



MAJOR

INFLECTION

POINT

ASX: PTX

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# 3 Key Messages

## 1 PTX-100

### A major inflection point with Phase 2 Initiation

- Phase 2a clinical trial has begun
- Encouraging data in an area of unmet need
- First in class with a wider application

## 2 CellPryme OmniCAR

### Lower risk exposure to cell therapy sector

- Improves 3<sup>rd</sup> party cell therapies
- Agnostic on cell type and targets

## 3 \$12.1 Million\*

### Well capitalised to deliver on milestones

\*Near-term Liquidity (Jan 2025): \$12.1m

- Cash (31 Dec): \$6.4m
- Deposits (matured 9 Jan): \$2.0m
- R&D Rebate (received 10 Jan): \$3.7m

4C Jan 2025 for December 2024 quarter

# PTX: Targeting Improved Oncology Patient Outcomes

Through novel approaches to treat cancer including targeted and cell therapies

Led by an experienced team of drug developers and deal makers with a track record in blood cancers

## PTX-100

- Targeted therapy – one of the most advanced cancer therapies on ASX
- Treats cancers with high mortality rates and high unmet need
- Promising Phase 1b results in T Cell Lymphoma (TCL)
  - ✓ 64% response either halting or reducing disease
  - ✓ Extended duration of response compared to approved alternatives
  - ✓ Safety profile may have advantages in comparison to other treatment options
- Phase 2 study in Cutaneous T Cell Lymphoma (CTCL) underway
- FDA Orphan Drug Designation provides market exclusivity, regulatory support and fast-track approval potential
- Total TCL market US\$1.8B market in 2030 for 8 major markets\*

## Pre-clinical assets

- OmniCAR and CellPryme platforms have potential to improve CAR-T therapies

# Experienced team

- Experienced team of drug developers and deal makers with track record in blood cancers

## Management Team



James McDonnell  
CEO



Dr. Marissa Lim  
Chief Medical Officer



Upaly Bahadure  
Director – Clinical Affairs &  
Operations



Mariam Mansour, PhD  
Director – Clinical  
Development and  
Translational Sciences



Luis Malaver-Ortega, PhD  
Director Research and  
Development

## Board of Directors



Dr James Campbell  
Non-Executive Chairman



Dr Allen Ebens  
Non-Executive Director



Dr Ellen Feigal  
Non- Executive Director



Dr Gavin Shepherd  
Non-Executive Director

Experienced gained in global companies

Bristol Myers Squibb®

Telix

CSL

BeiGene

AMGEN

IPSEN

NOVARTIS

JUNO  
THERAPEUTICS

Cephalon®

VIFOR  
PHARMA

Genentech

# Investment Snapshot

<b>ASX Ticker</b>	<b>PTX</b>
Total Issued Capital	805 M shares
Share Price <sup>1</sup>	A\$0.048
Top 20 Own	18%
<b>Market Capitalisation<sup>1</sup></b>	<b>\$39M</b>
<b>Near-term Liquidity<sup>2</sup></b>	<b>\$12.1M</b>
<b>Enterprise Value</b>	<b>\$27M</b>

1. As at 28 March 2025
2. Near-term Liquidity (Jan 2025): \$12.1m
  - Cash (31 Dec): \$6.4m
  - Deposits (matured 9 Jan): \$2.0m
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## Share price performance over past 12 months

