



Targeted therapies yielding a deep clinical pipeline

- » *3 clinical trials in oncology*
- » *First class team*
- » *All eyes on AML trial*

Prescient Therapeutics Limited (ASX: PTX)

Rodman & Renshaw 18th Annual Global Investment Conference

September 2016



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Company Overview

One of Deepest Clinical Pipelines on ASX	<ul style="list-style-type: none">• 3 clinical trials in cancer: 2 underway and 1 on track to initiate imminently• All under INDs
Two Clinical Stage Oncology Drugs	<ul style="list-style-type: none">• 2 novel drug candidates targeting key cancer pathways<ul style="list-style-type: none">» Akt (PTX-200) and Ras (PTX-100)
Distinguished Scientific Provenance	<ul style="list-style-type: none">• Compelling science from leading US institutions – Yale University & Moffitt Cancer Center• >65 peer reviewed publications
Significant investment already made	<ul style="list-style-type: none">• Over \$20 M invested to date<ul style="list-style-type: none">» Technologies have been awarded multiple prestigious US government grants
Proven Leadership & Management	<ul style="list-style-type: none">• Experienced and proven drug development team on board to aggressively drive product development
Rich Upcoming News Flow	<ul style="list-style-type: none">• Multiple milestone announcements and valuation inflection points across all clinical programs over next 12 to 18 months

Investment Highlights

- One of deepest clinical pipelines on the ASX
 - » Targeting hot areas of **unmet clinical need**
- Targeted therapies with impeccable scientific pedigree
- Funded through to value-accretive catalysts, with a fantastic share register
- **Phase 1b/2 AML trial is being led by renowned leukemia expert, Dr Jeff Lancet**
 - » Dr Lancet was also the Principal Investigator on Celator Pharmaceuticals' ground-breaking VYXEOS trial in AML
- Multiple shots on goal
- Brilliant scientific and clinical team with a proven record of success
- Catalysts not far away

Deep, Clinical Stage Product Pipeline

PTX has one of the **deepest** and **most mature** product pipelines on ASX

	Discovery	Screening	Preclinical	Phase 1	Phase 1b	Phase 2	Phase 3	
PTX-200	Breast Cancer					→	<i>Finishing</i>	
PTX-200	Ovarian Cancer					→	<i>Underway</i>	
PTX-200	AML					→	<i>Imminent</i>	
PTX-100	Solid tumors					→	<i>Completed</i>	

Corporate Snapshot

Key Metrics

ASX Ticker	PTX
Total Issued Capital	209.2 M shares
Options	53.8 M
Share Price ¹	A\$0.105
Market Capitalisation ¹	A\$22 M
Cash Position ²	A\$9.7 M
Top 20 Own	52%
6 month turnover ¹	37 M shares; A\$3.9 M

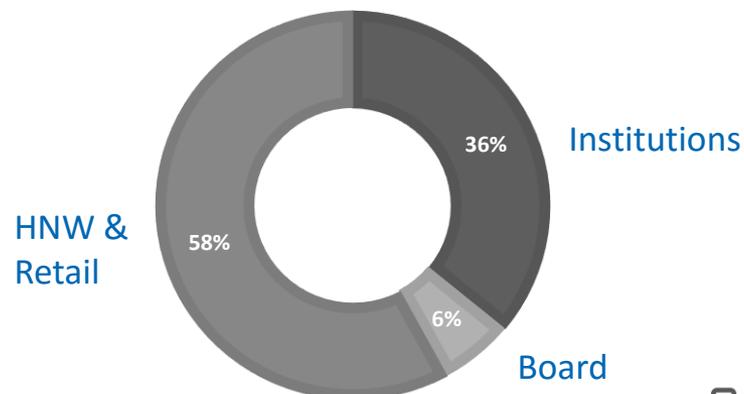
1 - As at 30 August 2016

2- As at 30 June 2016. Not included after this reporting period is \$1.3M shortfall placement and an anticipated R&D tax rebate of \$0.64M.

