
XOLREMDI™
(mavorixafor) capsules
X4 PHARMACEUTICALS
Investor Call
April 29, 2024
PROGRESS 4 PATIENTS

Forward-Looking Statements

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Any forward-looking statements in this presentation are based on management's current expectations and beliefs. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond X4’s control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including the risks that: X4’s launch and commercialization efforts in the U.S. with respect to XOLREMDI may not be successful, and X4 may be unable to generate revenues at the levels or on the timing we expect or at levels or on the timing necessary to support our goals; the number of patients with WHIM syndrome, the unmet need for additional treatment options, and the potential market for XOLREMDI may be significantly smaller than we expect; XOLREMDI may not achieve the clinical benefit, clinical use or market acceptance we expect or we may encounter reimbursement-related or other market-related issues that impact the success of our commercialization efforts; we may encounter adverse events for XOLREMDI at any stage that negatively impact commercialization; X4’s ability to establish and maintain an effective sales and marketing organization or suitable third-party alternatives for any approved products; X4’s, as an organization, lack of experience in sales, marketing or distribution of pharmaceutical products; X4’s other product candidates; X4’s possible exploration of additional opportunities and indications for XOLREMDI; the expected availability, content, and timing of clinical data from X4’s ongoing clinical trials of XOLREMDI; clinical trial design and enrollment for clinical trials as well as potential therapeutic benefits, including the current design for a potential Phase 3 clinical trial evaluating XOLREMDI in certain chronic neutropenic disorders and its expected initiation in the first half of 2024; patient prevalence; market opportunities; and X4’s use of capital and other financial results, including its financial runway. Actual events or results may differ materially from those expressed or implied by any forward-looking statements contained in this presentation on account of many factors, including, without limitation, risks of obtaining and maintaining regulatory approvals, including, but not limited to, potential regulatory delays or rejections; uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; the risk that trials and studies may be delayed and may not have satisfactory outcomes; the risk that the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the risk that initial or interim results from a clinical trial may not be predictive of the final results of the trial or the results of future trials; the potential adverse safety effects arising from the testing or use of our product and product candidates; the risk that patient prevalence, market, or opportunity estimates may be inaccurate; the impacts of general macroeconomic and geopolitical conditions on X4’s business; risks related to X4’s ability to raise additional capital; risks related to the substantial doubt about X4’s ability to continue as a going concern; changes in expected or existing competition; changes in the regulatory environment; risks related to X4’s ability to successfully market mavorixafor, if approved; unexpected litigation or other disputes; the need to align with our collaborators may hamper or delay our development and commercialization efforts or increase our costs; our business may be adversely affected and our costs may increase if any of our key collaborators fails to perform its obligations or terminates our collaboration; the internal and external costs required for our ongoing and planned activities, and the resulting impact on expense and use of cash, may be higher than expected which may cause us to use cash more quickly than we expect or to change or curtail some of our plans or both; and other risks and uncertainties, including those described in the section entitled “Risk Factors” in X4’s Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 21, 2024, and in other filings X4 makes with the SEC from time to time. X4 undertakes no obligation to update the information contained in this presentation to reflect new events or circumstances, except as required by law.

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Welcome and Overview

Paula Ragan, PhD, President & Chief Executive Officer

Label Highlights and Supporting Clinical Data

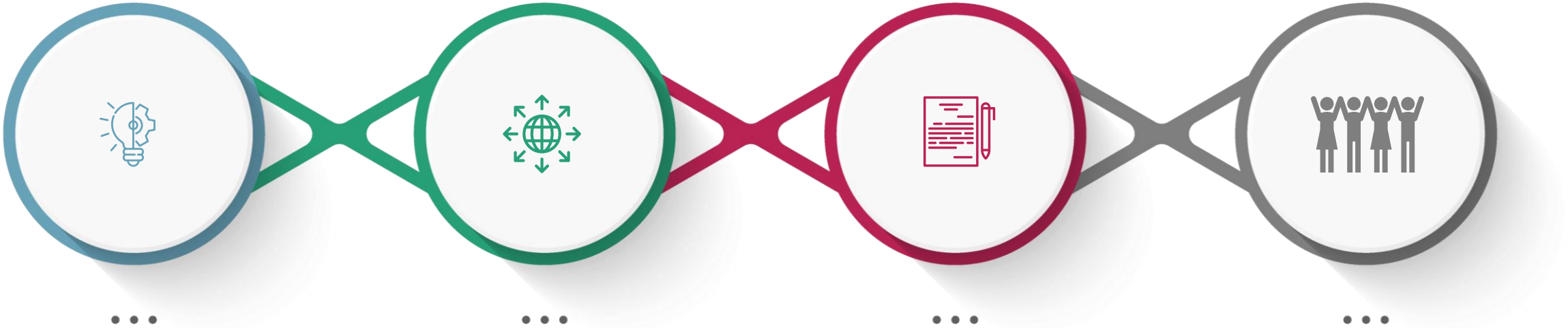
Christophe Arbet-Engels, MD, PhD, Chief Medical Officer

