



**DEVELOPING NOVEL TARGETED  
THERAPIES TO BEAT CANCER**

**SPOTLIGHT ON PTX-100**

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Prescient Therapeutics Limited (ASX: PTX)  
October 2017

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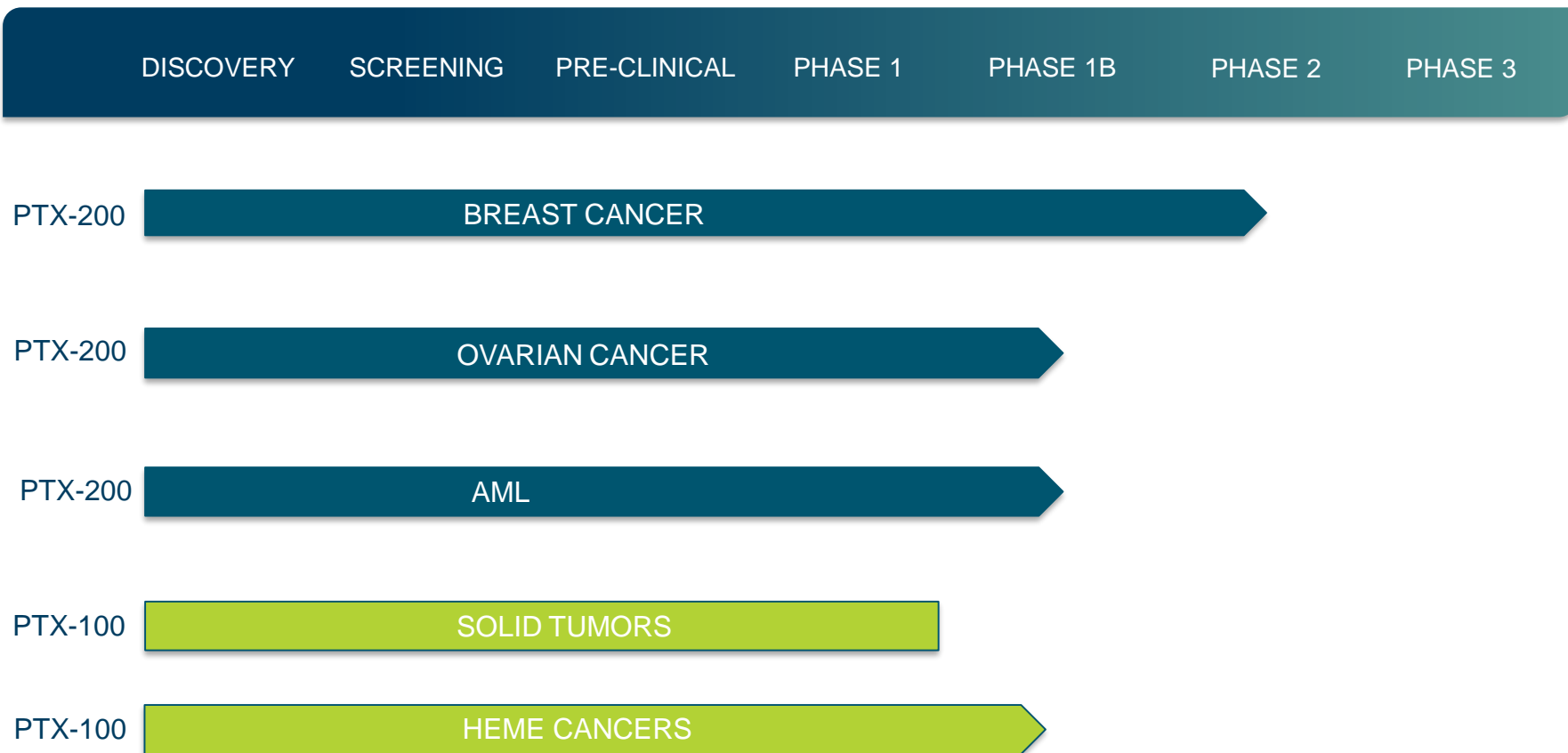
# INVESTMENT HIGHLIGHTS

