X4 PHARMACEUTICALS

INVESTOR PRESENTATION

APRIL 2021

FORWARD LOOKING STATEMENTS

The statements herein are subject to various risks and uncertainties. These risks and uncertainties include, without limitation, the risk that trials and studies may be delayed and may not have satisfactory outcomes; potential adverse effects arising from the testing or use of mavorixafor or other product candidates; the risk that costs required to develop mavorixafor or other product candidates or to expand our operations will be higher than anticipated; the ongoing direct and indirect effects of the ongoing COVID-19 pandemic on various aspects and stages of X4's clinical development process including the impact to expected site initiation, enrollment and participation in X4's clinical trials; and the risk that the PATH4WARD program and X4's relationship with Invitae will not be successful. Any forward-looking statements herein are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained herein, including, without limitation, the risks and uncertainties described in the section entitled "Risk Factors" in X4's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 19, 2021, and in other filings X4 makes with the SEC from time to time. X4 cautions investors not to place undue reliance on the forward-looking statements herein and undertakes no obligation to update the information contained in this presentation to reflect subsequently occurring events or circumstances.

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

OVERVIEW: BUILDING A GLOBAL RARE DISEASE COMPANY

- Leading discovery and development of novel therapies targeting diseases resulting from CXCR4 pathway dysfunction
- Novel therapeutics designed to improve immune cell trafficking
- Lead product candidate mavorixafor (X4P-001), first-in-class, oral, small molecule allosteric antagonist of chemokine receptor CXCR4
- Multiple clinical trials underway, including ongoing global registrational Phase 3 trial of mavorixafor in WHIM syndrome, a Phase 1b trial in Waldenström's macroglobulinemia and a Phase 1b trial in Severe Congenital Neutropenia
- Potential expansion opportunities across rare disease landscape
- Experienced leadership team in rare disease – includes several former members of Genzyme leadership team
- Headquarters in Boston, MA with R&D facility in Vienna, Austria

LEADERSHIP:
PROVEN TEAM WITH RARE DISEASE EXPERTISE

MANAGEMENT

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SHARIQ ALI, Ph.D.
VP of Medical Affairs
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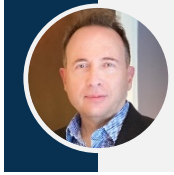

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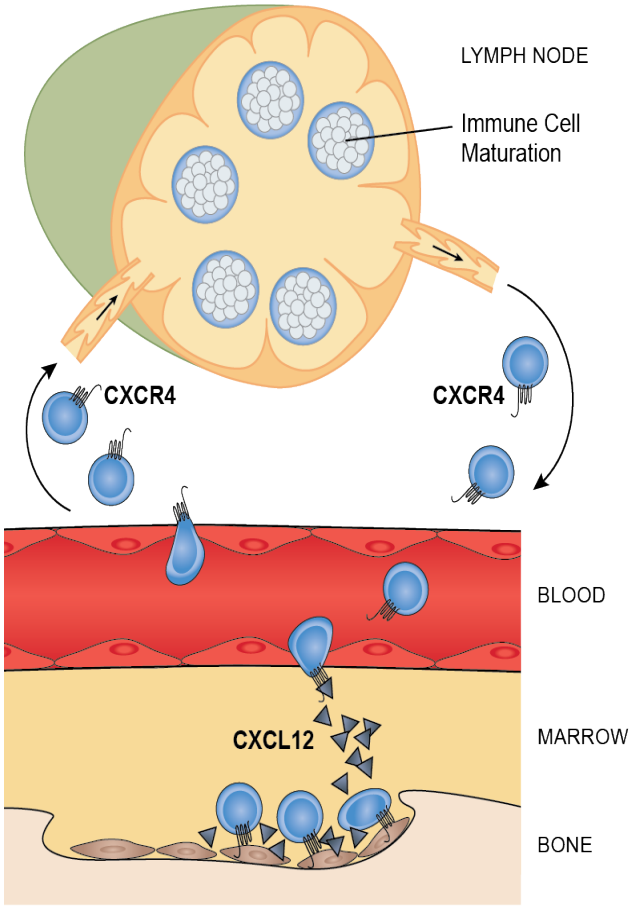
MAVORIXAFOR: TARGETED TREATMENT FOR DISEASES DRIVEN BY IMMUNE-CELL TRAFFICKING DEFICITS

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-suppression corrected by **blocking CXCR4 Signaling**

PIPELINE

PRECLINICAL PROGRAMS:
Additional primary immuno-deficiencies

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

OVERVIEW: MAVORIXAFOR

First-in-class CXCR4 antagonist

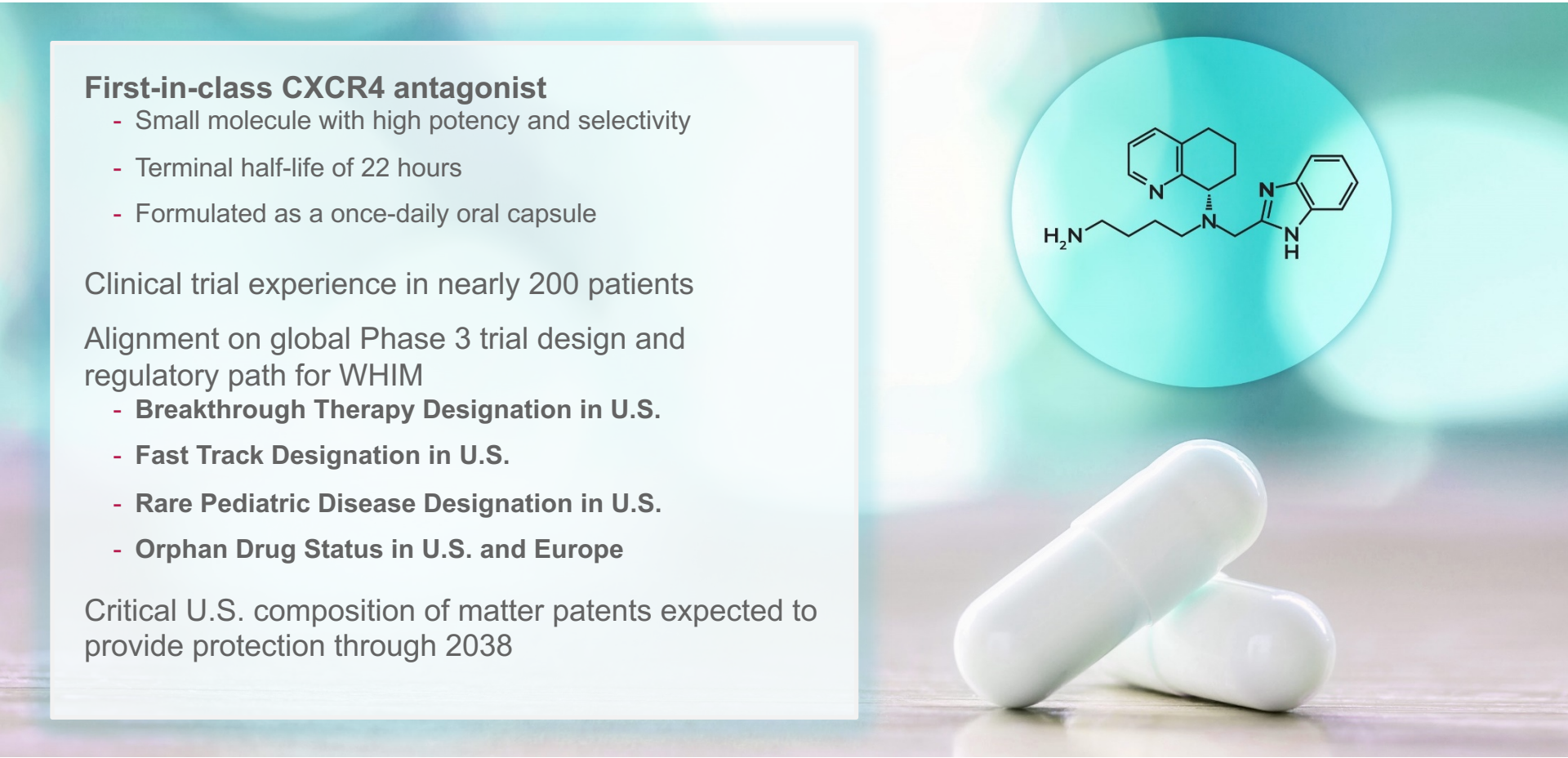
- Small molecule with high potency and selectivity
- Terminal half-life of 22 hours
- Formulated as a once-daily oral capsule