Commercial Launch Strategy & Execution

Mark Baldry, Chief Commercial Officer

Conclusion & Q&A

X4: delivering innovation for people with rare immune disorders



2014
X4 Founded to advance CXCR4-targeted therapeutics for people with rare diseases

2022
X4 announces positive top-line results from pivotal, global, Phase 3 trial of mavorixafor in WHIM syndrome

2023
FDA accepts NDA for mavorixafor in WHIM syndrome, granting Priority Review

2024
FDA approves XOLREMDI™ (mavorixafor), the first and only therapy indicated in patients with WHIM syndrome

CXCR4 = CXC chemokine receptor 4; WHIM = warts, hypogammaglobulinemia, infections, myelokathexis

Now FDA approved!

(zōl-RĚM-dee)

WHIM syndrome is a combined primary immunodeficiency and a chronic neutropenic disorder[1]

Heterogeneous presentation[2]

Most frequently characterized by:

Neutropenia (98%)

Hypogammaglobulinemia (65%)

Recurrent infections (92%)

Warts (40%)

Fewer than 1 in 4 patients present with all 4 manifestations in the WHIM acronym (warts, hypogammaglobulinemia, infections, and myelokathexis)

Based on an international cohort of 66 patients with WHIM syndrome, which included pediatric (65%) and adult (35%) patients.

Lifelong impact[2]

Chronic, congenital disorder

Commonly presents in childhood, with median age of diagnosis of 5.5 years of age

Lower life expectancy vs. the general population[3,4] due to sepsis, irreversible organ damage, recurrent pneumonia, and certain cancers

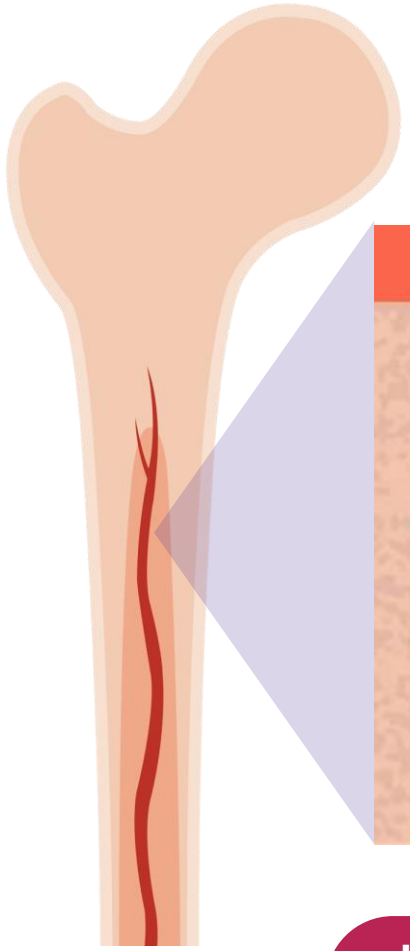
Ultra-rare population[5]

Estimated to be at least 1,000 people in the U.S.

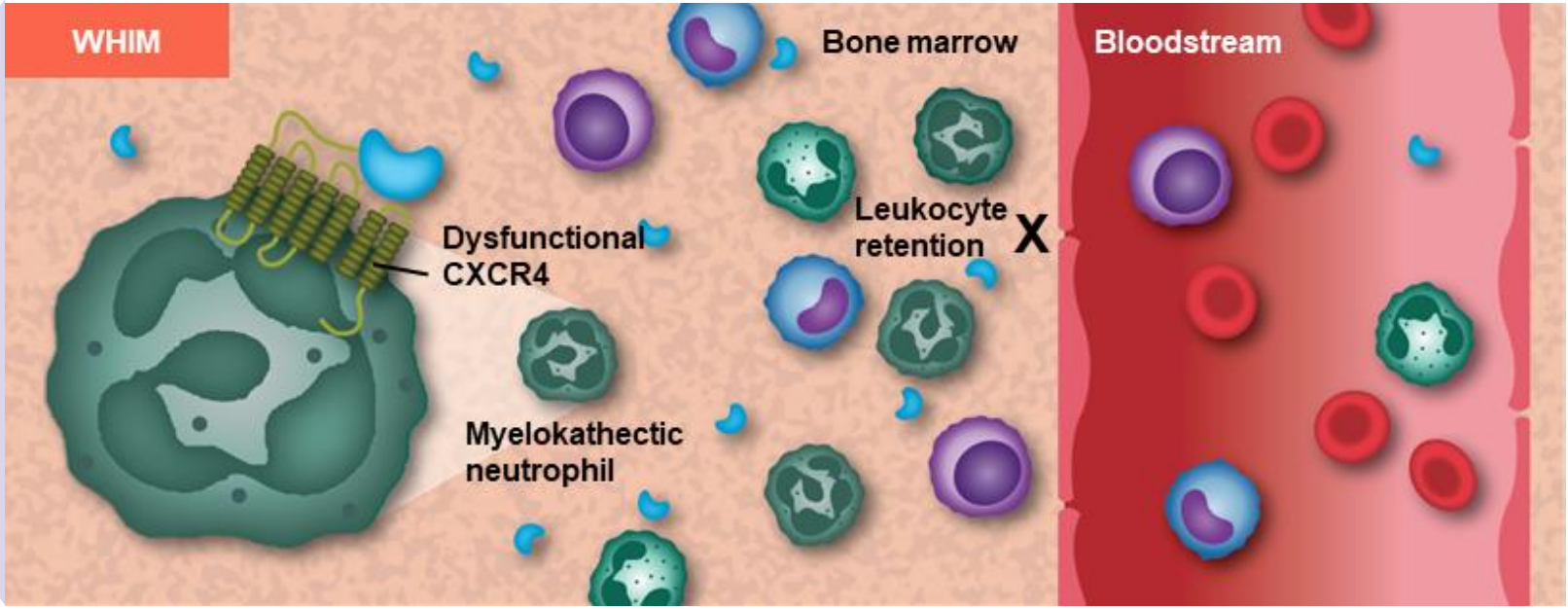
Based on X4 market research 2019, 2020.

