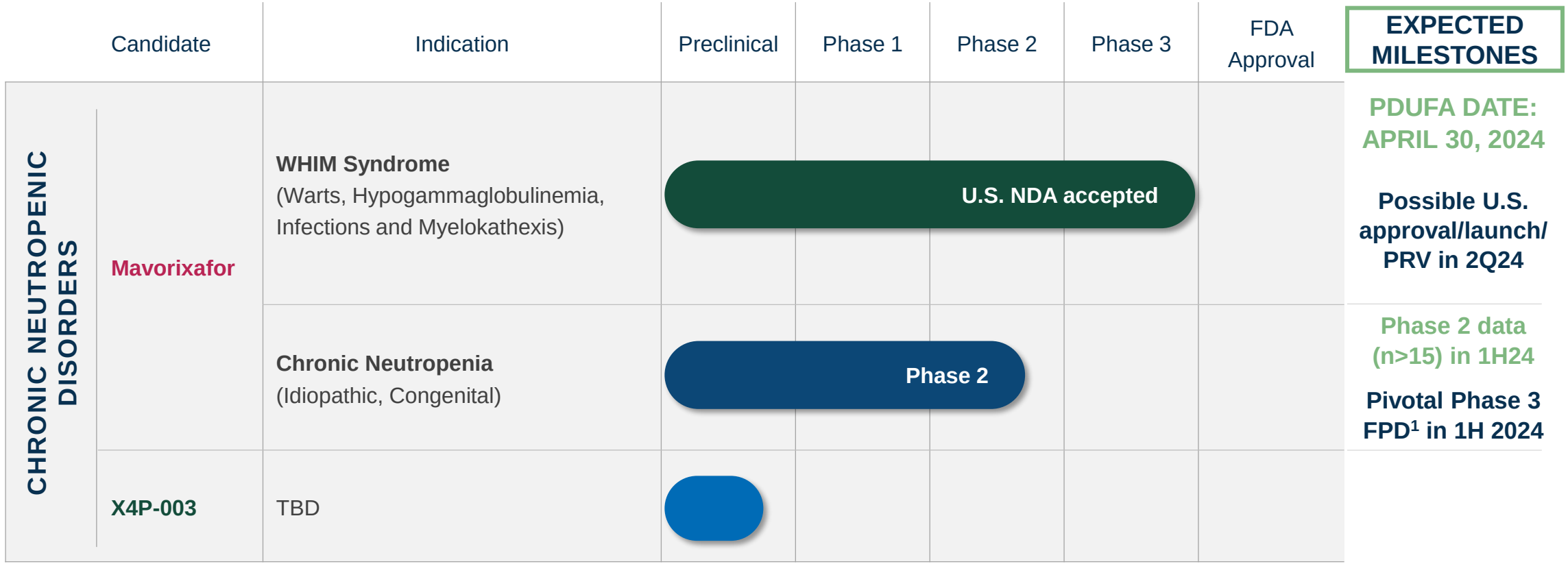
Potential Breakthrough With Oral, Once-Daily Treatment

- A once-daily, oral CXCR4 antagonist shown to raise blood levels of neutrophils and lymphocytes[5]
- Broad clinical potential across multiple rare immunodeficiencies
- For WHIM syndrome: mavorixafor granted Breakthrough Therapy Designation, Fast Track Designation, and Rare Pediatric Disease Designation in U.S. (PRV eligible); Orphan Drug Status in U.S. and Europe
- U.S. patent protection through 2038

1. Bainton DF (1980) The cells of inflammation: a general view. In Weissmann G (ed) *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. Stone, Nimalie D et al, *Antimicrobial Agents and Chemotherapy* vol. 51,7 (2007): 2351-8.

Advancing Mavorixafor in Chronic Neutropenic Disorders and WHIM Syndrome

Only oral candidate in clinical development across these indications



EXPECTED MILESTONES

PDUFA DATE: APRIL 30, 2024

Possible U.S. approval/launch/ PRV in 2Q24

Phase 2 data (n>15) in 1H24

Pivotal Phase 3 FPD[1] in 1H 2024

1. FPD = first participant dosed

WHIM Syndrome: Poorly Functioning Immune System with Chronic Neutropenia

Clinical symptoms driven by over-signaling in the CXCL12/CXCR4 pathway

Fewer than 1 in 4 WHIM patients present with all 4 manifestations making diagnosis a challenge

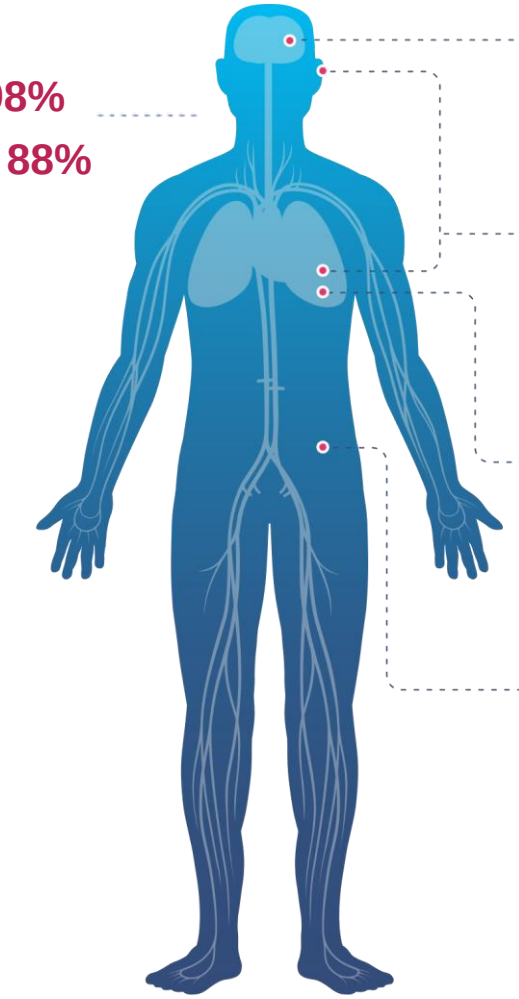
W Warts (**40% prevalence[1]**)
- can lead to HPV-related cancers

H Hypogammaglobulinemia (**65%**)
- low antibody levels

I Infections (**92%**)
- frequent bacterial and viral infections

M Myelokathexis (**100%**)
- retention of WBCs in bone marrow

A rare disease with no approved treatment options affecting at least 1,000 people[2] in the U.S.