Clinical trial experience in nearly 200 patients

Alignment on global Phase 3 trial design and 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**

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



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	Glioblastoma multiforme (GBM)				
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

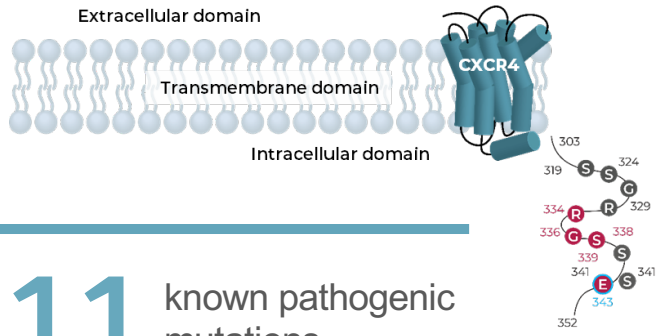
W **Warts** – can lead to HPV-related cancers

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," compromising immune cell trafficking and the ability to mount a healthy immune response



Up to

>3,500[1]

Potential U.S. WHIM patients

0

Approved targeted therapies

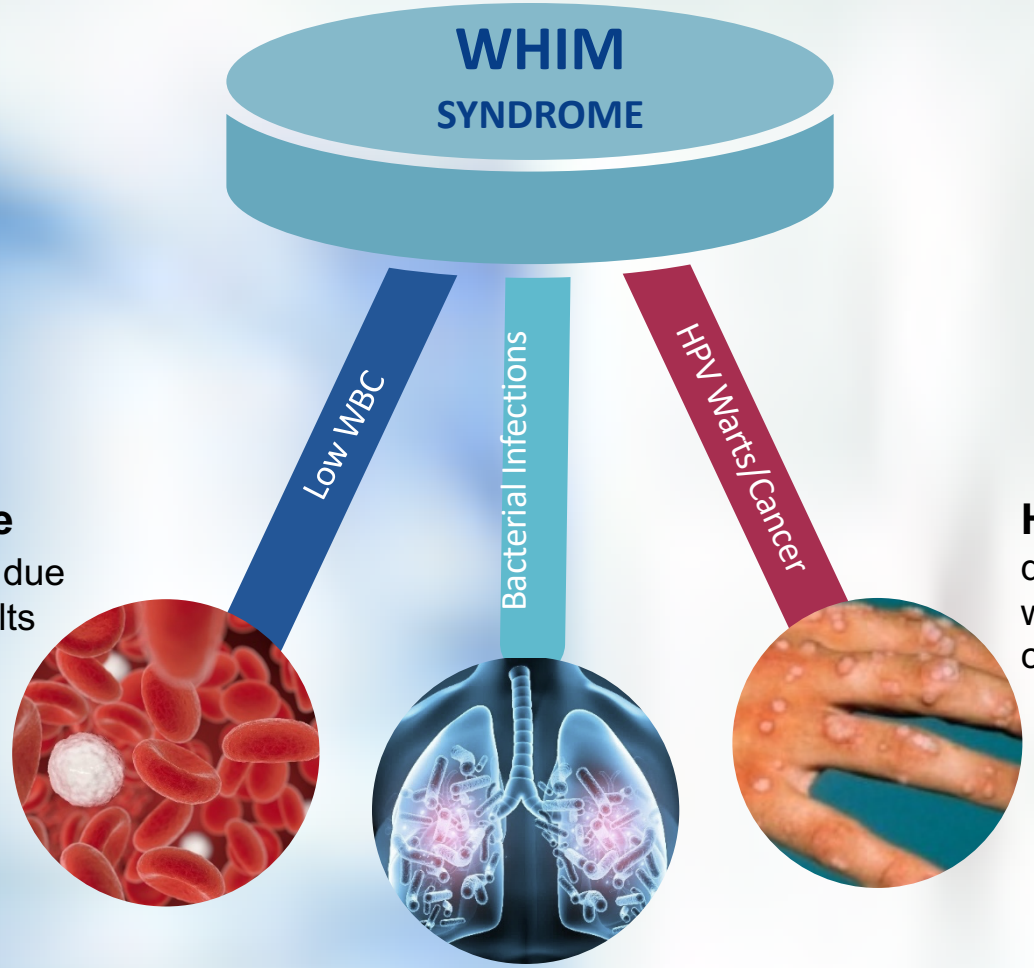
Symptomatic Rx; antibiotics, G-CSF, immunoglobulins

11 known pathogenic mutations

Genetic test to diagnose

1. IPM.ai 2020; study used artificial intelligence to identify likely WHIM patients from an insurance claims database of ~300M US persons

UNMET NEEDS IN WHIM



Critically low white blood cell counts, 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)