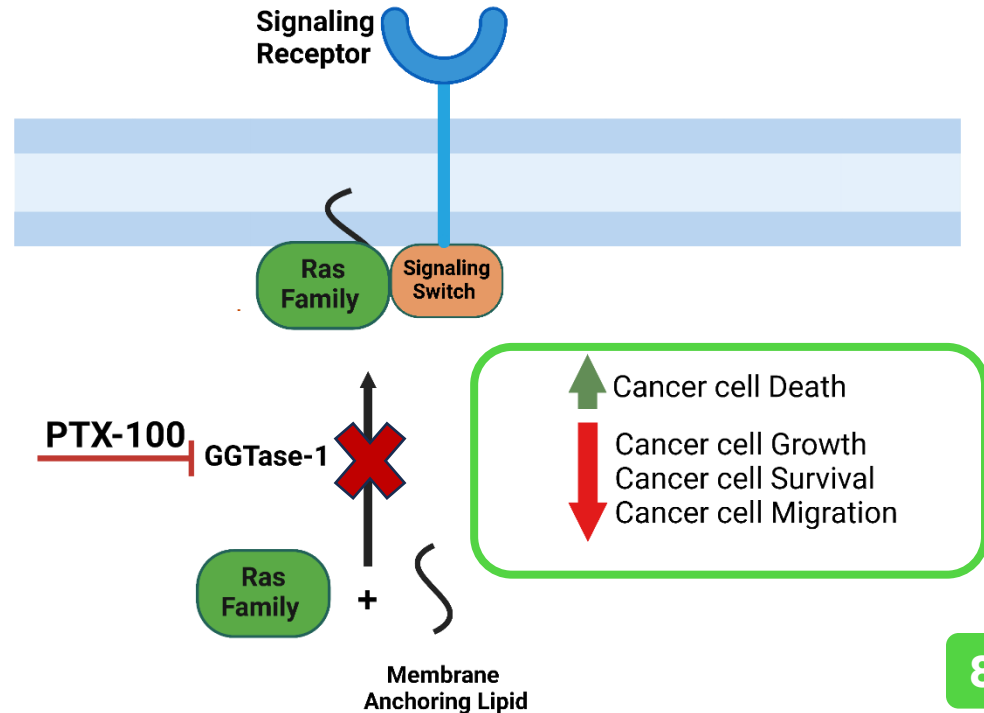


## Portfolio Overview

# PTX-100 First in Class Targeted Therapy

Inhibition of GGT-1 disrupts small GTPases including:  
**the RAS family pathway**

- Mutations in *RAS* are estimated to be responsible for approximately 22% of all human cancers<sup>1</sup>
- PTX-100 **targets and blocks** an enzyme called GGTase-1, **disrupting** the **RAS family pathways**
- This interferes with the way cancer cells grow and spread



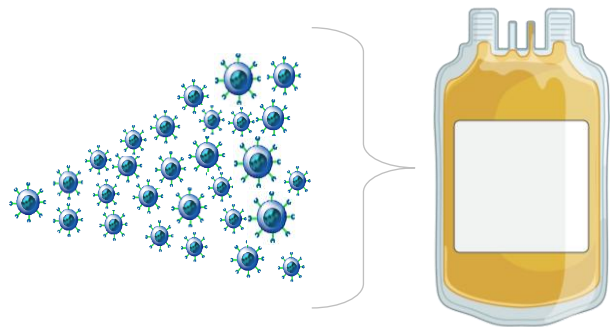
<sup>1</sup>. [The RAS Problem: Turning Off a Broken Switch - NCI](#)