References: 1. Dale DC, Firkin F, Bolyard AA, et al, *Blood*. 2020;136(26):2994-3003. 2. Geier CB, Ellison M, Cruz R, et al, *J Clin Immunol.* 2022;42(8):1748-1765; 3. Dotta L, Notarangelo L, Moratto D, et al. *J Allergy Clin Immunol*. 2019;7(5):1568-1577; 4. Beaussant Cohen S, Fenneteau O, Plouvier E, et al. *Orphanet J Rare Dis*. 2012;7:71; 5. Data on file. X4 Pharmaceuticals, Inc., 2024.

The underlying cause of WHIM syndrome, CXCR4 pathway dysregulation, impairs movement of leukocytes critical to a healthy immune response[1]



In WHIM syndrome, CXCR4 variants can result in decreased mobilization of white blood cells into peripheral circulation



Immunodeficiency in WHIM syndrome results in an increased susceptibility to severe, prolonged, and recurrent bacterial and viral infections[1]

References: 1. Dale DC, Firkin F, Bolyard AA, et al, *Blood*. 2020;136(26):2994-3003.

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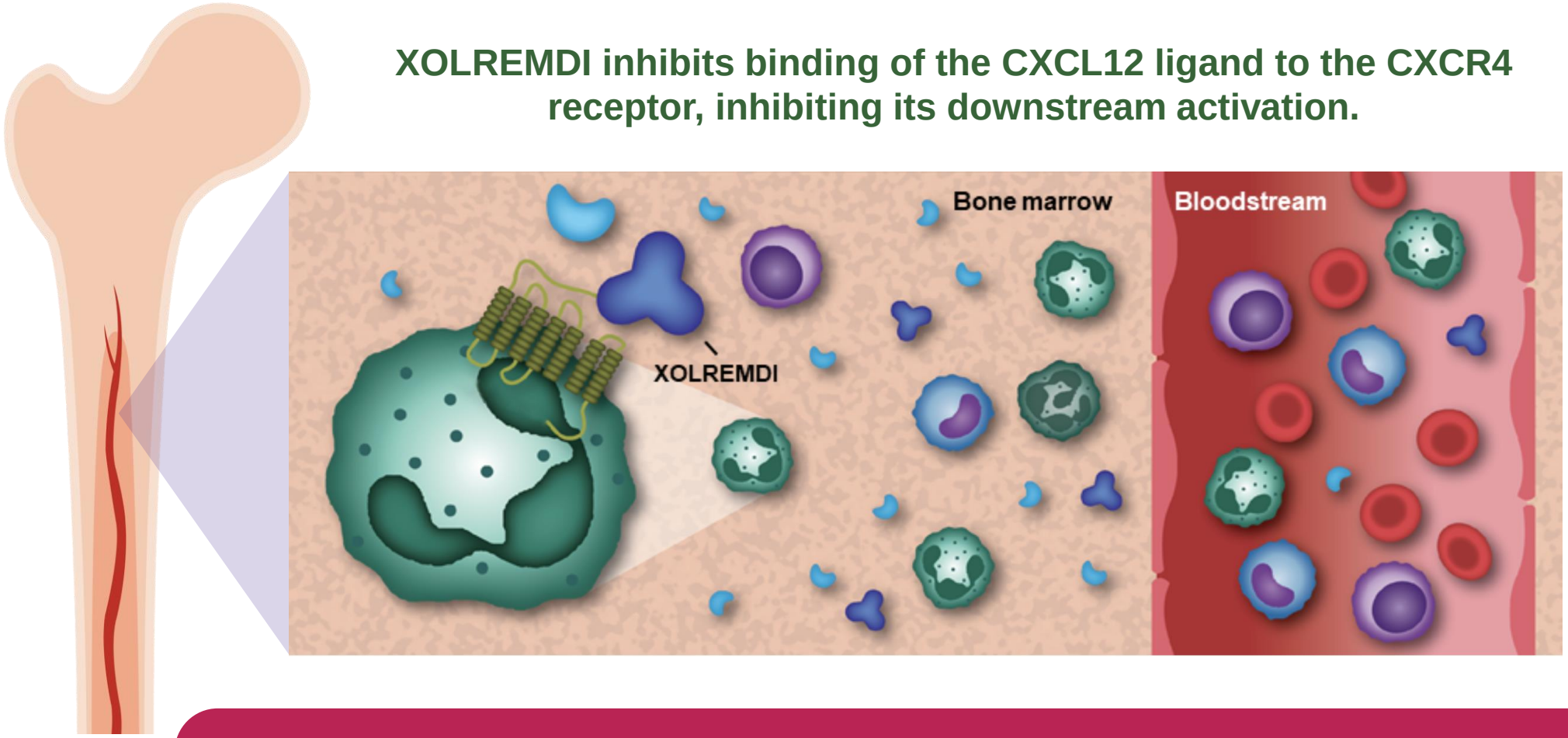
A selective, oral CXCR4 antagonist that targets the underlying cause of WHIM syndrome