PHASE 2 TRIAL INFORMS PHASE 3 TRIAL

PHASE 2 TRIAL DESIGN

INCLUSION

- Neutrophil count: ANC ≤400/µL *and/or*
- Lymphocyte count: ALC ≤650/µL *or* both

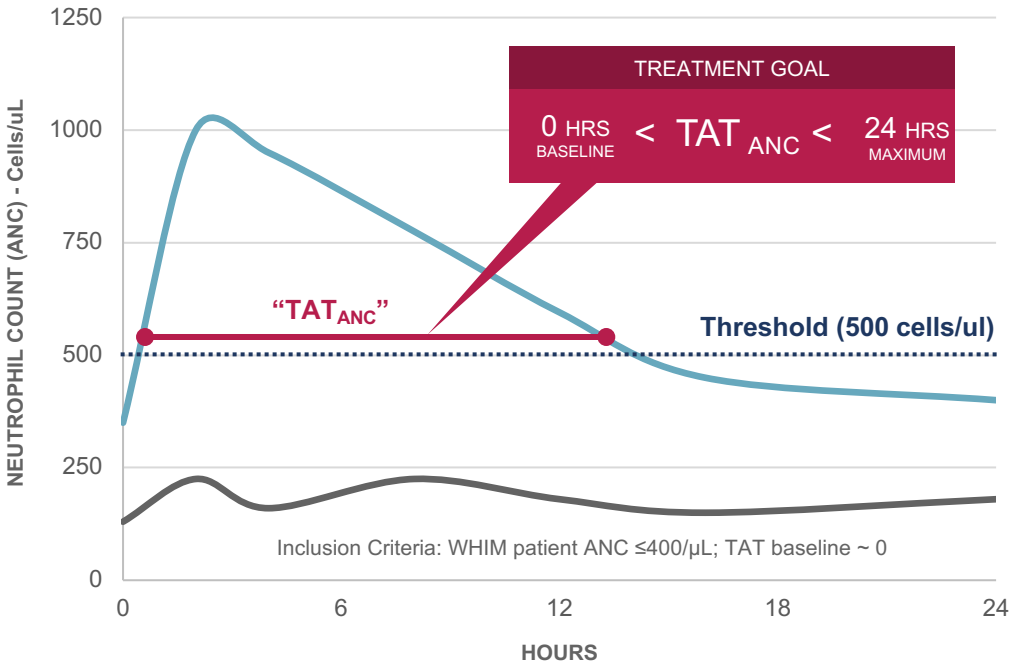
DOSE ESCALATION + OPEN-LABEL EXPANSION

- 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

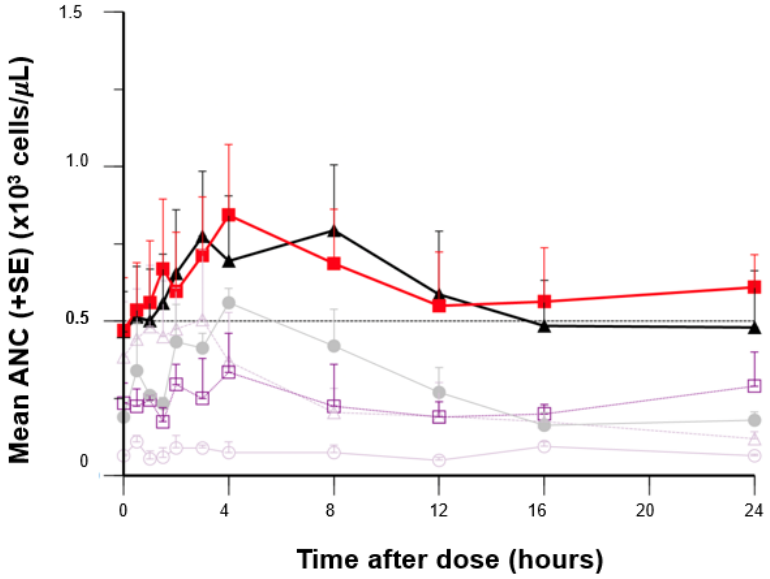


OBJECTIVE: INCREASE DAILY NEUTROPHIL COUNTS (ANC) ABOVE THRESHOLD AS MEASURED OVER 24 HOURS: TIME ABOVE THRESHOLD (TAT)

WHIM PHASE 2: OPEN-LABEL EXTENSION
SUCCESSFULLY ADDRESSED ALL 3 UNMET NEEDS

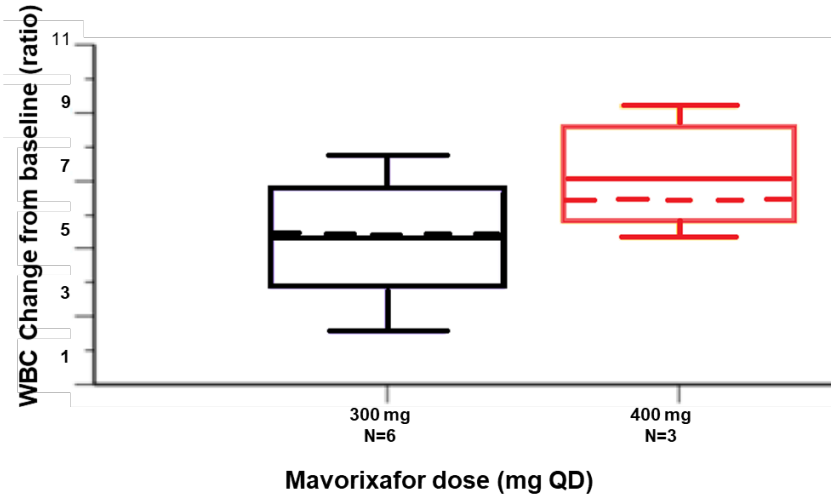
- Mavorixafor 400 mg orally once daily was well tolerated for >2 years without attributable serious AEs
- Durable, dose-dependent increases of WBC, ANC, and ALC counts
- TAT_{ANC} is an objective and consistent biomarker of clinical response to CXCR4 antagonist therapy
  - Primary endpoint in ongoing 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: OPEN-LABEL EXTENSION
SUCCESSFULLY ADDRESSED ALL 3 UNMET NEEDS

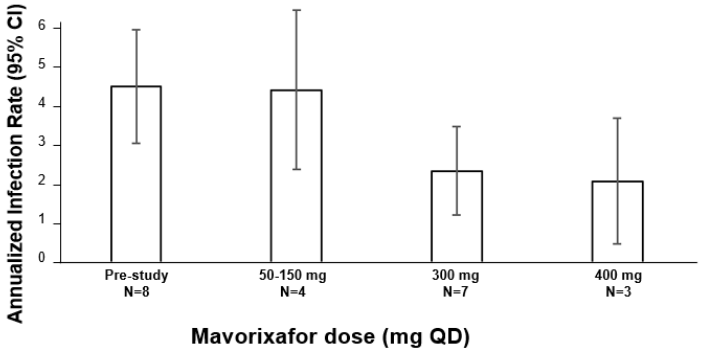
- Durable, dose-dependent increases of WBC, ANC, and ALC counts led to clinical benefits

