April 2019

X4 PHARMACEUTICALS

Forward Looking Statements

Certain statements in this presentation, particularly statements relating to: our 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 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 potential benefits of orphan drug designation; and our corporate strategies, prospects, projections and goals, may constitute "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and are usually identified by the use of words such as "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "will," "would," and variations of such words or similar expressions.

We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. Various important factors could cause actual results or events to differ materially from the forward-looking statements that we make, including, but not limited to, 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 risks associated with our capital needs and other risks described in the filings we make with the SEC from time to time. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. We do not assume any obligation to update any forward-looking statements, except as required by law.

A shelf registration statement on Form S-3 relating to the public offering of the shares of common stock described above was declared effective by the Securities and Exchange Commission (SEC) on February 19, 2019. Before you invest, you should read the prospectus in the registration statement and related preliminary prospectus supplement that we will file with the SEC for more complete information about our company and this offering. An electronic copy of the preliminary prospectus supplement and accompanying prospectus relating to the offering will be available on the website of the SEC at www.sec.gov. Copies of the preliminary prospectus supplement, when available, and the accompanying prospectus relating to the offering may be obtained by contacting Cowen and Company, LLC, at c/o Broadridge Financial Services, Attention: Prospectus Department, 1155 Long Island Avenue, Edgewood, New York 11717, telephone: 631-274-2806 or facsimile: 631-254-7140 or by contacting Stifel, Nicolaus & Company, Incorporated, Attention: Syndicate, One Montgomery Street, Suite 3700, San Francisco, CA 94104, telephone: (415) 364-2720 or email: syndprospectus@stifel.com.

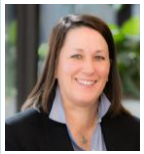
Overview: Building a Rare Disease Company

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

- **Novel therapeutics designed to improve immune cell trafficking**
- **Founded in 2014, listed on Nasdaq:XFOR in March 2019**
- **Lead product candidate mavorixafor (X4P-001), potentially first-in-class, oral, small molecule allosteric antagonist of chemokine receptor CXCR4**
- **Multiple clinical trials ongoing and planned, including Phase 3 trial of mavorixafor in WHIM syndrome anticipated to begin in Q2 2019**
- **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 and R&D facility in Vienna, Austria**

Proven Leadership Team with Rare Disease Expertise

Key management and advisors involved with R&D and launch of only approved CXCR4 antagonist - Mozobil

	Paula Ragan, PhD *CEO*	genzyme SANOFI
	E. Lynne Kelley, MD *CMO*	Histogenics Senseonics
	Adam Mostafa *CFO*	abpro CANTOR Fitzgerald

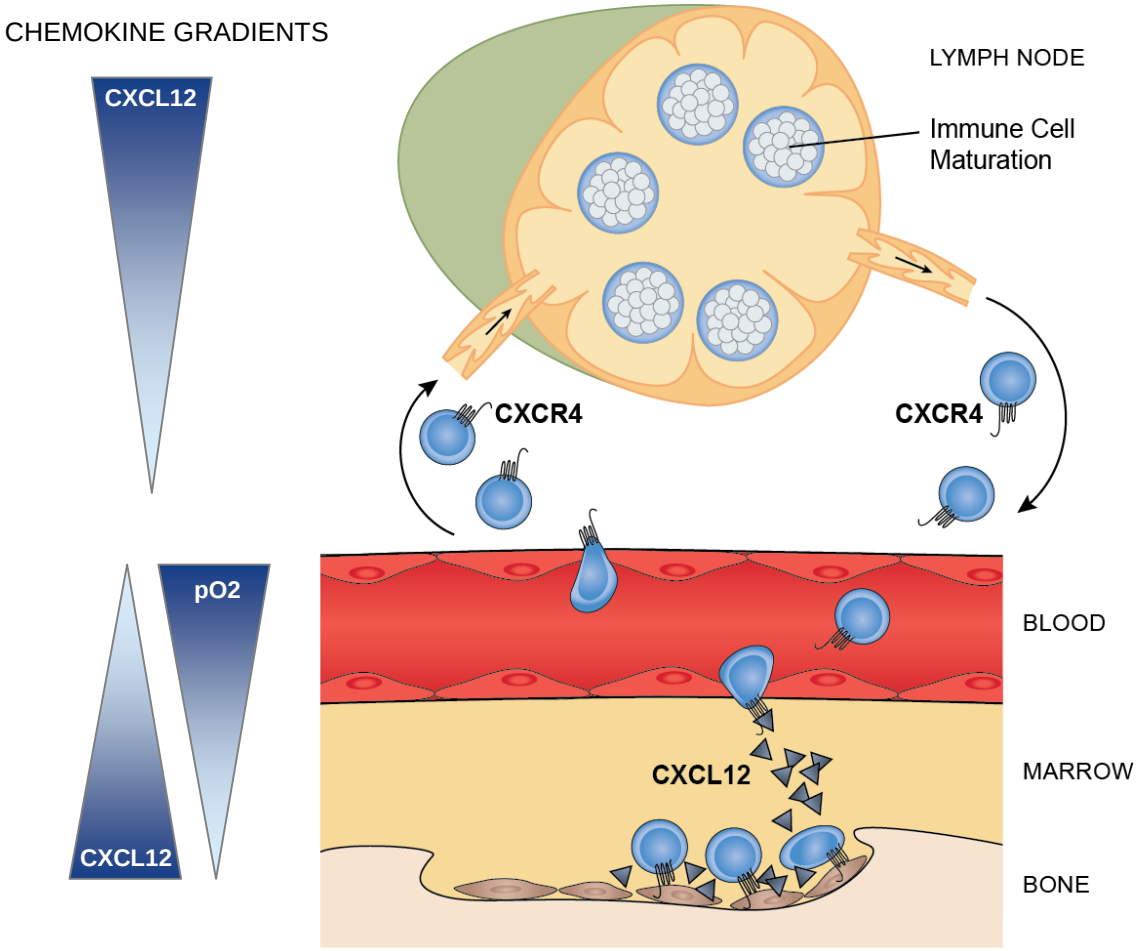
	Mary DiBiase, PhD *VP of Technical Operations and Quality*	Biogen EPIRUS BIOPHARMACEUTICALS
	Celeste DiJohnson *VP of Clinical Operations*	SAREPTA THERAPEUTICS SANOFI
	Tarek Ebrahim, MD *VP of Medical Affairs*	genzyme ALEXION
	Nic Scalfarotto, DVM *VP of Regulatory Affairs*	Aegerion Pharmaceuticals GE Healthcare

