X4 PHARMACEUTICALS

INVESTOR PRESENTATION

JUNE 2020

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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 7, 2020, 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 Cambridge, MA with R&D facility in Vienna, Austria

LEADERSHIP:
PROVEN TEAM WITH RARE DISEASE EXPERTISE

MANAGEMENT

PAULA RAGAN, Ph.D.
CEO

genzyme SANOFI

MARY DIBIASE, Ph.D.
SVP of Technical Operations & Quality

Biogen EPIRUS BIOPHARMACEUTICALS

ADAM MOSTAFA
CFO

abpro CANTOR Fitzgerald

NIC SCALFAROTTO, D.V.M.
SVP of Regulatory Affairs

Aegerion Pharmaceuticals GE Healthcare

DEREK MEISNER, J.D.
General Counsel

genocea RACapital

SHARIQ ALI, Ph.D.
VP of Medical Affairs

WAVE LIFE SCIENCES SANOFI genzyme

RENATO SKERLJ, Ph.D.
CSO

genzyme LTI

MICHELE RHEE, M.P.H., M.B.A.
VP of Patient Advocacy

ENZYVANT Takeda ONCOLOGY

BOARD OF DIRECTORS

MICHAEL WYZGA, Chairman
genzyme Radius

BILL ALISKI
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GARY BRIDGER, Ph.D.
AnorMED genzyme

DAVID MCGIRR, MBA
CUBIST PHARMACEUTICALS arsanis

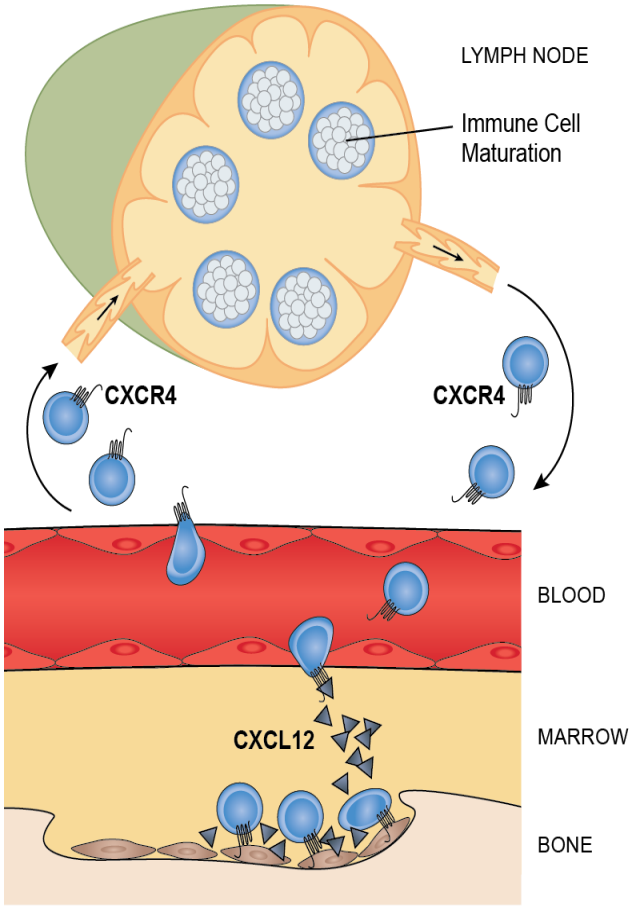
PAULA RAGAN, Ph.D.
genzyme SANOFI

MURRAY STEWART, M.D.
rhythm gsk

RENE RUSSO, PharmD, BCPS
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**

** Exploring potential strategic partnership(s) for future development and potential commercialization for mavorixifor for ccRCC and other potential immuno-oncology indications*

