X4 PHARMACEUTICALS

August 10, 2023

PROGRESS 4 PATIENTS

Developing the first oral treatments for chronic neutropenic disorders

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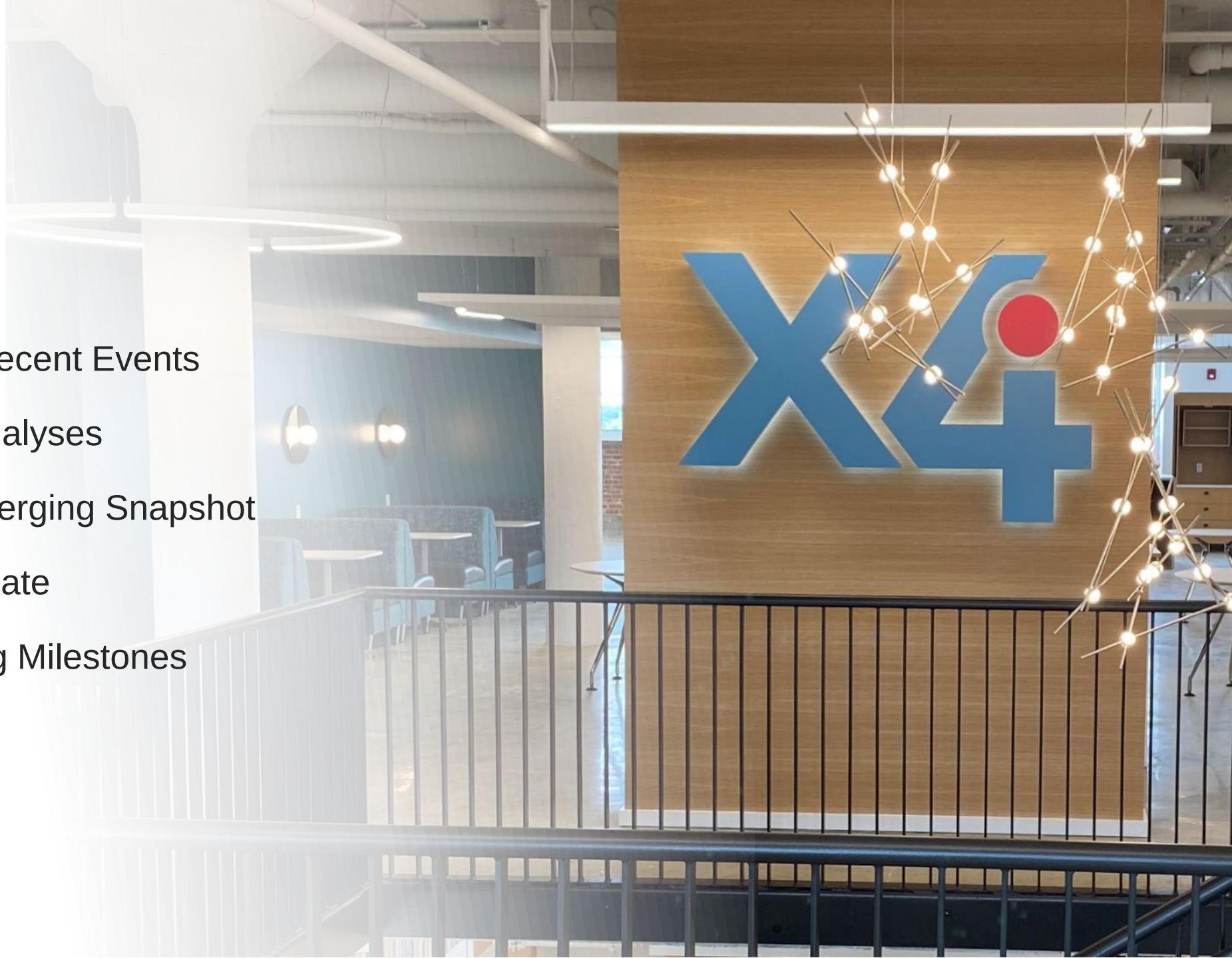
Today's Agenda

