



PRECISION ONCOLOGY: PTX-100 AND PTX-200

FROM ROBUST SCIENCE TO EVIDENCED-BASED CANCER TREATMENT

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Chief Scientific Officer
Prescient Therapeutics Limited (ASX: PTX)

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INTRODUCING SAID SEBTI, CSO OF PRESCIENT



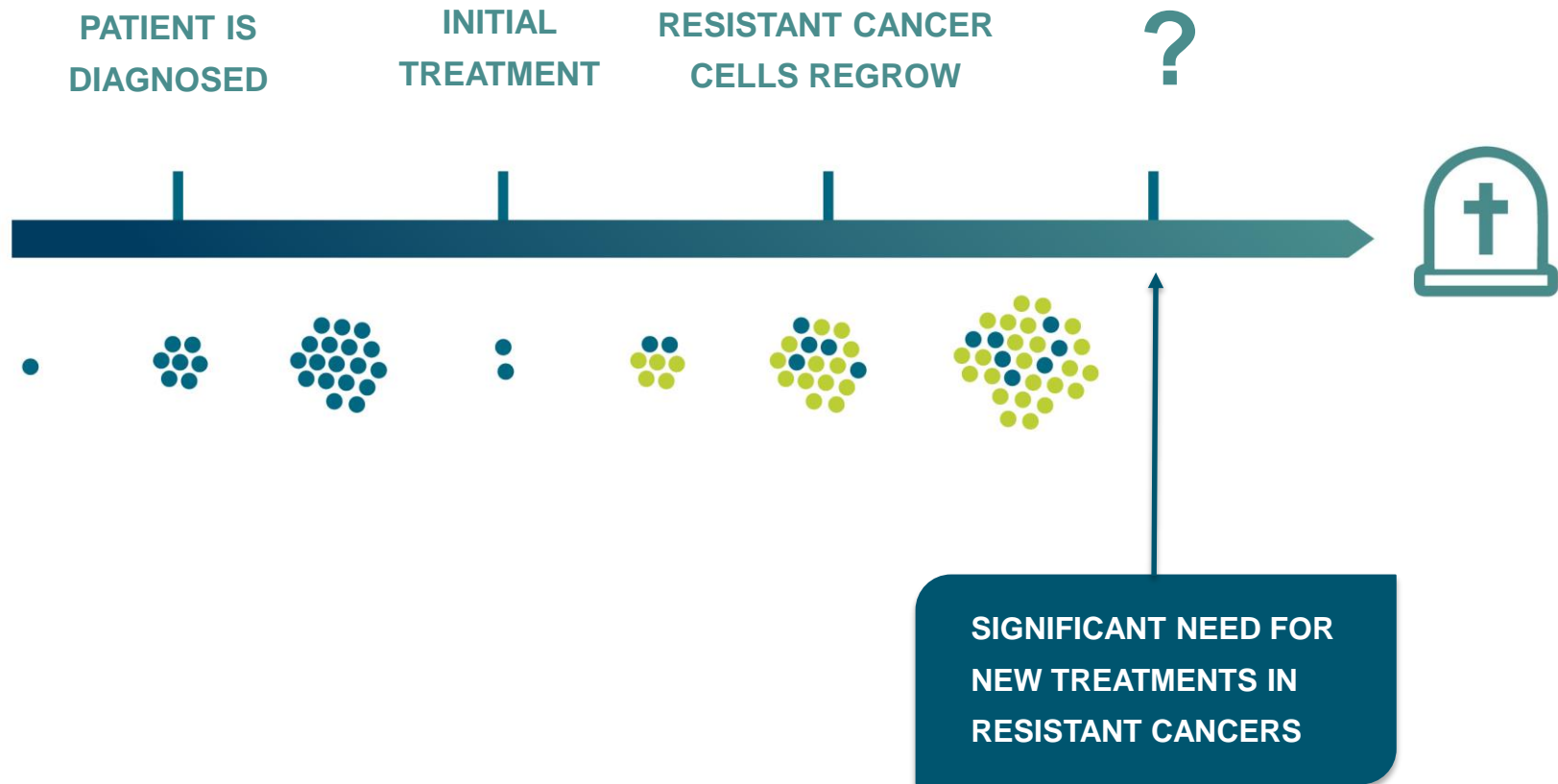
- Chief Scientific Officer, Prescient Therapeutics
- Professor and Chair, Drug Discovery Department, Moffitt Cancer Center in Tampa, FL
 - » 3rd largest cancer centre in the US
 - » 800 research scientists, postdocs, graduate students and support staff
- Co-inventor of PTX-100 & PTX-200
- Named among Top 20 Translational Researchers in the world by Nature Publishing Group



COMPANY OVERVIEW



WHAT DOES PRESCIENT DO?



INVESTMENT HIGHLIGHTS

2 DRUGS » IMMINENT CATALYSTS » FUNDING IN PLACE » UNDISCOVERED VALUE

- **Multiple shots on goal** with novel targeted (personalized) cancer therapies
- One of **deepest clinical pipelines** on the ASX
- Funded through to value-accretive catalysts, with a fantastic share register
- Great scientific and clinical team with a **proven record of success**
- **Transformative opportunity** in Ras/Rho mutant cancers
- Following in the footsteps of US targeted therapy companies that have enjoyed spectacular success
- **Multiple catalysts** this year
- **Encouraging Ph1b breast cancer results**

