X4 PHARMACEUTICALS

June 2023

PROGRESS 4 PATIENTS

Developing the first oral treatments for chronic neutropenic disorders

Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. These statements may be identified by the words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target,” or other similar terms or expressions that concern X4's expectations, strategy, plans, or intentions. Forward-looking statements include, without limitation, statements regarding the clinical development and therapeutic potential of mavorixafor for the treatment of WHIM syndrome, chronic and other neutropenias, and of X4’s other product candidates; X4’s possible exploration of additional opportunities for mavorixafor; the expected duration of patent protection; the expected availability, content, and timing of clinical data from X4’s ongoing clinical trials of mavorixafor; anticipated regulatory filings; clinical trial design; patient prevalence; market opportunities; and X4’s cash runway and ability to satisfy covenants in agreements with third parties, including the loan and security agreement with Hercules Capital, Inc. described in X4’s most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 4, 2023.

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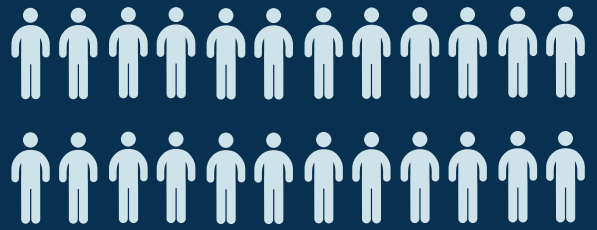
No Innovation for People with Chronic Neutropenia in More Than 30 years

~50,000[1]

Estimated Chronic Neutropenia Patients in the U.S.



- Low levels of neutrophils
- High risk of infections

1

Only One

Therapy Approved for Severe Chronic Neutropenia

Injectable Granulocyte Colony-Stimulating Factor (G-CSF)

- Inconvenient daily injections
- Can require months to titrate to optimal dose
- Frequent treatment-related, treatment-limiting bone pain and other adverse events
- Patients often under-dosed

Clear Need for a Simple, Oral, Well Tolerated Treatment

1. U.S. Prevalence Based on ICD-10 Code Research, Average Across 3 Years (2018, 2019, & 2021); >90% greater than 18 years of age, ~2/3 female, mixed G-CSF use.

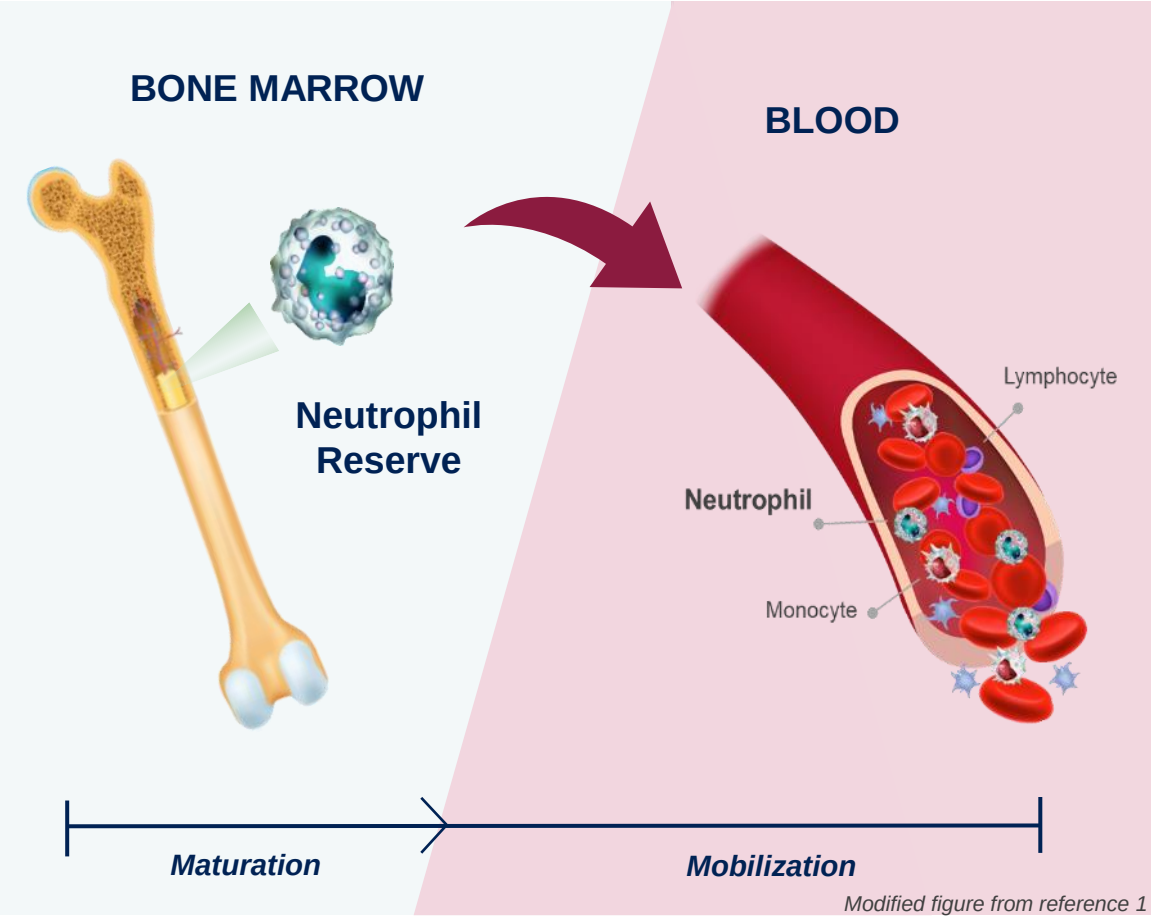
Mavorixafor: Potential Breakthrough for Treating Chronic Neutropenic Disorders

Only oral candidate in development to treat CN disorders, including WHIM syndrome

- Proven mechanism of action (MOA) / ability to increase circulating white blood cells, including neutrophils
- Demonstrated tolerability in >200 individuals, some for >4 years
- Breakthrough Therapy Designation (BTD) and Orphan Drug Designation in first indication: WHIM syndrome; Priority Review Voucher (PRV) eligible
- Patent protection expected through 2038

CXCR4 Antagonism Increases Circulating Immune Cells

Validated mechanism shown to correct neutropenia and lymphopenia



- Neutrophils are retained in the bone marrow by the CXCR4/CXCL12 axis, creating a “reserve”[2]
- Downregulation of CXCR4 leads to mobilization of white blood cells into the blood [2,3]
- Antagonism by mavorixafor shown to durably increase circulating levels of neutrophils, lymphocytes, and monocytes[4,5]

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. Kim HK, et al, Blood. 2006. 4. Mosi, RM, et al, Biochem Pharmacol, 2012. 5. Stone ND et al, Antimicrob Agents Chemother. 2007.