- 2Q 2023 Summary & Recent Events
- CN Market – Further Analyses
- CN Phase 2 Data – Emerging Snapshot
- CN Phase 3 Trial – Update
- Conclusion & Upcoming Milestones
- Q&A



2Q 2023 & Other Recent Events

Additional positive Phase 3 4WHIM results

- Phase 3 4WHIM Results:
  - Mavorixafor demonstrated good tolerability, increased white blood cells (including neutrophils), and reduced rate, severity, and duration of infections
  - Well attended X4 webinar in May & oral presentations at Annual Meetings of CIS (May) & EHA (June)
- Poised to submit mavorixafor U.S. NDA in WHIM syndrome
- Readying for potential launch in 1H 2024

New mavorixafor U.S. patent issued in June

- Broadens/strengthens patent portfolio protecting mavorixafor composition of matter through 2038

Christophe Arbet-Engels, MD, PhD to join as Chief Medical Officer

- Seasoned executive with significant experience in global drug discovery, translational research, clinical development, regulatory & medical affairs, and product launch and life-cycle management
- Experience spans broad range of therapeutic areas including rare and orphan diseases

2Q 2023 Financial Summary & Recent Highlights

Raised $65 million in gross proceeds	In May through a private placement (PIPE) priced at-the-market; participants included both new and existing life science investors
Russell 3000 Index	In late June, X4 was added to Russell 3000 Index
Completed $115 million loan facility	In early August with Hercules Capital; first tranche of $22.5 million drawn down at closing
Cash and equivalents at end of 2Q23 totaled $142.3 million	Including proceeds from loan facility, available funds of ~$160 million expected to fund operations into 2025

- Current runway projection does not include monetization of possible Priority Review Voucher received should mavorixafor gain U.S. approval for WHIM syndrome

Mavorixafor: Potential Breakthrough for Treating Chronic Neutropenic Disorders

Only oral candidate in development to treat CN disorders and WHIM syndrome

- ✓ Proven mechanism of action (MOA) / ability to increase circulating white blood cells, including neutrophils
- ✓ Demonstrated tolerability in >200 individuals, some for >4 years
- ✓ Successful Phase 3 trial in WHIM syndrome
- ✓ Successful Phase 1b clinical trial in chronic neutropenia (CN)
- Poised to submit U.S. NDA in WHIM syndrome
- Phase 2 CN clinical trial ongoing; Phase 3 expected to initiate in 1H 2024

Mavorixafor Targeting a Range of Chronic Neutropenic Disorders

~50,000 Estimated Chronic Neutropenia Patients in the U.S.[1]
(ANC <1,500 cells/uL for >3 months)

~40,000[1]

Chronic Idiopathic Neutropenia (CIN)

Most commonly diagnosed chronic neutropenia

Acquired neutropenia

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

~8,000[1]

Congenital Neutropenia

Rare hematological genetic diseases

Congenital

Diverse genetic etiologies

Younger, more severe population

Cyclic with *ELANE*

Typically, a 21-day cycle. Autosomal-dominant disorder. Can be caused by *ELANE* mutations