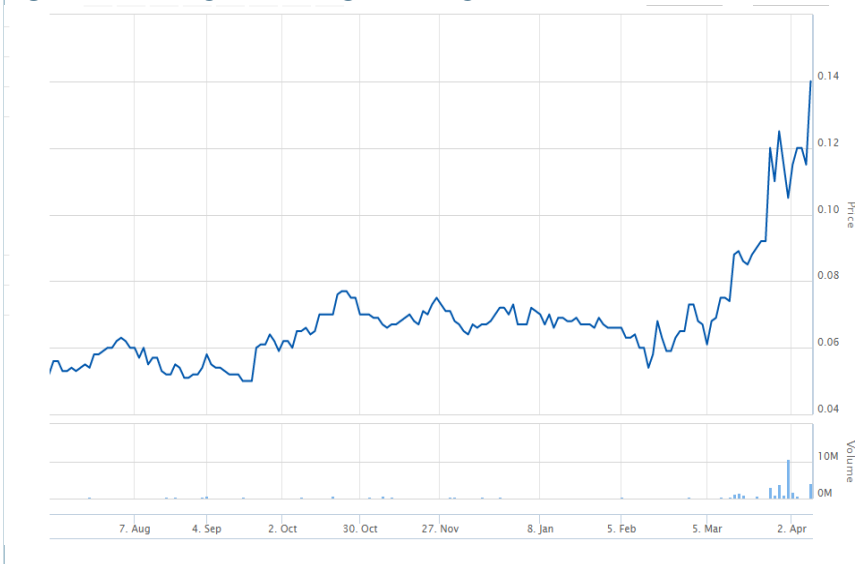
CORPORATE SNAPSHOT

KEY METRICS

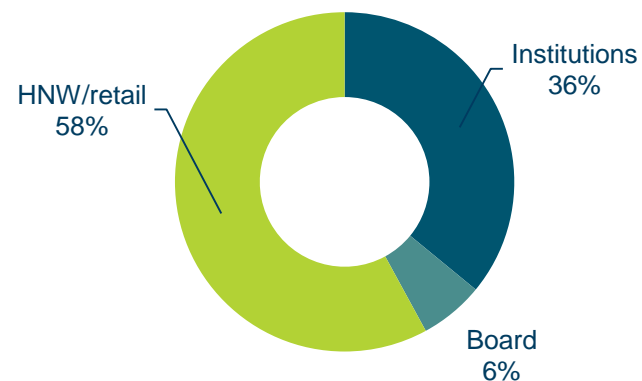
ASX Ticker	PTX
Total Issued Capital	211.3 M shares
Options	57.8 M
Share Price ¹	A\$0.12 (US\$0.09)

Market Capitalisation ¹	A\$25 M (US\$19.5 M)
Cash Position ²	A\$6.0 M (US\$4.7 M)
Top 20 Own	52%
6 month turnover ³	22.7 M shares; A\$1.6 M (US\$1.3 M)

SHARE PRICE PERFORMANCE

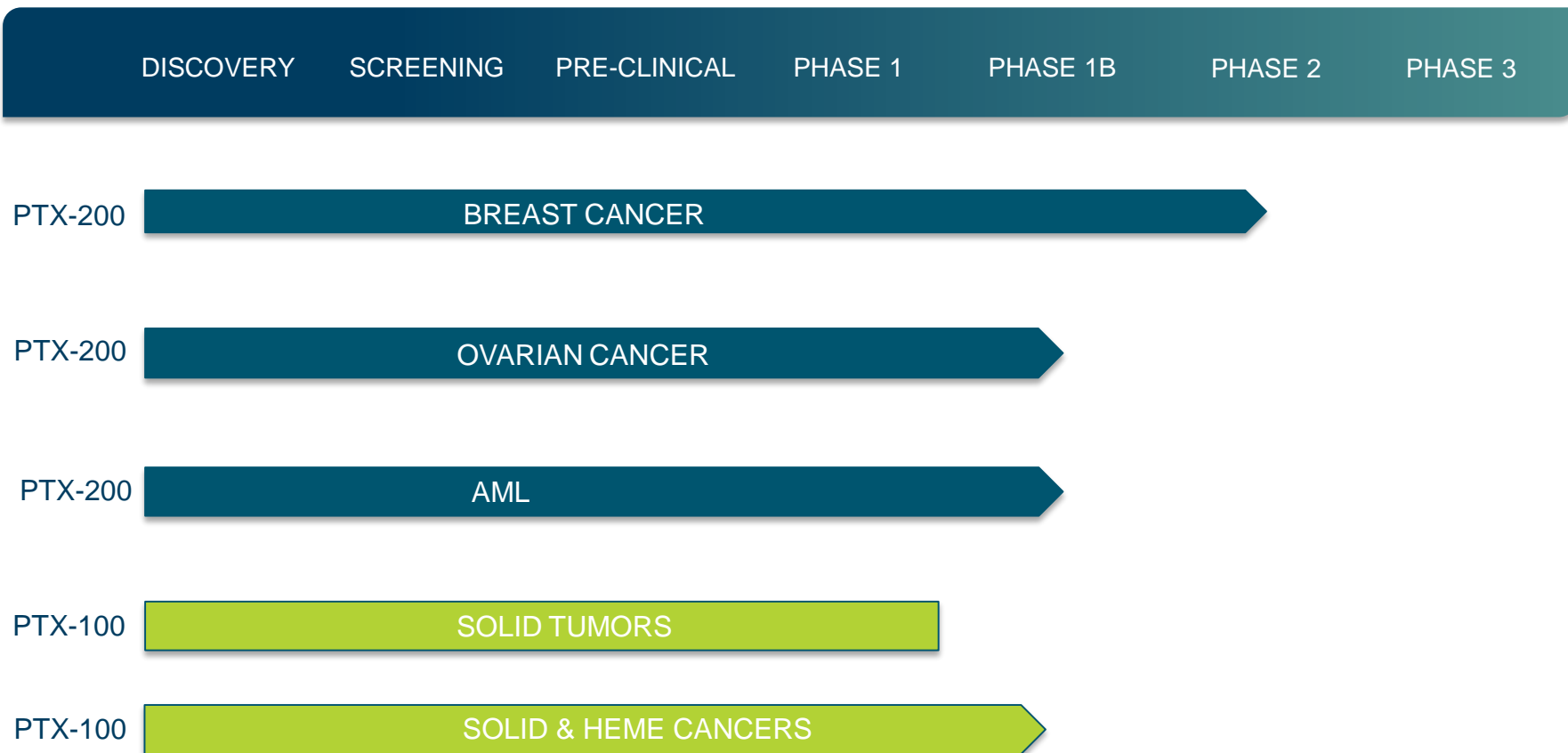


SHAREHOLDER BASE



DEEP, CLINICAL STAGE PRODUCT PIPELINE

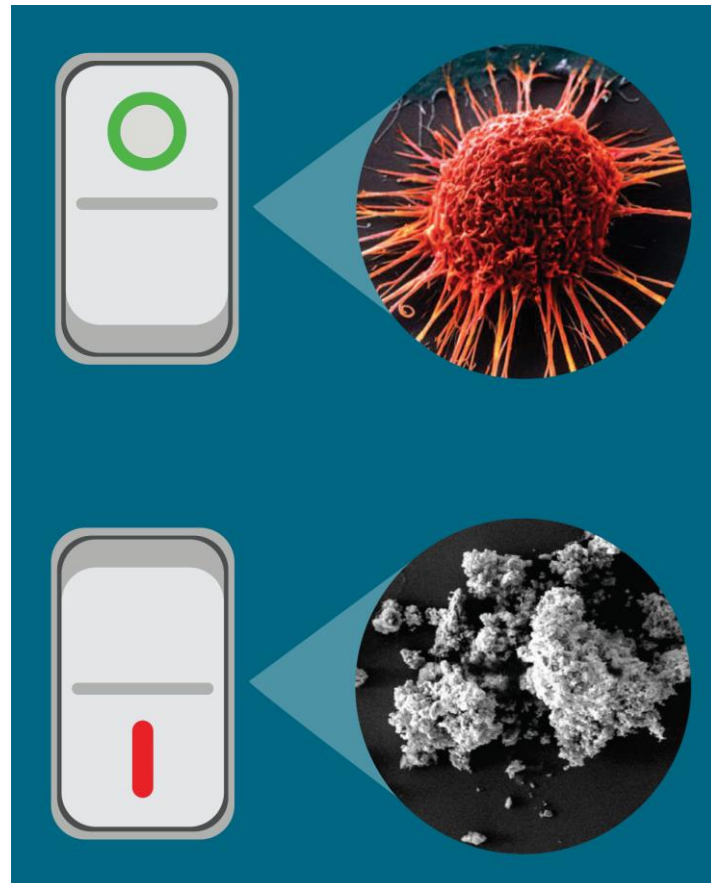
- PTX-200 currently in three clinical trials
- Advancing PTX-100 in basket studies of Ras and Rho mutant cancers - a transformative opportunity



