



**Near term opportunities.**  
**Long term value.**

**ASX: PTX**

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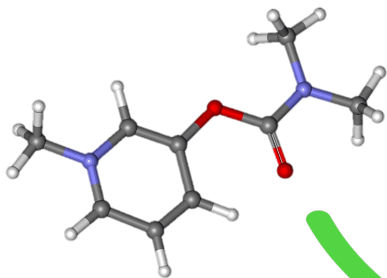
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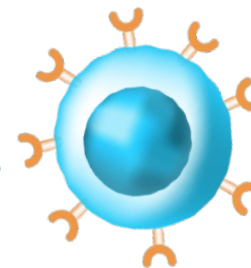
# Developing personalised novel therapies in diseases of unmet need

## Targeted therapies

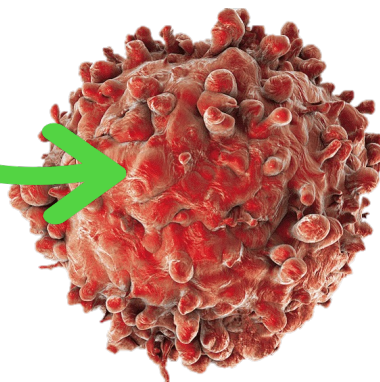


Targets switches  
**inside**  
cancer cells

## Cell therapies



Targets markers on the  
**outside**  
of cancer cells



# Investment highlights



## World class pedigree

We license from the best;  
and work with the best



## +\$20M cash

Long runway for multiple catalysts



## Many shots on goal

for substantial value creation



## Targeted therapies

Encouraging data in cancers with unmet need  
**Rapid pathway to commercialisation**



## 2 Cell Therapy platforms



CellPryme



OmniCAR

Internal & external opportunities



## Upcoming newsflow

from multiple programs

# Diversified portfolio of later stage and emerging assets

## Targeted therapies

### Encouraging Ph1b data in TCL

- Exceeding SoC expectations in an area of unmet need

**Aiming for Ph2 registration trial in 2024**



CellPryme

**Cell therapy enhancement platform with  
demonstrated benefits ready for the clinic**



OmniCAR

**Platform with potential to revolutionise cell  
therapy in pre-clinical development**

# Summary: a year of change and progress

## PTX-100

- Phase 1b demonstrated exciting progress and data
- Safety and patient responses beyond expect Standards of care
- Planning subsequent Phase 2 in TCL 2024



## CellPryme

- Excellent developmental progress throughout year; demonstrating cell therapy enhancement capabilities;
- Substantial effort to increase visibility in recognition of partners' capital limitations



## OmniCAR

- In adapting to a capital limited environment for cell therapies, shifted from expensive pre-IND work & clinical work to strengthening pre-clinical platform development

- Prolonged sector downturn, capital markets constraining biotech development and partnering appetite
- Prudent managing of company resources
- Portfolio prioritisation from early stage/high risk/high expense activities towards more prospective, later stage clinical activities



# Market backdrop

# Persistent sector headwinds

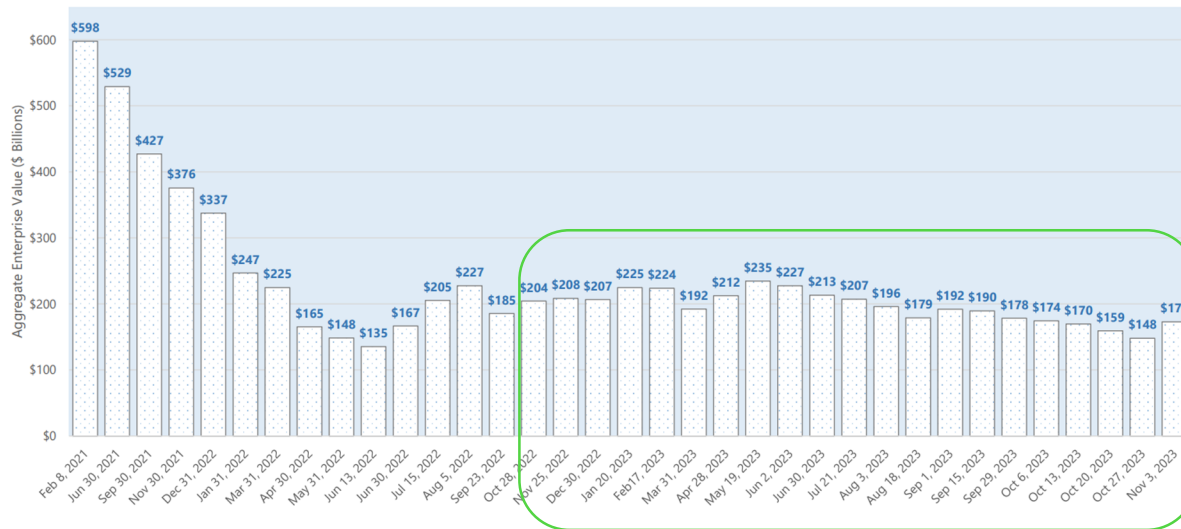
- Worst extended biotech bear market on record?
- Stubbornly high inflation and high interest rates combine to devalue the biotech sector
  - Impacts long-dated assets
  - Higher returns on low-risk asset classes drains funds from biotech sector
- Recession fears persist in Australia and abroad; outlook vacillates week-to-week
- Geopolitical and political uncertainty compounds risk aversion
- US Inflation Reduction Act (2022) lowering drug prices and future revenue expectations
  - Some programs being cancelled
- Extreme capital constraints for biotech as market largely remains shut
  - Forcing biotech companies to lay off staff and cut programs



