X4 PHARMACEUTICALS

INVESTOR PRESENTATION

AUGUST 2019

FORWARD LOOKING STATEMENTS

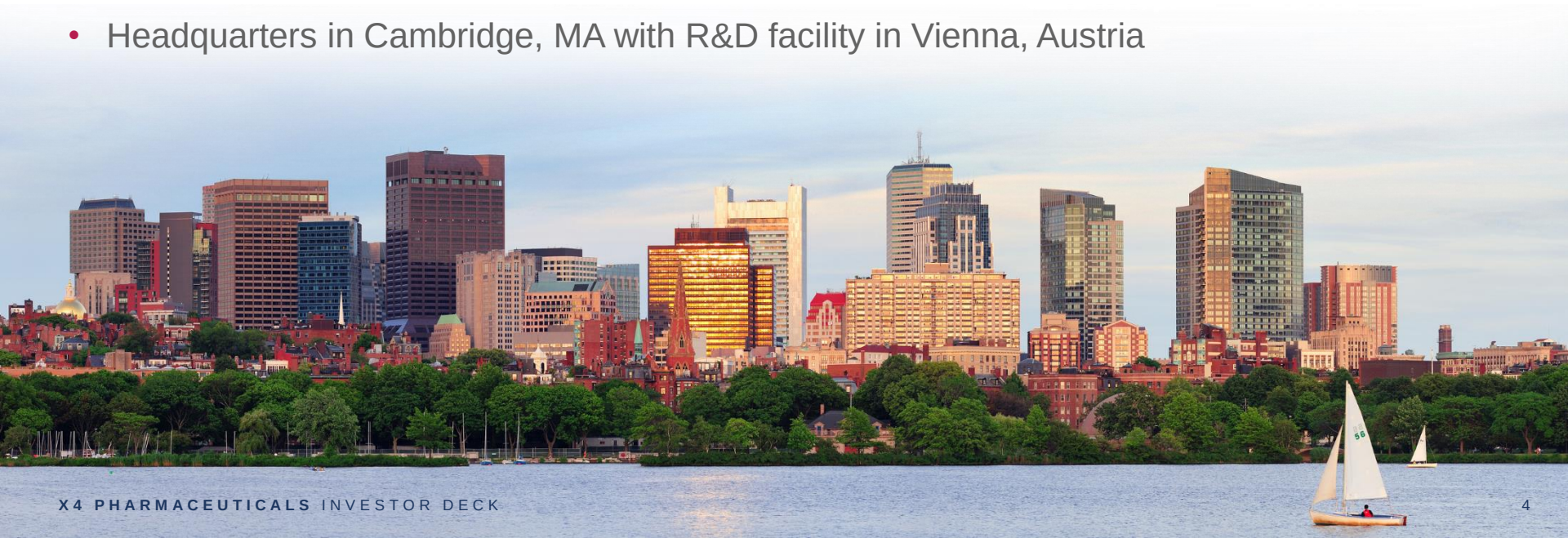
This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “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 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, or plans to commence clinical trials of mavorixafor in SCN and 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 as well as the expected offerings and benefits of the PATH4WARD program and X4’s relationship with Invitae.

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 press release. 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 press release 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 press release 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 registration Phase 3 trial of mavorixafor in WHIM syndrome
- 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

