3Q 2022 Earnings Call: The Road to 4WHIM Data and Beyond

November 3, 2022

Forward-Looking Statements

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A Unique Investment Opportunity with Significant Near-Term Milestones

Sharp focus on chronic neutropenic (CN) disorders, including WHIM syndrome

Bringing innovative treatments to ~50,000 estimated patients diagnosed with rare diseases of the immune system and high unmet needs

Mavorixafor – Oral, late-stage clinical CXCR4 antagonist candidate

Designed to be a once-daily oral therapy enabling the mobilization of immune cells from the bone marrow to the blood to improve immune system function

X4 PHARMACEUTICALS

Key recent & upcoming expected clinical milestones:

- ✓ Positive results from Phase 1b trial in chronic neutropenia – 3Q 2022
- **Results from global, pivotal Phase 3 trial in WHIM syndrome – 4Q 2022**
- Additional CN clinical data – 1H 2023
- U.S. NDA submission expected in WHIM – early 2H 2023
- Regulatory path clarity in CN – 2023

Unparalleled expertise in CXCR4 biology

Antagonizing the CXCR4/CXCL12 axis proven to increase the mobilization of white blood cells, including neutrophils

Strong balance sheet, with cash runway expected to fund operations into 3Q 2023

July 2022 fundraising, re-prioritization of resources, and other measures projected to extend runway though key upcoming milestones

X4 Focus on a Broad Range of Chronic Neutropenic Disorders

~50,000 Estimated Chronic Neutropenia Patients in the U.S.[1]

Idiopathic

~40,000

Most commonly diagnosed chronic neutropenia

Not attributable to drugs or specific infectious, inflammatory, autoimmune or malignant causes

Cyclic

~5,000

Typically, a 21-day cycle

Autosomal-dominant disorder

Can be caused by *ELANE* mutations

Congenital

~3,000

Rare hematological genetic diseases

Can be caused by *ELANE* and other mutations

WHIM Syndrome

Combined immunodeficiency

(low ANC, low ALC/AMC, hypogammaglobulinemia)

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

WHIM Syndrome: Immunodeficiency Disorder 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 can lead to devasting, irreversible morbidities

M **yelokathexis.** A “hyper-dense” population of immune cells in the bone marrow, reducing the ability to achieve a healthy immune response

No targeted therapies approved to treat underlying cause / pathophysiology

- Current treatments do not address mechanism of disease
- Treatments include antibiotics, G-CSF, and immunoglobulins; do not correct lymphopenia, are not effective against HPV related-disease, have no effect on cancer prevention

Range of Assessments Help Establish a WHIM Diagnosis

Primary Clinical Assessments

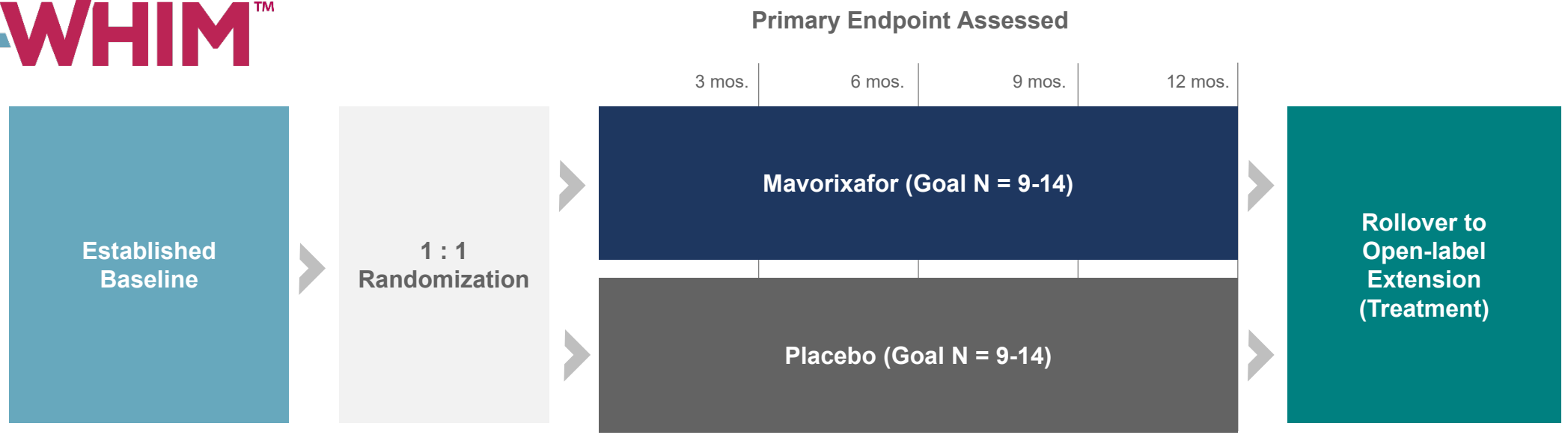
- **Neutropenia & lymphopenia (low ANC and ALC)**
- Repeat infections with long-term effects
- *In some:* wart lesions; cervical test for HPV
- *In some:* Low immunoglobulin (Ig) levels