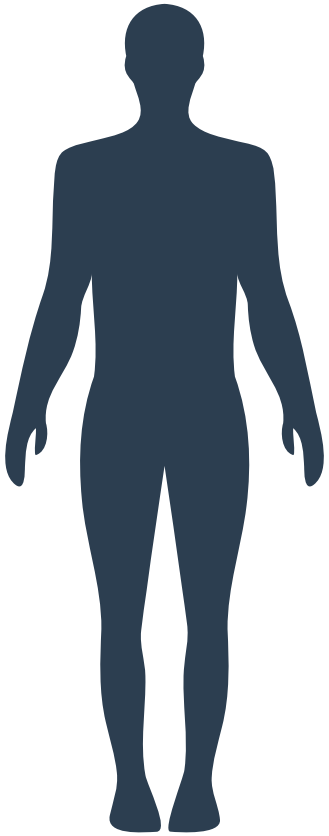
Advancing Mavorixafor in Chronic Neutropenic Disorders

	Candidate	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Expected Milestones	Target Patient Populations
CHRONIC NEUTROPENIC DISORDERS	Mavorixafor	**WHIM Syndrome** (Warts, Hypogammaglobulinemia, Infections and Myelokathexis)	**Successfully Completed Phase 3**				**NDA early 2H23** **Possible U.S. approval 1H24**	>1,000 U.S.[1]
	Mavorixafor	**Chronic Neutropenia** (Congenital, Cyclic, and Idiopathic)	**Phase 2**				**Add'l data / regulatory update 2Q/3Q 2023**	~50,000 U.S.[2]
	X4P-003	Not yet disclosed						

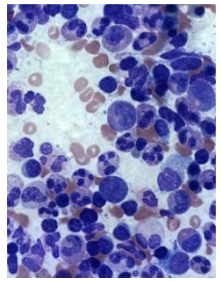
1. Company market research. Qessential market research, 2019 and IPM.ai artificial intelligence study, 2020. 2. U.S. Prevalence Based on ICD-10 Code Research, Average Across 3 Years (2018, 2019, & 2021); >90% greater than 18 years of age, ~2/3 female, mixed G-CSF use

WHIM[1] Syndrome: Poorly Functioning Immune System, Starting from Birth

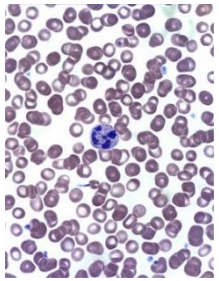
Clinical diagnosis driven by over-signaling in the CXCL12/CXCR4 pathway



WHIM Disease Bone Marrow[2]



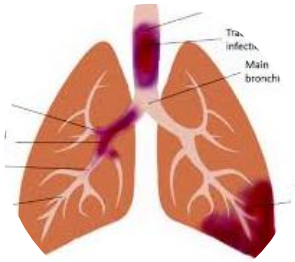
Healthy Bone Marrow[2]



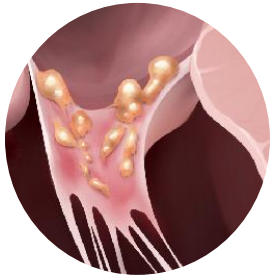
Decreased white blood cell counts & impaired cell maturation lead to immune system dysfunction and increased risk of infections

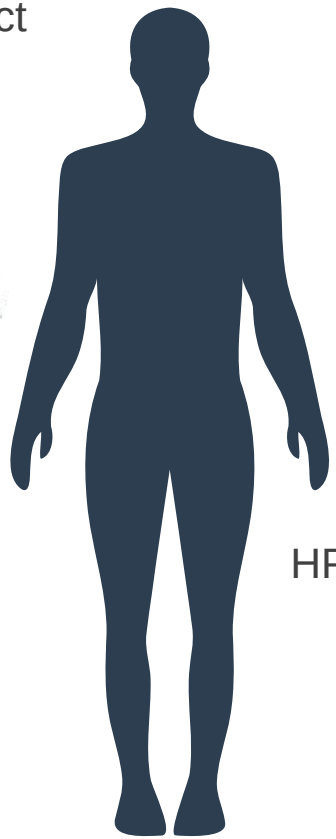
Increased Risk of Infections

Ear, Sinus, and Lung: Upper/Lower Respiratory Tract Infections

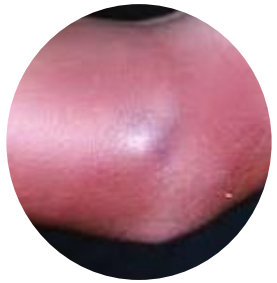


Heart: Endocarditis





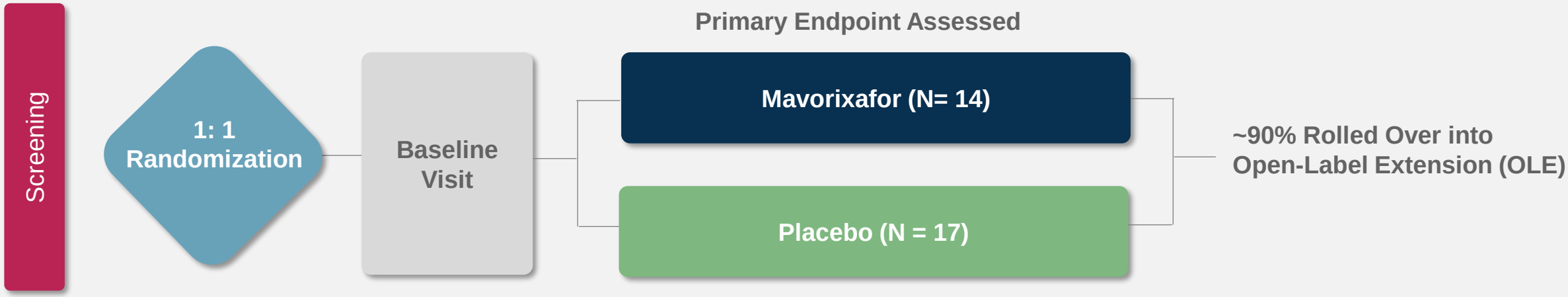
Skin: Cellulits



Genitourinary Areas: HPV-related Cancers

1. **WHIM = Warts, Hypogammaglobulinemia, Infections and Myelokathexis**
2. McDermott: Stiehm's Immune Deficiencies 2014, Pages 711-719

4WHIM Pivotal Phase 3 Clinical Trial Overview



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 the 13, 26, 39, and 52-week

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

GOAL LABEL: For the treatment of people aged 12 and above **diagnosed with WHIM syndrome**