XOLREMDI inhibits binding of the CXCL12 ligand to the CXCR4 receptor, inhibiting its downstream activation.

XOLREMDI increases mobilization of neutrophils and lymphocytes into the peripheral blood

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Label Highlights & Supporting Clinical Data

Christophe Arbet-Engels, MD, PhD
Chief Medical Officer

Until now, WHIM syndrome has been managed with treatments that do not address its underlying cause

Symptomatic Treatments

G-CSF

IVIg

Antibiotics & Antivirals

- Not specifically indicated for WHIM syndrome
- No adequate and well controlled trials evaluating safety and efficacy in patients with WHIM syndrome[1,2]
- G-CSF and IVIg associated with burdensome administration
- Long-term use of antibiotics associated with risk of developing antimicrobial resistance (AMR) and cumulative risk of adverse events[3]
  - 73% of surveyed HCPs (n=74) are concerned about antibiotic resistance in WHIM syndrome patients[4]

G-CSF: granulocyte colony-stimulating factor; **IVIg**: intravenous immunoglobulin.

References: 1. Dale DC, Firkin F, Bolyard AA, et al, *Blood*. 2020;136(26):2994-3003; 2. Geier CB, Ellison M, Cruz R, et al, *J Clin Immunol.* 2022;42(8):1748-1765; 3. Kiss C, Connoley D, Connelly K, et al, *Antibiotics*, 2022 Jan 11(1): 62; 4. X4 March 2024 Research; 74 HCPs (44 Immunologists and 30 HEM/ONCs).

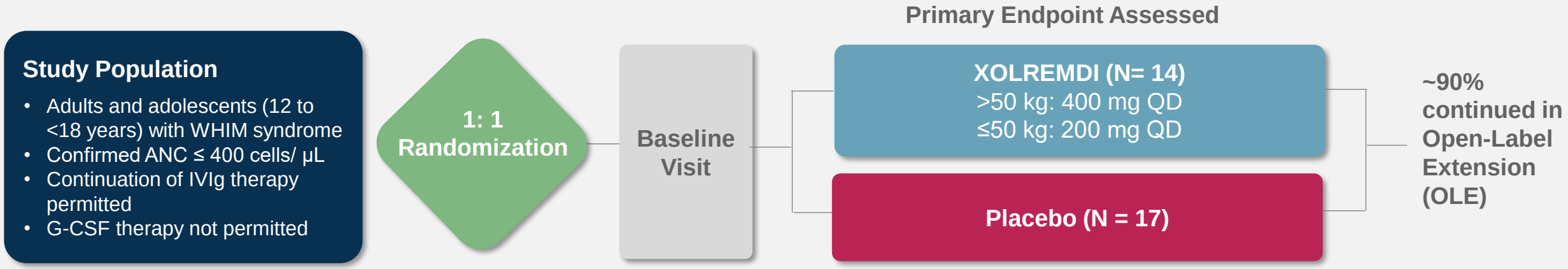
XOLREMDI™ (mavorixafor) U.S. Label highlights

Indication Statement	XOLREMDI is approved for use in patients 12 years of age and older with WHIM syndrome (warts, hypogammaglobulinemia, infections and myelokathexis) to increase the number of circulating mature neutrophils and lymphocytes.
Contraindication	Use with drugs highly dependent on CYP2D6 for clearance.
Boxed Warning	None
Dosing & Administration	Patients weighing >50 kg: 400 mg orally once daily Patients weighing ≤50 kg: 300 mg orally once daily
Dosage Forms and Strength	Capsules: 100 mg mavorixafor

Please see Important Safety Information and full Prescribing Information at www.xolremdi.com.

