

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 8-K**

**CURRENT REPORT**  
Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934

Date of Report (Data of earliest event reported): **August 5, 2022**

**X4 PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction of incorporation)

**001-38295**  
(Commission File Number)

**27-3181608**  
(IRS Employer Identification No.)

**61 North Beacon Street, 4th Floor**  
**Boston, Massachusetts**  
(Address of principal executive offices)

**02134**  
(Zip Code)

**(857) 529-8300**  
(Registrant's telephone number, including area code)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

**Securities registered pursuant to Section 12(b) of the Act:**

**Title of each class**  
**Common Stock, par value \$0.001 per share**

**Trading Symbol(s)**  
**XFOR**

**Name of each exchange on which registered**  
**The Nasdaq Stock Market LLC**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (240.12b-2 of this chapter). Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act

**Item 7.01**                      **Regulation FD Disclosure.**

X4 Pharmaceuticals, Inc. (the "Company") has updated its corporate presentation to be used from time to time when management of the Company presents at various industry and other conferences. On August 5, 2022, the Company updated its corporate presentation, which is available on the "Events and Presentation" section of the Company's investor relations website at <http://investors.x4pharma.com>. A copy of the updated corporate presentation is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Item 7.01 and in the corporate presentation attached as Exhibit 99.1 to this Current Report on Form 8-K shall not be deemed to be "filed" for purposes of Section 18 of the Securities Act of 1934, as amended, or otherwise subject to the liabilities of that Section, nor shall they be deemed incorporated by reference into any registration statement or other filing under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

The corporate presentation attached as Exhibit 99.1 to this Current Report on Form 8-K includes "safe harbor" language pursuant to the Private Securities Litigation Reform Act of 1995, as amended, indicating that certain statements contained therein are "forward-looking" rather than historical.

The Company undertakes no duty or obligation to update or revise the information contained in this Current Report on Form 8-K, although it may do so from time to time if its management believes it is appropriate. Any such updating may be made through the filing of other reports or documents with the Securities and Exchange Commission, through press releases or through other public disclosures.

**Item 9.01**                      **Financial Statements and Exhibits.**

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Corporate Presentation of the Company dated August 2022</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934 the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**X4 PHARMACEUTICALS, INC.**

Date: August 5, 2022

By: /s/ Derek Meisner  
Derek Meisner  
Chief Legal Officer

# Corporate Overview

August 2022



This presentation contains forward-looking statements within the meaning of 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, plans, or intentions. Forward-looking statements include, without limitation, statements regarding the clinical development and therapeutic potential of mavorixafor for the treatment of WHIM syndrome, chronic and other neutropenias, and of X4’s other product candidates; X4’s possible exploration of additional opportunities for mavorixafor; the expected duration of patent protection; the expected availability, content and timing of clinical data from X4’s ongoing clinical trials of mavorixafor; anticipated regulatory filings; clinical trial design; patient prevalence; market opportunities; and X4’s cash runway and ability to satisfy covenants in agreements with third parties.

Any forward-looking statements in this presentation are based on management’s current expectations and beliefs. Actual events or results may differ materially from those expressed or implied by any forward-looking statements contained herein, including, without limitation, 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, including, but not limited to, as a result of the effects of the ongoing COVID-19 pandemic, 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 effects arising from the testing or use of mavorixafor or other product candidates; the risk that patient prevalence, market or opportunity estimates may be inaccurate; 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; and other risks and uncertainties, including those described in the section entitled “Risk Factors” in X4’s Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on August 4, 2022, 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.

Certain information contained in this presentation relates to or is based on studies, publications, surveys and other data obtained from third-party sources and X4’s own internal estimates and research. While X4 believes these third-party sources to be reliable as of the date of this presentation, it has not independently verified, and makes no representation as to the adequacy, fairness, accuracy, or completeness of, any information obtained from third-party sources. Finally, while X4 believes its own internal research is reliable, such research has not been verified or validated by any independent source.