# Biotech valuations impacted globally

Enterprise valuations\* (a proxy for technology value) remain at low levels for the global biotechnology sector

Total Enterprise Value of Publicly Traded Global Biotech, Feb 8, 2021 to Nov 3, 2023 (\$ Billions)



EVs are:

**Down 71%** since peak

**Down 15%** last 12 months

**>200 companies trading below cash**

Even though these cash levels have fallen in last year

\*EV = Market capitalisation – cash + debt

Source: CapitalIQ;Stifel

# PTX-100

FIRST IN CLASS  
RAS PATHWAY INHIBITOR

# T-cell lymphomas (TCL)

- TCL occurs when T-cells undergo changes and become cancerous
- TCL is 10-15% of all non-Hodgkin lymphomas
- Rare (orphan) disease
  - Incidence of 27,263 cases in the 8 major markets in 2020
  - Prevalence of 90,275 cases in the 8 major markets in 2020
- TCL represents an area of high unmet need
  - Poor patient outcomes – even responders often relapse
  - New entrants, but outcomes are still poor, especially in relapsed and refractory disease
  - **Typically expect ORR ~30% and PFS ~4 months**
- TCL is not one disease
  - ~2/3 PTCL; 1/3 CTCL
  - Includes ~20 different sub-types of TCL
  - Diverse characteristics
- Makes treatment for any one sub-type even trickier

GlobalData

Weinstock; *et al*; Hematology; 2018

Lymphoma Australia

8 major markets: US, France, Germany, Italy, Spain, UK, Japan, and China

PTCL: Peripheral T-cell Lymphoma

CTCL: Cutaneous T-cell Lymphoma

ORR: Overall Response Rate

PFS: Progression Free Survival

# Advantages of Orphan Drugs



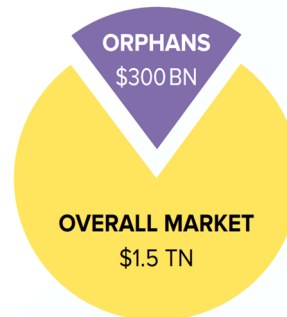
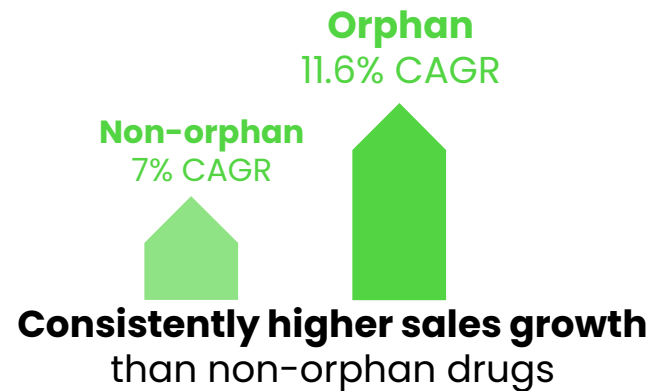
7 years of **guaranteed market exclusivity** in US



Enjoy **higher prices**



Sales are **more resilient** to cycles



Total orphan sales  
to reach  
**\$US300B** by 2028

# PTX-100's progress:

## What does it mean for PTX?

- PTX is on the verge of a major inflexion point with the start of a Phase 2 study for PTX-100
- Biggest catalyst in company's history, and the culmination of years of work
- Potential for the Phase 2 trial to be a registration study (i.e. **the study required to get a drug into the market\***)
  - Could accelerate clinical development
  - Greatly truncate the time and money required to approve PTX-100
- PTX could be the only ASX-listed biotech company with a drug in a registration study next year
- Orphan Drug Designation from FDA protects PTX-100 for 7 years post approval

