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.

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.

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 -

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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.

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 and LinkedIn.

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

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Supplemental COVID-19 Risk Factors

Please see our website : Supplemental COVID-19 Risk Factors