4WHIM was the largest Phase 3 clinical trial to date in WHIM syndrome

XOLREMDI was studied in a global, randomized, double-blind, placebo-controlled, Phase 3 trial conducted in 31 patients with WHIM syndrome



Primary endpoint

- Improvement in absolute neutrophil count (ANC) as measured by the mean time above ANC threshold of 500 cells/µL at 13, 26, 39, and 52 weeks

Secondary endpoints

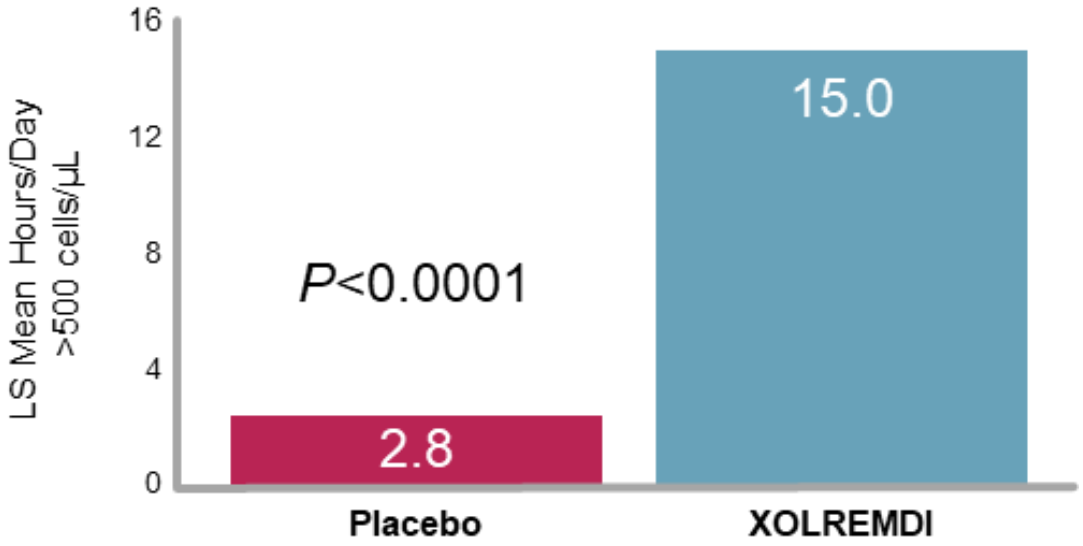
- Improvement in absolute lymphocyte count (ALC) as measured by the mean time above ALC threshold of 1000 cells/µL at 13, 26, 39, and 52 weeks
- Composite endpoint: Analysis of total infection score (rate, severity) and total wart change score

1. Badolato R, et al. *Blood*. Published online April 21, 2024;blood.2023022658.

4WHIM Results: XOLREMDI significantly increased the time patients stayed above key immune cell count thresholds over 52 weeks

Primary endpoint

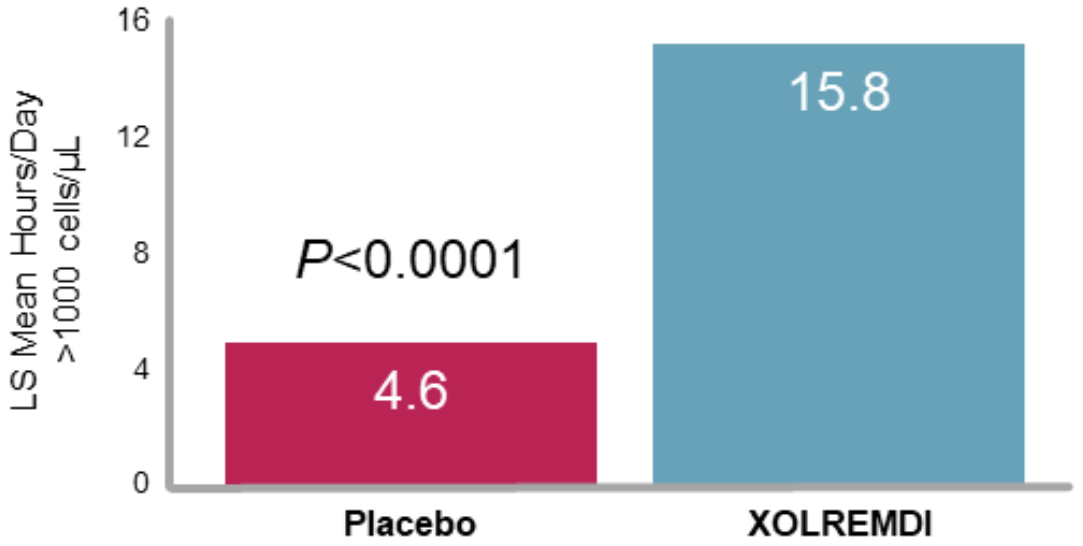
Significantly increased mean hours per day above the threshold for neutrophils