PAULA RAGAN, PhD
CEO

genzyme SANOFI

MARY DIBIASE, PhD
VP of Technical Operations & Quality

Biogen EPIRUS BIOPHARMACEUTICALS

E. LYNNE KELLEY, MD
CMO

Histogenics Senseonics

CELESTE DIJOHNSON, PhD
VP of Clinical Operations

SAREPTA THERAPEUTICS SANOFI

ADAM MOSTAFA
CFO

abpro CANTOR Fitzgerald

TAREK EBRAHIM, MD
VP of Medical Affairs

genzyme ALEXION

NIC SCALFAROTTO, DVM
VP of Regulatory Affairs

Aegerion Pharmaceuticals GE Healthcare

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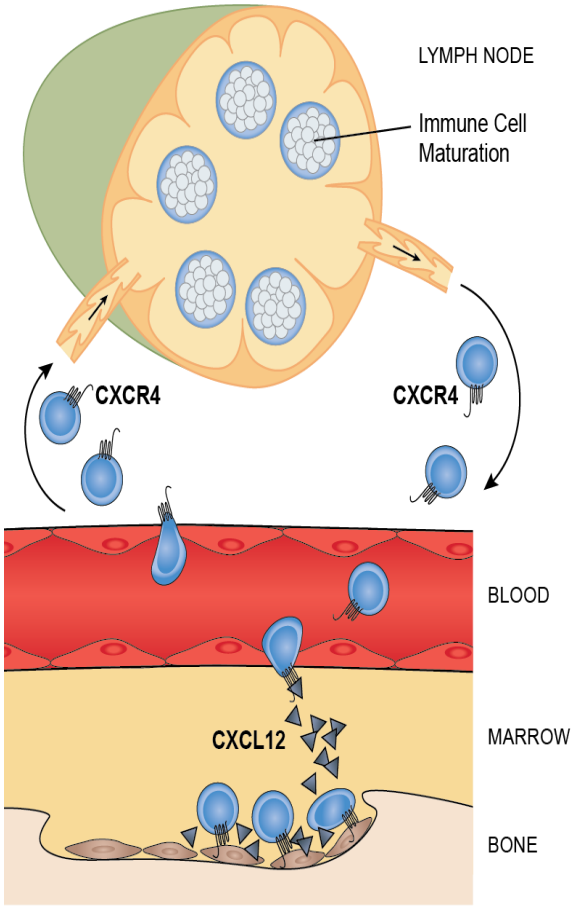
RENE RUSSO, PharmaD, BCPS, Director
arsanis CUBIST PHARMACEUTICALS

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

MECHANISM OF ACTION



LEAD INDICATIONS

PHASE 3:
WHIM Syndrome

PHASE 1/2:
Waldenström's Macroglobulinemia

Validated by Blocking **"Gain-of-Function" CXCR4 Genetic Mutations**

LABEL EXPANSION OPPORTUNITIES

PHASE 1:
Severe Congenital Neutropenia

PHASE 2A:
Renal Cell Carcinoma*

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

DISCOVERY

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

POTENTIALLY FIRST-IN-CLASS CXCR4 ANTAGONIST & ONLY 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 received from US FDA and EMA
- IP: Orphan exclusivity as well as issued and pending patents support exclusivity through late 2030's
- Favorable regulatory interactions with FDA and EMA

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 1/2		
	Severe Congenital Neutropenia (SCN)		PHASE 1		
	Clear cell renal cell carcinoma[2, 3] (ccRCC) *(Combination with Inlyta®)*			PHASE 2A	
X4P-002	Glioblastoma multiforme (GBM)				
X4P-003	Primary immuno-deficiencies (PID)				

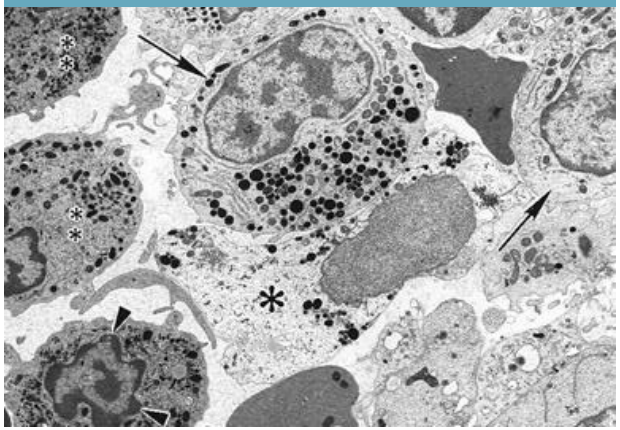
[1] *Phase 2 open label extension study for WHIM ongoing*
[2] *Two oncology trials have concluded: P1b biomarker in melanoma and P1b in ccRCC. Final publications expected in 2H19*
[3] *Intend to enter into a strategic partnership for future development and potential commercialization for mavorixifor for ccRCC and other potential immuno-oncology indications*