Neutropenia 98%

Lymphopenia 88%

Sepsis 13%
Resulting from bacterial meningitis and bacteremia

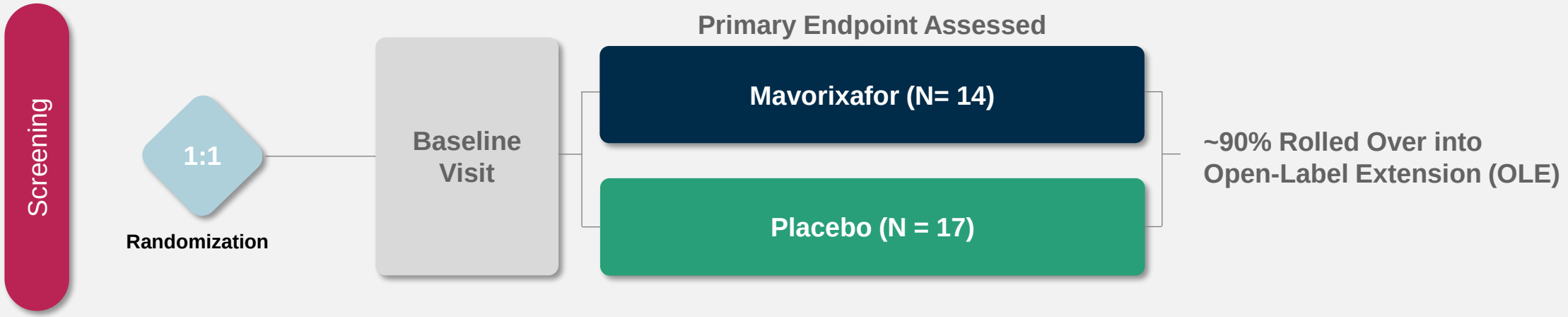
End-organ damage 20%
Bronchiectasis and hearing loss

Pneumonia 63%

Cancer 30%
Estimated overall risk by age 40, including HPV and EBV-associated malignancies

1. Prevalence numbers based on analysis of n=66 patients, as described in *Geier et al.* and on analysis on n=60 patients, as described in *Beaussant Cohen et al.*
2. Company market research. Qessential market research, 2019 and IPM.ai artificial intelligence study, 2020 (data on file).

Successful Pivotal, Global Phase 3 Clinical Trial in WHIM Syndrome



Baselines: 100% of patients had **severe chronic neutropenia (median ANC ~200 cells/µL)** and chronic lymphopenia (median ALC ~500 cells/µL)

Primary & First Secondary Endpoint: Time above Threshold (TAT) for ANC and ALC calculated as mean of 13, 26, 39, and 52-week assessments

Infection-Related Assessments: Data reviewed by independent, blinded, centralized adjudication committee for rate, severity, duration

Safety Assessments: Throughout the 52-weeks by an independent Data Safety Monitoring Board

POTENTIAL LABELED INDICATION: For the treatment of people aged 12 and above **diagnosed with WHIM syndrome**

ANC = absolute neutrophil count; ALC = absolute lymphocyte count

Mavorixafor Pivotal, Phase 3 Trial

Met primary and key secondary endpoints, demonstrating good tolerability[1]

4WHIM trial met primary endpoint of time above threshold (500 cells/µL) for absolute neutrophil counts (TAT_{ANC}) and certain key clinical benefit assessments

Mavorixafor achieved statistically significant increases in all white blood cells (WBCs) – including **neutrophils** & lymphocytes – versus placebo

Mavorixafor well tolerated with no treatment-related serious adverse events, no discontinuations due to safety events, no treatment-limiting toxicities

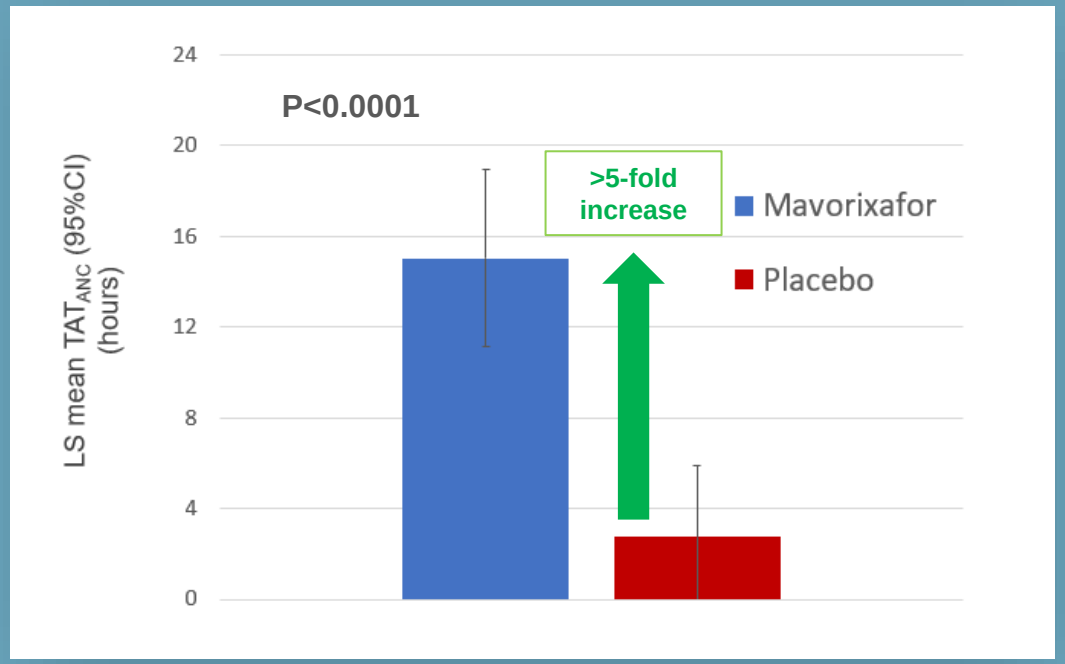
Significant reductions in the rate, severity and duration of infections associated with increases in TAT_{ANC} over the 52-week trial

