Developing treatments designed to have a clear and profound impact for patients with rare diseases, including WHIM syndrome and uncommon cancers

Investor Deck

September 2021

Forward-Looking Statements

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, Waldenström’s macroglobulinemia, Severe Congenital Neutropenia and other neutropenias and other primary immunodeficiencies, 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; 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 or delayed patient enrollment, 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; 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 3, 2021, 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.

Building a Global Rare Disease Company

Mavorixafor: late-stage disease-modifying therapeutic candidate targeting CXCR4

- Phase 3 trial in WHIM syndrome - enrollment completion 3Q21; top-line data expected 4Q22
- Phase 1b trial ongoing in Waldenström's macroglobulinemia
- Phase 1b trial ongoing in Severe Congenital Neutropenia
  - Exploring additional chronic neutropenia indications

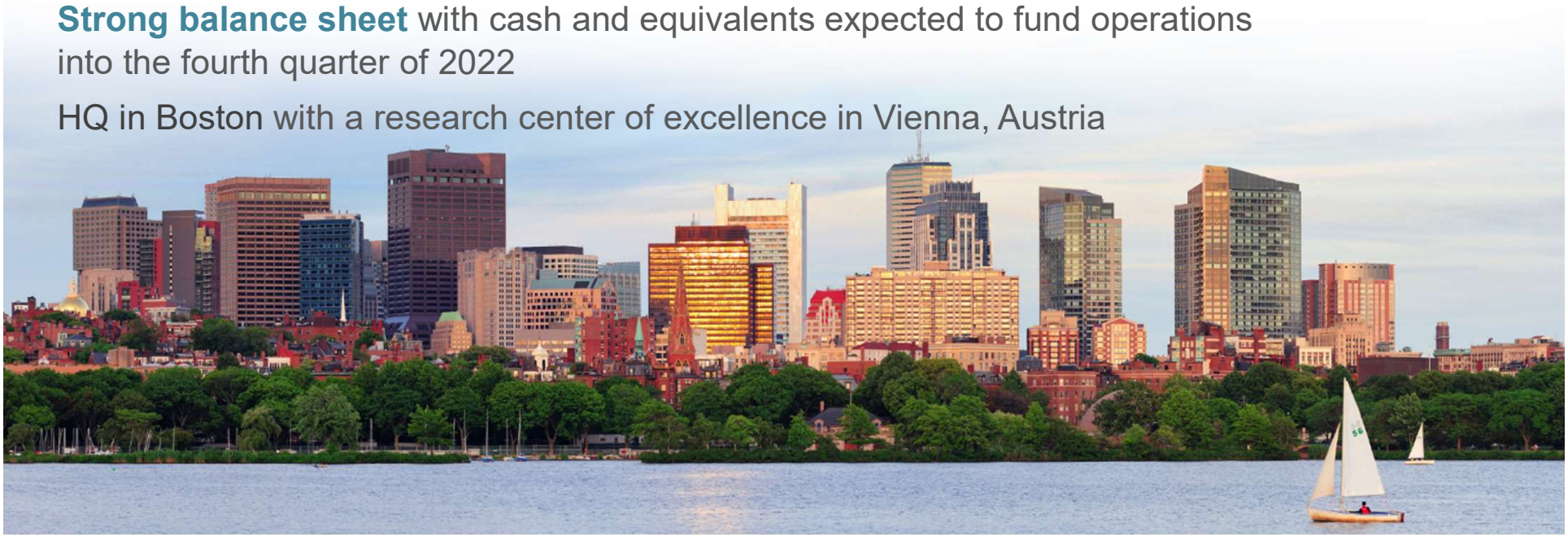
Initial therapeutic indications target >10,000 rare disease patients

Emerging pipeline with multiple pre-clinical candidates

Leadership with deep expertise in the science of the CXCR4 pathway and in successful development and commercialization of rare disease therapeutics

Strong balance sheet with cash and equivalents expected to fund operations into the fourth quarter of 2022

HQ in Boston with a research center of excellence in Vienna, Austria



Leadership: Proven Team with Rare Disease Expertise

MANAGEMENT

PAULA RAGAN, Ph.D.
CEO
genzyme · SANOFI

MARY DIBIASE, Ph.D.
COO
Biogen · EPIRUS

ADAM MOSTAFA
CFO
abpro · Cantor Fitzgerald

CARRIE MELVIN
SVP of Development Operations
TESARO · KURA ONCLOGY · EMD SERONO

DIEGO CADAVID, M.D.
CMO
Fulcrum Therapeutics · Biogen

SHARIQ ALI, Ph.D.
VP of Medical Affairs
WAVE · SANOFI · genzyme

ART TAVERAS, Ph.D.
CSO
Schering-Plough · Biogen

MIHAELA MACNAIR, Ph.D.
VP of Regulatory Affairs
ATEA Pharmaceuticals · Biogen

DEREK MEISNER, J.D.
General Counsel
genocea · RACapital

MICHELE RHEE, M.P.H., M.B.A.
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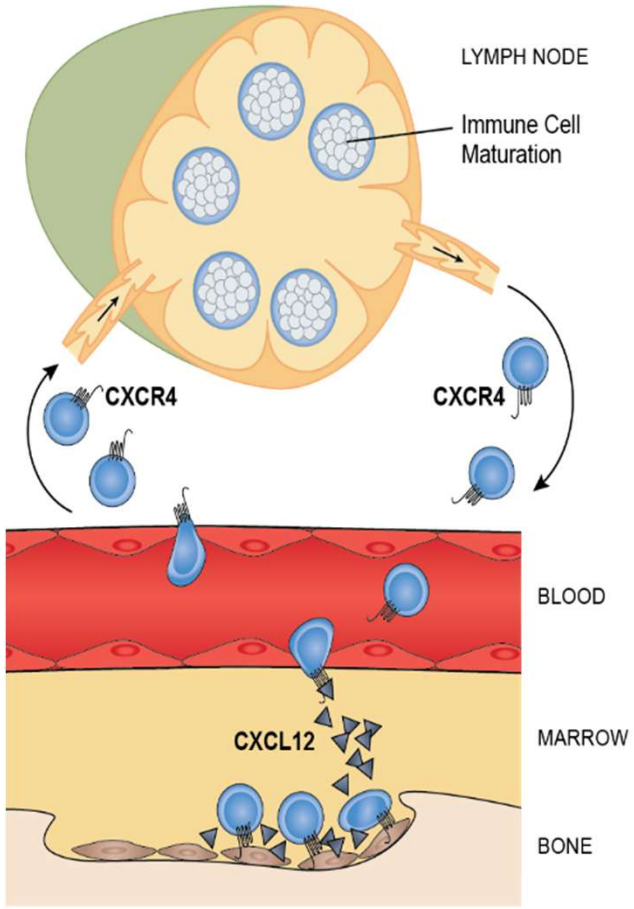
Mavorixafor: Targeting Broad Range of Disease States Expected to Benefit from CXCR4 Antagonism

MECHANISM OF ACTION



LEAD INDICATIONS

PHASE 3:
WHIM Syndrome

PHASE 1B:
Waldenström's Macroglobulinemia

→ Validated by blocking **"Gain-of-Function" CXCR4 genetic mutations**

LABEL EXPANSION OPPORTUNITIES

PHASE 1B:
Severe Congenital Neutropenia

→ Immune deficiency corrected by **blocking CXCR4 Signaling**

PIPELINE

PRECLINICAL PROGRAMS:
Additional immuno-deficiencies

→ Established linkages to **immune-system genetics/pathways**

Mavorixafor Overview

First-in-class CXCR4 antagonist

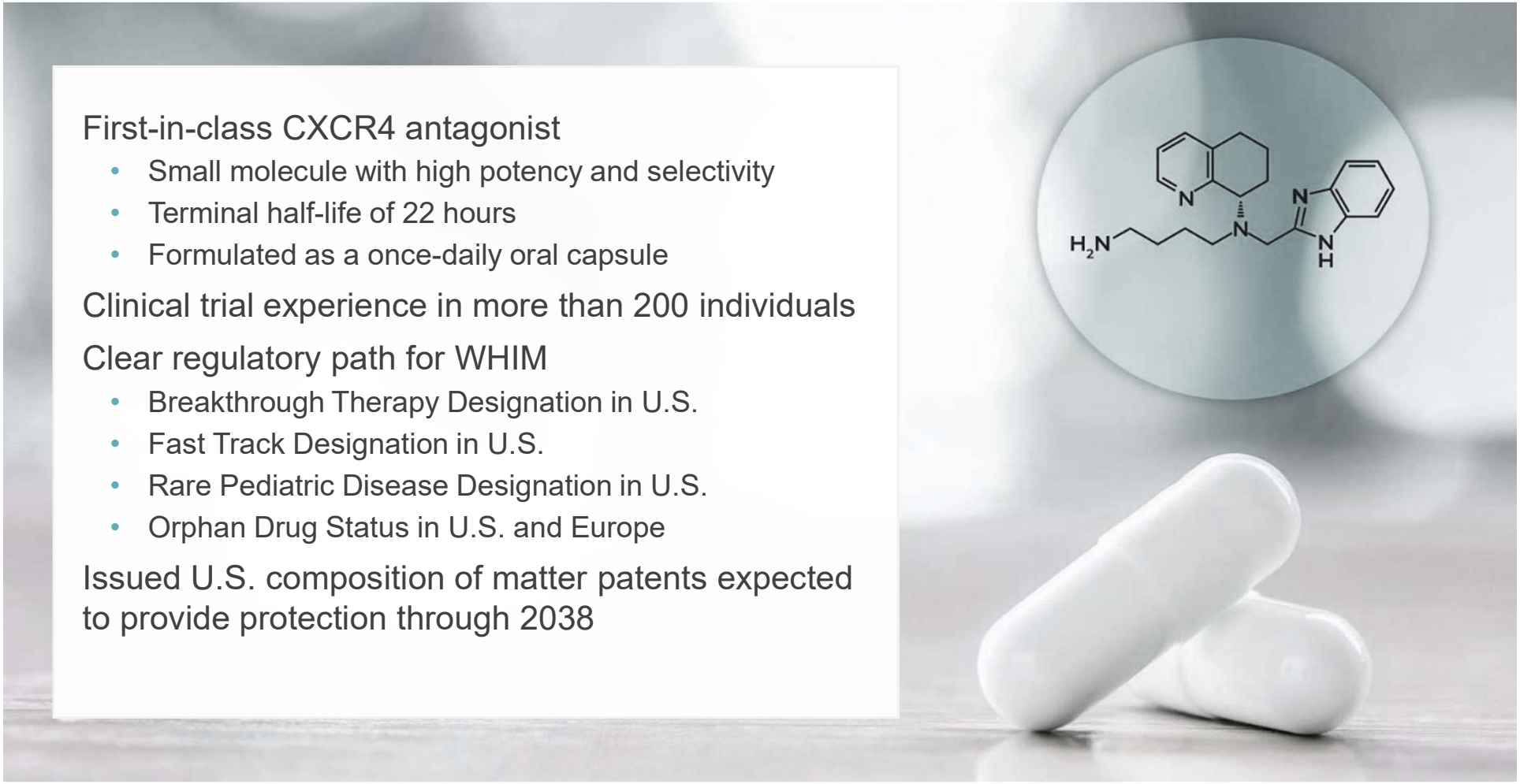
- Small molecule with high potency and selectivity
- Terminal half-life of 22 hours
- Formulated as a once-daily oral capsule