ANC = absolute neutrophil count; ALC = absolute lymphocyte count

4WHIM™

Phase 3 Clinical Trial

Mavorixafor

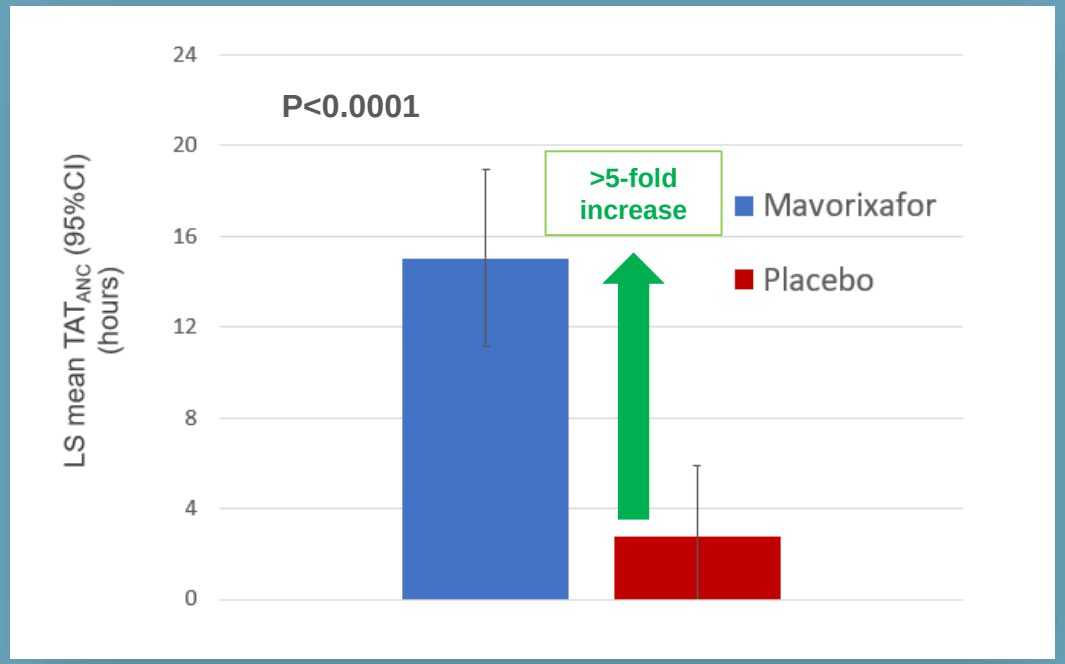
demonstrated significant clinical benefit & favorable safety profile

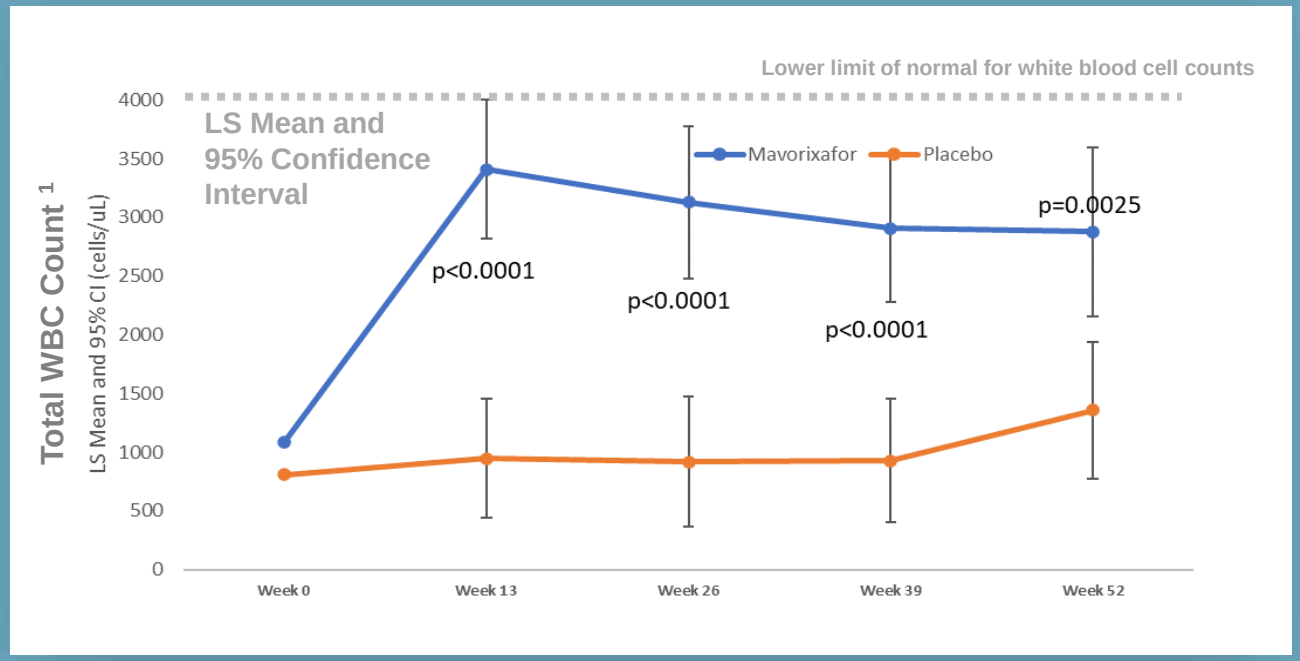
4WHIM trial met primary endpoint of time above threshold for absolute neutrophil counts (TAT_{ANC}) and key clinical benefit assessments

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

4WHIM Primary Endpoint Met; Total WBC Counts Increased Over 52 Weeks

Intent-to-Treat (ITT) Population Analysis





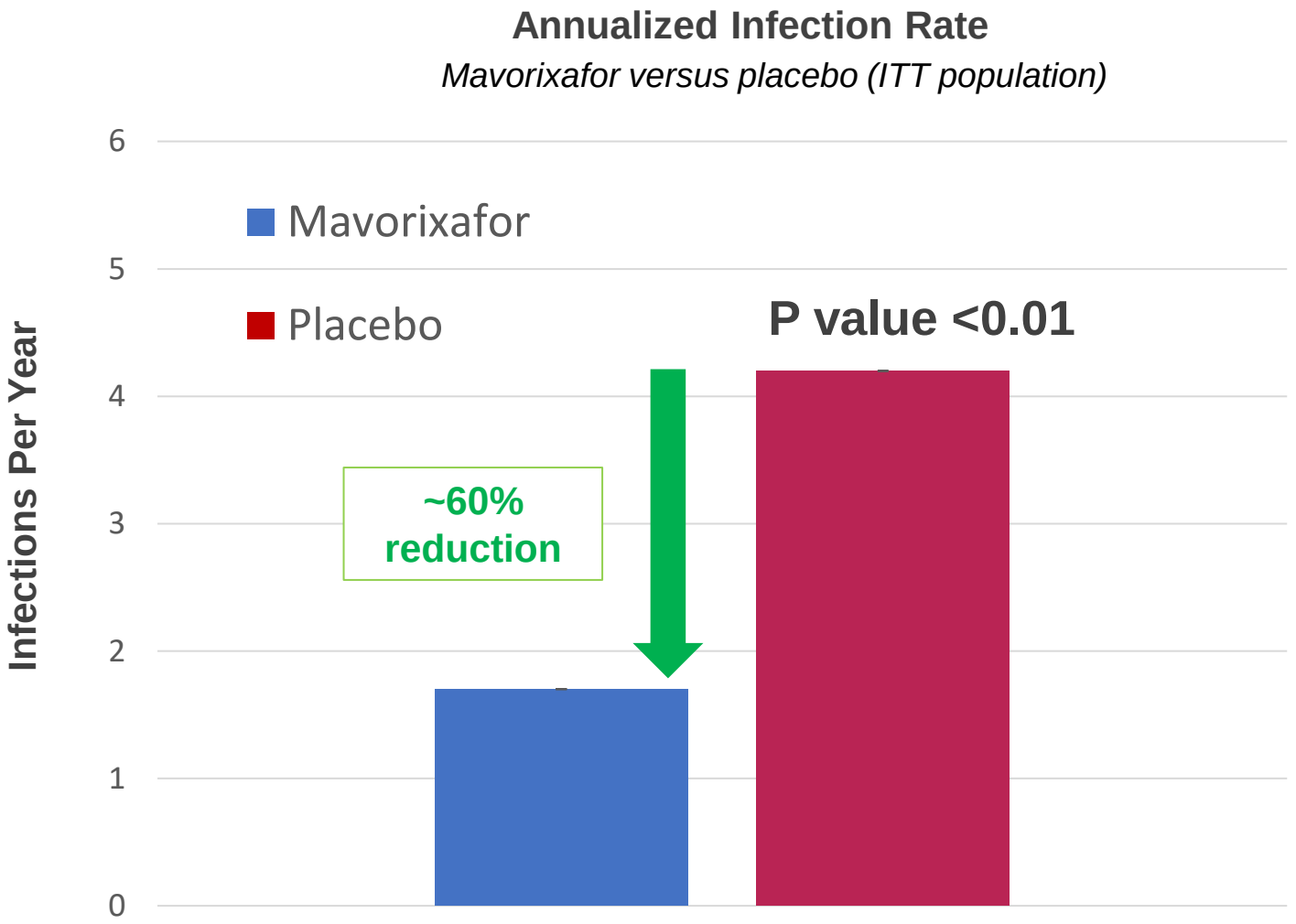
Mavorixafor significantly improved the time above threshold of ANC over 52 weeks vs. placebo