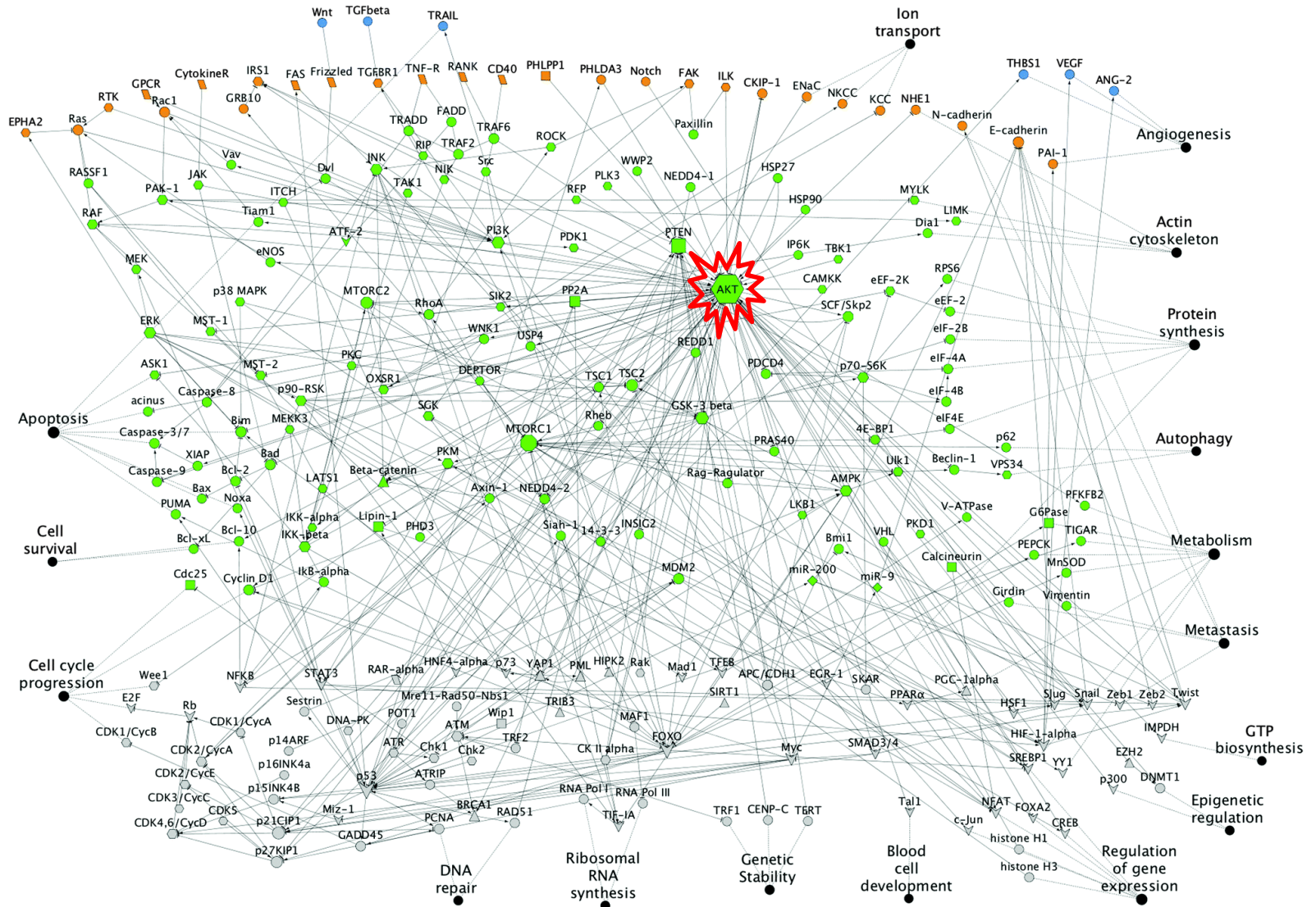
HOW OUR DRUGS WORK: “MOLECULAR SWITCHES”

Akt, Rho & Ras are growth molecules – when they are stuck “on”, they send **constant signals** to the cancer cell to **grow** and **cause resistance** to treatment

PTX’s drugs block the Akt, Rho & Ras growth pathways, switching the **growth signals off** and **causing the cancer cell to die**