Clinical trial experience in more than 200 individuals

Clear regulatory path for WHIM

- Breakthrough Therapy Designation in U.S.
- Fast Track Designation in U.S.
- Rare Pediatric Disease Designation in U.S.
- Orphan Drug Status in U.S. and Europe

Issued U.S. composition of matter patents expected to provide protection through 2038

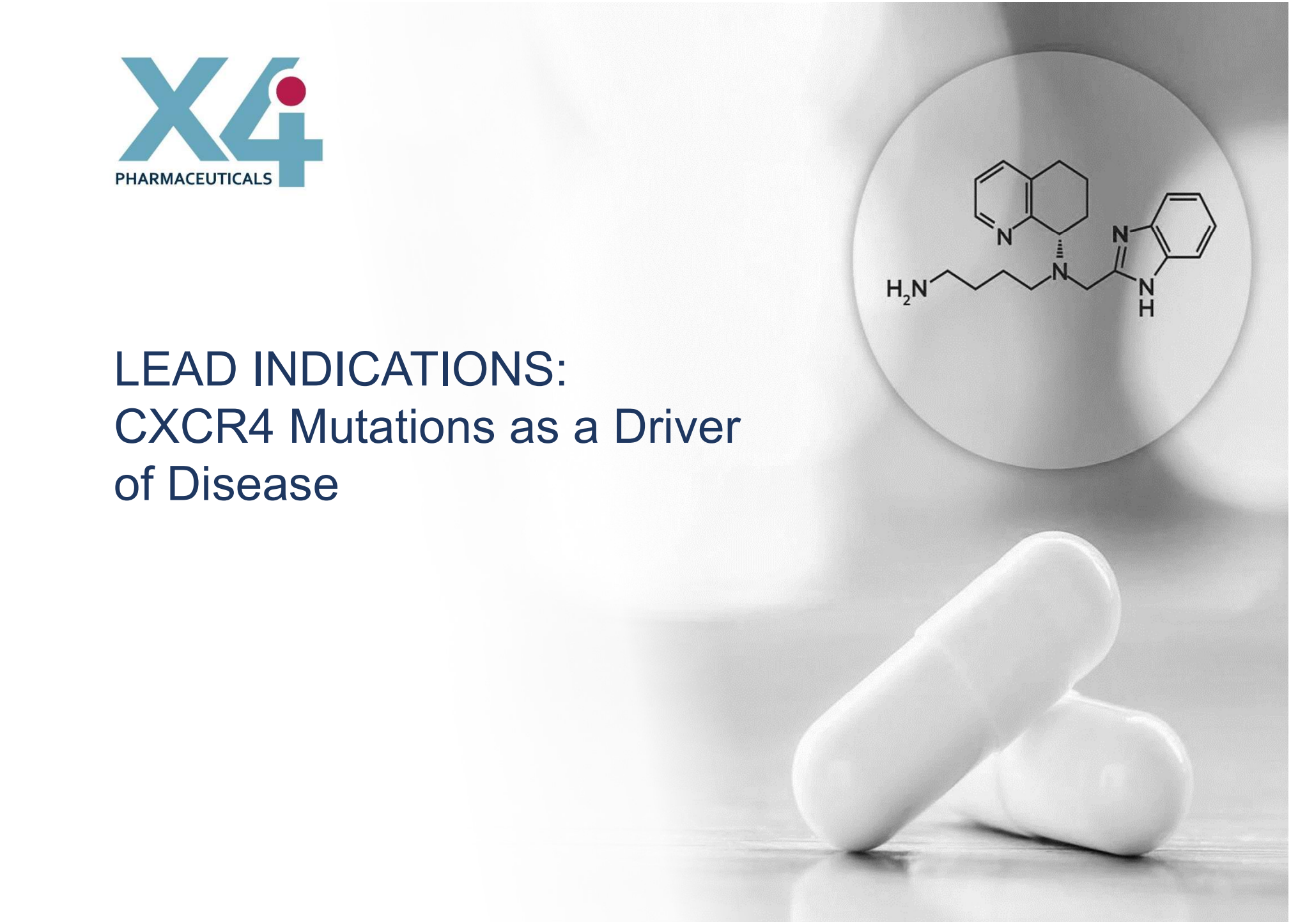


X4's Product Pipeline

CANDIDATE	INDICATION	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3
Mavorixafor (X4P-001)	Warts, Hypogammaglobulinemia, Infections and Myelokathexis (WHIM) syndrome[1]				PHASE 3
	Waldenström's Macroglobulinemia (WM)		PHASE 1B		
	Severe Congenital Neutropenia (SCN)		PHASE 1B		
X4P-002	Oncology indications				
X4P-003	Primary immuno-deficiencies (PID)				

[1] *Phase 2 open label extension trial for WHIM ongoing*

LEAD INDICATIONS: CXCR4 Mutations as a Driver of Disease



About WHIM Syndrome

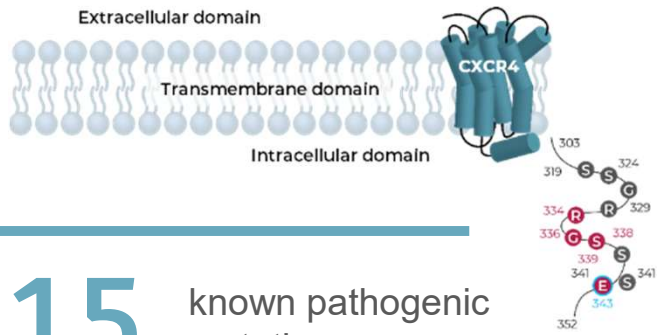
W **Warts** – can lead to HPV-related cancers

H **Hypogammaglobulinemia**

I **Infections**

M **Myelokathexis** – retention of mature neutrophils in the bone marrow

Immunodeficiency caused by gain-of-function mutations in the CXCR4 receptor that lead to excessive "on" signaling, dysfunctional immune cell trafficking, and an impaired ability to mount a healthy immune response



Up to

3,700[1]

Potential U.S. WHIM patients

0

Approved targeted therapies

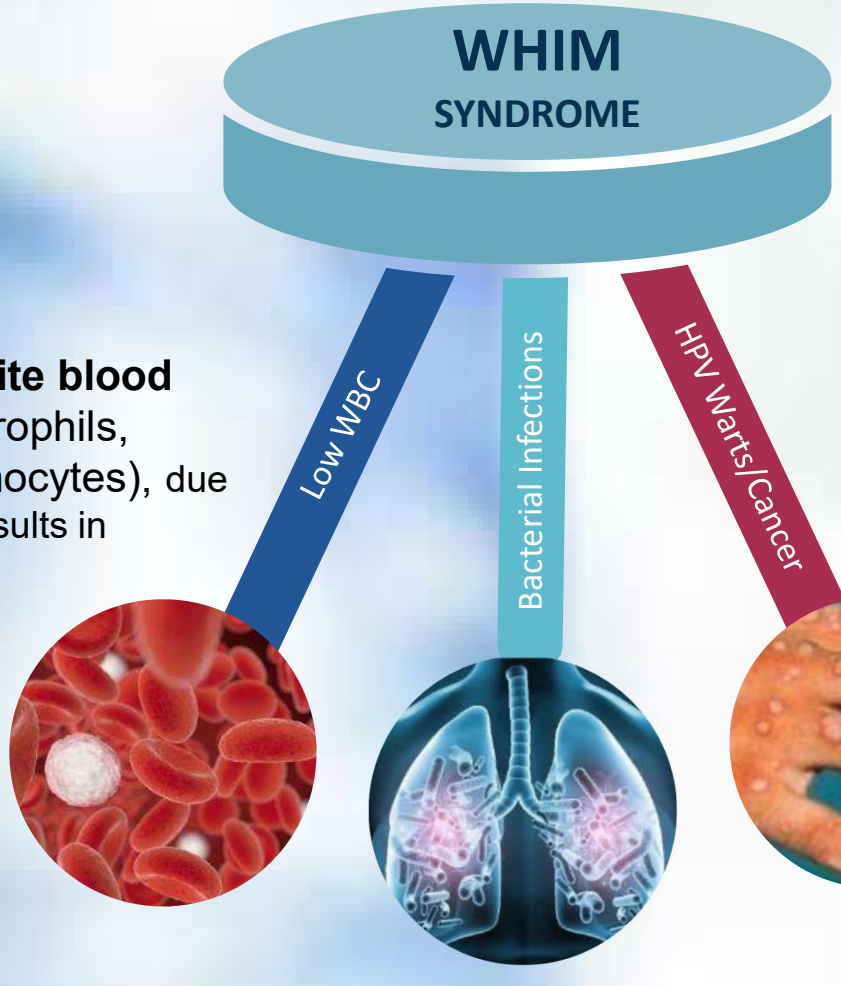
Symptomatic Rx; antibiotics, G-CSF, immunoglobulins

15 known pathogenic mutations

Genetic test to diagnose

1. IPM.ai 2020; study used artificial intelligence to matches to a known WHIM patient group from an insurance claims database of ~300M US persons

Unmet Needs in WHIM Syndrome



Critically low white blood cell counts (neutrophils, lymphocytes, monocytes), due to *myelokathexis*, results in immunosuppression

HPV infections
disfiguring recalcitrant warts; HPV-associated cancers

Severe bacterial infections in multiple organ systems
Bronchiectasis (lung), hearing loss (ear), cellulitis (skin)

WHIM Phase 2 Trial Informs Pivotal Phase 3 Trial

PHASE 2 TRIAL DESIGN

INCLUSION

- Absolute neutrophil count: ANC ≤400/µL *and/or*
- Absolute lymphocyte count: ALC ≤650/µL