LEAD INDICATIONS:
CXCR4 MUTATIONS AS A DRIVER OF DISEASE

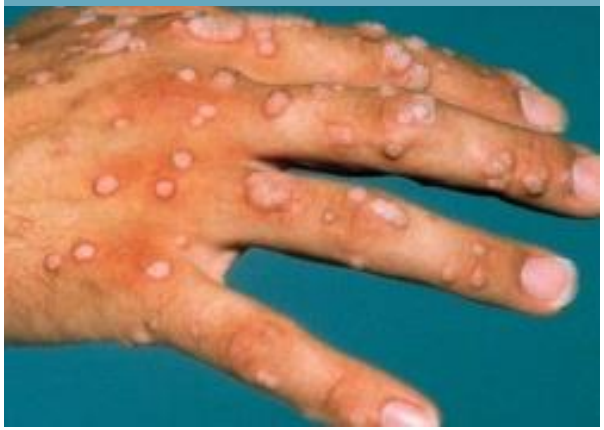
WHAT IS WHIM SYNDROME?

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-related cancers, hearing loss
  - FDA’s guidance (March 2019) that WHIM Syndrome is “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 STUDY DESIGN

INTRA-PATIENT DOSE ESCALATION

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

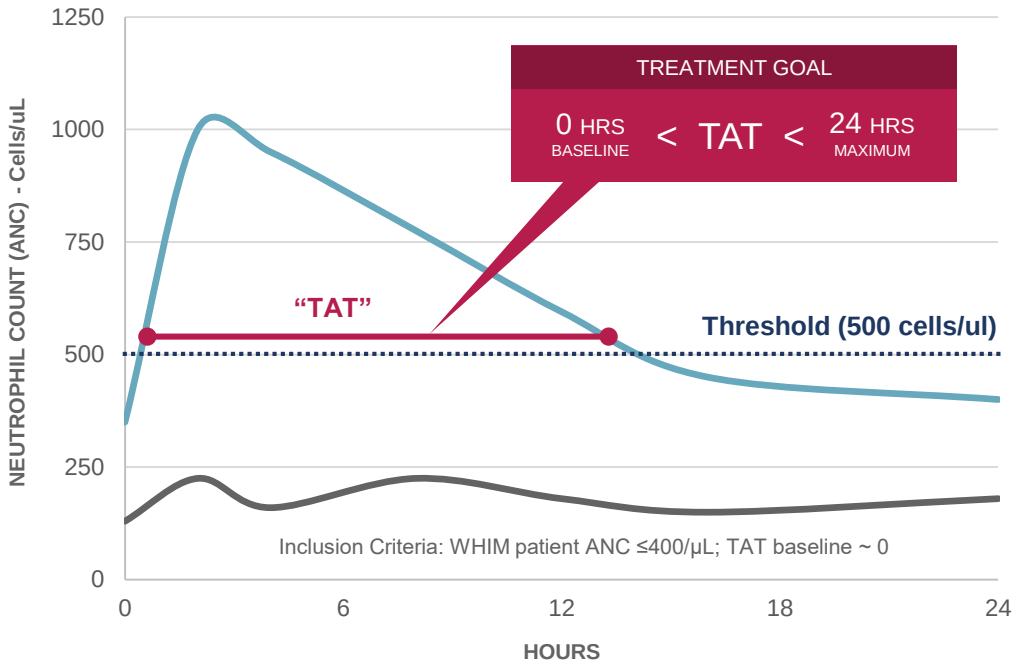
INCLUSION

