



**Spotlight on PTX-100:  
On the verge of a  
major inflexion point**

**December 2023**

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# Diversified portfolio of later stage and emerging assets

**Targeted  
therapies**

**Ph1b drug with potential for rapid clinical development.  
Encouraging activity in areas of unmet need**

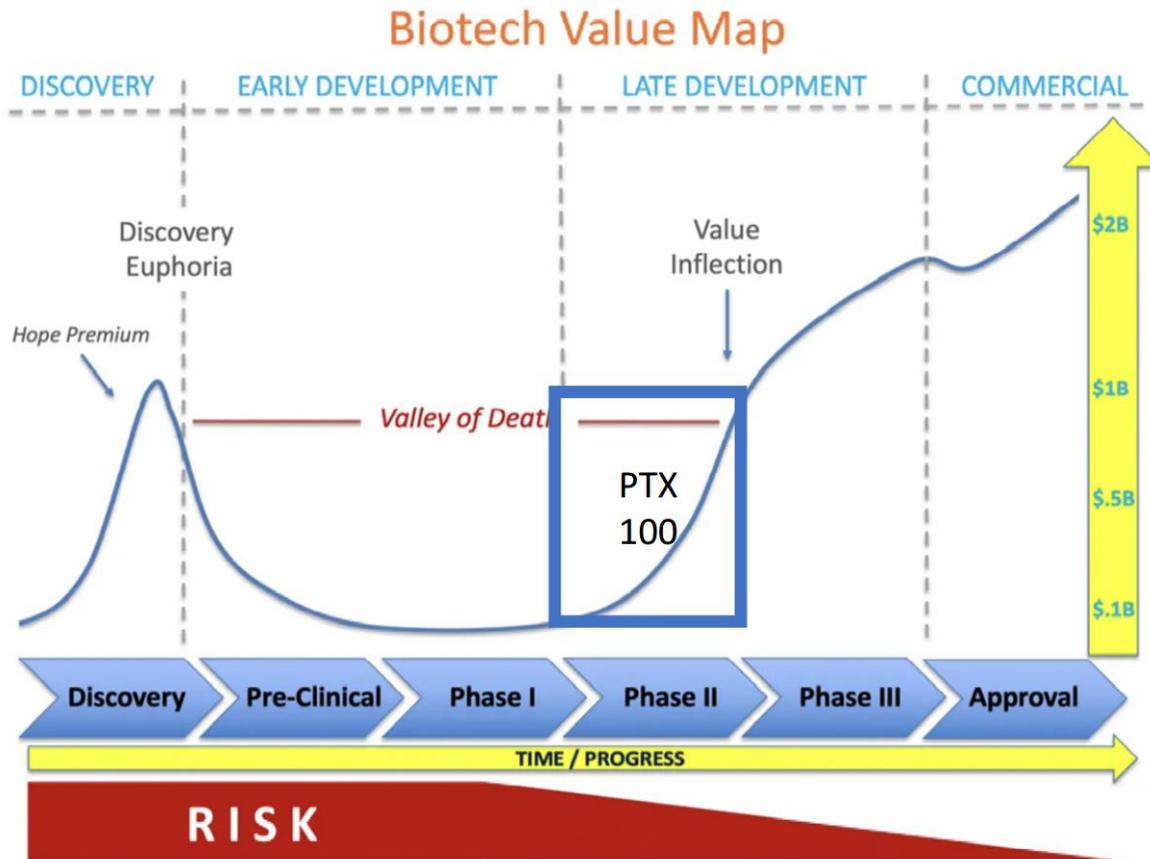
 **CellPryme**

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

 **OmniCAR**

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

# PTX is entering a new stage



# PTX-100 Background

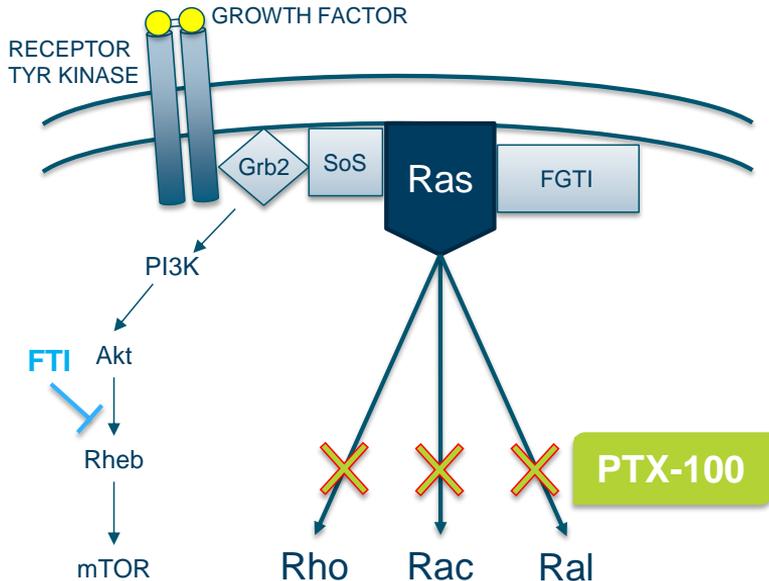
## First in Class inhibitor of Ras pathway

- Licensed from Yale