TURNING OFF TUMOUR MASTER SWITCHES

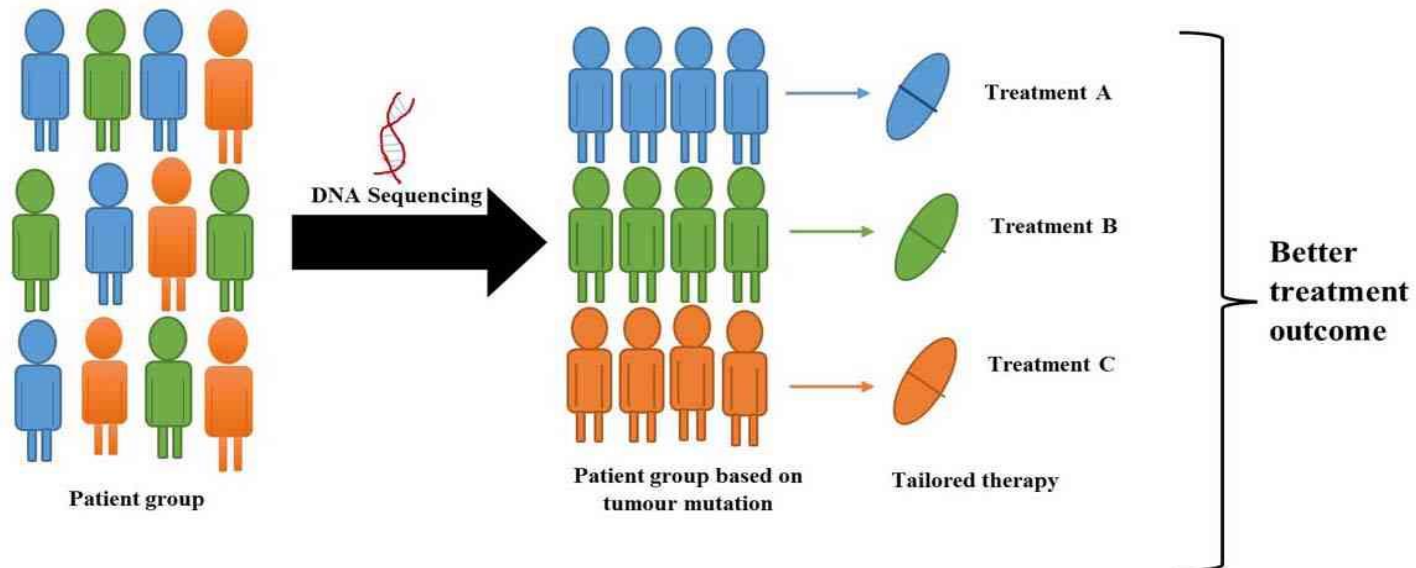


PRECISION ONCOLOGY EXPLAINED



PRECISION ONCOLOGY INTRODUCTION

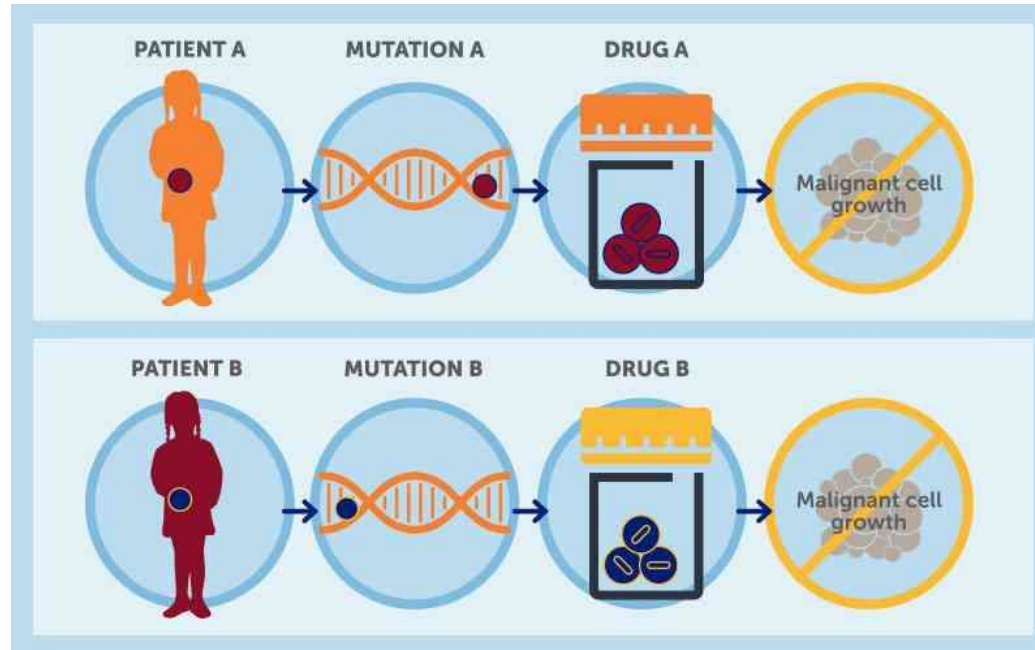
- Treatment takes into account **individual tumor variability**
- Doctors predict more accurately **which anti-cancer drug will work** in which groups of people
- **Not a one-size-fits-all approach** where treatment is for all patients, with no attention to differences between individuals
- This practice has precedent: For example, in blood transfusion the donor's blood type is matched to the recipient (no one is given blood from a randomly selected donor).



- The Precision Medicine industry was valued at **US\$39B in 2015**, and is expected to reach **US\$98B by 2023**, expanding at a rate of 12.3%

WHY PRECISION ONCOLOGY?

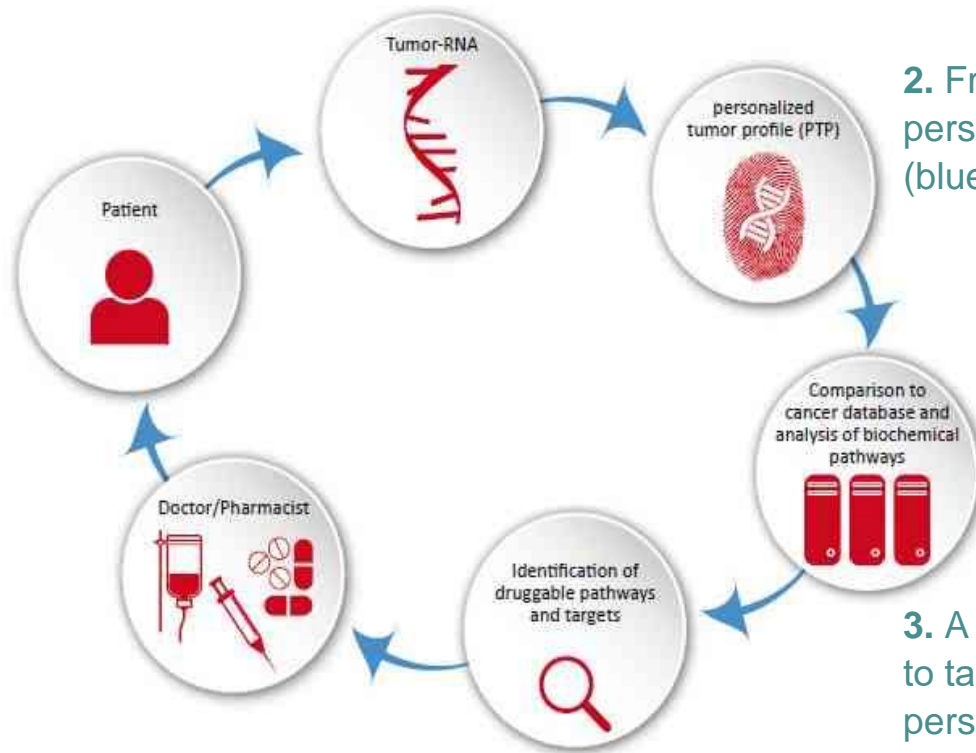
- Many types of cancers (breast, ovarian, lung, colon etc); within same cancer type, cancer patients' tumors can differ from one another: Tumors arise from different cancer-causing mutations and proteins.



- Precision Oncology tailors the treatment of a cancer patient with specific drugs that target the specific cancer-causing mutations or proteins that define the blueprint of that patients' individual tumor.

