

US FDA Grants Orphan Drug Designation to PTX-100 for broader TCL indications

Key points

- US FDA grants PTX-100 additional Orphan Drug Designation (ODD) for all T cell lymphomas
- ODD granted for a broader indication than requested by PTX
- Confers several developmental benefits and 7 years of market exclusivity
- Trial update due shortly

MELBOURNE Australia, 9 March 2023 – Prescient Therapeutics (“Prescient”; ASX: PTX), a clinical stage oncology company developing personalised therapies to treat cancer, is pleased to announce that the Office of Orphan Products Development at the US Food and Drug Administration (FDA) has granted Orphan Drug Designation for PTX-100 for the treatment of T-cell lymphomas (TCL), including cutaneous TCL (CTCL). Having separately received ODD for peripheral TCL (PTCL) in 2022, Prescient applied for ODD for CTCL. The FDA has now granted a broader designation than Prescient requested, which encompasses all TCLs.

TCLs describes a group of lymphomas that develop when a group white blood cells called lymphocytes grow out of control. There are different groups of TCL, including PTCL and CTCL, each with several distinct subtypes. Collectively, TCLs represent an area of unmet or poorly met patient need, especially in patients with relapsed or refractory disease. This ODD designation now covers all TCLs and their subtypes.

Relapsed and refractory TCLs are the focus of the current expansion cohort of the PTX-100 Phase 1b study under the leadership of globally-renowned lymphoma expert, Professor H. Miles Prince, AM. An update of the trial is due imminently.

The FDA’s Orphan Drug Designation program provides orphan status to drugs which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the US. It is designed to provide benefits to incentivize drug development in less common diseases.

For personal use only



The benefits of an Orphan Drug Designation are considerable and include guaranteed market exclusivity of seven years from granting of regulatory approval; and a waiver of Prescription Drug User Fee Act (PDUFA) fees for orphan drugs, which has a value of over US\$3.1 million in 2022¹.

Orphan Drug Designation will allow Prescient to benefit from incentives that can assist the development of PTX-100, a first-in-class prenylation inhibitor that disrupts oncogenic Ras pathways in cancer cells.

Prescient Managing Director and CEO Steven Yatomi-Clarke said “Prescient is delighted to be granted this Orphan Drug Designation by the FDA, and is pleasantly surprised for the granting of the designation that is broader than our request. This now confers the certainty of 7 years of market exclusivity for PTX-100 in a broader range of diseases with unmet or poorly met clinical need. We look forward to sharing updates on the PTX-100 trial shortly.”

- Ends -

To stay updated with the latest company news and announcements, [please update your details](#) on our investor centre.

About Prescient Therapeutics Limited (Prescient)

Prescient Therapeutics is a clinical stage oncology company developing personalised medicine approaches to cancer, including targeted and cellular therapies.

Targeted Therapies

PTX-100 is a first in class compound with the ability to block an important cancer growth enzyme known as geranylgeranyl transferase-1 (GGT-1). It disrupts oncogenic Ras pathways by inhibiting the activation of Rho, Rac and Ral circuits in cancer cells, leading to apoptosis (death) of cancer cells. PTX- 100 is believed to be the only GGT-1 inhibitor in the world in clinical development. PTX-100 demonstrated safety and early clinical activity in a previous Phase 1 study and recent PK/PD basket study of hematological and solid malignancies. PTX-100 is now in a Phase 1b expansion cohort study in T cell lymphomas, where it has shown encouraging efficacy signals and safety.

PTX-200 is a novel PH domain inhibitor that inhibits an important tumour survival pathway known as Akt, which plays a key role in the development of many cancers, including breast and ovarian cancer, as well as leukemia. Unlike other drug candidates that target Akt inhibition, PTX-200 has a novel mechanism of action that specifically inhibits Akt without non-specific kinase inhibition effects. This highly promising compound is currently in a Phase 1b/2 trial in relapsed and refractory AML, where it has resulted in 4 complete remissions so far. PTX-200 previously generated encouraging Phase 2a data in HER2-negative breast cancer and Phase 1b in recurrent or persistent platinum resistant ovarian cancer.

¹ www.fda.gov; updated May 2022

Cell Therapies

OmniCAR: is a universal immune receptor platform enabling controllable T-cell activity and multi- antigen targeting with a single cell product. OmniCAR's modular CAR system decouples antigen recognition from the T-cell signalling domain. It is the first universal immune receptor allowing post- translational covalent loading of binders to T-cells. OmniCAR is based on technology licensed from Penn; the SpyTag/SpyCatcher binding system licensed from Oxford University; and other assets.

The targeting ligand can be administered separately to CAR-T cells, creating on-demand T-cell activity post infusion and enables the CAR-T to be directed to an array of different tumour antigens. OmniCAR provides a method for single-vector, single cell product targeting of multiple antigens simultaneous or sequentially, whilst allowing continual re-arming to generate, regulate and diversify a sustained T-cell response over time.

