

ASX ANNOUNCEMENT

3 October 2019

Cynata Advances Toward Phase 2 Clinical Trial of Cymerus™ MSCs in Patients with Osteoarthritis

Melbourne, Australia; 3 October 2019: Cynata Therapeutics Limited (ASX: CYP), a clinical-stage biotechnology company specialising in cell therapeutics, is pleased to provide an update on start-up activities of a Phase 2 clinical trial to evaluate Cynata's Cymerus™ mesenchymal stem cells (MSCs) as a treatment for osteoarthritis, following the signing of an agreement with the University of Sydney.

Key Highlights

- Osteoarthritis is the third Phase 2 indication for Cynata**, highlighting demand for the company's Cymerus MSCs in multiple indications
- **448-patient Phase 2 clinical trial in osteoarthritis** expected to commence in Quarter 1 2020
- **World-class clinical team led by Professor David Hunter of the University of Sydney**
- Research Support Agreement signed to accelerate trial planning and start-up activities
- Trial design nearing completion and protocol development continues
- **Cynata retains full commercial rights to the use of Cymerus MSCs in osteoarthritis**
- One of the largest MSC trials to ever be conducted will **showcase Cynata's MSC manufacturing capability**

Significant progress has been made with the University of Sydney for the Phase 2 study to be undertaken using Cynata's Cymerus MSCs, and through a Research Support Agreement, Cynata's commercial rights have been confirmed. Planning and start-up activities are now advancing rapidly.

Dr Kilian Kelly, Cynata's Vice President, Product Development, said:

"We are very pleased that we are now able to explore the potential role of our Cymerus MSCs in improving the quality of life for patients living with the huge disease burden caused by osteoarthritis. Signing this Research Support Agreement with the University of Sydney confirms Cynata's rights to make this treatment commercially available, if successful, and enables us to finalize the trial design and move more quickly through the planning and start-up phase."

Clinical Trial Overview

The aim of the Phase 2 clinical trial is to assess the effect of Cymerus MSCs compared to placebo on clinical outcomes and knee joint structure over a two-year period, in 448 patients with osteoarthritis of the knee.

The trial will be led by Professor David Hunter, Florance and Cope Chair of Rheumatology, Chair of the Institute of Bone and Joint Research and Professor of Medicine at the University of Sydney, who has been a Chief Investigator of numerous clinical trials in osteoarthritis. He has more than 450 publications in high-impact journals, including the *New England Journal of Medicine*, *Journal of the American Medical Association* and *British Medical Journal*.

As announced on 13 December 2018, the conduct of the clinical will be led by the University of Sydney



and substantially funded by an Australian National Health and Medical Research Council (NHMRC) project grant, in addition to in-kind contributions from participating institutions and Cynata. Subject to obtaining relevant ethics and regulatory approvals, Cynata will manufacture and supply Cymerus MSCs for use in the trial.

Under the agreement, full commercial rights to the use of the Cymerus MSCs in osteoarthritis are retained by Cynata. In the event that results of this Phase 2 trial are used to support regulatory marketing approval of Cymerus MSCs for the treatment of osteoarthritis, Cynata will potentially pay fees to the University of Sydney totaling up to AU\$2.1 million, with the first payment (AUD \$100,000) due when the Phase 2 data are used in a submission for marketing approval to a regulatory authority.

Professor Hunter commented, *"We look forward to working with Cynata on this important clinical trial. If Cymerus MSCs demonstrate an improvement in symptoms and knee joint structure in this trial, it will reduce osteoarthritis disease burden and have a substantial impact on osteoarthritis management worldwide."*

About Osteoarthritis

Osteoarthritis is a chronic joint disease that causes pain and disability and affects around two million Australians and 30 million people in the USA. With no cure and an aging population, it is estimated that the incidence of osteoarthritis will increase by more than 50 percent in the next 15 years. Market research estimates that the resulting global osteoarthritis treatment market is expected to grow from a value of US\$8.0 billion in 2018 to US\$11.6 billion by 2025.¹

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About Cynata Therapeutics (ASX: CYP)

Cynata Therapeutics Limited (ASX: CYP) is an Australian clinical-stage stem cell and regenerative medicine company focused on the development of therapies based on Cymerus™, a proprietary therapeutic stem cell platform technology. Cymerus overcomes the challenges of other production methods by using induced pluripotent stem cells (iPSCs) and a precursor cell known as mesenchymoangioblast (MCA) to achieve economic manufacture of cell therapy products, including mesenchymal stem cells (MSCs), at commercial scale and without the limitation of multiple donors.

Cynata's lead product candidate CYP-001 met all clinical endpoints and demonstrated positive safety and efficacy data for the treatment of steroid-resistant acute graft-versus-host disease (GvHD) in a Phase 1 trial. Cynata plans to advance its Cymerus™ MSCs into Phase 2 trials for GvHD and critical limb ischemia. In addition, Cynata has demonstrated utility of its Cymerus MSC technology in preclinical models of asthma, critical limb ischemia, diabetic wounds, heart attack and cytokine release syndrome, a life-threatening condition stemming from cancer immunotherapy.

¹ Persistence Market Research 2018 research report: "Osteoarthritis Treatment Market: Global Industry Analysis (2012-2016) and Forecast (2017-2025)."