OVERVIEW: MAVORIXAFOR

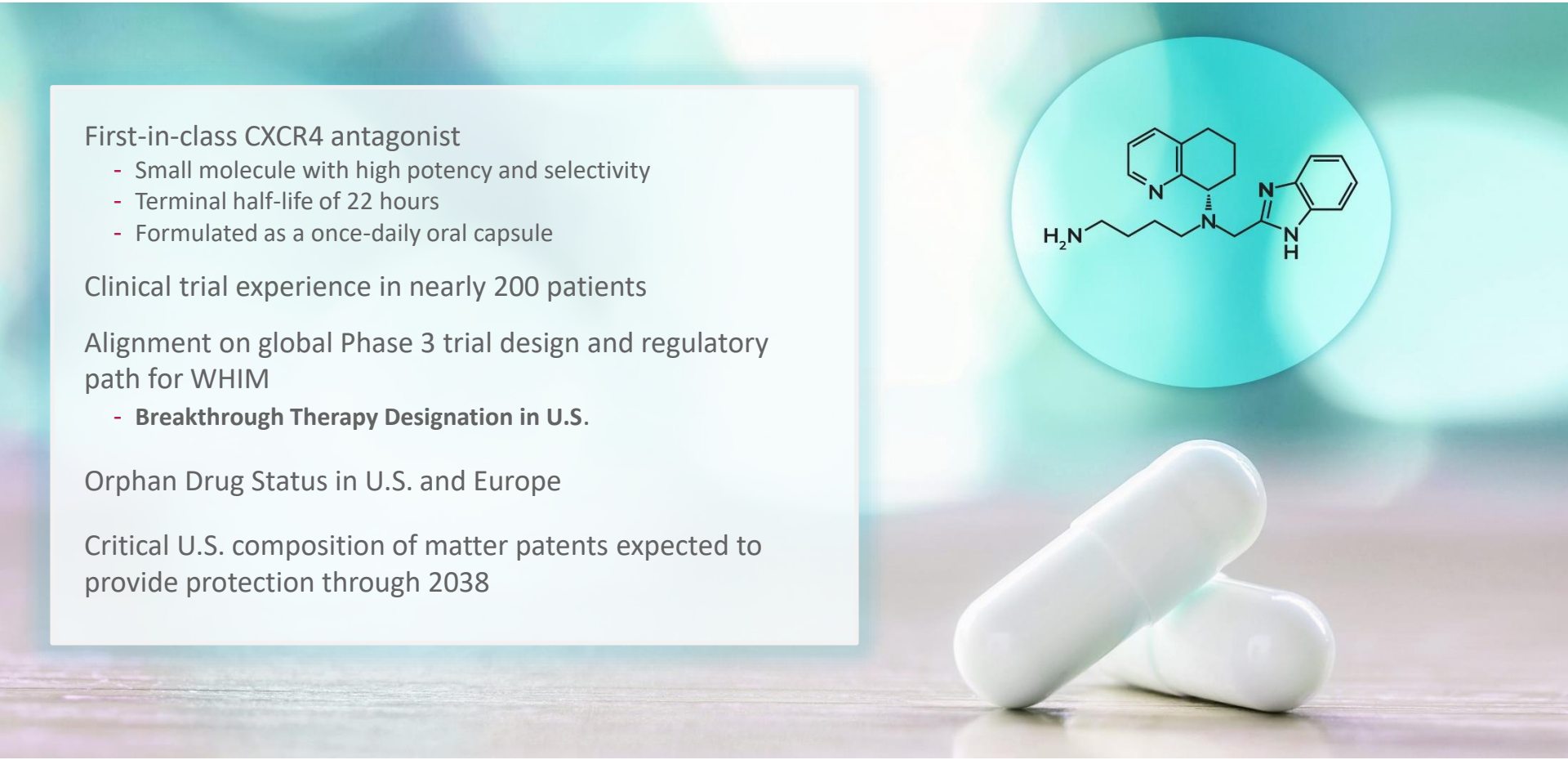
First-in-class CXCR4 antagonist
- Small molecule with high potency and selectivity
- Terminal half-life of 22 hours
- Formulated as a once-daily oral capsule

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.**

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		
	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

ABOUT WHIM SYNDROME

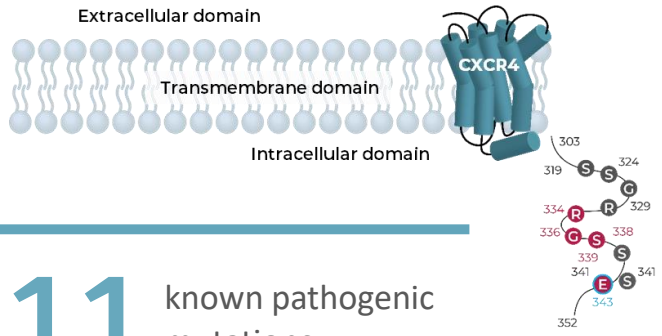
W **Warts** – can lead to HPV-related cancers

