X4 PHARMACEUTICALS

INVESTOR PRESENTATION

FEBRUARY 2020

FORWARD LOOKING STATEMENTS

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements include statements regarding X4’s plans for, or progress, scope, cost, duration or results or timing for the initiation, completion or availability of results of development of mavorixafor (X4P-001) or any of our other product candidates or programs, including regarding the Phase 3 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome, the Phase 2a portion of the Phase 1/2 clinical trial of mavorixafor in combination with axitinib in ccRCC, the Phase 1b clinical trials of mavorixafor for the treatment of Severe Congenital Neutropenia (SCN) and Waldenstrom’s Macrogloubulinemia (WM), the target indication(s) for development, the size, design, population, location, conduct, objective, duration or endpoints of any clinical trial, or the timing for initiation or completion of or reporting of results from any clinical trial, the potential benefits of mavorixafor, or any other product candidate or program or the commercial opportunity in any target indication, the expected offerings and benefits of the PATH4WARD program, X4’s relationship with Invitae and X4’s cash position.

These statements are subject to various risks and uncertainties, actual results could differ materially from those projected and X4 cautions investors not to place undue reliance on the forward-looking statements in this presentation. 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 and the risk that the PATH4WARD program and X4’s relationship with Invitae will not be successful. Any forward-looking statements in this presentation 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 in this press release, 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), as updated by X4’s Current Report on Form 8-K filed with the SEC on April 11, 2019, 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 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

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

LEADERSHIP:
PROVEN TEAM WITH RARE DISEASE EXPERTISE

MANAGEMENT

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ADAM MOSTAFA
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SAREPTA THERAPEUTICS SANOFI

BOARD OF DIRECTORS

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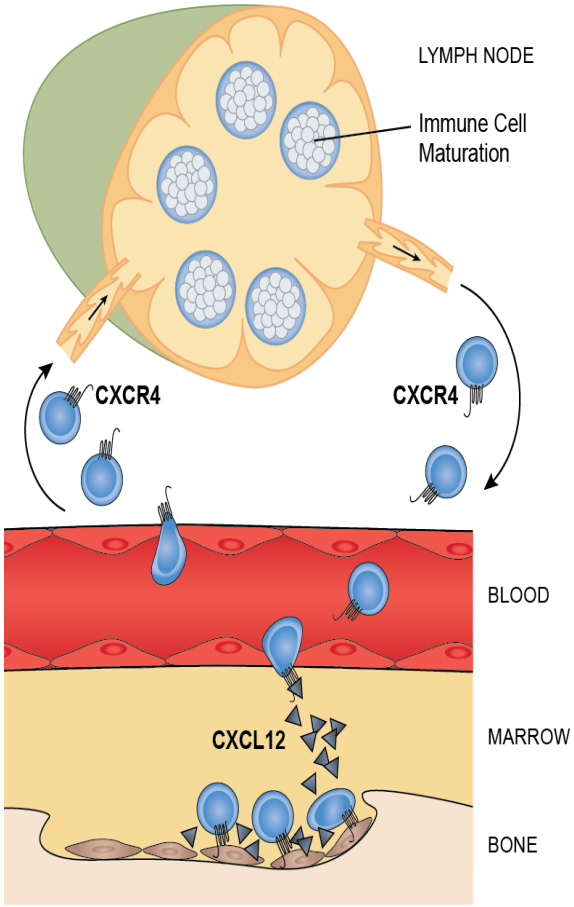
rhythm novelion THERAPEUTICS

RENE RUSSO, PharmaD, BCPS, Director

arsanis CUBIST PHARMACEUTICALS

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

PHASE 2A:
Renal Cell Carcinoma*

Immune-suppression corrected by **blocking CXCR4 Signaling**

PIPELINE

PRECLINICAL PROGRAMS:
Additional primary immuno-deficiencies

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

** Intend to enter into a strategic partnership for future development and potential commercialization for mavorixifor for ccRCC and other potential immuno-oncology indications*

