Positive 4WHIM Phase 3 Top-Line Results

Conference Call & Webcast

November 29, 2022

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On Today's Call

PAULA RAGAN, Ph.D.
President & CEO

MURRAY STEWART, DM, FRCP
Interim Chief Medical Officer

MARK BALDRY
Chief Commercial Officer

MARY DIBIASE, Ph.D.
Chief Operating Officer

ADAM MOSTAFA
Chief Financial Officer

ART TAVERAS, Ph.D.
Chief Scientific Officer

"I feel very passionate about helping other WHIM patients and am very grateful to all the staff at X4 who are helping us all."

- Leanne, WHIM patient

Mavorixafor – the First Potential Treatment for WHIM Syndrome

15.04 hours (P<0.0001)

Mean time of absolute neutrophil count above threshold[1] (TAT-ANC) over a 24-hour period, over 52 weeks, **treatment vs. placebo (2.75 hours)**

1. Threshold = 500 cells/μL; ANC levels below 500 are considered severe neutropenia

WHIM Syndrome: Congenital Immunodeficiency Associated with Chronic Neutropenia

Combined primary immunodeficiency affecting both children and adults

W **arts.** Driven by underlying HPV infection that can increase the risk of HPV-related cancer

H **ypogammaglobulinemia.** Low antibody production

I **nfections.** Multiple, chronic infections in WHIM patients due to neutropenia and lymphopenia can lead to devasting, irreversible morbidities, fatalities

M **yelokathexis.** A "hyper-dense" population of immune cells in the bone marrow, reducing the ability to mature, mobilize for immune surveillance

No targeted therapies approved to treat underlying cause

Range of Assessments Help Establish a WHIM Diagnosis

Primary Clinical Assessments

- **Neutropenia & lymphopenia (low ANC and ALC)**
- Repeat infections with long-term effects
- *In some:* wart lesions; cervical test for HPV
- *In some:* Low immunoglobulin (Ig) levels