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



>3,500[1]

estimated U.S. WHIM population

0

Approved targeted therapies

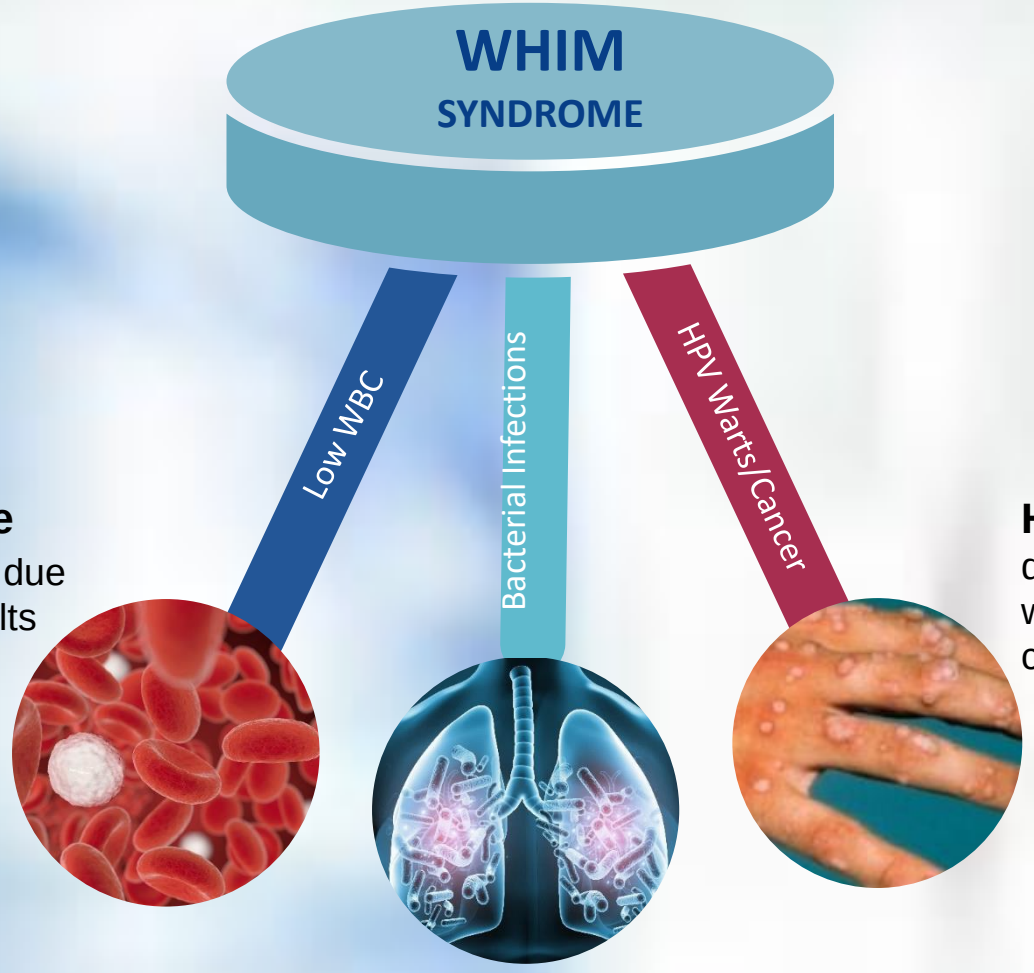
Symptomatic Rx; antibiotics, G-CSF, immunoglobulins