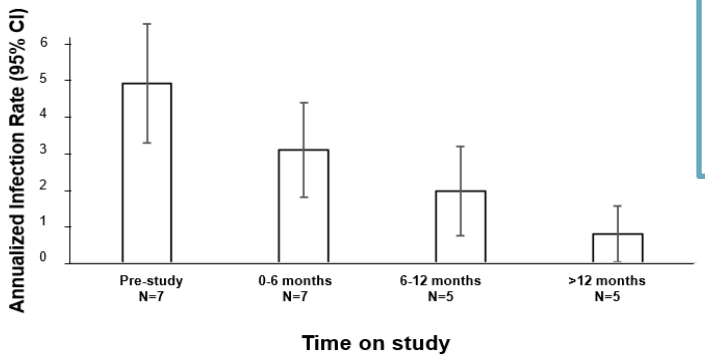
INFECTION RATES

- 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

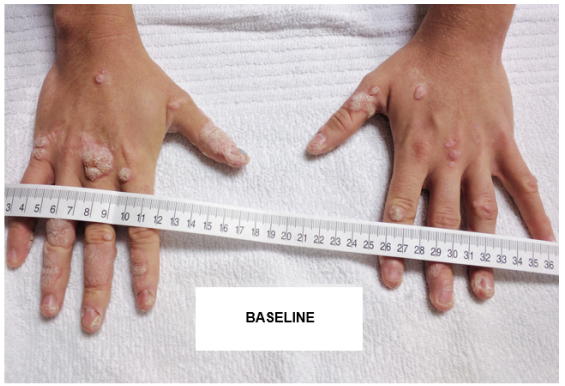
WART BURDEN

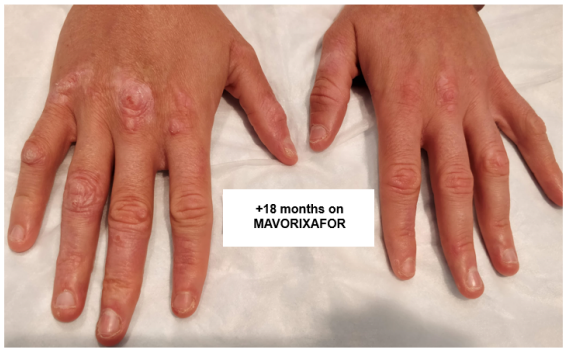
- Average 75% reduction in the number of warts
- Baseline vs. 18 months, following 14 months on 400 mg mavorixafor





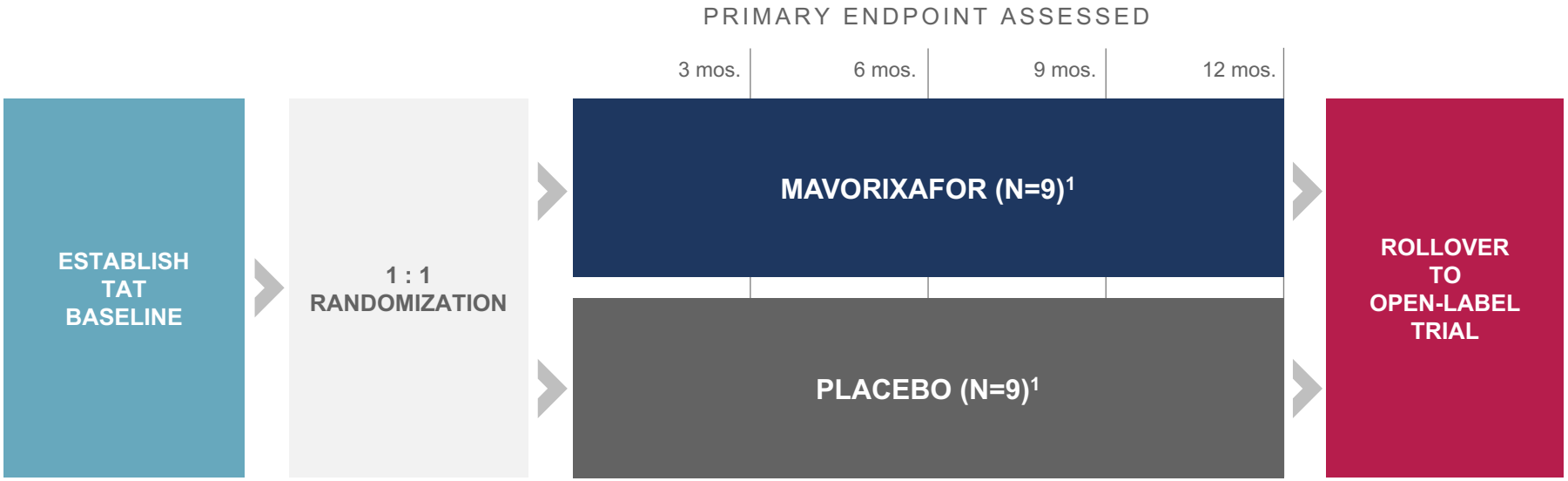
Two key secondary endpoints in ongoing pivotal Phase 3 trial





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
- **Enrollment:** Plan to enroll 18 to 28 subjects and activate approximately 20 to 25 sites globally
- **Phase 3 Top-line Data:** expected in 2022

1. Allowed to enroll up to 14 patients on drug and 14 patients on placebo

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

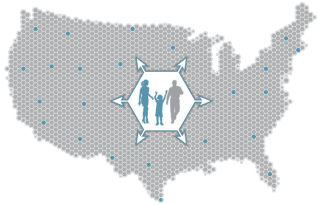
~8% reported having one or more WHIM patients

