



*Blue chip US science yielding  
a deep clinical pipeline*

» *Spotlight on AML  
ahead of imminent Phase 1b/2 trial*



*April 2016*

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# Investment Summary: Why all the excitement about AML?

- Acute Myeloid Leukemia (AML) is an area of substantial unmet medical need.
  - » One of the worst survival rates of all cancers
  - » Standard of care unchanged in 40 years
- A disease of intense interest for clinicians, pharma companies and investors
- Celator Pharmaceuticals (NASDAQ: CPXX) showed what can happen when enhancing the standard of care in AML
  - » Market cap recently **surged from small cap to \$780M**
  - » Dr Jeff Lancet was the Principal Investigator on CPXX's ground-breaking trial
- PTX had successful Phase 1 trial in AML (conducted at Moffitt and MD Anderson)
- **Dr Lancet is also the Principal Investigator on PTX's imminent Phase 1b/2 AML trial**
- AML trial about to commence; Phase 1b results due next year

# 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>1H 2016</i>		
PTX-100	Breast Cancer						
PTX-100	Multiple Myeloma						

# Corporate Snapshot

## Key Metrics

ASX Ticker	PTX
Total Issued Capital	93.7 M shares
Options	4.3 M (ex A\$0.10; 12 Oct 2017)
Share Price <sup>1</sup>	A\$0.12
Market Capitalisation <sup>1</sup>	A\$11.7 M
Cash Position <sup>2</sup>	A\$2.4 M
Top 20 Own	33%
6 month turnover <sup>1</sup>	33.5 M shares; A\$3.1 M

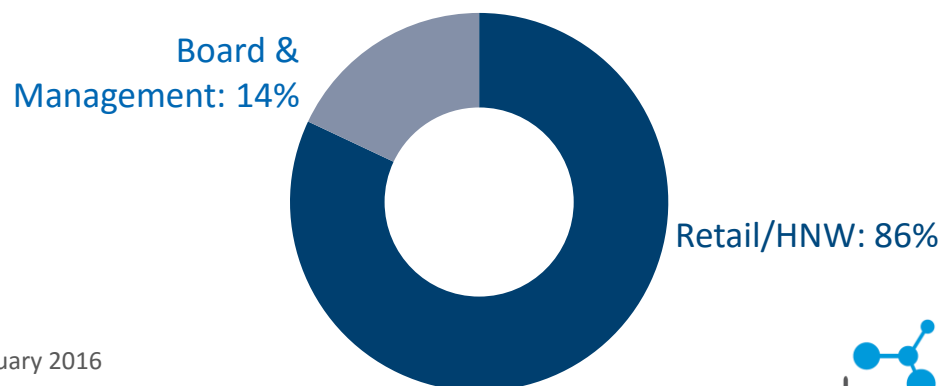
1 - As at 19 April 2016

2- \$1.9 M as at 31 Dec 2015 plus \$0.46M R&D incentive grant received in January 2016