## A Unique Investment Opportunity with Significant Near-Term Milestones

### Sharp focus on chronic neutropenic disorders

Bringing innovative treatments to >5,000 estimated patients with rare diseases of the immune system and high unmet needs



### Unparalleled expertise in CXCR4 biology

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

### Mavorixafor – a 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

### Key mavorixafor upcoming clinical milestones expected:

- Results from Phase 1b trial in chronic neutropenia in late September 2022
- Results from global, pivotal Phase 3 trial in WHIM syndrome in 4Q 2022
- U.S. NDA filing expected in WHIM in early 2H 2023

### 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 through key upcoming milestones

## Seasoned Executive Leadership Team Continues to Execute on Mission



PAULA RAGAN, Ph.D.  
President & CEO

genzyme SANOFI



DIEGO CADAVID, M.D.  
Chief Medical Officer

Fulcrum Therapeutics Biogen



ADAM MOSTAFA  
Chief Financial Officer

abpro CANTOR Fitzgerald



ART TAVERAS, Ph.D.  
Chief Scientific Officer

Schering-Plough Biogen



MARY DIBIASE, Ph.D.  
Chief Operating Officer

Biogen EPIRUS

## Immunodeficiencies (U.S. Prevalence)



Common Variable Immunodeficiency (CVID)<sup>1</sup>  
> 10,000 U.S. patients



Chronic Neutropenias<sup>2</sup>  
> 5,000 U.S. patients  
(severe and moderate)



WHIM Syndrome<sup>3</sup>  
> 1,000 U.S. patients

### Chronic Immunosuppression

- Life-long severe and/or life-threatening infections
- Reduced/no response to vaccines
- Increased cancer risk
- High morbidity (bronchiectasis, hearing loss) and life-threatening sepsis

### Caused By

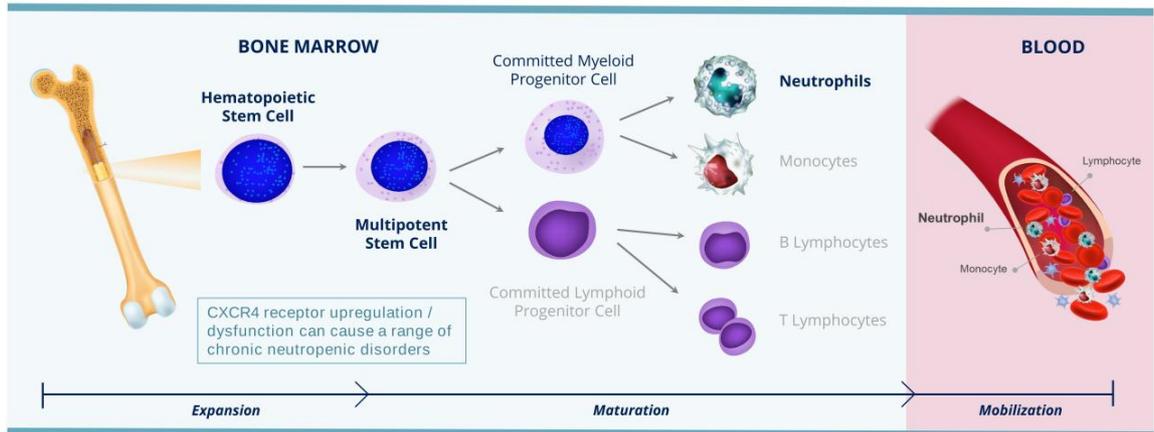
- Low white blood cell counts (cytopenias) and/or
- Dysregulated or dysfunctional immune cells

### Treated with Injectables

- Antibiotics for acute infections
- G-CSF for those with severe neutropenia
- IVIG for those with hypogammaglobulinemia (prevention)
  - Stem cell transplant in rare/severe cases

Unmet need: Oral treatment that corrects and regulates a range of immune system deficiencies

1. Odnoletkova et al *Orphanet Journal of Rare Diseases* v13, 201 (2018). 2. US estimate for severe plus 10% penetrance for moderate and prevalence from Andersen et al. *J Intern Med.* 2016 Jun;279(6):566-75. 3. Company market research. Qessential market research, 2019 and IPM.ai artificial intelligence study, 2020.