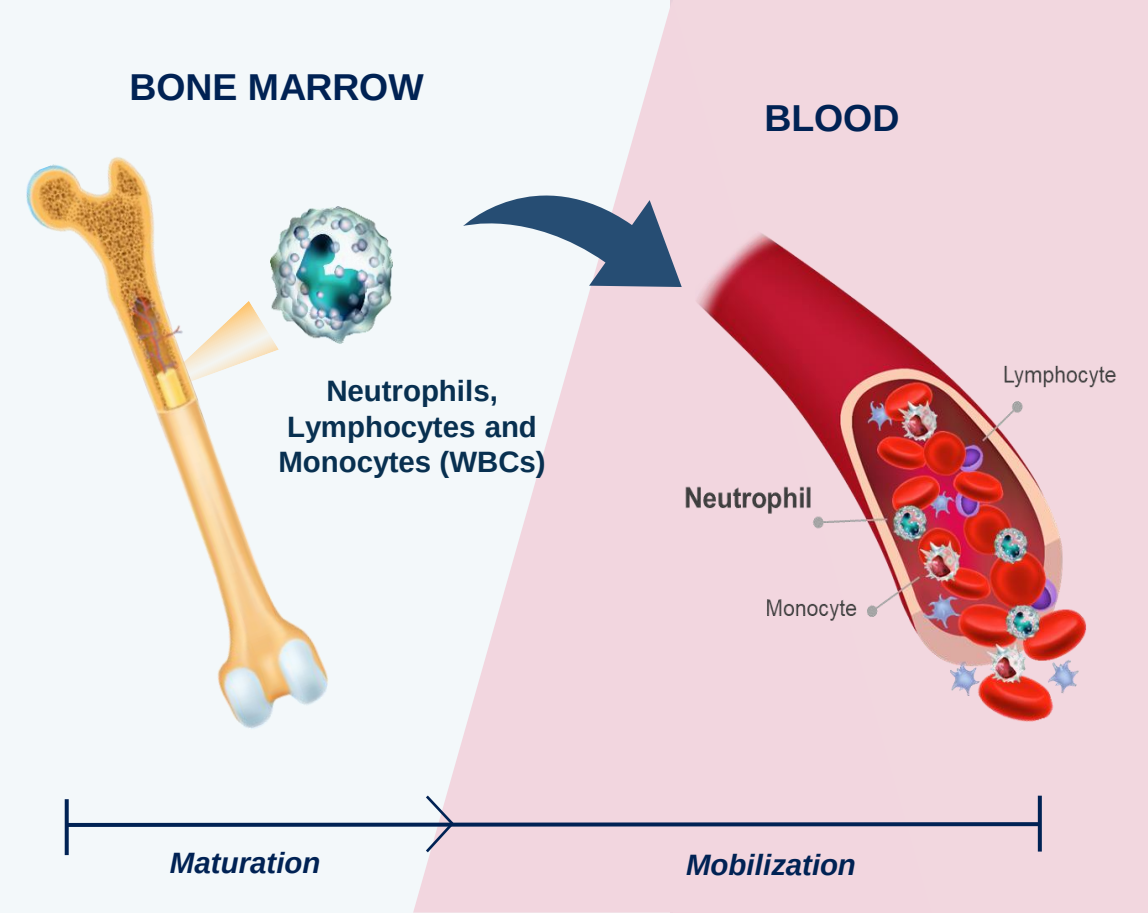
Additional Assessments

- Bone Marrow Biopsy
- Genetic Testing
- Family History

1. Michniacki et al, *Blood* (2019) 134 (Supplement_1): 3449

CXCR4: Key Role in Maturation & Mobilization of Neutrophils and Lymphocytes

Mavorixafor is a CXCR4 Antagonist



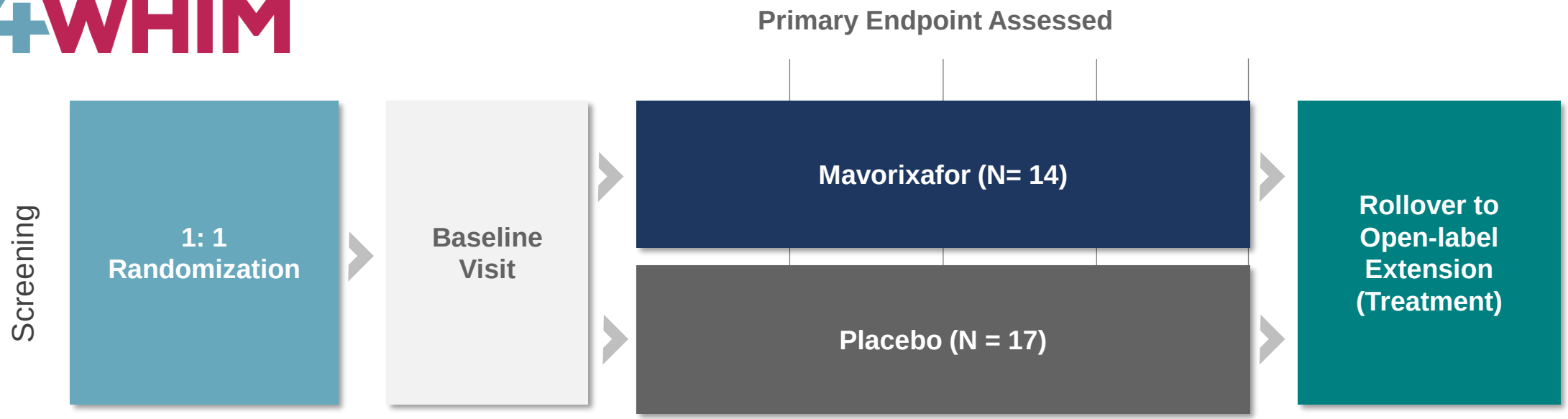
White Blood Cells (WBCs) are retained in the bone marrow by the CXCL12/CXCR4 axis creating a "reserve"

- Bone marrow produces WBCs, including neutrophils and lymphocytes
- Creates reserves to fight infection, short and long term

Mavorixafor shown to inhibit CXCR4 signaling and increase maturation and mobilization of WBCs into the blood

- Demonstrated reduction of neutropenia in Phase 1b CN and Phase 2 WHIM clinical studies
- Phase 3 study assessing impact on neutropenia, lymphopenia, and clinical aspects of WHIM syndrome

4WHIM Phase 3 Study Design



Top-Line Assessments (Today)

- Mean TAT_{ANC} - mean of the 13, 26, 39, and 52-week assessments
- Mean TAT_{ALC} - mean of the 13, 26, 39, and 52-week assessments
- Safety and tolerability across 52 weeks

>90% of patients have continued in open label extension (OLE)