1. Donadieu et al, EHA, 2023, Abstract S180.

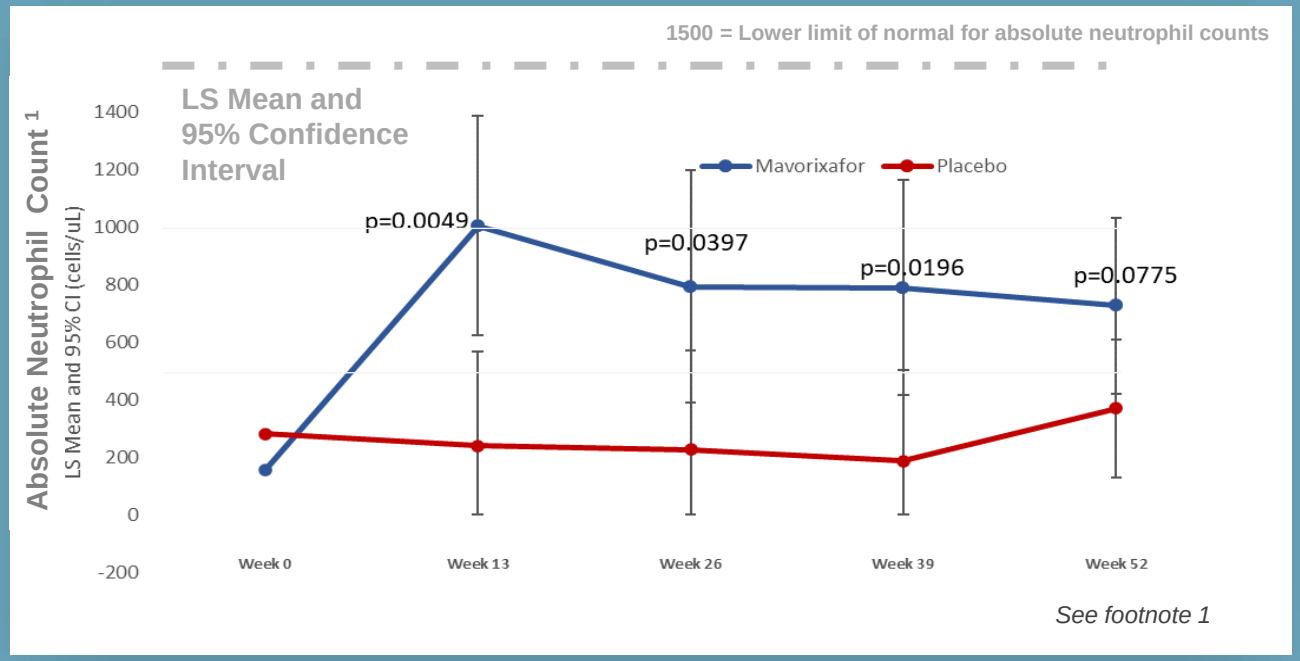
4WHIM Primary Endpoint Met; ANC Increases Maintained Over 52 Weeks

Intent-to-Treat (ITT) population analysis

Primary Endpoint



52-Week Absolute Neutrophil Counts (ANC)



Mavorixafor significantly improved the time above threshold of ANC (>500 cells/µL) over 52 weeks vs. placebo

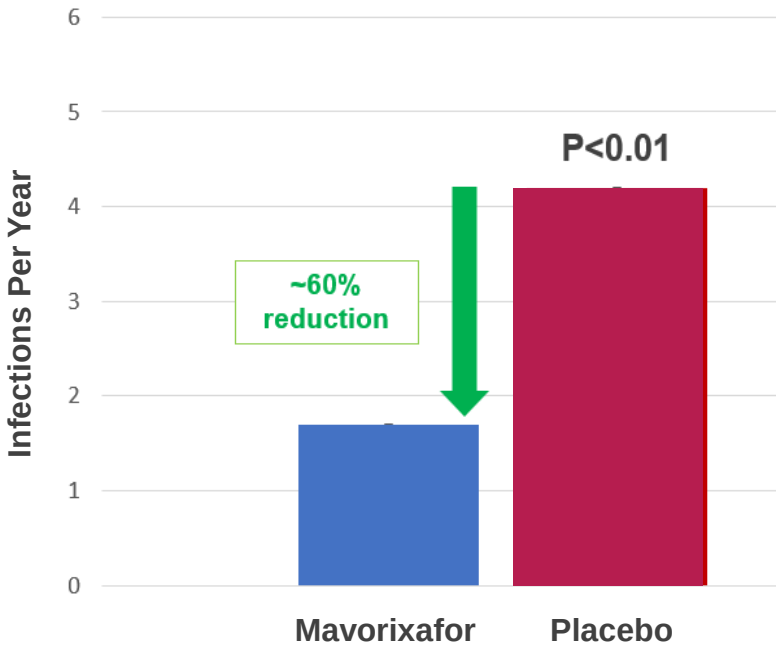
Mean TAT$_{ANC}$ was 15.04 hours for mavorixafor vs. 2.75 hours for placebo