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

Statistically significant, durable increases in all WBC subtypes[2]

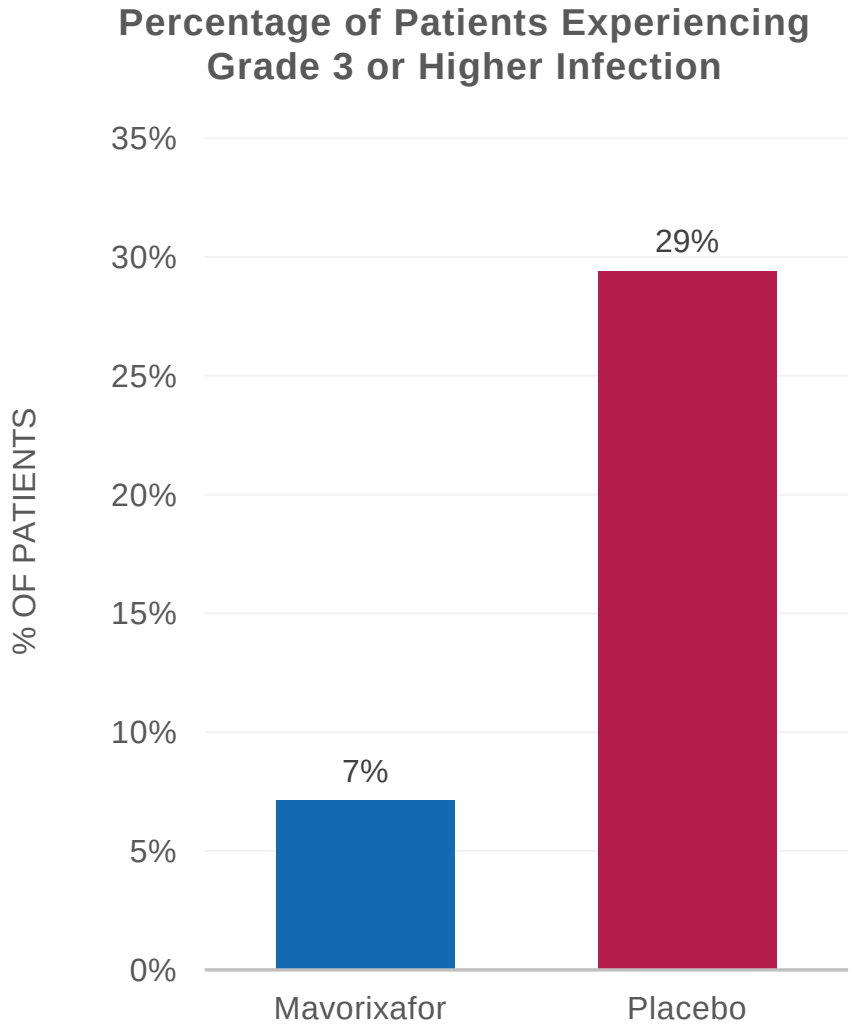
1. At week 52, 3 of 17 placebo patients were given mavorixafor in advance of their TAT measurements as they entered the open-label portion of the study. All data are included in ITT analysis. 2. Data on file.

Statistically Significant ~60% Reduction in Annualized Infection Rate



- **Deeper Reductions in Infection Rate with Time on Mavorixafor Treatment**
  - Infection rates decreased to <1.0 on mavorixafor vs. 4.5 for placebo ($p<0.005$) during months 6-12
- **Infection Rate Reductions With Mavorixafor Treatment Seen Across All Subgroups**

More Patients on Placebo Experienced Severe[1] Infections



	Mavorixafor (n=14)	Placebo (n=17)
CTCAE Criteria	N	N
Grade 1 / Grade 2	10	11
Grade 3	**1***	**4**
Grade 4	0	**1**
Grade 5	0	0

***Grade 3 infection on mavorixafor treatment occurred during first 3 months of treatment; rate of severe infections on placebo unchanged over 52-week period**

1. Severe infections are those Grade 3 or higher by CTCAE criteria; Scored by participants' highest grade CTCAE event.

Total Time With Infection Reduced by >70% with Mavorixafor

- Mean total time with infection: **~2 weeks on mavorixafor vs. ~7 weeks on placebo**
- Median total time with infection **showed similar (~75%) reduction**

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

Oral Mavorixafor was Well Tolerated in the Trial

Top-line safety data summary for randomization period; supports chronic dosing

Overall

- No treatment-related Serious Adverse Events (SAEs)
- No discontinuations due to safety events
- No treatment-limiting toxicities
- ~90% of patients continued into the Open Label Extension study

Treatment Arms

- Placebo arm had increased (3 to 4 times) infections/infestations and respiratory disorders
- Mavorixafor arm had increased skin & GI disorders
  - No discontinuations - all were mild, all resolved
- Other safety assessments showed balance between two arms and/or deemed non-drug related

Preparation Underway for Potential 1H 2024 US Launch in WHIM Syndrome

1 Building the WHIM Syndrome Community

- Establish X4 as a trusted partner with key stakeholders
- Educate on WHIM syndrome, highlighting unmet need and enabling better patient identification
- Support earlier diagnoses leading to better patient outcomes

2 Ensuring Broad Patient Access

- Communicate the mavorixafor value proposition
- Implement distribution and supply chain
- Engage with Payers to ensure rapid reimbursement

3 Evolving X4 to a Fully Integrated Biotech

- Build a rare disease commercial organization
- Establish infrastructure and capabilities
- Coordinate cross-functional launch readiness

Leveraging our Success in WHIM into Chronic Neutropenic Disorders

Advancing mavorixafor for the treatment of idiopathic, cyclic, and congenital chronic neutropenia

WHIM Syndrome

Positive Phase 3 Data: Trial Met Primary Endpoint

Reduction in Rate, Severity, and Duration of Infections

Improvements in Chronic Neutropenia and Lymphopenia (TAT_{ANC} and TAT_{ALC})

Phase 1b/Phase 2 Chronic Neutropenia Trial

Idiopathic

~40,000[1]

Most commonly diagnosed; with symptoms in young adulthood

Not attributable to other causes (drugs, HIV, autoimmune, etc.)