Additional secondary and exploratory clinical endpoints expected in 1H 2023

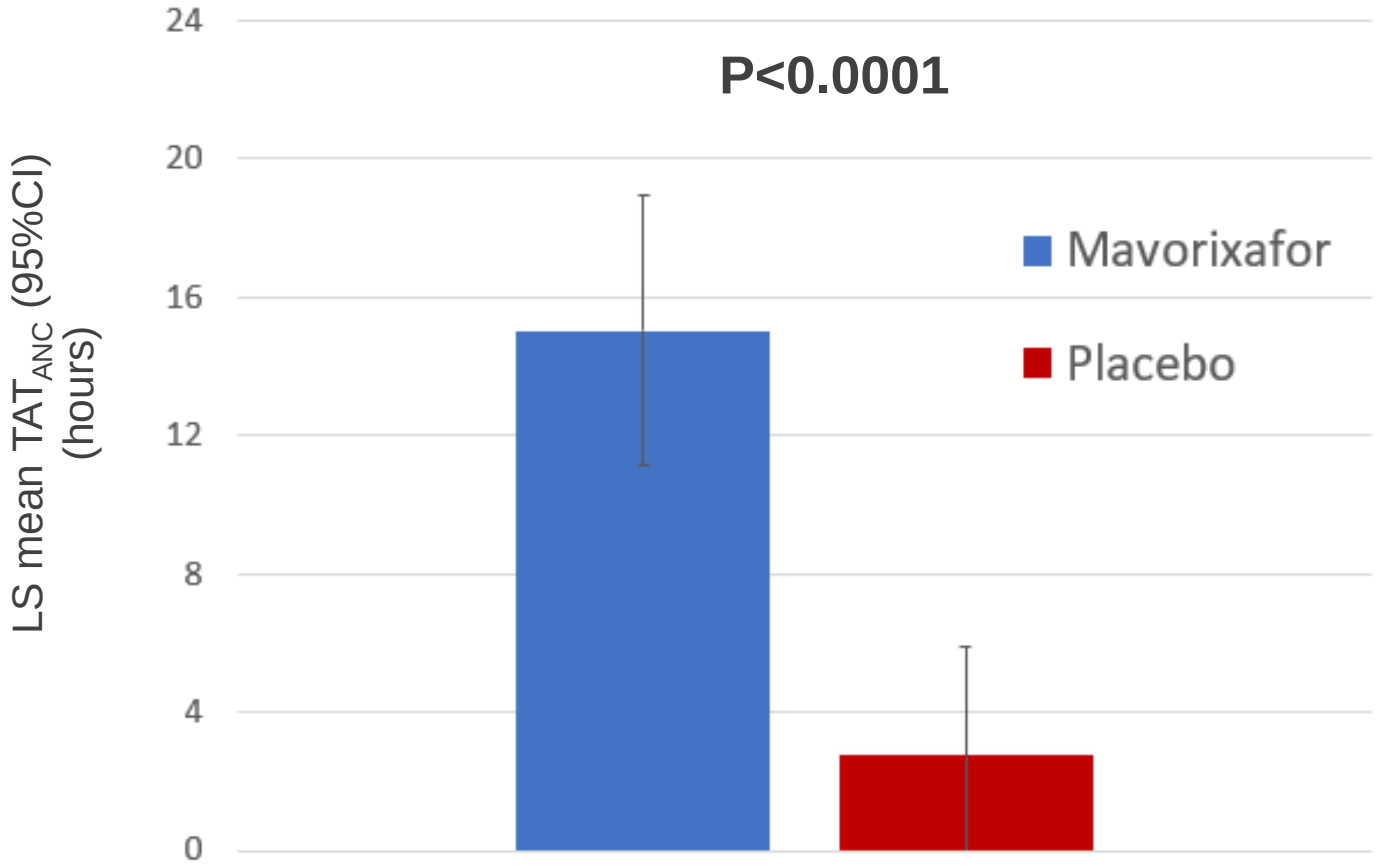
GOAL LABEL**: Indicated for the treatment of people aged 12 and above **diagnosed with WHIM syndrome