OVERVIEW: MAVORIXAFOR

POTENTIAL FIRST-IN-CLASS ORAL CXCR4 ANTAGONIST

- 350 Da small molecule with high potency (<10 nM) and selectivity
  - Administered as an oral capsule, once daily
  - Terminal half-life of 22 hours
- Previous clinical trials with >100 patients demonstrated dose-dependent mobilization of neutrophils and lymphocytes and favorable safety profile
- Orphan Drug Designation for WHIM syndrome received from FDA and EMA
- IP: orphan market exclusivity as well as issued and pending patents, if granted, support exclusivity into late 2030's
- Breakthrough Therapy Designation granted by FDA for treatment of adult WHIM patients

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		
	Clear cell renal cell carcinoma[2, 3] (ccRCC) *(Combination with Inlyta®)*			PHASE 2A	
X4P-002	Glioblastoma multiforme (GBM)				
X4P-003	Primary immuno-deficiencies (PID)				

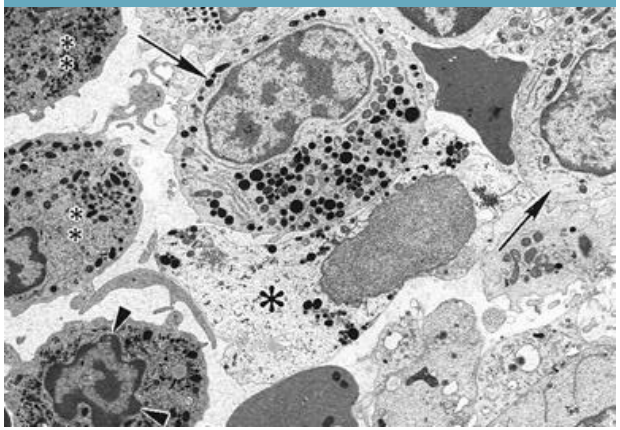
[1] Phase 2 open label extension trial for WHIM ongoing and Phase 3 trial initiated
[2] Two oncology trials have concluded: Phase 1b biomarker in melanoma and Phase 1b in ccRCC. Positive data from ccRCC Phase 2a trial reported at ESMO 2019
[3] Intend to enter into a strategic partnership for future development and potential commercialization for mavorixifor for ccRCC and other potential solid tumor immuno-oncology indications

LEAD INDICATIONS:
CXCR4 MUTATIONS AS A DRIVER OF DISEASE

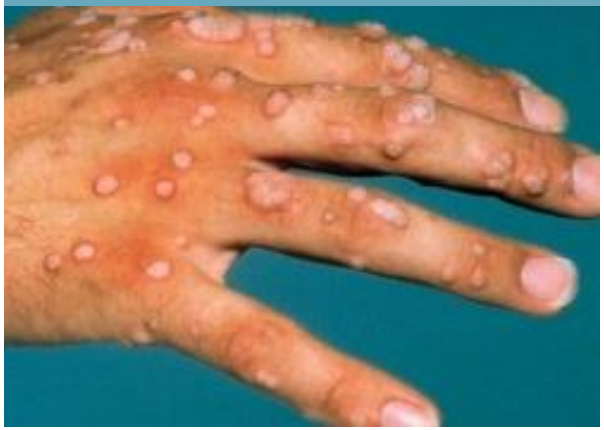
WHAT IS WHIM SYNDROME?

Warts **H**ypogammaglobulinemia **I**nfections **M**yelokathexis

MYELOKATHEXIS



HPV-WARTS



WHIM PATIENT:
ORAL HPV-ASSOCIATED CANCER

WHIM SYNDROME CARRIES A SIGNIFICANT UNMET MEDICAL NEED

NO THERAPIES ARE APPROVED TO ADDRESS THE UNDERLYING CAUSE OF WHIM: A GENETIC DEFECT OF THE CXCR4 RECEPTOR

- Immuno-deficiency caused by “gain-of-function” mutations in the CXCR4 receptor
  - Bone marrow filled with degenerating white blood cells including apoptotic neutrophils, due to defective trafficking caused by CXCR4 mutations
- Clinical impact of the genetic root cause
  - Critically low white blood cell counts, including neutrophils and lymphocytes, results in insufficient ability to clear serious bacterial and viral infections
  - Long-term impact of increased incidence of bronchiectasis (loss of lung function), HPV and EB related cancers, hearing loss
  - FDA’s March 2019 industry guidance is that WHIM Syndrome is a “Severely Debilitating or Life-Threatening Hematologic Disorder”
- Symptomatic treatments don’t address underlying disease
  - Antibiotics, G-CSF, and immunoglobulins can be used; none tested in WHIM trials