11 known pathogenic mutations

Genetic test to diagnose

1. Qessential Market Research 2019; IPM.ai AI research study, 2020

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

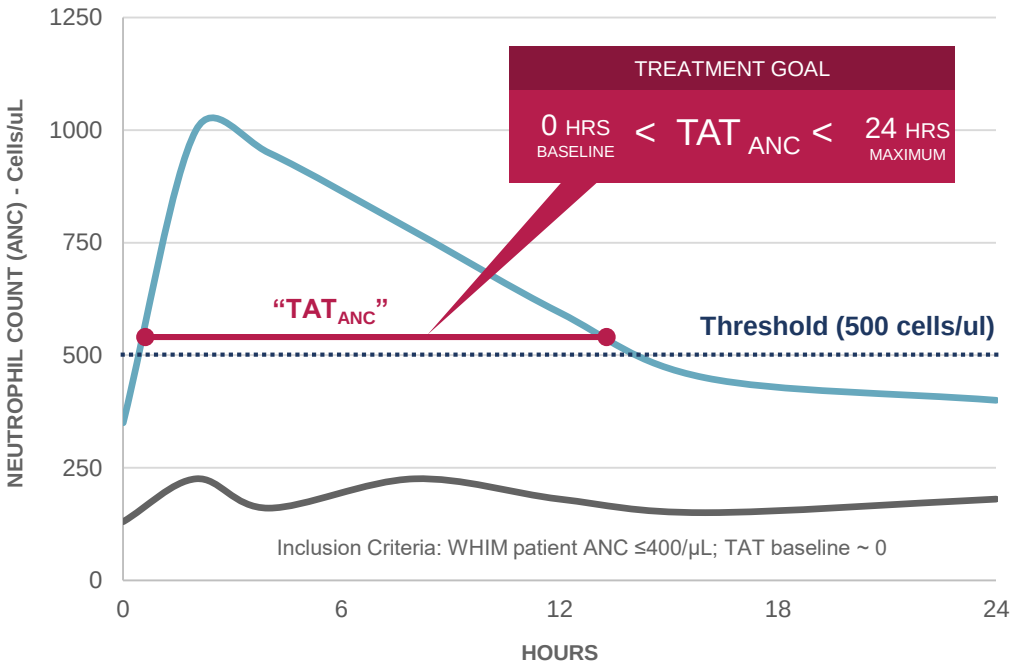
DOSE ESCALATION + OPEN-LABEL EXPANSION

- 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

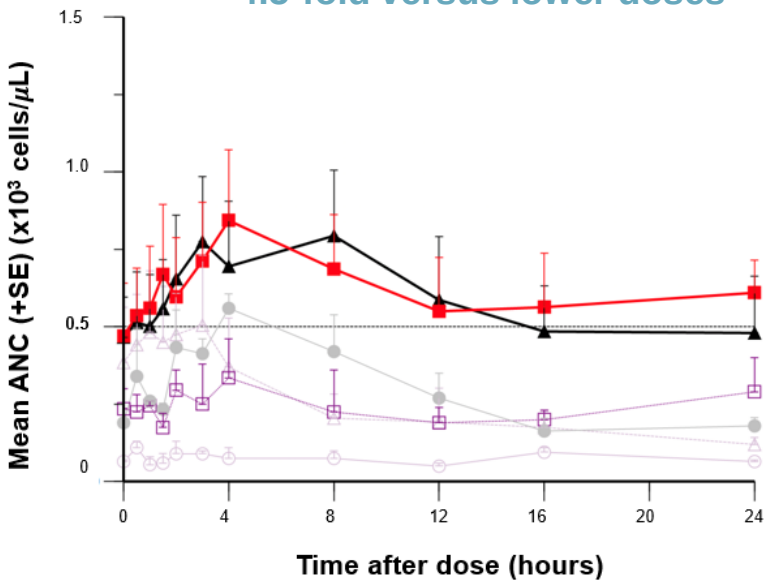


OBJECTIVE: INCREASE DAILY NEUTROPHIL COUNTS (ANC) ABOVE THRESHOLD AS MEASURED OVER 24 HOURS: TIME ABOVE THRESHOLD (TAT)

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

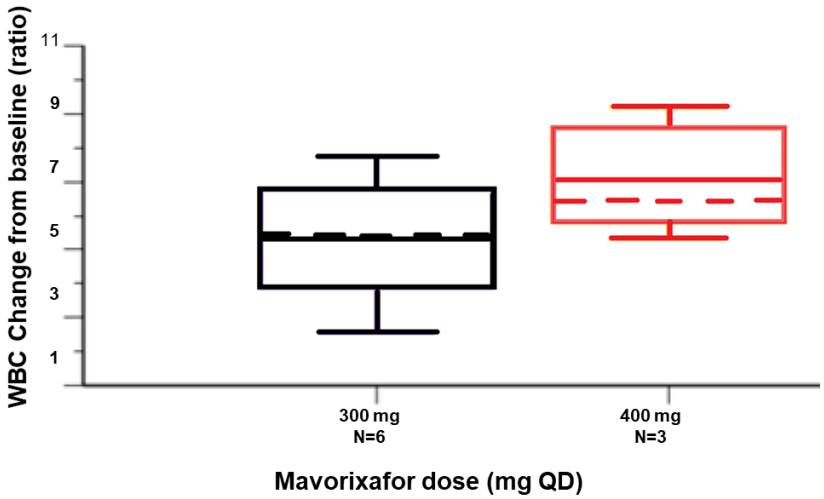
- Mavorixafor 400 mg orally once daily was well tolerated for >2 years without attributable serious AEs
- Durable, dose-dependent increases of WBC, ANC, and ALC counts
- TAT_{ANC} is an objective and consistent biomarker of clinical response to CXCR4 antagonist therapy
  - Primary endpoint in ongoing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