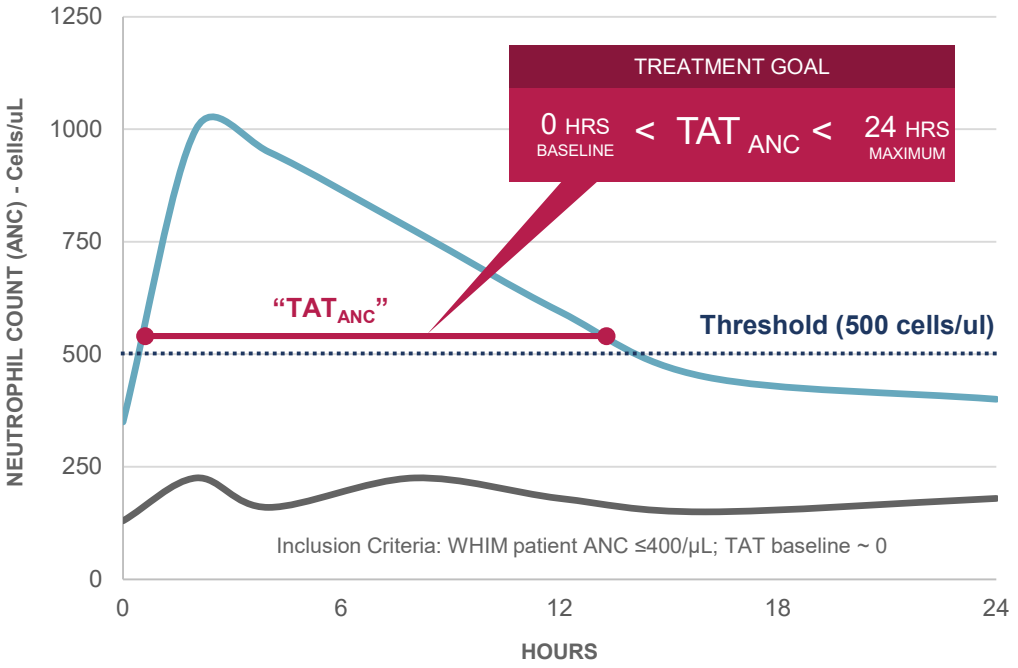
DOSE ESCALATION + OPEN-LABEL EXPANSION

- Dose Escalation: 50 to 400mg oral capsule once daily (QD), N = 8 patients
- Open-Label Expansion: If completed >24 weeks of dose escalation (N=5)

ENDPOINTS & ASSESSMENTS

- Safety, infections, warts, pharmacokinetics (PK) / pharmacodynamics (PD) to support dose-selection
- Open label extension examined infection rates, warts, long-term safety
- **Primary Endpoint for Phase 3:** 24-hr Time (hrs.) Above Threshold of Absolute Neutrophils Count (TAT_{ANC})

ILLUSTRATIVE TRIAL ENDPOINT EXAMPLE

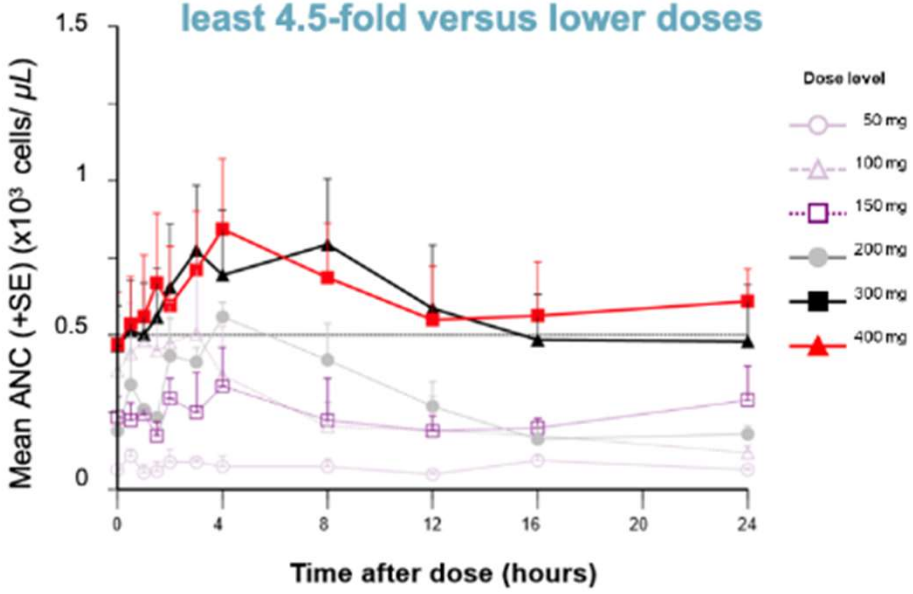


OBJECTIVE: INCREASE DAILY NEUTROPHIL COUNTS (ANC) ABOVE THRESHOLD AS MEASURED OVER 24 HOURS: TIME ABOVE THRESHOLD (TAT)

WHIM Phase 2 Trial: Open-Label Extension
Mavorixafor Successfully Addressed All 3 Unmet Needs

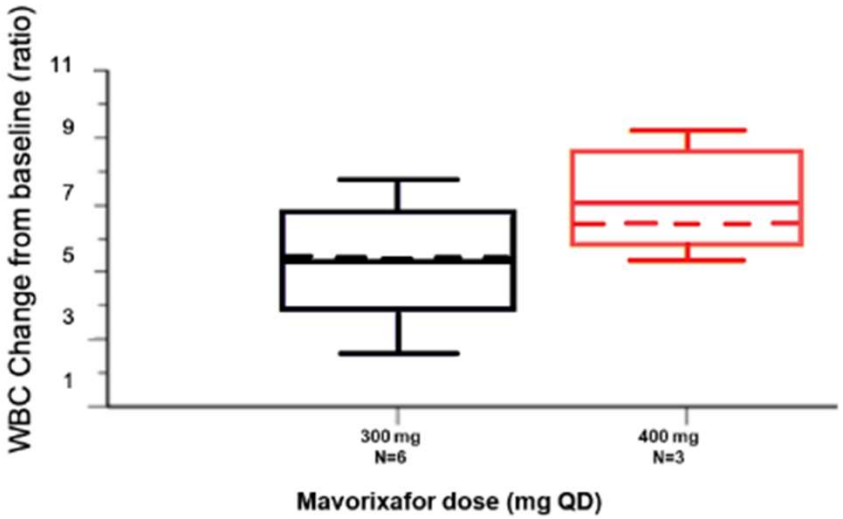
- Mavorixafor 400 mg orally once daily was well tolerated for >2 years without attributable serious AEs
- Durable, dose-dependent increases of WBC, ANC, and ALC counts
- TAT_{ANC} is an objective and consistent biomarker of clinical response to CXCR4 antagonist therapy
  - Measures reduction in duration of severe neutropenia
  - Primary endpoint in ongoing Phase 3 global clinical trial

Demonstrated increase in TAT_{ANC} at least 4.5-fold versus lower doses



At 300/400 QD Doses: Mean TAT_{ANC} was 12.6 hours

Increased total white blood cell counts



400 mg QD: largest WBC increase vs. baseline

Data from X4 poster presentation at 2020 EHA

WHIM Phase 2 Trial: Open-Label Extension
Mavorixafor Successfully Addressed All 3 Unmet Needs

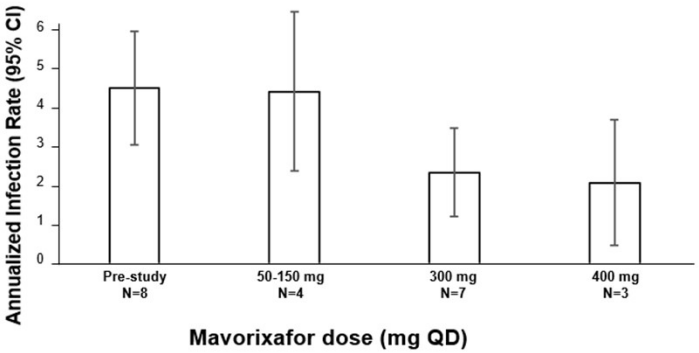
Increases of WBC, ANC, and ALC led to clinical benefits

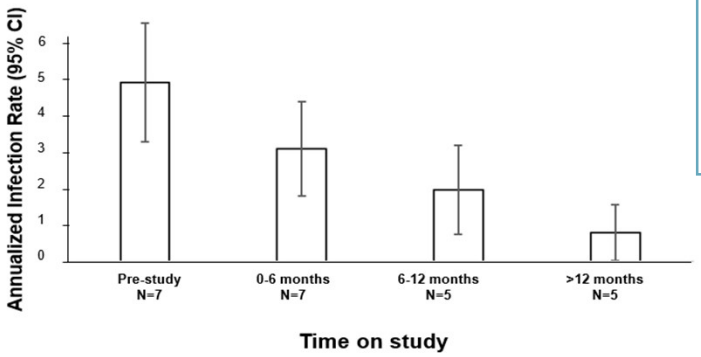
INFECTION RATES

- Infection rates decreased from 4.63 in the 12 months prior to the trial to 2.14 (a 54% reduction) at 400 mg
- Deepening reductions in infection rates with time

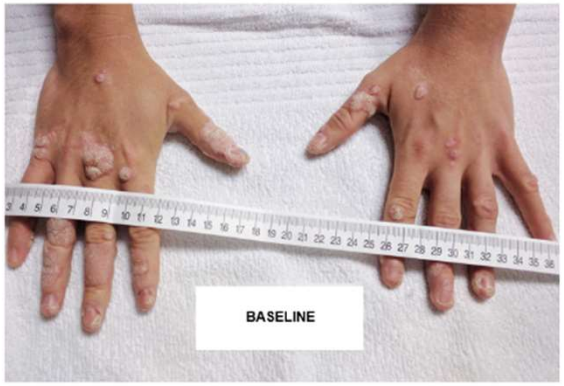
WART BURDEN

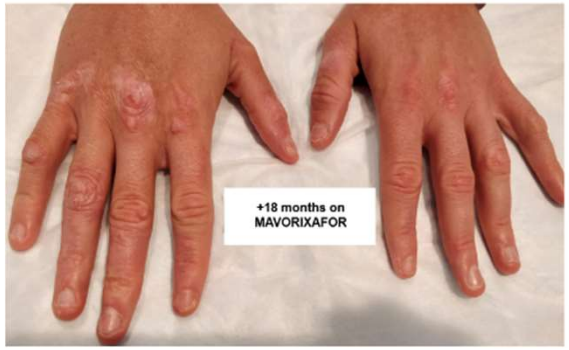
- 75% reduction in the number of warts
- Baseline vs 18 months, following 14 months on 400 mg mavorixafor