- Downstream MoA captures many Ras mutant variants
- PTX-100 is only RhoA inhibitor in clinical development
- Reduces cancer stem cells

## Clinical Status

- Phase 1b trial concluding in r/r TCL
- Excellent safety
- Encouraging efficacy vs expected from SoC
- Planning Phase 2 registration trial
- US Orphan Drug designation in all TCLs

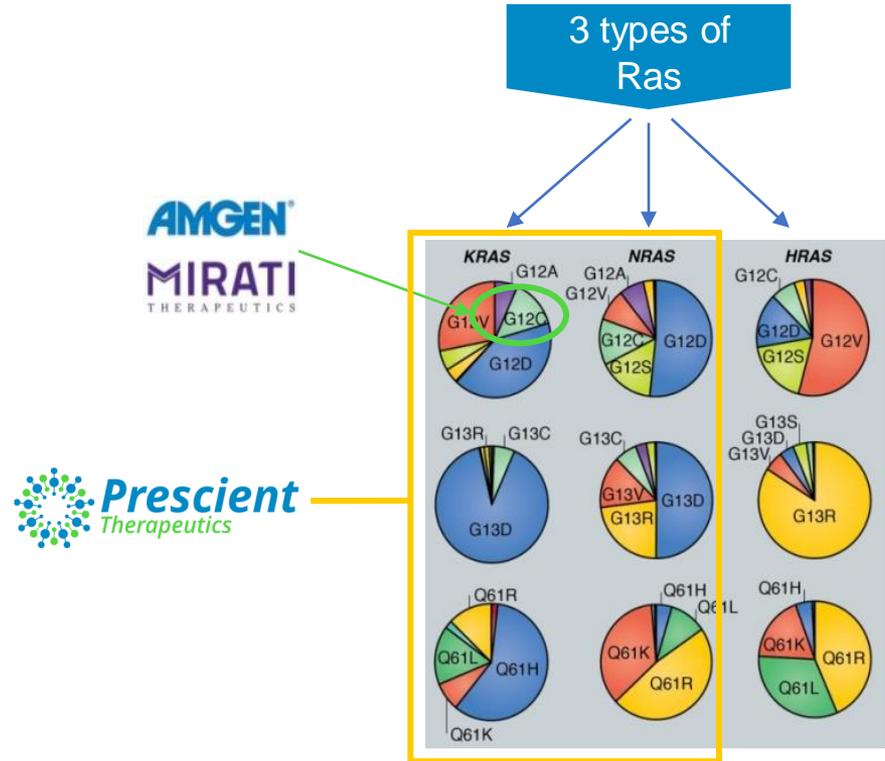
# Novel Ras pathway inhibition



PTX-100 disrupts the Ras pathway  
by **inhibiting the downstream activation of**  
**Ral, Rac and Rho**  
through **prenylation inhibition**

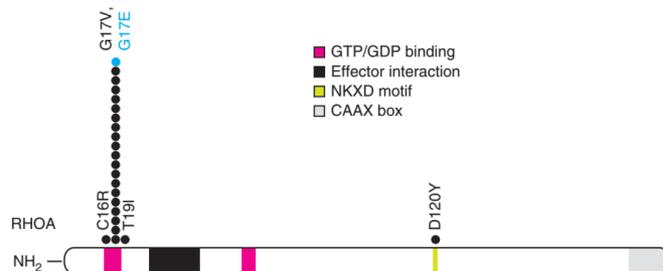
# PTX-100 addresses many types of Ras mutations