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

PATIENTS WITH THE "FACE" OF WHIM – IDENTIFIED IN DATABASE

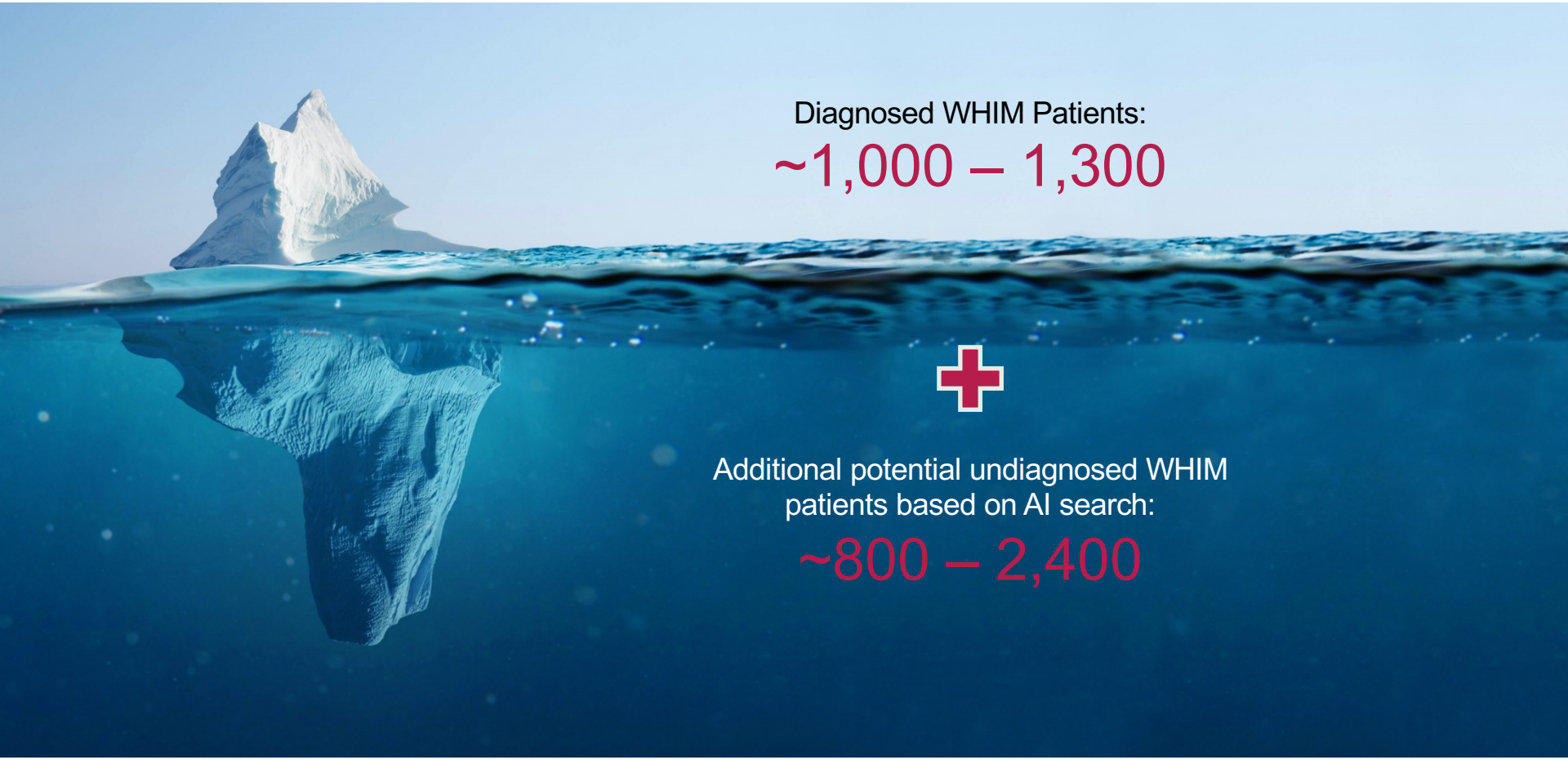
Conditions ***Required*** **for Inclusion**	Lower Estimate	Higher Estimate
Warts of Skin Condition	✓	✓
Hypogammaglobulinemia	✓	
Hypogammaglobulinemia *OR* Immunodeficiency Markers		✓
Respiratory Infection or Pneumonia	✓	✓

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

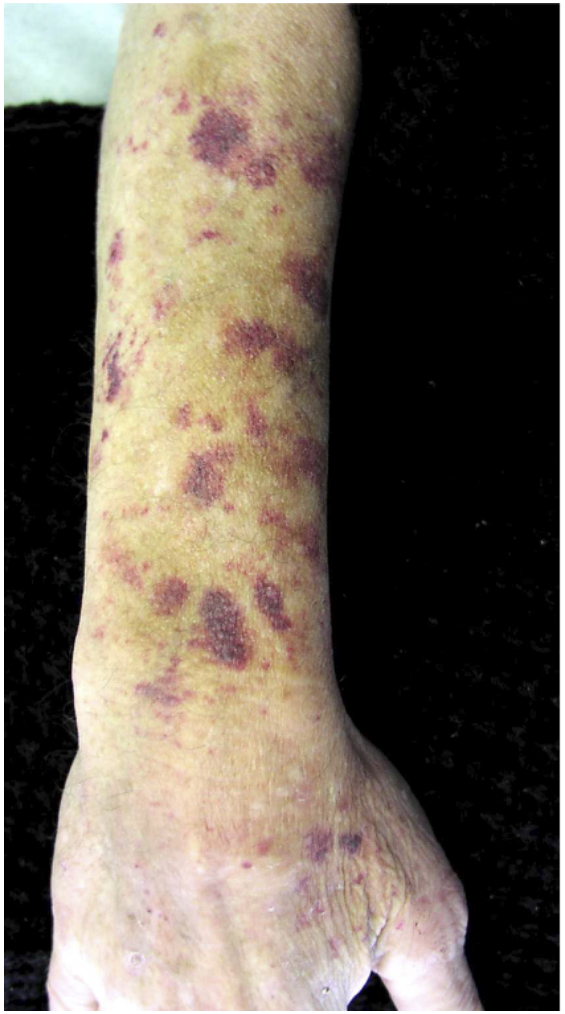
WHIM PREVALENCE OF 1,000 TO OVER 3,500 IN THE U.S.
RANGE INCLUDES POTENTIAL UNDIAGNOSED WHIM PATIENTS