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

- **2 targeted therapies** with impeccable scientific pedigree
- Multiple shots on goal with **Akt and Ras pathway inhibitors** in **multiple trials**
- **One of deepest clinical pipelines on the ASX**
  - » Targeting important areas of unmet clinical need
- **Multiple catalysts** for value creation
- **Funded through to value-accretive catalysts**, with a fantastic share register
- Phase 1b/2 AML trial is being led by globally renowned leukemia expert, Professor Jeff Lancet
  - » Professor Lancet also led Celator Pharmaceuticals' ground-breaking VYXEOS trial in AML
- Great scientific and clinical team with a **proven record of success**
- Recent **encouraging efficacy breast cancer results, despite SAE resulting in clinical hold**
- **Transformative opportunity for PTX-100 in rare blood (heme) cancers**

# DEEP, CLINICAL STAGE PRODUCT PIPELINE

- PTX-200 currently in three clinical trials (BRCA and OVCA recruitment currently on hold pending SAE response)
- Advancing PTX-100 in rare hematological cancers - a transformative opportunity



# HOW OUR DRUGS WORK: “MOLECULAR SWITCHES”

Akt & Ras are growth molecules found in cells – when they are stuck “on”, they send **constant signals to the cancer cell to grow**



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



# DRUGS DON'T DEVELOP THEMSELVES! DEVELOPMENT TEAM WITH BENCH TO BEDSIDE SUCCESS

Proven success from discovery and clinical development, through to FDA approvals



**Said Sebti, PhD**  
Chief Scientific Officer

- Professor and Chair, Department of Drug Discovery - Moffitt Cancer Center
- Co-inventor of PTX-100 & PTX-200
- **Named among top 20 Translational Researchers in the world by Nature Publishing Group**



**Terry Chew, M.D.**  
Chief Medical Officer

- Hematologist/oncologist with 20 years experience in biotech & pharma
- **5 New Drug Applications** including DaunoXome, Taxotere and DepoCyte
- **PTX is only 1 of only 2 ASX biotechs with a CMO that has successfully approved drugs**



**Mandeep Grewal**  
VP – Clinical Operations

- Extensive clinical trial management experience with pharma, biotech & CROs
- Certifications: CRCP, CCRA, CCRP
- Formerly Exelixis, Quark Pharma, Fibrogen, Cytokinetics, Chiron, Abbott, Quintiles



**Mike Preigh, PhD**  
VP - CMC

- **Led CMC at Array BioPharma for 10 years**
- Successfully brought >20 drug candidates to IND & clinical development
- Previously Pfizer



**Claudia Gregorio-King, PhD**  
VP - Operations

- Extensive experience in the management of pre-clinical and clinical research and intellectual property
- Regulatory affairs and clinical project management experience with small and large CROs



**Chaline Strickland, Pharm.D.**  
Regulatory Affairs

- Senior Director of Clinical Affairs at Ground Zero Pharmaceuticals
- Involved in dozens of New Drug Applications

# PTX-200

ACUTE MYELOID LEUKEMIA  
BREAST CANCER  
OVARIAN CANCER



# PHASE 1B AML TRIAL UNDERWAY

- Phase 1 results with PTX-200 (monotherapy) very encouraging
- Now PTX-200 + cytarabine in refractory or relapsed acute leukemia
  - » 15 -18 patients
  - » 3+3 design, single arm
  - » Up to 4 dose levels of PTX-200 starting at 25 mg/m<sup>2</sup> (days 1, 8, 15)
  - » Cytarabine held constant at 400 mg/m<sup>2</sup> as continuous infusion (days 2-6)
- Professor Jeff Lancet at Moffitt Cancer Center leading the trial
- Yale Cancer Center and Kansas University Medical Center also participating in trial
- **First cohort successfully completed (announced March 8)**
  - » 3 AML patients treated at 25 mg/m<sup>2</sup>
  - » Early signs of efficacy
- Now at second cohort at 35 mg/m<sup>2</sup>



Jeffrey E Lancet, M.D.  
Principal Investigator





# PHASE 1B BREAST CANCER TRIAL COMPLETED

- PTX-200 in combination with paclitaxel, followed by AC (doxorubicin & cyclophosphamide)
- Patients with metastatic and locally advanced HER2- breast cancer
  - » Albert Einstein College of Medicine Montefiore Medical Center and the H. Lee Moffitt Cancer Center
  - » Single arm
  - » Exploring 3 dose levels of PTX-200 15 -35 mg/m<sup>2</sup> (3/4 weeks up to 9 doses)
  - » Paclitaxel 80mg/m<sup>2</sup>/week x 12 weeks
  - » Expansion cohort: dose-dense AC every 2 weeks
- 29 patients dosed; 12 in expansion cohort at 35 mg/m<sup>2</sup>
- **Preliminary efficacy on 8 patients encouraging:**
  - » 1 complete response
  - » 4 partial responses
  - » 2 stable disease
  - » 1 progressive disease
- 5 patients from Phase 1b qualifying for Phase 2 analysis
- Company has paused recruitment following recent adverse event
- Revising risk management and patient protocols, to ensure superior safety in a high risk patient group



Joseph Sparano, M.D.  
Principal Investigator



Albert Einstein College of Medicine  
OF YESHIVA UNIVERSITY



Heather Han, M.D.