PHASE 2 TRIAL INFORMS PHASE 3 TRIAL

PHASE 2 TRIAL DESIGN

INTRA-PATIENT DOSE ESCALATION

- Open label
- 50mg to 400mg oral capsule once daily (QD)
- n = 8 patients
- Daily, assessed at one month and beyond

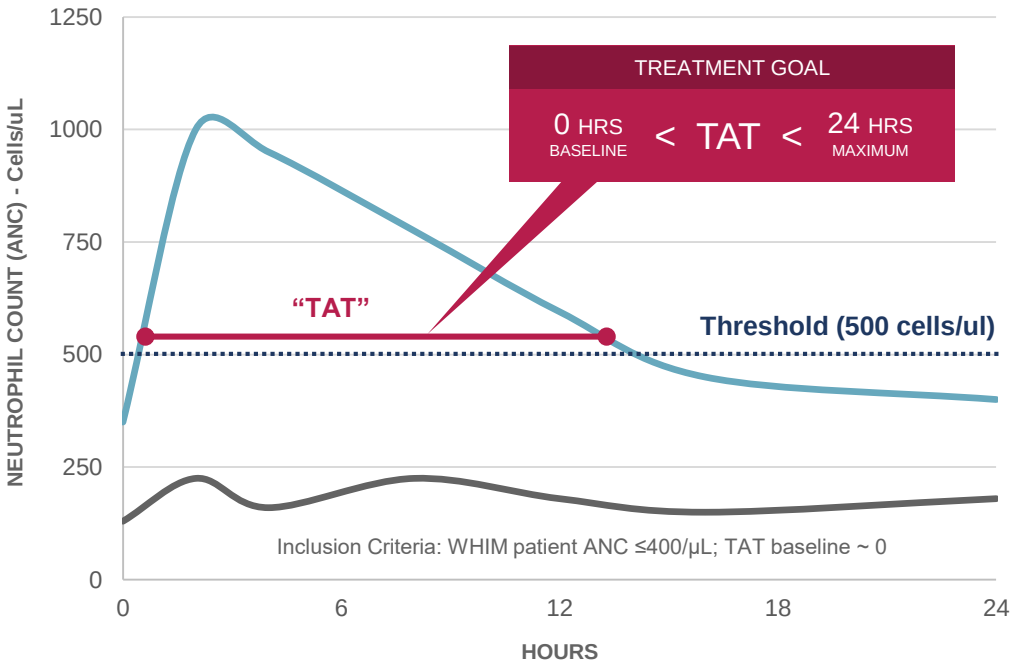
INCLUSION

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

ENDPOINTS & ASSESSMENTS

- Safety (infections, warts), pharmacokinetics (PK) / pharmacodynamics (PD)
- **Biomarker:** 24-hr Blood Counts of Neutrophils– Time (hrs.) Above Threshold