1. X4 Market Research, July 2023 – data on file; ICD-10 Code Research (2017-2023)

Initial Target Market for Mavorixafor in CN: Those With High Unmet Need

Significant Opportunity For Expansion

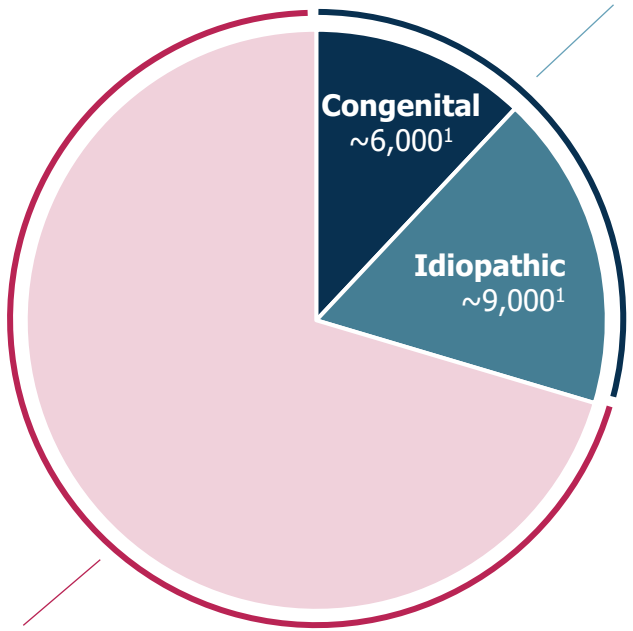
Methodology[1]

- Provides a real-world picture: U.S. CN landscape and needs
- Conducted interviews with and surveyed academic and community-based CN treaters (n=90)
- Reviewed detailed patient charts (n=300+)
- Triangulated using further claims (ICD-10 code) analysis using 6-year lookback

Defining Target Market & High Unmet Need

- **Excludes:** <12 years of age[2], CN disorders unlikely to respond to CXCR4 antagonism[2]
- **Includes:** only those with: history of severe/recurrent infections and/or history of G-CSF treatment
- **Finding:** ~90% of those with high unmet need (e.g., severe/recurrent infections) are also treated with G-CSF

Est. US Chronic Neutropenia Population (~50,000[1] Total)



~ 15,000 Initial Target Market with High Unmet Needs

adolescents and adults with history of severe / recurrent infections and/or G-CSF treatment

Significant Market Expansion Opportunities

Ages below 12 years old | Mild/Moderate Disease | QoL/G-CSF Intolerant

1. X4 Market Research, July 2023 – data on file; ICD-10 Code Research (2017-2023); 2. Consistent with planned CN Phase 3 trial.

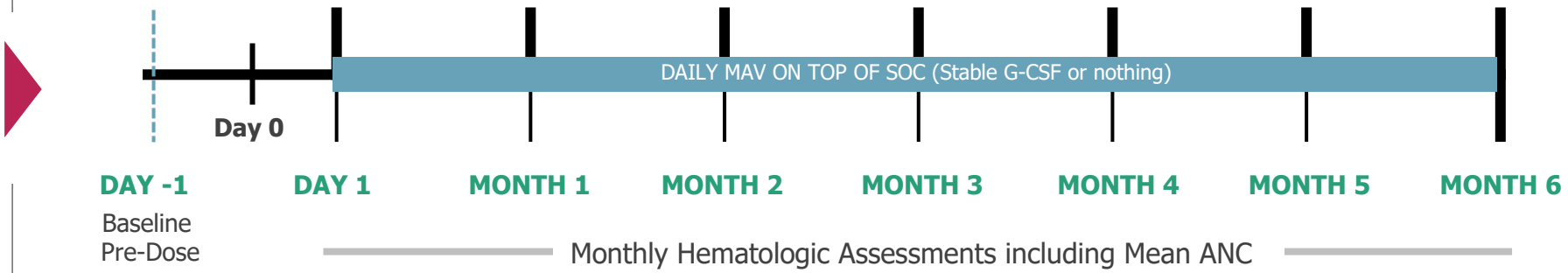
CN Phase 1b & Phase 2 Study: Review of Designs

Phase 1b Study:

Δ ANC Day 1 vs. Day -1 after single dose of mavorixafor +/- G-CSF

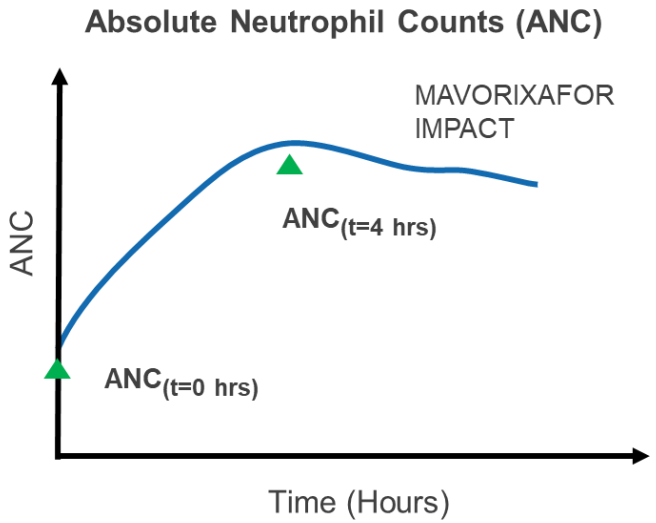
100% response; Published @ ASH 2022

Phase 2 Trial: Safety, Durability of ANC Levels, Exploring G-CSF Reduction



Mean ANC

Average of ANC at 0 and 4 hours



Preliminary Results[1]: Phase 2 Trial of Mavorixafor in Chronic Neutropenia

Emerging Data Show Durability & Successful G-CSF Reduction

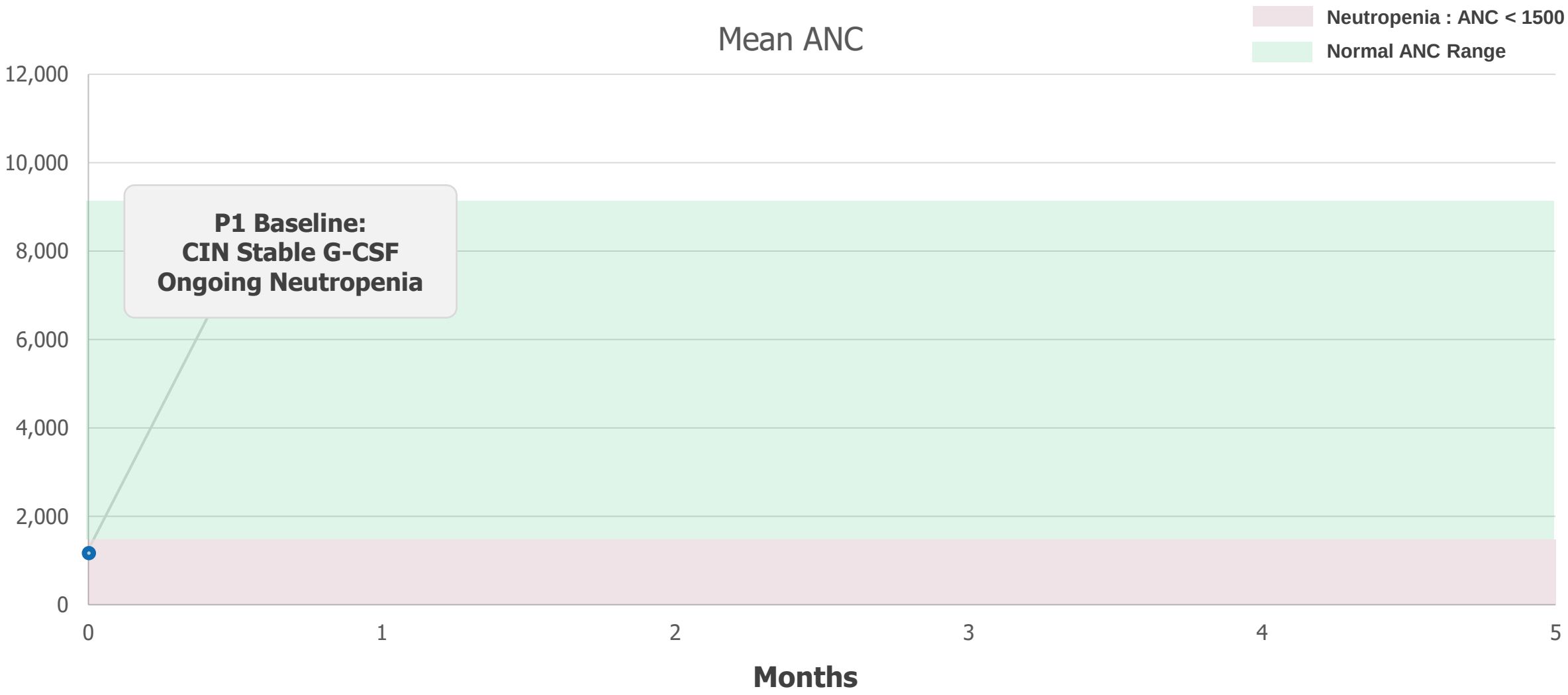
Participants		Baseline: Pre-Mav Dosing		After Mavorixafor Dosing		
	Diagnosis	G-CSF Use?	ANC Levels	Months Treated	ANC Levels	G-CSF Reduction
P1	CIN	Yes, chronic	Neutropenic with G-CSF	4	Increased response in ANC to normal ranges vs. BL	50% reduction at Month 2; off G-CSF after Month 4
P2	CIN	Yes, chronic	Normal with G-CSF	3	Increased response in ANC vs. BL	50% reduction at Month 2; off G-CSF after Month 3
P3	ELANE/Cyclic	Yes, chronic	Neutropenic with G-CSF	4	Increased response in ANC to normal ranges vs. BL	Recommended