Statistically significant, durable increases in neutrophils and lymphocytes (including B cells and T cells)[2]

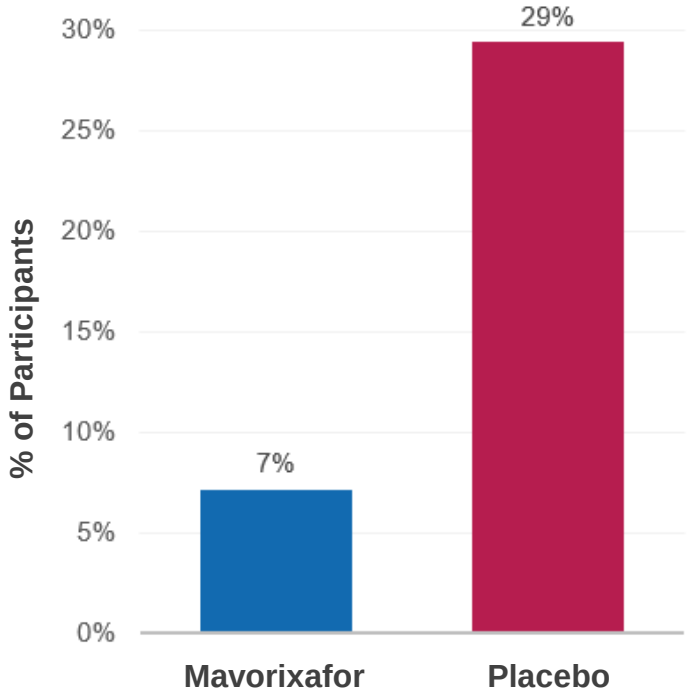
1. At week 52, 3 of 17 placebo patients were given mavorixafor in advance of their ANC measurements as they entered the open-label portion of the study. All data are included in ITT analysis. 2. Data on file.

Mavorixafor Reduced Annualized Infection Rate, Severity, & Duration

Annualized Infection Rate



Percentage of Patients Experiencing Grade 3 or Higher Infection Severity



Total Time with Infection (in days)

	Mavorixafor (n=14)	Placebo (n=17)
Mean (SD)	**14.1 (2 weeks)**	**49.1 (7 weeks)**
Median	8.5	32.0
Min, Max	0, 43	8, 134

FDA PDUFA Date of April 30, 2024

Preparations Underway for Potential 2Q 2024 U.S. Launch of Mavorixafor in WHIM Syndrome

Helping Build the WHIM Syndrome Community

- Establishing X4 as a trusted partner with key stakeholders
- Educating on WHIM syndrome, highlighting unmet need and enabling better patient identification
- Supporting earlier diagnoses to improve patient outcomes

Ensuring Broad Patient Access

- Communicating the mavorixafor value proposition
- Implementing distribution and supply chain
- Engaging with payers to ensure rapid reimbursement

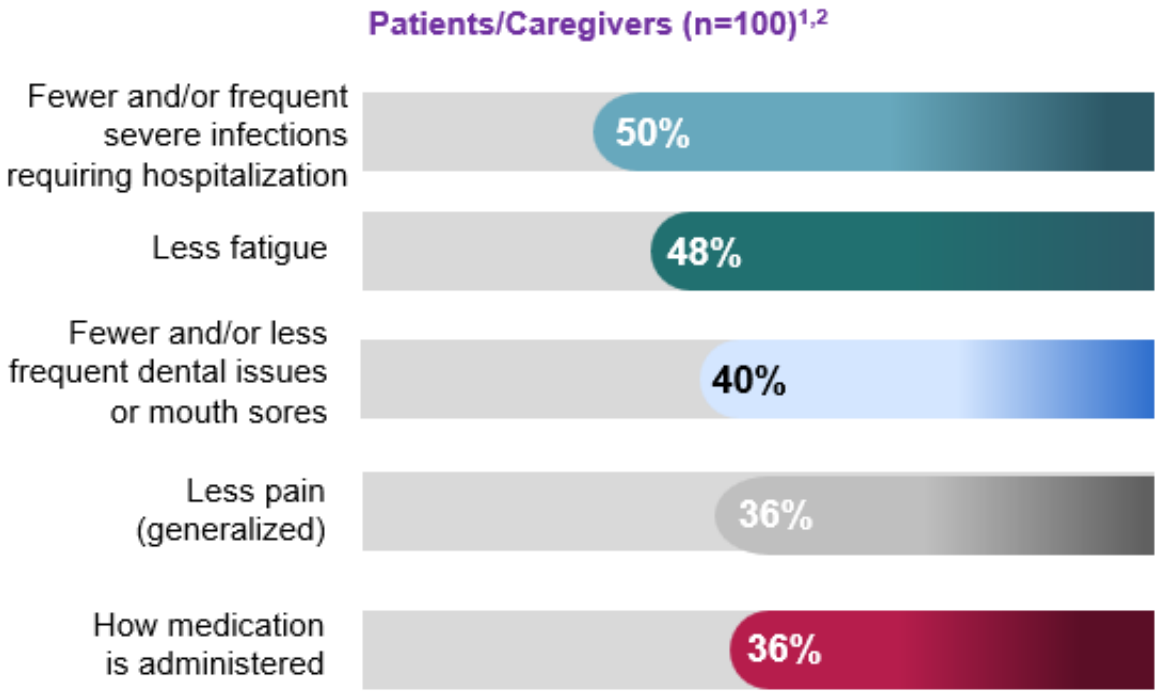
Evolving X4 to a Fully Integrated Biopharmaceutical Company

- Building a “fit for purpose” rare disease commercial organization
- Establishing infrastructure and capabilities
- Coordinating cross-functional launch readiness

What Makes a Difference to Chronic Neutropenia Patients and Their Physicians?