Pipeline

Product Candidate	Indication	Stage of Development			
		Preclinical	Phase 1	Phase 2	Phase 3
Mavorixafor (X4P-001)	Warts, Hypogammaglobulinemia, Infections and Myelokathexis (WHIM) syndrome			*Phase 2/3*	
	Severe Congenital Neutropenia (SCN)		**Phase 1**		
	Waldenström's Macroglobulinemia (WM)		**Phase 1/2**		
	Clear cell renal cell carcinoma* (ccRCC) *(Combination with Inlyta®)*			*Phase 2a*	
X4P-002	Glioblastoma multiforme (GBM)				
X4P-003	Primary immuno-deficiencies (PID)				

* Two oncology trials have concluded: P1b biomarker in melanoma and P1b in ccRCC. Final publications expected in 2H19
* Intend to enter into a strategic partnership for future development and potential commercialization for mavorixifor for ccRCC and other potential immuno-oncology indications

CXCR4/CXCL12 and Immune System Responses



Homeostasis
- Neutrophil homing
- Lymphocyte homing
- Dendritic cell trafficking

Infection Response
- Bacterial
- Viruses
- Fungal/Other

Immune Cell Signaling in Bone Marrow
- B cell homing
- Plasma cell homing

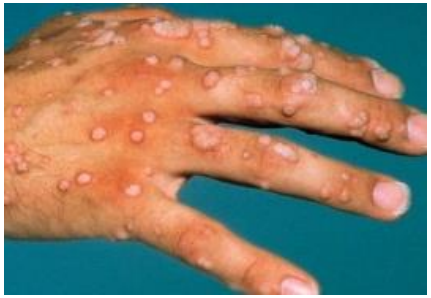
Cancer
- CTL trafficking
- Suppressor cell trafficking

Adapted from *Blood* 2013 121:1501-1509

WHIM Syndrome: Significant Unmet Medical Need

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

- Rare genetic primary immunodeficiency disease that results from “gain-of-function” mutations in the single gene that encodes the CXCR4 receptor
- Patients typically have chronic, critically low white blood cell counts, including neutrophils and lymphoctyes, which are necessary to mount a healthy immune response to bacterial and viral infections
- Debilitating disease progression given current treatment options limited to addressing different symptoms and focused on prevention and management of infections
- WHIM syndrome included in FDA’s guidance (March 2019) for Severely Debilitating or Life-Threatening Hematologic Disorders
- Proof of concept in WHIM previously demonstrated with Mozobil (twice-daily injectable CXCR4 antagonist)[1]



No therapies approved, or to our knowledge, in development to address underlying cause of multi-faceted disease: genetic defect of CXCR4 receptor