Severe neutropenia threshold = 500 cells/ µL

Key secondary endpoint

Significantly increased mean hours per day above the threshold for lymphocytes

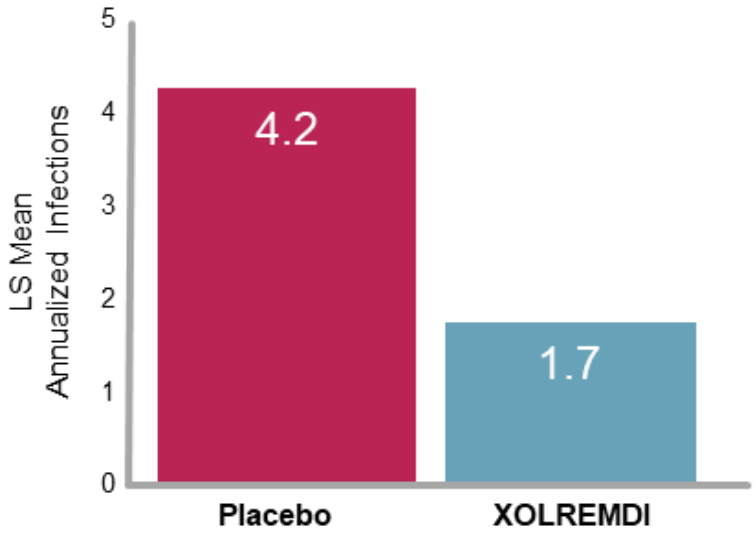


Severe lymphopenia threshold = 1000 cells/ µL

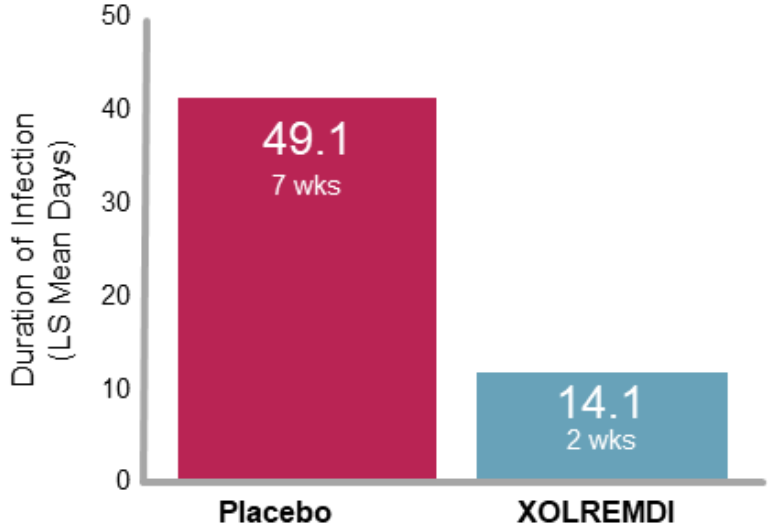
4WHIM Results: patients treated with XOLREMDI experienced improvements across infection assessments over 52 weeks versus placebo[1]

Total infection score 40% lower for patients on XOLREMDI versus those on placebo

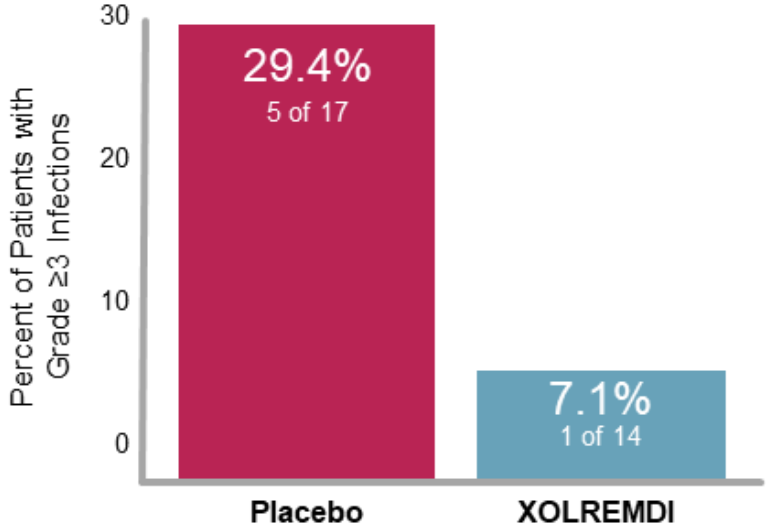
~60% reduction in annualized infection rate