Share price performance



Shareholder Base

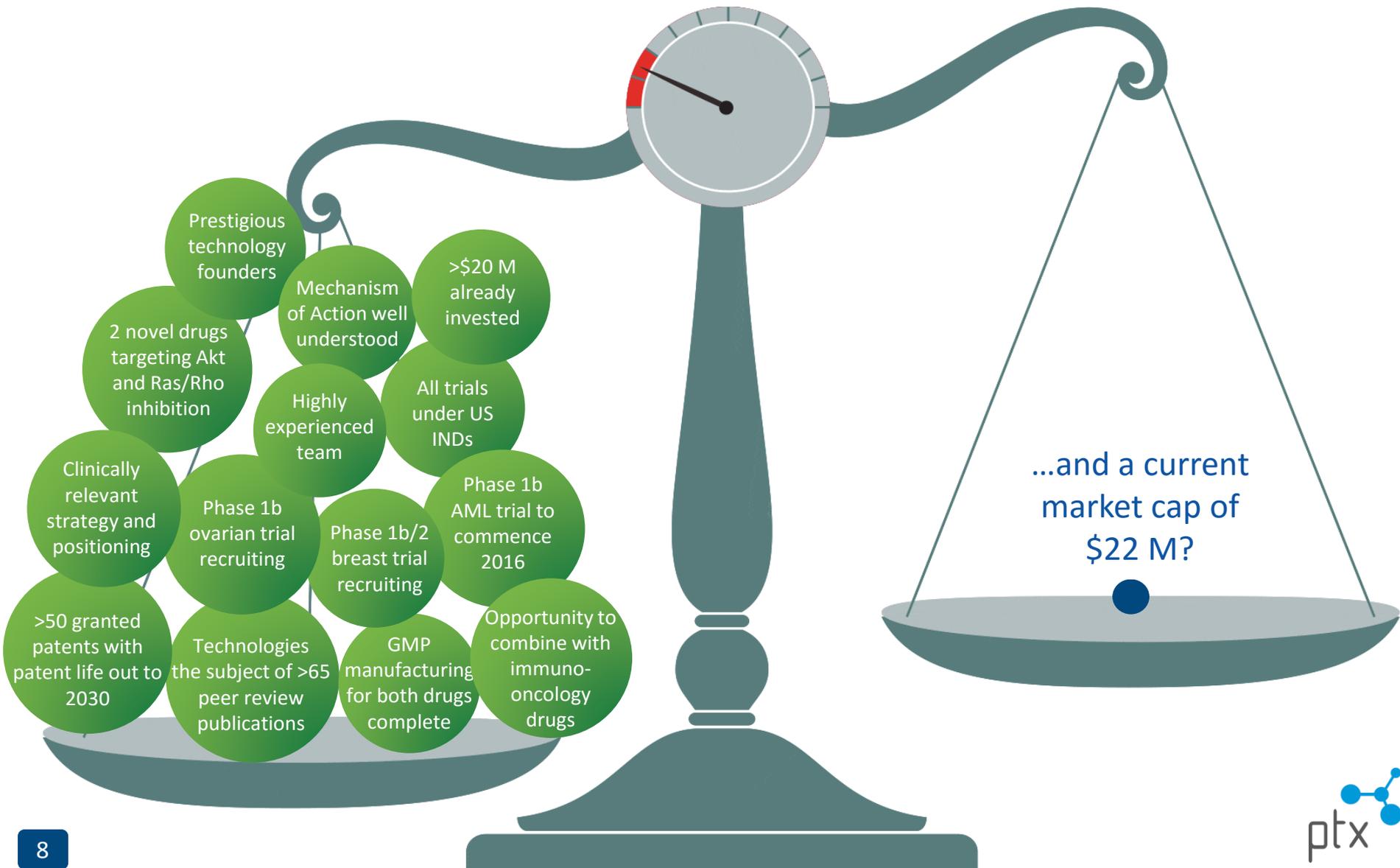


Underpinned by extensive peer reviewed work

Science validated by >65 peer reviewed publications, including:



PTX's valuation obviously doesn't weigh up!



How Our Drugs Work: “Molecular Switches”



Akt & Ras are growth factors found in cancer cells – when they are turned on, they send a signal to the cancer cell to grow



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

PTX-200

PTX-100



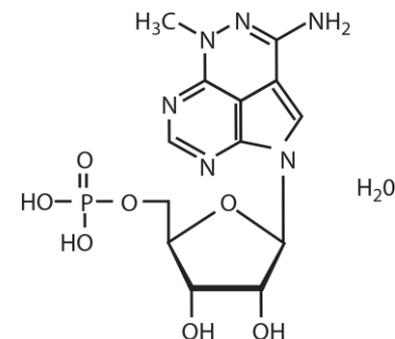
Akt is an Important Drug Target

- Akt pathway promotes cellular survival and growth
- **Hyperactive** Akt signaling has two deleterious effects:
 - » Plays key role in the **development of many cancers** including breast, ovarian, colorectal, prostate, pancreatic and hematologic cancers
 - » Confers **resistance to chemotherapy**
- Therefore there is strong pharma interest in Akt as a drug target
- Previous attempts at blocking Akt encountered fundamental problems leading to toxicities
 - » Focusing too far upstream (e.g. PI3K) or on single arms of pathways
 - » Multikinase inhibitors/ATP mimics
 - » Promiscuity leading to off target effects
 - » **All these non-specific, off-target effects lead to high toxicities**
- PTX-200 avoids these shortcomings!

PTX-200: Novel Akt inhibition

- A small molecule inhibitor of the Akt signaling pathway
- **Inhibits Akt without the toxicity** of other attempts
- **Anti-proliferative AND pro-apoptotic**
- Novel mechanism of action
 - » NOT an ATP mimic; not a direct kinase inhibitor
 - » Inhibits Akt by preventing Akt binding to the membrane
 - » Huge advantage in MoA; **avoids off target effects** of most kinase inhibitors
- PTX-200 synergistic with chemotherapy and biologics
- **Overcomes chemotherapy resistance and causes cancer cells to die**
- Completed Phase 1 trials demonstrated it is well tolerated, AML patients achieved stable disease (single cycle of monotherapy)

PTX-200 (TCN-P)



