X4 PHARMACEUTICALS

July 2024

PROGRESS 4 PATIENTS

Enabling a better future for people with rare immune disorders

Forward-Looking Statements

This presentation including any printed or electronic copy of these slides, the talks given by the presenters, the information communicated during any delivery of the presentation and any question and answer session and any documents or materials distributed at or in connection with the presentation, contains forward-looking statements within the meaning of applicable securities laws, including 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, business, plans, or intentions. Forward-looking statements include, without limitation, implied or express statements regarding X4’s expectations as to plans for commercial launch of XOLREMDI (mavorixafor), which is approved in the U.S. for use in patients 12 years of age and older with WHIM syndrome (the ”Indication”), including the success of its commercial launch in the U.S. through PANTHERx Rare; X4’s belief in its readiness for commercial launch of XOLREMDI; the potential benefit of XOLREMDI in the Indication; the potential number of patients in the United States with WHIM syndrome and the potential market for XOLREMDI; the initiation, timing, progress, and results of our current and future preclinical studies and clinical trials and related preparatory work and the period during which the results of the trials will become available, as well as our research and development programs; and the mission and goals for our business.

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X4's Momentum Addressing Unmet Needs in Rare Immune Disorders

Fully integrated company delivering on the promise of mavorixafor

PROVEN SUCCESS IN RARE DISEASE DRUG DEVELOPMENT & COMMERCIALIZATION

XOLREMDI™ (mavorixafor) approved by FDA in April 2024 - first therapy indicated for patients with WHIM syndrome[1]

- **Patients on commercial product** with U.S. launch ongoing and field team fully deployed
- Clinical safety and efficacy data published online in ASH Journal *Blood*
- EU MAA submission expected late 2024/early 2025

STRONG BALANCE SHEET SUPPORTS CONTINUED GROWTH

- Pro forma funds of $207 million[2]
- Balance sheet expected to fund operations into late 2025[3]

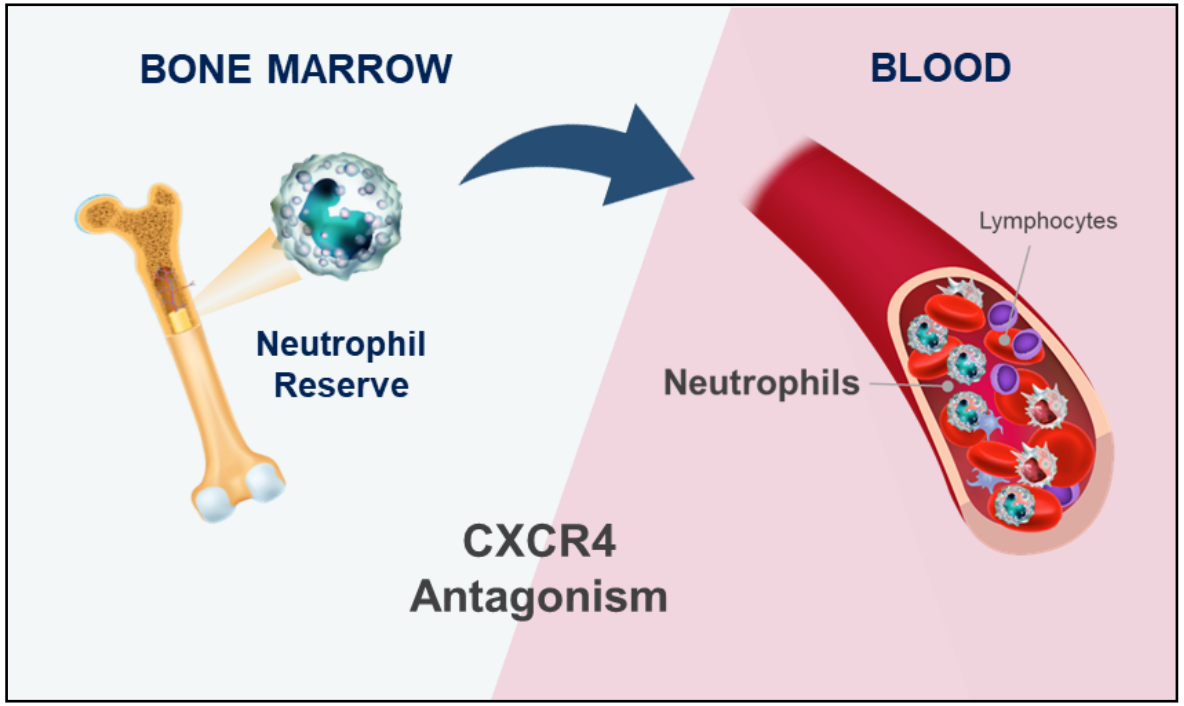
NEXT VALUE DRIVER: MAVORIXAFOR IN CHRONIC NEUTROPENIA

- Positive interim data from ongoing Phase 2 trial in CN presented in June 2024
- **Global, pivotal Phase 3 clinical trial in CN now initiated**

1. WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis); 2. Pro forma funds include $82 million in cash and equivalents as of March 31, 2024 + $105 million in proceeds from PRV sale (May 2024) + $20 million from debt draw down from loan facility with Hercules Capital, Inc. (May 2024); 3. Projected runway excludes any potential U.S. sales of XOLREMDI.