Additional Assessments

- Bone Marrow Biopsy
- Genetic Testing
- Family History

Phase 3 Study: Results Expected 4Q 2022

4WHIM™



- **Enrollment:** over-enrolled with 31 patients from 12 countries; **LPLV complete for placebo-controlled study**
  - **Severe neutropenia** at baseline = mean ANC of 220 cells/μL
  - 48% are pediatric (ages 12-17); supports potential for **Priority Review Voucher**
- **Open Label Extension:** >90% of eligible study participants transitioned to OLE
- **Safety:** ongoing evaluation from Data Safety Monitoring Board supports study continuation
- **Key Assessments:** assessing neutropenia, lymphopenia, safety & clinical benefit

Phase 3 Study: Primary Endpoint and Key Assessments

Assessment of correcting neutropenia

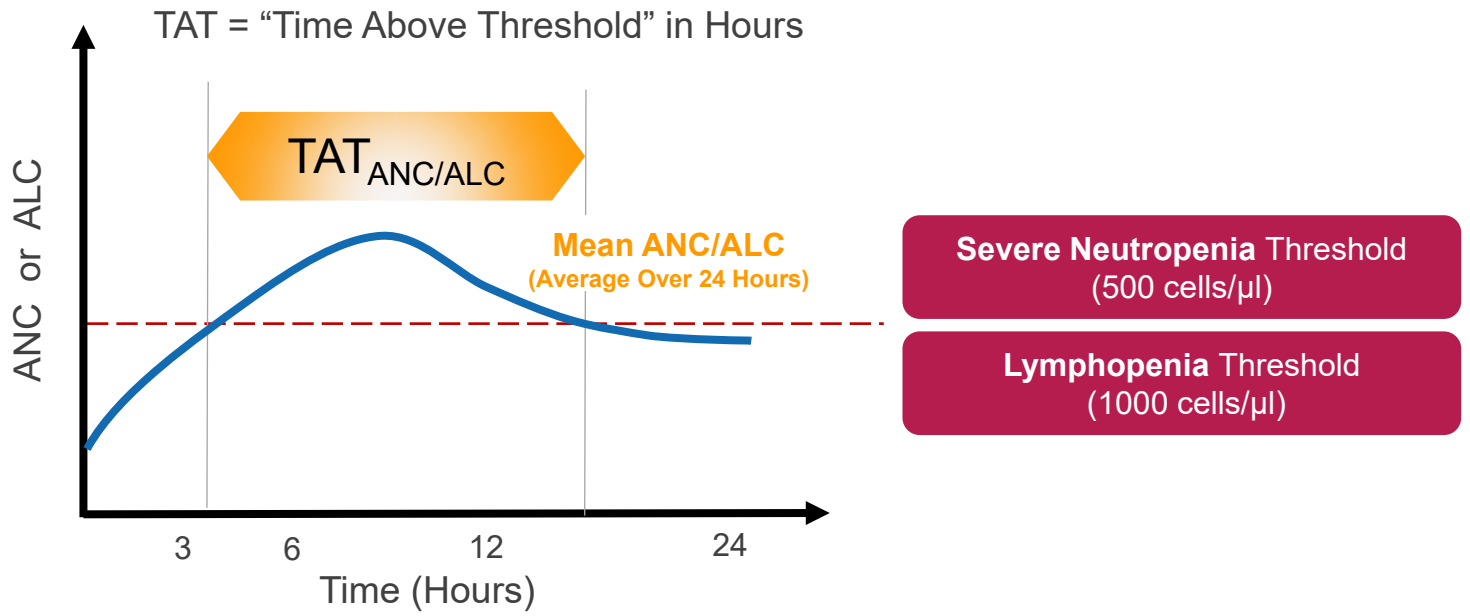
Assessment of correcting lymphopenia