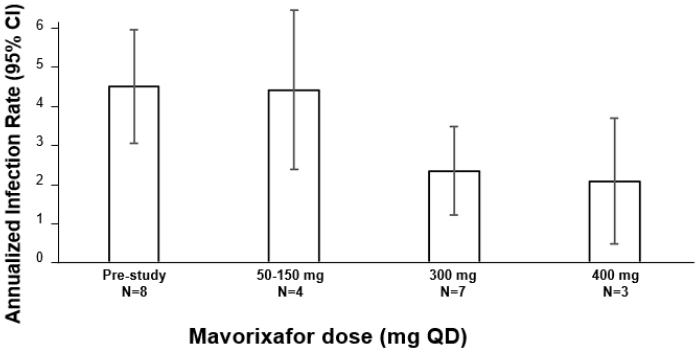
- Durable, dose-dependent increases of WBC, ANC, and ALC counts led to clinical benefits

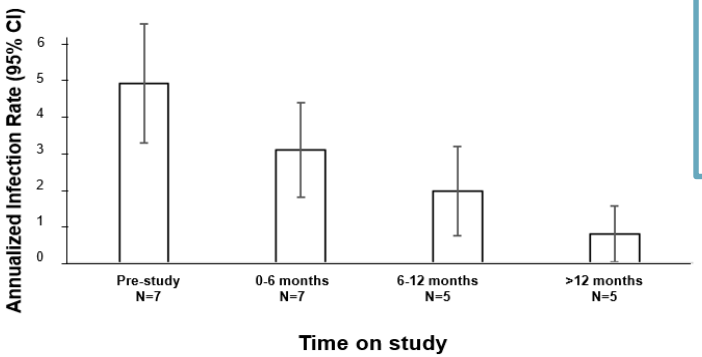
INFECTION RATES

- 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

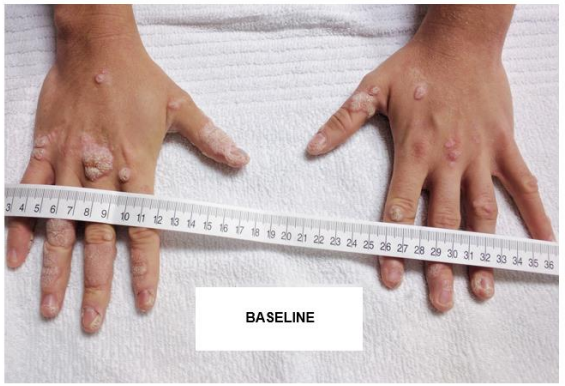
WART BURDEN

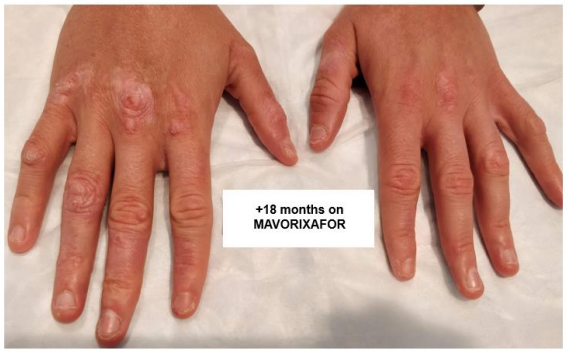
- Average 75% reduction in the number of warts
- Baseline vs. 18 months, following 14 months on 400 mg mavorixafor