ILLUSTRATIVE TRIAL ENDPOINT EXAMPLE

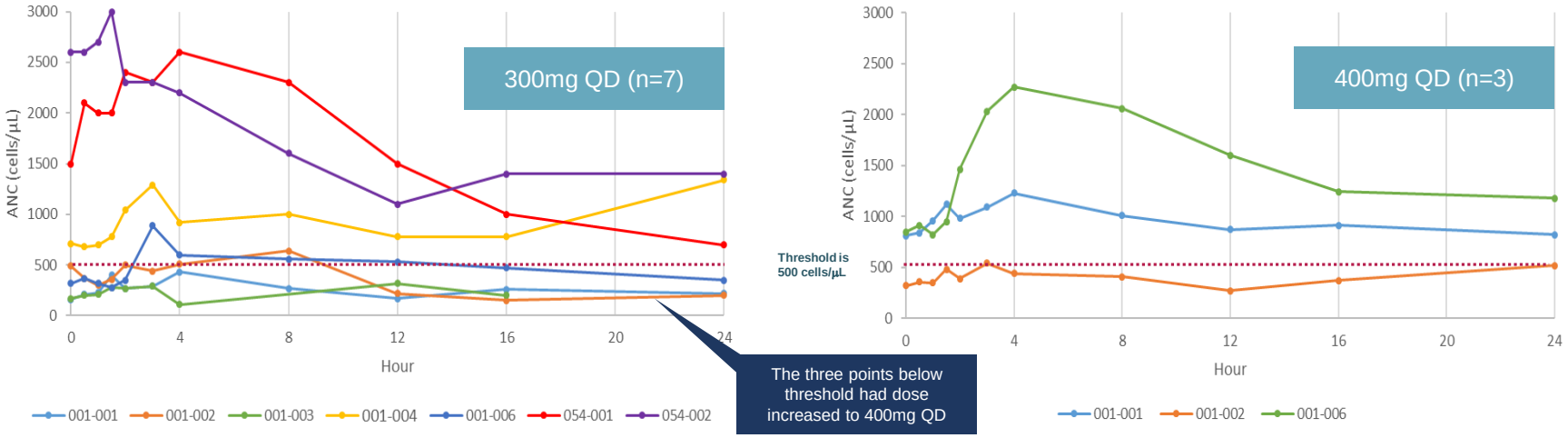


OBJECTIVE: INCREASE DAILY NEUTROPHIL COUNTS (ANC) ABOVE THRESHOLD AS MEASURED OVER 24 HOURS: TIME ABOVE THRESHOLD (TAT)

WHIM PHASE 2:
ACHIEVED MAXIMUM TAT IN MOST PATIENTS

NEUTROPHILS AND LYMPHOCYTES MOBILIZED; PAN-LEUKOPENIA ADDRESSED

Patients' baseline with an ANC of 50 – 200 cells/μL prior to treatment. Patients **dosed daily** and assessed at one month and beyond.

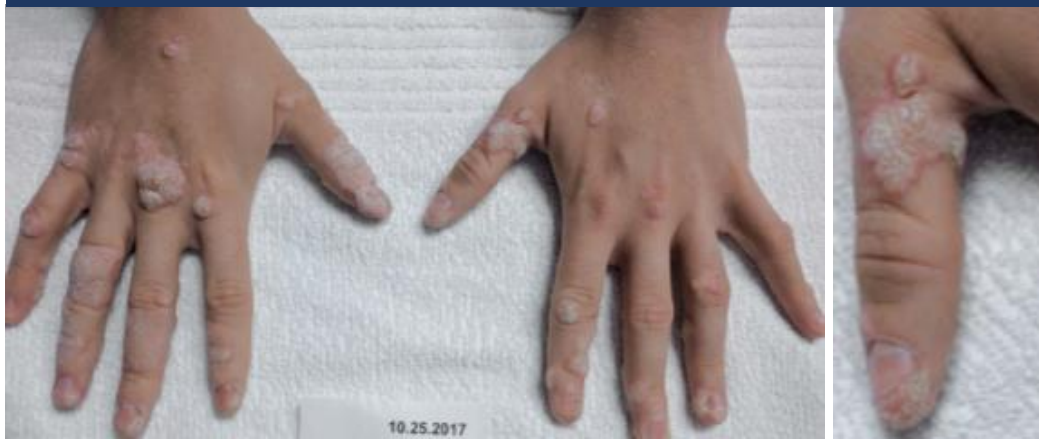


ASSESSMENTS		RESULTS
Neutrophil Counts > Threshold	✓	5 of 7 patients (71%): maximum TAT
Lymphocyte Counts > Threshold	✓	6 of 7 patients (85%): maximum TAT
Safety	✓	Acceptable; no Grade 3/4

400mg QD: Dose being tested in pivotal Phase 3 clinical trial which is open for patient enrollment

