X4 PHARMACEUTICALS

November 9, 2023

PROGRESS 4 PATIENTS

Developing the first oral treatments for chronic neutropenic disorders

Forward-Looking Statements

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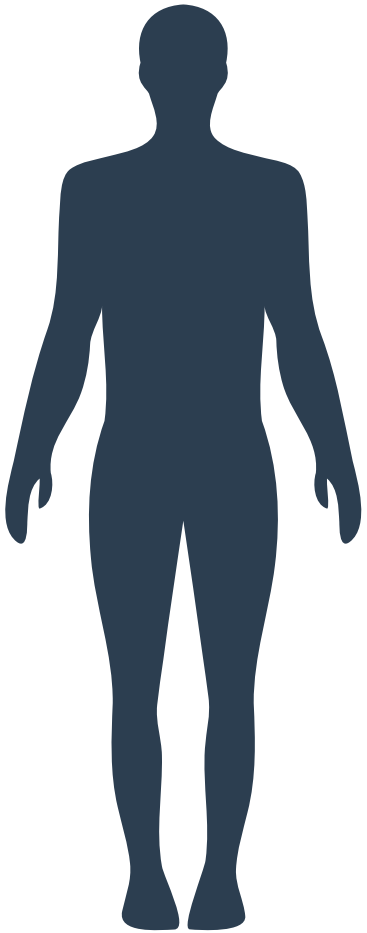
Key 3Q 2023 Events & Recent Highlights

Mavorixafor for WHIM NDA accepted for Priority Review	FDA sets PDUFA date of April 30, 2024; X4 eligible for Priority Review Voucher (PRV); preparing for 2Q 2024 U.S. launch
CN program advancing to Phase 3	>15 participants now enrolled in Phase 2 CN trial; key learnings and FDA input have enabled finalization of pivotal, global Phase 3 CN trial design
Strengthened leadership team and company BOD	Addition of Christophe Arbet-Engels, MD, PhD, as Chief Medical Officer and Keith Woods to Board of Directors adds significant rare disease drug development and commercialization expertise
Completed $115 million loan facility	Early August deal with Hercules Capital provides non-dilutive financing options
Cash and equivalents at end of 3Q23 totaled $142.7 million	Available funds expected to fund operations into 2025*

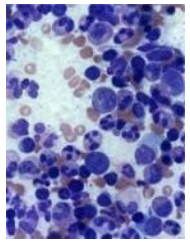
*Current runway projection does not include monetization of possible PRV received should mavorixafor gain U.S. approval for WHIM syndrome

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

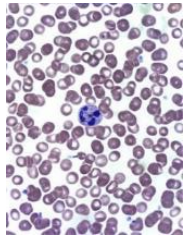
Clinical symptoms driven by over-signaling in the CXCL12/CXCR4 pathway



WHIM Disease Bone Marrow[2]



Healthy Bone Marrow[2]



- Decreased white blood cell counts lead to:
  - immune system dysfunction
  - increased risk of infections (lungs/upper & lower respiratory, skin/cellulitis, heart/endocarditis)
  - HPV-related cancers
- U.S. population estimated at ~1,000[3]
- Earlier diagnosis may improve patient quality of life, reduce morbidity/mortality[4,5]
- Successful Phase 3 4WHIM trial demonstrated mavorixafor's ability to raise absolute neutrophil and lymphocyte counts and reduce the rate, frequency, and severity of infection in adults and adolescents with WHIM syndrome.

Mavorixafor, an investigational oral CXCR4 inhibitor, the first potential disease-modifying agent for people with WHIM syndrome

1. WHIM syndrome (Warts, Hypogammaglobulinemia, Infections, and/or Myelokathexis) 2. McDermott: *Stiehm's Immune Deficiencies* 2014, Pages 711-719;
3. Company market research. Qessential market research, 2019 and IPM.ai artificial intelligence study, 2020 4. Heusinkveld LE, et al. *Expert Opin Orphan Drugs*. 2017;5(10):813-825. 5. Geier CB et al., *Clin Immunol*. 2022 Nov;42(8):1748-1765.