- 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 STUDY ENDPOINT EXAMPLE

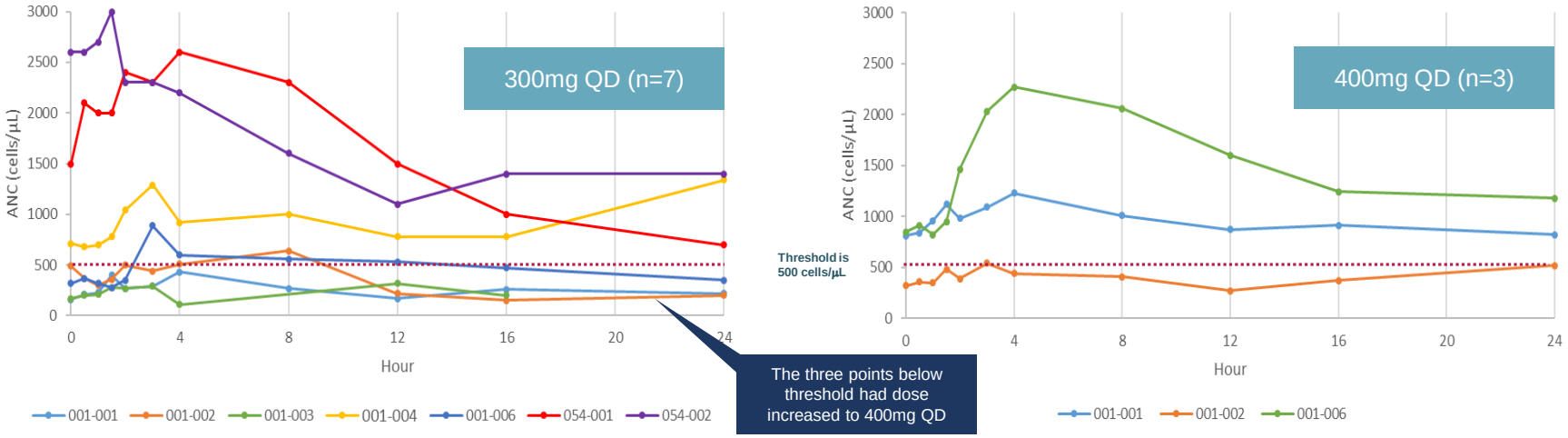


OBJECTIVE: INCREASE DAILY NEUTROPHIL COUNTS (ANC) ABOVE THRESHOLD AS MEASURED OVER 24 HOURS: TIME ABOVE THRESHOLD (TAT)

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.

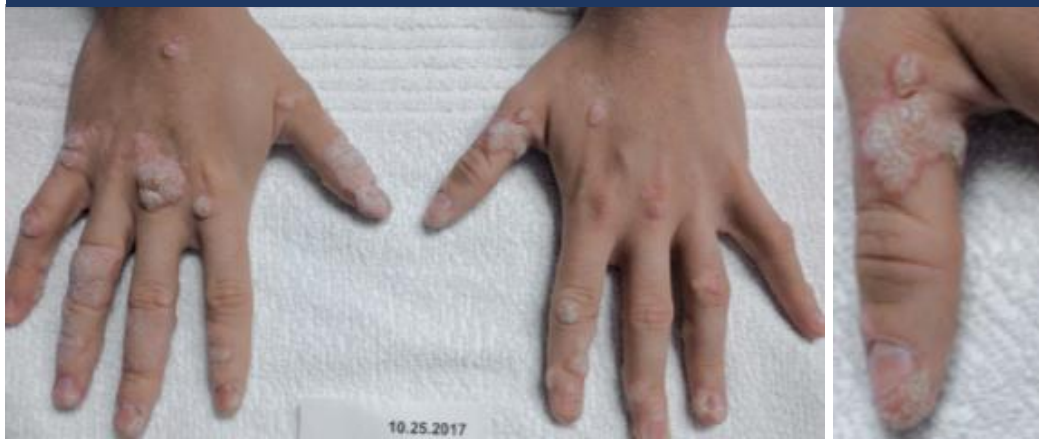


ASSESSMENTS		RESULTS
Neutrophil Counts > Threshold	✓	5 of 7 patients (71%): maximum TAT
Lymphocyte Counts > Threshold	✓	6 of 7 patients (85%): maximum TAT
Safety	✓	Acceptable; no Grade 3/4