Mavorixafor: Pipeline in a Product via CXCR4 Antagonism

Validated mechanism shown to alleviate neutropenia and lymphopenia



Modified figure from reference 1

Targeted Mechanism

- CXCR4 regulates movement of white blood cells throughout the body[2]
- **CXCR4 antagonism** has been shown to increase the migration of cells from the bone marrow, increasing circulating levels of neutrophils and lymphocytes[3,4]

Orally active CXCR4 Antagonist

- Mavorixafor has been shown to raise circulating blood levels of neutrophils and lymphocytes[4,5,6]
- Clinical potential across multiple rare immunodeficiencies
- U.S. patent protection expected through 2038

1. Bainton DF (1980) *The Cell Biology of Inflammation*, vol 2, pp 1–25. Amsterdam: Elsevier/North-Holland 2. Furze RC, et al, *Immunology*. 2008. 3. Mosi, RM, et al, *Biochem Pharmacol*, 2012. 4. Stone ND et al, *Antimicrob Agents Chemother*. 2007; 5. Badolato R, et al. *Blood*. Published online April 21, 2024;blood.2023022658; 6. Warren, JT et al, Oral Presentation at the 2022 annual meeting of the American Society of Hematology.

Maximizing the Potential of Mavorixafor for Patients

Only oral agent targeting rare immunodeficiencies

	Indication	Pre-clinical	Phase 1	Phase 2	Phase 3	FDA Approved	EXPECTED MILESTONES
XOLREMDI™ (mavorixafor) capsules	**WHIM Syndrome** (Warts, Hypogammaglobulinemia, Infections, Myelokathexis)	Approved in U.S. April 2024					Progress on U.S. launch **EU MAA submission late 2024 / early 2025**
Mavorixafor	**Chronic Neutropenia** (Congenital, Autoimmune, Idiopathic)	Phase 3 Initiated June 2024					**Full Phase 2 data by late 2024**
X4P-003	TBD						

WHIM Syndrome: a Combined Primary Immunodeficiency and CN Disorder[1]

Heterogeneous presentation of symptoms caused by CXCR4 dysfunction[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.

Until Now, WHIM Syndrome Managed with Treatments Not Addressing Underlying Cause of Disease

Symptomatic Treatments

G-CSF

IVIg

Antibiotics & Antivirals

- Not specifically indicated for WHIM syndrome
- No adequate or 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).

Now Available in the U.S.!

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.

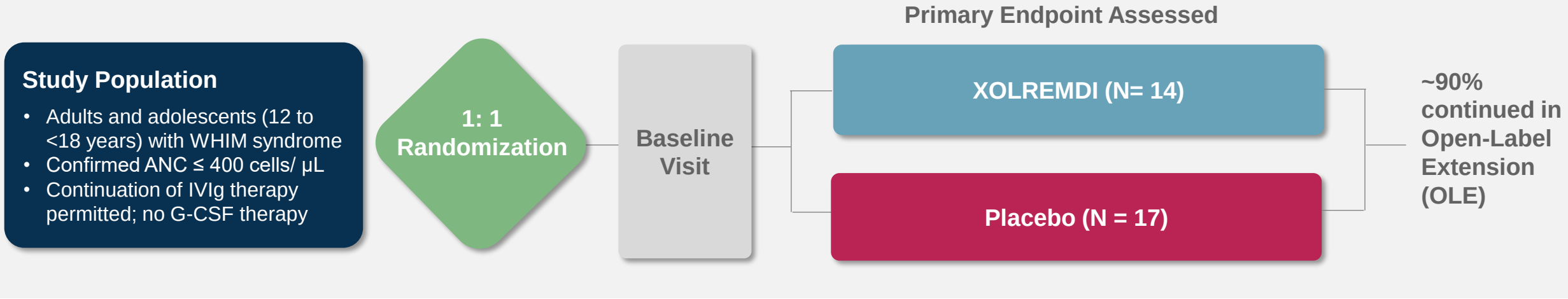
See full prescribing information at xolremdi.com

XOLREMDI™
(mavorixafor) capsules

(zōl-RĚM-dee)

4WHIM: 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

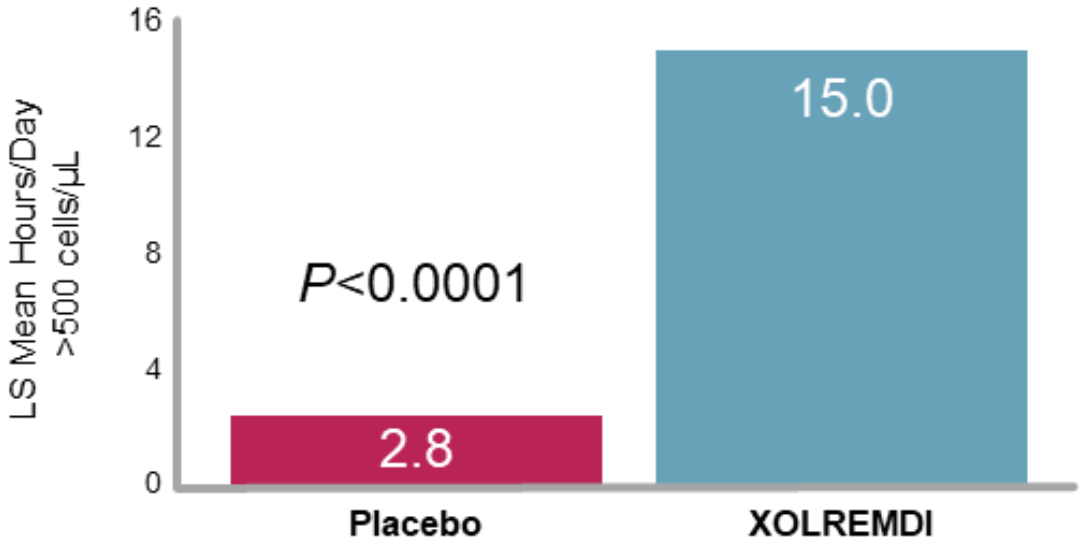
- Improvement in absolute lymphocyte count (ALC) as measured by the mean time above ALC threshold of 1000 cells/µL at 13, 26, 39, and 52 weeks
- Composite endpoint: Analysis of total infection score (rate, severity) and total wart change score