On average, infection duration was 5 weeks shorter



Fewer patients with severe infection



No difference in wart change scores between XOLREMDI and placebo arms

1. Badolato R, et al. *Blood*. Published online April 21, 2024;blood.2023022658.

Treatment was generally well tolerated; majority of adverse reactions were mild to moderate in severity

Adverse Reactions Section of Product Label[1]
(≥10% and at a frequency higher than placebo in 4WHIM)

Adverse Reaction	XOLREMDI (n=14)	Placebo (n=17)
Thrombocytopenia	3^	0
Pityriasis	2	0
Rash	2	0
Rhinitis	2	0
Epistaxis	2	1
Vomiting	2	1
Dizziness	2	1

^Serious adverse reactions of thrombocytopenia occurred in 3 of the 14 patients who received XOLREMDI, two of which occurred in the setting of infection or febrile neutropenia.

Warnings and Precautions: Embryo-fetal toxicity and QTc interval prolongation.

Published Phase 3 trial data results[2] showed:

- XOLREMDI (mavorixafor) was generally well tolerated in participants with WHIM syndrome
- No discontinuations occurred due to treatment-emergent adverse events (TEAEs), and none were deemed related to treatment
- No treatment-related serious TEAEs were observed

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Commercial Launch Strategy & Execution

Mark Baldry, Chief Commercial Officer

Commercial Strategy Overview: targeted education, engagement, and access

Support Patient Diagnosis

- Educate on WHIM syndrome
- Provide diagnostic support
- Engage at key medical conferences

Establish XOLREMDI as Standard of Care in WHIM syndrome

- Target key hematologists & immunologists
- Communicate targeted MOA and clinical profile
- Drive adoption and uptake in appropriate patients

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Gain Broad Access

- Mitigate access barriers
- Provide full suite of patient support services
- Help patients throughout their treatment journey

X4Connect™

Leveraging an agile commercial team to execute X4's first product launch

The right field team to launch an innovative rare disease medicine

Field team recruited from well known rare and ultra-rare organizations

Collectively more than 250 years of demonstrated success in commercial launches

Team Expertise

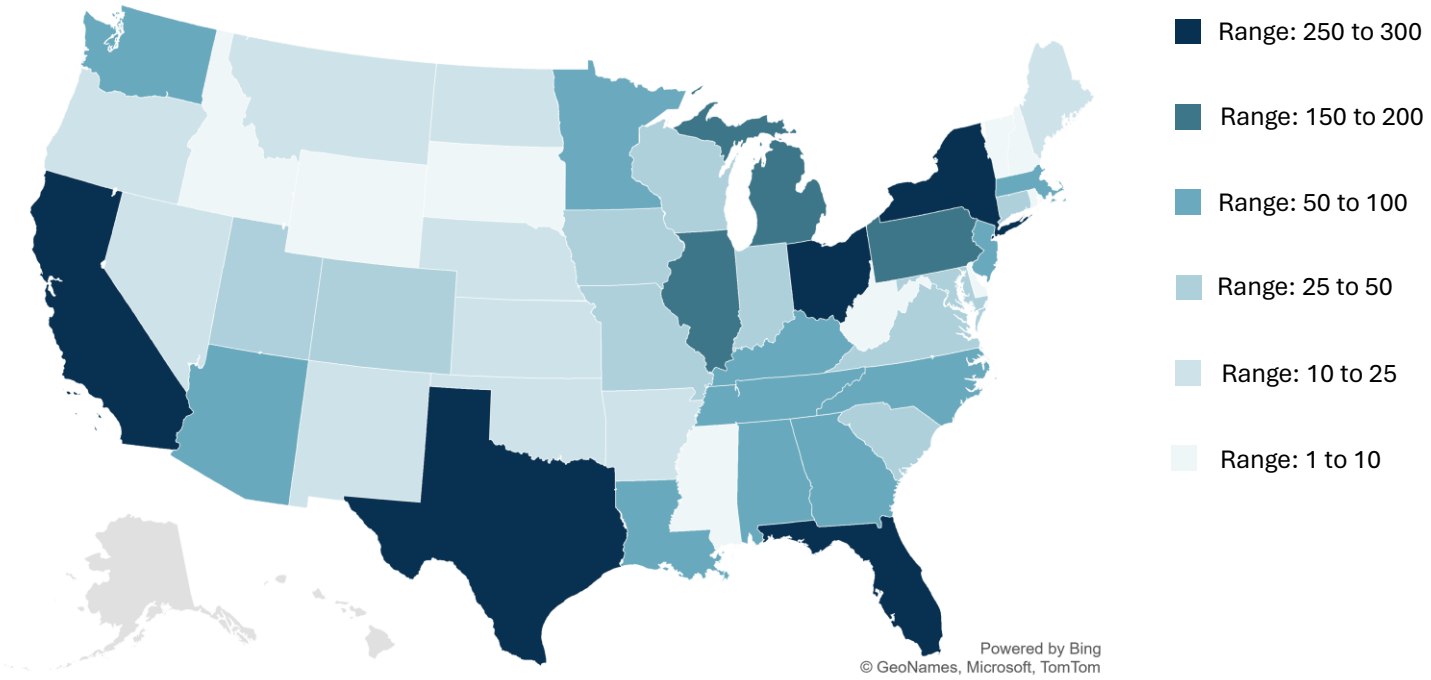
- Rare disease space / hematology & immunology
- Supporting diagnosis of rare diseases
- Understanding the patient journey
- Launching therapies into new markets
- Community relationships with physicians, thought leaders, and patient advocacy organizations