Shaping the WHIM Landscape – Turning Challenges into Opportunities

- No approved disease-modifying treatments
  - Symptomatic disease management
- Diagnosis: variable clinical presentation of disease
  - Only 23% of patients have all four classic WHIM features[1]
  - Not all symptoms are needed for diagnosis
  - Differing approaches to diagnosis – genetic testing can help
- Low disease awareness
- Limited historical support for WHIM patients and physicians

Significant unmet needs and no industry attention / innovation

Large potential undiagnosed patient population

1. Geier et al, 2022 *Journal of Clinical Immunology*

X4 Progress Towards Commercialization

Partnering with the WHIM community

- MSL team identifying and engaging with KOLs
- Fostering strategic collaborations with Patient Advocacy Groups & professional societies
- Supporting the medical community to further advance understanding and treatment of WHIM syndrome

Driving earlier diagnosis of WHIM

- Raising disease awareness / providing diagnostic support to reduce time to diagnosis
- Targeted conference presence, peer-to-peer symposia
- Diagnosis team engaging with clinicians who have identified symptomatic WHIM patients

Ensuring broad access for eligible patients

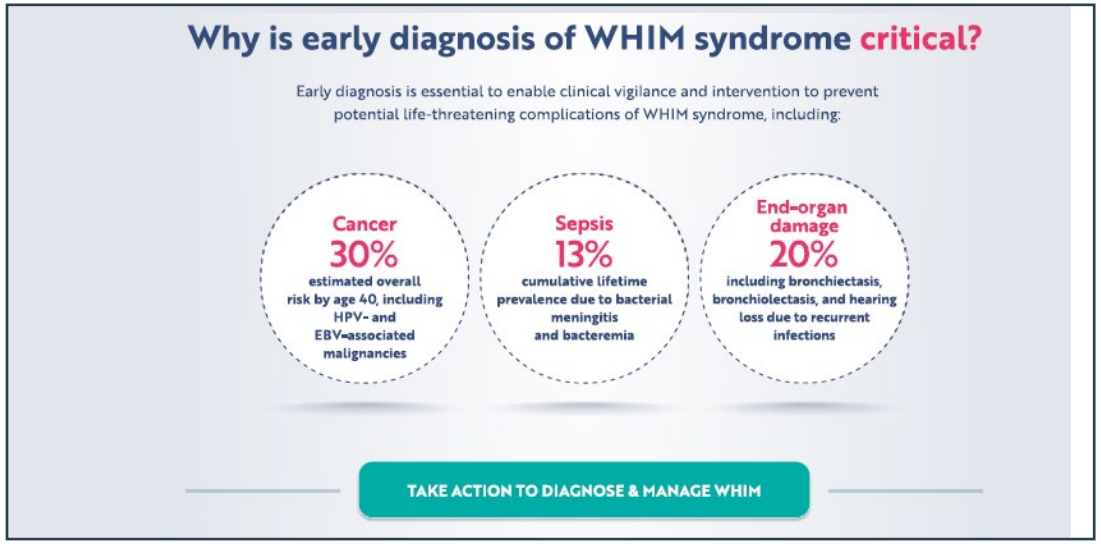
- Communications plan highlighting the compelling value proposition of mavorixafor
- Utilizing a rare disease distribution strategy to maximize prescription fulfillment and patient adherence
- Designing services and programs to ensure supportive patient experience