# CellPryme: enhancing cell therapies in two ways



**MANUFACTURING**



Seamless addition during manufacturing  
**Enhanced CAR-T phenotype**



**ADJUVANT**

**Synergy Effect**



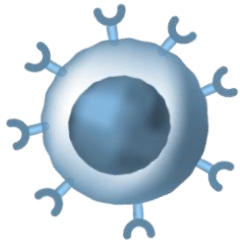
**Enhanced Effectiveness**



Safely administered to patient  
**Enhanced CAR-T therapy**

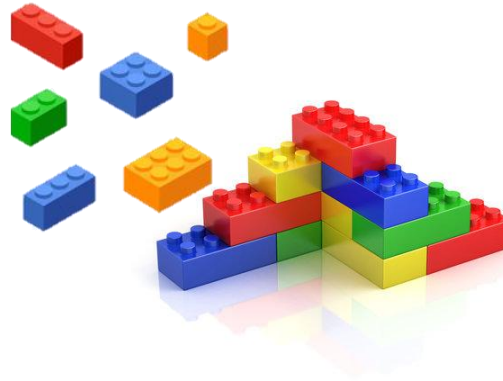
# OmniCAR: modular “plug & play” cells

## Traditional CAR-T products

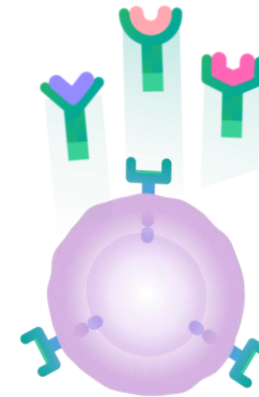


- Permanent target
- Single target
- Fixed

## “Plug & play” offers:

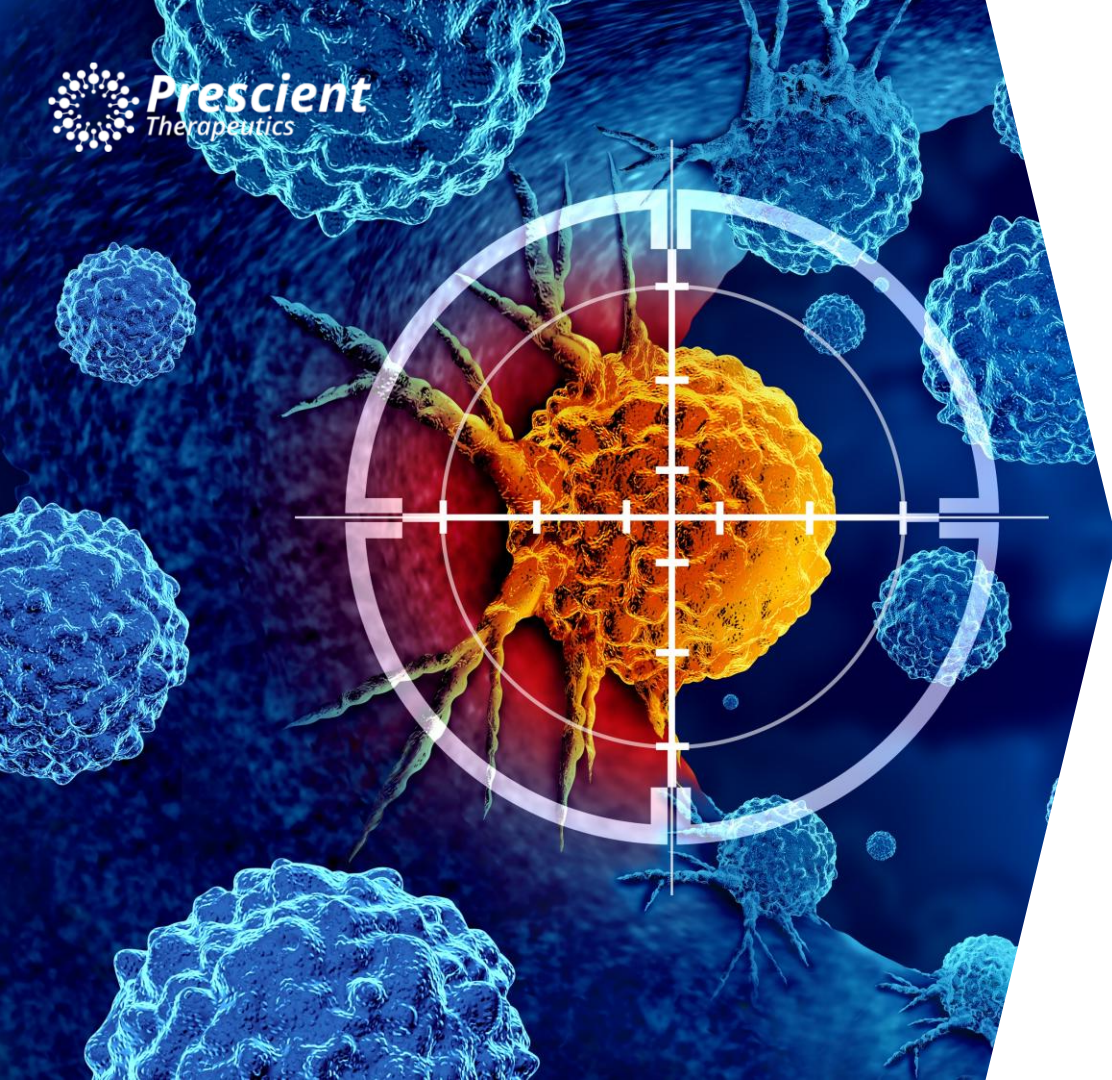
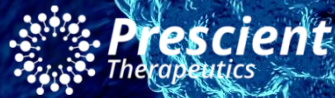