Cohort of patients <u>on G-CSF at baseline</u>, dosed for ≥ 3 months has shown:

- Tolerability: Well tolerated in combination with G-CSF: ***no serious adverse events (SAEs), chronic dosing supported***
- Durability: Large, sustained increases in ANC: ***into normal ranges***
- G-CSF: Reduction of G-CSF treatment: ***physicians elected to reduce G-CSF dose vs. mavorixafor***

1. Data cut as of July 14, 2023

Example (P1): Daily Mavorixafor Leads to Successful Reduction of G-CSF



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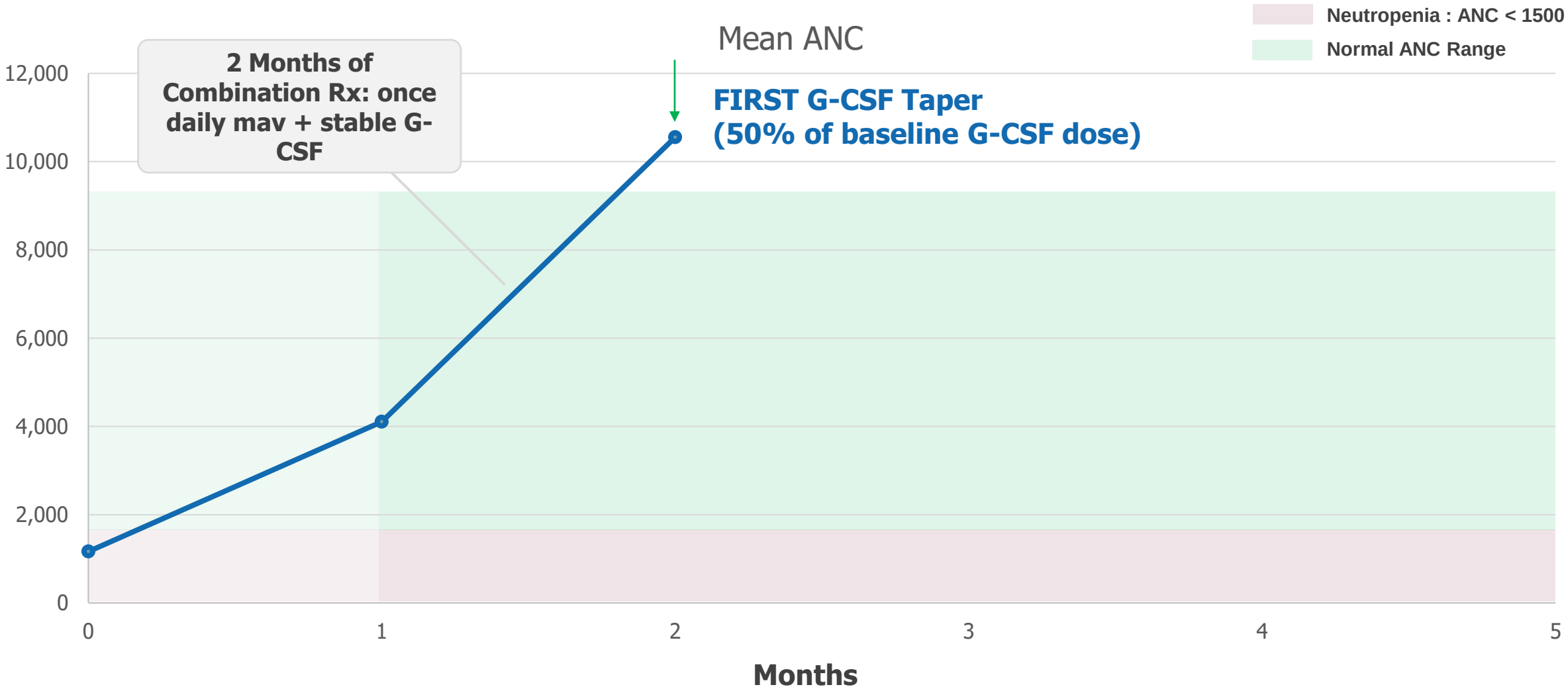