- 80% of cancer Ras cancer patients harbour **more than one Ras mutation!**
- Targeting one particular Ras variant (e.g. KRAS G12C) may lead to relapse driven by resistant clones
- PTX-100's unique MoA, working downstream, can potentially address **all K-Ras and N-Ras mutant cancers**



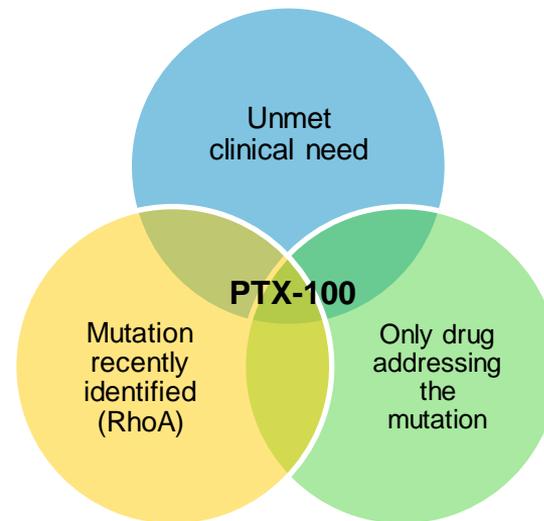
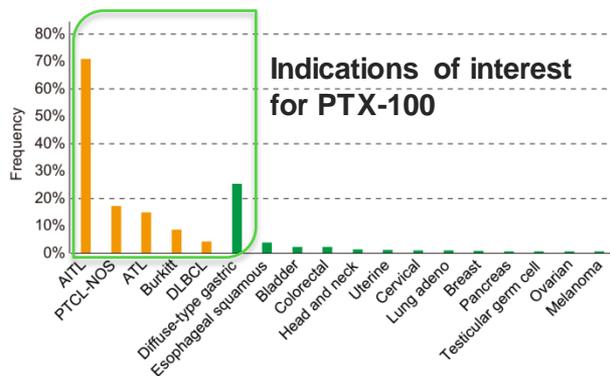
# PTX-100 is also the only drug targeting RhoA

## Schematic of RhoA protein structure, with alterations



- Various RhoA mutations drive a number of hematological and solid malignancies
- **PTX-100 a pan RhoA inhibitor** regardless of mutation
- **Only clinical stage RhoA inhibitor globally**

## Frequency of RhoA mutations in human malignancies



# 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

# Drugs in areas of unmet need can command higher prices

## Case Study

- Folutyn (pralatrexate)
- Approved 2009 for PTCL
- Overall Response Rate was 27%
- US \$842,585 per patient, per year



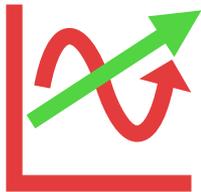
# Advantages of Orphan Drugs



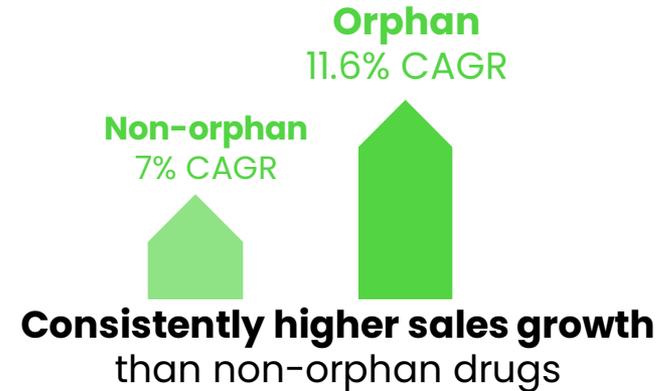
7 years of **guaranteed market exclusivity** in US



Enjoy **higher prices**



Sales are **more resilient** to cycles



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

# PTX-100

## Phase 1b study

# PTX-100: Ph1b Clinical Summary

- **Aims:** Phase 1b to evaluate safety PK/PD
- **Design:** Dose escalation in advanced malignancies; expansion cohort in relapsed & refractory T cell lymphomas
- **Results:**
  - Excellent safety
  - Target engagement at all 3 doses
  - **Response rates (incl 2 CRs) and mPFS in assessable pts with r/r TCL exceeding that expected with SoC**
- **Granted Orphan Drug Designation by US FDA for all TCLs**