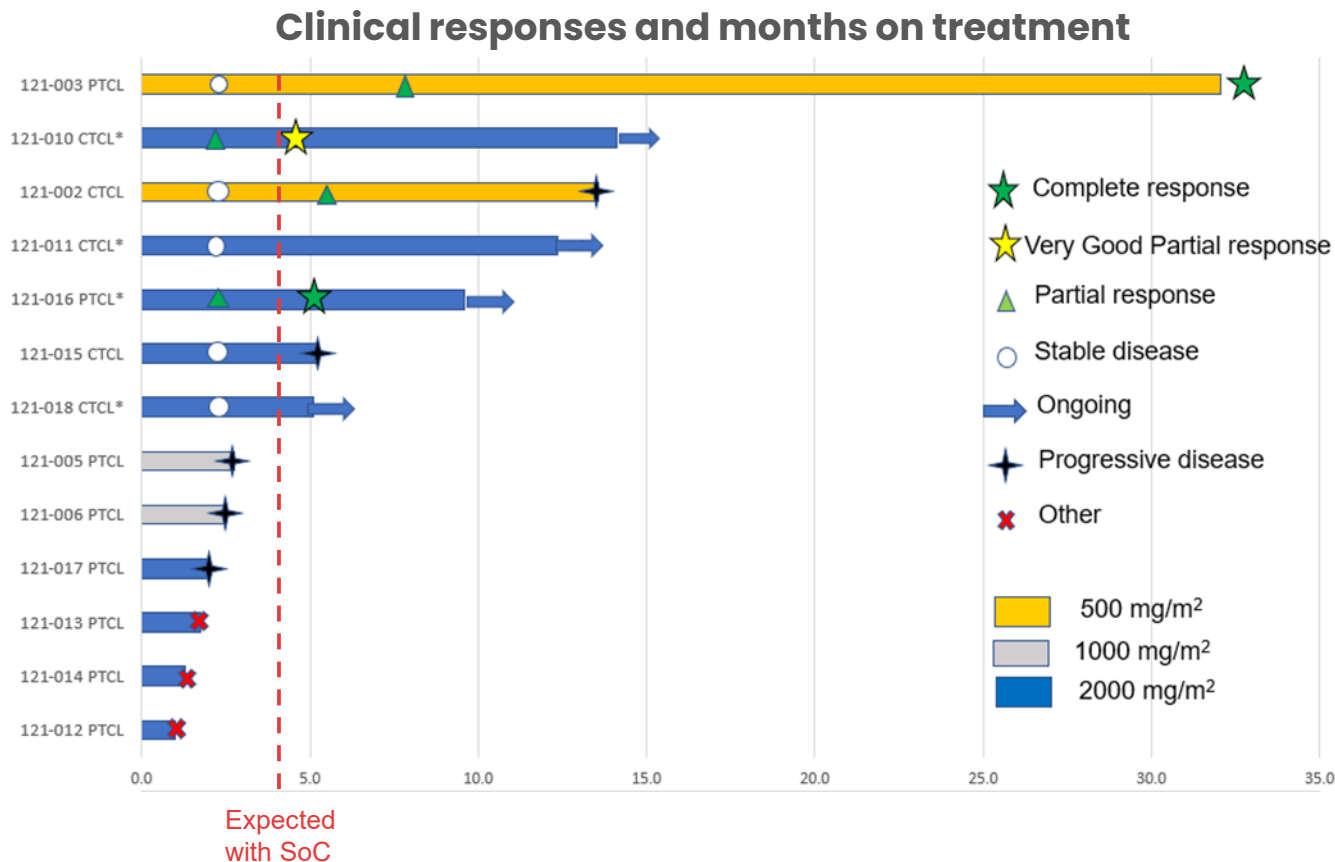
\* Subject to trial meeting or exceeding endpoints required by regulatory body

# Meaningful progress of PTX-100 Ph1b trial in r/rTCL

- Ongoing encouraging data continues to unfold from Ph1b study
- Data significantly more positive than 12 months ago
  - **This time last year there were only 2 responders (2 PRs; no CRs) and much shorter PFS**
- Currently n=14 r/r TCL pts; 10 evaluable<sup>1</sup>
  - Excellent safety profile
  - 4/10 responses (2CR & 2PR)
  - Additional 2 pts with durable SD >6 months
  - Clinical Benefit rate of 60% (6/10)
- **Granted Orphan Drug Designation by FDA for all TCLs**
- Abstract accepted for presentation at prestigious American Society of Hematology Meeting in Dec 2023
- Significant Phase 2 manufacturing campaign underway
- Substantial efficiencies gained in manufacturing process (improved yield; lower costs)
- Building awareness of PTX-100 with potential partners as data unfolds

1. Trial ongoing; data cut-off as at 10 July 2023  
r/r TCL: Relapsed and refractory T-cell lymphoma  
CR: Complete Response  
PR: Partial response  
SD: Stable Disease