400mg QD: Dosage in pivotal Phase 3 clinical trial that is underway

PHASE 2: SIGNIFICANT REDUCTION IN WART BURDEN THROUGH 55 WEEKS

PRE-TREATMENT



55 WEEKS POST-TREATMENT

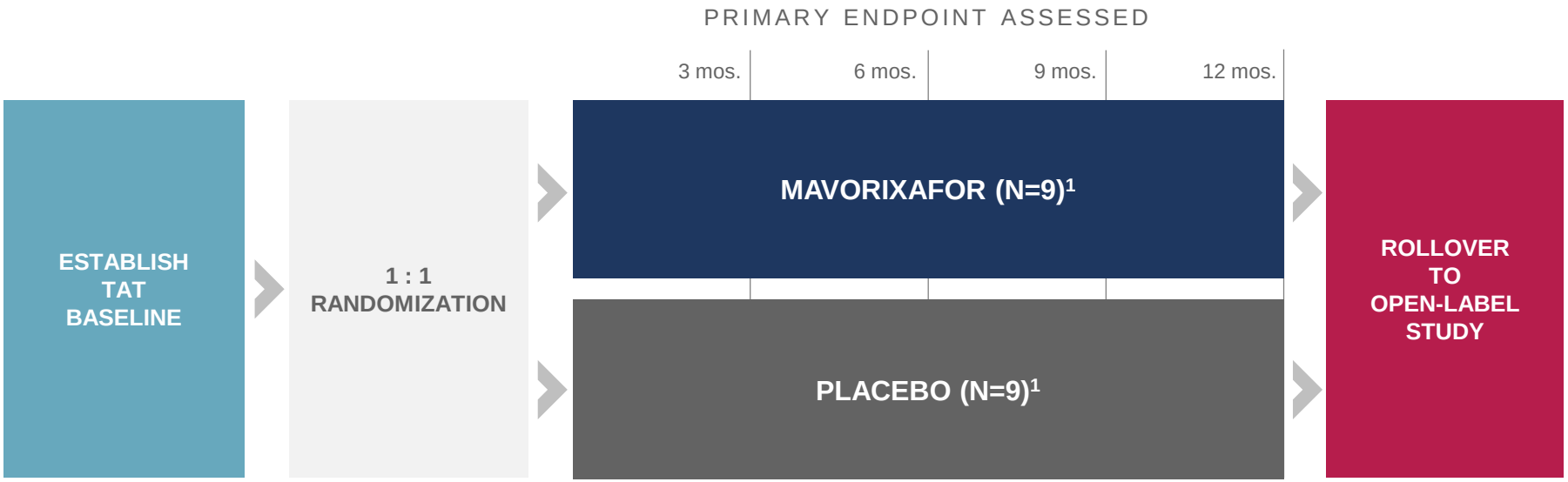
REDUCTIONS IN INFECTION RATE COMPARED TO HISTORICAL RATES

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, 2019; 2. McDermott, et a. Blood, 2014.