- Increased flexibility
- Multiantigen target
- Better control over cell function



- Modular/Adaptable
- Multi-target
- Tuneable





# PTX-100

## 1ST IN CLASS TARGETED THERAPY



Yale University



# Cutaneous T-cell Lymphoma (CTCL) Overview

- A rare type of cancer of white blood cells (T cells), normally involved in immune function
- These cancerous T cells travel to and live in the skin, where they grow and divide uncontrollably, attacking the skin
- CTCLs include subtypes, most commonly Mycosis Fungoides and Sezary Syndrome
- Can be indolent or aggressive, and range from rash-like patches through to plaques and tumours
- Limited options for patients with relapsed or refractory CTCL
- Orphan disease: 3,000<sup>#</sup> new cases in US each year and increasing
- Market projected to grow to US\$600M in the US by 2032





# Cutaneous T-cell Lymphomas (CTCL) a serious unmet need



**Professor Miles Prince**

*"Unfortunately, T-Cell lymphomas (...) is universally incurable in patients that have not responded to initial therapy. So, we are in desperate need of a treatment that will allow patients to respond and give them prolonged remissions."*

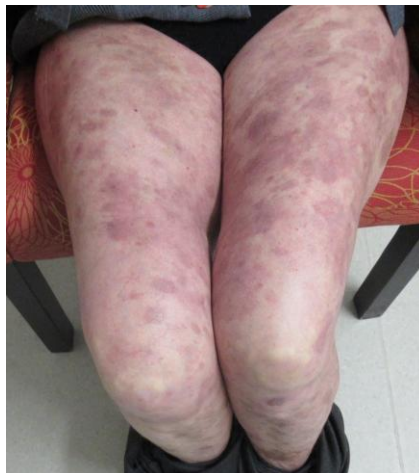


## Before



***“We are seeing responses  
in our patients who weren’t  
responding to any other  
treatments”***

Professor H. Miles Prince  
Principal Investigator