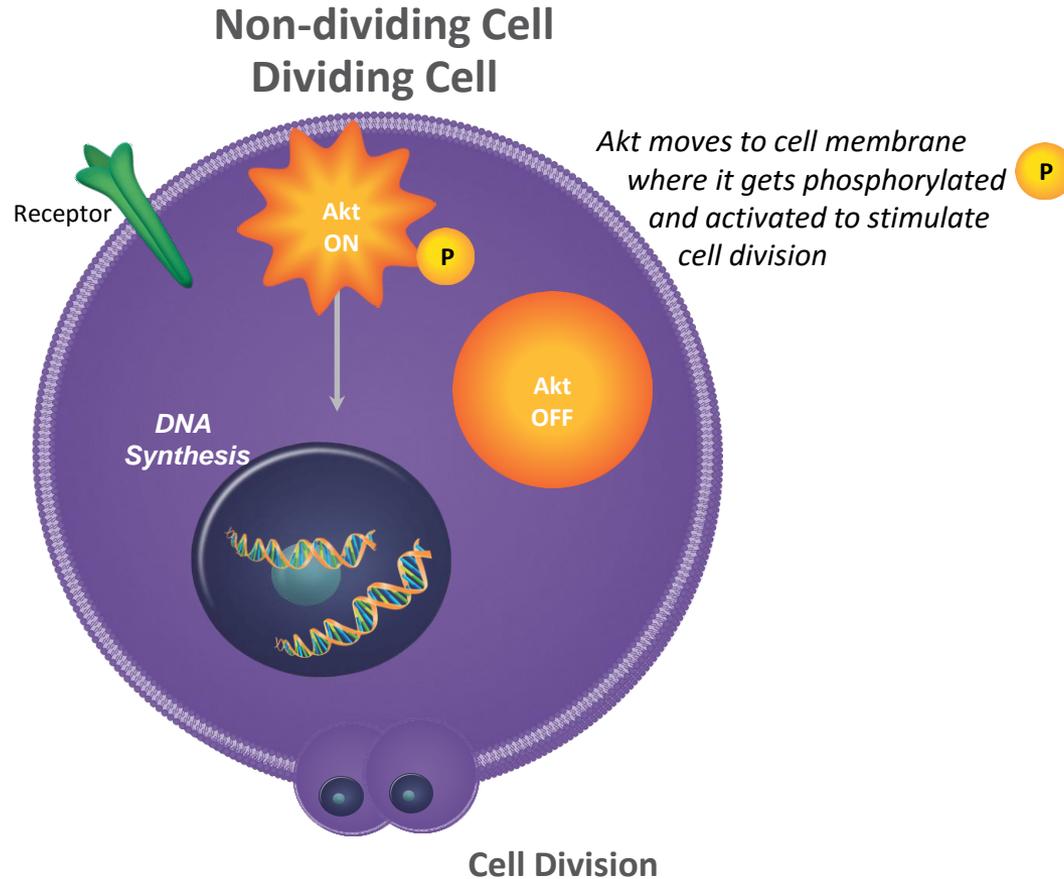
MOFFITT
CANCER CENTER

In normal cells, Akt switches growth ON & OFF

Add Growth Factor

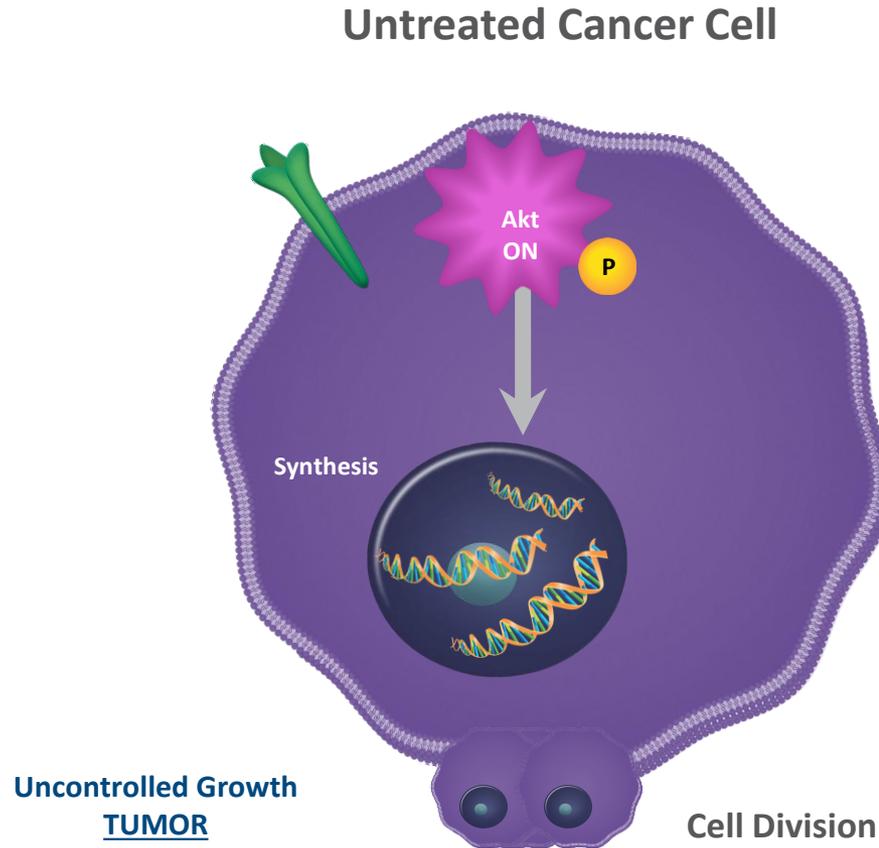


Akt is **ON** when phosphorylated and **OFF** when not



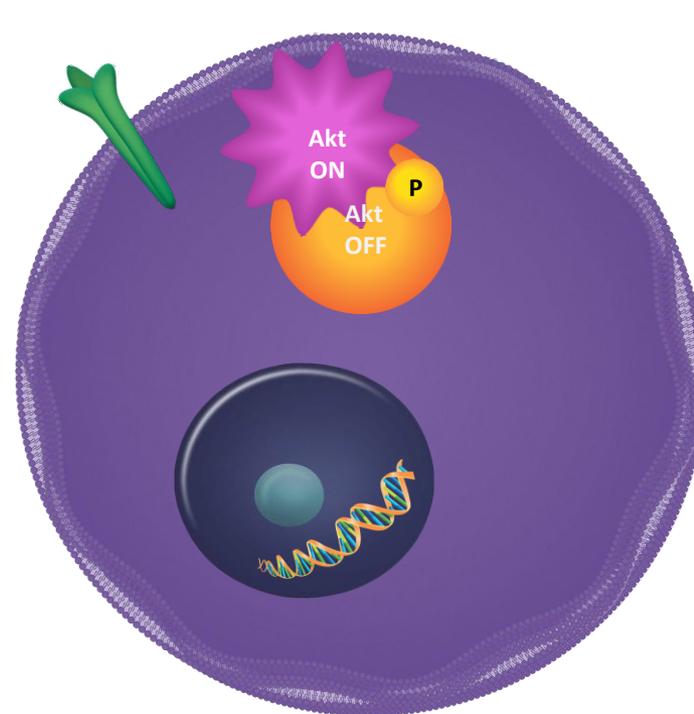
Controlled Growth

When Akt is permanently switched ON, uncontrolled growth (cancer) ensues



PTX-200: Mechanism of Action – switching Akt OFF

Cancer Cells treated with
PTX-200



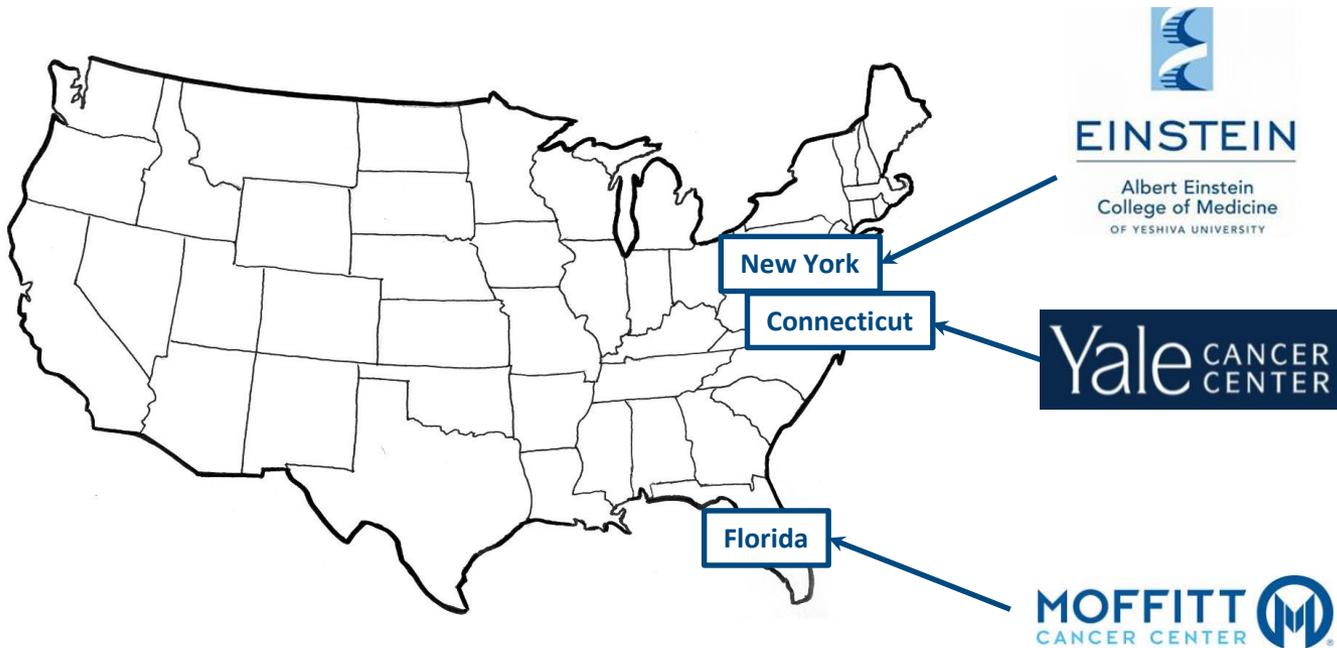
Treatment
With PTX-200

*Prevents Akt from
moving to membrane -
keeping it in the OFF position*



Tumor Cells
Stop Dividing

World Class Centers & Collaborations



Previous clinical trials conducted at:



Memorial Sloan Kettering
Cancer Center



Working with the Moffitt Cancer Center

- H. Lee Moffitt Cancer Center & Research Institute, established in 1986 in Tampa, Florida
- 3rd largest cancer centre in the US
- On one side of the campus is a world-leading “**comprehensive cancer clinic**” (1)
 - » offering patients medical oncology, surgical oncology and radiology, as well as ongoing care
- On the other side of the same campus is a renowned **cancer research institute** (2) dedicated to developing new cancer treatments
 - » 800 research scientists, postdocs, graduate students and support staff
 - » **Said Sebti is Head of Drug Discovery**
- The **perfect environment to conduct clinical trials**:
 - » Collaboration and synergy between leading researchers and clinicians