Assessment of impact on quality of life & other outcomes

Primary Endpoint = TAT_{ANC} → **Top Secondary Endpoint = TAT_{ALC}** → **Safety and Benefit**

(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)

Positive WHIM Phase 2 Results Informed Phase 3 Design

Increases in TAT_{ANC} seen in Phase 2

Increases in TAT_{ALC} seen in Phase 2

$TAT_{ANC/ALC}$ ranges of 2-24 hours seen in patients on Phase 3 dose

Used to power Phase 3 to >95% in primary & top secondary endpoint

All responded with >4x increase in peak ANC and ALC

100% of patients expressed benefit in Patient Reports

Observed Improvements in infection rate and wart burden

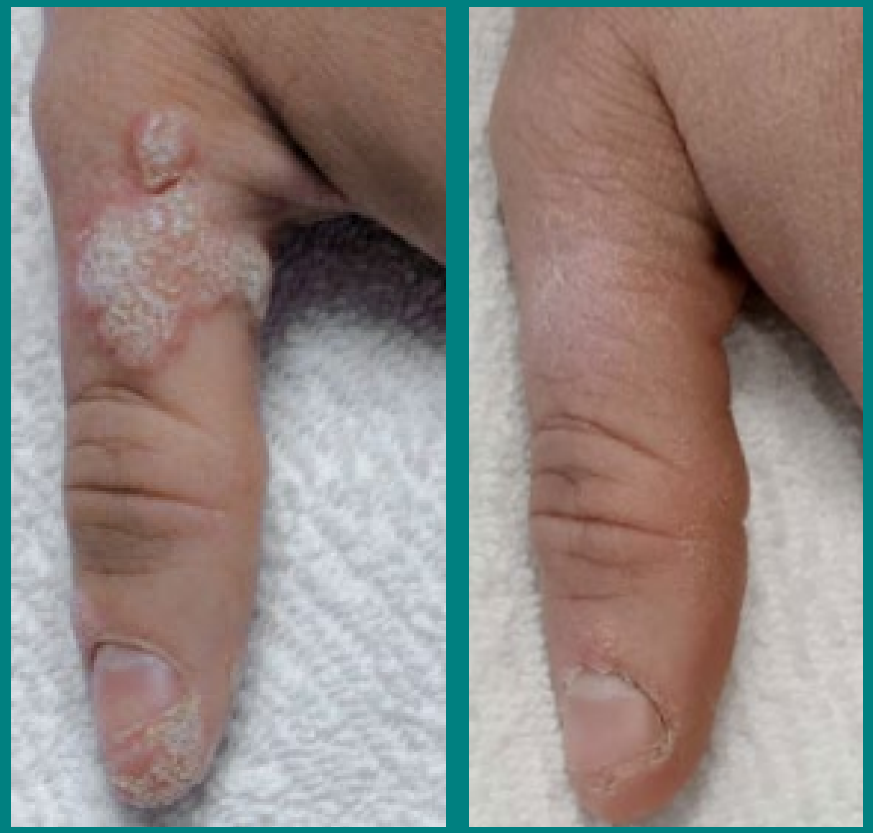
Well Tolerated over more than 3 years of treatment

Granted Breakthrough Therapy Designation in WHIM Syndrome

Data sources: Dale et al, *JCI*, 2020; Dale et al, Poster, ASH, 2021.