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

4WHIM: XOLREMDI Significantly Increased Time Patients Stayed Above Key Immune Cell Count Thresholds over 52 Weeks versus Placebo

Primary endpoint

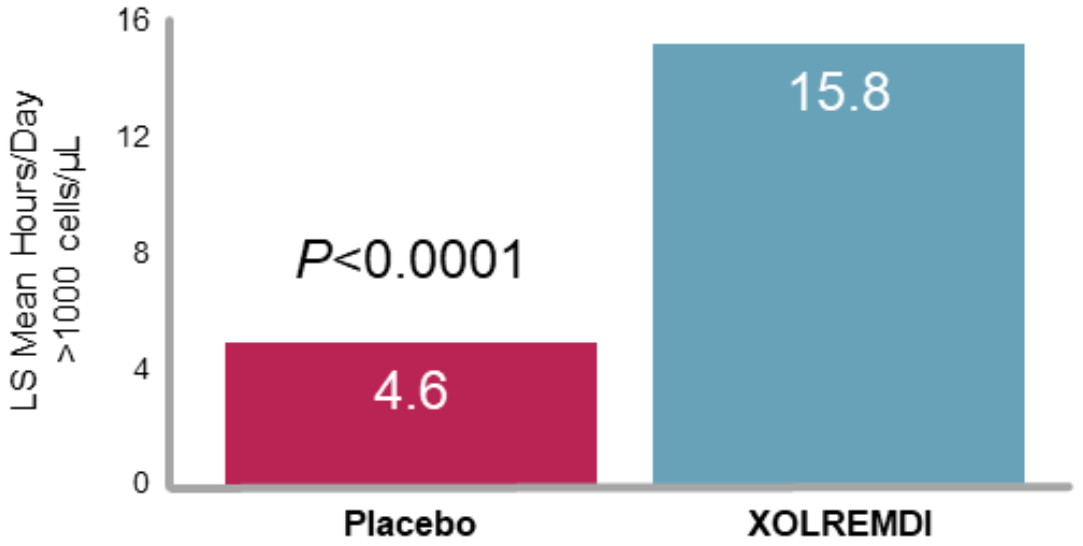
Significantly increased mean hours per day above the threshold for neutrophils



Severe neutropenia threshold = 500 cells/µL

Key secondary endpoint

Significantly increased mean hours per day above the threshold for lymphocytes



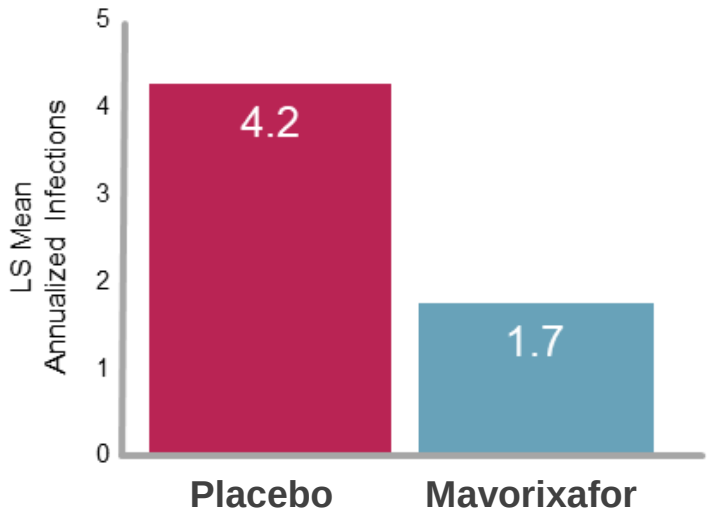
Severe lymphopenia threshold = 1000 cells/µL

4WHIM: ANC Increase Resulted in Clinical Infection Benefits[1,2]