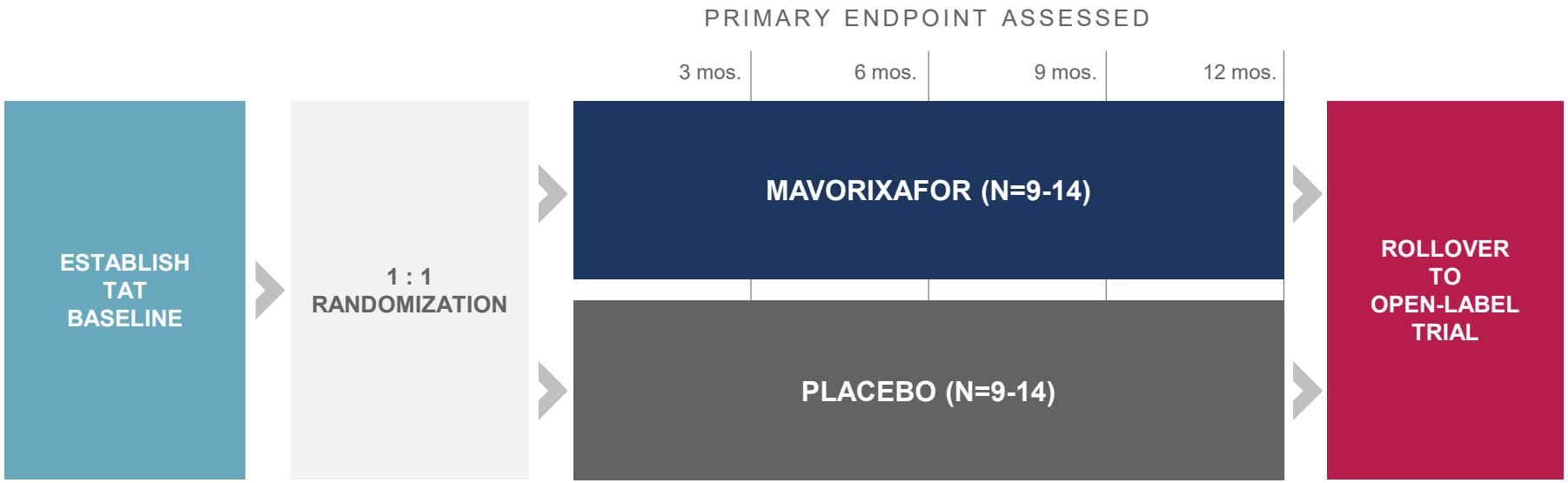
Two key secondary endpoints in ongoing pivotal Phase 3 trial





Data from X4 poster presentation at 2020 EHA

Global Registrational Phase 3 Trial in WHIM Syndrome



- **Primary Endpoint:** Biomarker of neutrophil count time above threshold (TAT) where the threshold is defined as 500 cells/uL; average of four assessment timepoints
- **Secondary Endpoints:** Infection rates and wart burden assessments
- **Dosing:** 400mg QD in patients 12 years of age or older (200mg QD for age 12-18 if weight is <50kg)
- **Enrollment:** 23 subjects enrolled (as of 8/1/2021); enrollment to complete in 3Q 2021
- **Phase 3 Top-line Data:** expected in 4Q 2022

2019 Market Research: Robust Study of WHIM Prevalence

1

Broad Quantitative Survey to Identify WHIM Patients

- Panel of ~56,000 Doctors (blinded, unbiased request to participate)
- >900 Doctors Completed Survey
- 11 Specialties Included

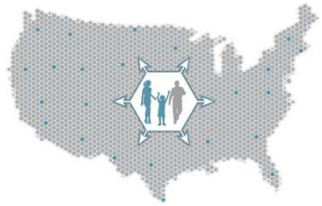
~8% reported having one or more WHIM patients

2

Telephone Interviews to Verify WHIM Diagnosis

- Conversations with 43% of doctors who reported diagnosing patients
- In-depth discussion of patient charts
- Required genetic diagnosis, biopsy, or clinical confirmation
- 33% verification rate rules out mis-reported patients

3



U.S. Prevalence Calculation

- Verified WHIM patients projected to universe of specialists
- Adjustment for duplication of patients across specialties

~1,000 – 1,300
Diagnosed WHIM Patients in the U.S.

Source: Qessential market research, 2019

2020 Market Research: Patients with the "Face" of WHIM Identified in Database Using AI

Face of WHIM → **Artificial Intelligence (AI)** → **Match to ~300M Lives**

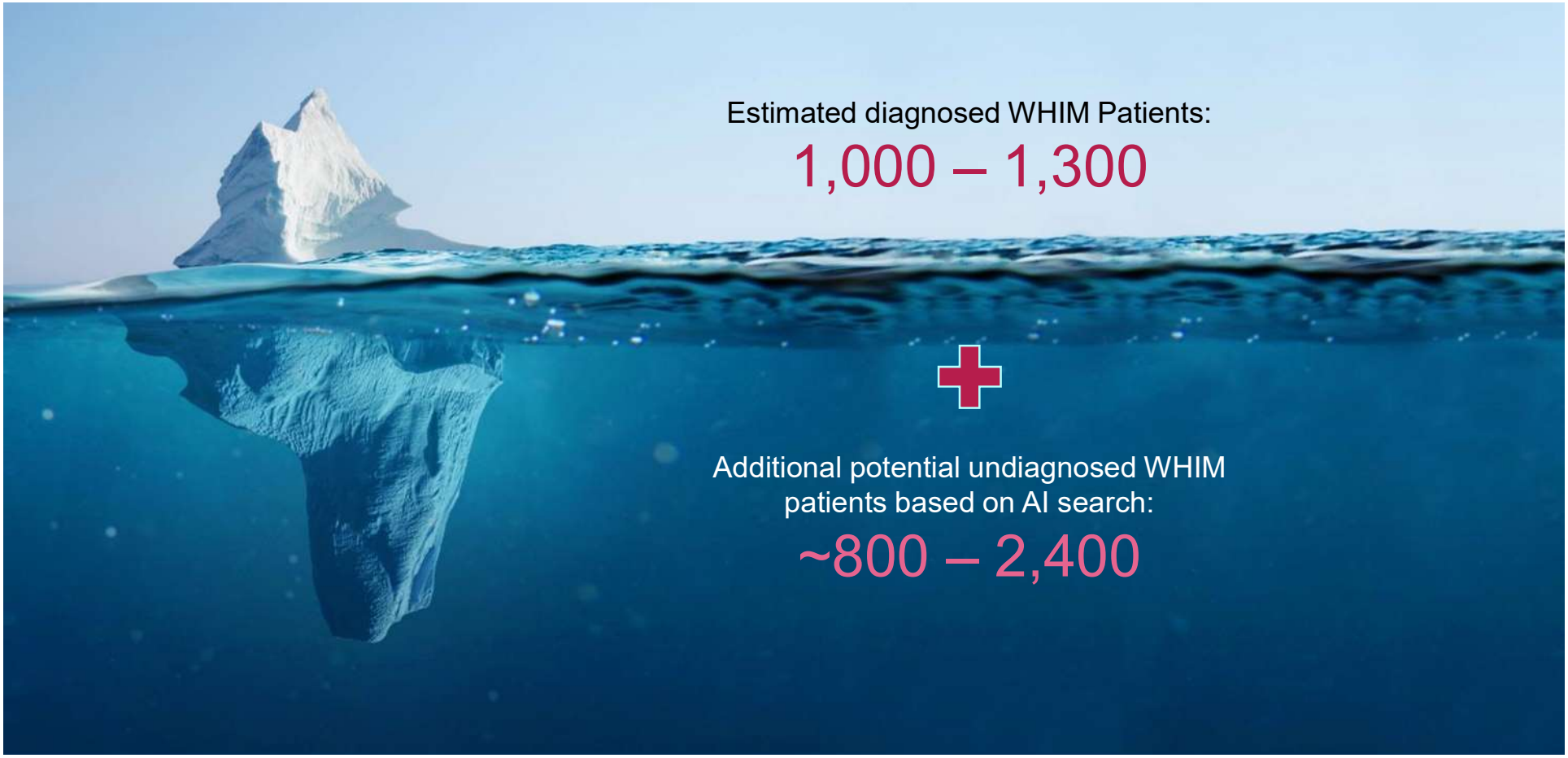
Conditions ***Required*** **for Inclusion**	Lower Estimate	Higher Estimate
Warts of Skin Condition	✓	✓
Hypogammaglobulinemia	✓	
Hypogammaglobulinemia *OR* Immunodeficiency Markers		✓
Respiratory Infection or Pneumonia	✓	✓

Additional ~800 to 2,400 potential WHIM patients

(no overlap with diagnosed patients projected from market research survey)

Source: IPM.ai artificial intelligence study, 2020

WHIM Prevalence of 1,000-3,700 in the U.S. Range Includes Potential Undiagnosed WHIM Patients



Source: Qessential market research, 2019 and IPM.ai artificial intelligence study, 2020

About Waldenström's Macroglobulinemia (WM)

A rare B-cell lymphoproliferative disorder of the bone marrow; a non-Hodgkin's lymphoma most often (>90%) caused by mutations in the MYD88 gene, which is involved in the body's innate immune response system

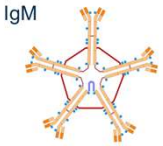
~8-10

year survival rate post diagnosis[1,2]

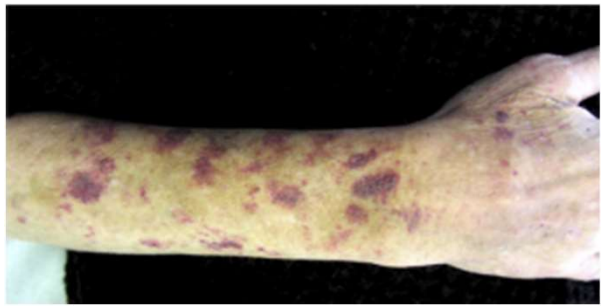
Limited Current Treatments

- Ibrutinib (BTKi)
- Chemo (bendamustine, R-CHOP)
- Rituximab
- Combinations and others

Signs & symptoms



- **Elevated IgM**
- Hyperviscosity syndrome
- Cryoglobulinemia – IgM clumping
- Pancytopenia, anemia
- Peripheral neuropathy
- Fever, night sweats, weight loss, fatigue



30-40%

Patients with identified WHIM-like CXCR4 mutations in addition to MYD88 mutation

Double-mutation

Waldenström's patients in the U.S. and EU[1]