Phase 2 Results Informed Phase 3 Design: Clinical Impact

Thumb of Affected WHIM Patient In Phase 2 study



Pre-Treatment

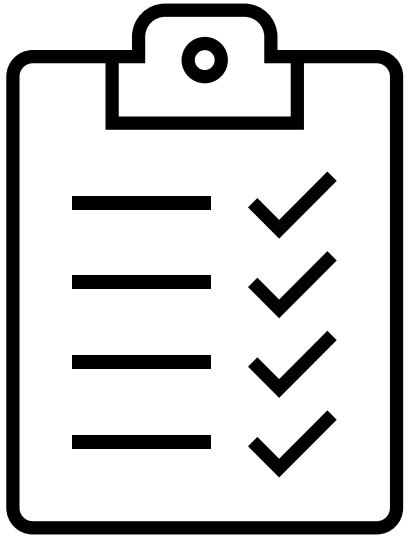
Following 55 weeks of treatment

- ✓*Overall positive Patient Global Impression of Change (PGIC)[1]*
- ✓*Improvement in warts*
- ✓*Improvement in infections*

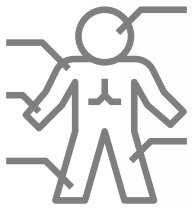
1. Dale et al, Poster, ASH, 2021.

Breakthrough Therapy Designation (BTD): FDA Guidance & Engagement

- **Multiple FDA interactions over several years**
- **FDA input:**
  - Informed on study design, endpoints, analyses
  - Recommended “composite endpoint” for infection burden
  - Recognition of the rarity, heterogeneity of WHIM syndrome



Mavorixafor: the First Potential Treatment for WHIM Syndrome



X4

SUPPORTING DIAGNOSIS

Diverse Efforts to Support Physician Education & Diagnosis

Clinical	Medical Education (MSLs) Publications, Conferences, Symposium Sponsorships
Patient Advocacy	Existing neutropenia registries (NNN, SCNIR) Global WHIM registry in development (IPOPI)
Bone Marrow Evaluations	Incidental findings from ruling out other diseases Working towards a Myelokathexis Center of Excellence
Genetic Testing	PATH4WARD and Invitae Patient Diagnostic Liaisons (Field)
Family History	Patient Education Liaisons (Field) Patient Advocacy outreach to communities

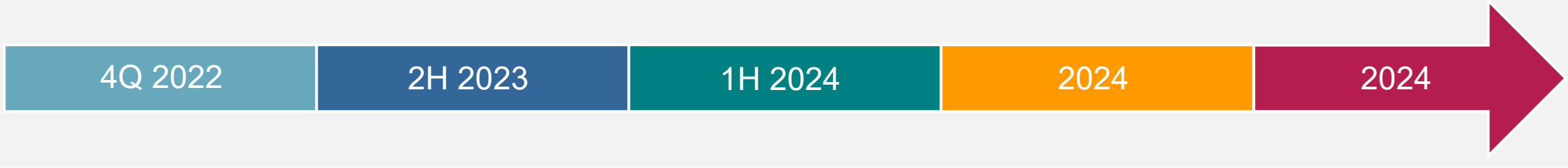
GOAL LABEL**: Indicated for the treatment of people aged 12 and above **diagnosed with WHIM syndrome

Timeline and Commercial Strategy

Initial Focus on the U.S.

EU Regulatory and Commercial Launch

Territories Outside of US / Key European countries



4Q 2022	2H 2023	1H 2024	2024	2024
Phase 3 Data in 4Q 2022	Expected NDA submission early 2H 2023	Expected U.S. launch in 1H 2024	EMA filing ~12 Months following U.S. submission Launch sequence: top 5-7 European countries will be prioritized	Partnership discussions to be explored

Commercial Leadership – Introduction to Mark Baldry, Chief Commercial Officer

- 30+ years' experience in global life science commercial strategy and operations
- Particularly skilled in:
  - Building high-performing commercial teams
  - Developing effective global strategies
  - Launching new products into rare disease and specialty markets
- Previously Chief Commercial Officer at Freeline Therapeutics and Wave Life Sciences
- Prior leadership positions at Amicus Therapeutics, Biogen, and the Human Genetic Therapies division of Shire
- MBA from Concordia University (Canada) and a BSc in Genetics from York University (UK)

Leveraging Significant Rare Disease Expertise for Potential 2024 U.S. Launch

Physician & Patient Education/Engagement

Building the Commercial Team

U.S. and EU Payer Engagement

Marketing/Branding

Manufacturing & Distribution (U.S.)