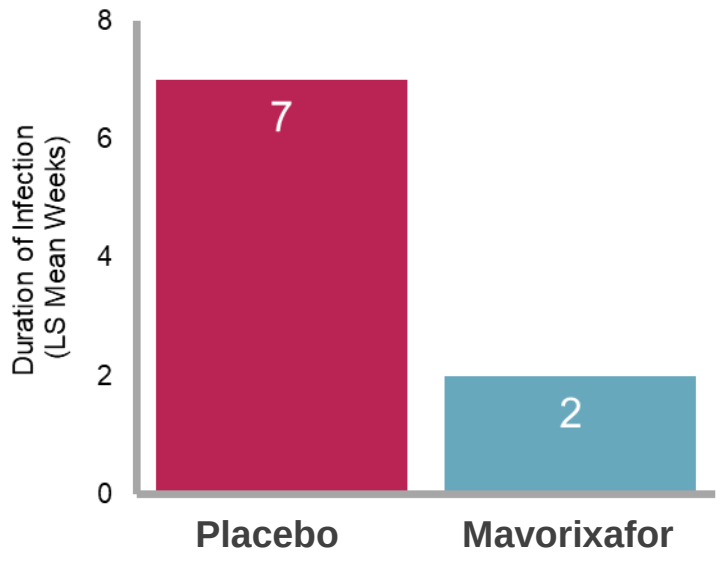
Mean ANC increases of >500 cells/µL reduced infection rate, duration, and severity

Total infection score[3] 40% lower for those on XOLREMDI versus placebo

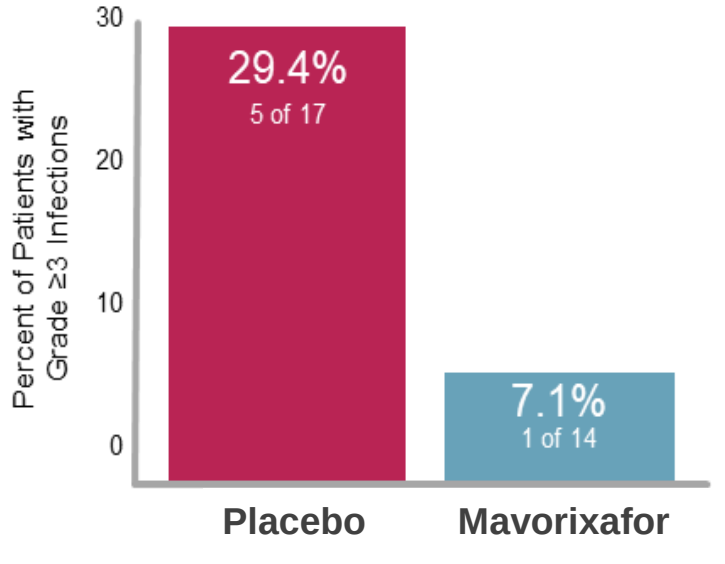
~60% reduction in annualized infection rate



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. 2. Badolato R. et al. Oral Presentation at Annual Meeting of the Clinical Immunology Society, May 2023. 3.Total infection score calculated by summing the number of infection events weighted by severity and divided by the total exposure time (in years).

4WHIM: Treatment Generally Well Tolerated; Majority of Adverse Reactions 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

Commercial Strategy: Targeted Education, Engagement, and Access

Supporting Patient Diagnosis

- Educating on WHIM syndrome
- Providing diagnostic support
- Engaging at key medical conferences

Establishing XOLREMDI as Standard of Care in WHIM syndrome

- Targeting key hematologists & immunologists
- Communicating targeted MOA and clinical profile
- Driving adoption and uptake

Gaining Broad Access

- Mitigating access barriers
- Providing full suite of patient support services (Co-pay Assistance, Quick Start Program, Bridge Program, Patient Assistance)
- Helping patients throughout their treatment journey

X4Connect™

Addressing High Unmet Need with Targeted Innovation

XOLREMDI™ (mavorixafor) capsules

- **First and only FDA-approved therapy indicated for WHIM syndrome**
- **Targets the underlying cause of WHIM syndrome via CXCR4 antagonism**
- **Demonstrated efficacy & safety profile with oral formulation**
- **Potential to address high burden of disease and strengthen relevant aspects of patients' immune function**

Targeted Approach to Covering the U.S. WHIM Market

- Field team recruited from well known rare and ultra-rare organizations
- Collectively more than 250 years of demonstrated success in commercial launches
- Mission-driven, patient-centric: bringing a novel therapy to a historically underserved population

Committed to Providing Innovative Solutions

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

Annual Price* Reflects Value

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

* Wholesale acquisition cost (WAC); assumes full compliance

Chronic Neutropenia: No Innovation in More Than 30 Years

~50,000[1]

U.S. Prevalence: total diagnosed with Chronic Neutropenia (CN)

~15,000[1]

Estimated subset with highest unmet need: minimum addressable market for mavorixafor in CN

Only One

Therapy Approved for Severe Chronic Neutropenia

Injectable Granulocyte Colony-Stimulating Factor (G-CSF)

- Approved to treat severe chronic neutropenia in 1995[2]
- Used as a chronic daily injection or as rescue during serious infection episodes
- Frequent treatment-related / treatment-limiting bone pain and other adverse events