~4,000-5,000

Mean progression-free survival (PFS) in CXCR4-mutation Waldenström's patients versus CXCR4-wild type[3]

<50%

1. Sekhar J, et.al., Waldenström macroglobulinemia: a Surveillance, Epidemiology, and End Results database review from 1988 to 2005. *Leuk Lymphoma* 2012;53(8):1625-1626;
2. https://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=33226 3. Treon *et al,* EHA 2018

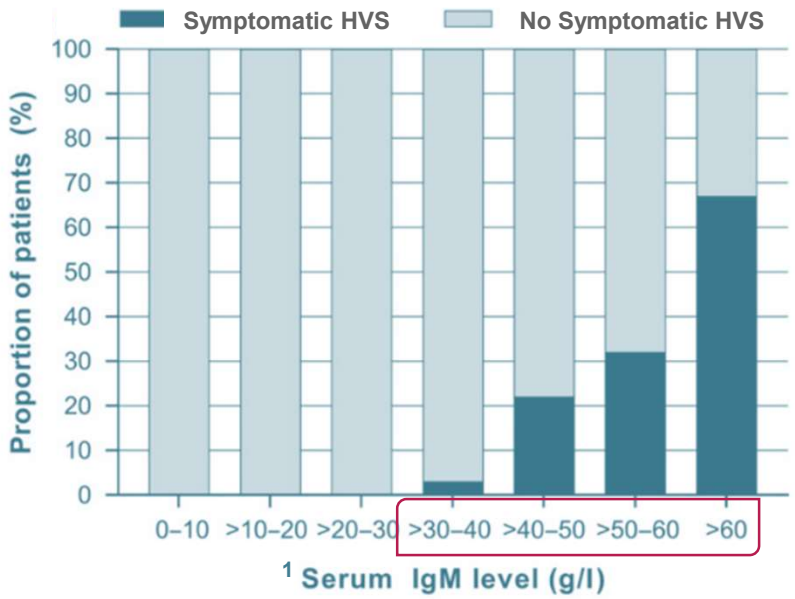
Impact of CXCR4 Mutations: More Severe Disease, Higher Risks, Greater Need for Treatment

	CXCR4 mutation	CXCR4 wild type
Serum IgM level at diagnosis*	+++	+
Risk of hyperviscosity (HVS)	+++	+
Bone marrow involvement	++	+
Lymphadenopathy	+	+++
Splenomegaly	+	+
Serum beta-2-macroglobulin	+	++
Thrombocytopenia	++	+
Leukopenia	+	+
Anemia	+	+
Acquired von Willebrand disease	+++	+
Time to therapy initiation	Shorter	Longer
# of WM patients (13,000)	***4,000-5,000***	***8,000-9,000***

**EXPERT REVIEW OF HEMATOLOGY 2019, VOL. 12, NO. 10, 873–881 https://doi.org/10.1080/17474086.2019.1649132*

Sekhar J, et.al.. Waldenström macroglobulinemia: a Surveillance, Epidemiology, and End Results database review from 1988 to 2005. *Leuk Lymphoma* 2012;53(8):1625-1626; https://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=33226

Patients with Higher IgM Have Symptomatic Hyperviscosity in Greater Proportion



CXCR4 Mutants Have Higher Serum IgM At Diagnosis[2]

Living with HVS: Clinical Perspectives

- Signs and symptoms: dizziness, vertigo, visual impairment, headache, coma, and seizures
- Retinopathy, hearing loss, cutaneous and mucosal bleeding,
- Irreversible neurological impairment
- Plasma volume expansion

1. *British Journal of Haematology*, 177, 717–725. 2017
2. *Journal Clinical Oncol* 36: 2755-2761. 2018

Waldenström's Phase 1b Trial Ongoing - Focus on Double-Mutation Population

Inclusion: Patients with *MYD88 + CXCR4* mutations who are naïve to ibrutinib

Design: Multi-national Phase 1b trial of mavorixafor in combination with ibrutinib (n=12 to 18)

- **Intrapatient dose-escalation:** cycles of 200 mg, 400 mg, and 600 mg QD
- **3 cohorts supporting dose selection of mavorixafor:**
  - Cohorts A & B: 6 patients enrolled in each
  - Cohort C (expansion): potential for additional 6 patients dosed up to 600 mg (enrolling)
- **Endpoints**
  - Safety, PK/PD
  - Assessments of serum IgM levels, hemoglobin, and clinical response

June 11, 2021: announced positive safety and initial efficacy data at low/mid doses

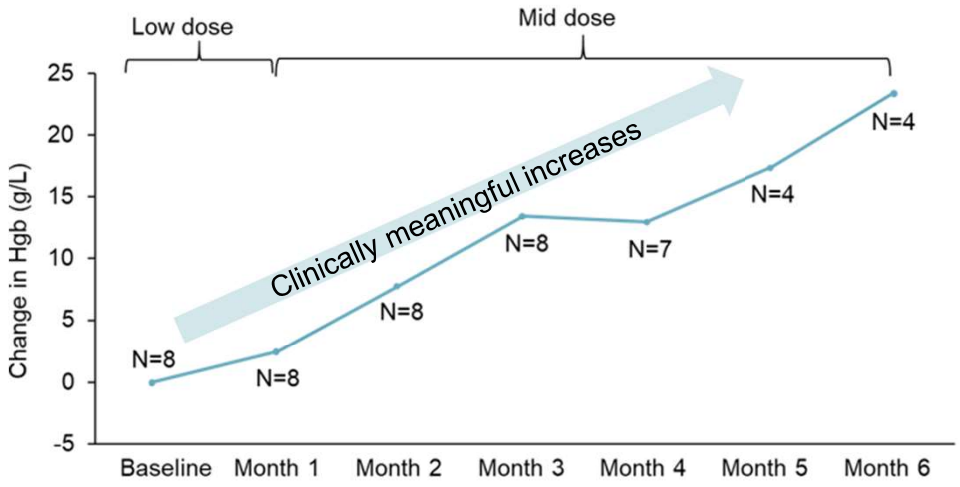
4Q21: Anticipate additional data at all doses to inform dose selection for potential further study

LEUKEMIA & LYMPHOMA SOCIETY®

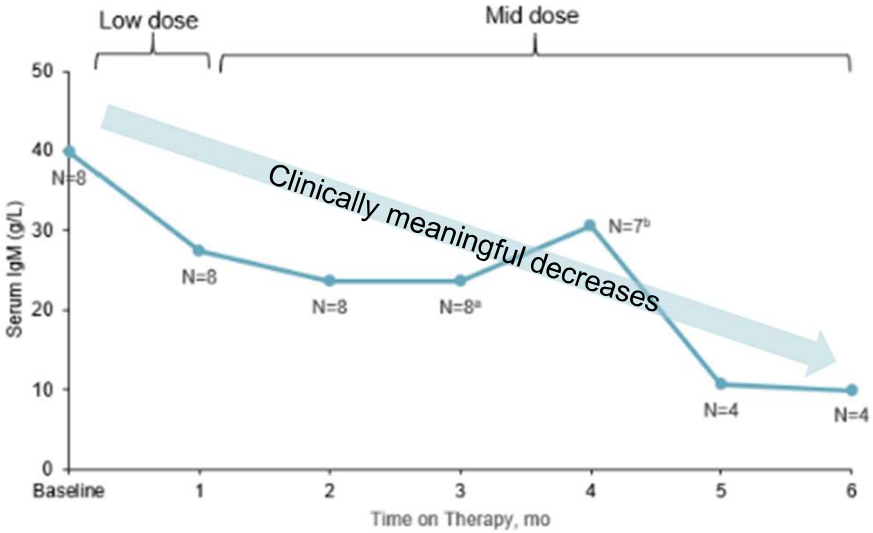
- Strategic collaboration with Leukemia & Lymphoma Society (LLS)
- Selected for LLS' Therapy Acceleration Program

EHA 2021: Mavorixafor + Ibrutinib Results in Clinically Meaningful Changes in IgM and Hemoglobin

Median Change from Baseline
Hemoglobin



Median Serum Levels
IgM



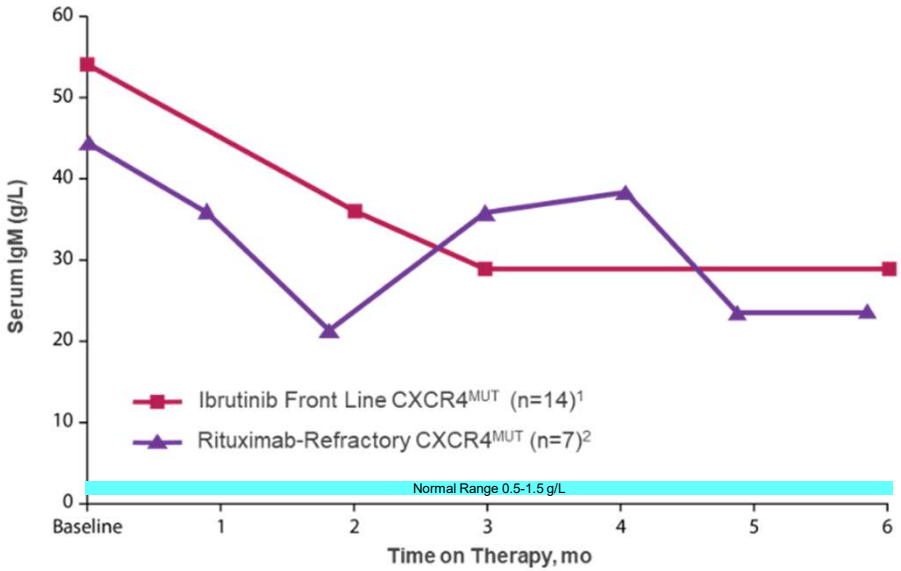
At 6 months:
- Hemoglobin levels approached normal levels over time
- Key biomarker for resolution of anemia/fatigue and bone marrow health
- ***Suggests reduction of cancer burden in the bone marrow***

At 6 months:
- 2 of 4 patients had >50% reduction from baseline
- 1 of 4 patients had absolute IgM levels within normal range
- ***Compares favorably (~2X) to historical data with ibrutinib alone***

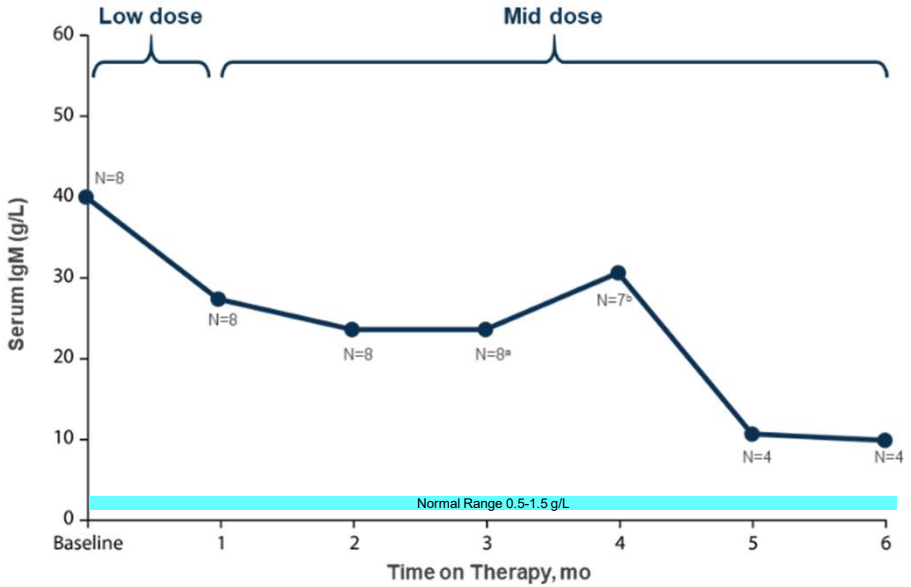
[a] IgM data of Patient 105-001 collected on May 10, 2021 were used to ensure 3 months' follow-up time.
[b] Participant 106-001 study treatment withheld due to an AE the week prior to month 4 IgM sample collection.