Professor H. Miles Prince, AM  
Principal Investigator



# Strong response rates in difficult diseases

|                        | Overall Response Rate | Clinical Benefit Rate |
|------------------------|-----------------------|-----------------------|
|                        | CR + PR               | CR + PR + SD>6months  |
| Benchmark <sup>1</sup> | <b>30%</b>            | <b>45%</b>            |
| r/r PTCL<br>(n=4)      | 50% (2/4)             | 50% (2/4)             |
| r/r CTCL<br>(n=5)      | 40% (2/5)             | 80% (4/5)             |
| r/r TCL<br>(n=9)       | 44% (4/9)             | 66% (6/9)             |

1. Considered a target benchmark by Prescient and its investigators, with reference to currently available therapies in r/r TCL

Enrollment completed but study ongoing; results as at 1 Dec 2023

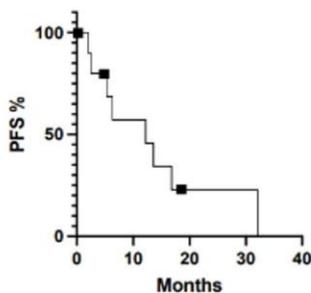
# Median Progression Free Survival (mPFS<sup>1</sup>) exceeds expectations

## mPFS benchmarks in r/r TCL:

Standard of care: ~4 months

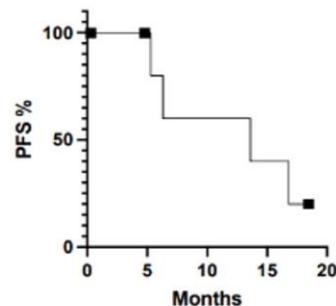
For a registration study: 5-6 months<sup>1</sup>

