US FDA Grants Orphan Drug Designation to PTX-100 for PTCL

Key points

- **US FDA grants PTX-100 Orphan Drug Designation**
- **Confers several developmental benefits and 7 years of market exclusivity**
- **PTCL is a blood cancer with high unmet clinical need**
- **Expansion cohort in TCL on track to fully recruit this year under the leadership of globally-renowned lymphoma expert, Professor H. Miles Prince, AM.**

MELBOURNE Australia, 15 July 2022 – 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 peripheral T-cell lymphomas (PTCL).

The 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.

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 2022[1].

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. PTX-100 showed an encouraging efficacy signal in PTCL in the dose escalation of a Phase 1b basket study, and is now in an expansion cohort of 12 patients with relapsed and refractory T cell lymphomas (TCL), including PTCL, under the leadership of globally-renowned lymphoma expert, Professor H. Miles Prince, AM. The expansion cohort is due to fully recruit this year.

PTCL is a disease of serious unmet need. Survival following relapse is poor and has not significantly improved in the last 20 years[2,3]. Whilst PTCL is not a common malignancy, the nature of disease and the paucity of effective treatment options for refractory patients creates a potentially shorter regulatory path for PTX-100 in this setting, and the fastest route to market in a high value area of unmet clinical need.

Currently available therapies for PTCL are typically characterised by high occurrence of serious toxicities; low response rates (<30%) and short duration of responses (3-4 months)[4]. So far, PTX-100 is exhibiting a favourable safety profile with encouraging efficacy signals, including one PTCL patient with a durable response that is still on therapy after 28 months.

[1] www.fda.gov; updated May 2022
[2] Mak V, *et al.*; *J Clin Oncol* 2013; 31:1970-6.
[3] Chihara D, *et al.*; *Br J Haematol* 2017; 176:750-8.
[4] Saleh *et al.*; *J Exp Pharmacol;* 2021:13 577–591

Prescient Managing Director and CEO Steven Yatomi-Clarke said "The granting of Orphan Drug Designation by the FDA is significant for Prescient's development of PTX-100. Orphan drugs often enjoy shorter and cheaper development pathways. Additionally, the Company now has the certainty of 7 years of market exclusivity in the event of regulatory approval of PTX-100 for PTCL.

PTCL is a disease of unmet need, where safer and more effective therapies will be welcomed by patients and clinicians. As our expansion cohort unfolds, ultimately it will be supporting clinical data and Orphan Drug Designation that will combine to help bring PTX-100 to patients with this challenging disease."

- Ends -

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About Prescient Therapeutics Limited (Prescient)

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

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.

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

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 and LinkedIn.

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Supplemental COVID-19 Risk Factors

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