WHIM PHASE 2: SIGNIFICANT REDUCTION IN WART BURDEN THROUGH 55 WEEKS

PRE-TREATMENT



55 WEEKS POST-TREATMENT

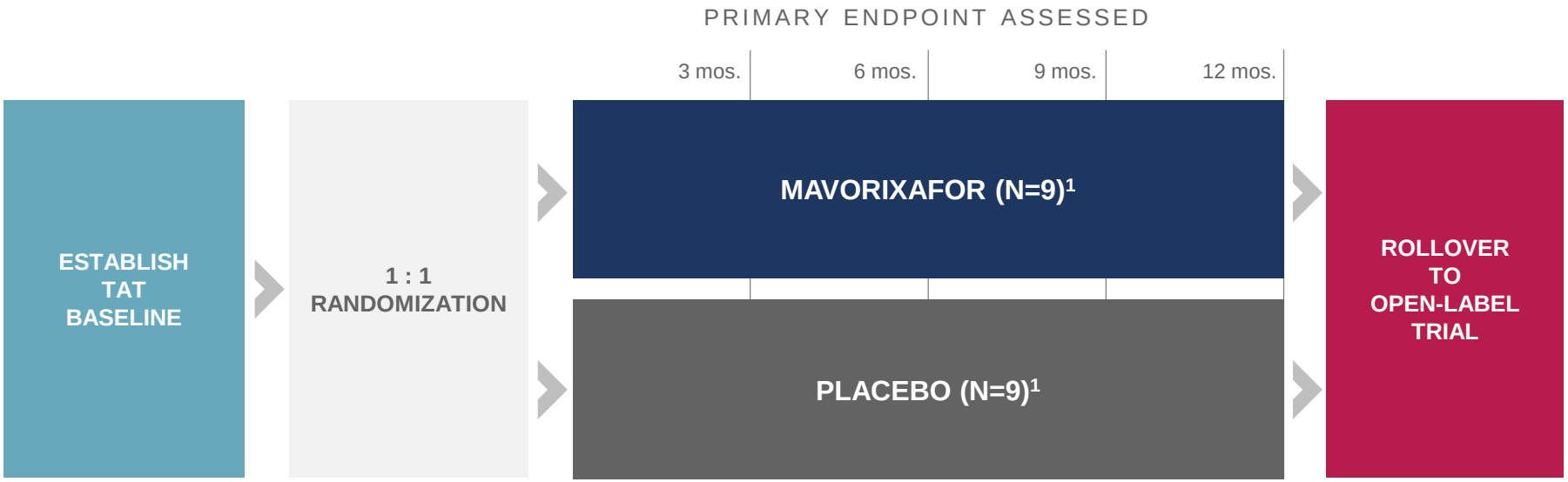
REDUCTIONS IN INFECTION RATE COMPARED TO HISTORICAL RATES

INFECTION RATES

- Minimal infections in three patients dosed for over 9-months (0.08 infections/pt/month)[1]
- Historical infection rates reported in WHIM (0.37 infections/pt/month)[2]

1. Dale et al, ASH, 2018; 2. McDermott, et a. Blood, 2014

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

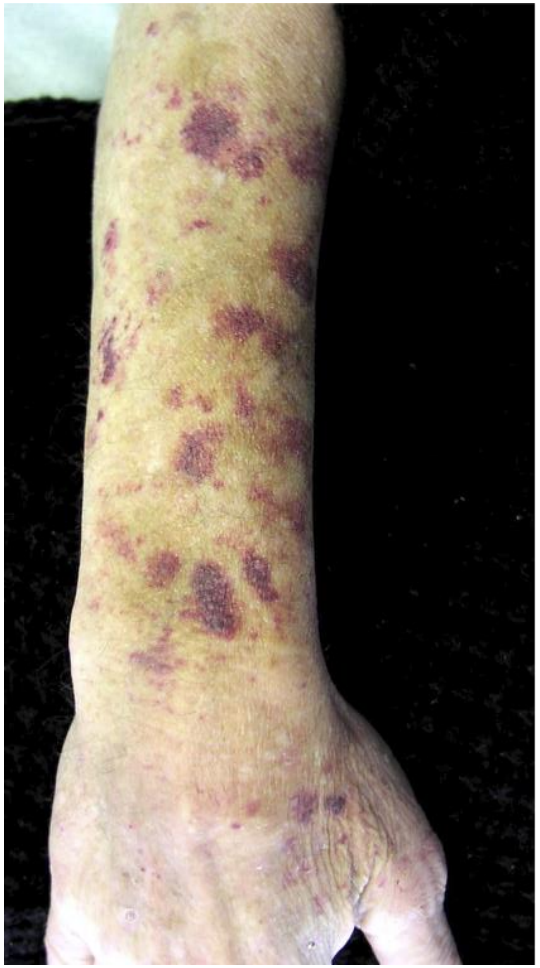
Dosing: 400mg QD in patients 12 years of age or older