 - » Currently over 350 clinical trials
 - » Huge influx of patients for trial recruitment
 - » Bird’s eye view of other cancer research and treatments



Drugs don't develop themselves!

- 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-Program Leader, Chemical Biology and Molecular Medicine - Moffitt Cancer Center
- 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
- Formerly with Argos and Peregrine Pharmaceuticals
- **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
Vice President –
Clinical Operations

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



Chaline Strickland
Clinical &
Regulatory Affairs

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

Clinical Advocates Driving Our Programs

Acute Myeloid Leukemia



Jeff Lancet, M.D.

- Section Chief of Leukemia in the Department of Malignant Hematology at Moffitt
- **Was Principal Investigator on Celator's transformative VYXEOS AML trial**

Breast Cancer



Joseph Sparano, M.D.

- Prof. of Medicine & Obstetrics, Gynecology, & Women's Health - Albert Einstein College of Medicine
- Assoc. Chairman for Clinical Research - Montefiore Medical Center Dept of Oncology



Heather Han, M.D.

- Assistant Prof. of Medicine at University of South Florida College of Medicine
- Medical oncologist, specializing in breast cancer
- The Center for Women's Oncology - Moffitt Cancer Center

Ovarian Cancer

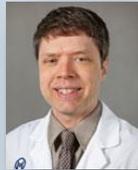


Robert Wenham, M.D.