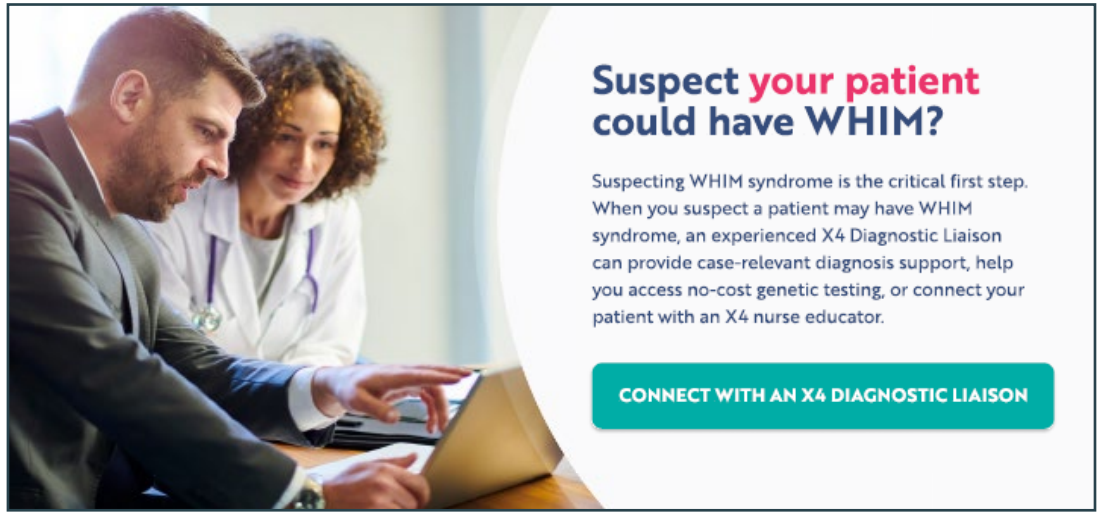
Delivering on the promise of mavorixafor

- Creating a “fit for purpose” organization, capabilities, processes, and infrastructure
- Readying for 2Q 2024 U.S. launch
- Expect to leverage commercial investment into CN opportunity

Disease Awareness → **Earlier Diagnosis** → **Patient Finding**

“What If It’s WHIM?” Disease Awareness Campaign





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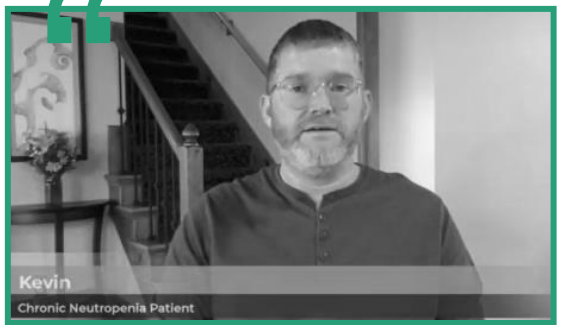
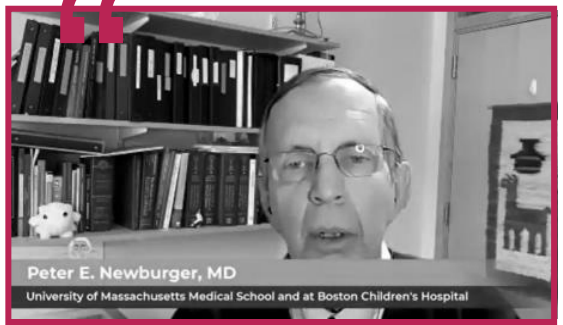
What Makes A Difference to CN Patients and Their Physicians?

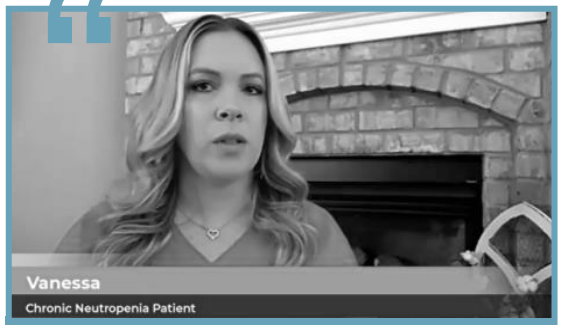
"there is a major diagnostic gap currently…, and also treatment options are extremely narrow"



"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 and maintaining the oral mucosa and dental health."