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

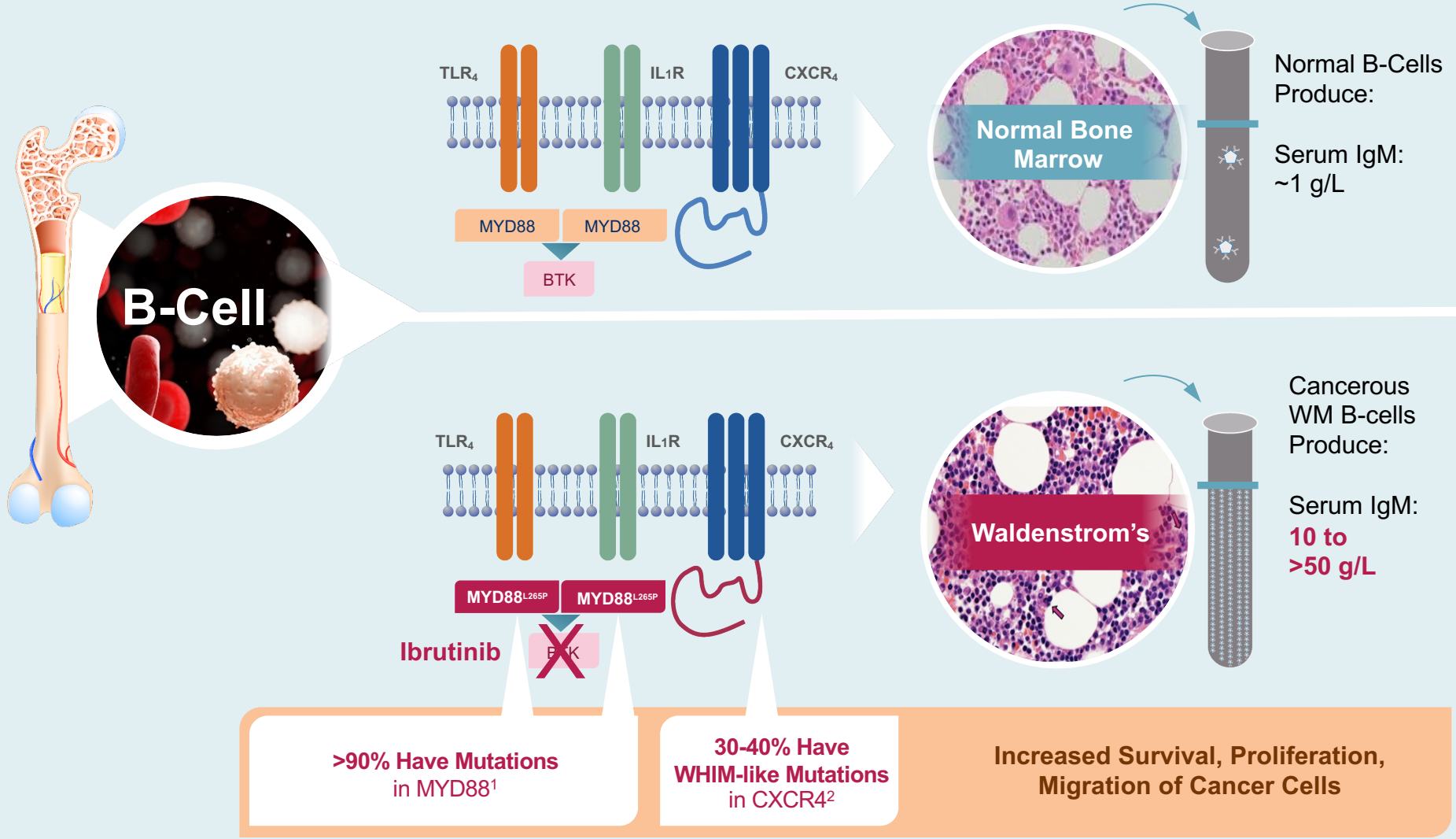
OVERVIEW: WALDENSTRÖM'S MACROGLOBULINEMIA (WM)

- Rare blood cancer: form of Non-Hodgkin's Lymphoma
- Current Standard of Care
  - Bendamustine
  - Rituximab
  - R-CHOP
  - BTK inhibitors: ibrutinib
  - Combinations & others
- Chronic, changing treatments as disease progresses
  - *Very* rare "Complete Responses"
- ~8-year survival rate post-diagnosis[1,2]
  - Improving with new treatments for some patients
- Genetic Drivers of WM Disease
  - MYD88 and CXCR4



[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

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



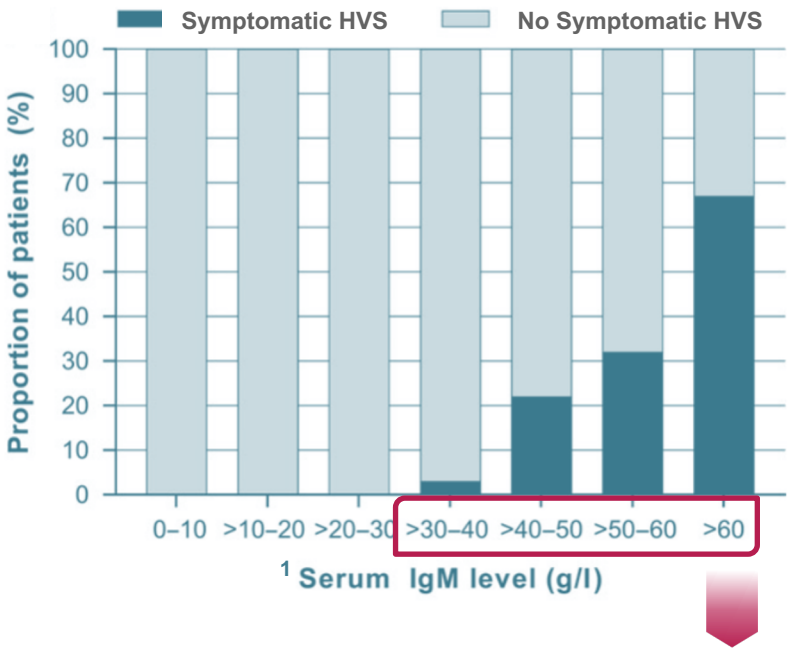
1. Treon et al. 2012. *New England Journal of Medicine,* 367, 826-833; 2. Hunter et al. 2014. *Blood*, 123, 1637–1646.

IMPACT OF CXCR4 MUTATIONS: MORE SEVERE DISEASE, HIGHER RISKS AND NEED FOR TREATMENT

	CXCR4 mutated	CXCR4 wild type
Serum IgM level	+++	+
Risk of hyperviscosity	+++	+
Lymphadenopathy	+	+++
Splenomegaly	+	+
Serum beta-2-macroglobulin level	+	++
Thrombocytopenia	++	+
Leukopenia	+	+
Anemia	+	+
Bone marrow involvement	++	+
Acquired von Willebrand disease	+++	+
Time to therapy initiation	Shorter	Longer
# of WMs 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
- Mucosal (oronasal) bleeding
- Irreversible neurological impairment
- Plasma volume expansion