- Section Head, Gynecologic Cancer Research
- Principal investigator Total Cancer Care Protocol

World Class Scientific Advisory Board

- Genuine international authorities, with particularly strong expertise in leukemia



**Jeff Lancet,
M.D.**

- Professor of Oncologic Sciences, H. Lee Moffitt Cancer Center and University South Florida
- Section Chief of Leukemia in the Department of Malignant Hematology at Moffitt
- Principal Investigator on Celator's groundbreaking VYXEOS trial in AML



**Farhad
Ravandi,
M.D.**

- Professor, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, Texas
- Chief, Section of Developmental Therapeutics, Texas University MD Anderson Cancer Center, Houston, Texas



**Thomas
Prebet,
M.D., PhD**

- Assistant Director of Myeloid Malignancy Research, Yale Cancer Center, New Haven Connecticut
- Previously Associate Professor of Clinical Hematology at Institut Paoli-Calmettes, Marseille, France



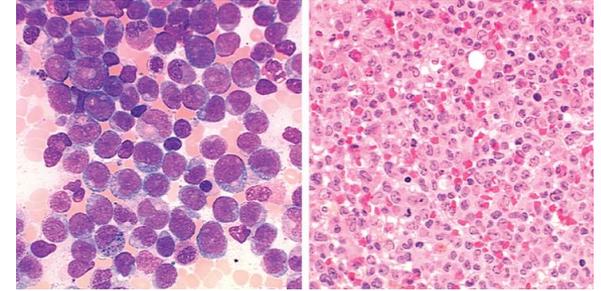
**Douglas
Joshua, PhD**

- Emeritus Professor of Hematology at the Sydney University Medical School
- Consultant Hematologist, Royal Prince Alfred Hospital, Sydney
- Member of the International Myeloma Foundation



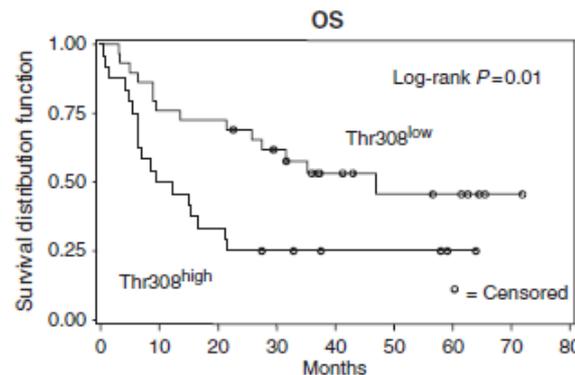
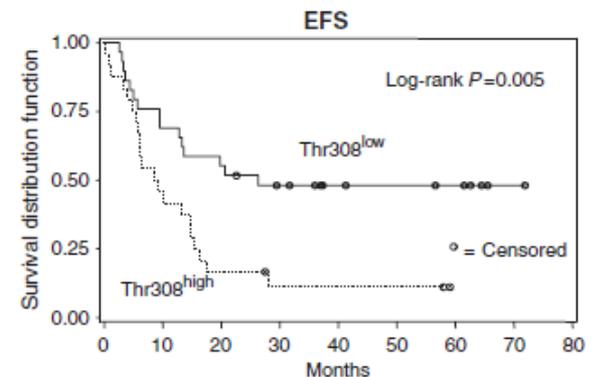
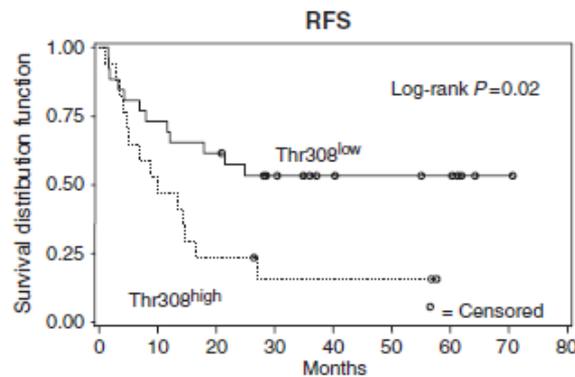
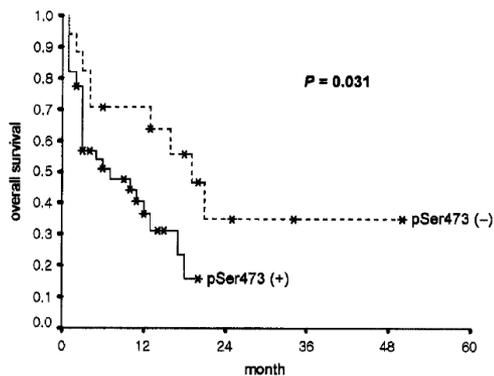
Acute Myeloid Leukemia Market Overview

- AML is a type of cancer that affects the blood and bone marrow
 - » Patient cannot produce normal blood cells
 - » Blood cells cannot function properly nor fight disease
- Progresses very quickly & 5 year survival is a dismal 25%
- More common in adults over 60 years old, so the market is growing rapidly in developed economies
 - » 50% increase in incidence since 2013 in the US alone!
- After initial chemo, most patients relapse
- There are poor options for relapsing and refractory AML patients. **Treatment options have barely changed in ~40 years!**
- These ingredients explain the massive interest in relapsing & refractory AML
 - » A growing, ageing disease in rich countries
 - » Dismal survival
 - » No treatment options!
- **PTX's AML program was a clear focus of interest from both clinicians and specialist biotech funds in the US last year due its compelling efficacy signals**