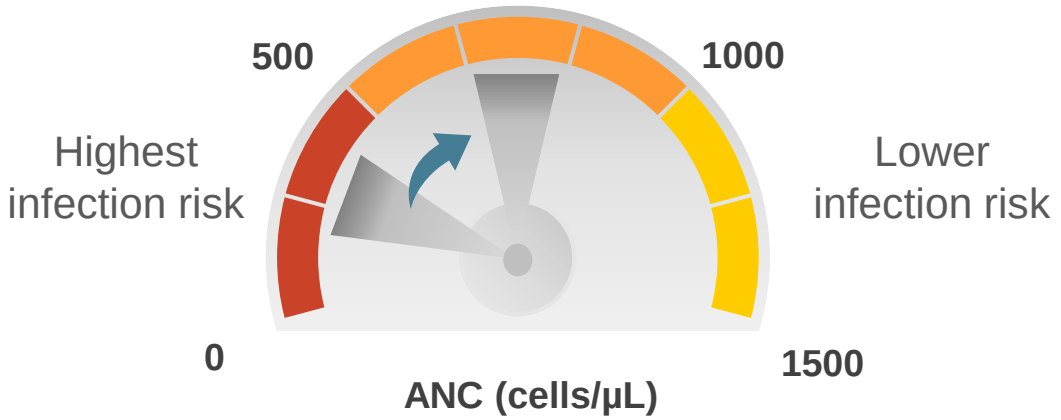
Innovation needed to address unmet patient needs

1. X4 Market Research, July 2023 – data on file; ICD-10 Code Research (2017-2023). 2. https://www.cancernetwork.com/view/fda-approves-new-indication-neupogen-chronic-neutropenia

Risk of Serious, Recurrent Infections Correlated to Severity of CN[1]

NIH Classification[2]	Absolute Neutrophil Count (ANC)
Severe (Grade 4)	<500 cells/µL
Moderate (Grade 3)	500 - 1,000 cells/µL
Mild (Grade 2)	1,000 - 1,500 cells/µL
Non-clinical (Grade 1)	1,500 = Lower Limit of Normal (LLN)

Increasing Neutrophil Counts >500 cells/µL Clinically Meaningful[6]



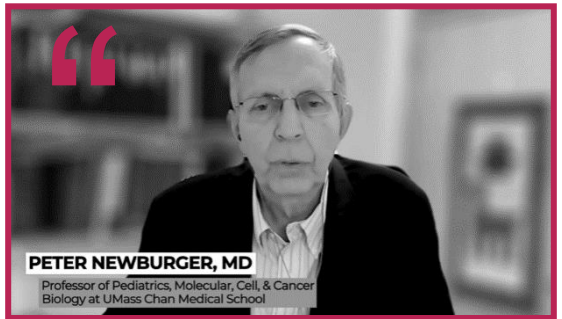
- Frequent and/or severe infections are the primary clinical consequence of chronic neutropenic disorders[3]
- Infections may lead to frequent hospitalizations or result in life-threatening complications, including death[4,5]

1. https://ctep.cancer.gov/protocoldevelopment/electronic_applications/docs/ctcae_v5_quick_reference_8.5x11.pdf ; 2. Jan Palmblad, Carlo Dufour, Helen A. Papadaki, *Haematologica*, Vol. 99 No. 7 (2014): July, 2014; 3. Sicre de Fontbrune F, et al. *Blood*. 2015;126(14):1643-1650; 4.Bodey, GP, *et al*, *Ann Intern Med*, 1966. 5. Donadieu J, et al. *Expert Rev Hematol*. 2021;14(10):945-960; 6. Salehi T, et al. Iran *J Allergy Asthma Immunol*. 2012;11(1):51-56.

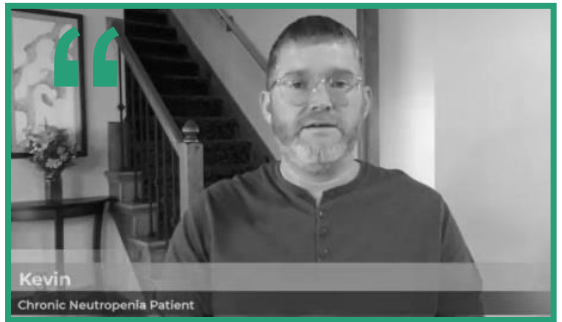
What Makes a Difference to Chronic Neutropenia Patients and Their Physicians?

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

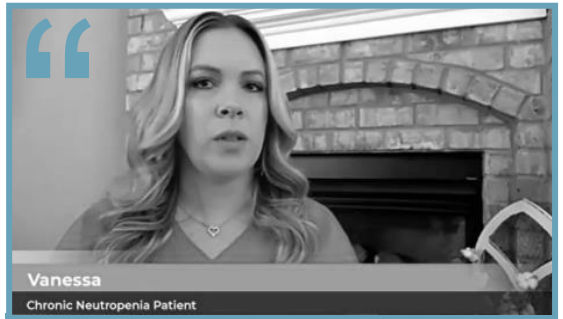
"We have to find something more compatible with normal quality of life."



"…maintaining an ANC above the 500 to 800 level correlates with a very significant decrease in severe infections."



"What I'd like to see with neutropenia would be a different way to administer the medicine… nobody likes needles."