GLOBAL PHASE 3 REGISTRATION 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: 400 mg QD in patients 12 years of age or older

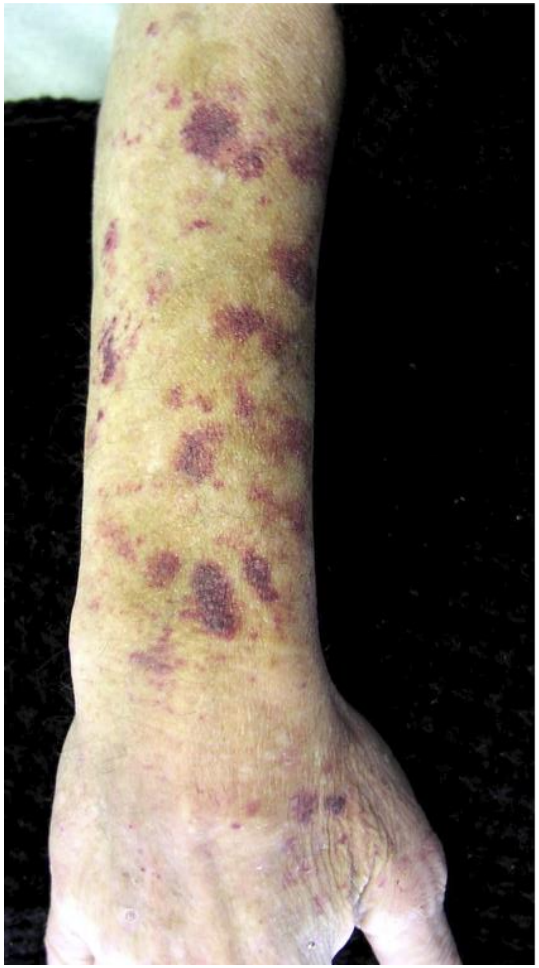
Enrollment: Opening approximately 20 sites globally

[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]
  - Annual incidence: 1,000-1,500 in US[2]; ~1,800 in EU[3]
- Signs and Symptoms
  - Elevated IgM and other blood-markers
  - Hepatomegaly, splenomegaly, skin purpura
- ~8-year survival rate post-diagnosis
- 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



*Sources [1]Prevalence estimate mathematically as incidence x median Survival X 50% (1/2 living and ½ dead at 8 years); Incidence derived mathematically as Prevalence/ 50%/ 8 years [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; [3]https://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=33226 (prevalence estimated at 1/102,220 for EU)*

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

RESPONSE PROFILE IN R/R WM			
	MYD88^{Mut} CXCR4^{WT}	MYD88^{Mut} CXCR4^{Mut}	P-Value
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 PLANNED PHASE 1B/2A TRIAL TARGETS DOUBLE-MUTANT R/R

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

Design: Multi-national Phase 1/2 of mavorixafor in combination with ibrutinib

- 3X3 dose escalation in combination; then expansion
- Endpoints: safety, PK/PD, VGPR and CR rates, other