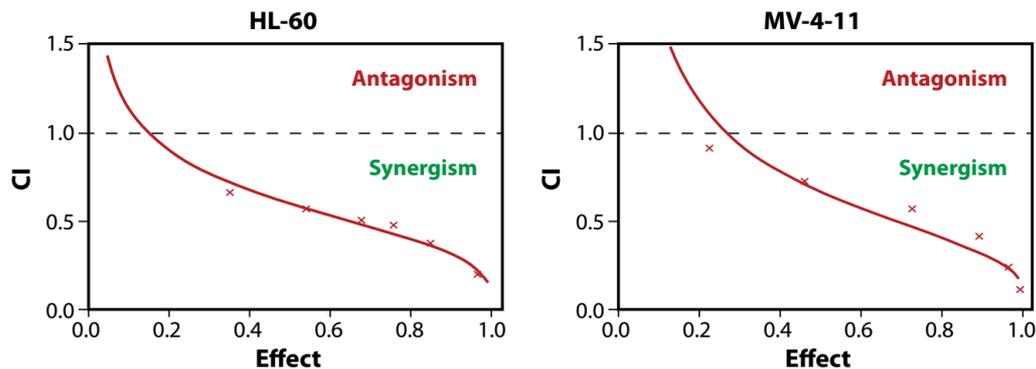
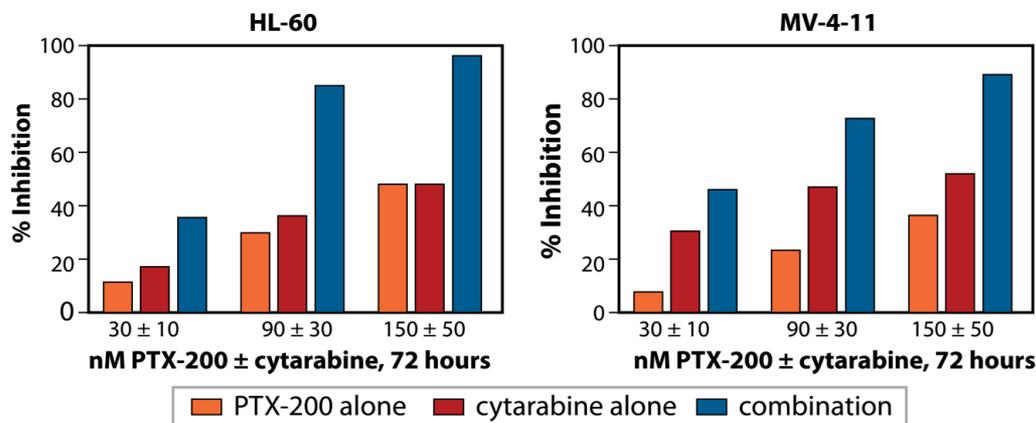
Relevance of Akt in AML

- Frequent constitutive Akt activation (phosphorylation) in AML
 - » Constitutive phosphorylation (Ser473 and Thr308) of Akt in AML compared to normal bone marrow cells in 44 out of 66 of patients (72 %)
- Implications for Akt as a modulator of chemotherapy resistance in AML
- High Akt phosphorylation = inferior survival



PTX-200 synergizes cytarabine in AML cells

- PTX-200 highly synergistic with cytarabine current standard of care in AML cells
 - » PTX-200 + cytarabine = much more effective effect than the simple additive effect of either compound (i.e. $1 + 1 \geq 2$)
- As cytarabine is the current standard of care in AML, this suggests that PTX-200 may potentiate the standard of care



PTX-200: Completed Phase 1 in AML (monotherapy)

Patients	32
Trial Centers	MD Anderson & Moffitt
Patient Inclusion	Advanced hematologic malignancies (mainly AML)
Methods	Administration 1 hour IV infusion on days 1, 8, and 15. Cycles repeated every 21 days.
Study Objectives	To establish dosing regime and biological dose
Summary	<ul style="list-style-type: none">• 17 out of 32 patients had stable disease after one cycle of treatment• 3 patients with AML achieved >50% bone marrow blast reduction• Compelling signals of efficacy• Further investigation of PTX-200 alone or in combination in patients with high Akt levels is warranted



Published Leuk Res.
2013
Nov;37(11):1461-7

Compelling evidence for PTX-200 in AML

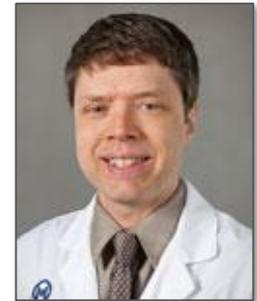
Efficacy hypothesis	PTX-200 Evidence
<ul style="list-style-type: none"> High p-Akt is correlated with inferior survival in AML. 	<ul style="list-style-type: none"> PTX-200 decreased pAkt in AML blasts.
<ul style="list-style-type: none"> Inhibiting p-Akt improves response to chemo in the clinical setting. 	<ul style="list-style-type: none"> PTX-200 decreased pAkt in AML blasts, suggesting this method of reducing pAkt would similarly improve clinical outcomes.
<ul style="list-style-type: none"> Phase I achieved safety? 	<ul style="list-style-type: none"> Yes.
<ul style="list-style-type: none"> Any evidence of clinical benefit? 	<ul style="list-style-type: none"> Yes. 53% SD in very sick patients with rapidly progressing disease, despite only using a single cycle of monotherapy. 3 patients had >50% blast reduction.
<ul style="list-style-type: none"> Is there a comparable with any other attempted Akt inhibitor in AML? 	<ul style="list-style-type: none"> PTX-200 had more compelling results than another Akt candidate MK2206 in Phase1 AML. (MK2206 development has since been discontinued by Merck).
<ul style="list-style-type: none"> » MK2206 successfully demonstrated apoptosis of AML cell lines in vivo, but failed to inhibit p-Akt in the clinical setting. 	<ul style="list-style-type: none"> » PTX-200 successfully inhibited p-Akt in the clinical setting.
<ul style="list-style-type: none"> » Only saw one response out of 19 patients (5% SD). 	<ul style="list-style-type: none"> » 17 out of 32 achieved stable disease (53% SD).
<ul style="list-style-type: none"> » Failed at MTD 	<ul style="list-style-type: none"> » Succeeded well below MTD
<ul style="list-style-type: none"> How does it combine with current standard of care? 	<ul style="list-style-type: none"> PTX-200 is highly synergistic with cytarabine in AML cells.
<ul style="list-style-type: none"> Lessons from other trials currently running? 	<ul style="list-style-type: none"> In current Phase 1b breast cancer trial for PTX-200, interim analysis showed encouraging efficacy (including biomarker data).