Mature neutrophil pool in bone marrow is **20 times higher** than in circulation<sup>1</sup>

Neutrophils are ~50% of circulating immune cells<sup>1</sup>

Source: Bainton DF (1980) The cells of inflammation: a general view. In Weissmann G (ed) The Cell Biology of Inflammation, vol 2, pp 1–25. Amsterdam: Elsevier/North-Holland



**Only oral CXCR4 antagonist in development**

- Small molecule with high potency and selectivity
- Durable half-life supporting once-daily dosing
  - 2 or 4 capsules, once per day (WHIM syndrome)

**Antagonizes Both Wild-Type and Mutated CXCR4**

- Demonstrated ability to increase the mobilization of white blood cells, including neutrophils, from bone marrow to bloodstream
- Potential utility across broad range of chronic neutropenic disorders with high unmet needs

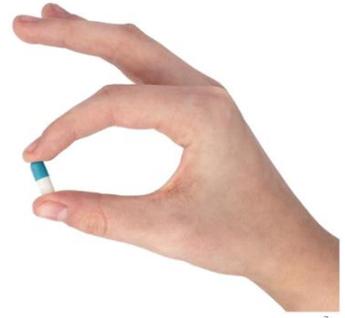
**Safety Profile Supports Chronic Use**

- >200 patients/subjects treated to date
- Some patients on Rx for several years

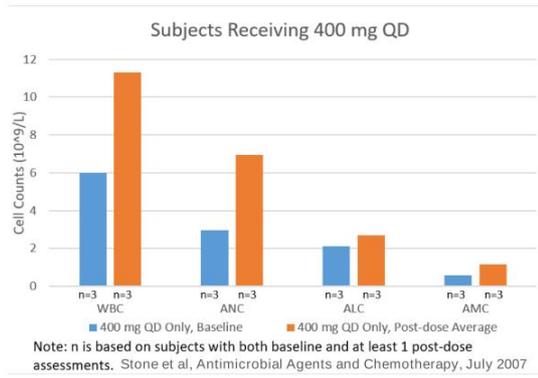
**Favorable Regulatory Designations (WHIM syndrome)**

- Breakthrough Therapy Designation (U.S.)
- Fast Track Designation (U.S.)
- Rare Pediatric Disease Designation (\*\*PRV eligible)
- Orphan Drug Status in U.S. and Europe

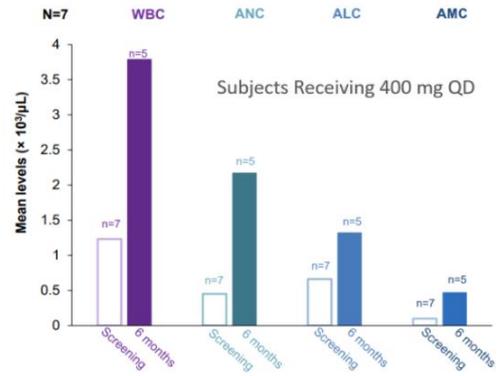
**Patent Protection Expected Through 2038 and Beyond**



Mavorixafor in Healthy Volunteers



Mavorixafor in WHIM Syndrome



ASH 2021. Poster #2186.

WBC: white blood cells; ANC: absolute neutrophil count;  
ALC: absolute lymphocyte count; AMC: absolute monocyte count

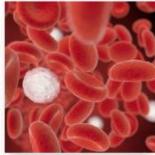
## Pipeline: Significant Upcoming Milestones Expected



	Candidate	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Expected Milestones	Target Patient Populations
CHRONIC NEUTROPENIC DISORDERS (In-house)	Mavorixafor	WHIM Syndrome <sup>1</sup> (Warts, Hypogammaglobulinemia, Infections and Myelokathexis)	Phase 3				Top-line data 4Q 2022 NDA Submission early 2H 2023	> 1,000 U.S. <sup>2</sup>
		Chronic Neutropenia (Congenital, Cyclic, and Idiopathic)	Phase 1b				Add'l data / clinical update in 3Q 2022	> 5,000 U.S. <sup>3</sup>
	X4P-003	TBD						
ONCOLOGY (Partnering)	Mavorixafor	Waldenström's Macroglobulinemia* (double-mutation patients)	Phase 1b					> 2,000 U.S. <sup>4</sup>
	X4P-002	Oncology indications*	IND-enabling					Other leukemias and lymphomas > 25,000 U.S. <sup>3</sup>

\*Programs only being advanced through partnership

Sources: 1. Phase 3 open label extensions (OLE) for WHIM ongoing 2. Company market research, Qessential market research, 2019 and IPM.ai artificial intelligence study, 2020. 3. Estimate using Andersen et al. J Intern Med. 2016 Jun;279(6):566-75. 4. WM Epidemiology Analysis The NemetzGroup.