Cyclic

~5,000[1]

Typically, a 21-day cycle

Autosomal-dominant disorder with symptoms from birth/infancy

Often linked to *ELANE* genetic variants

Congenital

~3,000[1]

Rare hematological genetic diseases with symptoms from birth/infancy

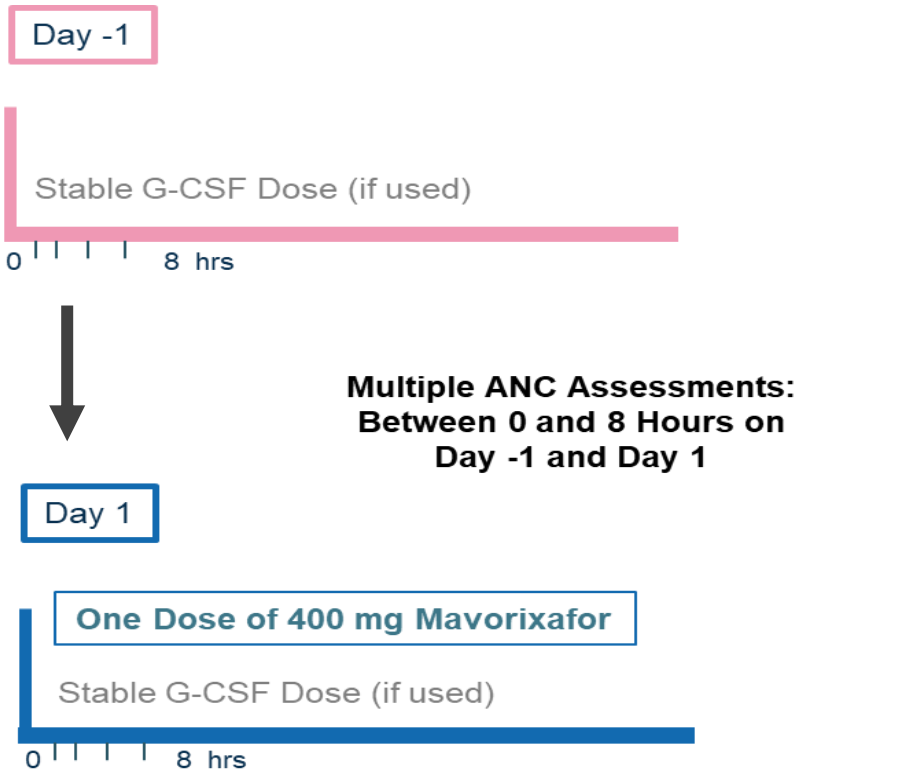
Caused by range of genetic variants

1. U.S. Prevalence Based on ICD-10 Code Research, Average Across 3 Years (2018, 2019, & 2021); >90% greater than 18 years of age, ~2/3 female, mixed G-CSF use

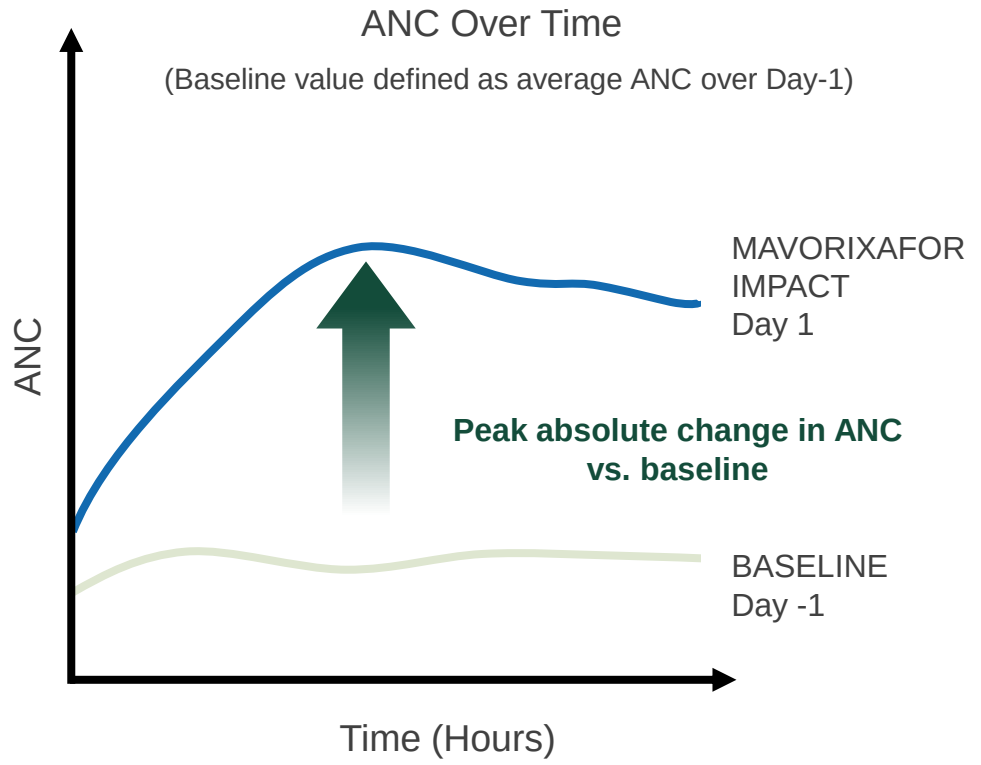
Phase 1b: Measuring Mavorixafor Potential in Chronic Neutropenic Disorders

