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### **INVESTMENT HIGHLIGHTS**

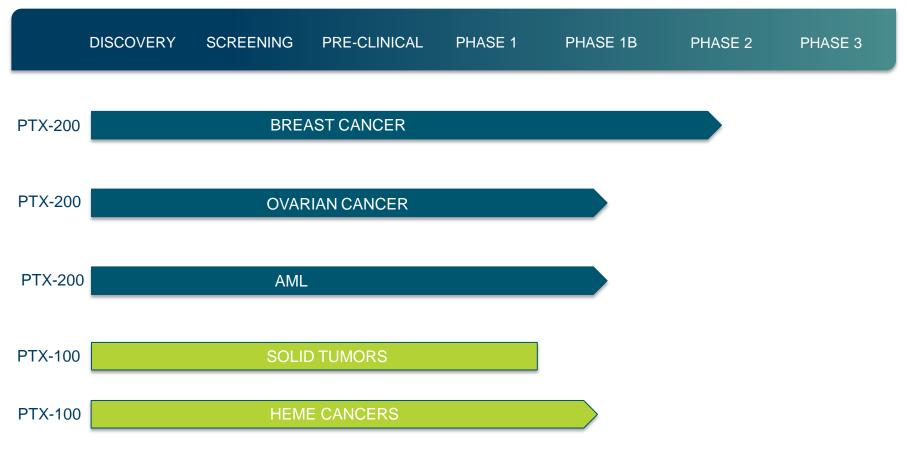
### 2 DRUGS » IMMINENT CATALYSTS » FUNDING IN PLACE » UNDISCOVERED VALUE

- 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 leukaemia 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 breast cancer results, despite SAE resulting in clinical hold
- Transformative opportunity in rare blood (haem) cancers



# DEEP, CLINICAL STAGE PRODUCT PIPELINE

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





# **HOW OUR DRUGS WORK: A REFRESH**

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







### INVESTMENT DECISION FUNNEL FOR ANY BIOTECH

Is the drug in a clinical trial, or still pre-clinical? **Both drugs clinical stage** Is the trial conducted to US FDA standard? ✓ All INDs (under US FDA) Do the indications make clinical & √ Targeting unmet/poorly met medical commercial sense? needs - relapse & refractory; hot areas Are there multiple drugs &/or ✓ 2 novel drugs. 3 clinical trials, with programs to mitigate risk? another being planned Where has the science come ✓ Blue chip provenance. multiple US grants from? Has it been validated? >65 peer reviewed publications! Is the clinical hypothesis ✓ Potentiating existing treatments; clinician buy-in; sound and clinically relevant? compelling efficacy signals provide confidence Who is prepared to put their name to it? ✓ International experts from world leading institutions Has the team √ Yes – from bench to bedside; FDA approvals; into market done it before? How long ✓ Multiple catalysts this year alone until catalysts? Cashed up Is the risk-adjusted Strong institutional investor support valuation Valuation a fraction of relevant peers attractive? Multiple layers of value with risk mitigation



### LAST YEAR WE SAID

AML trial currently screening for first patient Ph1b



Ovarian cancer currently screening next patient Ph1b



Breast Cancer Ph1b expansion cohort nearing completion



Manufacturing new batch of PTX-200



Interest in PTX-100 – planning development program



• Building awareness of company and our programs ahead of data





### THE YEAR IN REVIEW

- Manufacturing run of PTX-200
- Commencement of PTX-200 Phase 1b AML trial
- Successful completion of Phase 1b PTX-200 breast cancer trial
- Subsequent SAE on patient on breast cancer trial leading to clinical hold on all PTX-200 trials
- Progression of Phase 1b/2 PTX-200 ovarian cancer trial, with first cohort completed
- Granting of three additional US patents and a European patent for PTX-200
- Granting of Orphan Drug Designation for PTX-200 by U.S. FDA
- PTX-100 pre-clinical study published in prestigious scientific journal Nature
- · Lifting of clinical hold on PTX-200 trials in AML and ovarian cancer
- Strategic review of indications for PYTX-100 and identification of a truly transformative opportunity in RhoA mutant lymphomas
- Identification of Patent application for PTX-100 in RhoA mutant cancers
- Appointment of new highly-experienced individuals to the team
- Built global awareness of company and our programs



### THE PTX-200 CLINICAL HOLD

- After completion of the Phase 1b breast cancer trial, last patient passed away from liver failure
- Hold on all 3 PTX-200 programs
- Share price dropped 30%
- Review on all data in PTX-200 trials
- Enhanced safety measures include:
  - Excluding patients with liver disease
  - » More frequent liver function tests
- Hold lifted on AML trial Sept
- Hold lifted on ovarian cancer trial November
- Share price still recovering but trending in right direction