**Pancytopenia:** All White Blood Cells Affected & Reduced



**Severe bacterial infections**  
In multiple organ systems: bronchiectasis (lung), hearing loss (ear), cellulitis (skin)



**Viral infections & cancer risk**  
Disfiguring recalcitrant warts; EBV and HPV-associated cancers

**0** Approved targeted therapies; symptomatic treatment with G-CSF and IVIG

[www.whimsyndrome.com](http://www.whimsyndrome.com)

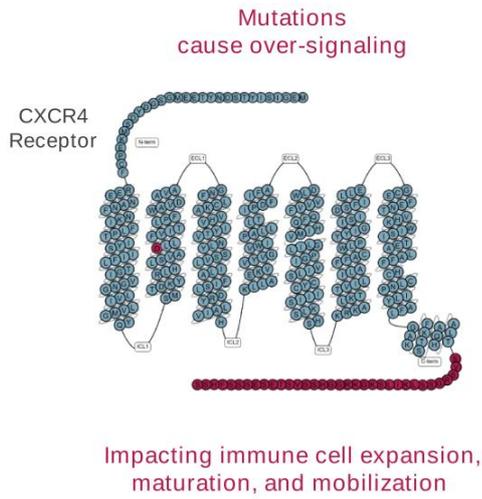
## Leanne's Story - Living with WHIM Syndrome



WHIM Patient  
New South Wales, Australia

[In early marriage], I had 8 episodes of pneumonia in 8 months, more likely one continual episode of pneumonia. I had a PICC line, I was having intravenous antibiotics.

Every time I was better, **they'd stop the antibiotics** and almost immediately **I'd become unwell again.**

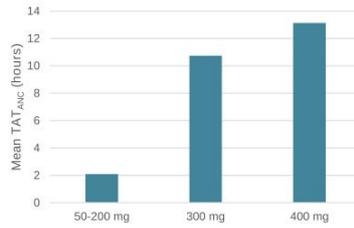


- WHIM Syndrome: Spectrum of clinical presentations mostly with (and sometimes without) CXCR4 mutations
  - W: warts
  - H: hypogammaglobulinemia
  - I: infections
  - M: myelokathexis (hyper-cellular bone marrow)
- In most cases, autosomal dominant disease driven by pathogenic CXCR4 mutations; mostly located in "tail" (c-terminus) of receptor
  - ~16 identified to date; # increasing with research
- Results in over-signaling of CXCR4 that impacts all white blood cells

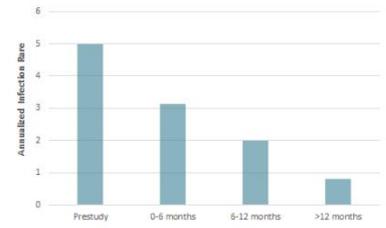
**WHIM Phase 2 Trial Design**

- Intra-patient dose-escalation: safety, PK
- Endpoint: clinically relevant blood measurement: "Time above threshold for absolute neutrophil count" (TAT<sub>ANC</sub>)
  - FDA & EMA agreement on use as P3 endpoint
  - Wart burden and infection rates also examined
- Patients eligible to participate in an open-label extension (OLE) phase of the trial

Major (>600%) Increase in Neutrophil Counts (TAT<sub>ANC</sub>)