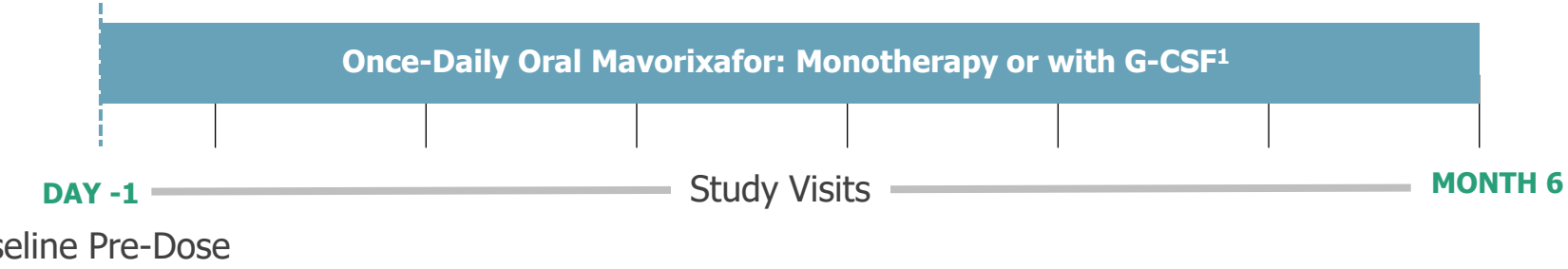
".. I'm using Neupogen… I use it daily on a low dose… If I get the extreme bone pain…, I am unable to sleep. It's unreal."

Assessing Mavorixafor in 6-Month CN Phase 2 Clinical Trial

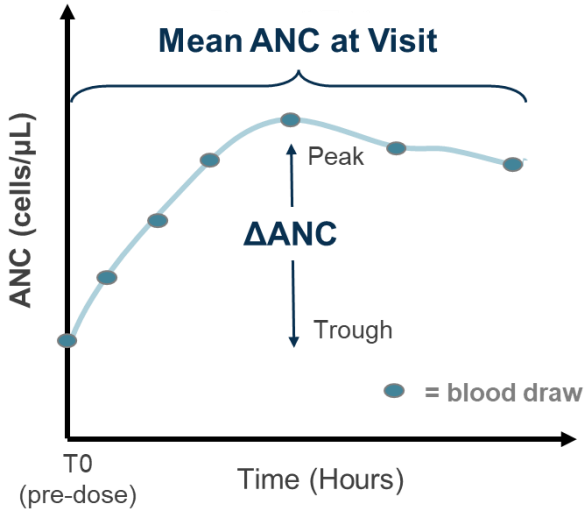
Mavorixafor dosed orally once-daily with or without background injectable G-CSF

Mavorixafor: Same Oral Dosing as 4WHIM Phase 3

Phase 2 Trial: Safety, Durability of ANC Levels over 6-Month Period



Timepoint Efficacy Assessments – Per Participant



Assessments at Baseline, Month 1, Month 3, and Month 6

- **At Each Visit:** up to 7 blood samples drawn over 8 hours
- **Mean ANC at Visit:** mean of absolute neutrophil counts from blood draws over the 8-hour period
- **ΔANC:** ANC at Peak minus ANC at Trough (T0)[2]

1. Modifications to G-CSF dosing allowed after Month 2 at doctor's discretion; additional data expected in late 2024. 2. For one participant, trough = first available sample

Interim Phase 2 Results Support Advancing to Pivotal Phase 3 CN Clinical Trial

✓ Mavorixafor durably increased ANC by >500 cells/μL as a monotherapy

- Raised participants' mean ANC above the lower limit of normal at Months 3 and 6
- Lowered potential infection risk by improving grade of neutropenia

✓ Mavorixafor monotherapy durably increased ANC in severe CN participants (baseline ANC<500 cells/μL)

- Achieved target ANC of ~800-1,000 cells/μL in this 'tougher-to-treat' population

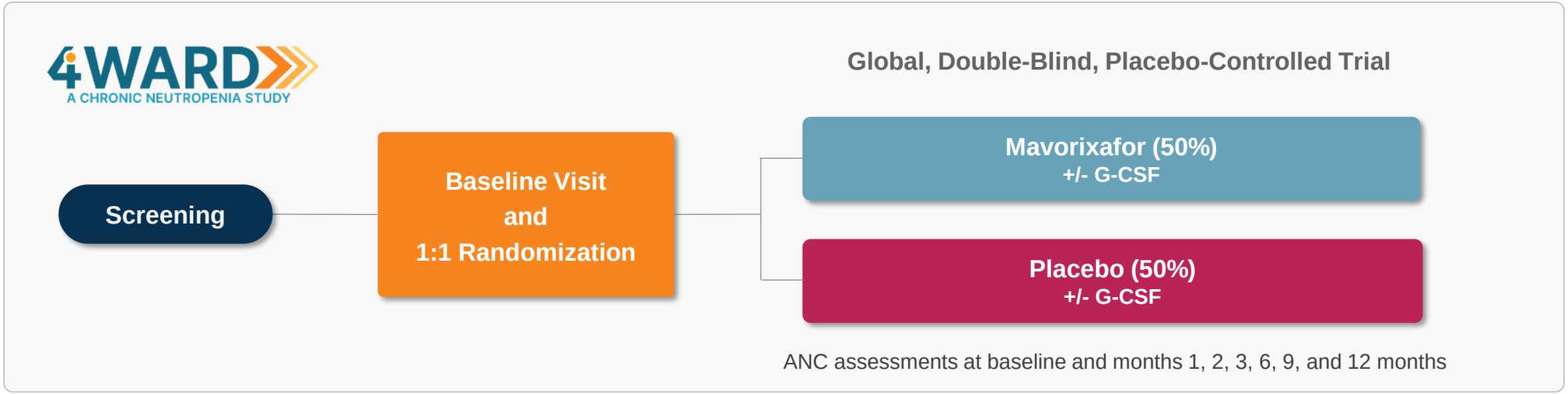
✓ Mavorixafor durably increased ANC by >1000 cells/μL in combination with stable-dose G-CSF