EHA 2021: Results Suggest More Rapid and Robust Response with Combo vs. Ibrutinib Monotherapy

Ibrutinib Monotherapy Median Serum IgM Levels



Mavorixafor + Ibrutinib Median Serum IgM Levels



1. Treon S., et. al *JCO* 2018 DOI: https://doi.org/10.1200/JCO.2018. 78.6426
2. Dimopoulous et al, IWWM9 Meeting, 2016, *Lancet Oncology*, 2017.

[a] IgM data of Patient 105-001 collected on May 10, 2021 were used to ensure 3 months' follow-up time.

[b] Participant 106-001 study treatment withheld due to an AE the week prior to month 4 IgM sample collection.

High Dose/Extended Dosing Is Ongoing In Phase 1b: Potential For Even Deeper, More Durable Responses

EHA 2021: Key Takeaways from Waldenström's Phase 1b Data

- **Preliminary data suggest mavorixafor + ibrutinib effects clinically meaningful benefits in reduction in serum IgM and increases in hemoglobin**
- **At 6 months**, mavorixafor plus ibrutinib showed greater decreases in IgM versus previously published ibrutinib monotherapy studies at the same time point in double mutation patients:

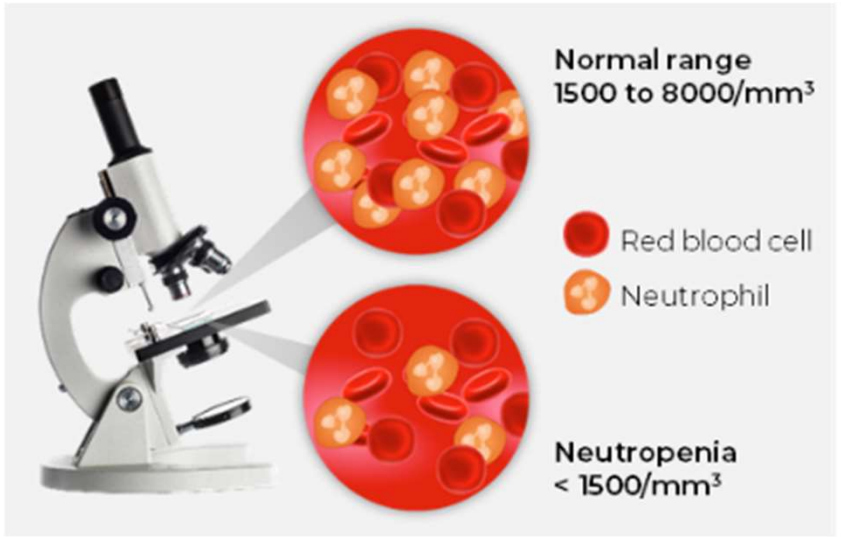
	Ibrutinib Monotherapy	Mavorixafor + Ibrutinib
% IgM Drop from baseline	38-45%	**60-75% (4-8 patient baseline)**
≥50% Reduction	28-38%	**50% (2 of 4 patients)**

- One patient had normalized IgM by 6 months on combination treatment
  - Prior published studies of ibrutinib monotherapy in double-mutation patients show very infrequent (≤10%) reductions of this magnitude (≥ 90%) and at a median time of ~11 months on treatment
- Patient enrollment and dose escalation to the highest (600 mg QD) level continue

MAVORIXAFOR LABEL EXPANSION OPPORTUNITIES

About Severe Congenital Neutropenia (SCN)

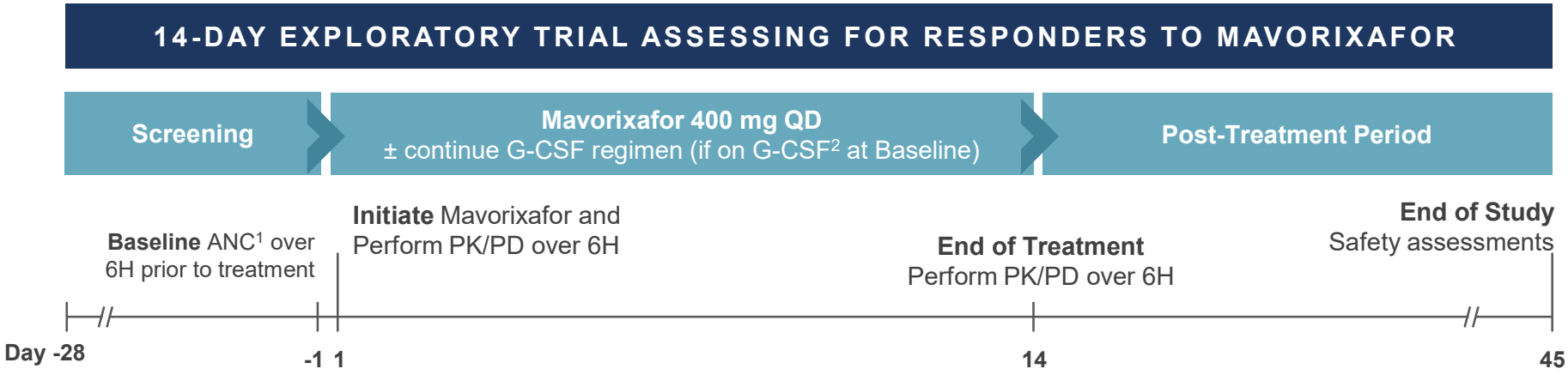
- Rare blood disorder
- Characterized by abnormally low levels of certain white blood cells (neutrophils <1,500 cell/ul)[1]
  - From birth, fevers, severe bacterial infections
    (at times life-threatening), pneumonias, oral ulcers, premature tooth loss
  - Treatment options: antibiotics and G-CSF
- Prevalence estimated 2,000-3,000 patients (US & EU)2
- Genetic drivers:
  - May be inherited as either an autosomal dominant or an autosomal recessive genetic trait
  - Many cases of SCN are the result of spontaneous, random mutations




Oral ulcers

1. https://rarediseases.org/rare-diseases/severe-chronic-neutropenia/ 2. https://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=en&Expert=42738

Phase 1b SCN Clinical Trial Underway:
Focus on Neutrophil Response to Mavorixafor



Inclusion: Up to 45 patients total (30 SCN, 15 exploratory sub-populations)

Endpoints: Safety and tolerability, percentage of patients with ANC >50% baseline

Goal: Achieve proof of concept to support FDA interactions regarding proposed registrational trial

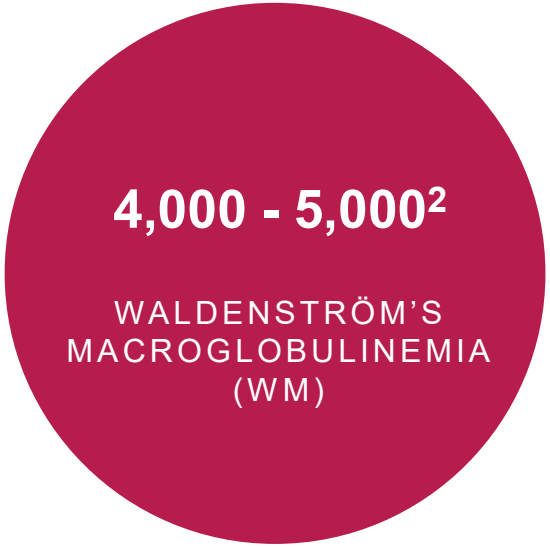
Initial Clinical Data expected in 4Q21

- These and additional data expected to support exploration of broader use of mavorixafor across neutropenia landscape

[1] ANC = absolute neutrophil count
[2] G-CSF = granulocyte-colony stimulating factor
Study NCT04154488

Targeting the CXCR4 Pathway Positions X4 to Target >10,000 Patients with Rare Diseases

- Strong Phase 2 results de-risk ongoing Phase 3
- Favorable Breakthrough Therapy Designation



- Near-term inflection point
- Large, well defined market opportunity

- Neutropenia expansion opportunities

[1] IPM.ai 2020; study used artificial intelligence to identify likely WHIM patients from an insurance claims database of ~300M US lives; represents US only
[2] Sekhar J, et.al.. Waldenström macroglobulinemia: a Surveillance, Epidemiology, and End Results database review from 1988 to 2005. Leuk Lymphoma 2012;53(8):1625-1626; https://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=33226 · Represents CXCR4-mutant patients; 30% to 40% of total WM estimate of 13,000 patients in the U.S. and EU;
[3] Estimated U.S. and EU based on prevalence of 5 per million: Dale et al (2017) Cur. Op. Hematology. 24 (1): 46-53