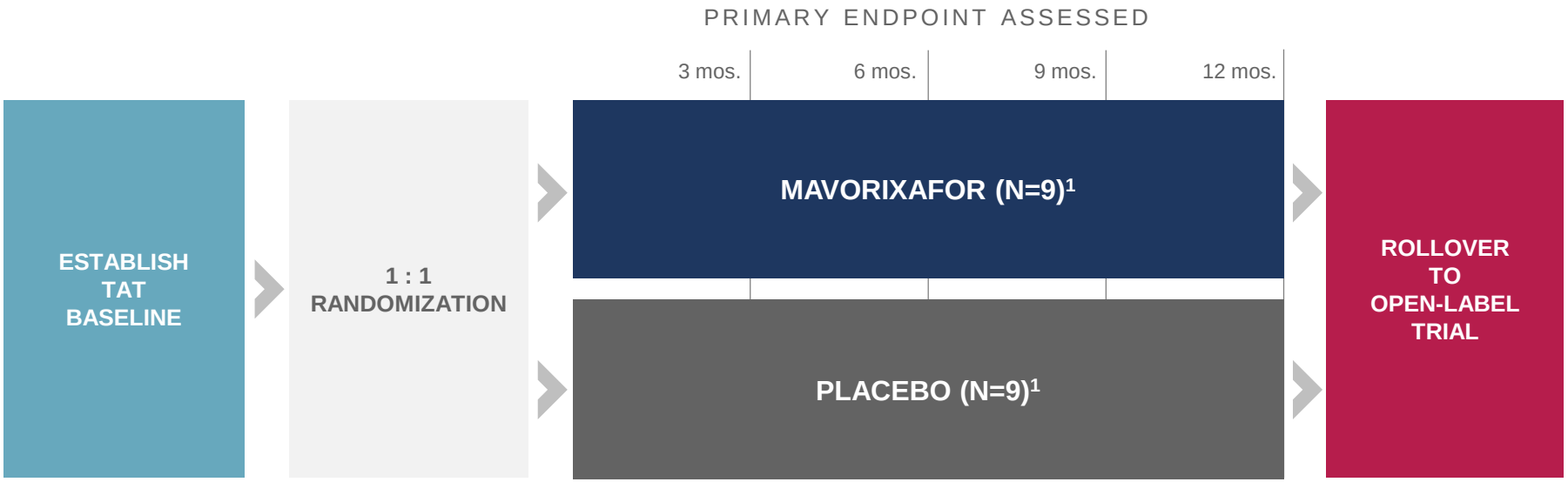
Two key secondary endpoints in ongoing pivotal Phase 3 trial





Data from X4 poster presentation at 2020 EHA

GLOBAL REGISTRATIONAL PHASE 3 TRIAL IN WHIM SYNDROME

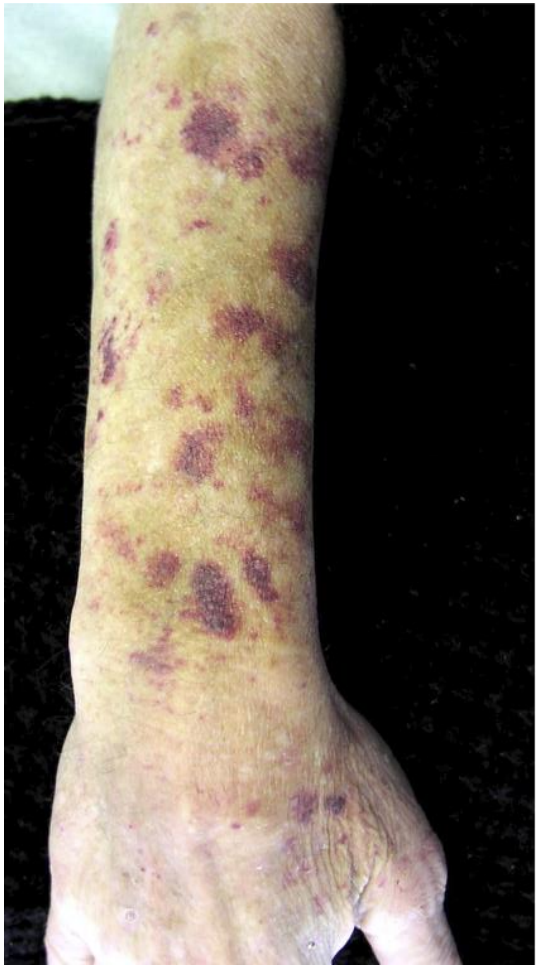


- **Primary Endpoint:** Biomarker of neutrophil count time above threshold (TAT) where the threshold is defined as 500 cells/uL; average of four assessment timepoints
- **Secondary Endpoints:** Infection rates and wart burden assessments
- **Dosing:** 400mg QD in patients 12 years of age or older
- **Enrollment:** 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