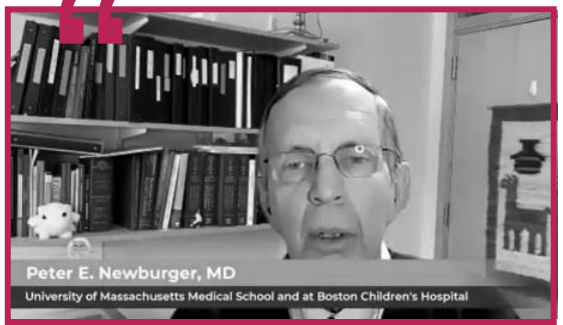
Expanded treatment options, ideally:

- Reduced infection rates
- Oral formulation
- Good safety profile
- Alternate therapy to injectable G-CSF and/or
- Reduced G-CSF-dose & related toxicities

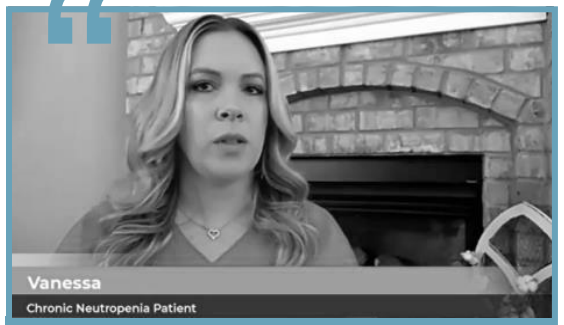


"There is a major diagnostic gap currently…, and also treatment options are extremely narrow"

"What I'd like to see with neutropenia would be a different way to administer the medicine… nobody likes needles."



"..an augmentation [of ANC] by 500 or 1000 would be adequate for clinical purposes of preventing infection."



".. I'm using Neupogen... I use it daily on a low dose... If I get the extreme bone pain…, I am unable to sleep…Yeah. It's unreal."

1. Ellis A, *et al*. poster presented at ASH Annual Meeting December 2022; 2. Other improvements included lower cost, fewer and/or less frequent short-term side effects from medication, fewer and/or less frequent gastrointestinal symptoms, fewer and/or less frequent long-term side effects from medication, and easier storage; respondents were allowed to select ≥1 options - total percentages may not add up to 100.

Segmentation of Unmet Needs in Chronic Neutropenia Patients

Chronic Neutropenia Patients Have A Range of Needs[1]

Alternative Monotherapy

Low neutrophil counts

High risk of infections

G-CSF intolerant / no alternative therapies

Highest need: well tolerated treatment that improves ANC and reduces infection risk

Improved Efficacy While On G-CSF

Remain neutropenic while on G-CSF

Infection risk remains

Experience bone pain, other AEs on G-CSF

Highest need: achieving normalized ANC and reduced infection risk, no additional adverse effects

Lower G-CSF Dose/Toxicity

Normalized ANC levels

Normalized infection risk

Experience bone pain, other AEs on G-CSF

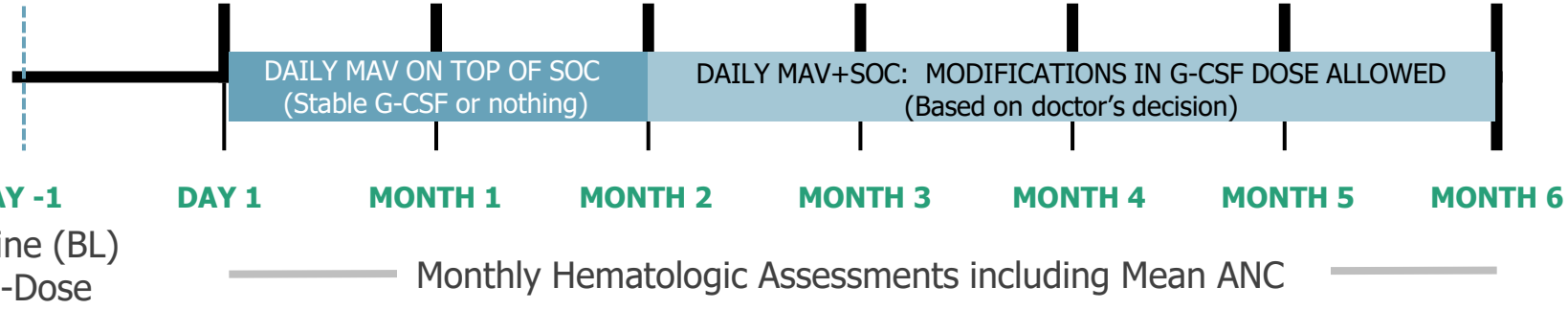
Highest need: reduced G-CSF while maintaining normalize ANCs and no increased infection risk

1. Ellis A, *et al*. ASH Annual Meeting, Poster Presentation, December 2022.

CN Phase 2 Study: Assessing Chronic Daily Dosing of Mavorixafor +/- G-CSF

Mavorixafor: Same Oral Dosing as WHIM Phase 3

Phase 2 Trial: Safety, Durability of ANC Levels, Exploring G-CSF Reduction



Primary Study Objectives:

Mavorixafor Monotherapy: Assess if mavorixafor raises ANC levels in neutropenic patients as a monotherapy

Mavorixafor + G-CSF in Neutropenic Patients: Assess if mavorixafor raises ANC levels in combination with G-CSF

Mavorixafor + G-CSF in Non-Neutropenic Patients: Assess if mavorixafor raises ANC levels in combination with G-CSF to enable/explore reducing or discontinuing G-CSF treatment

- **Inform the design of a global, pivotal Phase 3 clinical trial of mavorixafor in neutropenic patients**

Preliminary CN Phase 2 Results[1]: Addressing Individual Needs

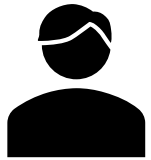
All (n=3) had clinically meaningful increases in ANC[2] with addition of mavorixafor

Data to Date: Three Cases Studies With All Participants on G-CSF

Alternative Monotherapy

- ✓ *~25% enrolled participants are not treated with G-CSF*
- ✓ *Data presentation planned for 1H 2024*

CIN, age 24, female
Baseline ANC ~1,170 cells/μL



CyN, age 39, male
Baseline ANC ~690 cells/μL

Improved Efficacy While On G-CSF

- ✓ Clinically meaningful[2] increases in ANC due to mavorixafor observed in all subjects
- ✓ Neutropenic participants achieved normalized ANC on combination
- ✓ Mavorixafor well tolerated in combination with G-CSF

CIN, age 20, female
Baseline ANC ~5,560 cells/μL

Lower G-CSF Dose/Toxicity

- ✓ G-CSF dosing reduced/removed in the 2 CIN participants
- ✓ Reductions in G-CSF dosing of 75% and 50% maintained normalized ANC

No infections seen after month 2 in all participants

1. Data presented at 2023 American Society of Hematology Annual Meeting, Poster #1160
2. Clinically meaningful: Increase in ANC > 500 cells/μL decreases risk of infection by one grade (e.g. Grade 3 to Grade 2).

CN Clinical Data to Date Support Advancing Mavorixafor into Phase 3 Trial

Overall learnings

First supporting evidence that mavorixafor treatment durably increases ANC

Levels of observed ANC increase (Δ ANC ≥ 500) correlate with infection risk reduction

Safety profile to date supports chronic treatment with mavorixafor

Preliminary data support responder criteria used as primary endpoint in planned CN Phase 3

Mavorixafor delivered on patient needs

Neutropenic participants achieved normalized ANCs

G-CSF could be reduced meaningfully (50% or more)

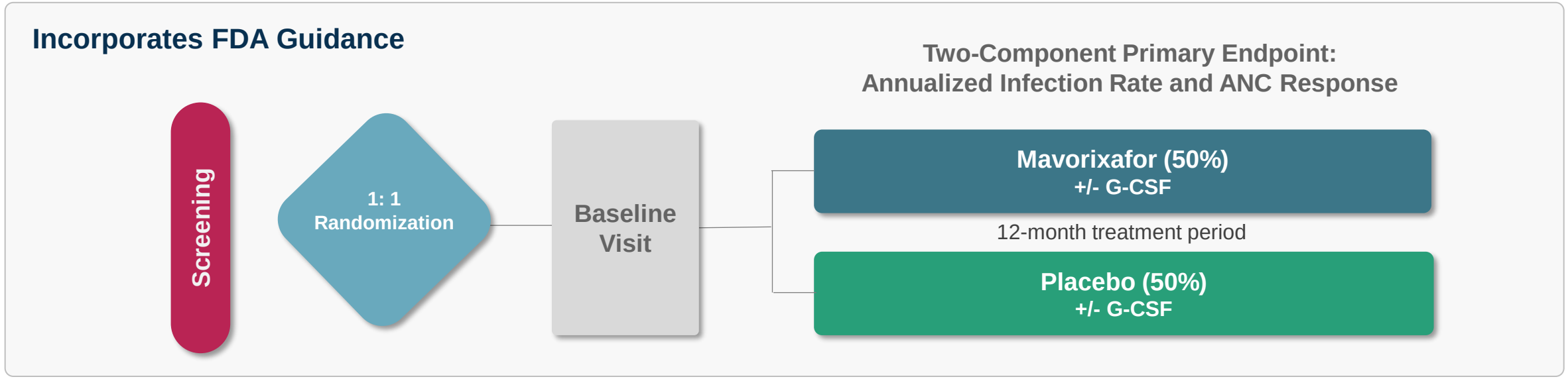
No additional adverse effects seen to date in combination with G-CSF

Phase 2 CN Trial Status

- >15 participants enrolled in trial
- Comprehensive Phase 2 data expected in 1H 2024

Data Across Multiple Studies to Date + Input from FDA Informed Pivotal, Global Phase 3 Trial Design

CN Pivotal, Global Phase 3 Trial Expected to Initiate in 1H 2024



Key Inclusion Criteria:

- **Diagnosis:** congenital, autoimmune, or idiopathic neutropenia
- **Absolute Neutrophil Count (ANC):** <1500 cells/µL
- **Infection history:** 2 infections requiring intervention within last 12 months

Design: double-blinded, randomized, placebo-controlled on top of standard of care (+/- G-CSF[1]); same mavorixafor dosing as 4WHIM trial

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

Endpoint and Power: 150 subjects, ≥90% on primary endpoints of annualized infection rate and ANC response

1. 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.