Single-dose study, with or without G-CSF treatment

Trial Design

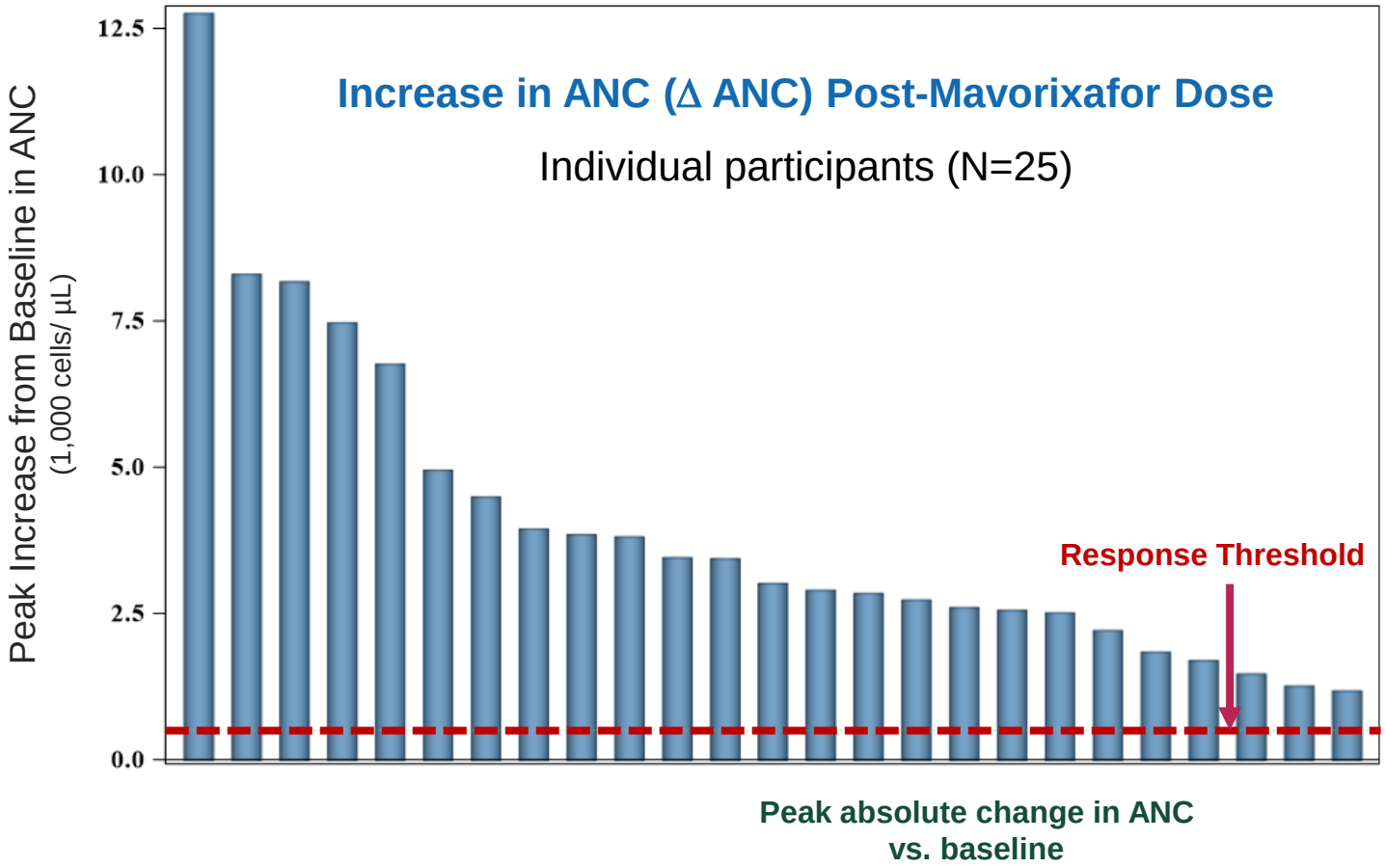


Trial Measurements



Phase 1b Results: 100% of Patients Responded Across All CN Disorders Studied

Response defined as increase in ANC >500 cells/ µL[1]



All participants responded

- Suggests bone marrow reserve of neutrophils can be accessed
- Responses exceeded 500 cells/µL for every individual participant

Mean ANC increase shows consistent correction of neutropenia across all CN types:

- Idiopathic
- Congenital
- Cyclic

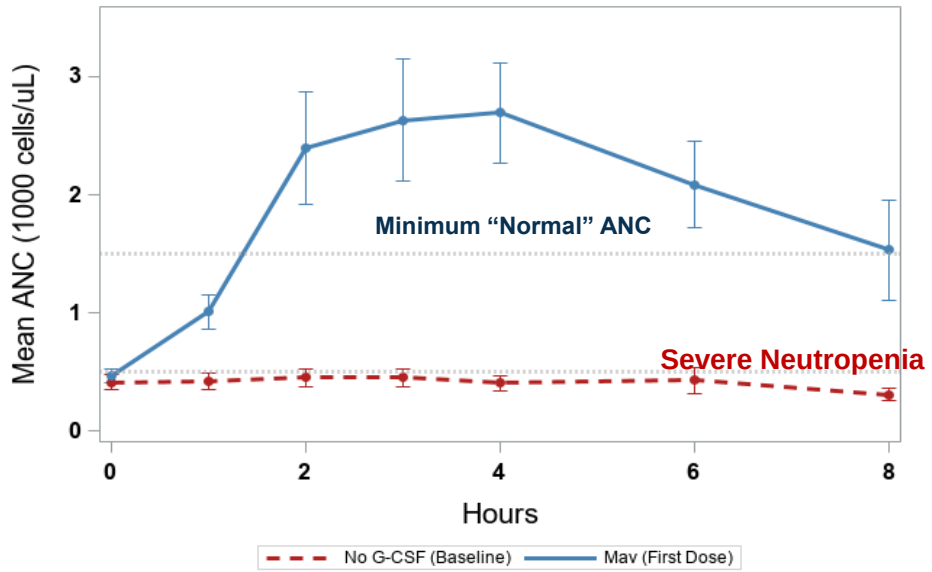
1. Increase of at least 500 cells/µL corresponds to improvement in at least one grade (e.g. severe neutropenia improves to moderate neutropenia); Chance in ANC determined using peak ANC (post-treatment) minus average baseline ANC (pre-treatment).

Mavorixafor Potential as Monotherapy or in Combination with G-CSF

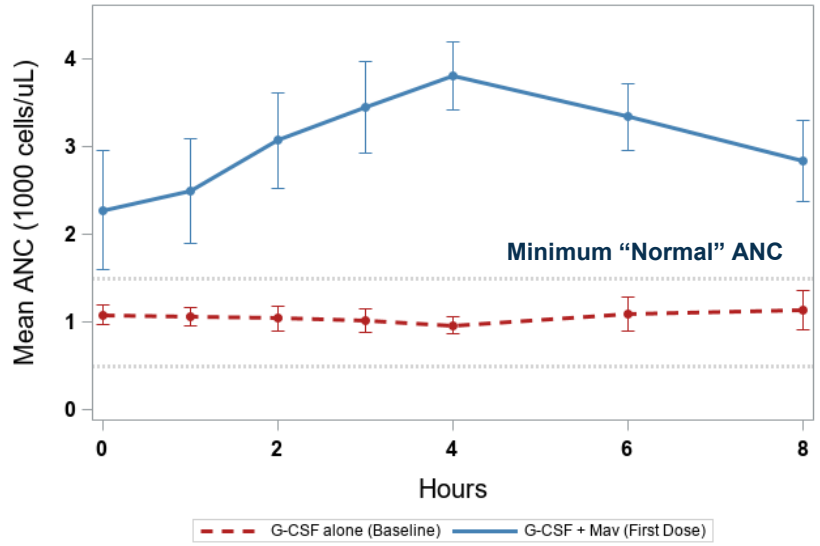
Supports exploring potential of mavorixafor to reduce or replace G-CSF

Subgroups: participants with moderate or severe neutropenia (pre-treatment)

MAVORIXAFOR MONOTHERAPY



MAVORIXAFOR + G-CSF



All (100%) participants responded

All (100%) achieved normalized ANC levels

Mean ANC increase of >2,500 cells/µL across all participants

Advancing Mavorixafor Towards Potential Registration in Chronic Neutropenic Disorders