### **BIG OPPORTUNITY IN RARE HEMATOLOGICAL NICHE**

- Recent discovery that RhoA mutations are a key driver in certain haematological cancers
- Some of these conditions are characterized high unmet need:
  - » Rare
  - » Poor prognoses with short median survival
  - » Very few existing treatment options
  - » Very few drugs in development
- PTX-100 is uniquely positioned to address this unmet need due to its mechanism of action
- Prescient recently lodged new patent application in this area.
- Case study: Folotyn (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

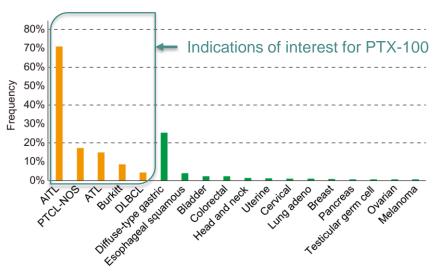






# RAS PATHWAY, AND RHOA IN PARTICULAR, ARE IMPLICATED IN A NUMBER OF HEMATOLOGICAL MALIGNANCIES

### Frequency of RhoA mutations in human malignancies



nature genetics

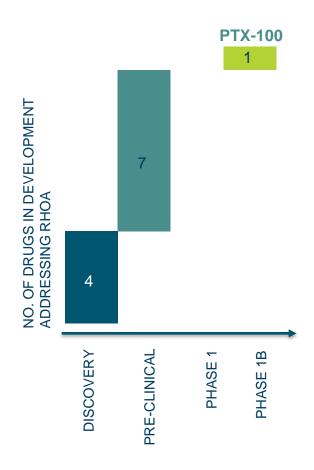




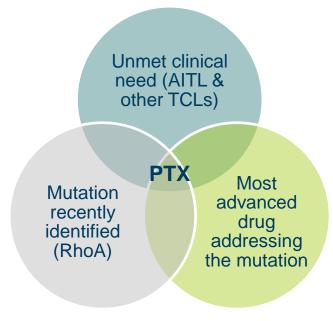
- In addition, N-Ras and K-Ras contribute to several heme malignancies including:
  - » Acute Myeloid Leukemia (AML)
  - » Chronic Myelomonocytic Leukemia (CMML)
  - » Juvenile Myelomonocytic Leukemia (JMML)
  - » Multiple Myeloma (MM)
  - » Ras-associated Autoimmune Leukoproliferative Disorder (RALD)



# 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





## WHEN TARGET THERAPIES MEET RARE DISEASES



- Loxo Oncology (NASDAQ: LOXO); \$2.3B market cap
- TRK inhibitor targeting cancers with TRK fusions
- Deal for LOXO-101 and LOXO-195
- In mid-stage clinical trials. ORR 75%
- US\$400m upfront
- Loxo could earn up to US\$1.55 Billion
- Bayer and Loxo co-promote in US; Bayer solo RoW







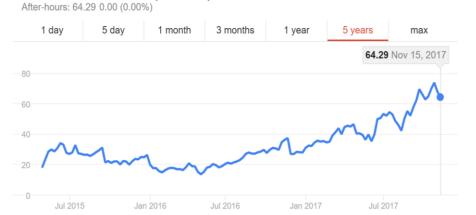
## WHEN TARGET THERAPIES MEET RARE DISEASES

- 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
- Valuation: US\$2.5 B; \$64/share Today:



#### **Blueprint Medicines Corp** NASDAQ: BPMC - 15 Nov., 5:28 pm GMT-5

64.29 USD **1**0.37 (0.58%)





# WHEN TARGET THERAPIES MEET RARE DISEASES

- Prescient Therapeutics (ASX: PTX); market cap \$14M
- Target therapies for cancer
- Hyper pAkt PTX-200
  - » Orphan drug designation in AML
- Ras pathway mutations PTX-100
  - » Ultra-orphan RhoA mutant lymphomas
- A step behind some US peers, but treading the same path







### IN THE NEXT 12 MONTHS WE WILL WORK TOWARDS:

- Removal of clinical hold on PTX-200 breast cancer trial
- Final data on the Phase 1b 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 in haematological malignancies
- Continuing to build awareness among investors, clinicians and corporates



## **THANK YOU**

...Thank you to our shareholders for their ongoing support

...Thank you to our Board, staff and clinical faculty for working successfully though the challenges this year

...Thank you to the brave patients we treat, and their families and support networks

Together we are all helping develop much needed new therapies to patients who are often on their last chance





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