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

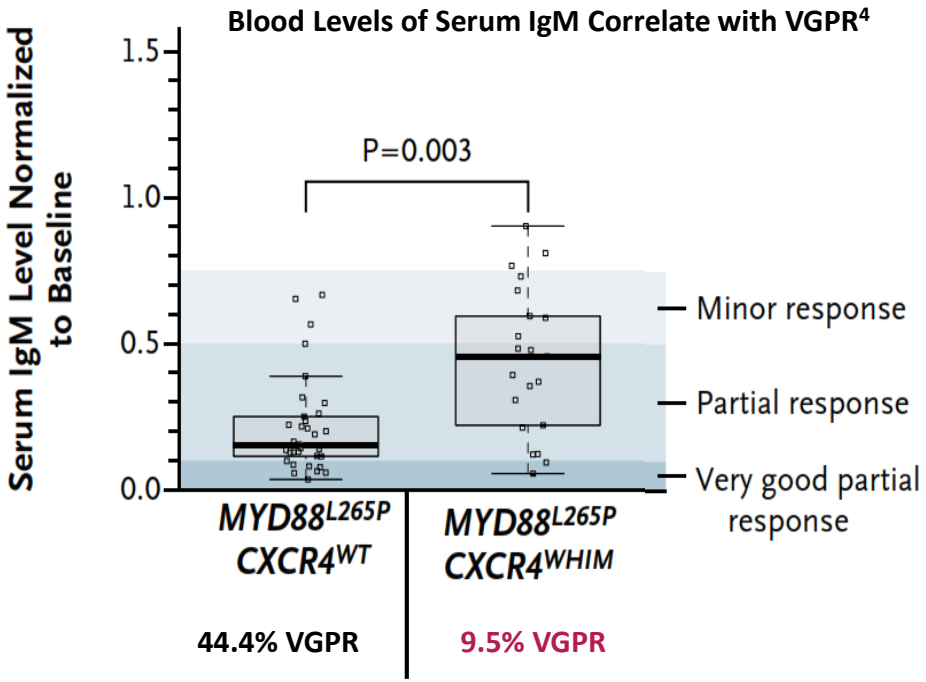
[1] *Sekhar J, et.al.. Waldenström macroglobulinemia: a Surveillance, Epidemiology, and End Results database review from 1988 to 2005. Leuk Lymphoma 2012;53(8):1625-1626;*
[2] *https://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=33226*

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

- Very Good Partial Response (VGPR) Rates: **9.5% for CXCR4-mutant vs. 44.4% 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 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



1. Table Recreated from: Treon et al, EHA 2018; 2. Treon et al, EHA 2018 ; 3. Gustine J. Am J Hematol. 2018; 4. Figure extracted and adapted from Treon et al, NEJM, 2015

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 other blood parameters

Timing: Initial data in 2H 2020

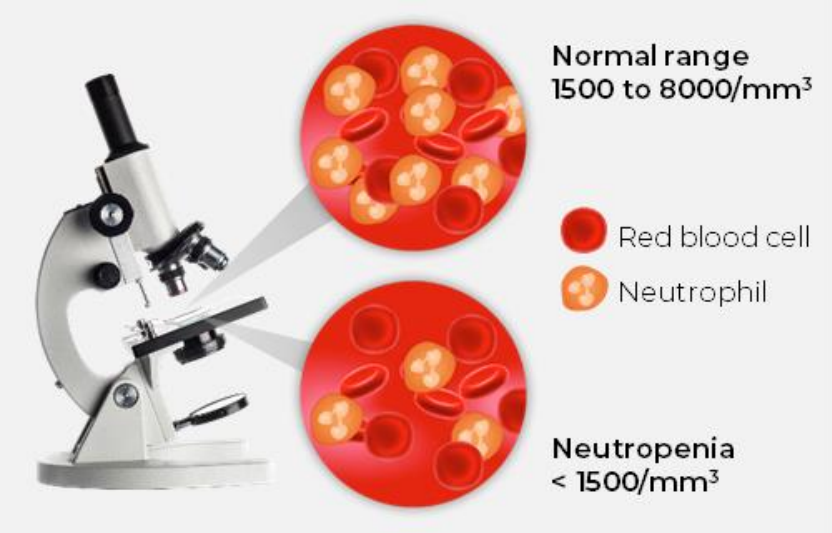
LEUKEMIA & LYMPHOMA SOCIETY®

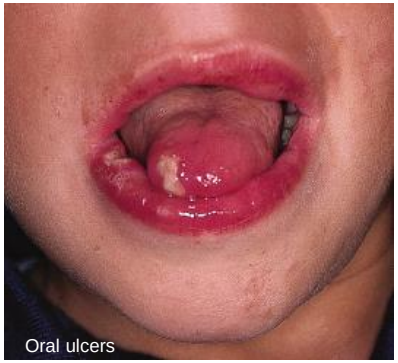
- Strategic collaboration with Leukemia & Lymphoma Society (LLS)
- Selected for LLS' Therapy Acceleration Program