Demographics & Screening Metrics

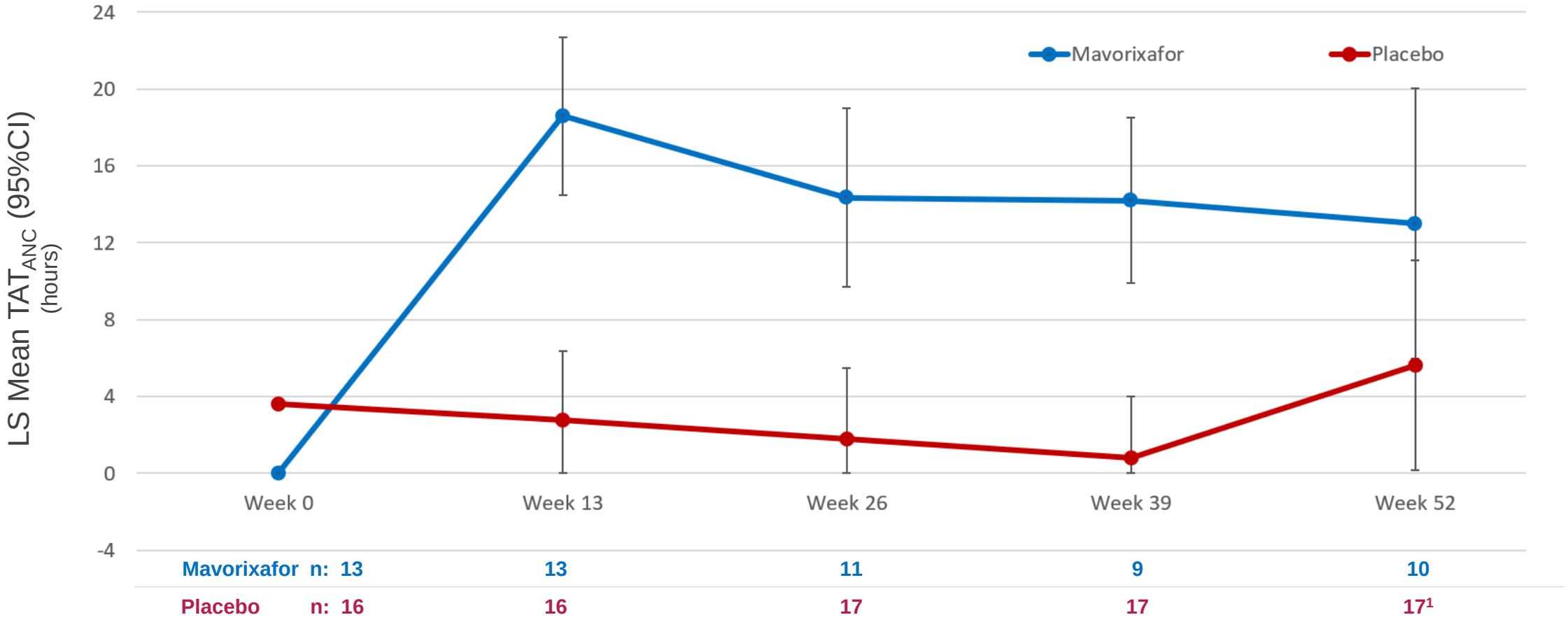
	Mavorixafor (N=14)	Placebo (N=17)
Adolescents (12 to <18 years)		
n (%)	7 (50)	8 (47)
Adults (≥18 years)		
n (%)	7 (50)	9 (53)
Female Gender		
n (%)	9 (64)	9 (53)
Previous Immunoglobulin Usage		
n (%)	6 (43)	8 (47)
Screening ANC (cells/μL)		
n mean (SD) median (min, max)	14 173 (112) 150 (40, 390)	17 194 (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)

Phase 3 Primary Endpoint (TAT_{ANC}) Met

- Mavorixafor significantly improved the time above the threshold of ANC vs. placebo in intent-to-treat (ITT) population
- Mean TAT_{ANC} was 15.04 hours for mavorixafor vs. 2.75 hours for placebo
- 5.5-fold improvement in TAT_{ANC} compared with placebo



TAT_{ANC} vs. Time on Treatment: Mavorixafor Durably Increased TAT_{ANC} Over 52 Weeks (ITT population)



