PITT STREET RESEARCH

Prescient Therapeutics

Phase 2 coming soon

Share Price: $0.044

ASX: PTX

Sector: Healthcare

4 September 2024

Prescient Therapeutics (ASX:PTX) is an ASX-listed oncology drug developer with a big FY25 ahead of it. The company's flagship asset is PTX-100, which is targeting T-Cell Lymphomas. It works by blocking the GGTase I enzyme and has Orphan Drug Designation.

PTX-100 is potentially one trial away from FDA approval

There's no shortage of oncology developers on the ASX, but while most are still at pre-clinical or Phase 1, PTX is about to commence Phase 2 with PTX-100, which will happen by the end of 2024. There is potential for this product to gain FDA approval after Phase 2 without a Phase 3 trial given the current lack of treatment options for T-Cell Lymphomas. In our view, shareholders can be very confident in the drug's success given its Phase 1 results, as well as for potential interest from commercial partners, if market precedent is anything to go by.

Not a one trick pony

PTX is not hinging all its bets on PTX-100. The company is also advancing its CellPryme and OmniCAR platforms. They are not CAR-T therapies in their own right, but (for reasons which we will come to in this report) can improve CAR-T efficacy when used in combination with them, in other words aid CAR-T therapies to be more effective in attacking tumours. CAR-T has emerged in recent years to become an important part of cancer therapy, but has some shortcomings including efficacy and lack of control post-administration that Prescient's platforms can help overcome.

Valuation range of 11.6-16.4c per share

We reiterate our valuation PTX as outlined in last November's initiation report, at 11.6c per share base case and 16.4c per share in an optimistic (or bull) case using a Sum of the Parts/DCF methodology.

The key catalysts for creation of shareholder value in the near-term will be the initiation of Phase 2 trial for PTX-100. We expect the ending of the Bear Market for Life Sciences, with interest rates peaking and set to decline over the next 12-18 months, to also be a crucial factor in this company's valuation. Please see p.7 for an outline of our valuation rationale and p.8 for the key risks.

Market Cap. (A$ m)	35.4
# shares outstanding (m)	805.3
# shares fully diluted (m)	837.2
Market Cap Ful. Dil. (A$ m)	36.8
Free Float	100%
52-week high/low (C$)	0.105 / 0.038
Avg. 12M daily volume ('1000)	853.9
Website	https://ptxtherapeutics.com

Source: Company, Pitt Street Research

Share price (A$) and avg. daily volume (k, r.h.s.)

Source: Refinitiv Eikon, Pitt Street Research

Valuation metrics	
DCF fair valuation range (A$c)	11.6-16.4
WACC	16.2%
Assumed terminal growth rate	None

Source: Pitt Street Research

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The investment case for Prescient Therapeutics

Prescient Therapeutics is a biotech company focusing on cancers with unmet needs technologies.

1. **Prescient Therapeutics (ASX: PTX) is one of the most advanced oncology biotechs on the ASX**, on the cusp of a Phase 2 trial for its PTX-100 drug that (if successful) could see the drug approved.
2. **PTX-100 targets T-Cell Lymphoma, a disease where there is an urgent need for new treatment options.** While only around 5,000-6,000 people a year will be diagnosed with a T-Cell Lymphoma in the US, it is fatal. Generally overall survival in relapsed or refractory disease is less than a year in the absence of an allogeneic hematopoietic stem cell transplantation, which can be expensive[1].
3. **The market opportunity could be lucrative** even though there is a small number of patients. In our model for PTX, using US$450,000 per patient (with Acrotech Biopharma's Folotyn as a guide) and assuming a 50% penetration of a market that is 5,500 patients generates a sales opportunity of US$2.5bn.
4. **There is an easier path to market.** As an Orphan Drug, there will be at least seven years of market exclusivity post-approval. And although ordinarily, a company would need to conduct Phase 2 and Phase 3, the agency has discretion to give a drug approval post-Phase 2 if the experimental therapy is particularly strong compared to the standard of care. Given the lack of treatment options in relapsed and refractory T-Cell Lymphoma, Prescient believes post Phase 2 approval is a serious possibility for PTX-100.
5. **Existing clinical data can provide confidence of success.** This is not just because there is such encouraging data, but also because it has substantially beaten existing standards of care, and worked where previous lines of treatment have failed.
6. **There is precedent for Orphan Drug Developers that commercialise their drugs to trade at substantial valuation.** Consider Amgen's buyout of Horizon Therapeutics in October 2023. Amgen bought Horizon for Tepezza, which is the only approved treatment for thyroid eye disease. Horizon earned $1.66bn in sales for Tepezza in 2021, in its first full year on the market, and US$1.97bn in 2022. Amgen paid a massive US$27.8bn for this new franchise. Obviously, this might be too high to consider for an ASX listed biotech, although it is unlikely it would continue to be capped at just A$40m.
7. **CellPryme and OmniCar offer further potential for upside.** These are platforms that facilitate CAR-T therapy. CAR-T has substantially taken off in the last decade because of its success against certain cancers, although it has some limitations that CellPryme and OmniCAR would help overcome. The company is preparing to put CellPryme into clinical trials, while OmniCAR is still at the pre-clinical development stage.