1. McDermott et al; Blood, 2014

mavorixafor: Phase 3 Ready for WHIM Syndrome

- Completed open-label, dose escalation Phase 2 trial
  - Clinically meaningful improvement in neutropenia
  - Patients showed improvements in certain other signs and symptoms
  - Favorable tolerability profile
- Five patients continuing to receive mavorixafor in Phase 2 open-label extension (OLE) study
  - Plan to provide future updates
- Randomized, placebo controlled double blinded Phase 3 pivotal trial expected to commence in second quarter 2019
- Diagnosis confirmed genetic testing; >1,000 estimated genetically confirmed WHIM patients in US
- Orphan drug designation received in October 2018 from US FDA and submitted request to EMA in March 2019

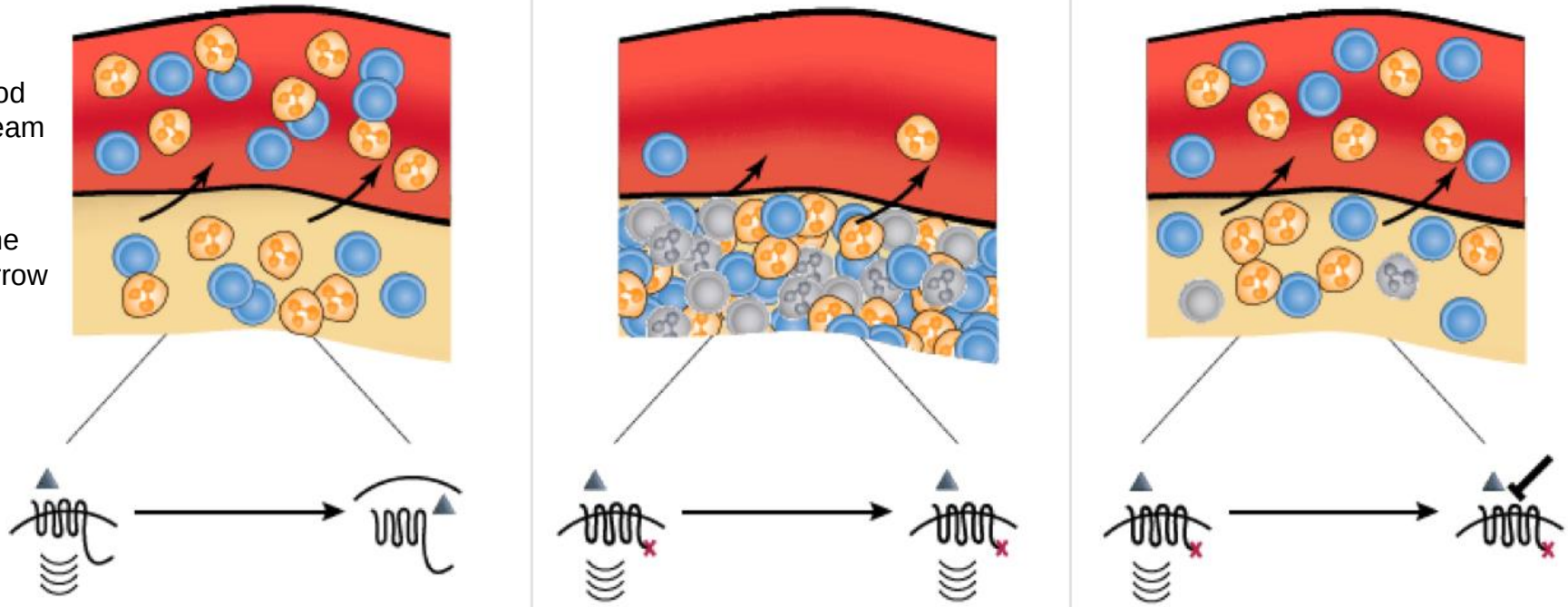
Phase 3 trial design intended to leverage key learnings from Phase 2

WHIM: Genetic Mutations in CXCR4 Create Abnormal Trafficking of White Blood Cells (WBCs)

Normal	WHIM Disease	Goal: Treatment with X4P-001

Blood Stream

Bone Marrow



Normal CXCR4 Signaling

Hyperactive CXCR4 Signaling

Corrected CXCR4 Signaling

WHIM Phase 2 Trial: Assess Neutrophil Counts Biomarker "Time Above Threshold" Metric