STEPS INVOLVED IN PRECISION ONCOLOGY

1. Each cancer patient tumor is first analyzed for mutations & other molecular biomarkers (i.e. DNA/RNA sequencing; Phosphorylation)



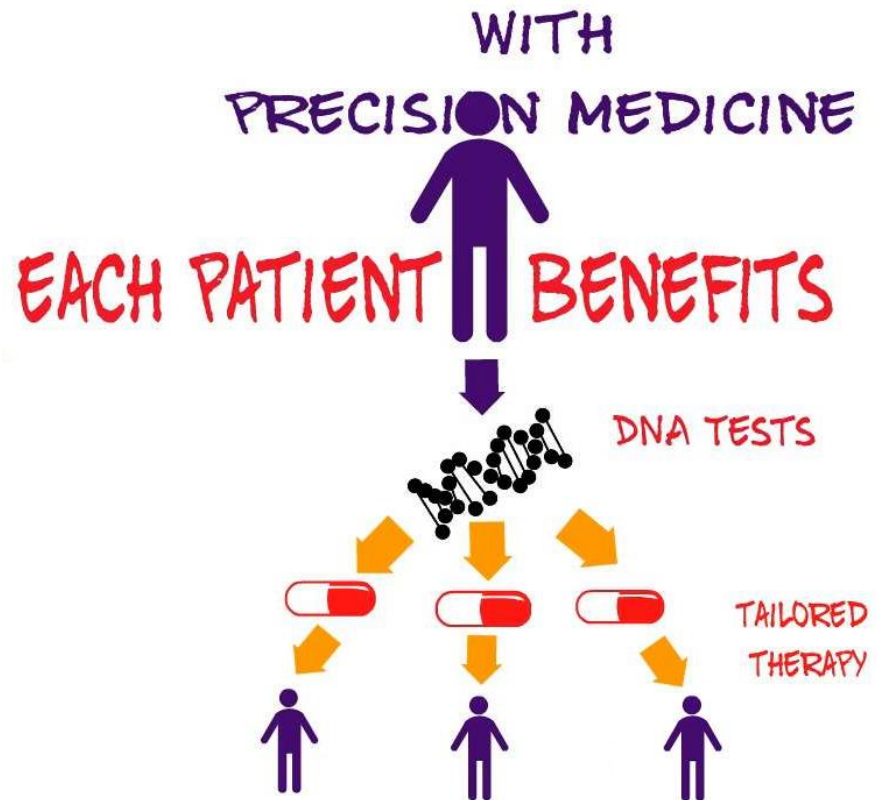
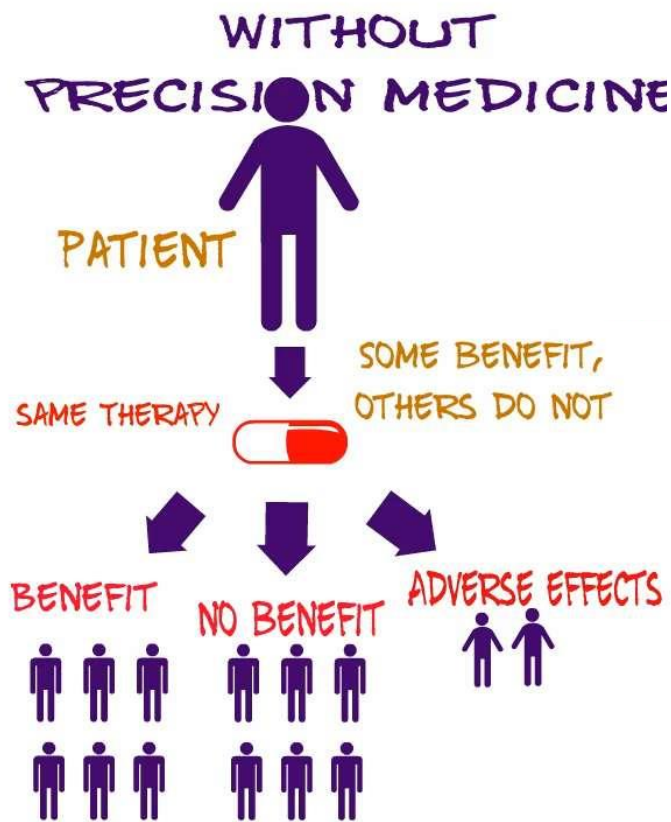
2. From this analyses a personalized tumor profile (blueprint) is obtained

3. A drug is then selected to target the tumor's personalized tumor profile

This way, only patients that are more likely to respond are treated with the **right drug** for the **right patient** with the **right tumor profile**

BENEFITS VS STANDARD TREATMENTS








This **precise, targeted** personalized therapy is a **more effective** way of treating cancer patients.



SUCCESS STORIES IN PRECISION ONCOLOGY



EXAMPLES OF PRECISION ONCOLOGY APPROACHES

Disease	Target	Drug	Status
Chronic Myeloid Leukemia	Bcr-Abl (95% of patients)	Gleevec 	Gleevec has increased 5-year survival rate for CML from 31% to 90% (1990s-2012)
Acute Myeloid Leukemia	FLT3 (25% of AML patients)	Gilteritinib 	NDA filed for R/R FLT3-positive AML
Acute Myeloid Leukemia	IDH2 (8% of AML patients)	Enasidenib 	Received FDA approval in 2017 for R/R AML with IDH2 mutations
Many cancer types	TRK fusions (various %s)	LOXO-101 	NDA filed for cancers with TRK fusion
Many cancer types	High p-Akt (various %s)	PTX-200 	In development for cancers with high p-Akt levels (regardless of the genetic defect that causes high p-Akt)
Various Lymphomas	RhoA mutations (up to 70%)	PTX-100 	In development for cancers with RhoA mutations
Gastric and pancreatic cancers	KRas mutations (up to 95%)	PTX-100 	In development for cancers with KRas mutations

TARGETED THERAPIES CASE STUDY #1: LOXO

Bayer agrees cancer drug deal with Loxo worth up to \$1.55 bln

November 14, 2017, 10:08:00 AM EDT By Reuters

- Loxo Oncology (NASDAQ: LOXO); US\$3.9B market cap
- TRK inhibitor targeting cancers with TRK fusions
- Deal for LOXO-101 and LOXO-195
- In mid-stage clinical trials ORR 75%
- Bayer deal - US\$400m upfront
- Loxo could earn up to US\$1.55 Billion
- Bayer and Loxo co-promote in US; Bayer solo RoW
- **923% return** in 4 years



TARGETED THERAPIES CASE STUDY #2: BLUEPRINT

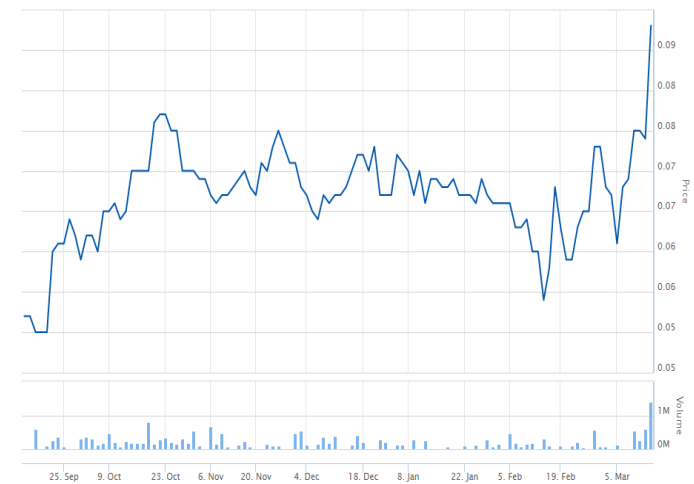


- Blueprint Medicines (NASDAQ: BPMC)
- Target therapies for cancer based on genomically defined diseases (abnormal kinase activation)
- 4x Phase 1 drugs
- 3 x discovery programs
- IPO April 2015: Valuation: US\$398M; \$16/share
- Today: Valuation: US\$3.8 B; \$78/share
- **342% return** in <3 years