WHIM Efforts Align Well with Potential Expansion into CN Disorders

WHIM syndrome and CN physicians and researchers significantly overlap

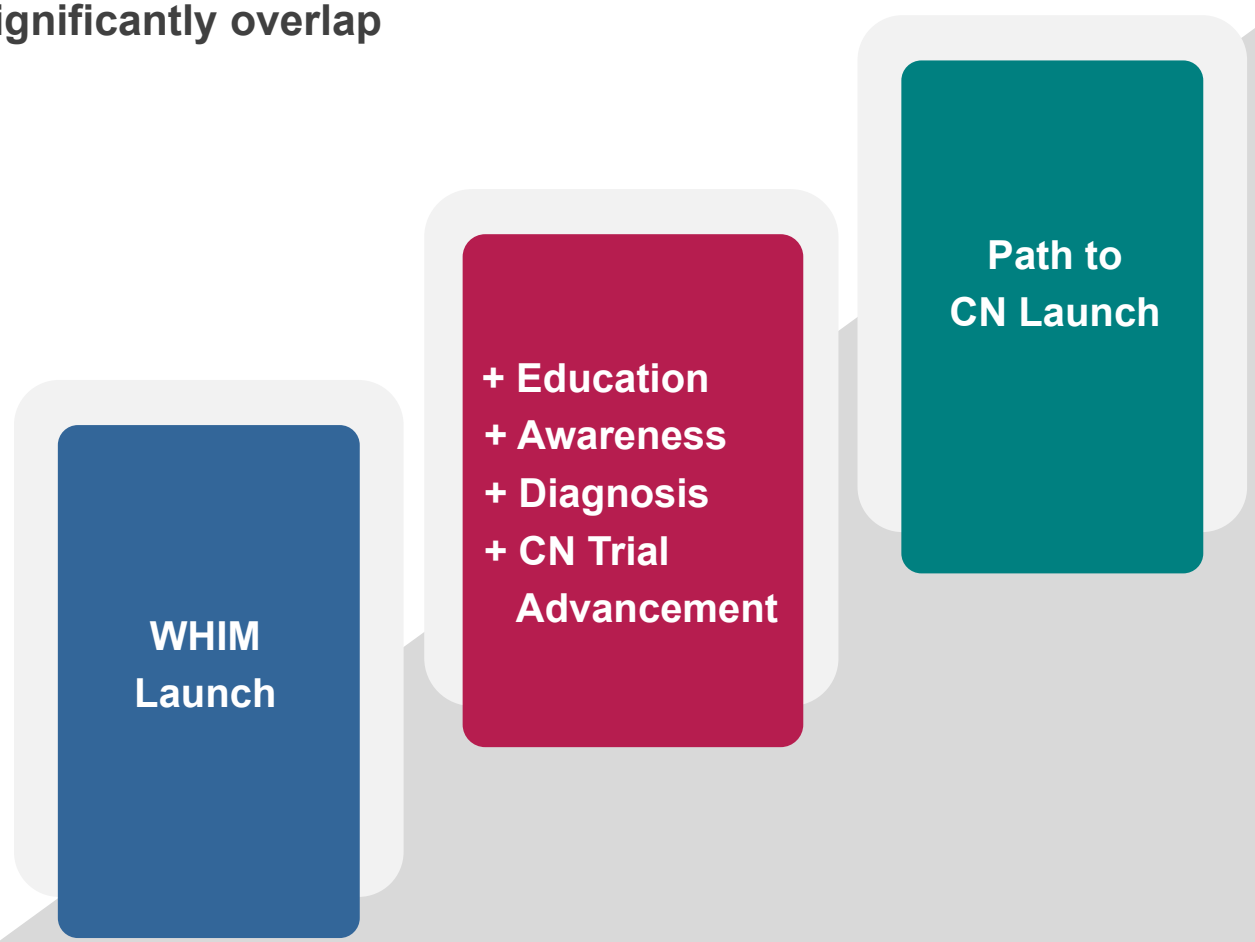
- Non-malignant hematology specialists
- Immunologists