A. All r/r TCL pts  
(11 evaluable)



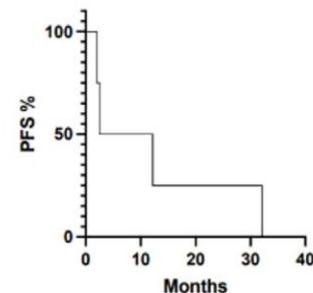
mPFS:  
12.2 months

B. r/r CTCL pts  
(7 evaluable)



mPFS:  
13.6 months

C. r/r PTCL pts  
(4 evaluable)

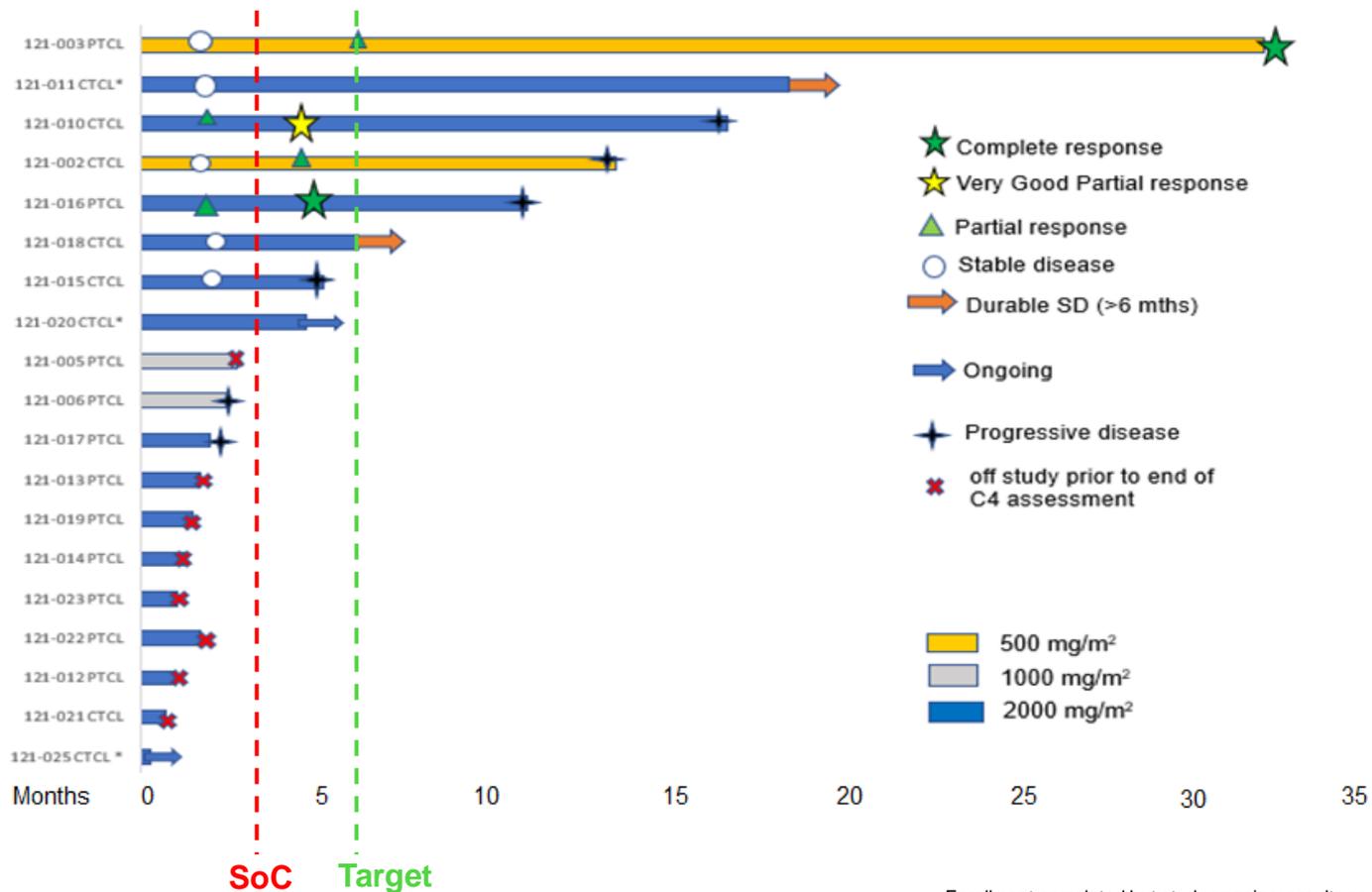


mPFS:  
7.4 months

1. Progression Free Survival is the time from first treatment until disease progression or death
2. Considered a target benchmark for a Ph2 or registration study in r/r TCL by key opinion leader (S.M. Horowitz *et al*; Blood; Dec 2021)

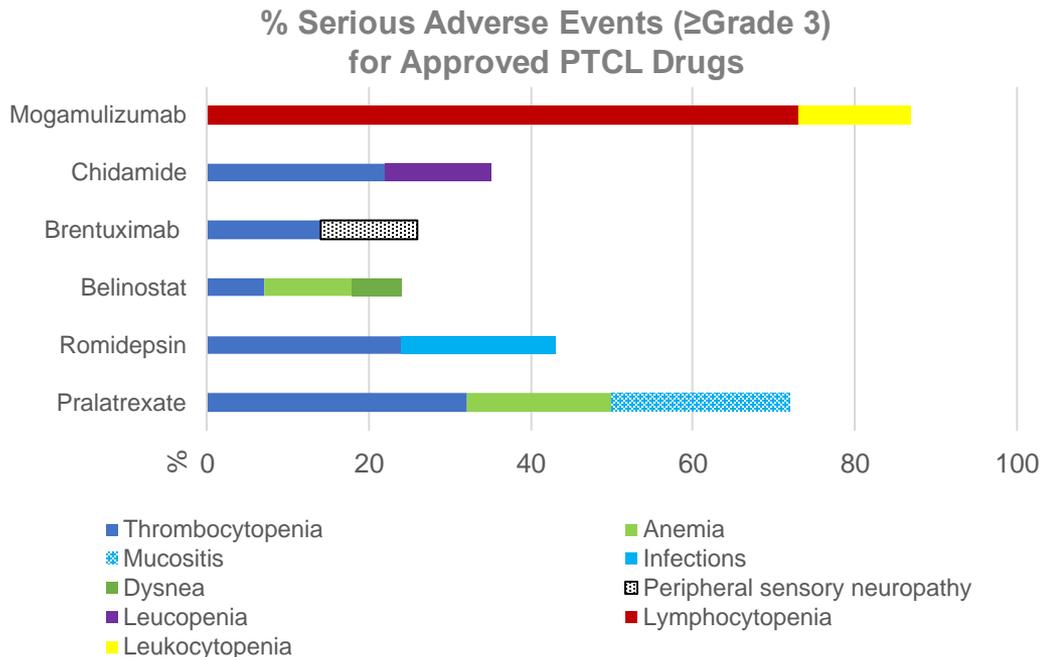
Enrollment completed but study ongoing; results as at 1 Dec 2023

