



ASX ANNOUNCEMENT

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Treatment Commences in Second Patient Cohort in Cynata's World First Clinical Trial

Melbourne, Australia, 24 January 2018: Australian stem cell and regenerative medicine company, Cynata Therapeutics Limited (ASX: CYP), is pleased to announce that the first patient in Cohort B has been treated with CYP-001, the Company's first mesenchymal stem cell (MSC) product for steroid-resistant acute graft-versus-host disease (GvHD).

Key Highlights:

- **First patient** with steroid-resistant acute GvHD treated in second cohort (Cohort B) in Cynata's phase 1 clinical study of its Cymerus™ MSC product, CYP-001
- **Follows encouraging safety and efficacy data** from the first patient cohort, and **recommendation from the Data and Safety Monitoring Board (DSMB)** to commence Cohort B, announced on 22 January

Dr Kilian Kelly, Cynata's Vice President of Product Development, said "The treatment of the first patient in Cohort B, and the ninth patient overall in this trial, is another milestone and value catalyst for the Company, bringing us closer to determining how safe and effective CYP-001 is in this devastating disease. In light of the very encouraging results from the initial cohort, and the higher dose level in Cohort B, we look forward to the final data with optimism."

CYP-001 is manufactured in a scalable process using Cynata's Cymerus platform with induced pluripotent stem cells (iPSCs) as the starting material. The Cymerus process overcomes both the need to source multiple donors and the inherent variability in products derived from multiple donations. Cynata is an industry leader with a sustainable and robust manufacturing process for therapeutic MSC products.

While GvHD is the focus of the current clinical trial, Cynata continues to actively explore additional high-potential target diseases, including asthma, heart attack, brain cancer, and others.

Phase 1 Clinical Trial and Next Steps

Following the advice from the Data and Safety Monitoring Board (DSMB) (as announced by the Company on 22 January 2018), the trial resumed at seven major transplant centres in the UK and Australia. Patients in Cohort B will receive two infusions of CYP-001 one week apart, each at a dose of two million cells/kg, up to a maximum dose of 200 million cells. This is twice (2x) the dose level received by patients in Cohort A. A total of 8 patients are expected to participate in Cohort B; when combined with the 8 patients already treated in Cohort A, this makes a total of 16 patients in the phase 1 trial.

Commencement of the second patient cohort (Cohort B) signifies advancement toward completion of the trial, expected later in 2018.

Ends

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About Graft-versus-host-disease

Graft-versus-host-disease (GvHD) is a complication that can occur after a bone marrow transplant or similar procedure, when the donor's immune cells (from the "graft") attack the recipient of the transplant (the "host"). The only approved treatment for GvHD is corticosteroid therapy, which is typically only effective in about 50 percent of patients. When GvHD fails to improve or worsens despite steroid treatment, patients are described as having steroid-resistant GvHD. The prognosis for these patients is poor, with mortality rates in excess of 90%.¹

About the Phase 1 Clinical Trial (Protocol Number: CYP-GvHD-P1-01)

The trial is entitled "An Open-Label Phase 1 Study to Investigate the Safety and Efficacy of CYP-001 for the Treatment of Adults With Steroid-Resistant Acute Graft Versus Host Disease". Participants must be adults who have undergone an allogeneic haematopoietic stem cell transplant (HSCT) to treat a haematological (blood) disorder and have subsequently been diagnosed with steroid-resistant Grade II-IV GvHD.

The first eight participants were enrolled in Cohort A and received two infusions of CYP-001 at a dose of one million cells per kilogram of body weight (cells/kg), up to a maximum dose of 100 million cells. There was one week between the two CYP-001 infusions in each participant. The next eight participants will be enrolled into Cohort B and receive two infusions of CYP-001 at a dose of two million cells/kg, up to a maximum dose of 200 million cells.

The trial's primary objective is to assess the safety and tolerability of CYP-001, while the secondary objective is to evaluate the efficacy of two infusions of CYP-001 in adults with steroid-resistant GvHD. The primary evaluation period concludes 100 days after the first dose in each participant. Efficacy is assessed on the basis of response to treatment (as determined by change in GvHD grade) and overall survival at 28 and 100 days after the administration of the first dose. After the completion of the primary evaluation period, participants enter a longer-term, non-interventional follow-up period, which will continue for up to two years after the initial dose.

About Cynata Therapeutics (ASX: CYP)

Cynata Therapeutics Limited (ASX: CYP) is an Australian clinical-stage stem cell and regenerative medicine company that is developing a therapeutic stem cell platform technology, Cymerus™, originating from the University of Wisconsin-Madison, a world leader in stem cell research. The proprietary Cymerus™ technology addresses a critical shortcoming in existing methods of production of mesenchymal stem cells (MSCs) for therapeutic use, which is the ability to achieve economic manufacture at commercial scale. Cymerus™ utilises induced pluripotent stem cells (iPSCs) to produce a particular type of MSC precursor, called a mesenchymoangioblast (MCA). Cymerus™ provides a source of MSCs that is independent of donor limitations and an "off-the-shelf" stem cell platform for therapeutic product use, with a pharmaceutical product business model and economies of scale. This has the potential to create a new standard in the emergent arena of stem cell therapeutics, and provides both a unique differentiator and an important competitive position.

¹ Westin JR, Saliba RM, De Lima M, et al. Steroid-Refractory Acute GVHD: Predictors and Outcomes. *Adv Hematol.* 2011; 2011:601953.