>80% Reduction in Annualized Infection Rates



>75% reduction in the number of warts while on treatment



## WHIM Phase 2 OLE: Continued Improvements Seen Across Patients

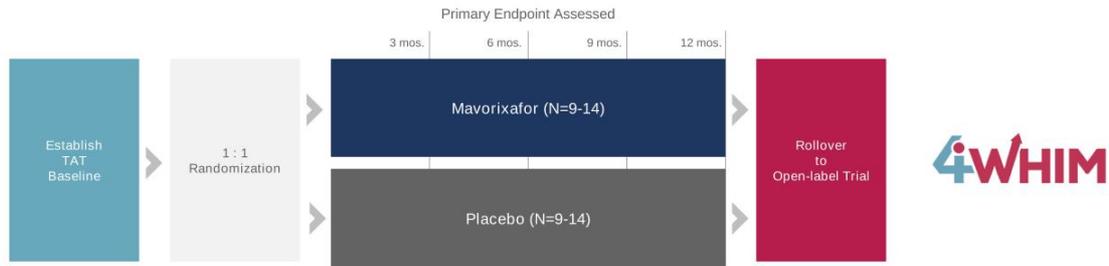


Patient Cases	Baseline WBCs & On-Treatment TAT	Patient Profiles & OLE Study Results
 <p>Frequent Infections</p>	Baseline WBC: 0.7    ANC: 0.19    ALC: 0.43 On-treatment <sup>1</sup> TAT:    ANC: 24 hrs    ALC: 15.78 hrs	42 y/o adult male electrician. Sick all of the time from WHIM. Sniffles turn into pneumonia. <b>Reported reduced healing times and no severe infections on study.</b>
 <p>Nothing Works Tried Every Therapy</p>	Baseline WBC: 0.54    ANC: 0.06    ALC: 0.35 On-treatment <sup>2</sup> TAT:    ANC: 9.51 hrs    ALC: 20.1hrs	Adult female, doctoral candidate. Frequent sinus infections and warts. Previously tried 20+ therapies with no success. <b>Reported no new warts and decreased to no infections while on study.</b>
 <p>Severe Bone Pain</p>	Baseline WBC: 0.9    ANC: 0.3    ALC: 0.5 On-treatment <sup>3</sup> TAT:    ANC: 22.5 hrs    ALC: 4.6 hrs	Adult female pharmacist. Hospitalized at least yearly for severe infection / IV antibiotics. Severe bone pain while on G-CSF. <b>Reported significantly fewer infections and reduced healing time on study.</b>
 <p>Unpredictable Life HPV Cancer Risk</p>	Baseline WBC: 4.1    ANC: 2.3    ALC: 1.4 On-treatment <sup>4</sup> TAT:    ANC: 24 hrs    ALC: 15.78 hrs	Adult female nurse experiencing hospitalizing infections since an infant (10 wks old). Multiple HPV-associated lesions excised. <b>No new infections after first few months of therapy.</b>

- 100% of WHIM participants interviewed experienced a meaningful treatment benefit due to mavorixafor
- All participants (n=4/4) stated infections were less severe and that improvements were consistent during the trial
- Most (n=3/4, 75%) WHIM participants reported a decrease in overall healing time for infections
- Major improvement in warts was seen in the one patient with significant wart burden\*

\* Improvements in warts categorized as either a reduction of wart size, decrease in number of warts, and/or the lack of new wart development.  
 Reference range. Hemogram (units/reference range) WBC - White blood cells (x10<sup>9</sup> / 3.5-10.5), ANC - Absolute Neutrophil Count (x10<sup>9</sup> / 2.1-7.8) ALC - Absolute Lymphocyte Count (x10<sup>9</sup> / 0.8-3.3)  
 TAT: Time Above Threshold. 1. OLE residence visit 3 post end of study receiving 400mg dose. 2. OLE residence visit 1 post end of study receiving 400 mg dose. 3. Week 21 study visit receiving 300mg dose.  
 4. OLE residence visit 1 post end of study receiving 300 mg dose.

## Design of Mavorixafor Global Phase 3 Trial in WHIM (*Patients ≥12 years of age*)



- Primary Endpoint: Biomarker of time above threshold (>500 cells per microliter) for absolute neutrophil count ( $TAT_{ANC}$ ); average of four assessment timepoints
- Secondary Endpoints:  $TAT_{ALC}$ , infections, wart burden, infection and wart composite, QoL assessment & others
- Dosing: 400mg QD in patients for subjects above 50 kg; 200 mg QD for those below 50 kg and <18 years
- Enrollment Complete: Over-enrolled with 31 patients

Phase 3 Top-line Data Expected in 4Q 2022

Targeted Mavorixafor  
U.S label

For the treatment of WHIM Syndrome in patients  $\geq 12$  years

Diagnosis of WHIM Syndrome:

Clinical and Laboratory Presentation\* consistent with WHIM ( $\geq 1$  of the following: warts, lymphopenia, hypogammaglobulinemia, neutropenia, and/or infections)

1. Pathogenic / likely pathogenic CXCR4 mutations – including variants causing gain-of-function CXCR4 signaling
2. Myelokathexis

Diagnostic paradigm is evolving; some leading clinicians would consider diagnosing WHIM without CXCR4 or myelokathexis (which can easily be missed)

The Different Faces of WHIM



1. "Combined Immunodeficiency" is defined as one or more of abnormal neutrophils (ANC) lymphocytes (ALC) and monocytes (AMC) and hypogammaglobulinemia. Clinical presentation of infections (bacterial and/or viral including HPV) is also required.