# r/r TCL swimmer plot: responses and duration



# Favourable safety profile compared to peers

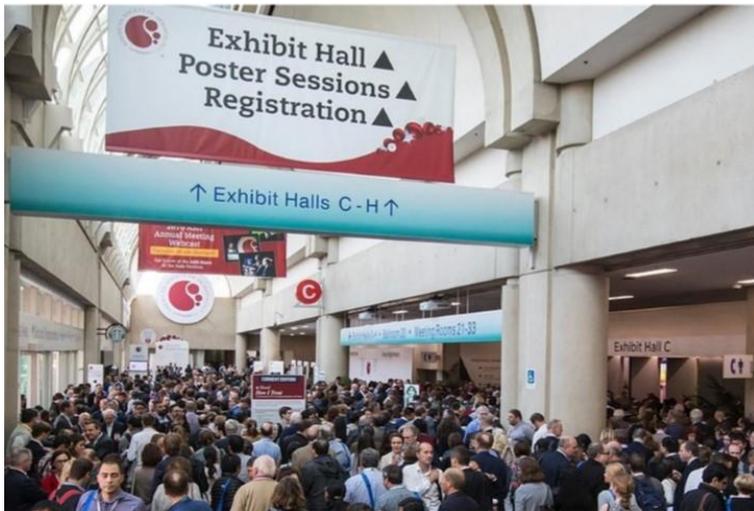
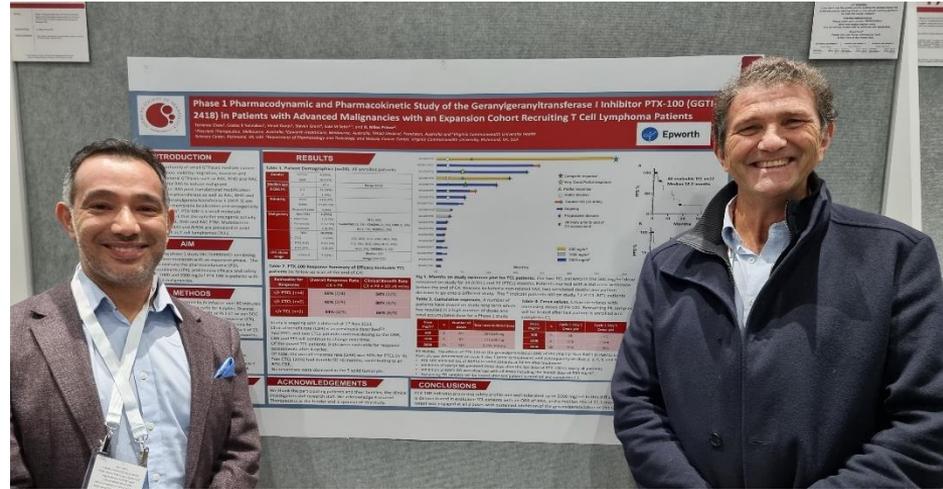
## Approved PTCL drugs have troublesome safety profiles



## PTX-100 HAS AN EXCELLENT SAFETY PROFILE

- No serious adverse events related to PTX-100
- Suits fragile patient population
- Good candidate for combination therapy

# Prescient at ASH Meeting 2023



# PTX-100 data at ASH

- PTX-100 data in TCL was received very positively by industry and KOLs
- The nature of PTX-100's efficacy observations seem unique :
  - Other TCL drugs only achieve durability when they achieve CR, which is a rare event
  - By contrast, PTX-100 patients experienced durability even with PRs and SD
- PTX-100's safety was considered a distinguishing feature
- For reference, two other posters on similar sized r/r TCL trials:

|  | Size        | ORR        | mPFS               | Safety                        |
|--|-------------|------------|--------------------|-------------------------------|
| PI3K $\gamma$ $\delta$ DNA-PK triple inhibitor | N=19        | 31%        | 5.6 months         | 88.5% pts had AE; 69% had SAE |
| GemDox (chemo)                                 | N=18        | 50%        | 5 months           | 100% pts had AE; SAE in 64%   |
| PTX-100  | <b>N=19</b> | <b>44%</b> | <b>12.2 months</b> | <b>0 pts had SAEs</b>         |