## After



# PTX-100 Phase 1b responses:

## Strong response rates in evaluable patients

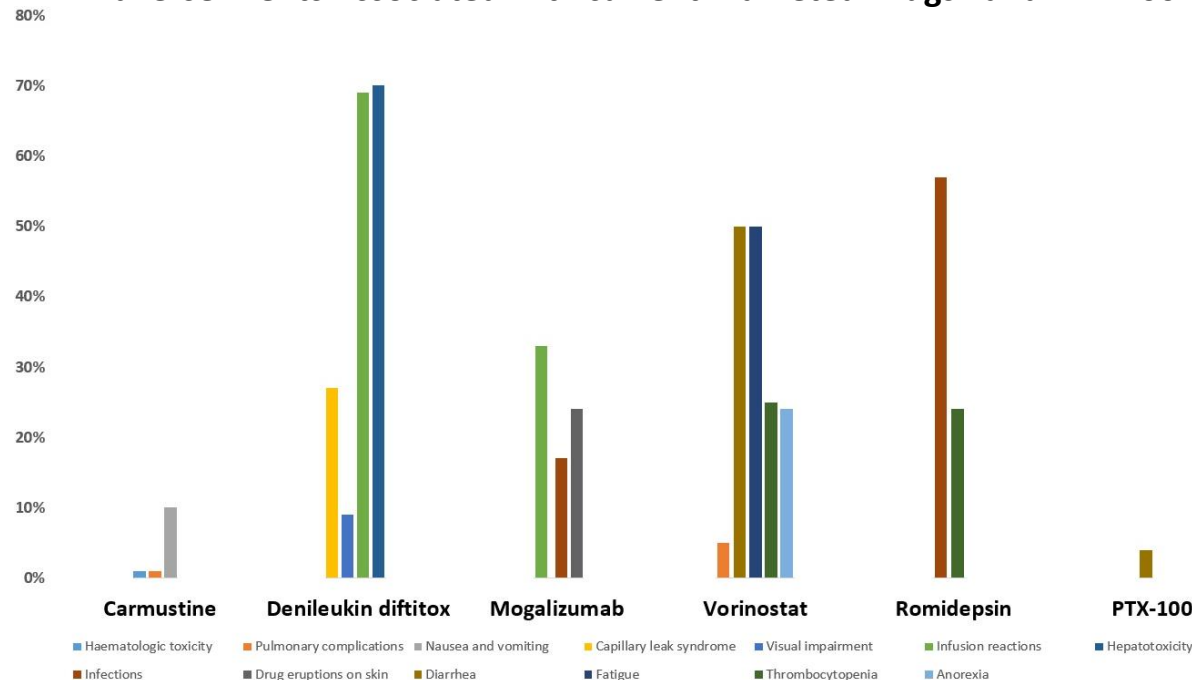
	Benchmark <sup>1</sup>	Lymphir <sup>2,3</sup>	PTX-100 (Phase 1B)
Response Rate	30%	36%	45%
Clinical Benefit Rate	45%	NA	64%
Duration of Response	9-13 months CTCL 3-4 Months PTCL	6.5 months (CTCL)	10.7 months
Serious Adverse Events <sup>4</sup>	>30%	36%	4%

1. Considered a target benchmark by Prescient and its investigators, with reference to currently available therapies in r/r TCL
2. Label as per FDA.gov; Fierce Pharma; EF Hutton report
3. Approved by the FDA 8 Aug 2024
4. Assessed as related to drug

# PTX-100: Favorable safety profile compared to peers

**Recommended CTCL drugs**, as outlined in international cancer treatment guidelines, have challenging safety profiles, **with adverse events occurring in up to 70% of patients**

## Adverse Events Associated with current Marketed Drugs\* and PTX-100



### PTX-100 HAS A FAVOURABLE SAFETY PROFILE

- Minimal Serious Adverse Events related to PTX-100
- Suits fragile patient population
- Good candidate for combination therapy

\*Other serious but less common events include Progressive multifocal leukoencephalopathy leading to death, Pancreatitis and Tumour Lysis syndrome. **Brentuximab vedotin** can cause rare but fatal progressive multifocal leukoencephalopathy, and more often pneumonitis, pancreatitis, opportunistic infections, infusion reactions and tumor lysis syndrome.