Timing: Expected to commence in 2019

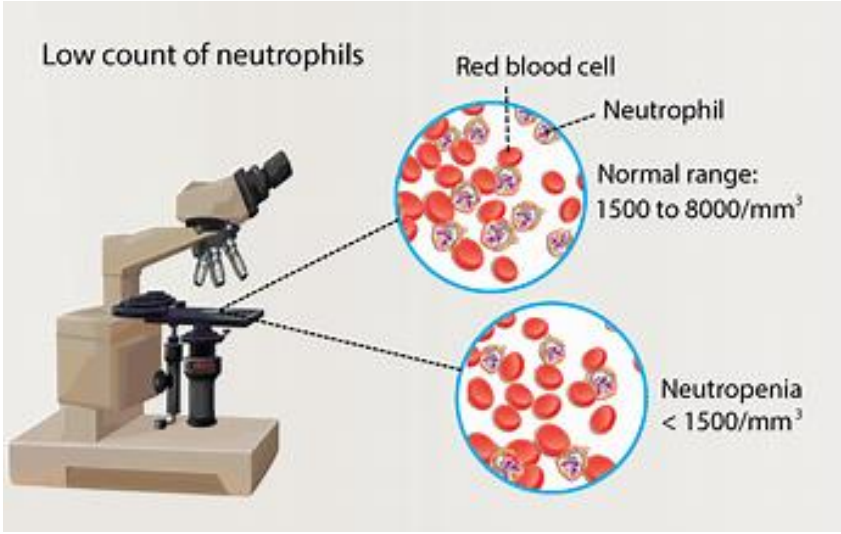
LEUKEMIA & LYMPHOMA SOCIETY®

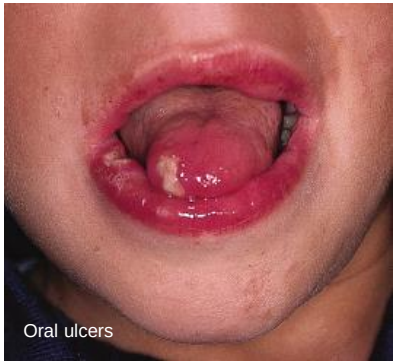
- Strategic collaboration with Leukemia & Lymphoma Society (LLS)
- Selected for LLS' Therapy Acceleration Program

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

PLANNED PHASE 1 SCN TRIAL:
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***	If Day 1 ANC >25% over baseline within 8 hours, **continue** daily mavorixafor	**Final ANC***

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

Phase 1 Endpoints: Safety and tolerability, percentage of patients with ANC >50% baseline

Goal: Achieve proof of concept to support FDA interactions regarding proposed Phase 3 trial

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

INTRODUCING **PATH4WARD**

PATH4WARD

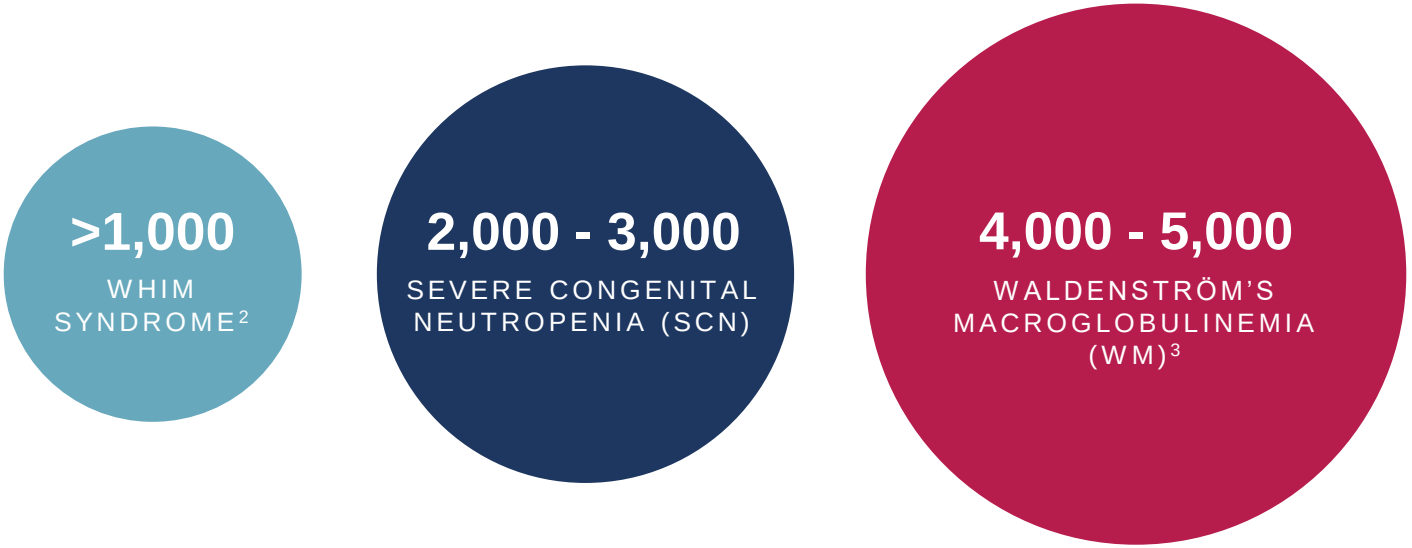
Patient Access To Hope

for Rare Primary Immunodeficiencies

- Genotyping initiative for Congenial 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 CIN
- Genotyping panel of up to ~200 genes related to immuno-deficiencies
- Individuals with CXCR4 mutations may enroll in WHIM Phase 3 trial or Phase 1 SCN 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

