



ASX Release

FDA Grants Orphan Drug Designation for PTX-200 in Acute Myeloid Leukemia

Melbourne, Australia (1 May 2017): Clinical-stage oncology company Prescient Therapeutics Ltd (ASX: PTX) 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-200 for the treatment of acute myeloid leukemia (AML).

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 7 years from granting of regulatory approval;
- Potential for accelerated review; and
- 50% tax credit on US trials.

Orphan Drug Designation will allow PTX to benefit from incentives that can assist the development of PTX's lead drug candidate PTX-200, which inhibits an important tumor survival pathway known as Akt which is aberrant in many blood cancers, including 72% of AML patients.

PTX's CEO and Managing Director, Steven Yatomi-Clarke said today "The granting of Orphan Drug Designation by the FDA is significant for PTX. 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-200 for AML.

AML is a key focus of our clinical program, with PTX-200 with an important Phase 1b/2 clinical trial currently underway at Moffitt Cancer Center under the leadership of Professor Jeffrey Lancet. Orphan Drug Designation is another positive step towards bringing new treatments to market in an area of urgent need."

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About Prescient Therapeutics Limited (PTX)

PTX is a clinical stage oncology company developing novel compounds that show promise as potential new therapies to treat a range of cancers that have become resistant to front line chemotherapy.

PTX's lead drug candidate PTX-200 inhibits an important tumor 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 which are non-specific kinase inhibitors that have toxicity problems, PTX-200 has a novel mechanism of action that specifically inhibits Akt whilst being comparatively safer. This highly promising compound is now the focus of three current clinical trials. The first is a Phase 1b/2 trial evaluating PTX-200 as a new therapy for relapse and refractory Acute



Myeloid Leukemia, being conducted at Florida's H. Lee Moffitt Cancer Center (Moffitt) and Yale Cancer Center in New Haven, Connecticut (Yale) under the leadership of Professor Jeffrey Lancet, MD.

PTX is also conducting a Phase 2 study examining PTX-200 in breast cancer patients at the prestigious Montefiore Cancer Center in New York and the Moffitt. The third trial is a Phase 1b/2 trial of PTX-200 in combination with current standard of care is also underway in patients with recurrent or persistent platinum resistant ovarian cancer at the Moffitt.

PTX's second novel drug candidate, PTX-100, is a first in class compound with the ability to block an important cancer growth enzyme known as geranylgeranyl transferase (GGT). It also blocks the Ral and Rho circuits in cancer cells which act as key oncogenic survival pathways, leading to apoptosis (death) of cancer cells. PTX-100 was well tolerated and achieved stable disease in a Phase I trial in advanced solid tumors.

Further enquiries:

Steven Yatomi-Clarke
CEO & Managing Director
Prescient Therapeutics Limited
+61 417 601 440

Kyahn Williamson
WE Buchan
kwilliamson@buchanwe.com.au
+61 401 018 828