2. WHIM syndrome has been diagnosed without CXCR4 mutations or myelokathexis. Heusinkveld LE, Yim E, Yang A, Azari AB, Liu Q, Gao JL, McDermott DH, Murphy PM. Pathogenesis, diagnosis and therapeutic strategies in WHIM syndrome immunodeficiency. *Expert Opin Orphan Drugs.* 2017, 5(10): 813-825. doi: 10.1080/21678707.2017.1375403. Epub 2017 Sep 25. PMID: 29057173; PMCID: PMC5648064.

## Chronic Neutropenia Beyond WHIM

Chronically immunocompromised patients due to sustained or intermittent low neutrophil counts (neutropenia); can be caused by

- G-CSF response defects
- Elevated bone marrow CXCL12 downregulating G-CSF production
- Stem cell loss
- Differentiation/maturation arrest
- Increased apoptosis
- Prolonged survival of senescent neutrophils preventing regeneration of nascent neutrophils
- Impaired release from bone marrow
- Impaired de-margination from periphery
- Functional upregulation of CXCL12/CXCR4 pathway

The magnitude of neutropenia correlates with higher risk of severe infections and greater frequencies of infections

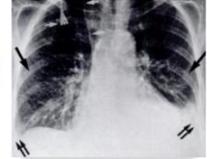
- Mild if ANC between 1,000 and 1,500/ $\mu\text{L}$
- Moderate if ANC between 500 and 1,000/ $\mu\text{L}$
- Severe if ANC  $<500/\mu\text{L}$  (estimated 5,000 U.S. patients<sup>1,2</sup>)

Skin Abscesses



Oral Ulcerations

Pneumonia



1. Eligible patients in the TriNetX USA database who had an ANC  $<1500$  cells/ $\mu\text{L}$  in calendar year of interest and two additional ANC  $<1500$  cells/ $\mu\text{L}$  in the subsequent two years.  
2. Numbers are extrapolated to reflect the total U.S. population each year (multipliers ranged from x6.4 in 2017 to x5.2 in 2019).

## Injections of G-CSF

- Once or twice-daily at 5-6  $\mu\text{g}/\text{kg}$  to reduce severe neutropenia and risk of infections
- “Dose down-titration” and reduced frequency often implemented to aid with tolerability for chronic use
- Neutrophil target: ~1,000-2,000 cells/ $\mu\text{L}$

## Challenges for patients on G-CSF

- 25% continue to experience severe bacterial infections while on chronic treatment<sup>1</sup>
- Increasing risk of myelodysplastic syndromes (MDS)<sup>2</sup>
- ~70% have moderate or severe bone-pain impacting compliance and QoL<sup>3</sup>

No alternative therapies, except for bone marrow transplantation



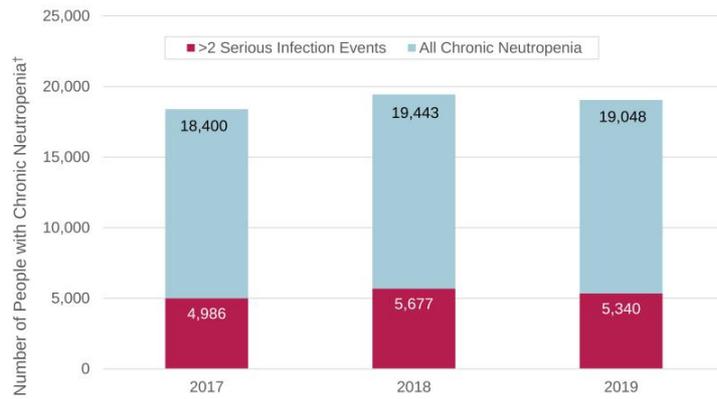
U.S. G-CSF Market For Severe Chronic Neutropenia: Estimated \$120-460 Million

1. Fontbruen et al, Blood, October 2015 – Volume 128(14). 2. Dale et al Support Cancer Ther 2006 Jul 1;3(4):220-31:. 3. Michniacki et al, Blood (2019) 134 (Supplement\_1): 3449.