# Encouraging ongoing PTX-100 Ph1b clinical results





# PTX-100 Phase 2 variables for consideration

Variable	Considerations for PTX
Disease	<ul style="list-style-type: none"><li>• PTCL, CTCL or both? Different diseases with diversity of characteristics; needs and endpoints. Do both simultaneously, or lead in with one, then add an arm?</li></ul>
Doses	<ul style="list-style-type: none"><li>• Activity seen at all doses, but most information at 2000mg/m<sup>2</sup>. FDA's Project Optimus guidelines, (which seeks to identify the <i>lowest</i> active dose) will almost certainly require at least 2 dose levels per group in the next study, which increases no. of patients required (and therefore timing and costs)</li></ul>
Design	<ul style="list-style-type: none"><li>• Two-stage study with interim go/no-go could reduce expenditure commitment for PTX if early hurdles are not met. This is current preferred option. Ideally n≈120. Aust and US sites.</li><li>• Cross-over arms could be another way of exploring what line of therapy PTX-100 is best suited for, but could increase the no. of patients required and may compromise long-term endpoints.</li></ul>
Control arm	<ul style="list-style-type: none"><li>• A comparative study using approved drugs as controls. Increases the size and cost of the study, and perhaps the hurdle. However, the resultant data would be compelling for the regulators and clinicians, and potential corporate partners. Another factor is availability of each comparator drug in different territories (not all drugs are approved everywhere).</li></ul>
Registration study?	<ul style="list-style-type: none"><li>• It is PTX's aspirations for the Ph2 to be a registration study. If allowed by FDA, this would be highly significant, as this single study could lead to an approval. Would open pathways for rapid commercialisation. However, an additional confirmatory study upon approval is still required. PTX may be the only ASX company with a drug in a registration study.</li><li>• If not allowed, then a "regular" Phase 2 trial would still be a great outcome for PTX! It would require a subsequent study, as per conventional drug development pathways.</li><li>• Importantly, either pathway enables PTX to advance towards the goal of an approval.</li></ul>

# PTX-100 challenges and path forward

## Challenges

- Recruitment challenges in a rare disease, but looking to expand to multiple sites in next study
- 5 day I.V. dosing schedule is demanding on patients
  - Any changes to formulation will only be investigated after an approval of the initial formulation (or an unequivocal efficacy signal in Ph2)
- **MANUFACTURING IS EVERYTHING!**
  - GMP manufacturing and documentation for drug product to registration standard is on much more stringent level than Phase 1!!!
  - PTX bolstering capabilities in this area
- A changing regulatory environment creates uncertainty about Phase 2 trial design and potential for registration study & requires careful liaison with KOLs and regulator(s)

## Going forward

- Presentation of Ph1b data at ASH (Dec 2023)
- Manufacturing underway (current)
- Quality systems & controls (current, ongoing)
- Liaison with global KOLs (Q4 2023/Q1 2024)
- Protocol drafting (current)
- FDA meeting (Q1/Q2 2024)
- Manufacturing completion and drug delivery (2Q 2024)
- Phase 2 trial open (~mid 2024)
- Expansion to US sites (2H 2024)
- To partner or go solo? Depends on factors including territory; market size; costs to take forward; partner interest; territory economics
  - Conceptually, a potential deal could comprise: R&D costs + upfront, milestones and royalties; depending on rights and level of success of clinical data

# PTX-200

NOVEL AKT INHIBITION

## Status

- 27 patients treated with PTX-200 at escalating doses in combination with 200-400 mg/m<sup>2</sup> cytarabine (chemo)
  - 4 patients with CR/CRi
  - 1 patient with PR
- After 40 years bereft of innovation, the AML landscape has shifted significantly in recent years, with many new therapies available
- This has significantly impacted recruitment

## Going forward

- Reviewing package – possibility of not proceeding
- Finalise current study and complete data package. Requires minimal resources from PTX
- Review entire data to determine clinical and commercial viability of PTX-200
  - Future activities that add value for PTX without much additional resources from PTX?
    - e.g. new combination studies in investigator-initiated trials
  - Consideration must be given to limitations of patent protection (no composition of matter)