## 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

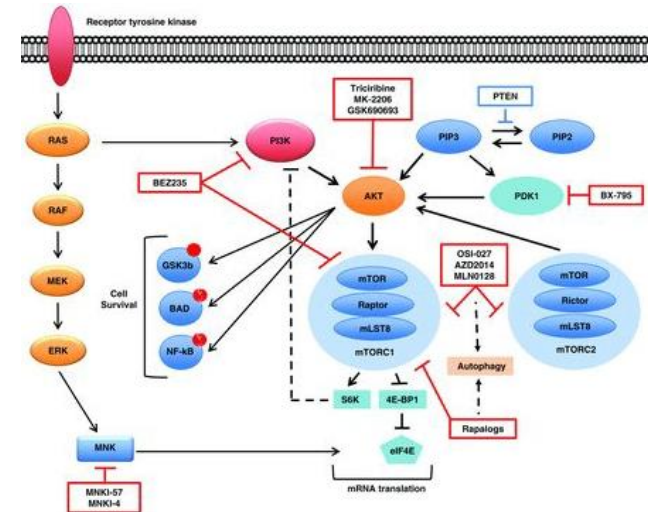
PTX-100





# Akt is an important drug target

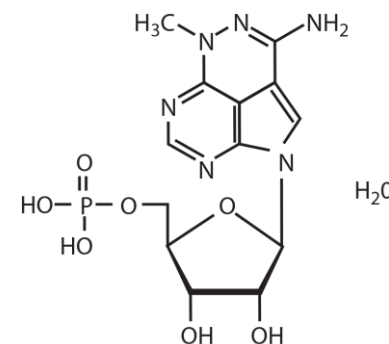
- Akt pathway promotes cellular survival and growth
- **Hyperactive** Akt signaling has two deleterious effects:
  - » Plays key role in the **development of many cancers** including breast, ovarian, colorectal, prostate, pancreatic and hematologic cancers
  - » Confers **resistance to chemotherapy**
- 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)



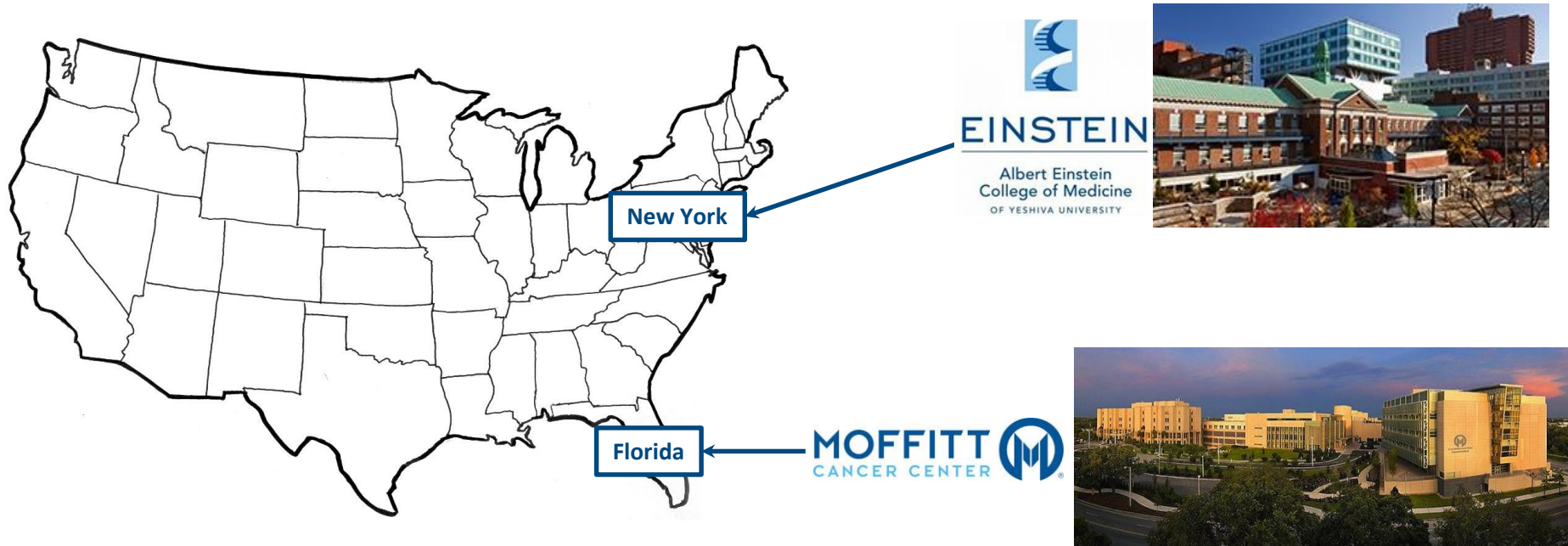
MOFFITT  
CANCER CENTER

# What is Chemoresistance?

- Despite the many advances in cancer treatment over many decades, 90% of advanced cancer patients become resistant to chemotherapy
- Resistance comes about when some of the cells that are not killed by the chemotherapy mutate (change) and become resistant to the drug
- At this point, the chemotherapy stops working and the cancer starts re-growing. Cancer patients then often have no treatment options left
- PTX's drugs are designed to prevent or reverse chemoresistance
- This **gives a new lease of life to existing drugs** when they stop working  
→ “great for patients; great for drug companies”
- Once PTX establishes its drugs in the chemoresistant setting, PTX will then seek to position its drugs earlier in the disease process as a monotherapy.