> 20,000 People (aged >12) Estimated with Chronic Neutropenia in the U.S.<sup>1</sup>:  
Thousands Have Major Health Impact with >2 Serious Infection Events per Year



**CN Patients Experience Serious Infection Events (SIEs):** Any infection that required hospitalization, intravenous antibiotics and/or resulted in disability or death



~25% of all CN patients have two or more SIEs<sup>2</sup> per year

Source: X4 Data on file; TriNetX USA EMR Database analysis using inclusion criteria and medical record coding, data and confirmation. Patients 12 years of age and above.

<sup>1</sup> Eligible patients in the TriNetX USA database who had an ANC <1500 cells/μl in calendar year of interest and two additional ANC <1500 cells/μl in the subsequent two years.  
<sup>2</sup> Numbers are extrapolated to reflect the total U.S. population each year (multipliers ranged from x6.4 in 2017 to x5.2 in 2019).

Phase 1b trial: Studies Activity of Mavorixafor in Broad CN Population

- Severe and moderate CN – congenital, idiopathic, cyclic
- With or without genetic causes
- With or without concomitant G-CSF

Endpoints: Safety and tolerability, change in ANC (and other WBCs) vs. pre-treatment baseline

Goal: Define broad CN populations responsive to mavorixafor in support of FDA interactions regarding proposed registrational trial(s)

Studying mavorixafor across broad chronic neutropenic disorders

### Patients 12 years old and older

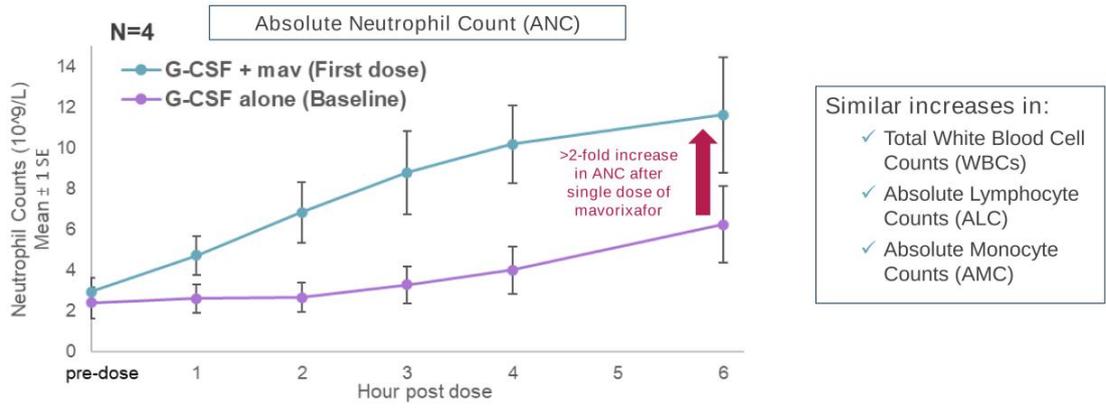
#### Protocol Version 5

- 14 days of treatment with mavorixafor
- Blood sampling over 6 hours on days -1, 1 and 14
- Severe (ANC<500/uL) chronic idiopathic neutropenias on concomitant G-CSF
- Selected severe congenital neutropenia conditions with or without concomitant G-CSF
- Clinic only study

#### Protocol version 6

- 1 day of treatment
- Blood sampling over 8 hours on days -1 and 1
- Any congenital, cyclic, or idiopathic neutropenic disorder regardless of concomitant G-CSF use
- Neutrophil count <1,000  $\mu$ L if not on G-CSF
- Study can be done in the clinic or at home

Meaningful Responses\* to Single Dose in Idiopathic Chronic Neutropenia Patients



\* ASH 2021, Poster #2186.