Mission-driven, patient-centric: bringing a novel therapy to a historically underserved population

Targeted approach to covering the U.S. WHIM market

Refined Target List of ~3,500 HCPs

(primarily immunologists and hematologists)



Focused KOL Engagement

~20 top thought leaders

Partnering with Patient Advocacy Networks

Jeffrey Modell Foundation (JMF)

Immune Deficiency Foundation (IDF)

HCPs are seeking a targeted therapy for WHIM Syndrome, a serious condition with high burden and unmet need

HCP Survey

92% of HCPs believe WHIM syndrome is a serious immune disorder with potential for long-term complications

89% believe there is a high unmet need for a targeted + effective therapy for WHIM syndrome

76% are dissatisfied with current options for treating the symptoms of WHIM syndrome

Shown an unbranded profile of XOLREMDI, interest in prescribing was high across HCPs in all key specialties, receiving an average 9 out of 10 rating

Drivers of Appeal/Interest

HCPs were particularly impressed by:

- Reduction in infections
- Targeted nature of the treatment
- Oral formulation

Sources: X4 Pharmaceuticals HCP Market Research and Testing from February 2024 (N=29 HCPs) and March 2024 (N=74 HCPs)

Patient Support

X4Connect™ helps eligible patients navigate insurance coverage, prior authorization requirements, and financial assistance programs to help support access

X4Connect Care Coordinators • Investigate coverage and help navigate prior authorizations, appeals, and financial assistance options

X4 Nurse Educators • Provide education and available resources to patients and their caregivers about XOLREMDI and WHIM syndrome

X4Connect Specialty Pharmacists • Counsel patients on their XOLREMDI prescription

Financial Assistance Programs • Financial assistance offerings include Quick Start, Copay Assistance, Bridge Program, and Patient-Assistance Program for eligible patients

Specialty Pharmacy

Processes and fills XOLREMDI prescriptions with clinical pharmacists available to:

- Answer questions about treatment
- Provide information about potential side effects
- Confirm treatment dosing and any adjustments

PANTHERx RARE

XOLREMDI addressing high unmet need with targeted innovation

XOLREMDI™ (mavorixafor) capsules

- **Targeted Breakthrough therapy for ultra-rare patient population**
- **First and only FDA-approved therapy indicated for WHIM syndrome**
- **Demonstrated efficacy & safety profile**
- **Potential to address high burden of disease and strengthen relevant aspects of patients' immune function**

Annual Price* Reflects Value

- Patients >50 kg = 400 mg daily = $496,400 annually
- Patients ≤50 kg = 300 mg daily = $372,300 annually

Committed to Providing Innovative Solutions

Dedicated support and education available through X4Connect and PANTHERx Rare for all eligible patients

Helping unite the WHIM syndrome community through collaborations, targeted education, and support of earlier diagnosis

XOLREMDI targets CXCR4 dysregulation, the underlying cause of WHIM syndrome

* Wholesale acquisition cost (WAC); assumes full compliance

Delivering on the promise of XOLREMDI (mavorixafor)

Supporting Patient Diagnosis

Establishing XOLREMDI as Standard of Care in WHIM syndrome

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Gaining Broad Access

X4Connect™

Conclusion

Paula Ragan, PhD

President & Chief Executive Officer

X4: continuing to deliver progress for patients

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U.S. Approval & Launch of XOLREMDI for WHIM syndrome April 2024

Laying a strong foundation for XOLREMDI sales & market growth

Additional Phase 2 CN trial data expected in 1H 2024

Initiation of pivotal Phase 3 CN trial anticipated in 1H 2024

Potential pipeline expansion opportunities

Priority Review Voucher received

PROGRESS 4 PATIENTS

Courtney, living with
WHIM syndrome

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