Primary Endpoints: Infection Rate & Responder Criteria Align with Unmet Needs

Primary Infection Endpoint

Powered to ≥90% for annualized infection rate and ANC response

Primary ANC/Biomarker Endpoint

Unmet Need for Participants	ANC Inclusion Criteria at Baseline ($Baseline_{ANC}$)	Responder Criteria for Phase 3 ANC Endpoint
Alternative Monotherapy[1]	$Baseline_{ANC} \leq 500$ cells/μL	2 out of 3 ANC measurements in first 3 months of study exceed 2x the value of the $Baseline_{ANC}$
Improved Efficacy with G-CSF	500 cells/μL < $Baseline_{ANC} \leq 1500$ cells/μL	2 out of 3 ANC measurements in first 3 months of study exceed 1500 cells/μL

1. A majority of subjects not treated with G-CSF are likely to have ANC<500 cells/μL; inclusion criteria will also allow those with >500 cells/ /μL to enter the study.

Maximizing Mavorixafor Potential to Address Infection Risk in CN and Beyond

WHIM Syndrome
>1,000 est. US Patients

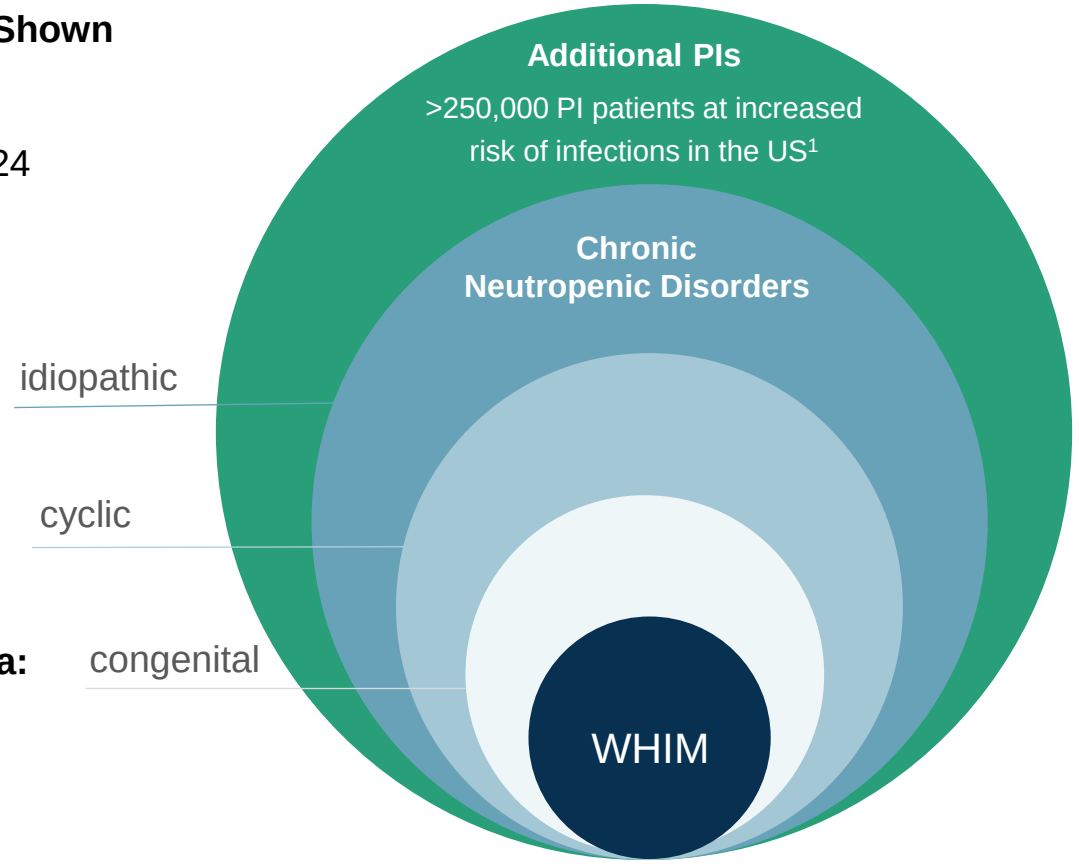
Significant Infection Benefit Shown
PDUFA date of April 30, 2024
Potential U.S. launch in 2Q 2024

Chronic Neutropenic Disorders
~15,000 minimal addressable US market; ~50,000 est. total US Patients

Initiation of Pivotal Phase 3
Expected in 1H 2024

Primary Immunodeficiencies (PIs)
>250,000[1] est. US Patients
(potential subset for further study)

Additional WHIM Phase 3 Data: Innate & adaptive immunity
Data anticipated 1H 2024



1. Boyle JM, Buckley RH. Population prevalence of diagnosed primary immunodeficiency diseases in the United States. *J Clin Immunol*. 2007 Sep;27(5):497-502

An Exciting 2024 Expected: Delivering Innovation to Patients in Need

FDA PDUFA date set for April 30, 2024

U.S. NDA for mavorixafor in WHIM accepted 4Q 2023

X4 eligible for Priority Review Voucher

U.S. launch of mavorixafor for WHIM expected in 2Q 2024

Preparing for potential launch of first-ever treatment for WHIM syndrome

4WHIM trial open-label extension update expected in 1H 2024

Additional Phase 2 CN trial data expected in 1H 2024

>15 participants enrolled

Comprehensive Phase 2 data set expected in 1H 2024

Initiation of Phase 3 CN trial anticipated in 1H 2024

Key learnings and FDA input have enabled finalization of pivotal, global Phase 3 chronic neutropenia trial design