".. 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."

Emerging Phase 2 Trial Results Support Mavorixafor Making a Positive Difference for CN Patients

Expanded treatment options beyond G-CSF, ideally:

- Oral formulation & fixed dosage
- Durable, increased and/or normalized ANCs
- Good safety profile
- Reduce infection rates
- Enable G-CSF reduction & reduce G-CSF-related toxicities

Progress in CN Phase 2 Trial Continues To Support Mavorixafor's Potential Benefit

>15 Participants Now Enrolled in Trial

Initial 3 Participants on G-CSF Completed 6-Month Study

Full data of three subjects to be presented at ASH 2023 in December

- Oral formulation, fixed dosage,
- Well tolerated, acceptable safety profile
- Durable, increased/normalized ANCs
- Preliminary data supports G-CSF reduction or withdrawal

Additional Data Expected During

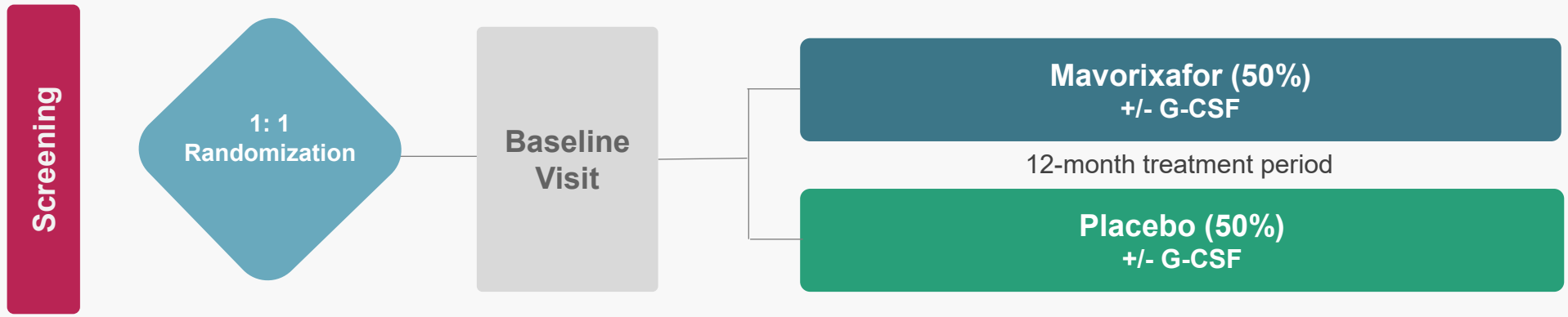
1H 2024

Robust Data Across Multiple Studies De-Risk CN Phase 3 Pivotal Trial

- Phase 1b CN data – 100% response in 25+ subjects
- Phase 2 CN data – durable ANC responses, acceptable safety profile in combination with G-CSF in initial cohort (n=3) of subjects
- WHIM Phase 3 pivotal – major reductions in rate, severity, and duration of infections, with durable ANC increases

CN Pivotal, Global Phase 3 Trial: Delivering A Potential New Treatment Option

Incorporates FDA Guidance



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[2]); same mavorixafor dosing as 4WHIM trial