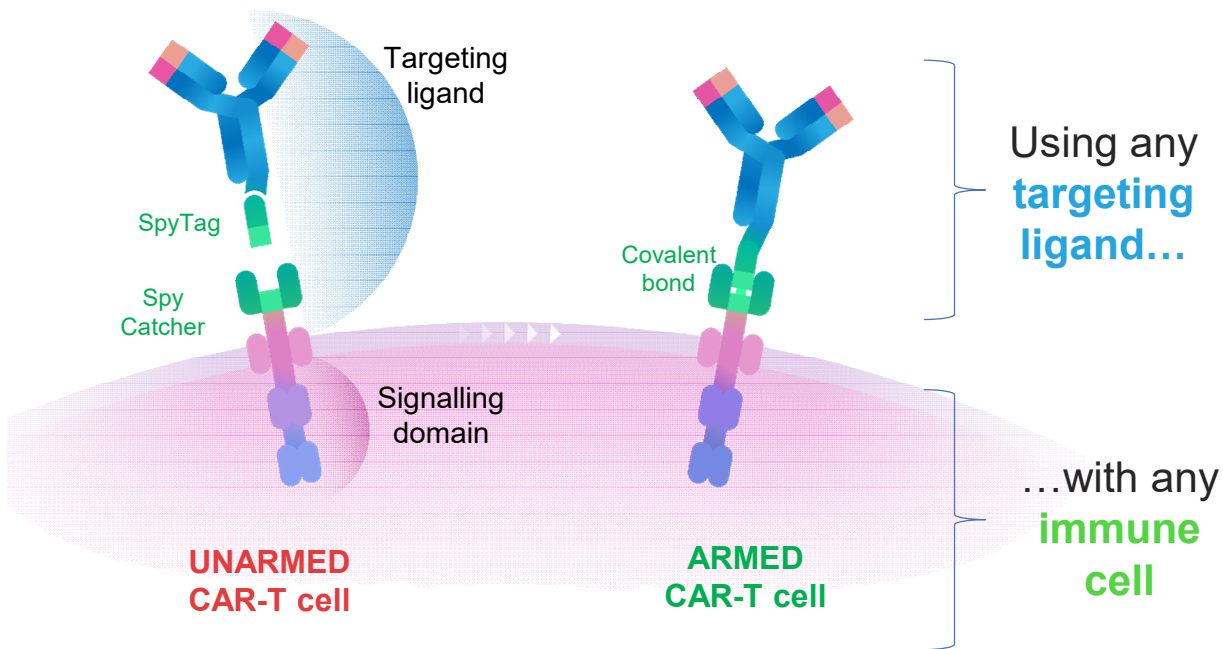
**Canceling unproductive projects after appropriate testing and after considering creative alternatives is part of what biotech companies are paid to do, and of course we are prepared to do it now.**



# OmniCAR

**Universal, Next Generation  
CAR-Therapies**

# OmniCAR: flexible, modular CAR platform



Associate Professor  
Daniel J. Powell, Jr



Professor  
Andrew Tsourkas



UNIVERSITY OF  
OXFORD

- As previously discussed, adapted to a capital limited environment for cell therapies, shifted from expensive pre-IND work & clinical work to strengthening pre-clinical platform development
- Progress of pre-clinical platform development
  - Explored binder PK - doses and scheduling against dynamic background of varying cell numbers
  - Binder resting led to superior performance
  - Retrogenix studies showed no undesirable binding to library of over 6,500 human proteins
  - Further immunogenicity studies confirmed acceptable immunogenicity of SpyCatcher & SpyTag. AML binders were slightly more immunogenic but considered acceptable by KOL
  - Assay development: developed and patented unique antibody against SpyCatcher
    - Technically challenging
    - Important for detection of any OmniCAR cell product – another barrier of IP protection
- Successful collaboration completed with large US partner:
  - Successful proof of concept of non-viral transduction; automated, scalable manufacturing
  - Business development interactions
- Continued business development. Met with dozens of companies during the year, including at key conferences including CAR-TCR; International Society of Cell & Gene Therapy (ISCT) & BIO
  - Clear that cell therapy headwinds are significantly stifling partnering appetite



## Challenges

- Huge expenses for internal clinical development: not only of T-cells and GMP viral vectors, but also GMP-like binders. Portfolio re-prioritisation reflects the risk-averse environment
- Some models showed less control (tonic signalling) of unarmed T-cells – under investigation and requires solving
- Potential partners/collaborators now significantly capital constrained, trimming their own teams and pipelines with greatly diminished appetite for:
  1. Additional programs; and
  2. Especially those requiring product re-design and early work

## Going forward

- Still believe that modularity can play a transformative role in cell therapies
- Taking the opportunity to optimise the platform preclinically; investigate unarmed activity; improve safety and control features
- Position OmniCAR favourably for when cell therapy sector regains buoyancy
- Can aim to bring in-house programs back on clinical path when environment improves