[1] Cancers (Basel). 2023 Feb; 15(3): 589. Published online 2023 Jan 18.

Prescient's progress with PTX-100

PTX has been working on PTX-100 for a decade, having picked it up in 2014, following 5 years of research work conducted at American universities, including a small Phase I with 13 patients. Given work on since discontinued assets (particularly PTX-200), it took until 2019 for the company to start a Phase 1 study of its own. The study, which was initially a dose escalation study in a variety of tumour types, reported in July 2021 with an excellent safety profile and a clinical signal seen in T-Cell Lymphoma. Ever since, PTX-100 has been focused on T-Cell Lymphomas.

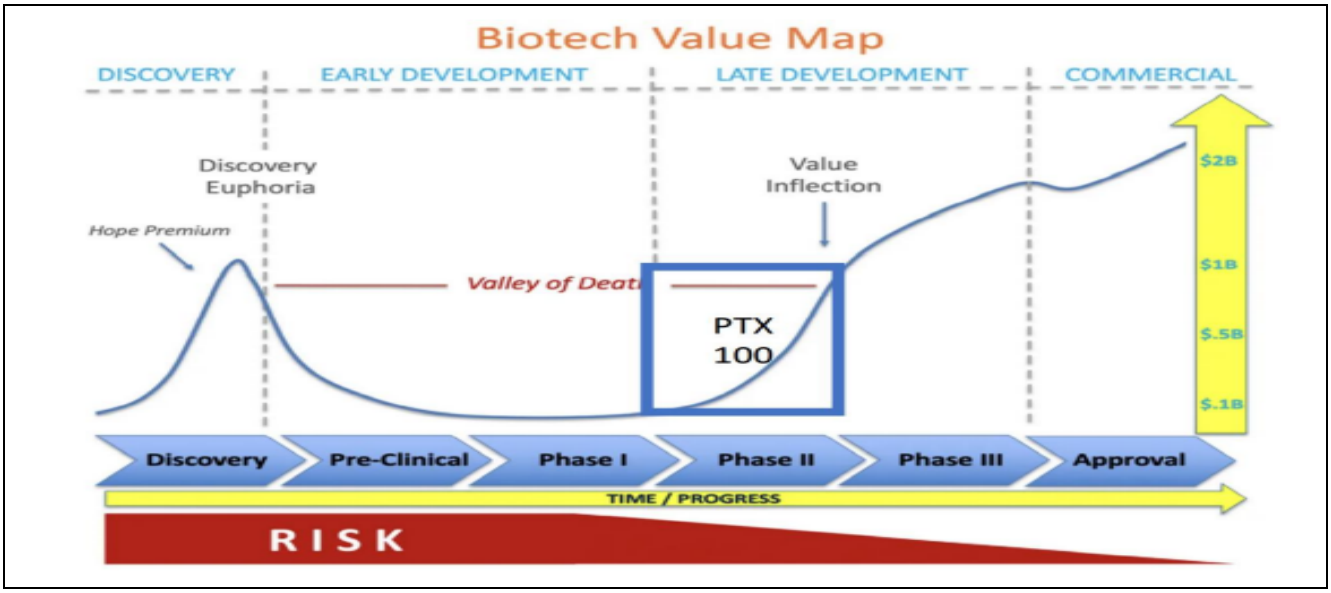
Phase 1b results with PTX-100 were strongly encouraging

Since our initiation report last November, the company has made even further favourable progress with their Phase 1b trial and the results were strongly encouraging. These endpoints were successfully met – there were no drug-related SAEs (Serious Adverse Events). Early-stage efficacy results were also measured and were very encouraging. Eleven of the 25 patients were eligible for assessment. Five patients saw clinical responses, three of which experienced complete eradication of the disease, representing an overall response rate (ORR) of 45%. A further two patients saw Stable Disease (SD) for over 6 months, thus representing a Clinical Benefit Rate (CBR) of 64%. This is very promising for a disease as deadly as T-Cell Lymphoma – especially in the relapsed and refractory setting.