Phase 2 Study Underway

Phase 2 trial ongoing to assess chronic dosing (up to 6 months) and G-CSF down-titration

Initial data to evaluate durability after 1 to 3 months of treatment: 2Q/3Q 2023

Targeting 20 or more participants in the study

Expected Near-term Milestones: 2Q/3Q 2023

Initial durability data from Phase 2 study

Regulatory clarity on CN Phase 3 trial design

Potential Phase 3 Design

Target patient population: Idiopathic, Cyclic and Congenital Chronic Neutropenia

- Clinical history consistent with infection risk
- Those with severe and potentially moderate neutropenia

Placebo-controlled study

Developing the first oral treatments for chronic neutropenic disorders

Expected Upcoming Milestones

Cash expected to fund operations into 4Q 2024[1]

4Q22

- Achieved primary endpoint and key secondary endpoint in Phase 3 4WHIM clinical trial ✓

1H23

- ✓ Mavorixafor/WHIM Pre-NDA meeting with U.S. FDA
- Presented additional positive 4WHIM trial results

2Q/3Q 2023

- Additional chronic neutropenia clinical data
- Registration path clarity for mavorixafor in CN disorders

2H23

- Mavorixafor U.S. New Drug Application (NDA) in WHIM syndrome
- WHIM launch readiness update

1H24

- Possible approval[2], PRV grant, & launch of mavorixafor for WHIM in the U.S.
- OLE data from 4WHIM
- Initiate Phase 3 clinical trial of mavorixafor in chronic neutropenia

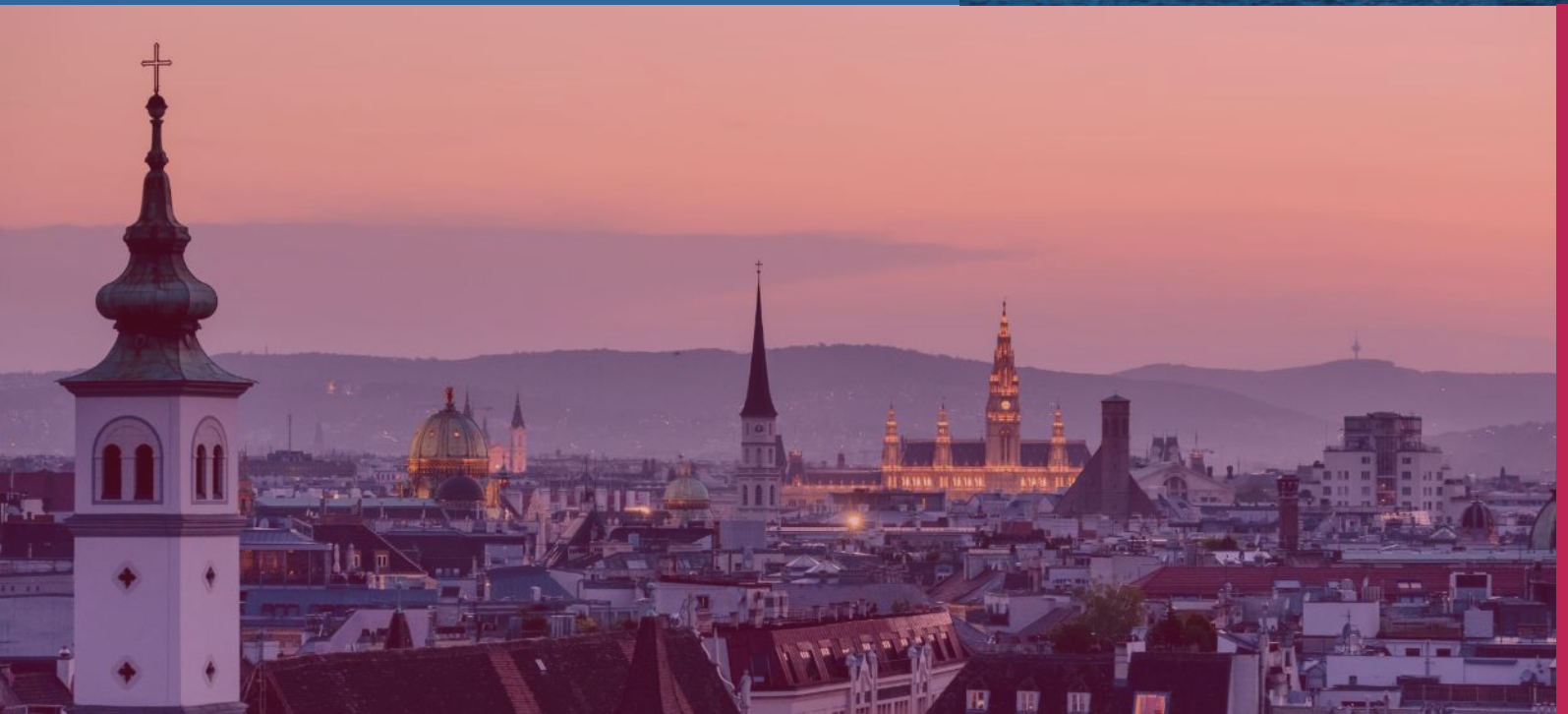
1. Cash runway estimate includes cash and restricted cash balance of $94.4 million as of March 31, 2023 plus anticipated net proceeds from a PIPE financing announced in 2Q 2023. 2. Timeline assumes granting of priority review by U.S. Food and Drug Administration.

U.S. Headquarters

61 North Beacon Street, 4th Floor

Boston, MA 02134

NASDAQ: XFOR



Research Center of Excellence

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Amicus Therapeutics
Biogen

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Chief Operating Officer

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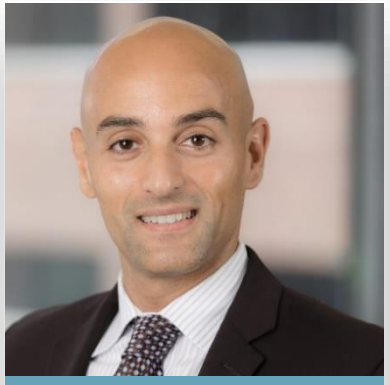

ADAM MOSTAFA
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MURRAY STEWART, DM, FRCP
Interim Chief Medical Officer

Rhythm PHARMACEUTICALS
gsk

ART TAVERAS, Ph.D.
Chief Scientific Officer

Schering-Plough
Biogen

Strong Balance Sheet Supports Expected Upcoming Milestones

~$155 million[1]

Cash expected to fund operations into 4Q 2024[2,3]