Intra-Patient Dose Escalation

- Open label
- 50 mg to 400 mg once daily (QD)
- n = 8 patients

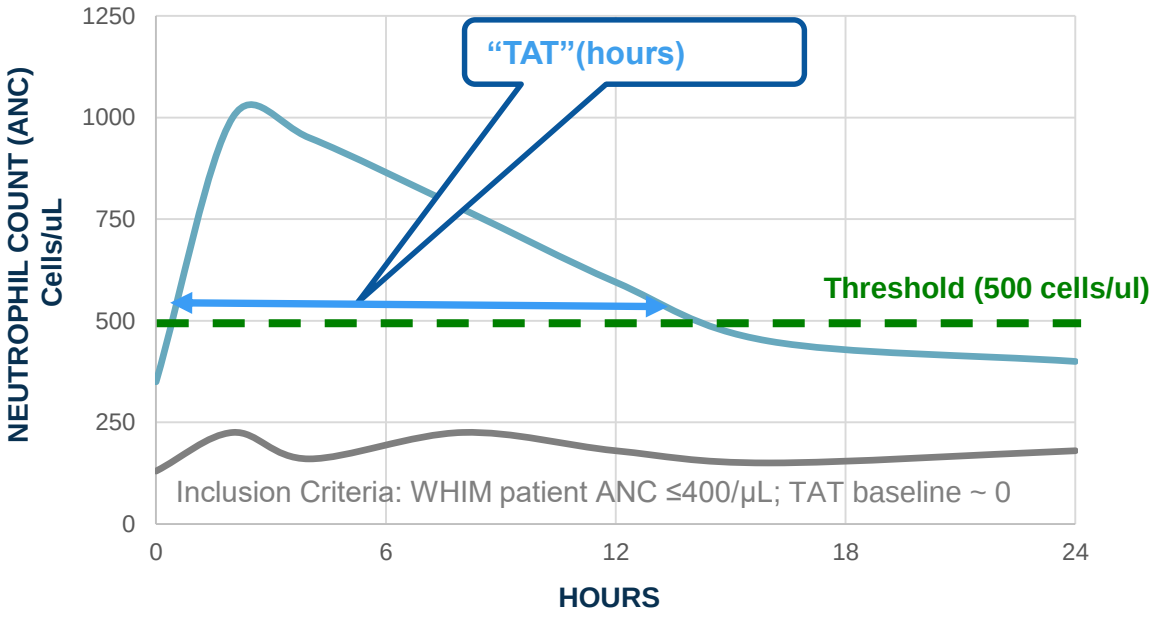
Inclusion

- 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

Objective: Increase Daily Neutrophil Counts (ANC) Above Threshold As Measured Over 24 hours: Time Above Threshold (TAT)



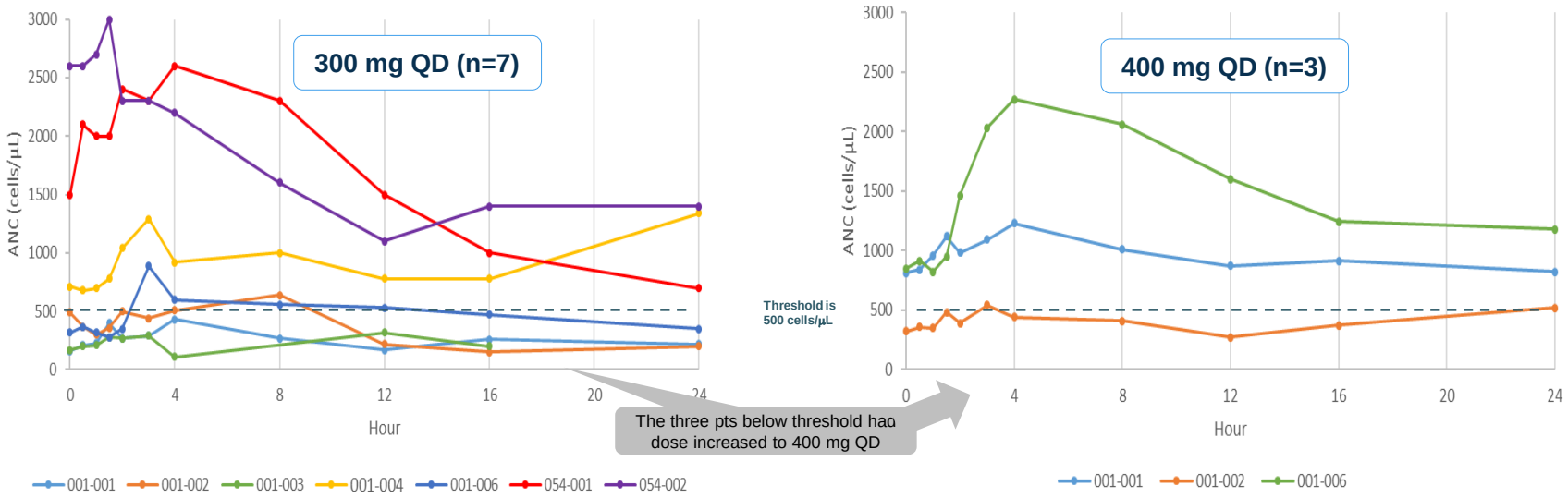
0 hours baseline < TAT < 24 hours maximum