Expansion Opportunities for Mavorixafor

Global regulatory filings anticipated for WHIM in 2024/2025

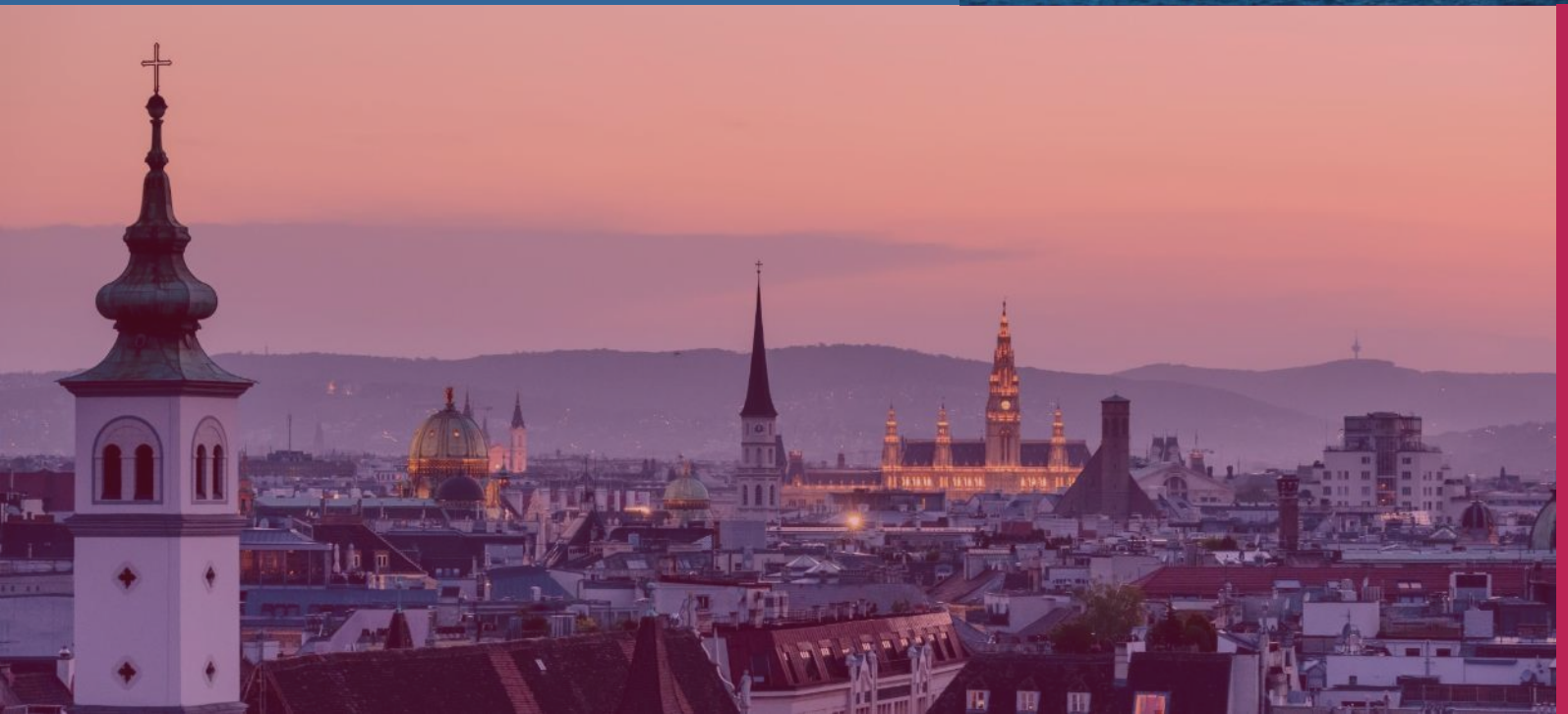
Exploring additional immunodeficiency indications

U.S. Headquarters

61 North Beacon Street, 4th Floor

Boston, MA 02134

NASDAQ: XFOR



Research Center of Excellence

Helmut-Qualtinger-Gasse 2

A-1030 Vienna, Austria

www.x4pharma.com

Seasoned Executive Leadership Team

Experienced in research, development, & commercialization of first-in-class, innovative therapies

PAULA RAGAN, Ph.D.
President & CEO

SANOFI
genzyme

CHRISTOPHE ARBET-ENGELS, M.D., Ph.D.
Chief Medical Officer

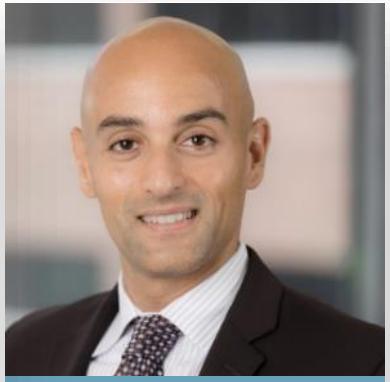
MILLENDO THERAPEUTICS
Biogen

MARK BALDRY
Chief Commercial Officer

Amicus Therapeutics
Biogen

MARY DIBIASE, Ph.D.
Chief Operating Officer

Biogen
EPIRUS BIOPHARMACEUTICALS



ADAM MOSTAFA
Chief Financial Officer

abpro
CANTOR Fitzgerald

ART TAVERAS, Ph.D.
Chief Scientific Officer

Schering-Plough
Biogen

Strong Balance Sheet Supports Expected Upcoming Milestones

~$115.2 million[1]

Funds expected to support operations into 2025[2]

Top-tier Life Science-Focused Institutional Shareholder Base

Analyst Coverage

B RILEY FBR

BROOKLINE CAPITAL MARKETS

CANTOR Fitzgerald

PIPER | SANDLER

STIFEL

HCW H.C. WAINWRIGHT & CO.

1. As of December 31, 2023; 2. Potential for runway extension through commercialization of mavorixafor and monetization of priority review voucher, if approved/received.