Phase 2 is next

The next step for PTX-100 is a Phase 2 trial, and the company aspires to open one during the current quarter (the September quarter of CY24). PTX is discussing with the FDA potential for such a study to be an Accelerated Approval Study. This would mean, subject to successful data, PTX-100 could be submitted for FDA approval if and when it passed this study without the need for a Phase 3 study. Merely entering Phase 2 is a major step where there is most potential for shareholder value creation, as the first step where efficacy of the drug is the primary focus (Figure 1).

Figure 1: PTX's progress



Source: Company

The company has also been presenting its data to TCL experts, particularly at the 5th World Congress of Cutaneous Lymphomas (WCCL) and 15th Annual T-Cell Lymphoma Forum, both in, California. The company reported the data was received with significant interests from lymphoma specialists due to its safety profile and efficacy in aggressive cases of TCL. While conference presentations may seem to be a 'time filling' exercise to some investors, this may be important for the company. As we saw with Cyclopharm (ASX:CYC) last year, significant awareness of a drug or device prior to its approval in the US medical community, can significantly aid the company's cause for regulatory approval – there were dozens key opinion leaders in CYC's field that essentially lobbied the FDA for approval given the efficacy of CYC's device Technegas and the need for solutions like it. We wouldn't be surprised to see interest in the US medical community aid PTX similarly.

PTX-100 could potentially be commercialised in as little as 3 years from now.

Assuming success in all regards (FDA approval to the Phase 2 trial to be pivotal, successful Phase 2 results and FDA approval for commercialisation), commercialisation could occur in as little as 3-4 years from now, if Phase 2 initiation happened by the end of this calendar year and that it takes another 2 years to complete the study and read out the data. PTX is also expanding the current trial to create a more robust regulatory package.

Even if a conventional Phase 3 is required, PTX is still compelling

It is plausible that the company may have to undertake a subsequent Phase 3 trial, as per conventional drug development paths. Phase 3 trials are more comprehensive, time-consuming and expensive. Nonetheless, PTX should still be an attractive company for investors of all kinds because there are few other (if any) oncology stocks with a drug in Phase 2 and with such encouraging Phase 1 and pre-clinical results.

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Developers of orphan drugs are able to obtain substantial benefits that non-orphan drug developers get including tax credits, long-term periods of market exclusivity and even waiving of certain fees. Orphan Drugs can also fetch premium prices (several hundreds of thousands of dollars for example), and this is often reflected in the valuation of companies. One example Horizon Therapeutics – it has Tepezza, the only approved treatment for thyroid eye disease, and was bought by Amgen's in October 2023 for a staggering US$27.8bn. This followed over $3.5bn in sales in the first two full years on the market (2021 and 2022). A figure of US$27.8bn might be too high for PTX at this stage.

Nonetheless, it is not unreasonable to imagine there could be M&A or partnering interest for this drug – the latter could occur even prior to commercialisation. Consider that Neuren Pharmaceuticals (ASX:NEU) tripled in value to ~$2.6bn over CY23 after a partnering deal and commercialisation for its Rett-syndrome treatment during that year. This occurred following its FDA approval, although Dimerix (ASX:DXB) depicts that it is not impossible for deals to be struck prior to approval and even during Phase 3. Such a deal would not only facilitate royalties on sales, but likely provide for payment of certain R&D and commercialisation costs as well as potential upfront payments that could help fund development of OmniCar and CellPryme. Neuren's initial payment, received in July last year, was US$100m.

CellPryme and OmniCAR

PTX is also progressing its work with CellPryme and OmniCAR, both platforms to assist with CAR-T (Chimeric Antigen Receptor T-Cell) therapy. CAR-T therapy is a therapy that utilises the body's own T-cells, which are isolated from the patient's peripheral blood, endowed with enhanced specificity and killing efficacy towards the patient's cancer cells, and then reinjected into the host, where they aid in tumour clearance. The treatments on the market today show very encouraging remission rates – of over 80%[2]. Many CAR-T therapies, however, remain inaccessible to many due to high costs and have some challenges holding them back, particularly efficacy, durability of CAR-T cells, multi-targeting and post-infusion control. This is where CellPryme and OmniCAR come in.

CellPryme is a cell therapy enhancement platform, with two applications (CellPryme-M and CellPryme-A) that can be used separately or concurrently with significant power together and in their own right. CellPryme-M, through the introduction of molecules during a cell manufacturing process, allows better kinds of cells to be produced, which improves cellular therapies like CAR-T. CellPryme-A is an intravenous drug administered alongside cellular immunotherapy and allows the tumour microenvironment to be overcome.