# World Class Centers & Collaborations



Previous clinical trials conducted at:



INDIANA UNIVERSITY



**Memorial Sloan Kettering  
Cancer Center**



# Working with the Moffitt Cancer Center

- H. Lee Moffitt Cancer Center & Research Institute, established in 1986 in Tampa, Florida
- 3<sup>rd</sup> 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



# Management and Board of Directors

- Experienced, complementary, and collaborative



**Steven  
Yatomi-Clarke**  
CEO & Managing  
Director

- 16 years' experience in healthcare investment banking
- Collaborator on clinical trials conducted in Australia and the US in cancer immunotherapy
- BSc (Hons, Biochemistry & Molecular Biology); BCom (Economics)



**Paul Hopper**  
Executive Director

- 25 years experience in international public company markets with a focus on life science and biotechnology
- Chairman of Viralytics Ltd. and Executive Chairman of Imugene Ltd.
- Former Director of Somnomed, pSivida, Fibrocell and Founder of Polynoma



**Steve Engle**  
Non-Executive  
Chairman

- Former Chairman and CEO of US-listed XOMA (NASDAQ:XOMA) and La Jolla Pharmaceuticals (NASDAQ: LJPC)
- Currently CEO of Averigon Consulting, an advisory firm to life science industry

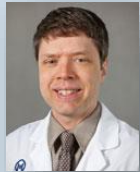


**James Campbell,  
PhD**  
Non-Executive  
Director

- CEO of Patrys Limited (ASX:PAB)
- Previously CFO and COO of Chemgenex Pharmaceuticals
- Non-Executive Director of Invion (ASX:IVX), Medibio Limited (ASX:MEB)

# World Class Scientific Advisory Board

- Genuine international authorities, with particularly strong expertise in leukemia



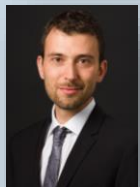
**Jeff Lancet,  
M.D.**

- 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



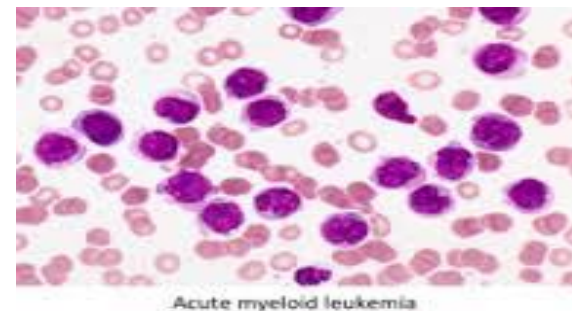
**Douglas  
Joshua, PhD**

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



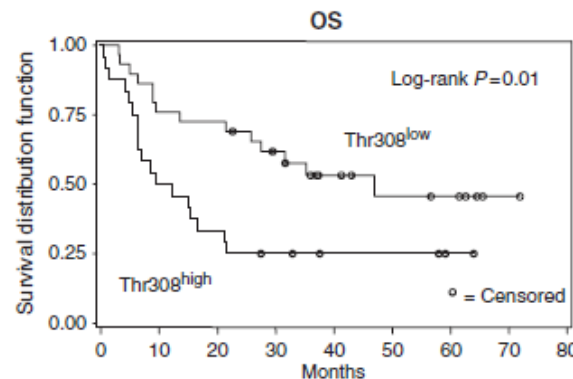
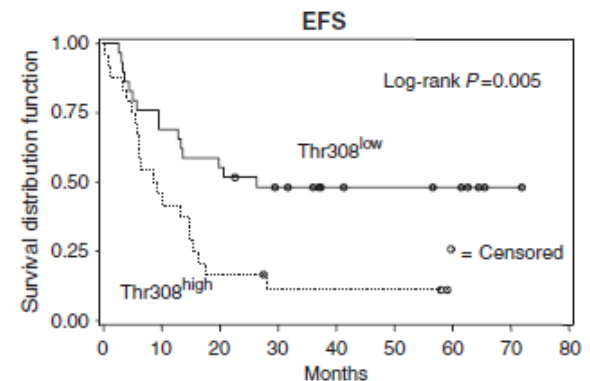
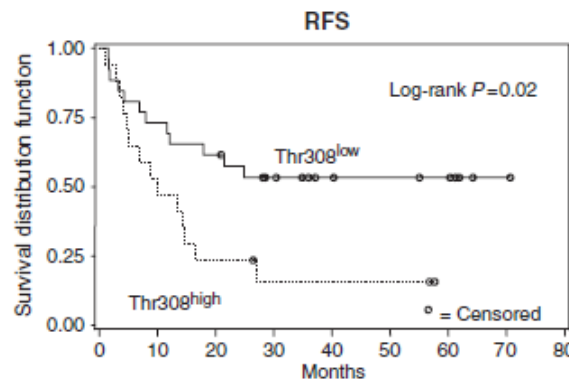
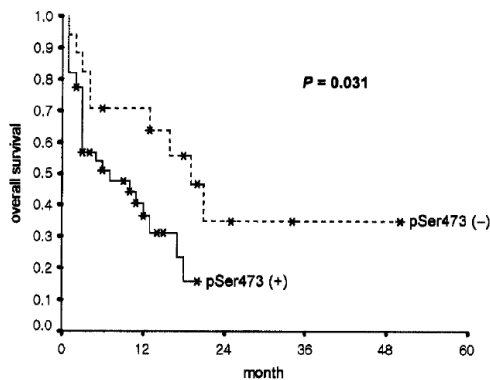
# Acute Myeloid Leukemia Market Overview