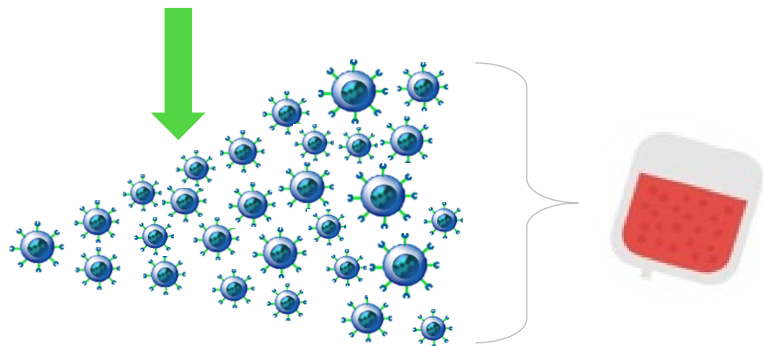
CellPryme

**CELL THERAPY  
ENHANCEMENTS**

# CellPryme enhances cell therapies in two ways



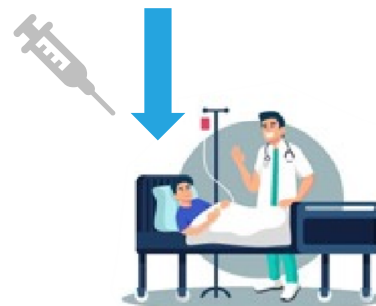
## MANUFACTURING ENHANCEMENT



Non-disruptive additive  
during cell manufacturing

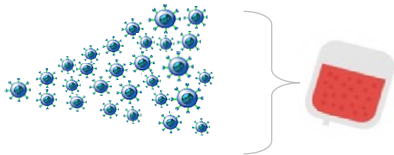


## ADJUVANT THERAPY



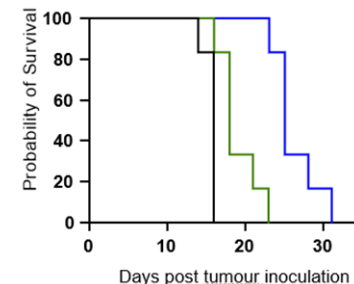
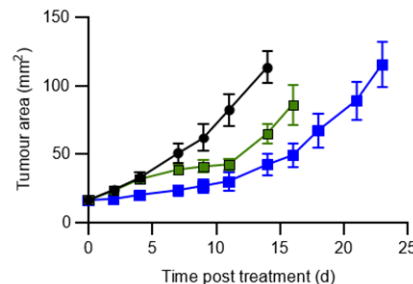
Administered to patient  
alongside cell therapy

## MANUFACTURING ENHANCEMENT

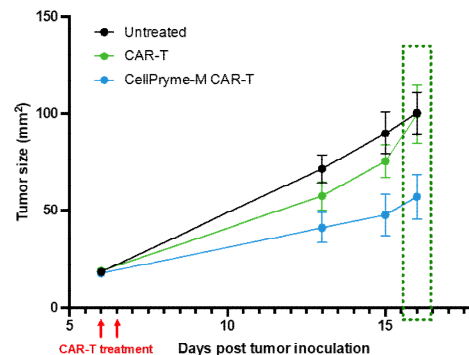


### PRODUCES SUPERIOR CELLS

- 50% more “youthful” Tcm cells
- Last longer; potent killing
- Doubles helper Tcells
- **Doubles tumour control & survival**



— Control  
 — Conventional CAR-T  
 — Conventional CAR-T + CellPryme-M



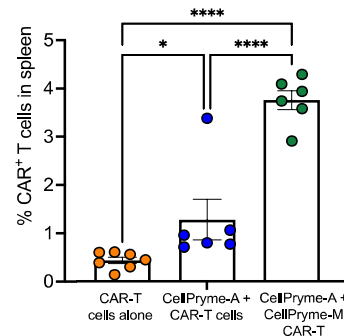
## ADJUVANT THERAPY



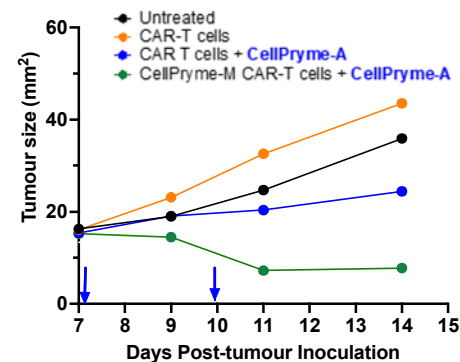
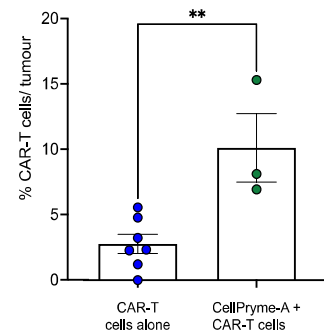
## BREACHES THE CANCER'S CASTLE WALLS

- 9X more CAR-T cells
- 4x penetration the cancer's protective barriers
- **Very strong cancer killing synergies with CellPryme-M!**