# Rationale of prioritising r/r CTCL for Ph2 trial

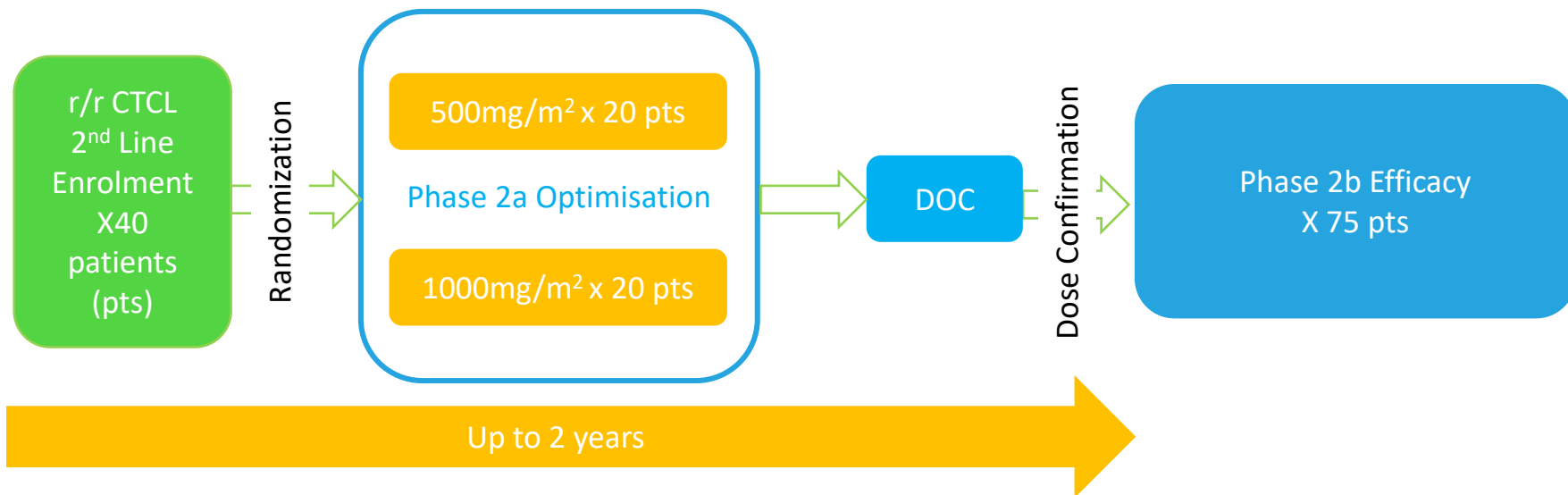
## CTCL

- **Higher confidence** of PTX-100 in CTCL (more data; more responders)
- **Greater need** for new therapies
- Likely to **recruit faster** than PTCL because of lack of trial competition
- **Larger patient pool** because of high prevalence/longer patient life expectancy
- Likely **smaller, faster, cheaper trial design**

## PTCL

- Peripheral T Cell Lymphoma (PTCL) is more prevalent than CTCL, but even though PTCL is still an unmet need, it has more existing and emerging competition
- PTCL more likely to require larger, more expensive studies that may require a comparator arm
- Further studies will be conducted under investigator led programs

# Progressing PTX-100 to Phase 2



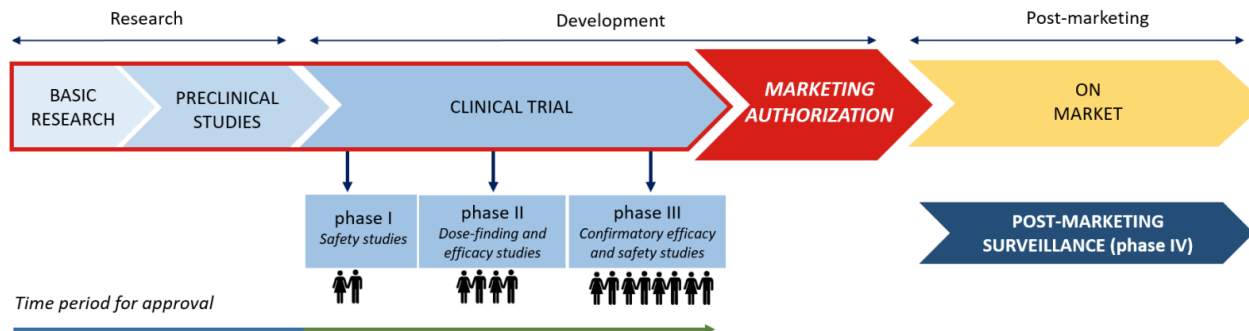
## Multicenter clinical trial

Australia (3)    USA (6)  
France (3)      Italy (3)