OmniCAR, in simple terms, operates as an immune receptor platform and a molecular binding system. Binders recognising cancer cells are administered intravenously after the administration of CAR-T cells and activates these cells resulting in on-demand tumour killing.

Readers interested in further details on how exactly these platforms work and the market opportunity should see our initiation report from November 2023.

Current developments with CellPryme and OmniCAR

PTX is nearing completion of pre-clinical development of CellPryme and is preparing regulatory packages for both CellPryme-M and CellPryme-A so that these may enter the clinic in due course. The advantage of these platforms is that they can integrate easily into partner programs without highly disruptive changes to manufacturing processes or protocols mid-stream.

PTX is progressing platform optimisation of OmniCAR to investigate unarmed T-cell activity and improving control features. As a unique and multi-modal platform, this program is involving domain experts across protein and cell engineering and other areas.

[2] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC10094630/

Our valuation of PTX

We value PTX at 11.6c per share in our base case and 16.4c per share in our bull case.

We reiterate our valuation of PTX from our initiation report. Using a Sum of the Parts rationale (with 2 risk-adjusted DCFs for PTX-100 and CellPryme), we have valued PTX at 11.6c per share in our base case and 16.4c per share in our bull case (Figure 2). Our total valuation of PTX is the sum of the NPV of PTX-100 and CellPryme as well as the company's net cash position (worth 0.7c per share). Our assumptions are summarised in Figure 3.

Figure 2: Our valuation of PTX

Sum of the Parts Valuation	Base Case		Bull case	
Drugs	**A$m**	**A$ps**	**A$m**	**A$ps**
PTX-100	60.52	0.075	85.78	0.107
CellPryme	27.06	0.034	39.91	0.050
rNPV	**87.58**	**0.109**	**125.69**	**0.157**
Cash (close of FY23)	5.90	0.007	5.90	0.007
Debt (close of FY23)	-	-	-	-
Equity Value	**93.48**	**0.116**	**131.59**	**0.164**
Current Price		0.044		0.044
Upside		164%		272%

Estimates: Pitt Street Research

Figure 3: Our key DCF assumptions

DCF Assumptions (Base case)	PTX-100	CellPryme
Launch	CY28	CY31
Estimate market size (patient numbers)	5,500	242,100
Growth	2%	1%
Potential market penetration	50%	1%
Realised price (US$)	450,000	450,000
Peak sales (US$m)	2,595	3,528
Peak royalty revenue (US$m)	519	706
Gross milestone revenue (US$m)	75	40
Commercial exclusivity period (years)	10	7
Drug development cost (US$m)	40	58
Partner's share of costs	50.0%	50.0%
Discount rate	16.2%	16.2%
Royalty rate	20.0%	20.0%
Tax rate	21.0%	21.0%
Probability of success	30.00%	15.00%
Risk-adjusted NPV (A$m) - base case	60.52	27.06
rNPV per share (A$) - base case	0.075	0.034

Estimates: Pitt Street Research

Prescient Therapeutics has a market capitalisation on the ASX of less than $50m, which is only US$32m. Whatever currency you use, we think the current market capitalisation of Prescient does not begin to take account of the way in which clinical success with PTX-100 in T-Cell Lymphoma can yield a marketed drug in only around three years, where that drug's market opportunity is at least in the hundreds of millions.

Key risks facing PTX

Risks specific to PTX. We see the following major risks for PTX:

- **Timing risk.** There is the risk that the company's products may take longer than expected to move through the clinic.
- **Technical risk.** Some of the technologies that PTX is working with are relatively new and therefore may not therefore be 'bug-free'.
- **Regulatory risk**. There is the risk that regulators may decline to approve PTX products, even if PTX considers the data submitted to be adequate.
- **Commercial risk**. There is the risk that PTX may fail to find commercial partners for its products.
- **Uptake risk**. There is the risk that PTX products are still too expensive in the healthcare markets in which it wants to participate.
- **Funding risk**. There is the risk of future capital raisings proving dilutive to existing shareholders.
- **Key personnel risk**. There is the risk that the company may lose key personnel and be unable to replace them and/or their contribution to the business.

Risks related to pre-revenue Life Science companies in general.

The stocks of biotechnology and medical device companies without revenue streams from product sales or ongoing service revenue should always be regarded as speculative in character.

Since most biotechnology and medical device companies listed on stocks exchanges in Australia and around the world fit this description, the 'term' speculative can reasonably be applied to the entire sector.