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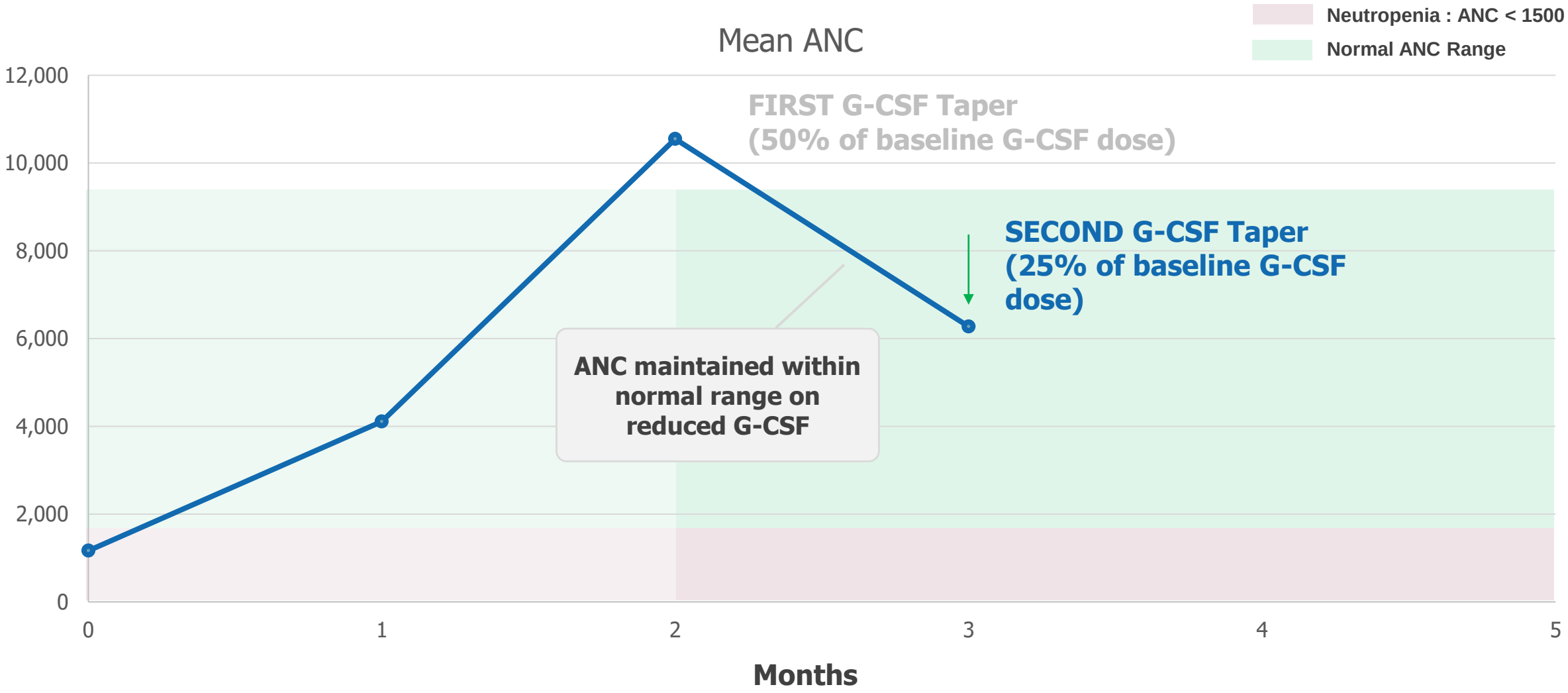