↑9x expansion

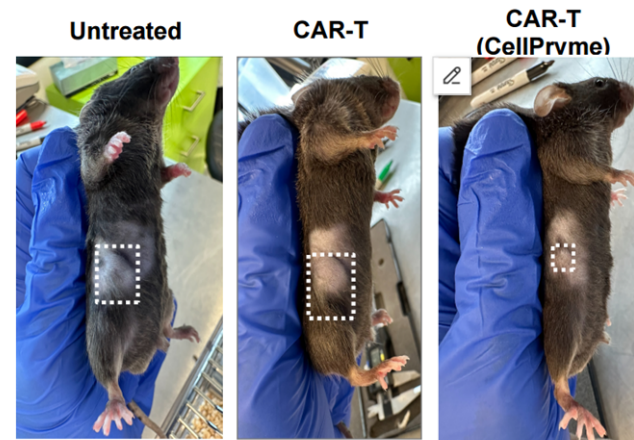


↑4x tumour penetration



# CellPryme: technical progress

- Validation from 10 repeated *in vitro* studies and 3 repeated *in vivo* studies
- CP-M: Improvements to exposure protocol
  - Stabilised CAR expression
  - Further increase in central memory CAR-T cells
- Repeat antigen stimulation models
  - Prevented CAR-T exhaustion
  - Downregulation of immune checkpoints
- Demonstrated that manufacturing conventional anti-Her2 CAR-T with CP-M enabled dramatic improvement in tumour control in difficult-to-treat (CAR-T resistant) *in vivo* colon cancer model
- Demonstrated head-to-head superiority of CellPryme-M vs current industry standard cytokines IL7/15



**Adding CellPryme-M dramatically improved ability of anti-Her2 CAR-T cells to kill tumours in resistant colon cancer model**

# CellPryme: building awareness

## Progress

- Strong promotion to potential partners and collaborators
- Presentation of CP-A & CP-M at 10 conferences including Phacilitate; CAR-TCR; ISCT; OCTS; BIO; ARM Meeting on Mesa & Med; BIO Europe
- Engaged with dozens of parties representing biotech, pharma, CDMOs
- CP-M being tested by third parties under MTA (biotech and CDMO)
- Building appreciation of the potential benefits vs integration hurdle integrating CellPryme into 3<sup>rd</sup> party programs

## Context & learnings

- These parties did not even think of PTX as a partner a year ago. Now PTX has dozens of new relationships and multiple ongoing conversations based on reputation of innovation and trust.
- After over a year of intense, effective BD and marketing activities, PTX understands the market and is on top of the opportunities. PTX has built the relationships by generating confidence with potential partners.
- BD strategy has been enhanced by learning about the potential of incorporation of CellPryme-M into CDMOs
- CellPryme-M has a substantially lower hurdle to integrate than CellPryme-A.
  - CP-M likely about higher scale/lower value
  - CP-A requires new trial protocols and changes to reg packages to be used in combination with other CAR-T therapies. This requires significant effort & investment.
  - Some parties may use both. Case by case basis



# Cellpryme: challenges & path forward

## Challenges

- Clarity on regulatory documentation required for both CellPryme-M and CellPryme-A (as it straddles different regulatory divisions)
- User variation: Working closely with 3<sup>rd</sup> parties to ensure it works in their hands
- Capital constraints of partners and willingness to alter programs to incorporate CellPryme (a lower hurdle, but a hurdle nonetheless).
- Parties already in the clinic are more reticent to change their manufacturing protocol mid-stream, until they encounter problems that CP-M can solve. Therefore earlier and more persistent engagement is required
- Why aren't the major players engaging so far?
  - Feedback is that priorities are overcoming current challenges with access; pricing; label expansion; manufacturing capacity & logistics
- Trademark challenge (but Prescient is unlikely to be using the name to end users anyway)