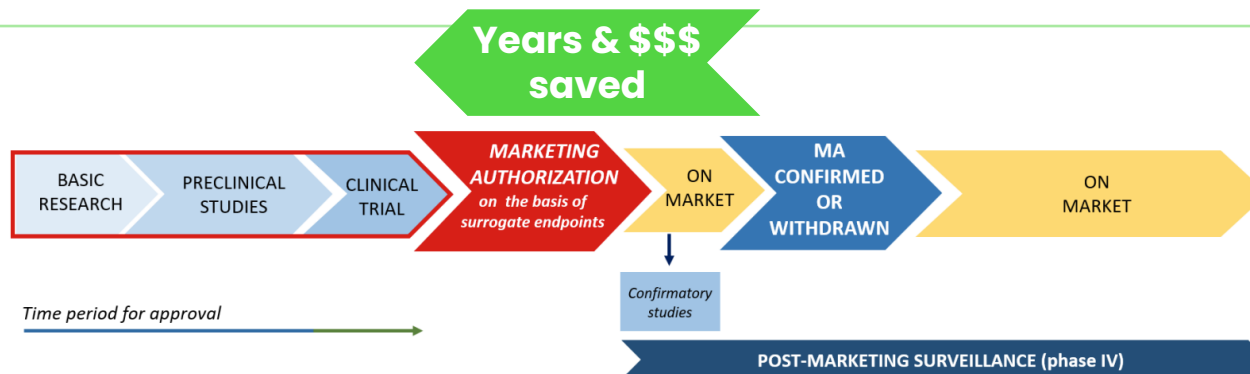
- **Phase 2a:** N=40 pts with r/r CTCL (dose optimization)
- **Phase 2b:** N=75 pts with r/r CTCL will be treated at the recommended dose from Phase 2a
- **Involving international experts in CTCL treatment**

# Aiming for shortened registrational pathway

## Regular drug development process

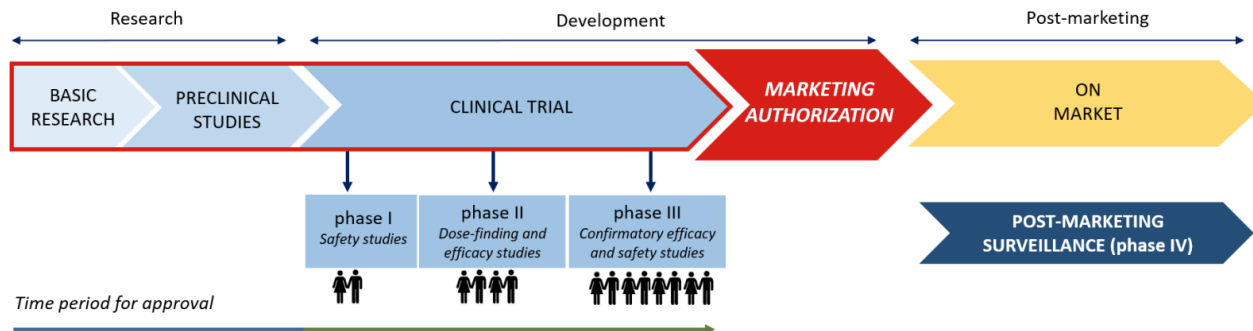


## Potential for Registrational study

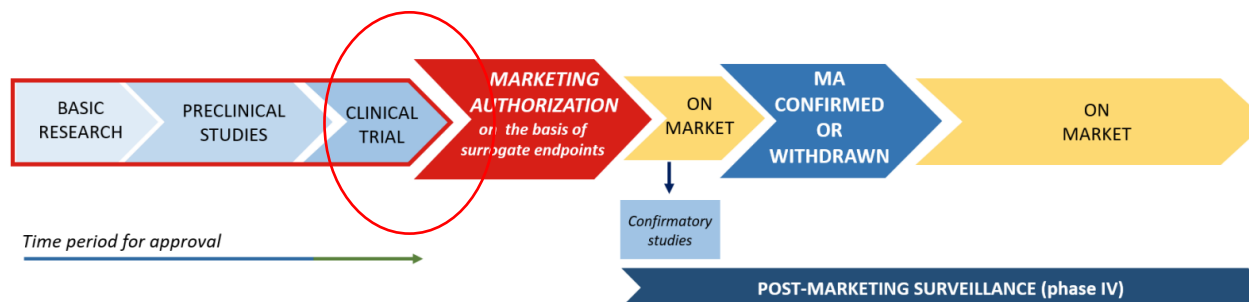


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# Advantages of Orphan Drugs