Efforts to Maximize Mavorixafor Potential

Sponsored Genetic Testing

MSL Deployment to engage concentrated, targeted physician population

Disease Education on WHIM and Waldenstrom's

Ongoing Collaboration with key Patient Advocacy Groups

Catalyst-Rich Period Anticipated Next 12-18 Months

- **Mavorixafor Pivotal Phase 3 Trial in WHIM Syndrome**
  - Trial enrollment completing in 3Q 2021
  - Updates on WHIM patient prevalence expected in 4Q 2021
  - Longer-term data from ongoing Phase 2 open-label extension study expected in 4Q 2021
- **Mavorixafor Waldenström's Phase 1b Clinical Trial**
  - Cohorts A & B fully enrolled; Cohort C enrolling; dose escalation continues
  - Safety, dose, and clinical response being assessed
  - Additional data expected in 4Q 2021
- **Additional Program Updates**
  - Mavorixafor SCN Phase 1b trial – initial data expected in 4Q 2021
  - Research publications and Pipeline advances anticipated
- **Future Expected Milestones – 2022 and Beyond**
  - Pipeline candidate IND filing in 2022
  - WHIM Phase 3 top-line data in 4Q 2022
  - Potential study of mavorixafor in additional neutropenia indications and more broadly in cellular immunodeficiencies
  - Potential mavorixafor WHIM NDA filing in 1Q 2023

Selected Financial Highlights

$96M[1]

Cash Expected to Fund Operations into Q4 2022[2]

Share and Warrant Information:

- **26.6M shares outstanding**
  (24.8M common shares and 1.8M pre-funded warrants)
- **5.4M class B warrants**
  (expiry 30 days post WHIM P3 data)
- **3.9M class A warrants**
  (2024 expiry)

BIOTECH-FOCUSED INSTITUTIONAL SHAREHOLDER BASE

ANALYST COVERAGE

Canaccord Genuity
COWEN
STIFEL
OPPENHEIMER
B RILEY FBR
ROTH Capital Partners
HCW H.C. WAINWRIGHT & CO.
BROOKLINE CAPITAL MARKETS

[1] As of June 30, 2021, as reported in Company's form 10Q filed with the SEC on August 3, 2021.
[2] 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 April 1, 2022. Based on our current financial projections, which do not include additional funding from third parties or potential amendments to the Hercules agreement, we expect that we would be in violation of this covenant in the second quarter of 2022, which could result in accelerated principal and interest payments due that could shorten our cash runway.

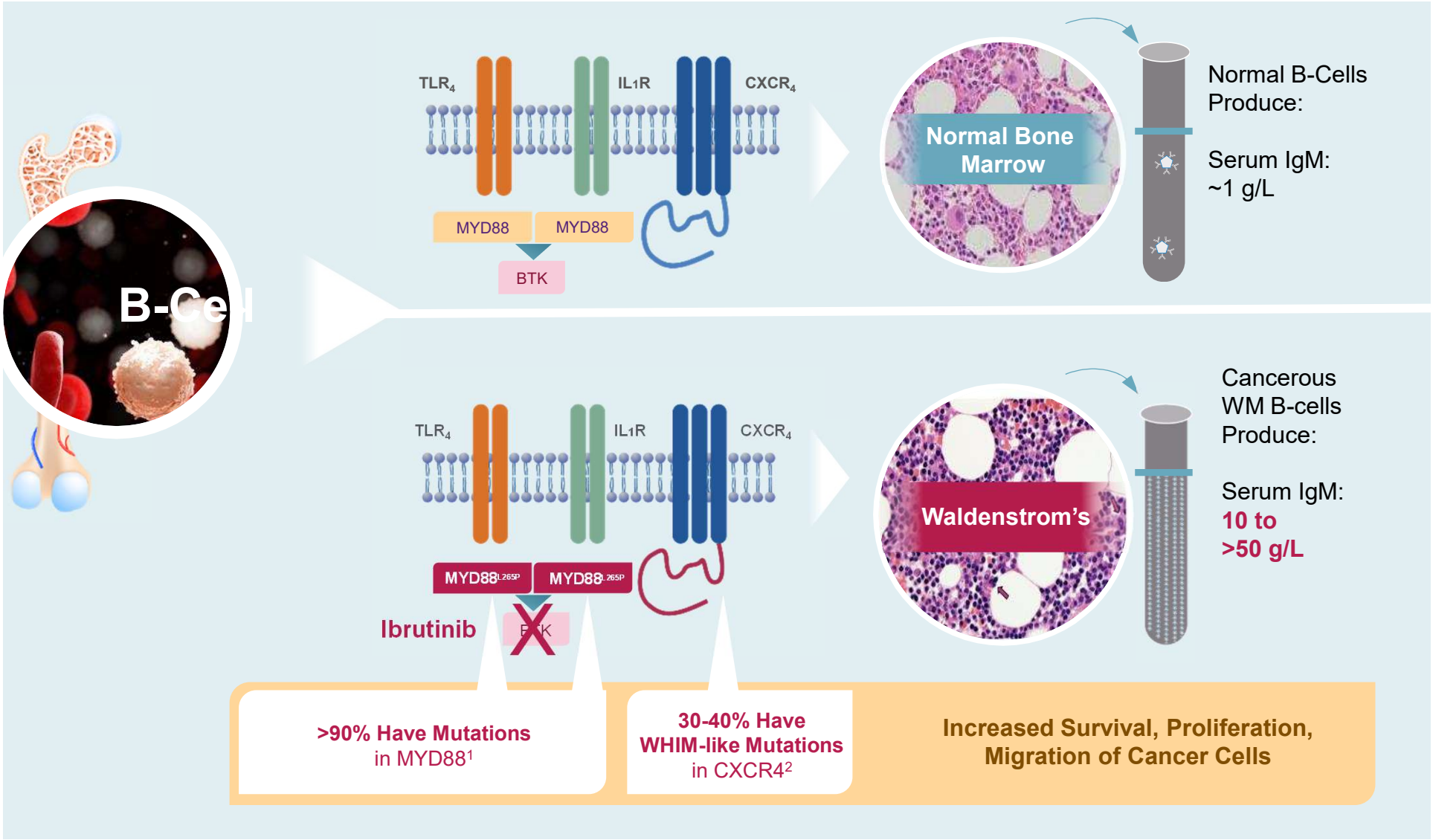
61 North Beacon Street
4th Floor
Boston, MA 02134

www.x4pharma.com

PHARMACEUTICALS

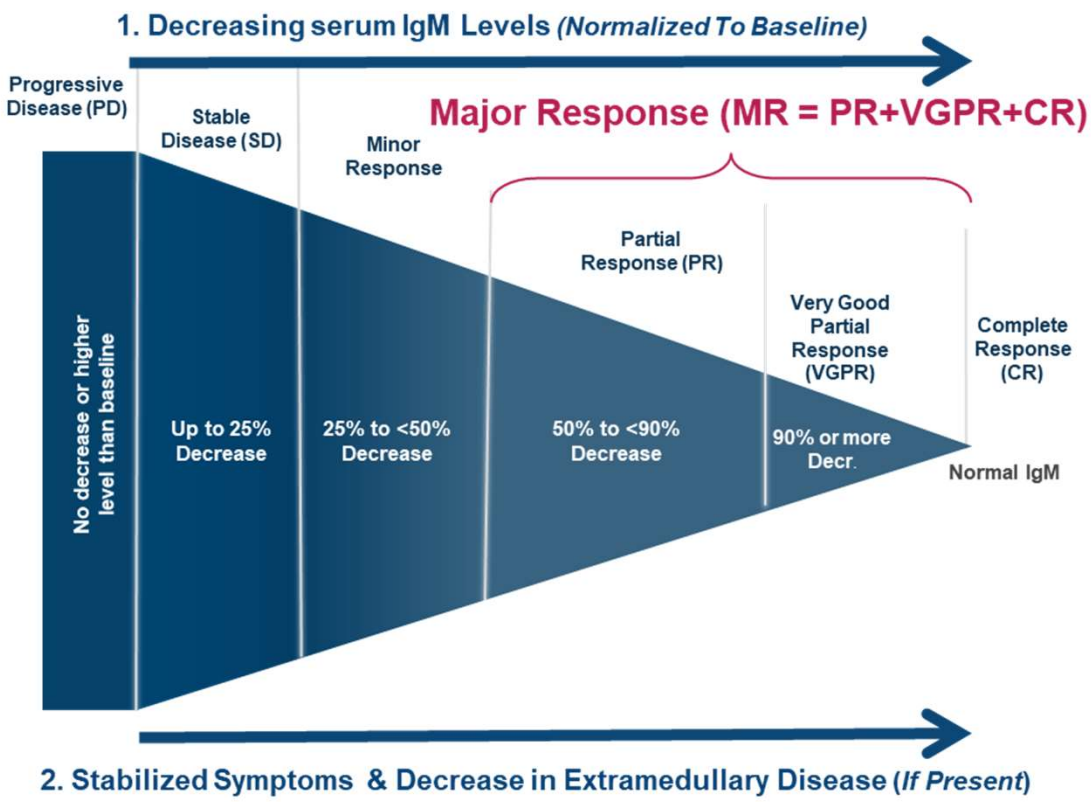
APPENDIX:
Additional Waldenström's Data (as of April 2021)

INCREASE IN CANCEROUS B-CELLS AND SERUM IGM IN WM: DRIVEN BY GENETIC MUTATIONS IN MYD88 & CXCR4



1. Treon et al. 2012. *New England Journal of Medicine,* 367, 826-833; 2. Hunter et al. 2014. *Blood*, 123, 1637–1646.

CONNECTING IGM REDUCTIONS TO CLINICAL RESPONSE IN WALDENSTRÖM'S



Major Response Criteria

- **>50% reduction in normalized serum IgM**
  - using "best response"
- No new/worsening of symptoms
- Decrease (via imaging) lymphadenopathy / splenomegaly disease if present at baseline

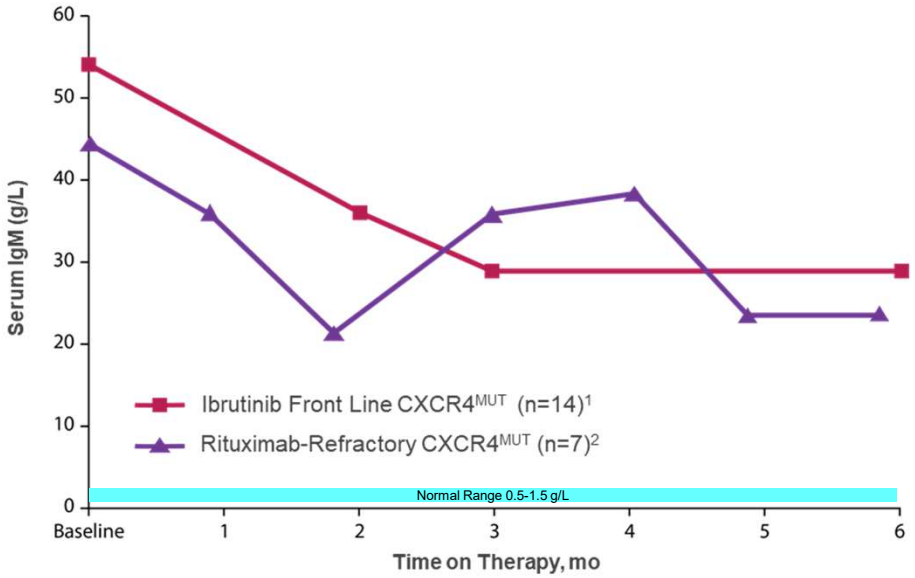
Note: Bone marrow normalization (via BM aspirate) is required only for Complete Response evaluation

Response Assessments Require Longer-Term Study

- CT/MRI images every 3 or 6 months
- Bone marrow aspirates at 6 or 12 months or when clinically indicated

DOUBLE-MUTATION WM PATIENTS RESPOND POORLY TO BTK INHIBITION

Ibrutinib Monotherapy Median Serum IgM Levels



Effect of Ibrutinib Monotherapy on IgM Levels

	PREVIOUSLY TREATED[1,2]	FRONT LINE[3]
Time to >50% Response (mo)	6.0	7.3
Percent at >50% reduction at 6 months	38.1%	28.6%

1, 2. Treon, NEJM, 2015, Treon, EHA, 2018; 3. Treon, *JCO*, 2018.