- 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

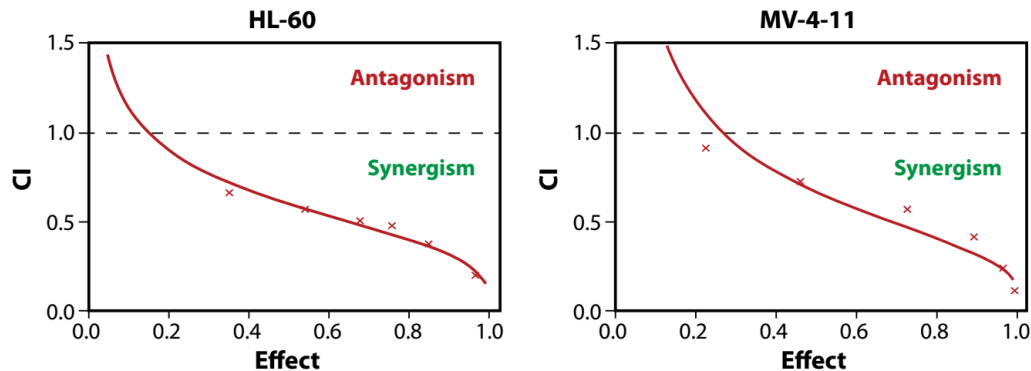
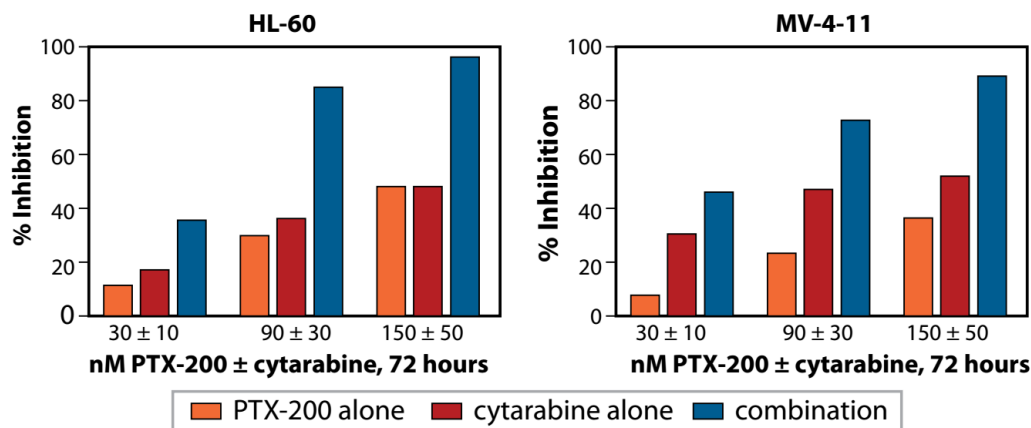
- Frequent constitutive Akt activation (phosphorylation) in AML
  - » Constitutive phosphorylation (Ser473 and Thr308) of Akt in AML compared to normal bone marrow cells in 44 out of 66 of patients (72 %)
- Implications for Akt as a modulator of chemotherapy resistance in AML
- High Akt phosphorylation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