## Going forward

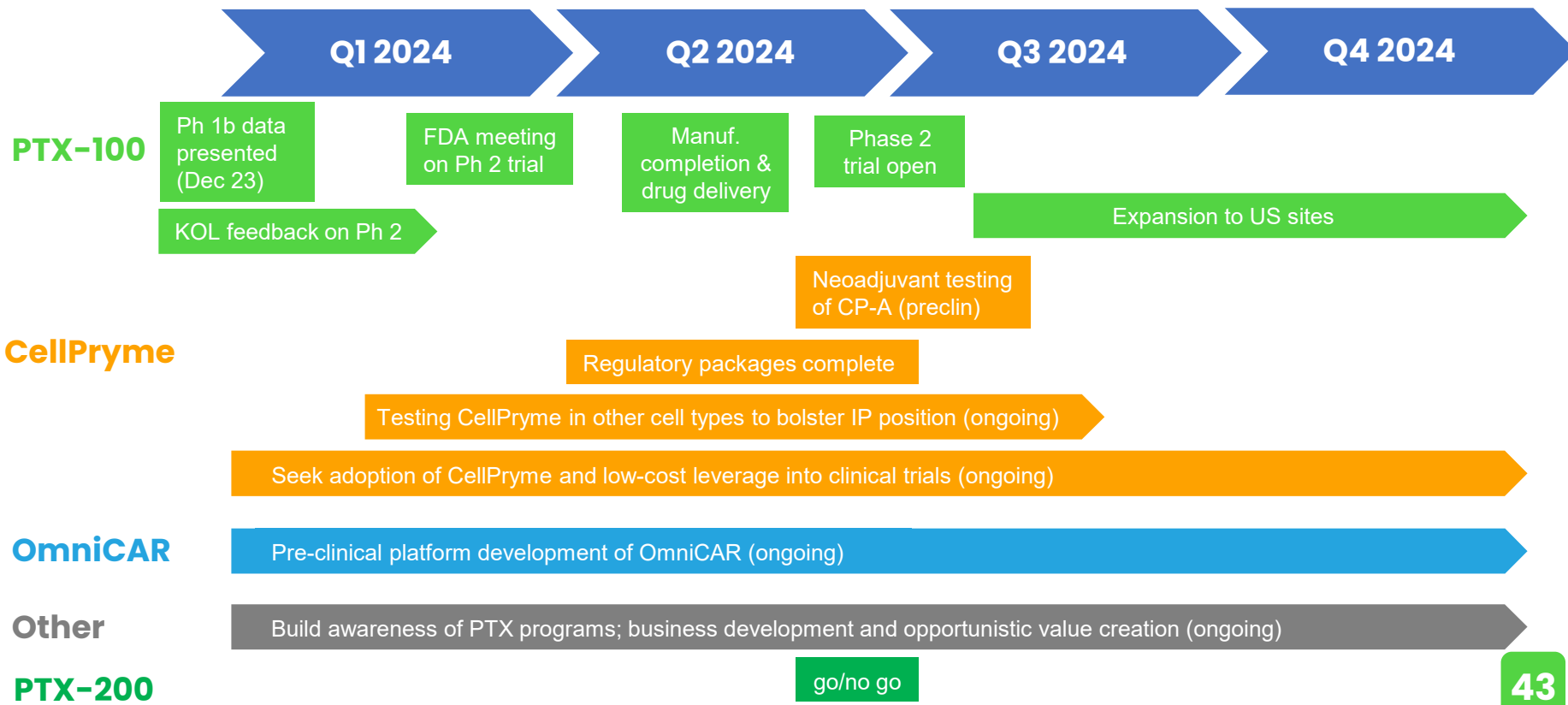
- Preparation of regulatory packages
- Seek leverage into clinical trials with partners & collaborators
- CellPryme-A schedule testing
  - Neoadjuvant (i.e. prior to CAR-T therapy)
  - Optimise frequency and doses
  - Important to better inform clinical studies
- Testing in other cell types
- Continue to build awareness of CellPryme with 3<sup>rd</sup> parties
  - Aiming for additional MTAs and agreements in 2024

- Dozens of active business development discussions
  - Numbers are highly fluid
  - Trade off between our interest in them vs their interest in us
- Most discussions are for CellPryme-M (easier to test & integrate)
- As conversations proceed, some invariably drop off and others progress
- Standard process for BD discussions:
  - Non-confidential discussions → confidential discussions → deeper due diligence (including how CP could work with partner's specific technology) → MTA → evaluation of results → troubleshooting & retesting → agreement
- Timelines vary greatly depending on partner on a case-by-case basis, but can take ~ 4-12 months from initial discussions to MTA

# Summary

# Major catalysts to work towards next year

Prescient will continue to progress the development of programs across its pipeline. Some notable catalysts to work towards include, but are not limited to:



# Aspirations for each program in 5 years if they succeed

**Not everything will go to plan, but any one of these successes could be transformative for PTX**

- PTX-100
  - Approved in r/r TCLs
  - In additional Phase 2 trials to dominate TCL:
    - Combination therapies
    - Earlier lines of therapy
    - Indications beyond TCL
  - New formulations to extend IP and broaden applications
- PTX-200?
- CellPryme
  - CellPryme-M generating modest but recurring revenues from a number of biotech and CDMO partners
  - CellPryme-A with 1st approval in combination with CAR-T and undergoing additional clinical trials for approvals with other CAR-T products
- OmniCAR
  - >1 Internal program in Ph1/2 trial
  - 3-4 External programs in clinical development, more in pre-clinical development
- Portfolio expansion in areas of expertise

## 3 Key Messages

### 1 PTX-100

#### Encouraging Ph1b data in TCL

- Exceeding SoC expectations in an area of unmet need

**Aiming for Ph2 registration trial in 2024**

### 2



CellPryme  
OmniCAR

#### Lower risk exposure to cell therapy

- Improve existing and emerging cell therapies
- Agnostic on cell type and targets

### 3

~\$19M cash

**Well capitalised to deliver on milestones**



**Thank you!**

**ASX code: PTX**

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