# 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 (recruitment on hold)
- Currently recruiting at H. Lee Moffitt Cancer Center
- Up to 12 patients with an additional 18 in expansion cohort
- **Now at second dose level**



Robert Wenham, M.D.  
Principal Investigator



# PTX-100

Phase 1 in solid tumors completed.

Now pursuing a transformative opportunity in rare blood cancers.



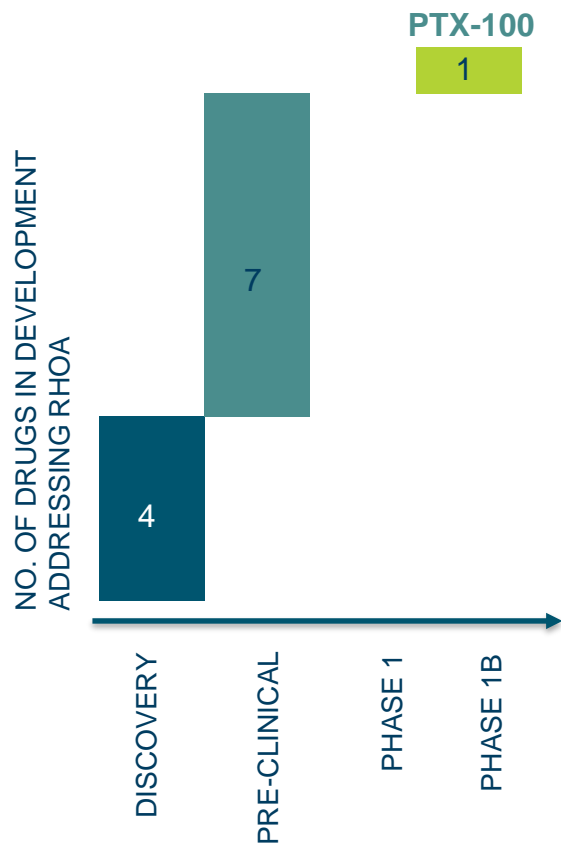
# TRANSFORMATIONAL OPPORTUNITY IN RARE HEMATOLOGICAL NICHE

- PTX-100 has a unique opportunity in RhoA mutant lymphomas
- Prescient recently lodged new patent application in this area.
- Rare diseases can transform biotech companies
  - » **Less competition** from Big Pharma
  - » Typically much **smaller trials** required = Lower **cost + Faster** development
  - » Support from regulators, including potential **expedited review**
  - » **Guaranteed market exclusivity** post approval (irrespective of patent status) 7 years in US; 10 years in EU
  - » Can often carry program without having to partner (**much more lucrative**)
- Case study : Folutyn (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 year

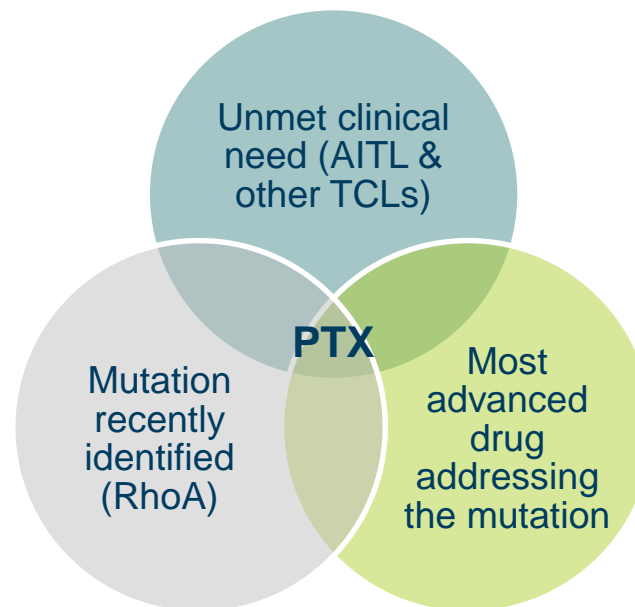
**FOLOTYN**   
(pralatrexate injection)