1. Min YH, et al. Leukemia 2003; 17:995
2. Gallay, et al. Leukemia 2009; 23:1029

# 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 I in AML (monotherapy)

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

THE UNIVERSITY OF TEXAS  
MD Anderson  
Cancer Center

MOFFITT  
CANCER CENTER



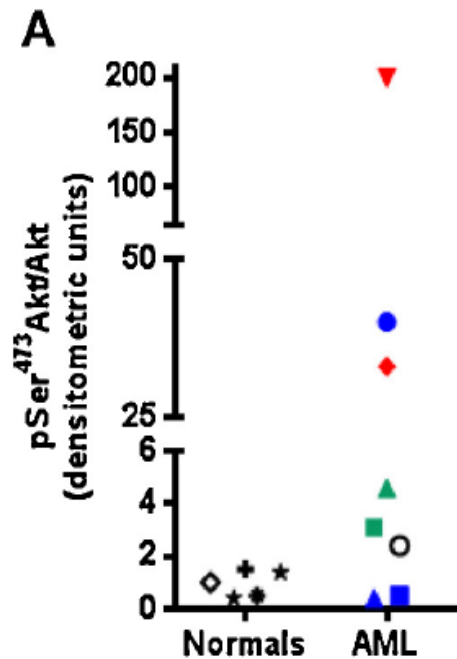
Published Leuk Res.  
2013  
Nov;37(11):1461-7



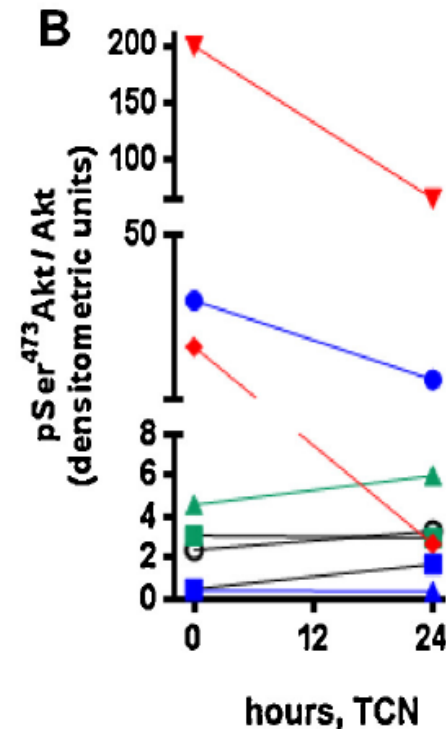
# PTX-200 reduced p-Akt in AML blasts

- High p-AKT cells more sensitive to PTX-200

» p-Akt/AKT in AML samples before therapy



» Action of PTX-200 on Akt phosphorylation in AML blasts



# Compelling evidence for PTX-200 in AML

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

# Spectacular rise of Celator Pharmaceuticals

- Celator (NASDAQ: CPXX) soared to ~\$780M valuation on positive Phase III data in AML (naïve/newly diagnosed secondary AML)
- Reformulation of existing standard of care (liposomal cytarabine + daunorubicin)
- Jeff Lancet was the Principal Investigator – also leading PTX's AML trial
- **Fantastic precedent for PTX in improving current standard of care in AML!**



# 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
- Ready to initiate trial 1H 2016
- 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



# Pipeline Yielding Multiple Layers of Value

- Edison Investment Research: Sum-of-parts valuation DCF valuation
- Risk-adjusted valuation of **\$0.53 per share (\$0.45 fully diluted)**