Treatment Goal

Phase 2: Achieved Maximum TAT in Most Patients

Neutrophil and Lymphocytes Mobilized; Pan-Leukopenia Addressed

Patients Started with an ANC of 50 – 200 cells/μL Prior to Treatment

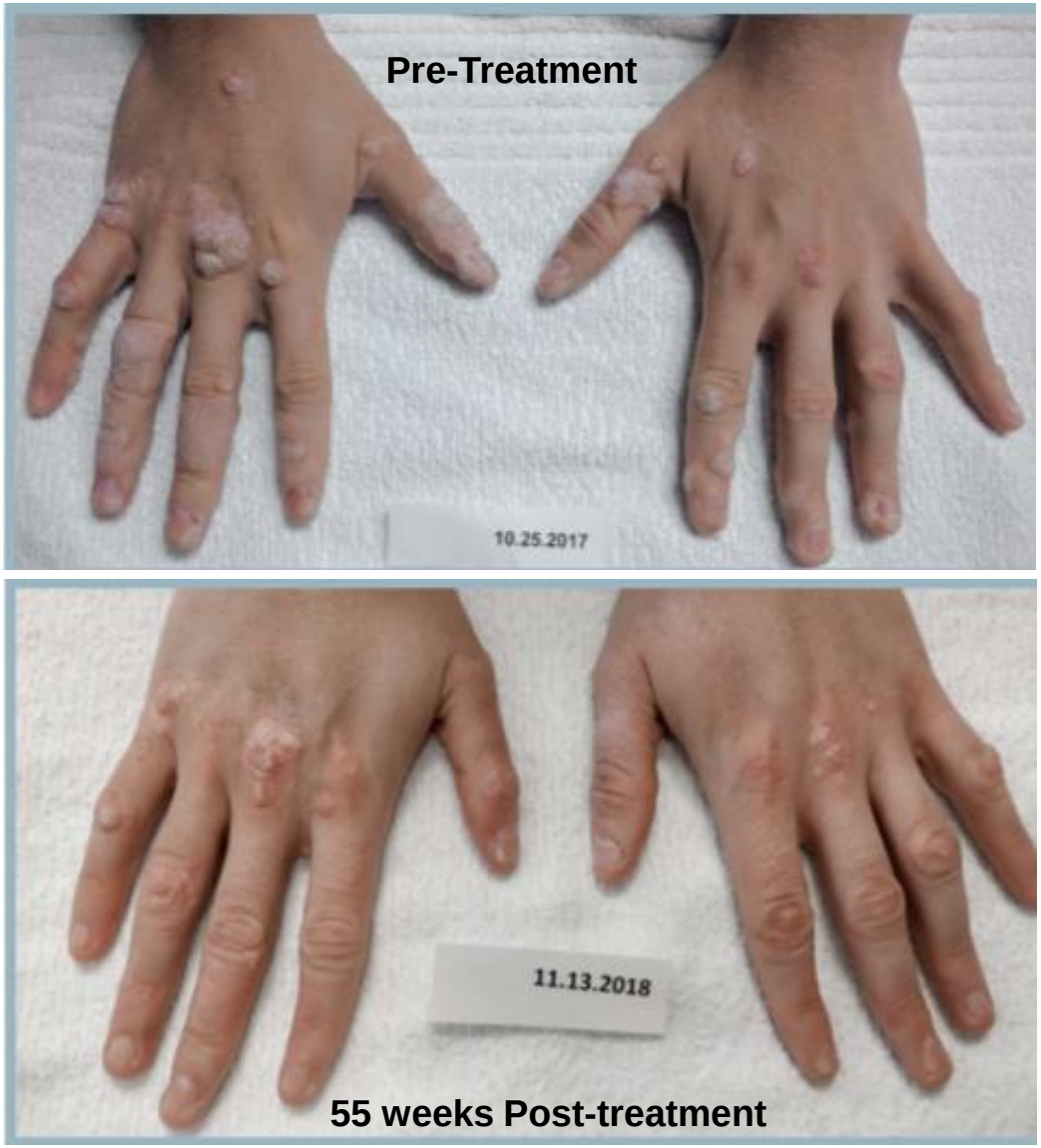


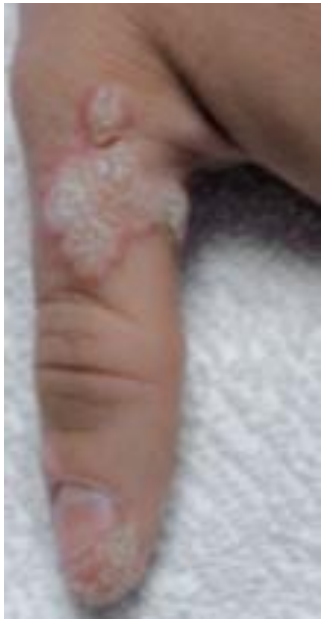
Assessments	Result	
Neutrophil Counts > Threshold	✓	**5 of 7 patients (71%): maximum TAT**
Lymphocyte Counts > Threshold	✓	6 of 7 patients (85%): maximum TAT
Safety	✓	Acceptable; no Grade 3/4

