ASX ANNOUNCEMENT **21 June 2018**

Cynata Reports Positive 28-day Data from Cohort B of Phase 1 Trial of CYP-001 in GvHD

Melbourne, Australia, 21 June 2018: Australian stem cell and regenerative medicine company Cynata Therapeutics Limited (ASX: CYP) is pleased to announce positive safety and efficacy data from a day 28 analysis of patients in Cohort B of its Phase 1 clinical trial of CYP-001, the Company's lead Cymerus™ mesenchymal stem cell (MSC) product candidate, in steroid-resistant acute graft- versus-host disease (GvHD).

Key Highlights – Cohort B Day 28 Analysis

- **Overall Response rate by Day 28 was 86%** (six out of seven patients treated with CYP-001 showed an improvement in the severity of GvHD by at least one grade compared to baseline)
- **Complete Response rate by Day 28 was 57%** (GvHD signs/symptoms completely resolved in four out of seven patients treated with CYP-001)
- **Higher dose of CYP-001 administered in Cohort B elicited a faster response** than the lower dose in Cohort A. By Day 28, Cohort A had a Complete Response rate of 12.5%, compared to 57% in Cohort B
- **No treatment-related serious adverse events** or safety concerns have been identified
- **All seven patients treated with CYP-001 survived at least until Day 28**
- **Data support advancement of CYP-001 into Phase 2 trial**

Dr Ross Macdonald, Chief Executive Officer of Cynata said: *"The 28-day results for Cohort B are highly encouraging and, together with the excellent data from Cohort A, support the advancement of CYP-001 into a Phase 2 trial in GvHD. Importantly, CYP-001's strong safety profile may enable us to advance the therapy directly into Phase 2 trials in other indications beyond GvHD where there is a high unmet medical need for a consistent and scalable source of high-quality MSCs. We are evaluating our options strategically and expect to provide more details at the appropriate time."*

Eight patients with steroid-resistant acute GvHD were enrolled in Cohort B, as originally planned. Seven out of eight patients enrolled in Cohort B received two infusions of CYP-001 at a dose level of 2 million cells per kilogram of body weight, up to a maximum of 200 million cells per infusion. All treated patients have now reached the pre-specified Day 28 endpoint.

As previously announced, the clinical investigator determined that one patient in Cohort B was no longer a suitable candidate for treatment, due to a medical complication that occurred shortly after enrolment but prior to treatment with CYP-001. This patient has been excluded from analysis, as they did not receive CYP-001 treatment.

All seven patients treated with CYP-001 in Cohort B survived until Day 28. The Overall Response and Complete Response rates were 86% and 57%, respectively. In one case, the GvHD grade remained stable up to Day 21. However, the patient withdrew from the trial on Day 22 to commence palliative care.

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Dr Kilian Kelly, Cynata's Vice President of Product Development, said, *"These results build on the very encouraging data from Cohort A of this trial and are particularly impressive given that all 15 patients enrolled in the Phase 1 trial had failed to respond to corticosteroid therapy, the only approved treatment for GvHD. The response to treatment is compelling, with an even higher Complete Response rate in Cohort B than in Cohort A and no safety concerns reported in either patient cohort. Moreover, the data suggest that the higher dose level has elicited a much quicker treatment response. This is important considering the severely debilitating nature of acute GvHD symptoms in many patients."*

Next Steps

The Primary Evaluation Period will be completed when the final patient in Cohort B reaches 100 days after the first infusion of CYP-001. Cynata will announce Day 100 Cohort B data once an analysis has been completed.

In view of the very encouraging results from this trial Cynata is exploring options to evaluate its Cymerus MSCs in trial in indications beyond GvHD and will provide further information to the market when appropriate.

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About Acute Steroid-Resistant 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 percent.[1]

The global market opportunity for GvHD has been estimated to reach approximately US$500 million p.a. by 2021.[2]

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 were required to be adults who received an allogeneic haematopoietic stem cell transplant (HSCT) to treat a haematological (blood) disorder, and were subsequently 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 1 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 were enrolled into Cohort B, seven of whom received two infusions of CYP-001 at a dose of 2 million cells/kg, up to a maximum dose of 200 million cells. As previously announced (24 May 2018), the clinical investigator determined that one patient in Cohort B was no longer a suitable candidate for treatment, due to a medical complication that occurred shortly after enrolment but prior to treatment with CYP-001. This patient has been excluded from analysis, as they did not receive CYP-001 treatment.

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. Results of the Primary Evaluation Period in Cohort A were announced on 27 February 2018. 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.

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

[2]https://www.visiongain.com/Report/1794/Global-Graft-versus-Host-Disease-(GVHD)-Market-2017-2027

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