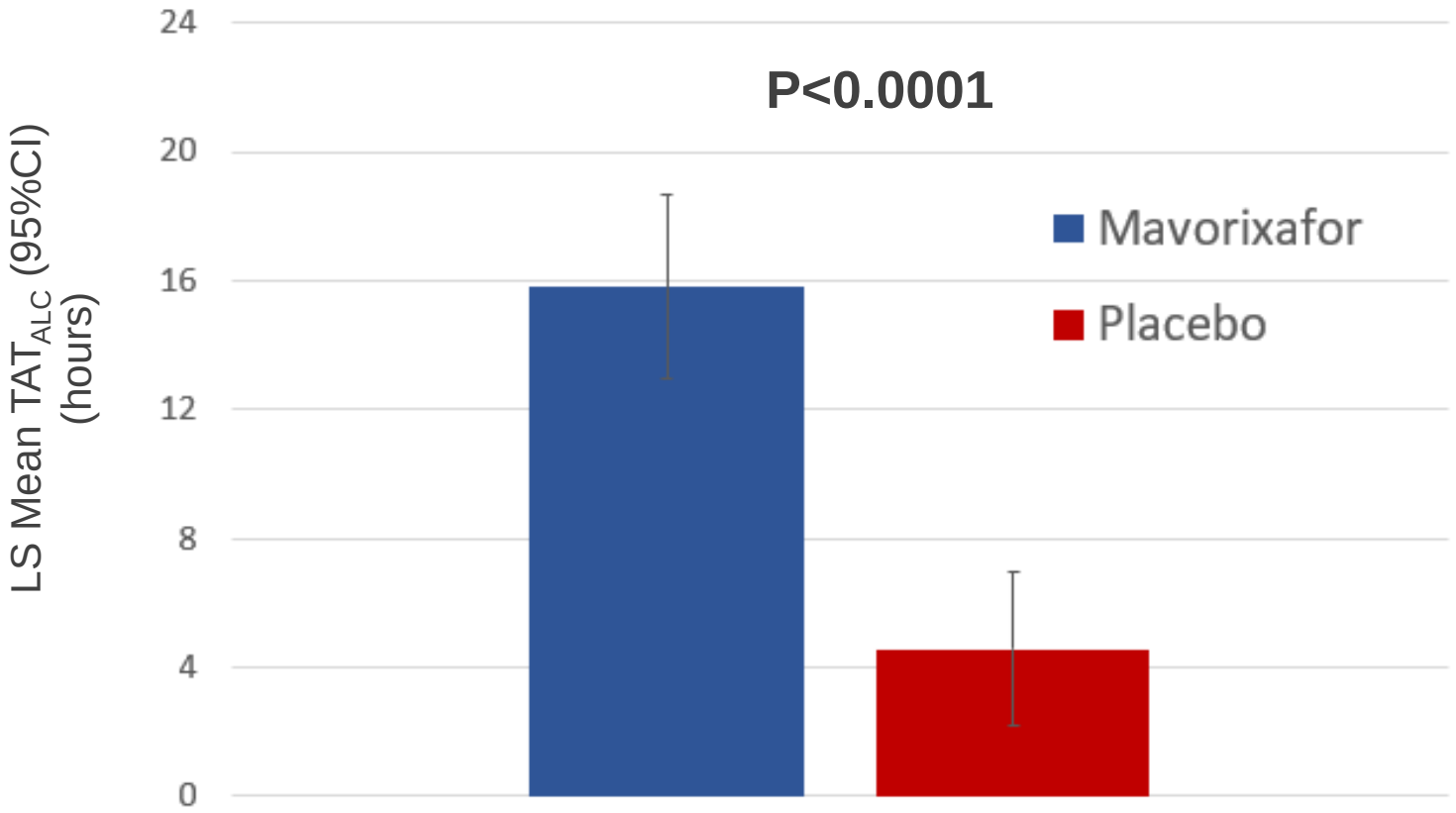
TAT_{ANC} was increased and maintained over 52 weeks vs. placebo and vs. baseline