# Selected KOL quotes on PTX-100 in r/r TCL

## Overall very positive scientific and clinical feedback!

- *“This is more than an efficacy signal”*
- *“That swimmers plot looks good...hang on, that’s not weeks; it’s in months! Wow!”*
- *“Safety profile is the stand-out feature and could be the attraction vs competitors”*
- *“Heterogeneity of disease is going to be a problem. But Miles Prince is the world expert in different subtypes.”*

## Other observations:

- Current dosing scheduling is not ideal (can this be made more convenient?)
- Lots of competition for a small number of patients for trials
- Combinations are going to (eventually) be important for all PTCL players

# Moving ahead

# 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

- 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)

# PTX-100 Phase 2 variables for consideration

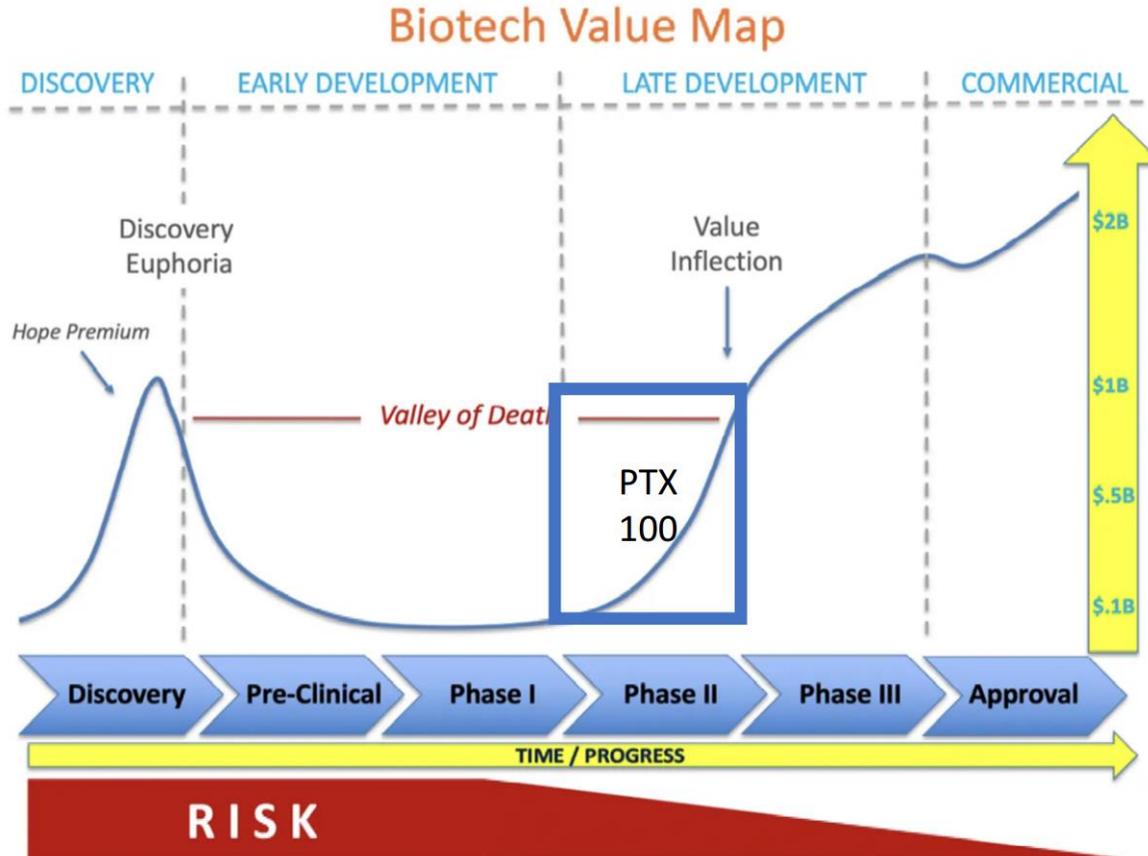
| Variable                    | Considerations for PTX   |
|-----------------------------|--|
| <b>Disease</b>              | <ul style="list-style-type: none"><li>• PTCL, CTCL or both? Different diseases with diversity of characteristics; needs and endpoints. Do both simultaneously, or lead in with one, then add an arm?</li></ul>   |
| <b>Doses &amp; Schedule</b> | <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>  |
| <b>Design</b>               | <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>   |
| <b>Control arm</b>          | <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>  |
| <b>Registration study?</b>  | <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> |