Top-tier Life Science-Focused Institutional Shareholder Base

Analyst Coverage

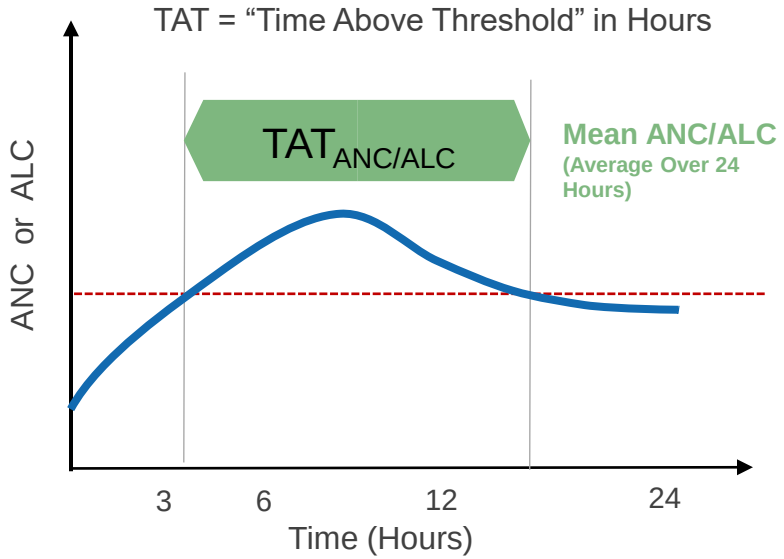
B RILEY FBR | BROOKLINE CAPITAL MARKETS | CANTOR Fitzgerald | COWEN | OPPENHEIMER

PIPER SANDLER | STIFEL | HCW H.C. WAINWRIGHT & CO.

1. Pro forma cash and cash equivalents balance includes $94.4 million as of March 31, 2023 plus anticipated net proceeds from a PIPE financing announced in 2Q 2023. 2. As described in detail in our most recent Form 10-Q, our agreement with Hercules Capital, Inc. contains a minimum cash covenant. Our current cash runway projections assume continued compliance with this covenant. Failure to satisfy this covenant could result in accelerated principal and interest payments due that could shorten our cash runway. Outstanding debt balance as of 03/31/2023 is $32.5 million. 3. Potential for runway extension through commercialization of mavorixafor and monetization of priority review voucher.

Phase 3 4WHIM Study: Primary Endpoint and Key Secondary and Exploratory Assessments

Assessment of correcting neutropenia	Assessment of correcting lymphopenia	Assessment of clinical benefit & other outcomes
Primary Endpoint = TAT_{ANC}	**1st Key Secondary Endpoint = TAT_{ALC}**	**Safety and Clinical Impact**
(p value of treatment vs placebo)	(p value of treatment vs placebo)	



Severe Neutropenia Threshold (500 cells/µl)

Lymphopenia Threshold (1000 cells/µl)

Patient/Physician Reports

Infections & Warts

Response to Vaccination

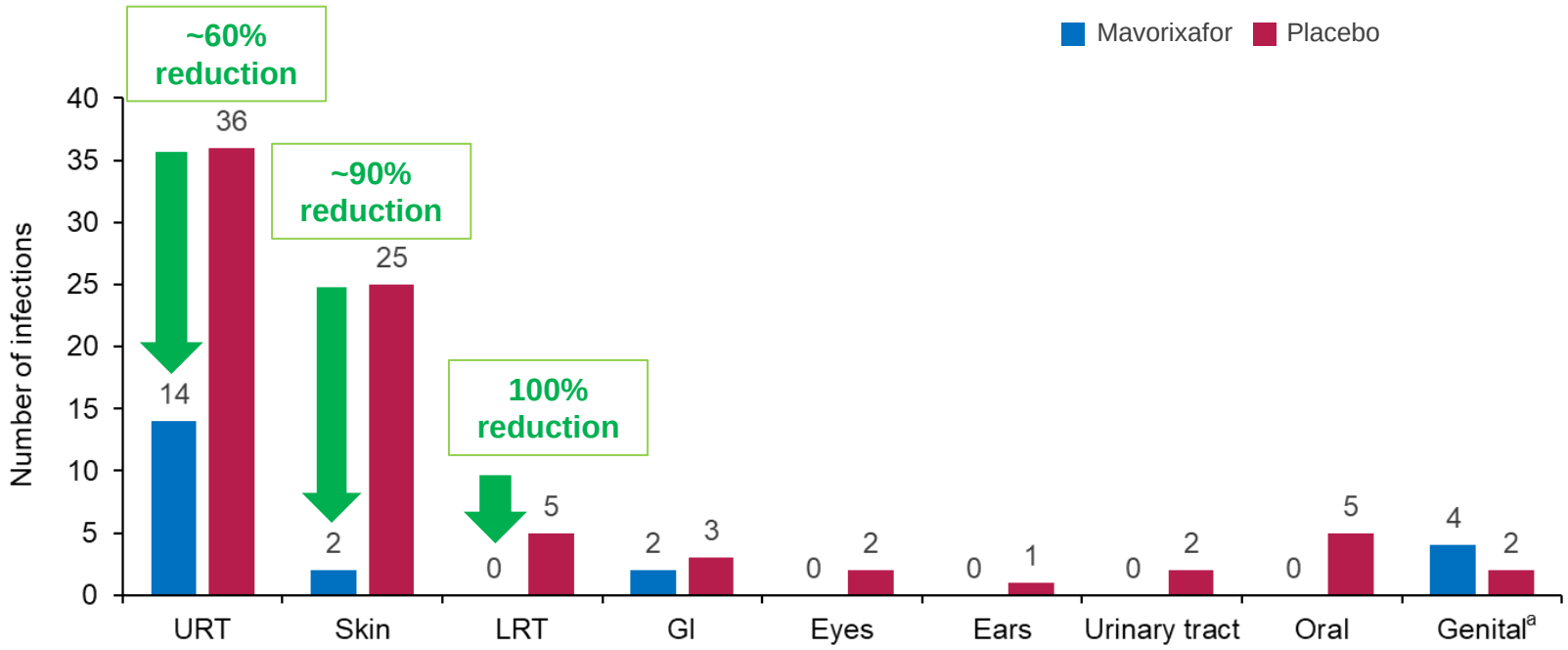
Safety, tolerability and pharmacokinetics (PK)

4WHIM Demographics & Screening Metrics

	Mavorixafor (N=14)	Placebo (N=17)
Adolescents (12 to <18 years)		
n (%)	7 (50)	8 (47)
Adults (≥18 years)		
n (%)	7 (50)	9 (53)
Female Gender		
n (%)	9 (64)	9 (53)
Previous Immunoglobulin Usage		
n (%)	6 (43)	8 (47)
Screening ANC (cells/μL)		
n mean (SD) median (min, max)	14 173 (112) 150 (40, 390)	17 194 (123) 200 (0, 400)
Screening ALC (cells/μL)		
n mean (SD) median (min, max)	14 496 (237) 420 (260, 1070)	17 1015 (1983) 520 (100, 8560)

Mavorixafor Reduced Infections Across Most Organ Systems

Reported Benefit Observed in Bacterial, Viral, and Fungal Infections



URT, upper respiratory tract; GI, gastrointestinal; LRT, lower respiratory tract. [a]Excluding warts.

Assessment of Warts

First placebo-controlled study assessing warts in WHIM

- ~70% of patients had warts
- Warts assessed at weeks 0, 26, and 52
- Photographic images captured
- Visual changes scored via central, blinded committee

Results

- No difference between groups in reducing pre-selected, existing warts
- Fewer participants on mavorixafor developed new warts at week 52