Enrollment: Plan to enroll 18 to 28 subjects and activate approximately 20 to 25 sites globally

[1] *Allowed to enroll up to 14 patients on drug and 14 patients on placebo*

OVERVIEW: WALDENSTRÖM'S MACROGLOBULINEMIA (WM)

- Rare form of Non-Hodgkin's Lymphoma
- Estimated prevalence of >13,000 in US and EU[1,2]
  - Annual incidence: 1,000-1,500 in US; ~1,800 in EU [1,2]
- Signs and symptoms
  - Elevated IgM and other blood-markers
  - Hepatomegaly, splenomegaly, skin purpura
- ~8-year survival rate post-diagnosis [1,2]
- Current treatment
  - Imbruvica ($136,000 per year)
  - Chemo and Rituxan in certain lines/settings
- Mechanism: genetic drivers in WM
  - >90% have mutations in MYD88 gene
  - 30-40% have WHIM-like mutations in CXCR4 gene



CXCR4 MUTATIONS DRIVE POOR RESPONSE IN RARE LYMPHOMA

[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*

CXCR4^{WHIM} REFRACTORY/RECURRENT WM: POOR CLINICAL OUTCOMES VS. WILD-TYPE

RESPONSE PROFILE IN REFRACTORY/RECURRENT WM (IBRUTINIB TREATMENT ONLY)			
	MYD88^{Mut} CXCR4^{WT}	MYD88^{Mut} CXCR4^{Mut}	P-Value
Patients (n) =	36	21	
ORR	100%	85.7%	0.005
Major (>PR)	97.2%	66.6%	<0.001
VGPR	44.4%	9.5%	0.007
Time to Minor Response (mos.)	1.0	1.0	0.10
Time to Major Response (mos.)	2.0	6.0	0.05

- Very Good Partial Response (VGPR) Rates: 9.5% vs. 44.4% for wild-type; no Complete Responses (CRs) in either[1]
- Median time to major response of 6 months vs. 2 months for wild-type[1]
- Median Progression Free Survival (mPFS) for CXCR4^{WHIM} is less than half that of mPFS for wild-type[2]
- ~4-fold likelihood ibrutinib discontinuation in CXCR4^{WHIM} WM[3]

1. Table Recreated from: Treon et al, EHA 2018; 2. Treon et al, EHA 2018 ; 3. Gustine J. Am J Hematol. 2018

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

Inclusion: Patients with MYD88 + CXCR4 mutations who have failed prior Rx

Design: Multi-national Phase 1b trial of mavorixafor in combination with ibrutinib

- Intrapatient dose-escalation with extension on highest tolerated dose for additional 3 months
- Endpoints: safety, PK/PD, and assessments of serum IgM levels and Hgb