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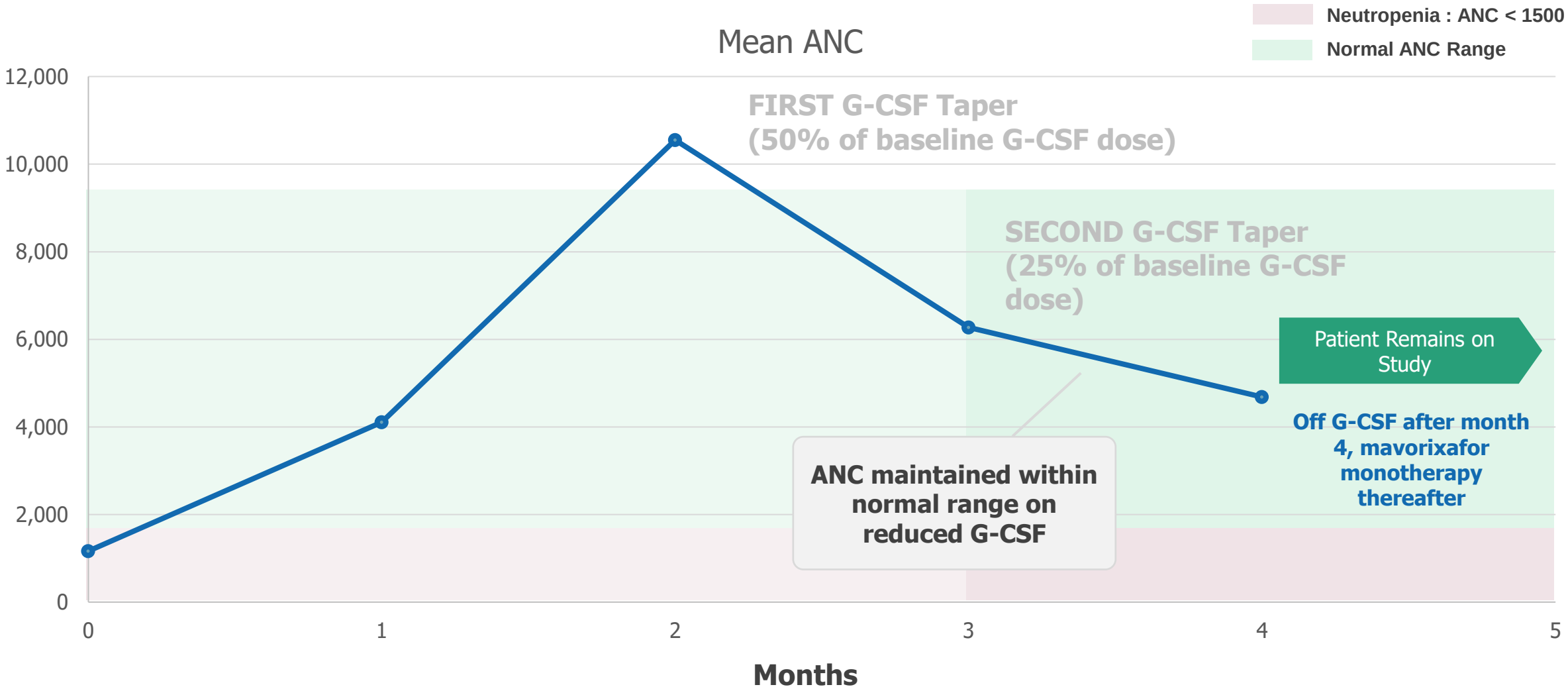