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

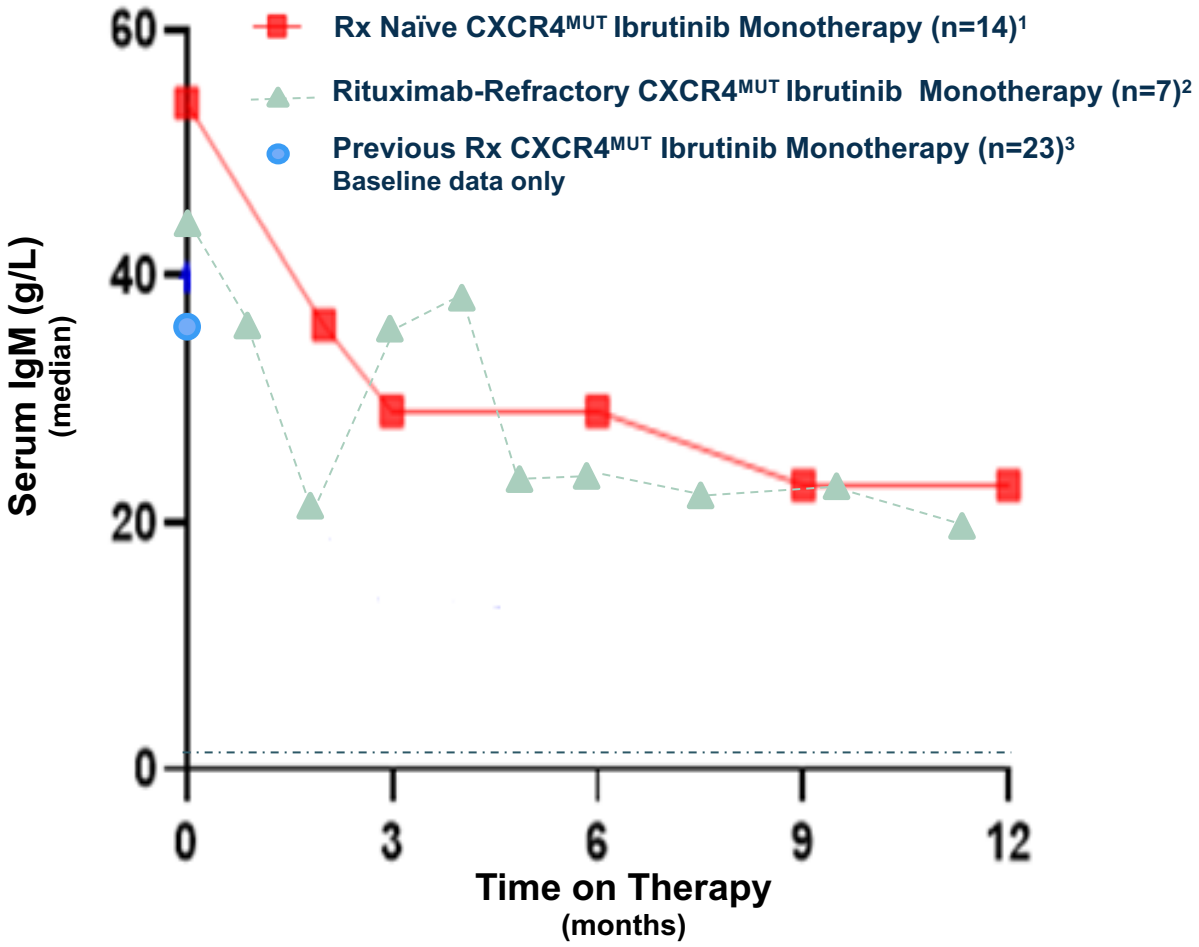
IMPROVED STANDARD OF CARE IS NEEDED FOR CXCR4-MUTANT WALDENSTROM'S PATIENTS

- Deeper absolute reductions in serum IgM levels
  - Reduce risk of symptomatic HVS, an emergent condition
  - Major component and leading indicator of Objective Response Rates
- Improvements in Major Response Rates Are Needed
  - Ibrutinib FDA approval based on overall response rates in 2015

CXCR4-MUTANT WM PATIENTS: Phase 3 Trial Benchmarks
IgM KINETICS

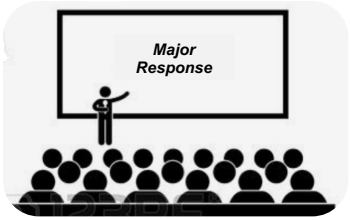
Treatment Goal

Absolute IgM levels as low as possible, as quickly as possible



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.
3. Treon S. et al, JCO 2020 DOI https://doi. org/10.1200/JCO.20. 00555

What Is the Definition of "Major Response" in WM?



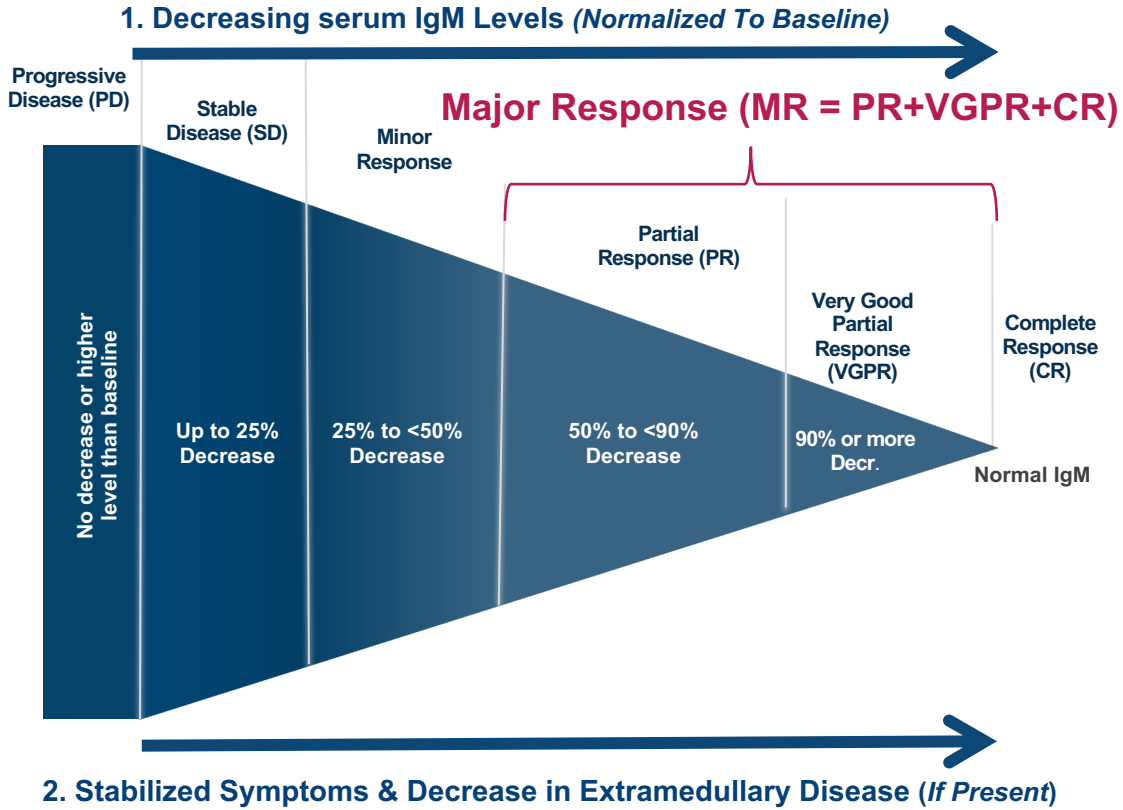
Response Assessments Require Longer Term Study

CT/MRI images every 3 or 6-months

Bone marrow aspirates at 6 or 12 months or when clinically indicated

Major Response

- **>50% reduction in normalized serum IgM**
  - using "best response"
- No new/worsening of symptoms
- Decrease (via imaging) lymphadenopathy/ splenomegaly disease if present at baseline
- Bone marrow normalization (via BM aspirate) for Complete Response eval.