400 mg QD: Phase 3 Pivotal Trial for Patients ≥ 12 years of age

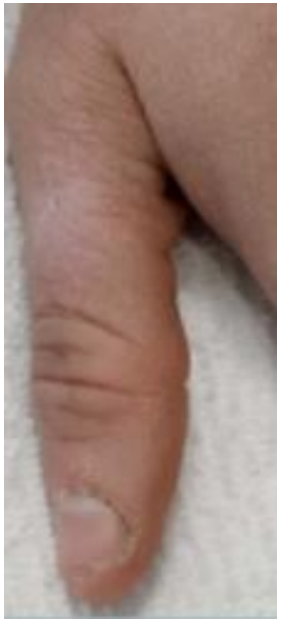
Significant Reduction in Wart Burden Through 55 Weeks

Reductions in Infections Rate Compared to Historical Rates





Pre-Treatment



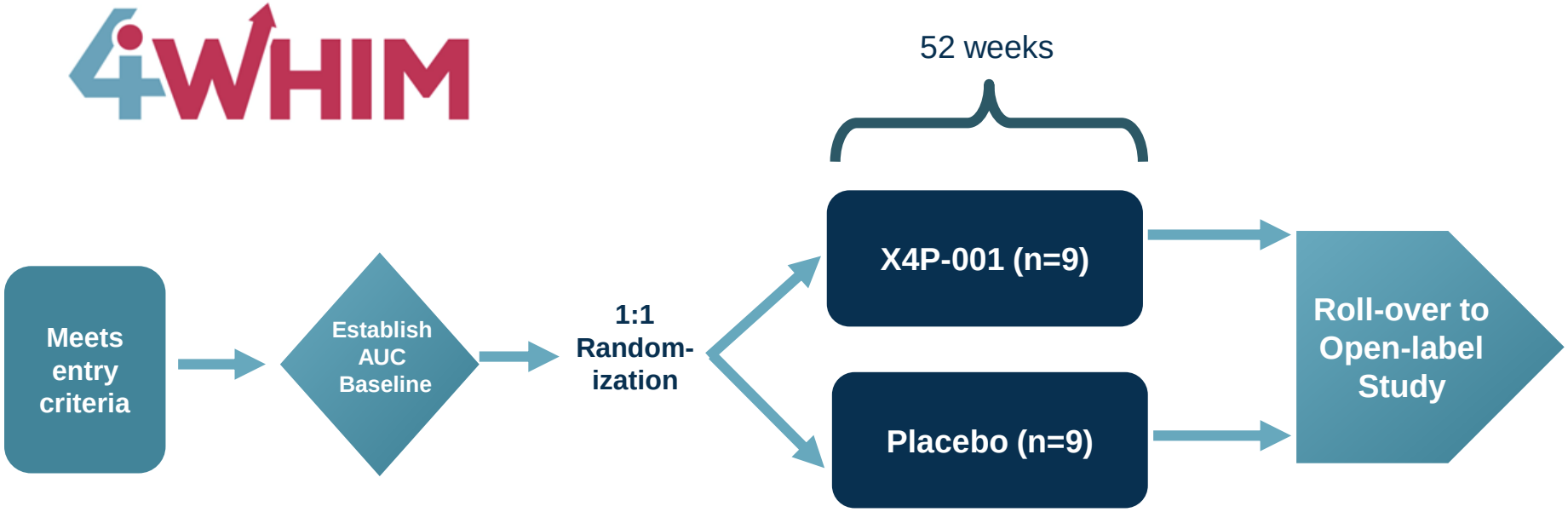
55 weeks Post-treatment

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.

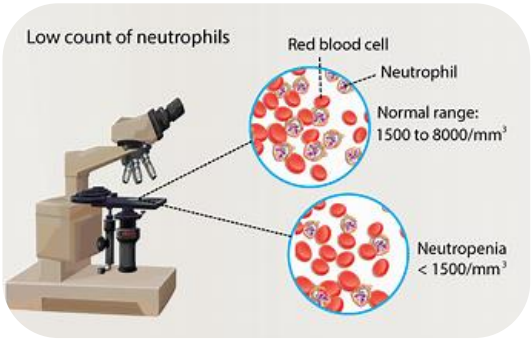
Phase 3 Trial in WHIM Syndrome – Expected 2Q19 Initiation