Average increase of ~12.3 hours vs. placebo over the 52-week treatment period

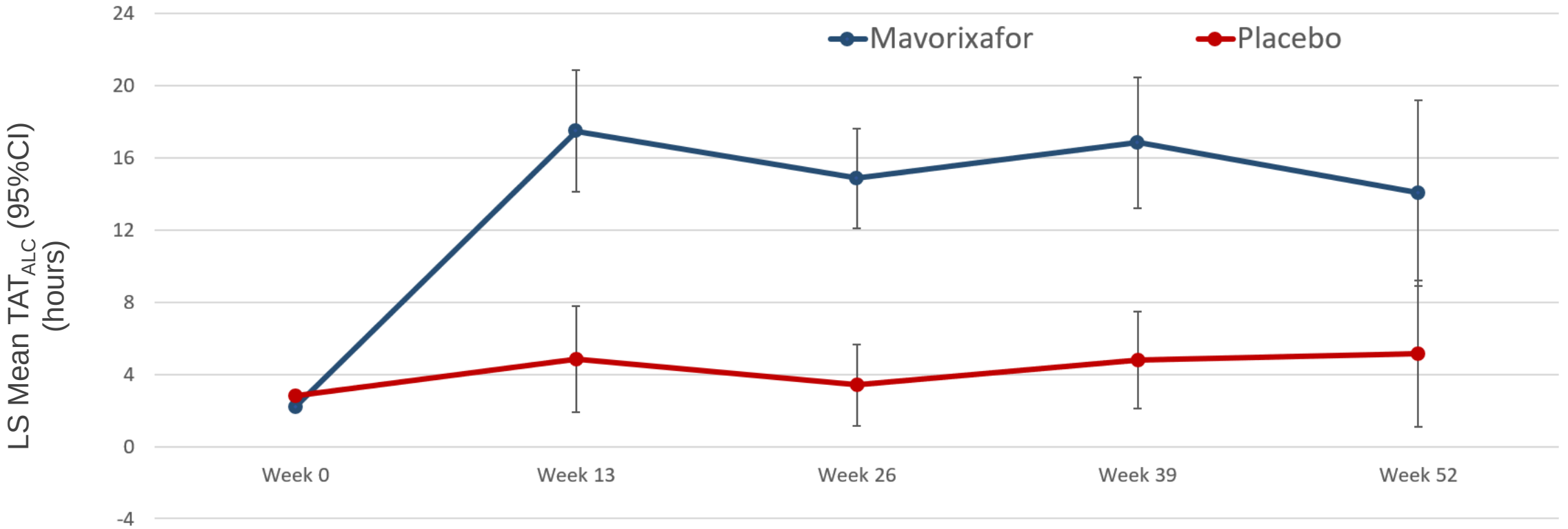
1. At week 52, 3 of 17 placebo patients were given mavorixafor in advance of their TAT measurements as they entered the open-label portion of the study. All data are included in ITT analysis.

Top Key Secondary Endpoint (TAT_{ALC}) Met

- Mavorixafor significantly improved the time above the threshold of ALC vs. placebo in ITT population
- Mean TAT_{ALC} of 15.80 hours for mavorixafor vs. 4.55 hours for placebo
- 3.5-fold improvement in TAT_{ALC} compared with placebo



TAT_{ALC} vs. Time on Treatment: Mavorixafor Durably Increased TAT_{ALC} Over 52 Weeks (ITT population)



Mavorixafor	n: 13	13	11	9	10
Placebo	n: 16	16	17	17	17[1]

TAT_{ALC} was increased and maintained over 52 weeks vs. placebo and vs. baseline

Increase of ~11.3 hours vs. placebo over the 52-week treatment period

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.

Top-Line Safety Data Summary for Randomization Period

	Mavorixafor (N=14)	Placebo (N=17)
	n (%)	n (%)
Summary		
Any TEAE	14 (100)	17 (100)
Treatment-related TEAE	7 (50)	3 (18)
Any Serious AE	5 (36)	2 (12)
Treatment-related Serious AE	0	0
Discontinuations due to AE	0	0
Treatment-limiting toxicity	0	0

Mavorixafor was generally well tolerated

No treatment-related Serious Adverse Events (SAEs)