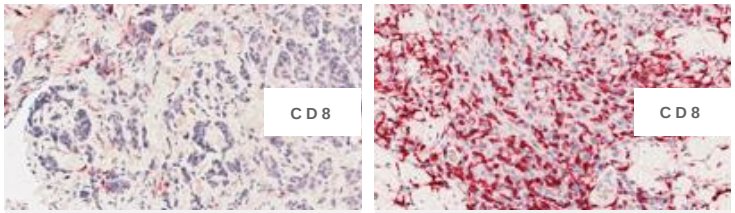
[1] Unless otherwise notes, these number represent US & EU; [2] US only; [3] Represents CXCR4-mutant patients; 30% to 40% of total WM estimate of 13,000 patients

IMMUNO-ONCOLOGY STRATEGY:
PARTNERSHIPS TO CAPTURE GLOBAL VALUE

COMPLETED TRIALS DEMONSTRATE SINGLE AGENT ACTIVITY & PROOF OF MECHANISM

PHASE 1B IN TREATMENT NAÏVE MELANOMA

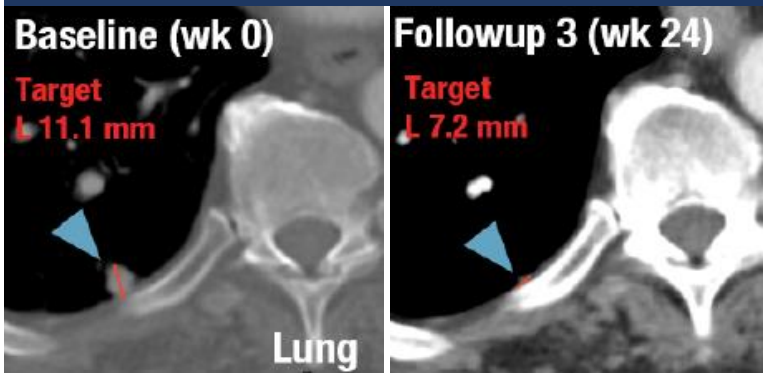
CD8+ T Cell Staining in Melanoma Tumor Biopsy (Patient #5)



DAY 1 (PRE-DOSE)

DAY 21 (X4P-001 MONOTHERAPY)

PHASE 1B IN PROGRESSING RCC WITH OPDIVO



ON-GOING PHASE 2A ccRCC TRIAL: MAVORIXAFOR + AXITINIB

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

Data Expected: 2H 2019

Strategy: Identify strategic collaborators to advance in IO; keying off data readout

SIGNIFICANT PROGRESS ANTICIPATED 2019 TO 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	Initiate Phase 1 trial in SCN
2019	Initiate Phase 1/2 in Waldenström's
4Q19-1Q20	WHIM patient identification update
1H 2020	New Pipeline: X4P-003 IND filing
Mid 2020	Phase 1 Trial in SCN: topline results
2H 2020	Phase 1/2 in Waldenstrom's: initial data readout
2H 2020	New Pipeline: X4P-002 IND filing
2021	Phase 3 Trial in WHIM: topline results

CASH EXPECTED TO BE SUFFICIENT TO FUND OPERATIONS THROUGH MID-2021

12.4 MM

SHARES OUTSTANDING
AS OF 06.30.19

$95.6 MM

CASH, CASH EQUIVALENTS & SHORT-TERM INVESTMENTS AS OF 06.30.19

RAISED $85.8 MILLION IN GROSS PROCEEDS IN APRIL 2019 OFFERING

ANALYST COVERAGE

- Canaccord Genuity
- Citi
- COWEN
- STIFEL

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