Prescient is developing OmniCAR programs for next-generation CAR-T therapies for Acute Myeloid Leukemia (AML); Her2+ solid tumours, including breast, ovarian and gastric cancers; and glioblastoma multiforme (GBM).

CellPryme-M: Prescient's novel, ready-for-the-clinic, CellPryme-M technology enhances adoptive cell therapy performance by shifting T and NK cells towards a central memory phenotype, improving persistence, and increasing the ability to find and penetrate tumours. CellPryme-M is a 24-hour, non-disruptive process during cell manufacturing. Cell therapies that could benefit from additional productivity in manufacturing or increased potency and durability in-vivo, would be good candidates for CellPryme-M.

CellPryme-A: CellPryme-A is an adjuvant therapy designed to be administered to patients alongside cellular immunotherapy to help them overcome a suppressive tumour microenvironment. CellPryme-A significantly decreases suppressive regulatory T cells; increases expansion of CAR-T cells in vivo; increases tumour penetration of CAR-T cells. CellPryme-A improves tumour killing and host survival of CAR-T cell therapies, and these benefits are even greater when used in conjunction with CellPryme-M pre-treated CAR-T cells.

Find out more at www.ptxtherapeutics.com or connect with us via Twitter [@PTX_AUS](https://twitter.com/PTX_AUS) and [LinkedIn](https://www.linkedin.com/company/ptxtherapeutics).

The Board of Prescient Therapeutics Limited has approved the release of this announcement.

Find out more at www.ptxtherapeutics.com or connect with us via Twitter [@PTX_AUS](https://twitter.com/PTX_AUS) and [LinkedIn](https://www.linkedin.com/company/ptxtherapeutics).

Steven Yatomi-Clarke
CEO & Managing Director
Prescient Therapeutics
steven@ptxtherapeutics.com

Investor enquiries:
Sophie Bradley – Reach Markets
+61 450 423 331
ir@reachmarkets.com.au

Media enquiries:
Andrew Geddes – CityPR
+61 2 9267 4511
ageddes@citypublicrelations.com.au

Disclaimer and Safe Harbor Statement

Certain statements made in this document are forward-looking statements within the meaning of the safe harbor provisions of the United States Private Securities Litigation Reform Act of 1995. These forward-looking statements are not historical facts but rather are based on the current expectations of Prescient Therapeutics Limited (“Prescient” or the “Company”), their estimates, assumptions, and projections about the industry in which Prescient operates. Material referred to in this document that use the words ‘estimate’, ‘project’, ‘intend’, ‘expect’, ‘plan’, ‘believe’, ‘guidance’, and similar expressions are intended to identify forward-looking statements and should be considered an at-risk statement. These forward-looking statements are not a guarantee of future performance and involve known and unknown risks and uncertainties, some of which are beyond the control of Prescient or which are difficult to predict, which could cause the actual results, performance, or achievements of Prescient to be materially different from those which may be expressed or implied by these statements. These statements are based on our management’s current expectations and are subject to a number of uncertainties and risks that could change the results described in the forward-looking statements. Risks and uncertainties include, but are not limited to, general industry conditions and competition, general economic factors, global pandemics and related disruptions, the impact of pharmaceutical industry development and health care legislation in the United States and internationally, and challenges inherent in new product development. In particular, there are substantial risks in drug development including risks that studies fail to achieve an acceptable level of safety and/or efficacy. Investors should be aware that there are no assurances that results will not differ from those projected and Prescient cautions shareholders and prospective shareholders not to place undue reliance on these forward- looking statements, which reflect the view of Prescient only as of the date of this announcement. Prescient is not under a duty to update any forward-looking statement as a result of new information, future events or otherwise, except as required by law or by any appropriate regulatory authority.

Certain statements contained in this document, including, without limitation, statements containing the words “believes,” “plans,” “expects,” “anticipates,” and words of similar import, constitute “forward- looking statements.” Such forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause the actual results, performance or achievements of Prescient to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. Such factors include, among others, the following: the risk that our clinical trials will be delayed and not completed on a timely basis; the risk that the results from the clinical trials are not as favourable as we anticipate; the risk that our clinical trials will be more costly than anticipated; and the risk that applicable regulatory authorities may ask for additional data, information or studies to be completed or provided prior to their approval of our products. Given these uncertainties, undue reliance should not be placed on such forward-looking statements. The Company disclaims any obligation to update any such factors or to publicly announce the results of any revisions to any of the forward-looking statements contained herein to reflect future events or developments except as required by law.

This document may not contain all the details and information necessary for you to make a decision or evaluation. Neither this document nor any of its contents may be used for any other purpose without the prior written consent of the Company.

Supplemental COVID-19 Risk Factors

Please see our website : [Supplemental COVID-19 Risk Factors](#)