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Summary of CN Phase 2 Emerging Data

Safety	✓ Acceptable tolerability demonstrated in participants receiving G-CSF + mavorixafor, no SAEs reported ✓ Mavorixafor monotherapy well tolerated (see 4WHIM results) ✓ Supports continued chronic dosing of mavorixafor in CN population +/- G-CSF
Changes in ANC: Durability and Normalization	✓ Initial participants have all demonstrated increased ANC vs. baseline ✓ Increases sustained over months in normal range
Potential for G-CSF Taper	✓ Given sustained increases in ANC, participating physicians have explored reduction in G-CSF dosing ✓ Multiple participants are now off G-CSF and continue on study
Trial Continuing to Enroll	✓ Abstract submitted to December ASH meeting • Additional data expected to be shared at that time

Ongoing FDA discussions supported by emerging Phase 2 data

Phase 3 Study on Track to Initiate in 1H 2024

FDA input incorporated into design

Progress on Phase 3 Design

Population

Chronic Idiopathic, Congenital, and Acquired Primary neutropenia diagnosis

12 years and older

ANC < 1500 cells/µL and with history of recurrent/severe infections

On or off G-CSF at baseline

Planned Design

Randomized, placebo-controlled, 12-month trial

Likely endpoints to measure changes in ANC, infection burden, Quality of Life, G-CSF-related metrics, and others

Consideration of G-CSF taper

Dosing

Same as Phase 3 4WHIM clinical trial:

- Once-daily oral mavorixafor (adults and adolescents weighing >50 kg, 400 mg; adolescents weighing ≤50 kg, 200 mg)

Finalizing Endpoints & Statistical Plan in 2H 2023

Likely co-primary endpoint: ANC and clinical benefit

Statistical analysis plan (SAP) / size of trial

Developing the first oral treatments for chronic neutropenic disorders

Expected Upcoming Milestones

Cash expected to fund operations into 2025[1]

4Q22

- Achieved primary endpoint and key secondary endpoint in Phase 3 4WHIM clinical trial ✓

1H23

- ✓ Mavorixafor/WHIM Pre-NDA meeting with U.S. FDA
- Presented additional positive 4WHIM trial results
- Raised $65 million in PIPE financing

3Q23

- Preliminary Phase 2 chronic neutropenia clinical data
- Additional clarity on regulatory path for CN disorders
- Completed $115 million loan facility ✓

2H23

- Mavorixafor U.S. New Drug Application (NDA) submission in WHIM
- WHIM launch readiness update
- Additional Phase 2 CN data & final CN Phase 3 protocol

1H24

- Possible approval[2], PRV grant, launch of mavorixafor for WHIM in the U.S.
- OLE data from 4WHIM
- Initiate Phase 3 clinical trial of mavorixafor in chronic neutropenia

1. Cash runway estimate includes cash and equivalents of $142.3 million as of June 30, 2023 plus initial drawdown of $22.5 million from expanding debt facility completed in early August 2023. 2. Timeline assumes granting of priority review by U.S. Food and Drug Administration.

Q&A