Timing: Initiated December 2019

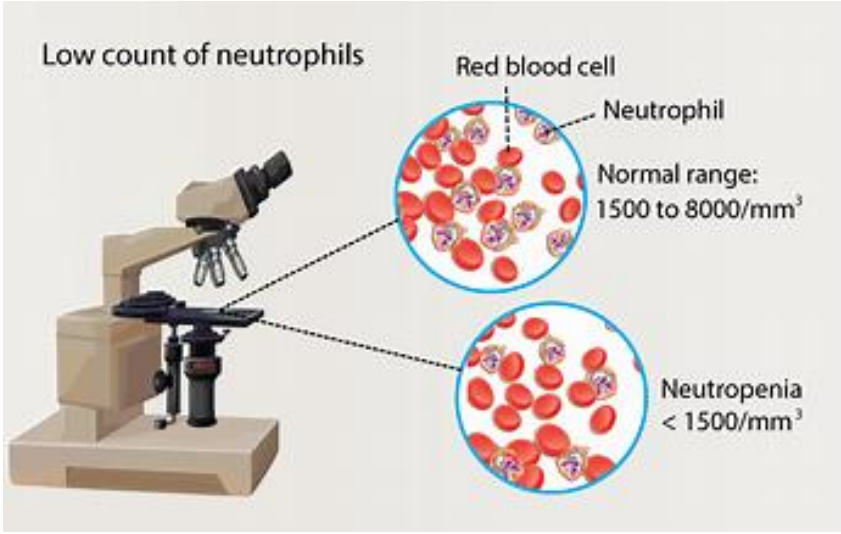
LEUKEMIA & LYMPHOMA SOCIETY®

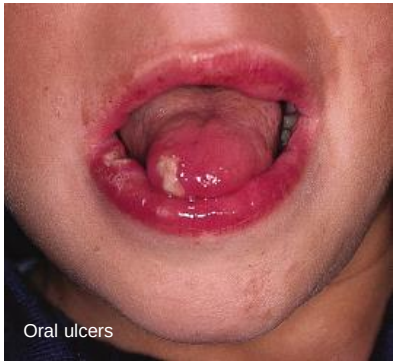
- Strategic collaboration with Leukemia & Lymphoma Society (LLS)
- Selected for LLS' Therapy Acceleration Program

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





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*

INTRODUCING **PATH4WARD**

PATH4WARD

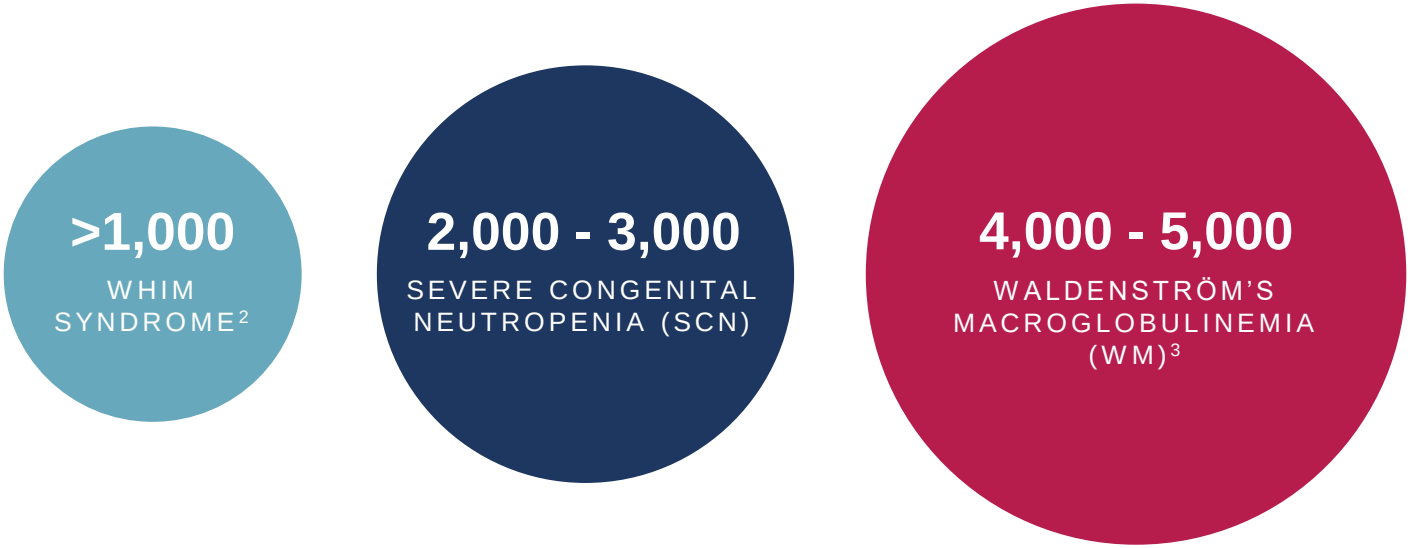
Patient Access To Hope

for Rare Primary Immunodeficiencies

- Genotyping initiative for Congenital Neutropenia including WHIM
- Collaboration with INVITAE
- Individuals with history of chronic severe neutropenia (ANC < 500/uL)
- Permanent or intermittent (cyclical) neutropenia of unknown origin and with a clinical presentations of SCN or Congenital Idiopathic Neutropenia (CIN)
- Genotyping panel of up to ~200 genes related to immuno-deficiencies
- Individuals with CXCR4 mutations may enroll in WHIM Phase 3 trial or SCN Phase 1b trial