- Supports potential for mavorixafor use to reduce G-CSF therapy

✓ Mavorixafor well tolerated +/- stable-dose G-CSF

- Safety profile consistent with prior studies of mavorixafor; supports chronic dosing +/- G-CSF

CN Pivotal, Global Phase 3 Trial Initiated



Key Inclusion Criteria for 150 participants with congenital, autoimmune, or idiopathic chronic neutropenia

- **Absolute Neutrophil Count (ANC):** <1500 cells/µL
- **Infection History:** 2 or more infections requiring intervention within last 12 months

Primary Endpoint: Two-component endpoint: positive ANC response and annualized infection rate

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

Note: For those treated with G-CSF (or other therapies) at baseline, dose and frequency are required to remain constant throughout the trial unless adjustment is needed for safety reasons.

Clinical Results to Date Support 4WARD Phase 3 CN Trial Primary Endpoint

Two-component Primary Phase 3 endpoint

Positive ANC Response

From Phase 2 CN trial interim analysis:

- 10 evaluated Phase 2 participants met Phase 3 inclusion criteria of baseline ANC<1500 cells/µL
  - **80% (8/10) demonstrated ANC increases meeting Phase 3 response criteria** for at least 1 visit[1]

Annualized Infection Rate

From Phase 3 4WHIM Phase 3 trial:

- **ANC elevation >500 cells/ µL** resulted in:
  - ~60% reduction in annualized rate of infection
  - 5 weeks shorter duration of infection
  - Fewer patients with severe infections

1. Plan for durability analysis of ANC response is being finalized with U.S. FDA

Significant Opportunity to Address Unmet Needs in the CN Community

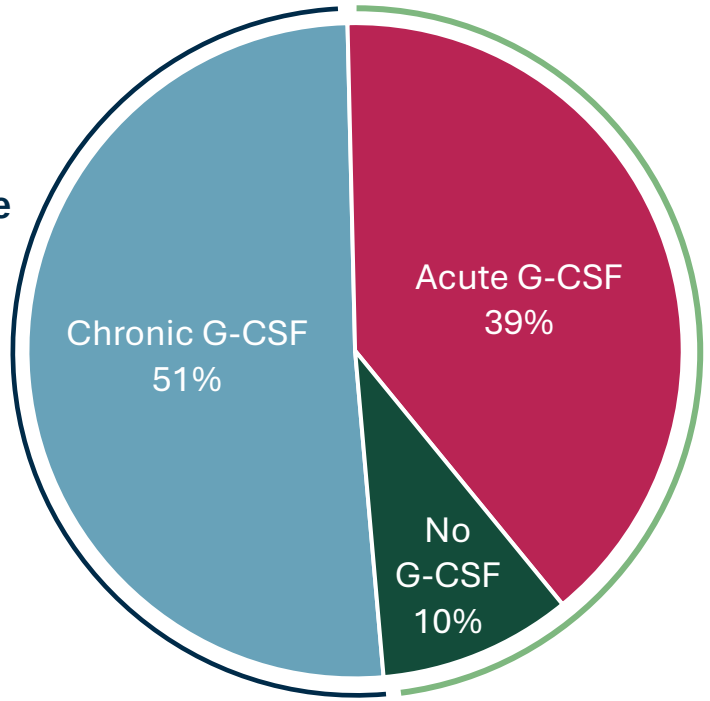
High unmet needs in ~15,000 patients in the U.S.

- Patients diagnosed with idiopathic, autoimmune, or congenital CN
- Adolescents and adults with history of severe/recurrent infections and/or previous/ongoing treatment with G-CSF

Current use of G-CSF within these patient populations

- ~51% of patients on chronic G-CSF therapy
- ~49% of patients not using G-CSF or on rescue use only

Current Use of G-CSF in ~15,000 U.S. CN Population with High Unmet Needs



Potential role of mavorixafor

X4 Market Research, July 2023 – data on file; ICD-19 Code Research (2017-2023).