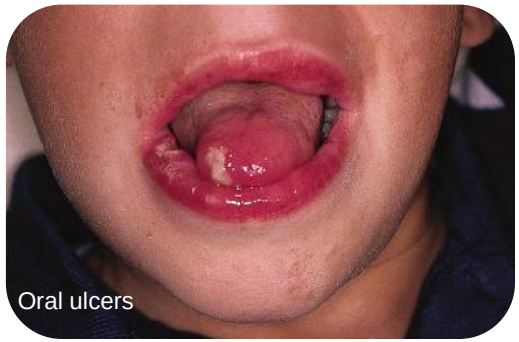


- 400 mg QD dosing in patients 12 years of age or older
- Primary endpoint: biomarker of neutrophil count time above threshold (TAT) where the threshold is defined as 500 cells/uL
- Secondary endpoints include infection rates and wart burden assessments

Primary Immunodeficiency Label Expansion: Phase 1 Trial in Severe Congenital Neutropenia

- 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
- Phase 1 trial planned for 2019 and data expected in mid 2020
  - Designed to determine the genetic profile of adult SCN patients and assess/correlate their pharmacodynamic response to mavorixafor

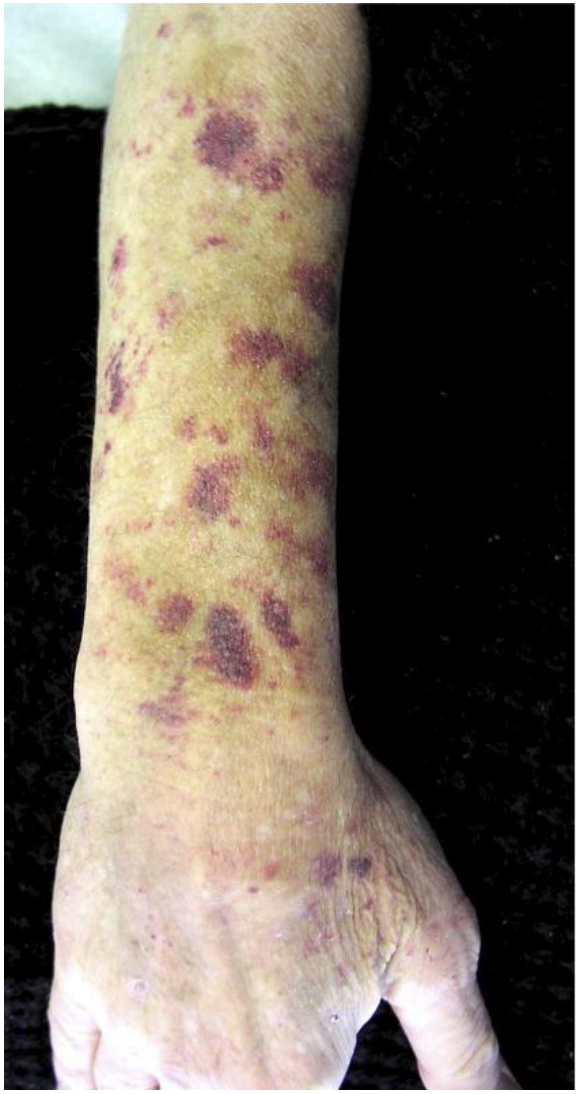




1. https://rarediseases.org/rare-diseases/severe-chronic-neutropenia/ 2. https://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=en&Expert=42738

CXCR4 in Cancer: 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**



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)

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CXCR4^{WHIM} R/R Waldenström's: Poor Clinical Outcomes vs. Wild-type

Phase 1/2 Trial Targets CXCR4-Mutant Population

- 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]

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

1. Table Recreated from: Treon et al, EHA 2018

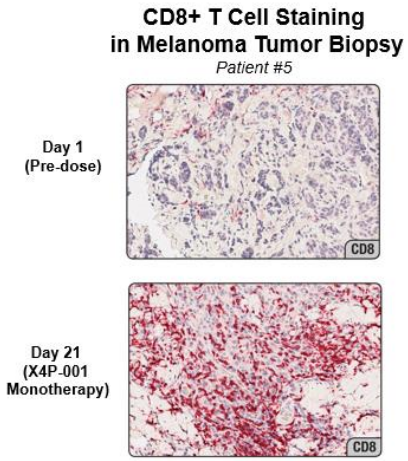
Planned Waldenström's Trial: Double-Mutant R/R WMs

- Inclusion: Patients with MYD88 + CXCR4 mutations who have failed prior Rx
- Design: Multinational 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
- Expected to commence in 2019