→ PTX-200 has lots of supportive data and efficacy signals that combine to give confidence leading into the Phase 1b/2 trial.



Phase 1b Trial imminent: Acute Myeloid Leukemia

- PTX-200 plus cytarabine in refractory or relapsed AML
- Phase 1 results very encouraging
- Phase 1b/2 IND recently allowed by FDA
- Jeff Lancet at Moffitt Cancer Center leading the trial
- Yale Cancer Center second site participating in trial
- Ready to initiate trial imminently
- 15 -18 patients
- Expected recruitment ~12 months
- Final reporting Phase 1b expected Q2-Q3 2017
- Recently bolstered PTX's Scientific Advisory Board with world class leukemia expertise from Moffitt, Yale and MD Anderson.



Jeffrey E Lancet, M.D.
Principal Investigator



Breast Cancer Market Overview



- Breast cancer market currently US\$10 B; due to double by 2023
- Most breast cancer drug sales are for HER2+ cancers, but this only represents ~20% of all breast cancers
- By contrast, **PTX is targeting “HER2 negative” (HER2-) breast cancer**
- HER2- has “flown under the radar” of drug developers, due to high profile successes in HER2+ drugs...
- ...but **~80% of breast cancers are still HER2-**
- Comparative lack of new drug development for HER2- patients, despite the need
- Evidenced by American Society of Clinical Oncology (ASCO) issuing a new practice guidelines in 2014
 - » Concluded that doctors should encourage HER2- patients to enroll in clinical trials for new HER2- drugs

Phase 1b Breast Cancer Trial Almost Completed

- PTX-200 in combination with paclitaxel, followed by doxorubicin and cyclophosphamide
- Patients with metastatic and locally advanced breast cancer
 - » Recruiting at Albert Einstein College of Medicine Montefiore Medical Center and the H. Lee Moffitt Cancer Center
 - » Partially funded by National Cancer Institute grant
- **Encouraging early data**
 - » Evidence of safety & anti-tumor activity
 - » Inhibits important tumor survival pathway (Akt)
- **17 patients already dosed – now in expansion phase**
 - » n = 12 patients in Phase 1b expansion stage



Joseph Sparano, M.D.
Principal Investigator



Albert Einstein College of Medicine
OF YESHIVA UNIVERSITY

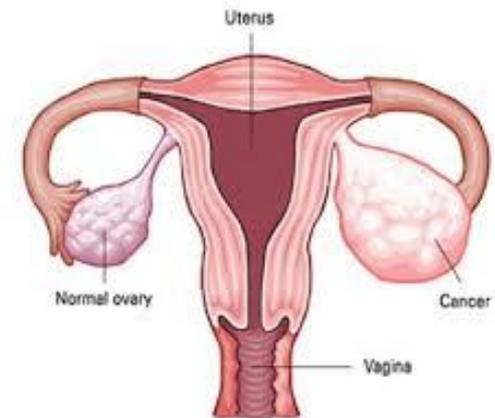


Heather Han, M.D.



Ovarian Cancer Market Overview

- One of the most common cancers in women -increasing with an ageing population
- Due to reach US\$1.7 B by 2019
 - » Market size currently constrained by old generic drugs that just aren't good enough
- Standard of care has not changed in decades (often generic paclitaxel & carboplatin)
 - » Initially effective, with 70% of patients entering remission, but...
 - » ...almost all patients eventually relapse
 - » They have become chemoresistant
- **There remains a severe gap in the market for new drugs for relapsing patients and platinum resistant patients**
- **This is the gap that PTX is pursuing in ovarian cancer**