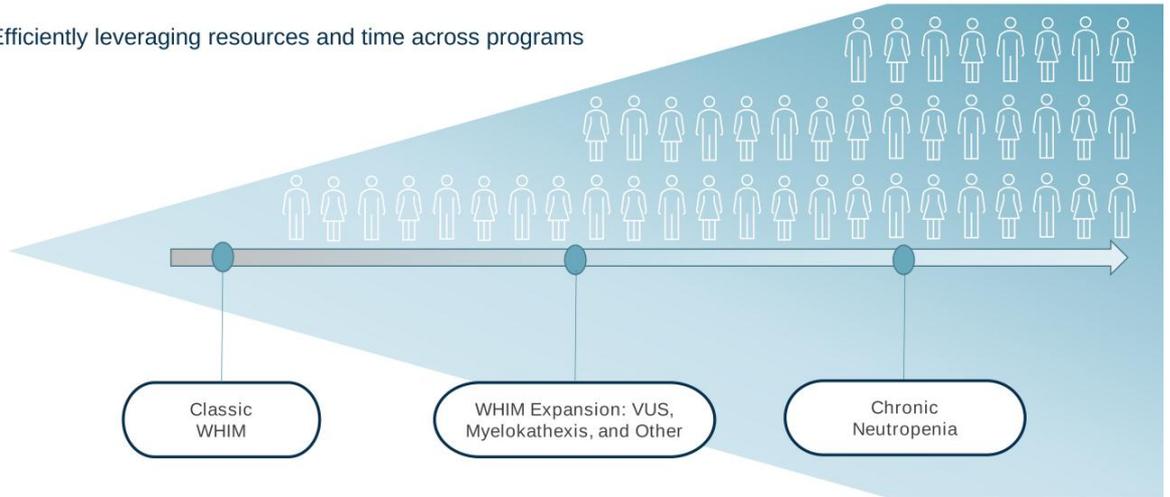


- ✓ More than 5,000 patients in the U.S. with chronic neutropenia at significantly increased rates of hospitalization with severe infections
- ✓ Only available treatment is injectable, with limited tolerability and frequent injections
- ✓ **Mavorixafor single dose activity:** Initial data from the ongoing Phase 1b trial in adult patients with idiopathic CN show clear elevation of neutrophils and other WBCs
- ✓ Further positive data from this ongoing trial to inform registrational trial to investigate mavorixafor as the potential first oral treatment to reduce the burden of infections in patients with chronic neutropenia

Fully enrolled Phase 1b trial;  
Readout expected late September 2022



Efficiently leveraging resources and time across programs



### Recent Events:

- Raised gross proceeds of ~\$56 million from PIPE financing in June 2022
- Amended debt facility with Hercules Capital extending interest-only payment period into 2024
- Announced sharpening of corporate focus and resources on development of mavorixafor for chronic neutropenic disorders, including WHIM syndrome
- Updated X4 oncology programs
  - Positive results from Phase 1b data of mavorixafor for the treatment of Waldenström's Macroglobulinemia (WM)
  - Announced granting of U.S. Orphan Drug Designation for mavorixafor in WM regardless of CXCR4 mutation status
  - Advancement of mavorixafor WM and pre-clinical X4P-002 programs only to proceed upon strategic partnership(s)

### Upcoming Expected Milestones

- Late September 2022 presentation of data from fully enrolled Phase 1b trial of mavorixafor in chronic neutropenia patients
- 4Q 2022 unveiling of data from global, pivotal Phase 3 (4WHIM) clinical trial of mavorixafor for the treatment of patients with WHIM syndrome
- Early 2H 2023 submission for U.S. regulatory approval of mavorixafor for WHIM
- Clarification of regulatory path forward for mavorixafor in CN indications

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**\$101M<sup>1</sup>**  
(pro-forma including recent  
PIPE financing)

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Cash Expected to Fund Operations into 3Q 2023<sup>2</sup>

Top-tier Life Science-Focused Institutional Shareholder Base

Analyst Coverage

STIFEL

OPPENHEIMER

HCW  
H.C. WAINWRIGHT & CO.

COWEN

BROOKLINE  
CAPITAL MARKETS

B RILEY FBR

<sup>1</sup> Pro-forma for June 30, 2022 cash and restricted cash balance of \$48.7 plus estimated net proceeds from PIPE financing that closed on July 6, 2022. <sup>2</sup> As described in detail in our most recent Form 10-Q, our agreement with Hercules Capital, Inc. contains a minimum cash covenant that becomes effective on September 1, 2022, subject to certain exceptions. Our current cash runway projections assume continued compliance with this covenant. Failure to satisfy this covenant could result in accelerated principal and interest payments due that could shorten our cash runway. Outstanding debt balance as of 6/30/2022 is \$32.5 million.

NASDAQ: XFOR

U.S. Headquarters  
61 North Beacon Street, 4th Floor  
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Research Facilities  
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