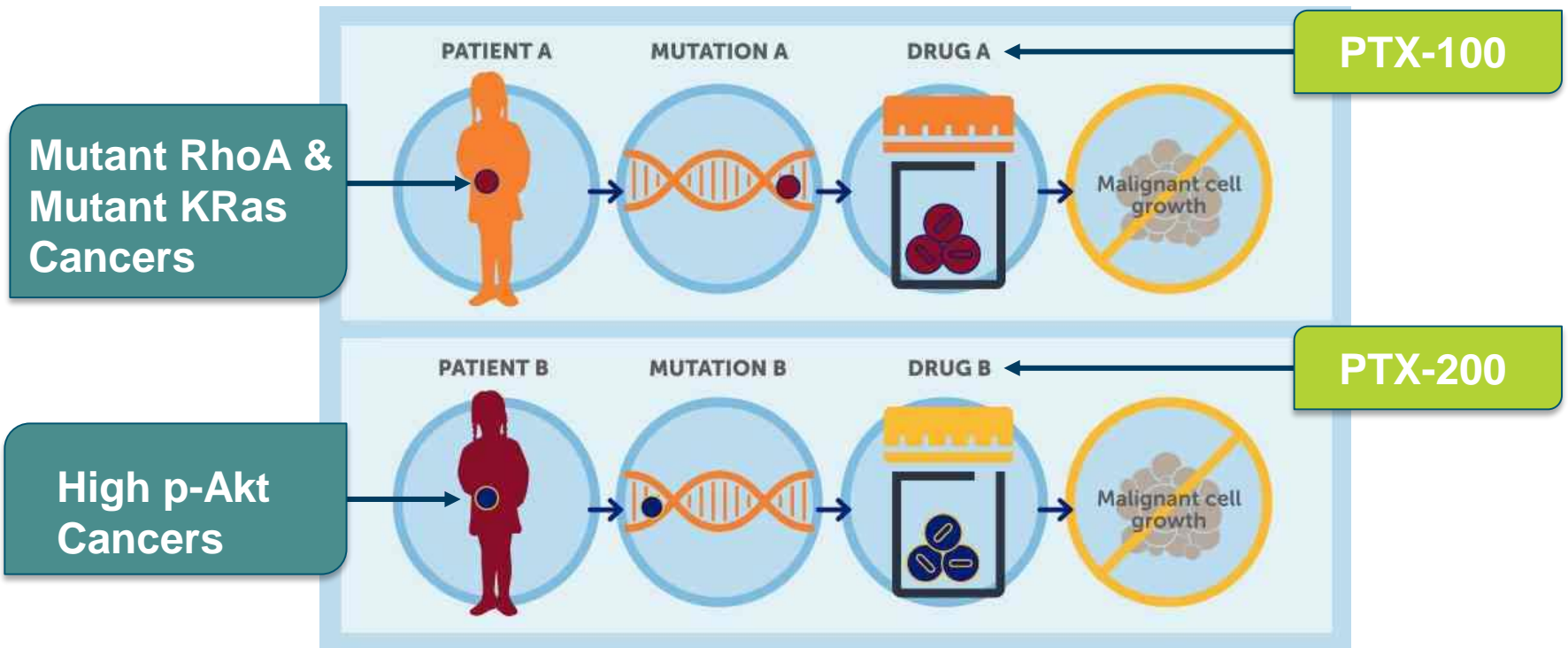


PRESCIENT FOLLOWING THE SAME DEVELOPMENT PATH

- Prescient Therapeutics (ASX: PTX); market cap \$22M
- Target therapies for cancer
- Hyper pAkt – PTX-200
 - » Orphan drug designation in AML
- Ras/Rho mutations – PTX-100
 - » Including ultra-orphan RhoA mutant lymphomas
- **A step behind some US peers, but treading the same path!**



PTX-100 AND PTX-200 PRECISION ONCOLOGY



PTX-200

NOVEL AKT INHIBITION

AML

Breast cancer

Ovarian cancer



PTX-200 PRECISION ONCOLOGY VALIDATION

- PTX-200 stops the growth of **only tumors that harbor high p-Akt levels**

- » PTX-200: Inhibitor of AKT hyper-activation-common in 50-70% of breast, ovarian, colorectal, prostate, pancreatic and Leukemia.
- » Activated AKT causes chemotherapy resistance
- » PTX-200 “switches off” pathway overcomes chemotherapy resistance and causes cancer cells to die



- In addition to high P-Akt levels, other biomarkers that may predict response to PTX-200 are:

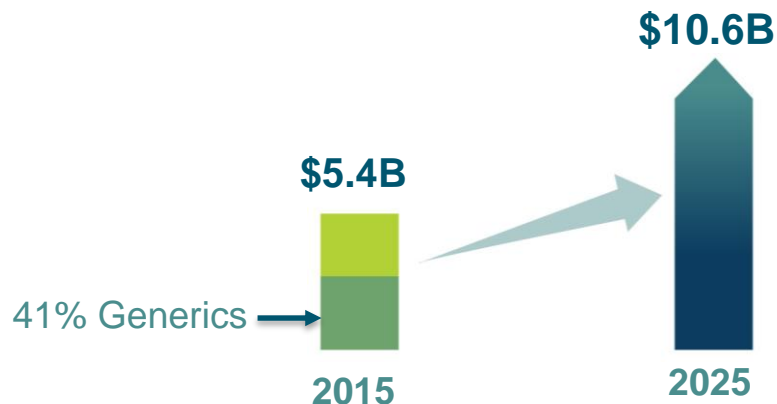
- » Akt mutation (E17K)
- » High levels of the ZNF217

Control Group No. 01		Experimental Group No. 01		No. 02	
Left OV5	Right OV3	Left OV5	Right OV3	Left OV5	Right OV3
0.2089g	0.2582g	0.4837g	0.0507g	0.2258g	0.0573g

HER2 - BREAST CANCER OVERVIEW



80% OF BREAST CANCERS ARE **HER2-**
BUT THIS IS **STILL UNDERSERVED** BY NEW DRUGS



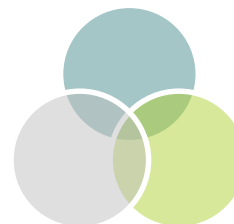
MARKET NEED:

LACK OF PIPELINE AGENTS ADDRESSING

- » RESISTANT ER+ DISEASE
- » NEOADJUVANT THERAPY



- » AKT IS ADVERSE PROGNOSTIC FACTOR
- » CORRELATED WITH WORSE DISEASE-FREE SURVIVAL
- » DRIVES RESISTANCE TO ENDOCRINE THERAPY



PTX'S NICHE:
NEOADJUVANT
TARGETED THERAPY
FOR HER2- DISEASE

PHASE 1B BREAST CANCER TRIAL SUCCESSFULLY COMPLETED; NOW IN PHASE 2

- PTX-200 in combination with paclitaxel, followed by AC (doxorubicin & cyclophosphamide)
- Patients with metastatic and locally advanced HER2- breast cancer
- 28 patients dosed; 12 in expansion cohort at 35 mg/m²
- 5 patients from Phase 1b qualifying for Phase 2 analysis
- **Phase 2 trial currently underway in locally advanced breast cancer**



Joseph Sparano, M.D.
Principal Investigator



Albert Einstein College of Medicine
OF YESHIVA UNIVERSITY



Heather Han, M.D.

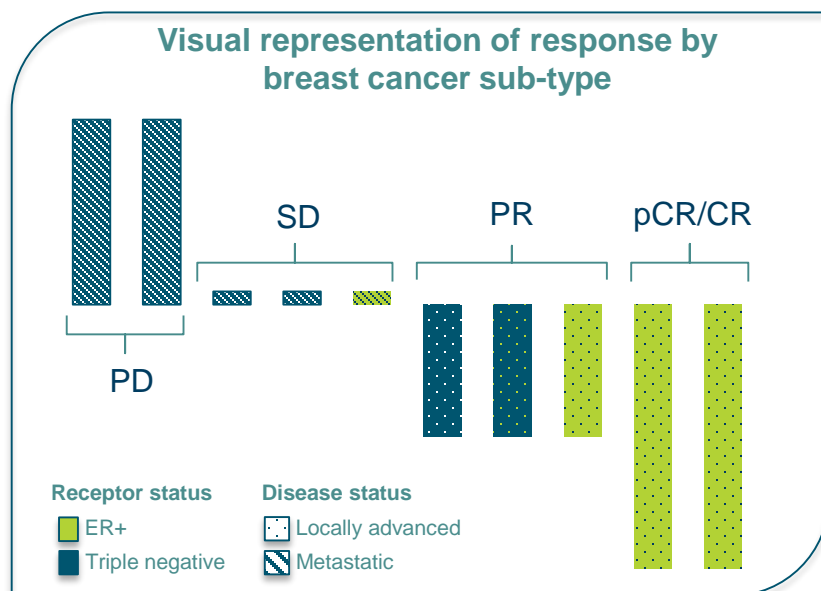


WHAT DOES SUCCESS LOOK LIKE FOR THIS DISEASE?

- For women with locally advanced **ER+, HER2 negative breast cancer**, typical expectations are:
 - » **Complete response (complete eradication) of 16% (11-22%)**
 - » **Overall response rate of 25%**
- A meaningful improvement on these response rates would be seen as very encouraging
- Whilst Prescient is not measuring PFS in this study, pCR is recognised by the FDA as an endpoint to accelerated approval

PHASE 1B EFFICACY RESULTS VERY ENCOURAGING

- 10 patients evaluable for efficacy:
 - » 2 complete responses (both in ER+)
 - » Overall response rate of 50%
 - » In ER+ disease, ORR (pCR+ PR) was 75%
- **Small numbers, but very encouraging efficacy results, particularly in difficult to treat ER+ and locally advanced disease**
- 5 patients from Phase 1b qualifying for Phase 2 analysis

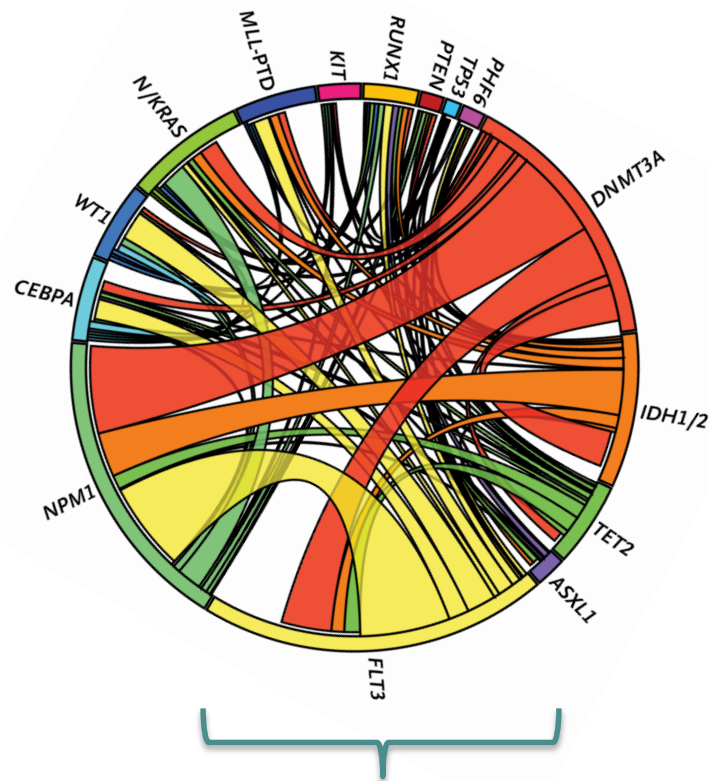


Metastatic & locally advanced patients			
	ER+	Triple negative	Total
pCR/CR	2	0	2
PR	1	2	3
SD	1	2	3
PD	0	2	2
ORR	75%	33%	50%

Locally advanced patients			
	ER+	Triple negative	Total
pCR/CR	2	0	2
PR	1	2	3
SD	0	0	0
PD	0	0	0
ORR	60%	40%	100%

AML: MUTATIONAL COMPLEXITY NEEDS PRECISION APPROACH

- Regardless of the mutational complexity of AML, **72% of AML patients have high p-Akt**
- **PTX-200 has the potential to complement other targeted therapies, and capture what they cannot**



1 drug approved &
15 in development
just targeting FLT3

PHASE 1B AML TRIAL UNDERWAY

- Phase 1 results with PTX-200 (monotherapy) very encouraging
- Now PTX-200 + cytarabine in refractory or relapsed acute leukemia
- World renowned expert Professor Jeff Lancet at Moffitt Cancer Center leading the trial
- Yale Cancer Center and Kansas University Medical Center also participating in trial
- Due to complete Phase 1b around middle of 2018