# PTX-100 THE MOST ADVANCED DRUG TARGETING RHOA

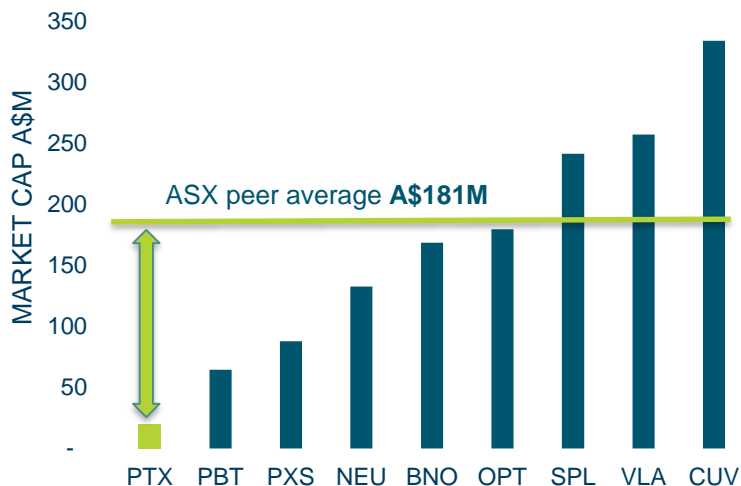


- Only 12 RhoA inhibitors in development in oncology
  - » No others are in the clinic
  - » None are in hematology indications
  - » PTX-100 is the most advanced, with Phase 1 trial in solid tumours completed
- **PTX-100 has a head start and unique position in RhoA mutant lymphomas**

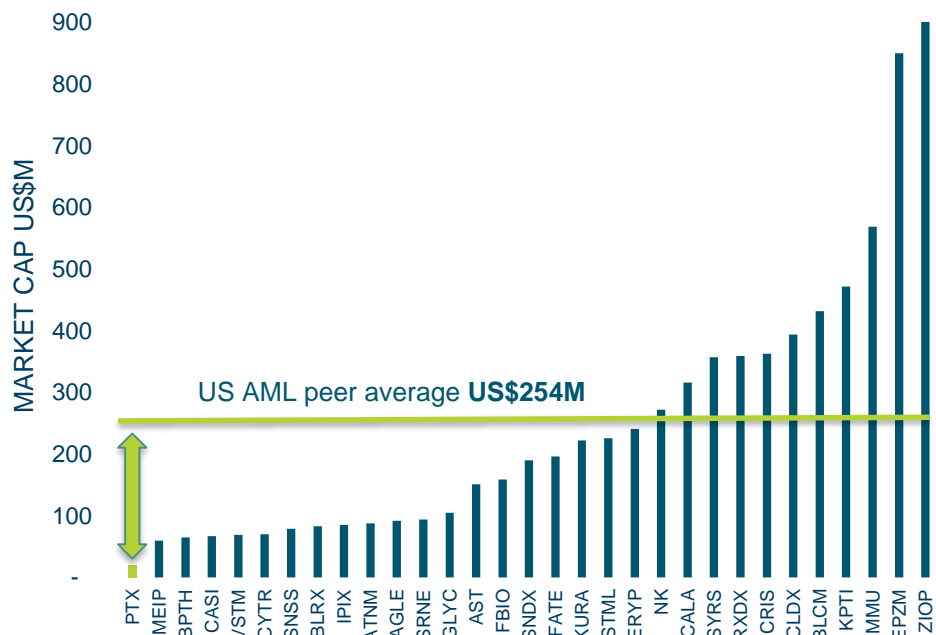


# SIGNIFICANT VALUATION ARBITRAGE

Significant valuation arbitrage against comparable ASX peers...



...Arbitrage against US AML peers is even more pronounced



Comparisons are complicated by most companies in having multiple indications (as does PTX). For illustrative purposes this comparison was narrowed to US biotechs with AML drugs in development and no revenue

# INVESTMENT SUMMARY

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

- **Underpinned by PTX-200**
  - » Any one of 3 trials a potential company maker
- **Moonshot on PTX-100**
- Excellent team with successful track record
- Overlapping catalysts
- A great time to be entering the stock!



## CONTACT

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