1. Treon S., et. al *JCO* 2018 DOI: https://doi.org/10.1200/JCO.2018. 78.6426
2. Dimopoulous et al, IWWM9 Meeting, 2016, *Lancet Oncology*, 2017.

PRELIMINARY WM PHASE 1B RESULTS: DEMOGRAPHICS

Table 1: Demographics, Clinical Characteristics and Mutational Status of All Patients

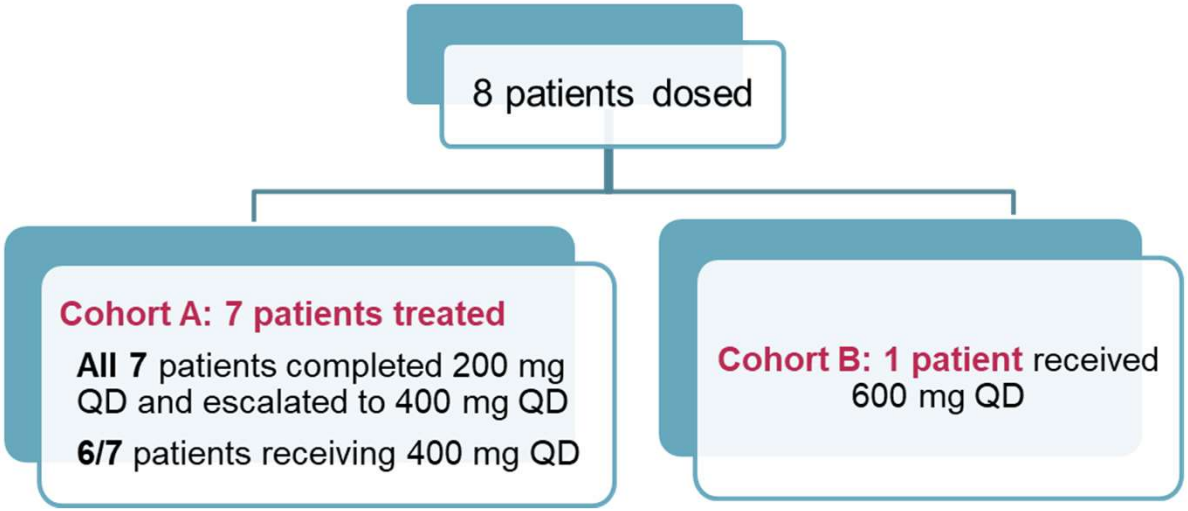
Characteristic	
Patients with both *MYD88* and *CXCR4* mutations, n (%)	8 (100)
Mean age (range), y	67 (38–80)
Male sex, n (%)	6 (75)
Mean disease duration (range), y	4.5 (0–11)
Mean prior lines of treatment, n (range)[a] Frontline therapy, n (%) Relapse/refractory therapy, n (%)	1 (0–3) 3 (37.5) 5 (62.5)
Median baseline IgM levels (range)[b], g/L	39.75 (11.88–58.50)
Median baseline hemoglobin levels (range)[c], g/L	110.5 (76–161)
Median baseline platelet levels (range)[d], 10^9/L	189 (108–453)
Patients with baseline extramedullary disease, n (%)	3 (38)
Patient baseline IPSS WM score, n	Low–2 Intermediate–3 High–3
CXCR4 mutational status Frameshift mutation, n (%) Nonsense mutation, n (%)	 4 (50) 4 (50)

IPSS, International Prognostic Scoring System
[a]3 patients were previously untreated.
[b]Normal range, 0.5–2 g/L.
[c]Normal range: male, 138–172 g/L; female, 121–151 g/L.
[d]Normal range, 150–400 10^9/L.

PRELIMINARY WM PHASE 1B RESULTS:
ENROLLMENT AND COHORTS

- 8 patients were enrolled in the study
- 4 patients treated for ≥6 28-day cycles
- Median duration of treatment = 156 days
- Cohort A fully enrolled, Cohort B enrolling

Figure 2. Patient Disposition



MAVORIXAFOR + IBRUTINIB WELL TOLERATED TO DATE

- No serious AEs were reporteda
- 77% of AEs were mild (Grade 1)
- 18 AEs were related to combination therapy, 13 were attributed to ibrutinib treatment only and 6 to mavorixafor use onlyb
  - AEs related to use of mavorixafor only occurred in 2 patients and were grade 1 or grade 2 and included nausea, acid reflux, constipation, elevated WBC count, and worsening pain/numbness in the shoulder/hands/wrists
- 3 DLT AEs were reported in 2 patients

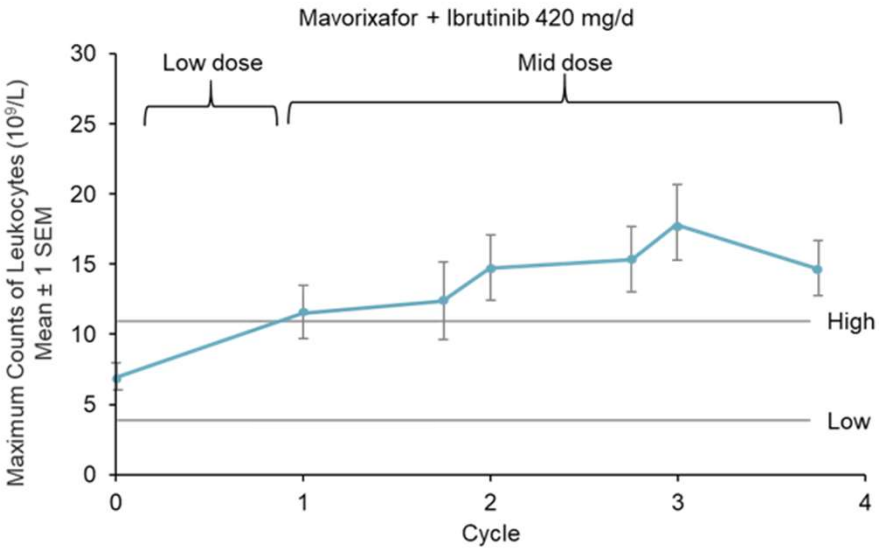
AE	Grade	Causality
Hypertension	3	Combination therapy
Worsening pain and numbness in right shoulder, bilateral hands/wrists[c]	2	Possibly mavorixafor
Worsening pain, numbness, and tingling in left hand and shoulder[d]	3	Ibrutinib

- Mavorixafor and ibrutinib exposures were consistent with previous single-agent studies
- No apparent drug–drug interactions between mavorixafor and ibrutinib were observed

AE, adverse events; CTCAE, Common Criteria for Adverse Events; DLT, dose-limiting toxicity.
[a]Interim early data analysis performed with data cutoff at April 15, 2021.
[b]Only AEs with a completed assessment for a causal relationship to the study drug(s) at the time of the data cutoff are included.
[c]Upon review with the investigator post the data cut, the AE does not meet DLT criteria per protocol and is pending removal of the DLT flag
[d]Upon review with the investigator post data cut, the AE does not meet Grade 3 CTCAE criteria and is pending downgrade to Grade 2 and removal of the DLT flag.

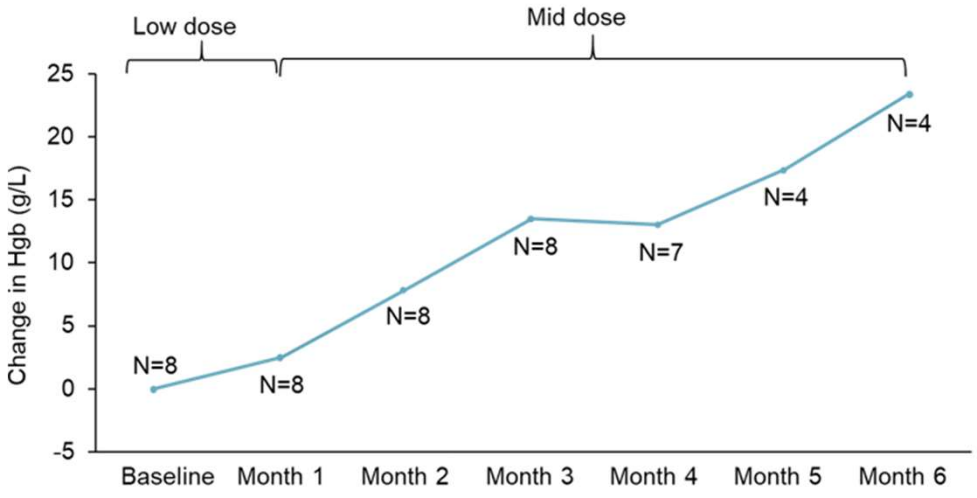
MAVORIXAFOR + IBRUTINIB PHASE 1B RESULTS

Mobilization of Leukocytes with Mavorixafor Exposure



- Mavorixafor exposures tracked with increases in key WBC counts in all patients
- Confirms target engagement and mavorixafor MOA
- Durable and sustained effect

Median Change from Baseline Hemoglobin



- Patients with pretreatment hemoglobin below normal had increases during treatment
- Median hemoglobin increased by >20 g/L for patients on treatment for 6 cycles (n=4)
- Hemoglobin levels approached normal levels
- Key biomarker for resolution of anemia/fatigue and bone marrow health

Interim early data analysis performed with data cutoff at April 15, 2021.