Jeffrey E Lancet, M.D.
Principal Investigator



PHASE 1B OVARIAN CANCER TRIAL

- Significant need for new products to treat platinum-resistant ovarian cancer
- Testing PTX-200 plus carboplatin in patients with platinum resistant ovarian cancer
- PTX-200 already proven **overcome cisplatin resistance** and **synergize with cisplatin** in pre-clinical studies
- Phase 1b underway
- Currently recruiting at H. Lee Moffitt Cancer Center



Robert Wenham, M.D.
Principal Investigator



PTX-100

PHASE 1 IN SOLID TUMORS COMPLETED

NOW PURSUING A TRANSFORMATIVE OPPORTUNITY IN
RAS AND RHO MUTANT CANCERS

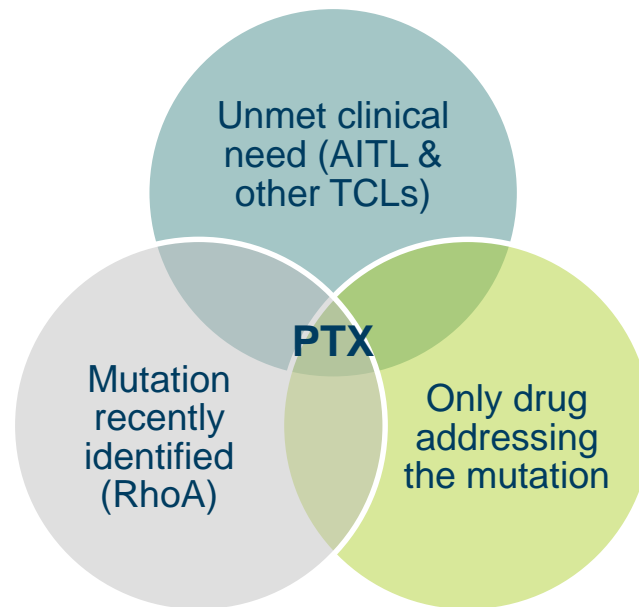


PTX-100 PRECISION ONCOLOGY

- PTX-100 is predicted to inhibit tumors that harbor **mutant RhoA** and **mutant KRas**
 - » PTX-100: 1st in class/man inhibitor of Ras (mutated in 30% of human cancers) and Rho (mutated in up to 70% of AITL) pathways
 - » PTX-100 “switches off” these pathways by targeting GGT-1, enzyme
 - » PTX-100 completed Phase 1: Safe and patients achieved stable disease
 - » Soon to enter Pilot and Phase II
- In addition to high mutant RhoA and mutant KRas, other biomarkers that may predict response to PTX-200 are:
 - » p27Kip
 - » PTEN
 - » RalA/B

PTX-100 THE MOST ADVANCED DRUG TARGETING RHOA

- Only RhoA inhibitor in the clinic
- Phase 1 trial in solid tumours completed
- **PTX-100 has a unique position in RhoA mutant lymphomas**



RARE DISEASES CAN TRANSFORM SMALLER COMPANIES

RARE DISEASES



TYPICALLY MUCH
SMALLER TRIALS
REQUIRED



LOWER
DEVELOPMENT
COST



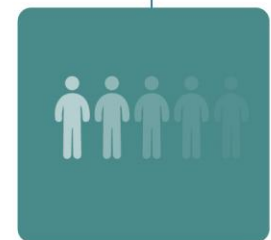
FASTER
DEVELOPMENT
TIME



SUPPORT FROM
REGULATORS,
INCLUDING POTENTIAL
EXPEDITED REVIEW



GUARANTEED MARKET
EXCLUSIVITY POST
APPROVAL
(IRRESPECTIVE OF
PATENT STATUS)
7 YEARS IN US;
10 YEARS IN EU



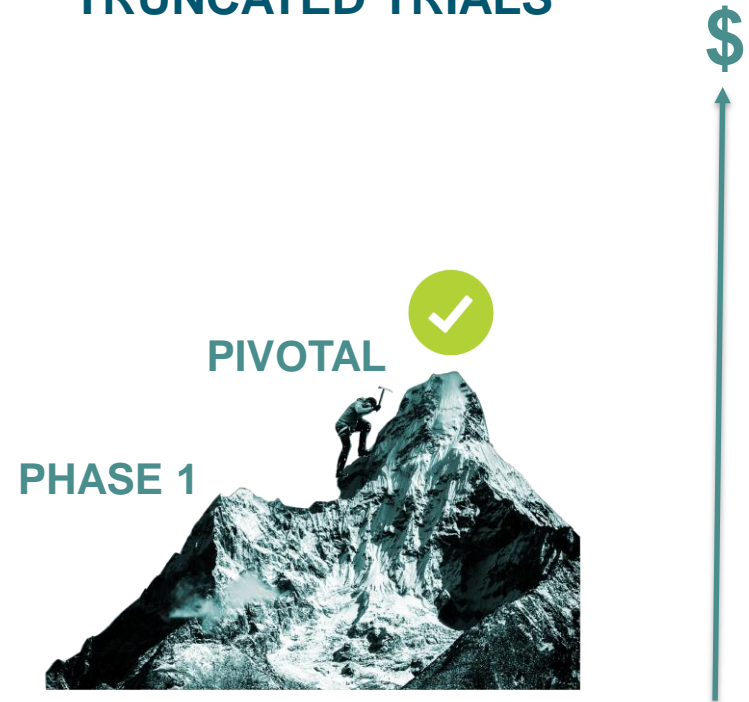
TYPICALLY
REQUIRE FEWER
RESOURCES

A QUICKER, CHEAPER ROUTE TO MARKET

TRADITIONAL TRIALS



TRUNCATED TRIALS



CASE STUDY : FOLOTYN

- Developed by Allos, acquired by Spectrum Pharmaceuticals
- For relapsed & refractory Peripheral T-cell lymphoma
 - » 5,600 cases/year in US
- Approved on overall response rate of 27%
- Currently priced at US\$450,540 per patient, per year

FOLOTYN
(pralatrexate injection) 



12 MONTHS GOALS AS SET OUT AT 2017 AGM

- Removal of clinical hold on PTX-200 breast cancer trial 
- Final data on the Phase 1b PTX-200 breast cancer trial 
- Continuing Phase 2 PTX-200 breast cancer trial
- Manufacturing run of PTX-100 and additional inventory of PTX-200
- Pre-clinical work in PTX-100 in RhoA mutant cancers
- Completion of PTX-200 AML Phase 1b trial
- Completion of PTX-200 ovarian cancer Phase 1b trial
- Re-entering the clinic with PTX-100
- Continuing to build awareness among investors, clinicians and corporates

INVESTMENT SUMMARY



- Multiple shots on goal with novel targeted (precision) cancer therapies
- Robust body of science underpinning programs
- Great scientific and clinical team with a proven record of success
- Transformative opportunity in Ras/Rho mutant cancers
- Following in the footsteps of US targeted therapy companies that have enjoyed spectacular success
- Multiple catalysts this year
- Encouraging efficacy signal in Ph1b breast cancer



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