Continuing to Deliver Progress for Patients

U.S. approval & launch for WHIM syndrome April 2024

Priority Review Voucher monetized

Positive interim data from Phase 2 study support and de-risk Phase 3 CN trial

Global, pivotal 4WARD Phase 3 CN trial initiated

4WARD
A CHRONIC NEUTROPENIA STUDY

Full Phase 2 chronic neutropenia data by late 2024

EU MAA submission in WHIM late 2024 / early 2025

Potential Market Opportunities

WHIM

>1,000 U.S. patients

Chronic Neutropenia

>15,000 U.S. patients

U.S. Headquarters

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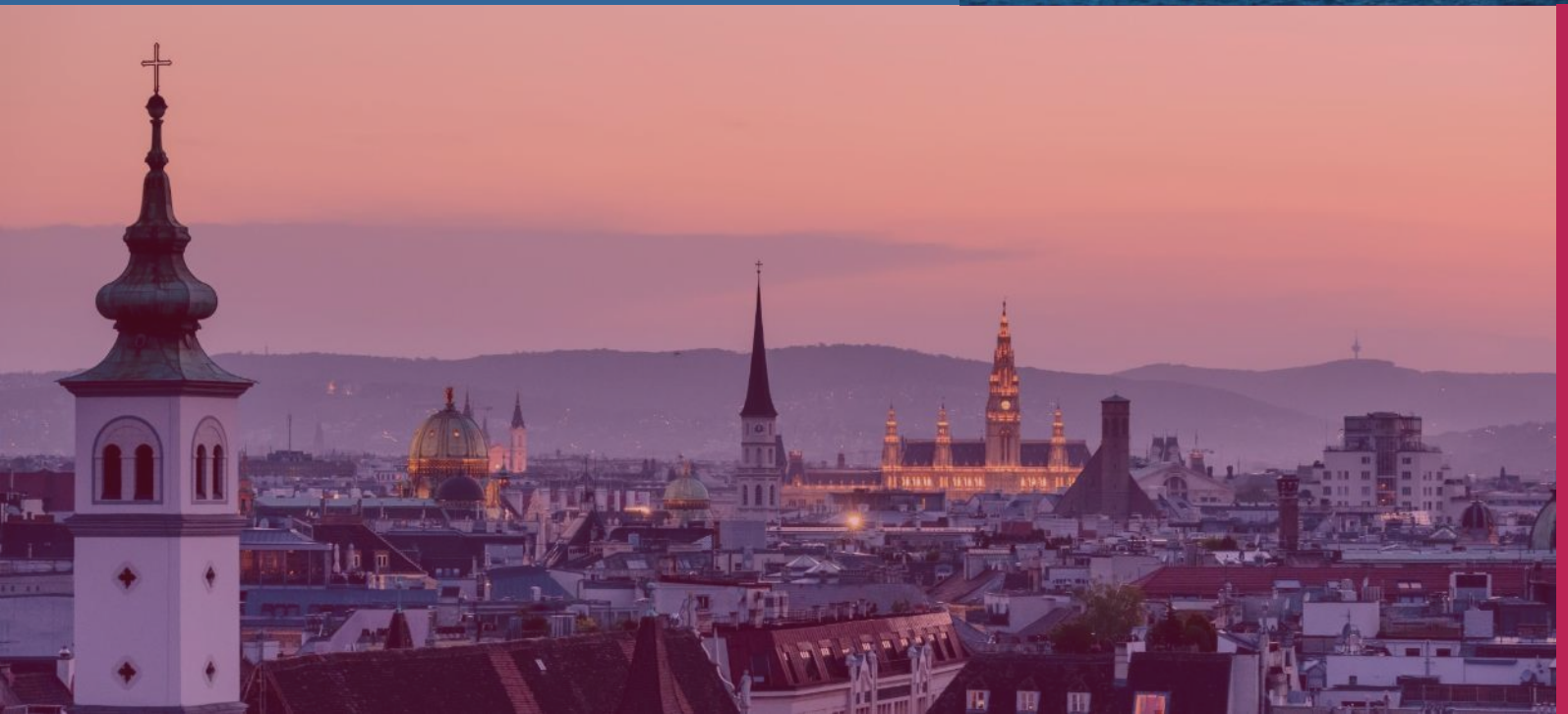
Boston, MA 02134

NASDAQ: XFOR



Research Center of Excellence

Helmut-Qualtinger-Gasse 2

A-1030 Vienna, Austria

www.x4pharma.com

Seasoned Executive Leadership Team

Experienced in research, development, & commercialization of first-in-class, innovative therapies

PAULA RAGAN, Ph.D.
President & CEO

SANOFI
genzyme

CHRISTOPHE ARBET-ENGELS, M.D., Ph.D.
Chief Medical Officer

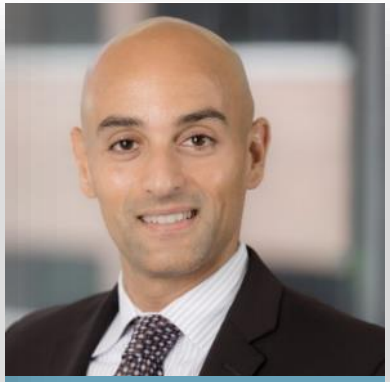
MILLENDO THERAPEUTICS
Biogen

MARK BALDRY
Chief Commercial Officer

Amicus Therapeutics
Biogen

MARY DIBIASE, Ph.D.
Chief Operating Officer

Biogen
EPIRUS BIOPHARMACEUTICALS



ADAM MOSTAFA
Chief Financial Officer

abpro
CANTOR Fitzgerald

ART TAVERAS, Ph.D.
Chief Scientific Officer

Schering-Plough
Biogen

Strong Balance Sheet Supports Expected Upcoming Milestones

~$207 million[1]

Funds expected to support operations into late 2025[2]

Top-tier Life Science-Focused Institutional Shareholder Base

Analyst Coverage

B RILEY FBR

BROOKLINE CAPITAL MARKETS

CANTOR Fitzgerald

PIPER | SANDLER

STIFEL

HCW H.C. WAINWRIGHT & CO.

1. Current funds include $82 million in cash and equivalents as of March 31, 2024 + $105 million in proceeds from PRV sale (May 2024) + $20 million from debt draw down from loan facility with Hercules Capital, Inc. (May 2024); 2. Projected runway excludes any potential U.S. sales of XOLREMDI.