CXCR4-MUTANT WM PATIENTS: Phase 3 Trial Benchmarks
Major Response Metrics

Effect of Ibrutinib Treatment As Monotherapy

	PREVIOUSLY TREATED[1,2]	FRONT LINE[3]
Time to Major Response (months)	6.0	7.3
Major Response (%) at 24 weeks	38.1%	28.6%

- Improvements in Major Response Rates Are Needed Both Short and Long-Term
  - Chronic studies (duration >12 months) report out on sub-components of Major Responses: Partial Response (PR), Very Good Partial Response (VGPR) and Complete Response (PR)

1.2. Treon, NEJM, 2015; Treon, EHA, 2018
3. Treon, JCO, 2018.

WM PHASE 1B TRIAL UNDERWAY:
FOCUS ON DOUBLE-MUTANT REFRACTORY/RECURRENT

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 with 3-month extension
- **3 cohorts supporting dose selection of mavorixafor:**
  - Cohort A: 6 patients dosed up to 400 mg*
  - Cohort B: additional 6 patients dosed up to 600 mg
  - Cohort C (expansion): potential for additional 6 patients dosed up to 600 mg
- **Endpoints**
  - Ssafety, PK/PD, and ~monthly assessments of serum IgM levels and other blood parameter
  - Objective responses assessed every 6 months

1H21: EHA abstract submission on initial data in low/mid doses
2H21: safety, dose selection, and response rates

LEUKEMIA & LYMPHOMA SOCIETY®

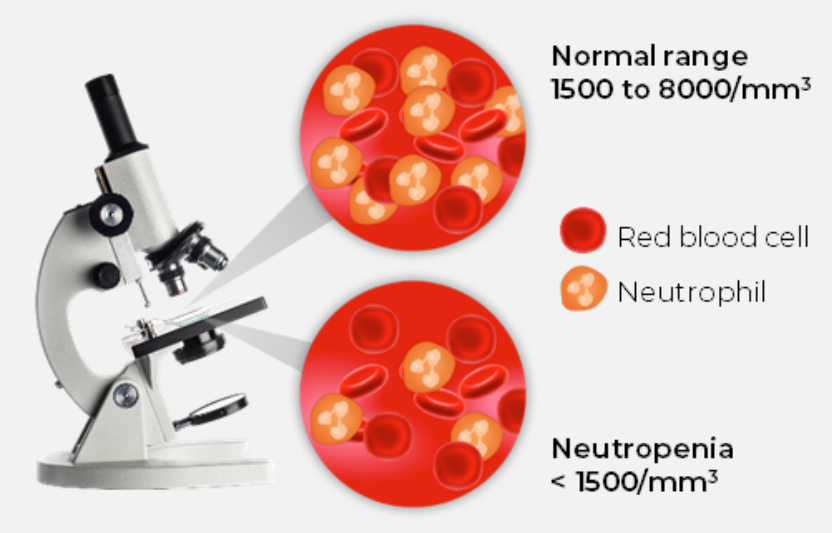
- Strategic collaboration with Leukemia & Lymphoma Society (LLS)
- Selected for LLS' Therapy Acceleration Program

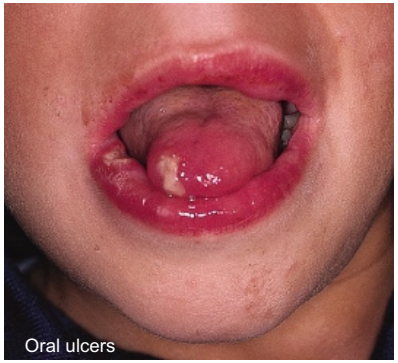
* Cohort A are eligible to dose escalate to 600 mg after Cohort B completes first 600 mg cycle.

LABEL EXPANSION OPPORTUNITIES

OVERVIEW: 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 TRIAL UNDERWAY:
FOCUS ON NEUTROPHIL RESPONDERS

14-DAY EXPLORATORY TRIAL ASSESSING FOR RESPONDERS TO MAVORIXAFOR

PATIENT PROFILE	DAY 0	DAY 1, DOSE 1	DAY 14, DOSE 14
Severe Chronic Idiopathic Neutropenia population and exploratory sub-populations	**Baseline ANC**[1]	If Day 1 ANC >25% over baseline within 8 hours, **continue** daily mavorixafor	**Final ANC**[1]

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

[1] *Measured over first 8-hours of baseline assessment or dose*

CLINICAL EPIDEMIOLOGY SUGGESTS SIGNIFICANT MARKET OPPORTUNITY

De-risked MOA targeting the CXCR4 pathway positions X4 to treat >10,000 total 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 ANTICIPATED MILESTONE ACHIEVEMENTS

2021

- 1H: Announce initial WM P1b data
- Q2/Q3: Update on WHIM Phase 3 Enrollment
- 2H: Additional WHIM Prevalence Research
- 2H: Announce initial SCN P1b data
- 2H: Announce additional WM P1b data
- Q4’21/Q1’22: WHIM P2 OLE update

2022

- Initiate WM P2/3 trial
- Announce WHIM P3 topline data
- IND Filing (X4P-002 or X4P-003)
- Q4’22/Q1’23: File WHIM NDA

SELECTED FINANCIAL INFORMATION

$117M[1]

Cash Expected to Fund Operations into Q4 2022[2]

Share and Warrant Information:

- **26.5M shares outstanding**
  (23.6M common shares and 2.9M 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] Unaudited figure as of March 31, 2021 (includes proceeds received by the Company in connection with its $55 million PIPE transaction announced on March 19, 2021).
[2] Assumes the retirement of the Company's debt with Hercules Capital, Inc. as a condition to closing the Company's co-development agreement with Abingworth LLP.