Patient foundations provide support to all

- National Neutropenia Network (NNN)
- Jeffrey Modell Foundation (JMF)
- Immunodeficiency Foundation (IDF)
- International Patient Organisation for Primary Immunodeficiencies (IPOPI)

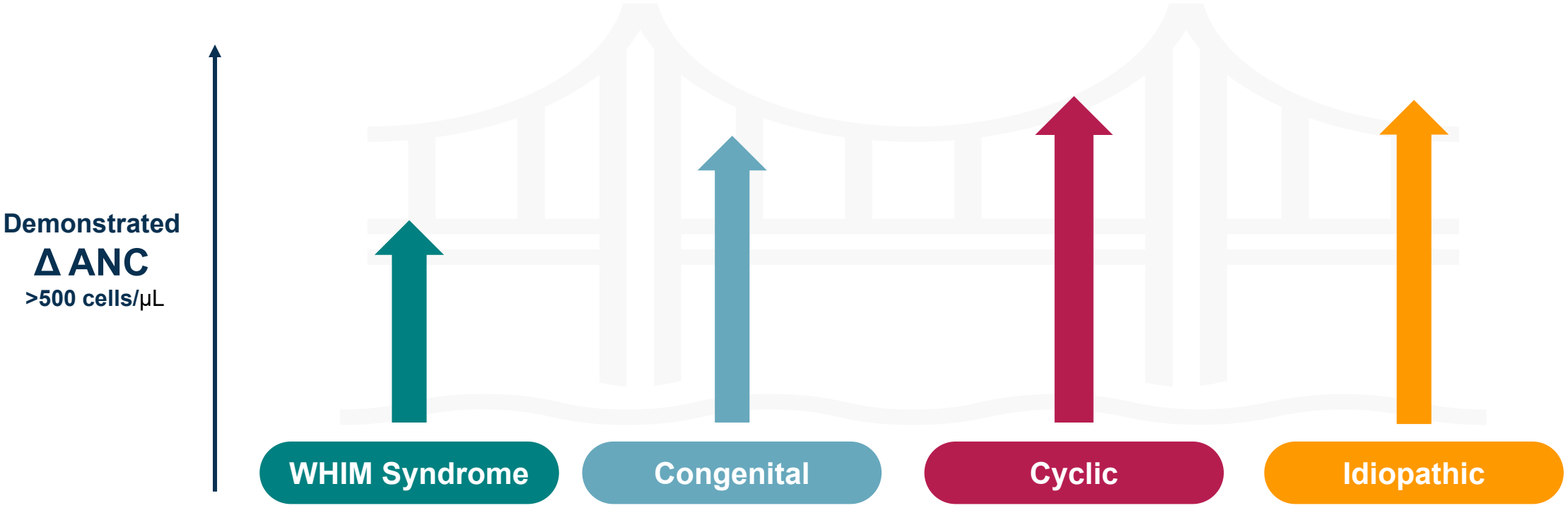
Ability to leverage X4's core commercial operational capabilities and infrastructure across:

- Supply chain, distribution channel, advocacy, sales, medical affairs and market access



Mavorixafor: Data Support Further Development across Broad CN Disorders

Potential to be Only Oral Therapy to Treat Chronic Neutropenia



~50,000 diagnosed U.S. patients

Significant Near-Term Milestones / Meaningful Growth Potential

Q3 2022

Phase 1b CN Results

Q4 2022

4WHIM Phase 3 Trial Readout 4Q 2022

Advancing mavorixafor into Phase 1b/Phase 2 CN clinical trial

1H 2023

Initial CN Regulatory Path Clarity

Additional CN Data Expected:

- Durability & ANC data
- Potential data on G-CSF taper
- Chronic safety data

2H 2023

WHIM NDA Submission

Advancing Towards CN Pivotal Trial

Q&A Session / Conclusion