The fact that the intellectual property base of most biotechnology and medical device lies in science not generally regarded as accessible to the layman adds further to the riskiness with which the sector ought to be regarded.

Caveat emptor. Investors are advised to be cognisant of the abovementioned specific and general risks before buying any the stock of any biotechnology and medical device stock mentioned in this report, including PTX.

Appendix I – Glossary

Acute Myeloid Leukemia (AML) – A blood cancer characterised by proliferation and accumulation of myeloid blasts in the bone marrow that are blocked at various stages of differentiation. The disease is called acute because patients develop abnormal numbers of these cells very quickly.

Adoptive T-cell therapy – Cancer treatment in which a patient's ownT-cells are engineered to increase their cancer-fighting properties, and then returned to the patient.

Blockbuster – A pharmaceutical drug with more than US$1bn in annual sales.

CellPryme – Prescient's platform technology for creating better quality cellular medicines.

Chimeric antigen receptor T-cells (CAR-T cells) – Chimeric antigen receptor T-cells (also known as CAR-T cells) are T-cells that have been genetically engineered to produce an artificial T-cell receptor for use in immunotherapy.

Complete Response – Elimination of a tumour brought about by a cancer drug.

Clinical Benefit Rate (CBR) - the percentage of advanced cancer patients who achieve complete response

Lymphoma – A cancer of the lymphocytes which the immune system needs to create T and B cells as well as Natural Killer cells. There are two main types of lymphoma, Hodgkin, and Non-Hodgkin, with Hodgkin Lymphoma being characterised by a particular cell type.

OmniCAR – Prescient's platform technology for creating modular cell therapies.

Orphan Drug – A drug that targets a disease affecting less than 200,000 potential patients in the US. Orphan drug designation provides tax benefits as well as market exclusivity in both Europe and the US.

Overall Response Rate (ORR) - The proportion of patients who have a partial or complete response to therapy[3].

Partial Response – A partial reduction in tumour size brought about by a cancer drug.

Pathway – A succession of signals between molecules within a cell to carry out the growth and functions of the cell. Well-known pathways include, but are not limited to MYC, PI3K/AKT, WNT and NOTCH.

pharmacokinetics (PK) and pharmacodynamics (PD)

Progression-Free Survival (PFS) – The length of time a cancer patient undergoing treatment can see no worsening of his or her cancer.

Serious Adverse Effects (SAEs) -

Stable Disease (SD) – Where the disease has not gotten worse, but has not gotten better either.

T Cells – White blood cells that are responsible for killing cells infected by viruses (in the case of 'Cytotoxic T-cells') and inducing B lymphocytes to produce antibodies (in the case of 'Helper T-cells').

T-Cell Lymphomas – A group of Non-Hodgkin Lymphomas impacting only T-cells.

[3] ORR does not include patients that have stable disease but otherwise no response to the therapy.

Appendix II – Analyst Qualifications

Stuart Roberts, lead analyst on this report, has been an equities analyst since 2002.

- Stuart obtained a Master of Applied Finance and Investment from the Securities Institute of Australia in 2002. Previously, from the Securities Institute of Australia, he obtained a Certificate of Financial Markets (1994) and a Graduate Diploma in Finance and Investment (1999).
- Stuart joined Southern Cross Equities as an equities analyst in April 2001. From February 2002 to July 2013, his research speciality at Southern Cross Equities and its acquirer, Bell Potter Securities, was Healthcare and Biotechnology. During this time, he covered a variety of established healthcare companies, such as CSL, Cochlear and Resmed, as well as numerous emerging companies. Stuart was a Healthcare and Biotechnology analyst at Baillieu Holst from October 2013 to January 2015.
- After 15 months over 2015–2016 doing Investor Relations for two ASX-listed cancer drug developers, Stuart founded NDF Research in May 2016 to provide issuer-sponsored equity research on ASX-listed Life Sciences companies.
- In July 2016, with Marc Kennis, Stuart co-founded Pitt Street Research Pty Ltd, which provides issuer-sponsored research on ASX-listed companies across the entire market, including Life Sciences companies.
- Since 2018, Stuart has led Pitt Street Research's Resources Sector franchise, spearheading research on both mining and energy companies.

Nick Sundich, lead analyst on this report, is an equities research analyst at Pitt Street Research.

- Nick obtained a Bachelor of Commerce/Bachelor of Arts from the University of Sydney in 2018. He has also completed the CFA Investment Foundations program.
- He joined Pitt Street Research in January 2022. Previously he worked for over three years as a financial journalist at Stockhead.
- While at university, he worked for a handful of corporate advisory firms.

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