7 years of **guaranteed market exclusivity** in US  
(10 years in Europe)



**Higher prices**



Sales are **more resilient**  
to cycles

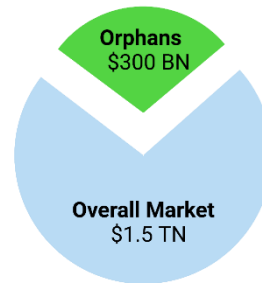
Non-orphan  
7% CAGR



Orphan  
11.6% CAGR



**Consistently higher sales growth**  
than non-orphan drugs



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

# T-cell lymphomas (TCL):

## High unmet need = Large market opportunity

### Total Addressable Market (TAM)

- 27,263 new cases / year in the 8 major markets
- Almost all will relapse
- Potential of **\$1.8B / year by 2030** (67% in the US)

### CTCL US alone

- Incidence 3,000 patients /annum<sup>#</sup>
- Almost all will relapse
- Combination therapy likely development
- Potential of **\$600M / year in 2032**

# Key Milestones in the near future: Implementation will drive value

Key Milestones	Expected Timing (CY)
First patient in and dosed with PTX-100 (FPID)	April/May
Potential FDA Fast Track designation	Q2
First US site activated and recruiting	Q2/3
First European site activated and recruiting	Q2/3
Continuous review of data during the Phase 2a	Q4 +
Validation of the new OmniCAR receptor and targets for AML	End Q4
Potential channel partner for CellPryme-M	Discussions ongoing

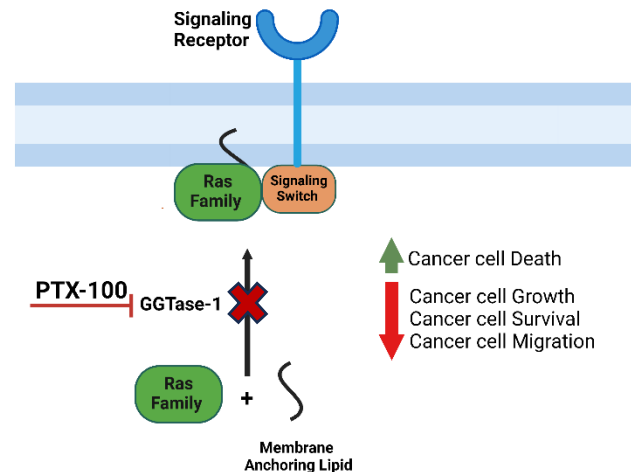


# First in Class PTX-100 beyond TCL

- First in class enzyme inhibitor disrupting the RAS super - family pathway, in particular, RHO, RAC and RAL
- 22% of all cancers have RAS involvement

## Examples:

- The RAS super family of genes consists of RAL, RAC, RHO-A/B, plus N-RAS and K-RAS. There are up to 153 proteins. Some examples of cancer types involving mutations of members of the RAS super family are listed below:
  - RAL mutations: Lung, bladder, prostate, hepatocellular, ovarian, pancreatic cancers
  - RHO-A mutations: Burkitt's lymphoma, gastric and breast cancers, PTCL
  - RAC mutations: Breast and prostate cancer, germ cell tumours including testicular cancer



# Summing up PTX-100:

## Driving a major inflection point

### Results:

#### Phase 1b

- 64% Clinical Benefit
- 10.7 months Duration of Response
- 4%  $\geq$  Grade 3 SAE
- Confidence to move to Phase 2a

### Timelines:

- Phase 2a is starting now
- Multiple sites globally
- International experts involved
- Recruitment will drive timing

### Regulatory Pathway/milestones:

- Orphan Designation
- IND acceptance
- Potential Fast Track designation
- TCL aligns with FDA interest in sponsors developing treatments for unmet medical needs
- Registrational potential

### Market Size:

- TCL market estimated US\$1.8B in 8 major markets in 2030
- CTCL market in US alone estimated at US\$600M in 2032

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**Prescient**  
Therapeutics

ASX: **PTX**

**THANK  
YOU**