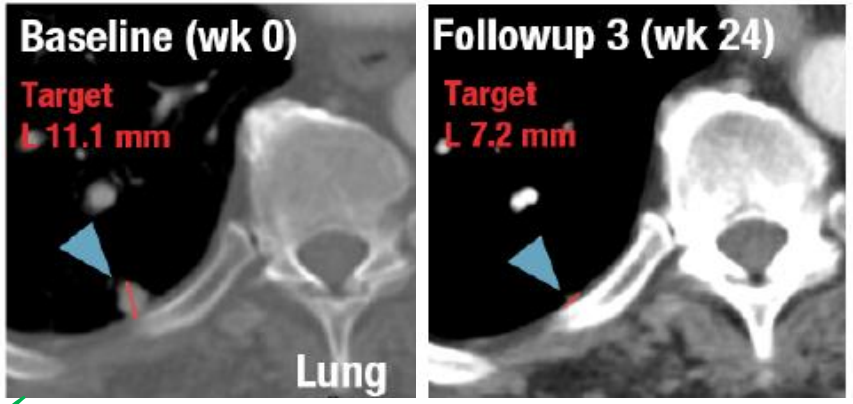
2. Treon et al, EHA 2018 ; 3. Gustine J. Am J Hematol. 2018.

Immuno-Oncology Strategy: Goal of Leveraging Biological Expertise Via Partnering

Completed Trials Demonstrate Single Agent Activity & Proof of Mechanism



✓ Phase 1b In Treatment Naïve Melanoma



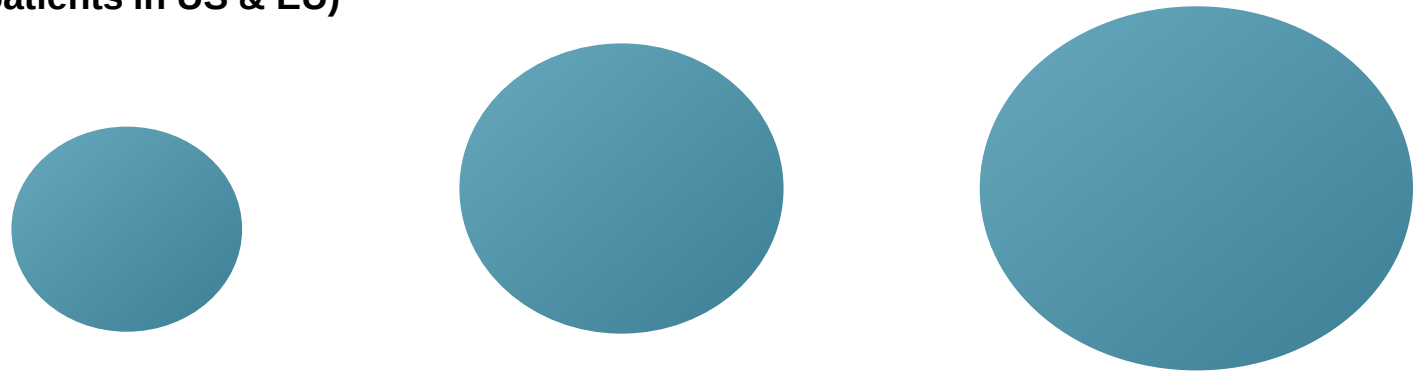
✓ Phase 1b In Progressing RCC with Opdivo

On-Going Phase 2a ccRCC Trial: Mavorixafor + Axitinib

- 65 patients; multi-national, fully enrolled
- Assessment: mPFS
  - 4.8 months mPFS with axitinib in patients with immediate prior TKI
- Data expected: 2H 2019
- Strategy: Identify strategic collaborators to advance in IO; keying off data readout

Epidemiology Suggests Significant Market Opportunity

Clinical Epidemiology
(Estimated patients in US & EU)



>1,000
WHIM Syndrome

2,000 – 3,000
Severe Congenital Neutropenia (SCN)

4,000 – 5,000
Waldenström's Macroglobulinemia (WM)[1]

Partnering with World Class Organizations to Increase Awareness in Primary Immunodeficiencies

Jeffrey Modell Foundation

University of Washington 1861

Immune Deficiency Foundation

Hôpitaux Universitaires Est Parisien
TROUSSEAU LA ROCHE-GUYON

1. Represents CXCR4-mutant patients; 30% to 40% of total WM estimate of 13,000 patients

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Significant Progress Anticipated 2019 to 2021

Target Date	Milestones
2Q 2019	Commence Phase 3 trial in WHIM syndrome
Mid 2019	EMA Orphan Drug Designation for WHIM
2H 2019	Phase 2a ccRCC PFS data readout
2019	Commence Phase 1 trial in SCN
2019	Commence Phase 1/2 in Waldenström's
4Q19-1Q20	WHIM patient identification update
1H 2020	New pipeline molecules: X4P-002 and X4P-003 INDs
Mid 2020	Phase 1 Trial in SCN: topline results
2H 2020	Phase 1/2 in Waldenstrom's – Safety, Dose and Activity
2021	Phase 3 Trial in WHIM – topline results

April 2019

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