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




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] Qessential Market Research 2019 and IPM.ai, 2020 - number of potential undiagnosed represents estimates for US only from AI study
[2] 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 https://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=en&Expert=42738

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

COMPLETED AND ANTICIPATED MILESTONE ACHIEVEMENTS

TARGET DATE	MILESTONES
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 prevalence update: raised guidance ✓
Mid-2020	Positive WHIM Phase 2 open-label extension data presented at EHA ✓
2H 2020	Phase 1b trial in Waldenström's: initial data readout
2021	Phase 1b trial in SCN: initial results
2022	Phase 3 trial in WHIM: topline results

SELECTED FINANCIAL INFORMATION

$117.0M[1]

Cash Expected to Fund Operations into 2022

Share and Warrant Information:

- **20.0M shares outstanding**
  (16.1M common 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
HCW H.C.WAINWRIGHT&CO.

[1] As of March 31, 2020, as reported in the Company's form 10-Q filed with the SEC on May 7, 2020. Cash figure does not include potential additional borrowing availability of $25 million under amended credit agreement with Hercules Capital, Inc. and $3.0 million milestone payment received from Abbisko Therapeutics in April 2020.

X4 PHARMACEUTICALS

INVESTOR PRESENTATION

JUNE 2020

APPENDIX

IMMUNO-ONCOLOGY STRATEGY:
PARTNERSHIPS TO CAPTURE GLOBAL VALUE

COMPLETED TRIALS DEMONSTRATE SINGLE AGENT ACTIVITY & PROOF OF MECHANISM

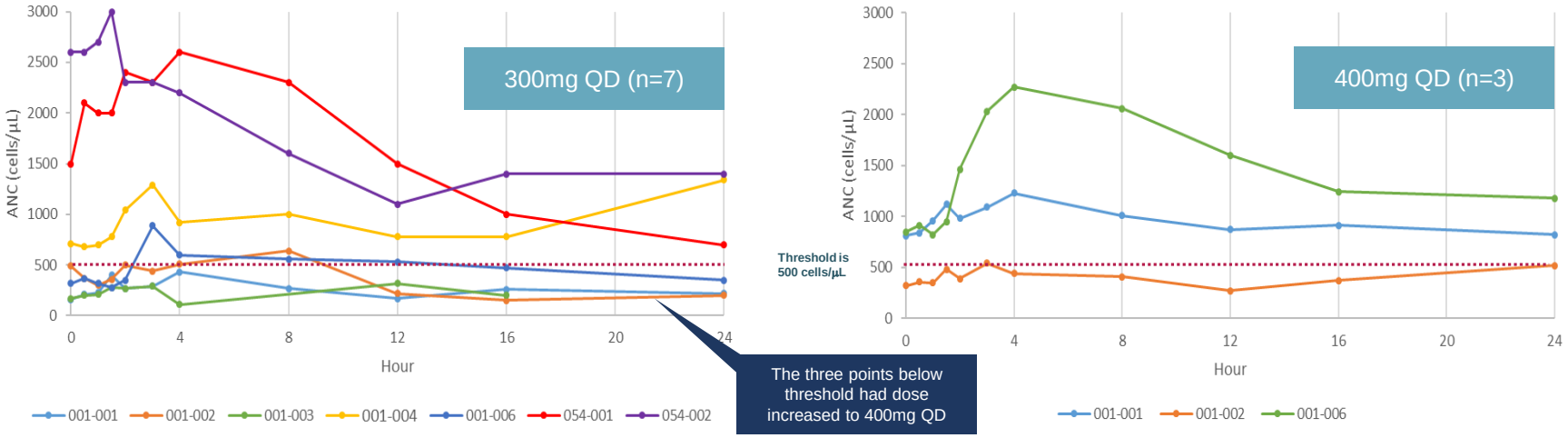
POSITIVE DATA FROM PHASE 2A ccRCC TRIAL: MAVORIXAFOR + AXITINIB PRESENTED AT ESMO 2019

- **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

WHIM PHASE 2 DOSE ESCALATION:
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 chosen for ongoing pivotal Phase 3 clinical trial