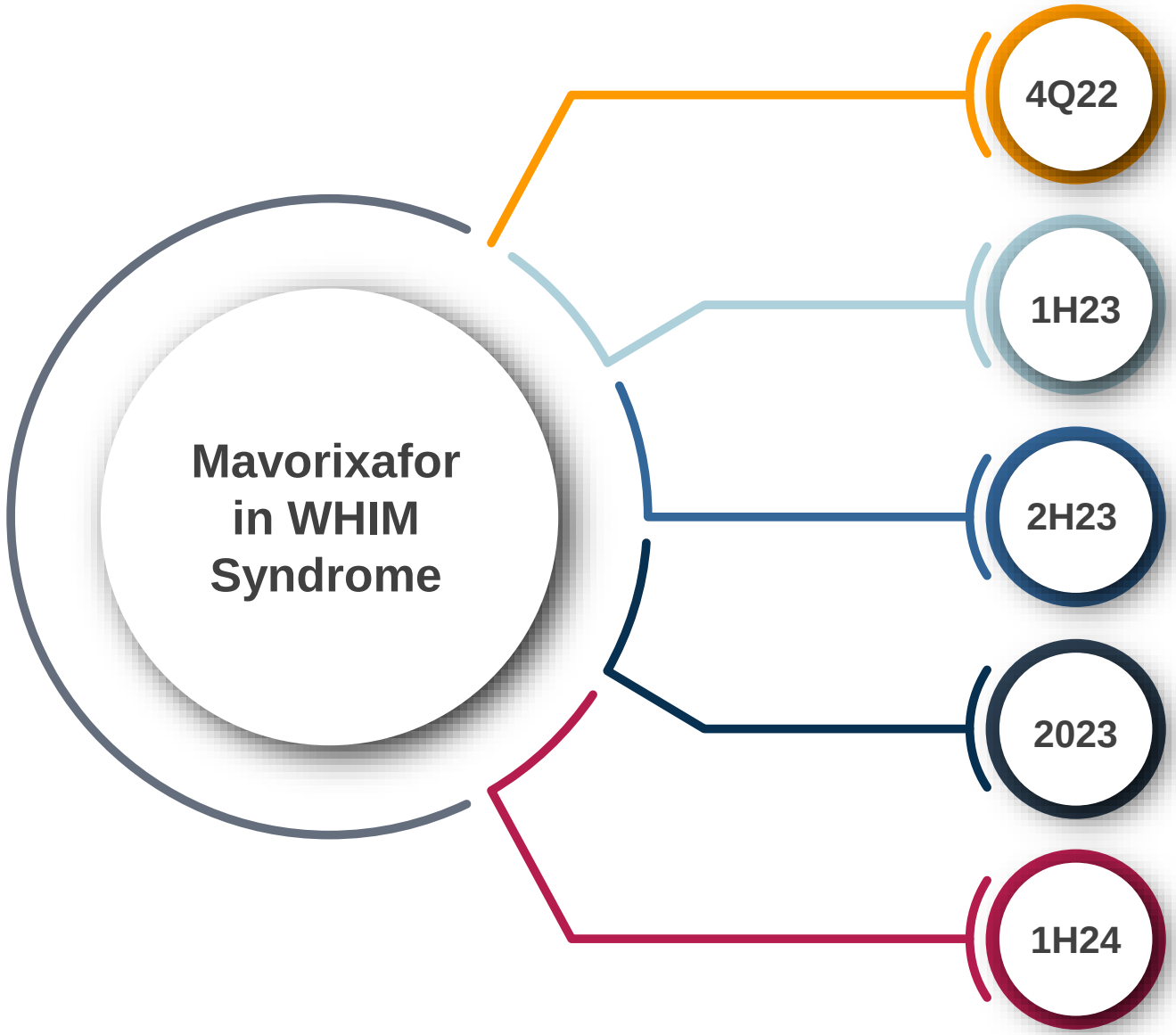
No discontinuations due to safety events

SAEs included: infections, glioma, thrombocytopenia - none deemed treatment related

Summary: 4WHIM Results

- Met primary endpoint (P<0.0001) of TAT_{ANC} – clinically meaningful correction of severe chronic neutropenia
- Durability of TAT_{ANC} response shown over 52 weeks of treatment
- Met first secondary endpoint (P<0.0001) of TAT_{ALC} - clinically meaningful correction of lymphopenia
- Durability of TAT_{ALC} response shown over 52 weeks of treatment
- Mavorixafor was generally well tolerated with no treatment-related serious adverse events reported

Next Steps/Expected Milestones to Advance Mavorixafor for the Treatment of WHIM



Achieved primary endpoint and key secondary endpoint in 4WHIM ✓

Pre-NDA meeting with U.S. FDA

Present additional 4WHIM trial results

U.S. New Drug Application (NDA) submission for mavorixafor in WHIM syndrome

Publish complete 4WHIM trial results

Continue with launch preparedness

Possible approval[1], PRV grant, and launch of mavorixafor in the U.S. for WHIM syndrome

1. Timeline assumes granting of priority review by U.S. Food and Drug Administration

THANK YOU!