Phase 1b Ovarian Cancer Trial Commenced

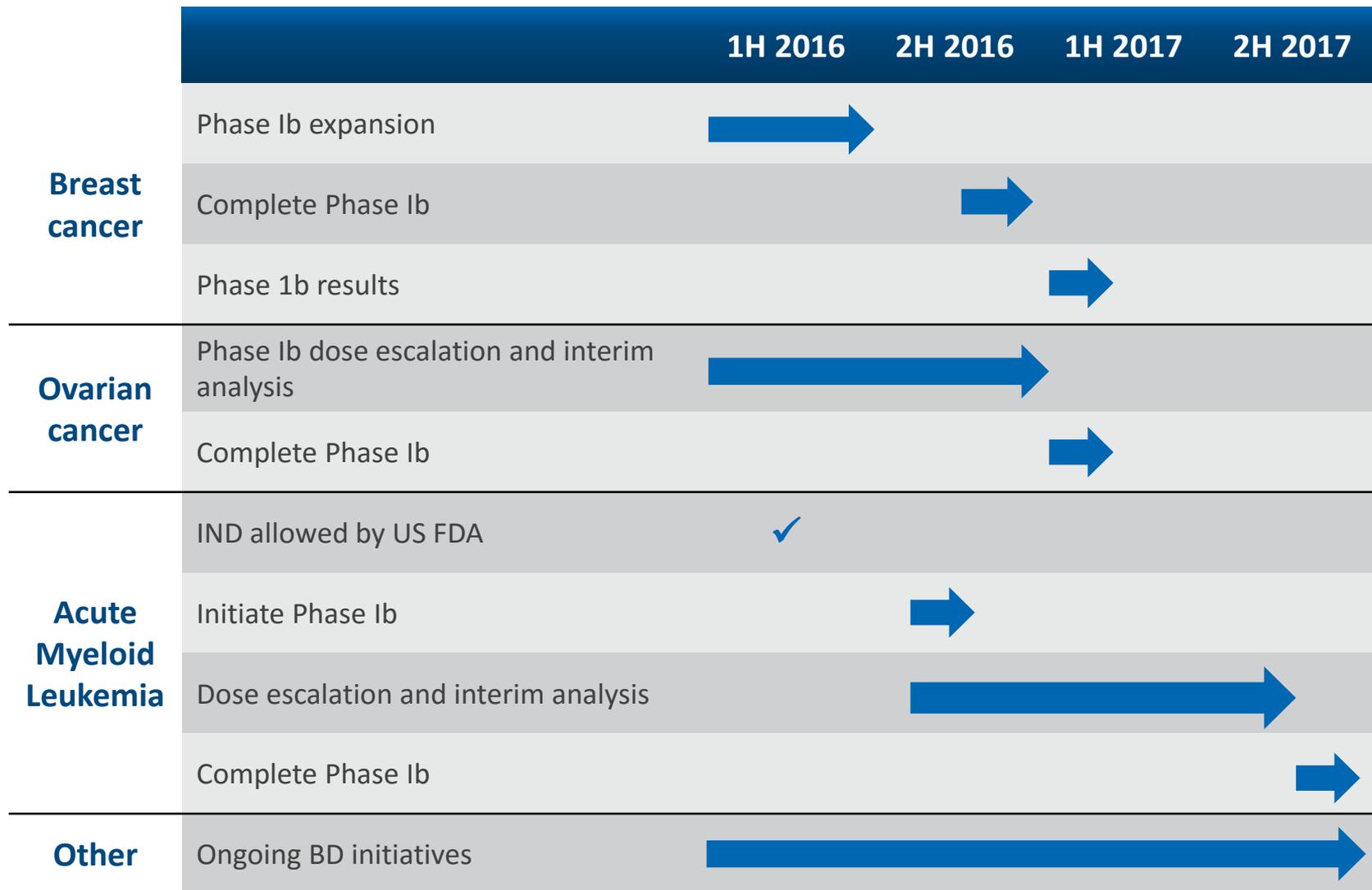
- Significant need for new products to treat platinum-resistant ovarian cancer
- Testing PTX-200 plus carboplatin in patients with platinum resistant ovarian cancer
- PTX-200 already proven **overcome cisplatin resistance** and **synergize with cisplatin** in pre-clinical studies
- **Phase 1b already underway**
- Currently recruiting at H. Lee Moffitt Cancer Center
- Up to 12 patients with an additional 18 in expansion cohort
- **6 patients already dosed**



Robert Wenham, M.D.
Principal Investigator



Catalysts To Watch Out For

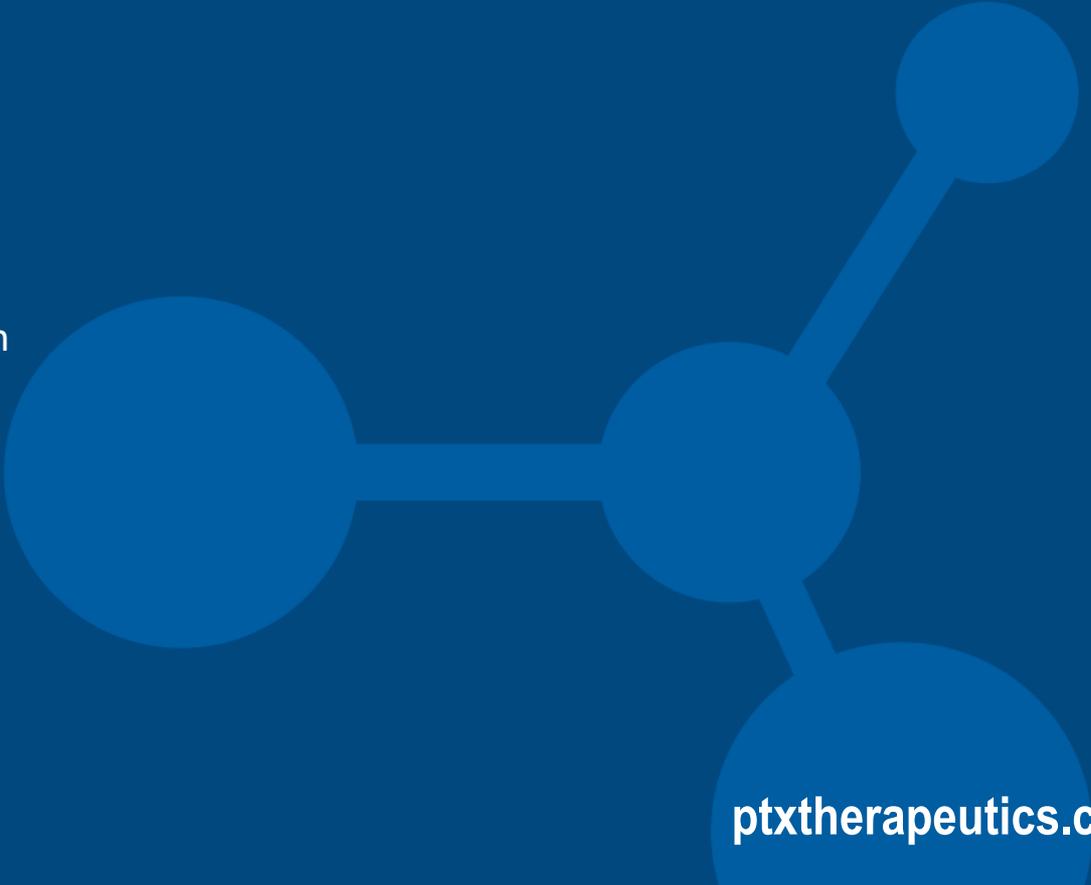




Contact

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M: +61 417 601 440

A large, stylized blue molecular structure graphic is positioned in the background of the slide. It consists of four circles of varying sizes connected by lines, mirroring the smaller graphic in the top left corner. The circles are arranged in a roughly triangular pattern with a fourth circle at the top right.

ptxtherapeutics.com