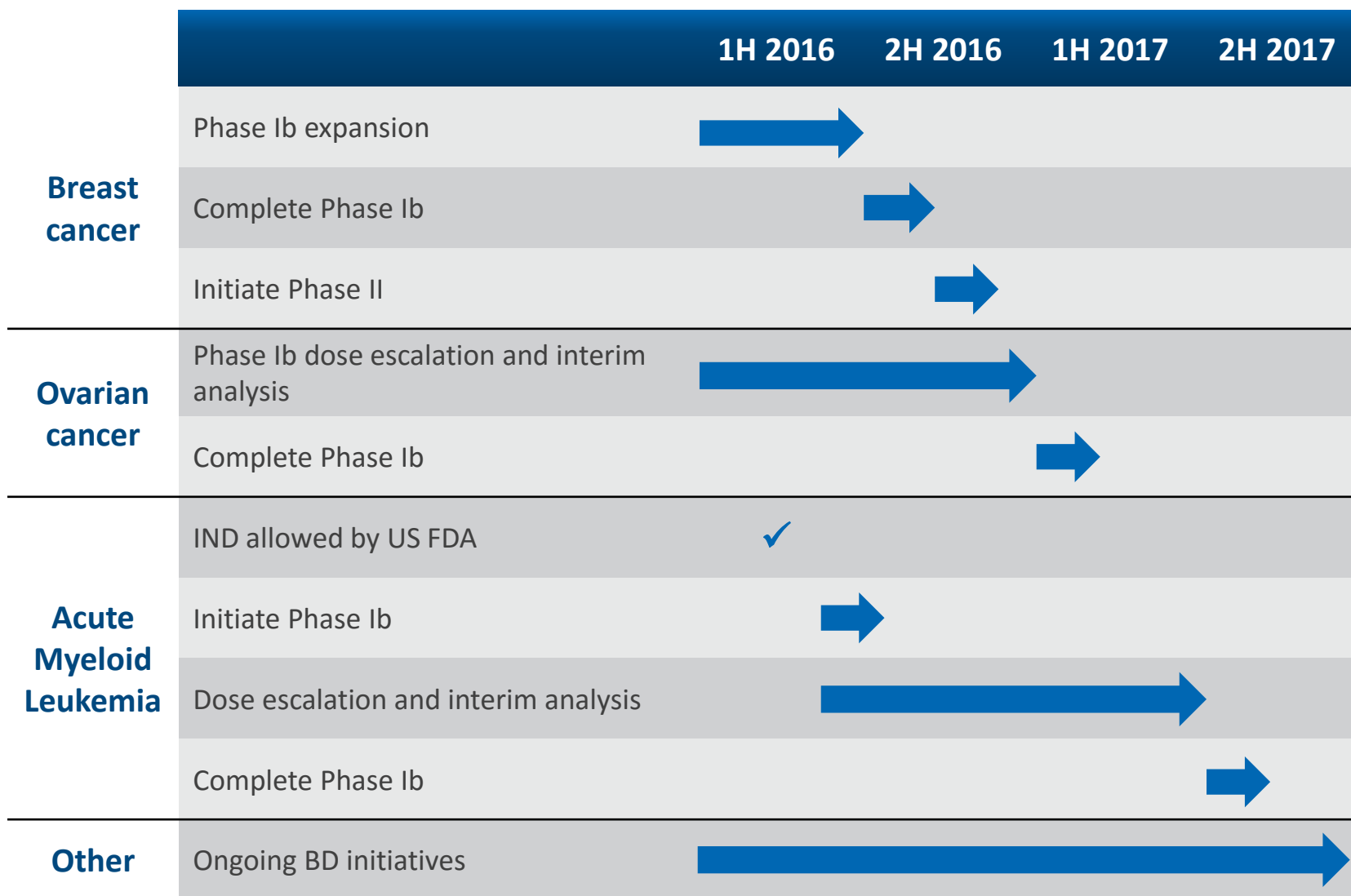


	rNPV (A\$ M)	rNPV/share (A\$)
Breast cancer (PTX-200)	\$13.9 M	\$0.15
Ovarian cancer (PTX-200)	\$5.4 M	\$0.06
AML (PTX-200)	\$17.6 M	\$0.19
Rest of pipeline (PTX-100; PTX-200)	\$21 .0 M	\$0.22
SG&A to 2022	(\$10.4 M)	(\$0.11)
<b>Portfolio total</b>	<b>\$47.5 M</b>	<b>\$0.53</b>
<b>PTX current market price<sup>1</sup></b>	<b>\$11.7 M</b>	<b>\$0.12</b>