# What does PTX-100's progress 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

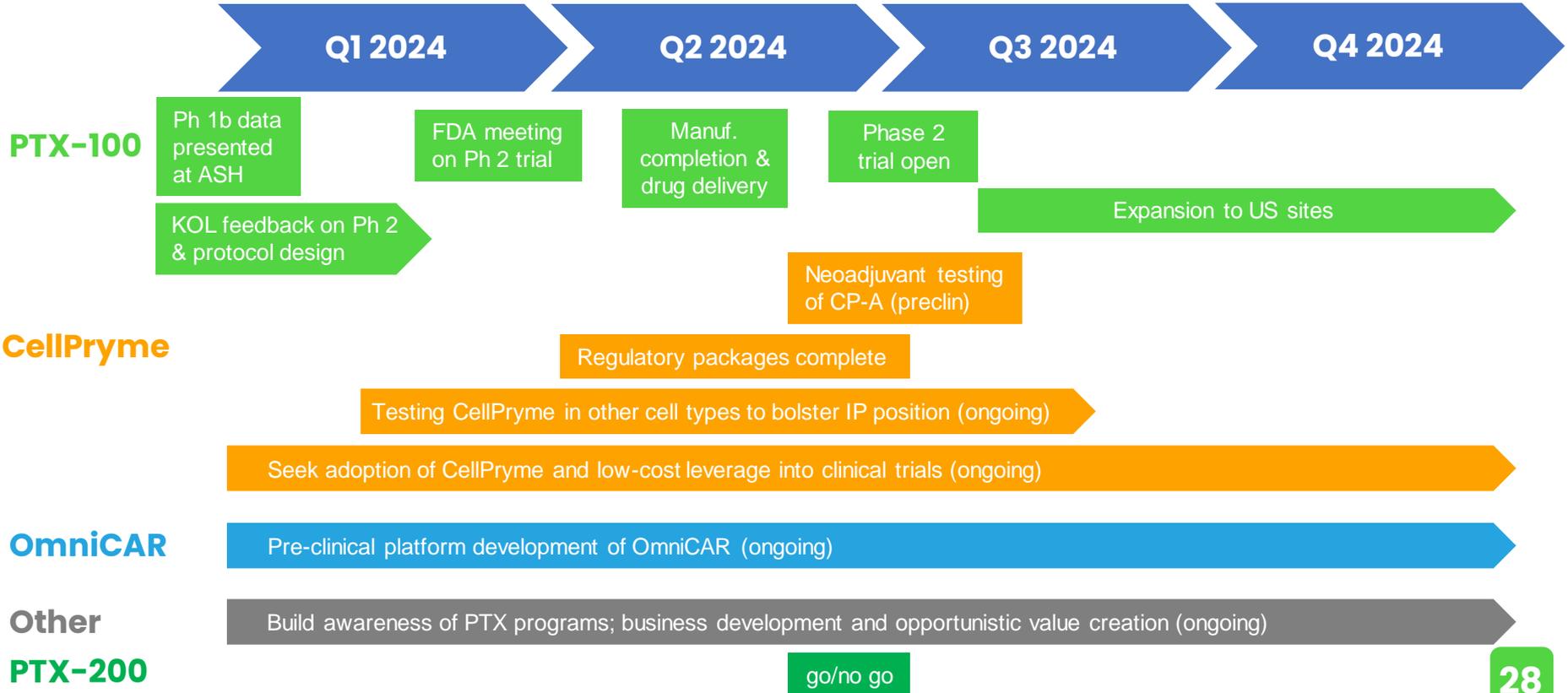
# PTX is entering a new stage



# 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:



## 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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