ASX Announcement

**Prescient Therapeutics Announces First Patient Dosed in Phase 1b Clinical Trial for First-in-Class Cancer Therapy PTX-100**

**MELBOURNE Australia, 14 November 2019:** Prescient Therapeutics (ASX: PTX) ("Prescient"), a clinical stage oncology company, today announced treatment of the first patient with its second targeted anti-cancer drug, PTX-100, in a Phase 1b trial enrolling patients with multiple cancer types. The first patient dosed is a multiple myeloma patient, who successfully completed a week of therapy with PTX-100 without any notable drug-related side effects.

The study will seek to determine the safety, dose regimen and treatment schedule of PTX-100, a first in class drug, in several cancers where Ras and RhoA mutations are prevalent. These mutations are present in many cancer types, but there remains no approved therapy against either Ras or RhoA mutant cancers. As a result, there remains a significant yet unserved market opportunity for treatments addressing Ras and RhoA mutant cancers, which has drawn strong attention from industry, clinicians and researchers.

The study at Epworth HealthCare in Melbourne is led by Professor Miles H. Prince AM, an internationally renowned oncologist who has contributed to the successful development of several new breakthrough cancer therapies.

Prescient will seek to identify the mutational status of each patients' malignancies and, within the constraints of a small sample size, seek to correlate this status with any clinical activity. Several cancer biomarkers will be investigated with the aim of identifying patients that may be most likely to respond to PTX-100 therapy.

The new study takes a ‘basket’ approach to assess the drug on multiple cancers with a view to addressing specific mutations, rather than tumor origin. Basket studies pioneered by several US companies have quickly identified patient populations who could benefit from the investigational drug, sometimes leading to fast track approval.

Professor Prince said, "All of us working to treat cancer are excited to see the first patient dosed in Australia with this compound and the prospect of advancing a new molecularly targeted therapy for people who currently have few medical options."

Prescient’s Managing Director and CEO Steven Yatomi-Clarke said, "Dosing the first patient in this study is a pivotal milestone in our efforts to develop effective new treatments for a range of hard to treat cancers. We are very pleased with our productive collaboration with Professor Prince and his team at Epworth in advancing this important clinical program."
Earlier studies in the US at Pennsylvania State University and Indiana State University in patients with advanced solid tumors showed PTX-100 was well tolerated and achieved stable disease in patients.

PTX-100 is licensed by Prescient from Yale University, and was invented by Prescient Chief Scientific Officer, Professor Said Sebti, recently appointed as Associate Director of Basic Research at Virginia Commonwealth University Massey Cancer Center, and Professor Andrew Hamilton, the President of New York University.

**PTX-100 -- a targeted cancer therapy**

PTX-100 is a first-in-class drug candidate that works by disrupting several oncogenic Ras pathways by inhibiting the activation of Rho, Rac and Ral in cancer cells, leading to the death of cancer cells.

The open-label, non-randomized trial will enrol up to 24 participants to evaluate the pharmacokinetics and pharmacodynamics of PTX-100, as well as safety and efficacy of up to three different doses in patients with advanced malignancies. Patients will receive the drug by intravenous infusion over 60 minutes on days one to five of a 14-day cycle for four cycles unless toxicity is observed. The aim is to identify the optimal time and dose-dependent effect of multiple doses of PTX-100.

**Professor Miles H Prince AM**

Professor Prince is involved in major clinical research programs ranging from the use of stem cells to the mechanism of the immune systems control of blood and cancer growth. He holds major Australian, American and European research grants and has published over 400 journal articles. He is a member of Australian, American and European Societies of Haematology and Oncology, and is on the boards of International Society of cutaneous lymphoma, International Waldenstrom’s Macroglobulinemia Foundation and chairman of the Medical Scientific Advisory Group of Myeloma Australia. He is the Director of Molecular Oncology and Cancer Immunology at Epworth HealthCare.

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About Prescient Therapeutics Limited (Prescient)

Prescient Therapeutics is a clinical stage oncology company developing targeted therapies that address specific mutations that drive cancer and contribute to resistance.

**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 RhoA inhibitor in the world in clinical development. PTX-100 is currently in a PK/PD basket study of hematological and solid malignancies, focusing on cancers with Ras and RhoA mutations. In a previous Phase 1 trial in advanced solid tumors, PTX-100 was well tolerated and achieved stable disease.

**PTX-200** is a novel PH domain inhibitor that 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:

- Phase 2 study examining PTX-200 in breast cancer patients at the prestigious Montefiore Cancer Center in New York and Florida’s H. Lee Moffitt Cancer Center (Moffitt). PTX-200 showed encouraging efficacy signals in the Phase 1b study, with twice the expected response rate. Responses have demonstrated durability in the study so far.
- Phase 1b/2 trial evaluating PTX-200 as a new therapy for relapsed and refractory Acute Myeloid Leukemia, being conducted the Moffitt; Yale Cancer Center in New Haven, Connecticut (Yale) and Kansas University Medical Center (KUMC) under the leadership of Professor Jeffrey Lancet, MD.
- 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.

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