1 - As at 19 April 2016

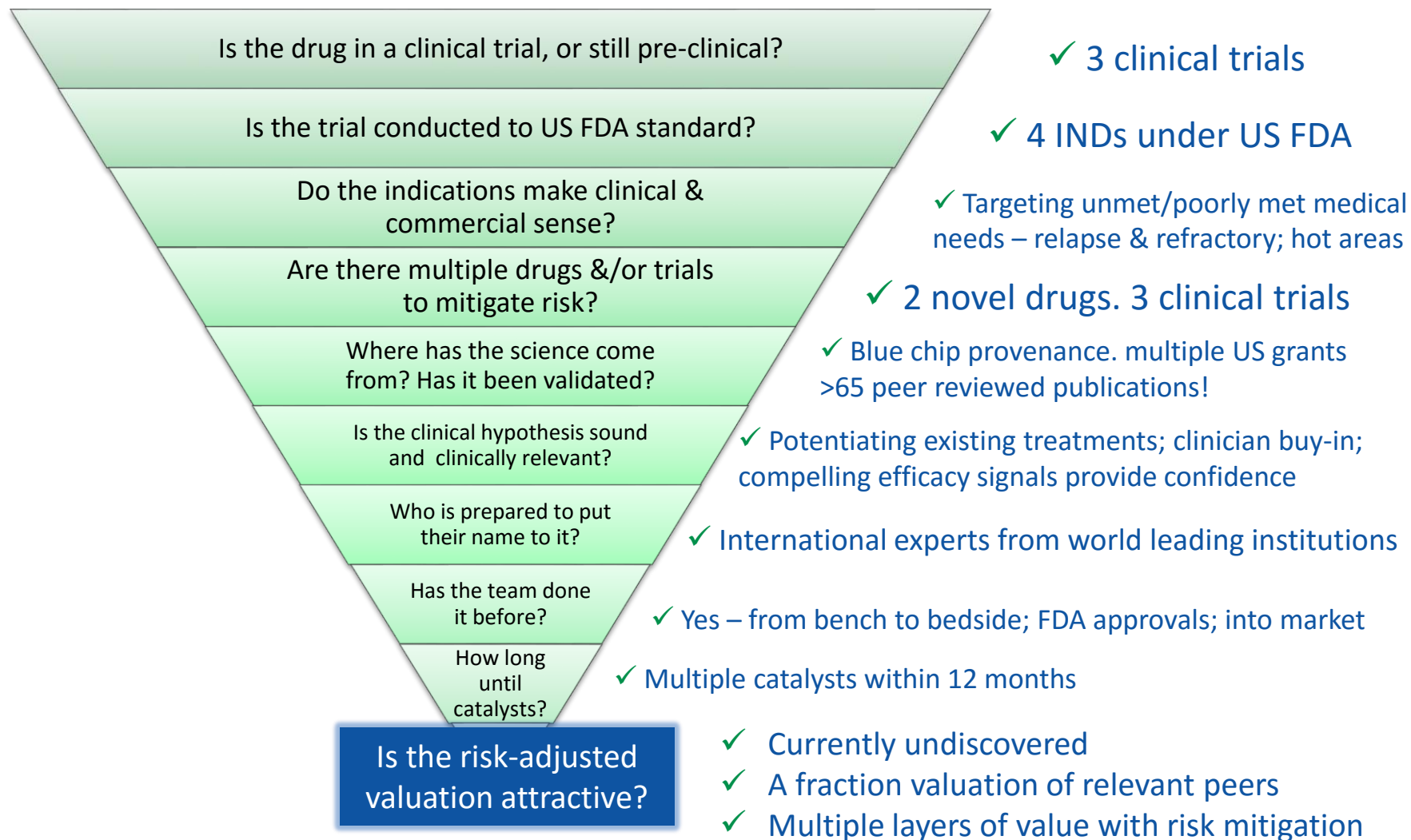
Report dated 2 March 2016. Full report available at: <http://www.edisoninvestmentresearch.com/research/company/prescient-therapeutics>

# Catalysts To Watch Out For





# Investment decision funnel for any biotech





## Contact

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A large, stylized blue molecular structure graphic is positioned in the background of the slide. It features four large circles connected by thick blue lines. One circle is on the left, connected to a central circle. This central circle is then connected to two other circles, one above and one below it.

[ptxtherapeutics.com](http://ptxtherapeutics.com)