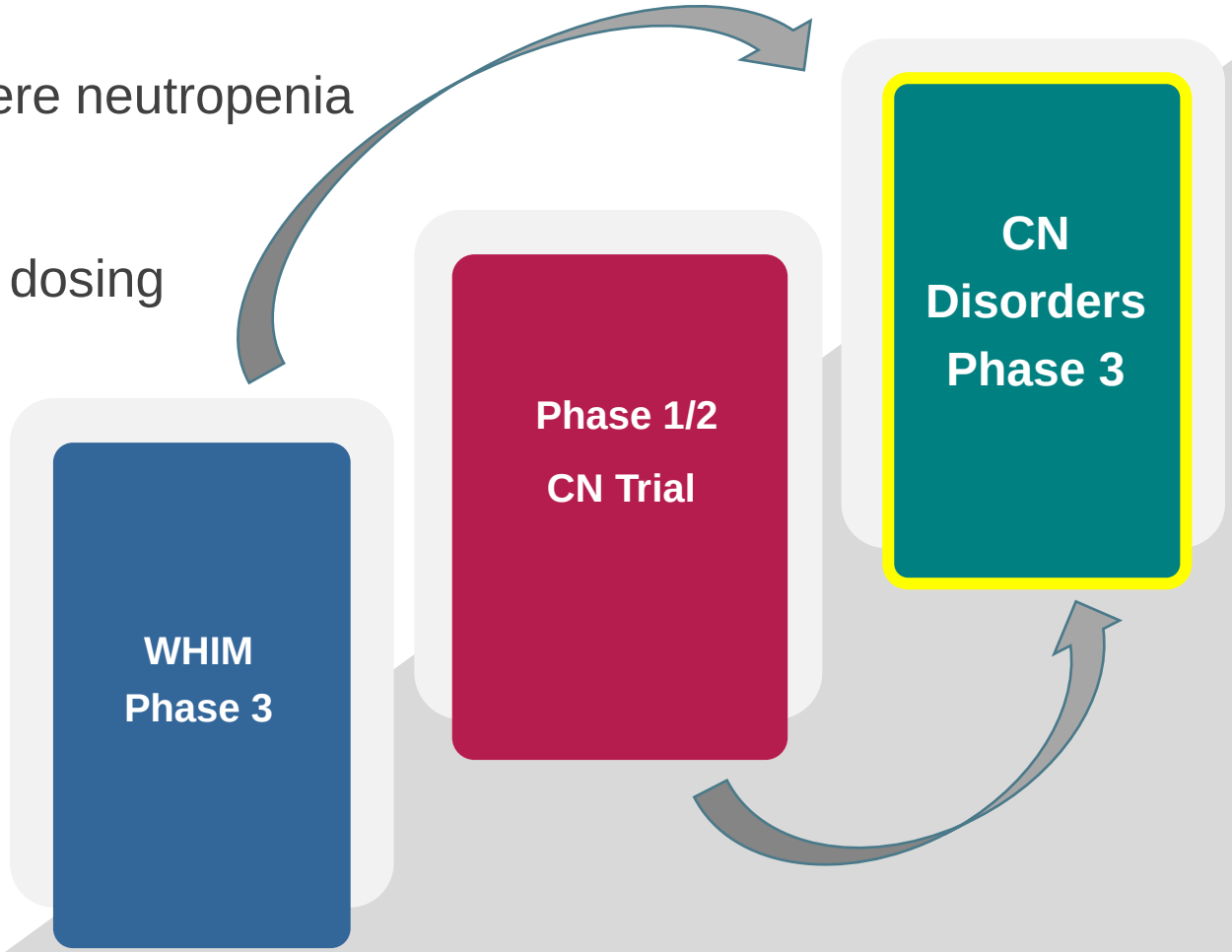
Study participants and their families

Clinicians, healthcare providers & study sites

X4 employees

Successful WHIM Phase 3 Supports Plan to Initiate CN Phase 3

- ✓ Primary Endpoint (TAT_{ANC}) = reduction of severe neutropenia
  - ✓ Placebo-controlled design
- ✓ Favorable tolerability profile over 52 weeks of dosing
- ✓ Durable response



Significant Near-Term Milestones Expected / Meaningful Growth Potential

2H 2022

Phase 1b CN Trial Data

4WHIM Phase 3 Topline Data

1H 2023

Initial CN Regulatory Path Clarity

Additional CN Data Expected:

- Durability & ANC data
- Explore G-CSF taper
- Long term safety data

2H 2023

WHIM NDA Submission

Advancing Towards CN Pivotal Trial

1H 2024

Potential WHIM NDA Approval & Launch

Potential Receipt of Priority Review Voucher

Potential Phase 3 CN Trial Initiation

Q&A