EXPLORING THE CAUSES OF NEUTROPENIA AND POTENTIAL FOR TREATMENT WITH MAVORIXAFOR

CLINICAL EPIDEMIOLOGY[1] SUGGESTS SIGNIFICANT MARKET OPPORTUNITY



WORLD-CLASS PARTNERSHIPS TO INCREASE PATIENT AND CLINICIAN AWARENESS

LEUKEMIA & LYMPHOMA SOCIETY®

INVITAE

Jeffrey Modell Foundation

UNIVERSITY OF WASHINGTON 1861

Immune Deficiency Foundation

Hôpitaux Universitaires Est Parisien
TROUSSEAU LA ROCHE-GUYON

[1] Unless otherwise noted, these number represent US & EU; [2] US only; [3] Represents CXCR4-mutant patients; 30% to 40% of total WM estimate of 13,000 patients

IMMUNO-ONCOLOGY STRATEGY:
PARTNERSHIPS TO CAPTURE GLOBAL VALUE

COMPLETED TRIALS DEMONSTRATE SINGLE AGENT ACTIVITY & PROOF OF MECHANISM

POSITIVE DATA FROM PHASE 2A ccRCC TRIAL: MAVORIXAFOR + AXITINIB PRESENTED AT ESMO 2019

- **Phase 2a Trial:**
  - Inclusion: 65 patients, multi-national, fully enrolled
  - Assessment: 4.8 months mPFS with axitinib in patients with immediate prior TKI
  - Objective: >50% improvement in medium PFS
- **Conclusions:**
  - Combination therapy with 400mg QD mavorixafor + 5mg BID axitinib observed to be generally well-tolerated with a manageable safety profile
    - Overall mPFS across clinically evaluable patients (n=62): 7.4 months
  - Demonstrated encouraging mPFS in this heavily pretreated advanced RCC patient population
    - mPFS with immediate prior IO therapy (n=18): 11.6 months
    - mPFS with immediate prior TKI therapy (n=34): 7.4 months
    - 8 patients remain on study > 17 months
  - Results suggest that mavorixafor may enhance clinical responses to axitinib and other TKIs that target tumor angiogenesis, as well as immunotherapy agents, such as CPIs
- **Strategy: Identify strategic collaborators to advance in IO**
  - Entered into partnership with Abbisko Therapeutics to develop mavorixafor in solid tumor oncology indications. We have retained all ex-China rights and can leverage data generated by Abbisko

Continued Milestone Achievements Anticipated in 2020 & 2021

TARGET DATE	MILESTONES
2Q 2019	Phase 3 trial in WHIM syndrome: commenced ✓
Mid 2019	EMA Orphan Drug Designation for WHIM ✓
2H 2019	Phase 2a ccRCC PFS data readout ✓
2019	Phase 1b trial in SCN: initiated ✓
2019	Breakthrough Therapy Designation granted by FDA for treatment of adult WHIM patients ★
2019	Phase 1b trial in Waldenström's: initiated ✓
1H 2020	WHIM patient identification update
2H 2020	Phase 1b trial in SCN: topline results
2H 2020	Phase 1b trial in Waldenström's: initial data readout
2H 2021	Phase 3 trial in WHIM: topline results

Selected Financial Information

~$130M

cash and cash equivalents*

Cash Expected to Fund Operations into 2022

Share and Warrant Information*:

- **20M shares outstanding**
  (16.1M basic shares and 3.9M pre-funded warrants)
- **5.4M class B cash-only warrants at $15.00**
  ($80M / expiry just post WHIM P3 data)
- **3.9M class A warrants at $13.20**
  ($50M / 2024 expiry)

BIOTECH-FOCUSED INSTITUTIONAL SHAREHOLDER BASE

ANALYST COVERAGE

citi

Canaccord Genuity

COWEN

STIFEL

OPPENHEIMER

B RILEY FBR

ROTH Capital Partners

*ALL FINANCIAL DATA IS AS OF DECEMBER 31, 2019; actual cash and cash equivalents as of December 31, 2019 may differ from this estimate due to the completion of X4's closing procedures for the fiscal year ended December 31, 2019, final adjustments and other developments that may arise between now and the time the financial results for 2019 are finalized.

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