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

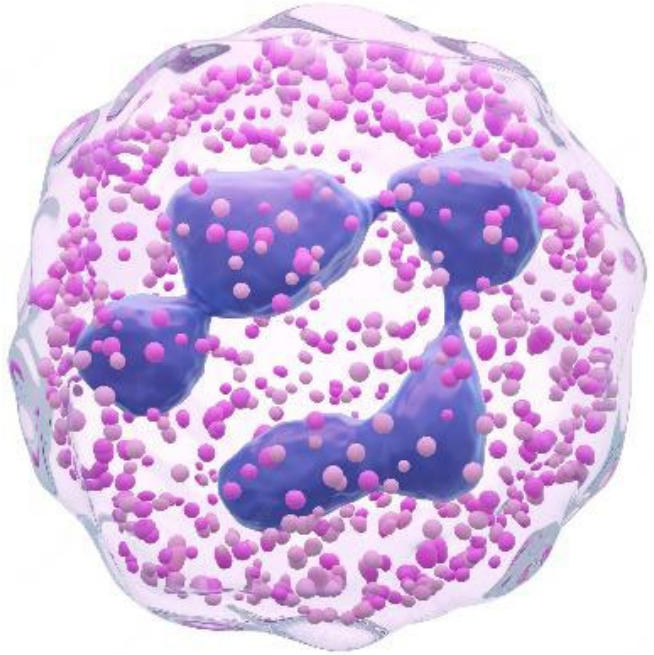
Endpoint and Power: 150 subjects, ≥90% on primary endpoints of annualized infection rate and ANC response

1. ANC response: two of three ANC measurements in the first 3 months reach >1500 cells/uL (for participants with 500<ANC<1500 at BL) or 100% increase over baseline (for those ANC <500 at BL)
2. Fors 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.

CN Pivotal, Global Phase 3 Clinical Trial: Progress and Upcoming Milestones

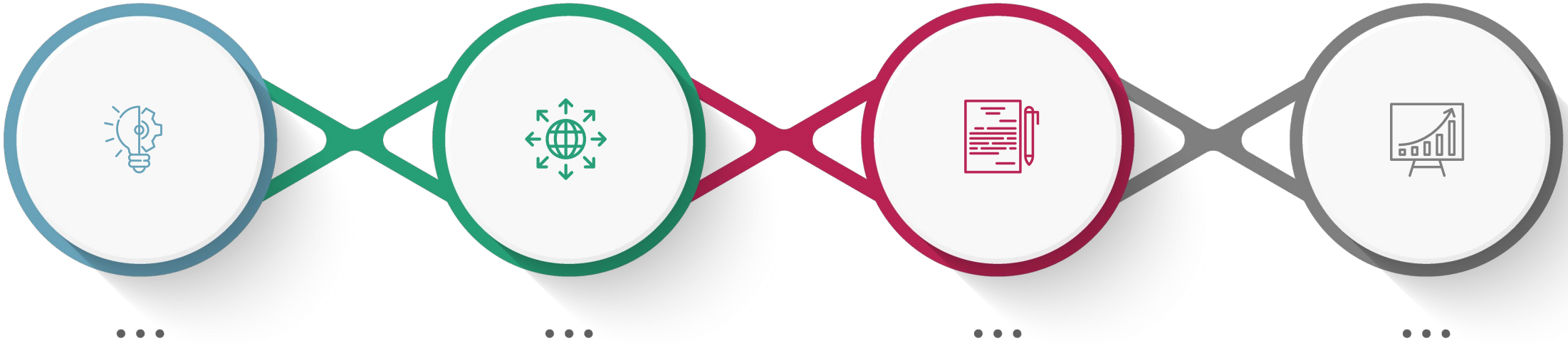
On track for first patient dosed (FPD) in 1H 2024

- Protocol finalized
- CRO in place, advancing towards activation of global trial sites
- Large pool of potential trial participants already identified
  - Estimating ~12-months to full enrollment



GOAL LABEL: Indicated to reduce infections rate in adults and children 12 years and older with congenital, autoimmune, or idiopathic chronic neutropenia

Exciting Look-Ahead to 2024



Comprehensive Data from >15 Participants in CN Phase 2 Trial Expected in 1H 2024

Pivotal, Global, Chronic Neutropenia Phase 3 Clinical Trial Initiation Planned in 1H 2024

Potential Approval and Launch of First-Ever Treatment for WHIM Syndrome in 2Q 2024

X4's Potential to Grow into a Global Commercial Enterprise in 2024 and Beyond

Q&A