ASX ANNOUNCEMENT **22 January 2018**

Encouraging Early Safety and Efficacy Data in Cynata's Phase 1 Trial of CYP-001 in GvHD; DSMB Recommendation to Initiate Enrolment of Second Patient Cohort

Melbourne, Australia; 22 January 2018: Australian stem cell and regenerative medicine company, Cynata Therapeutics Limited (ASX: CYP) is pleased to announce that the independent Data Safety Monitoring Board (DSMB) has recommended that Cynata's clinical trial of its lead Cymerus™ mesenchymal stem cell (MSC) product CYP-001 should progress to the next stage as planned.

Key Highlights:

- **All eight participants in Cohort A (lower dose cohort) have demonstrated at least a Partial Response** (defined as an improvement in the severity of GvHD by at least one grade compared to baseline)
- **No treatment-related serious adverse events** or safety concerns have been identified
- **DSMB recommendation to progress** clinical trial to second cohort (Cohort B)
- **Patient enrolment in Cohort B (higher dose cohort) now open** at seven trial sites in the U.K. and Australia

Cynata's clinical trial, which is the first clinical trial in which patients have been treated with an allogeneic, induced pluripotent stem cell (iPSC)-derived therapeutic MSC product, consists of a planned total of 16 patients with steroid-resistant acute graft-versus-host disease (GvHD). The recommendation to progress to the next stage (Cohort B) followed an independent review by the DSMB of the eight participants in Cohort A. Recruitment for Cohort A commenced in May 2017, and there are currently seven trial sites active and ready to enrol participants into Cohort B.

Steroid-resistant GvHD patients today have a dismal prognosis, where mortality rates are very high. At this time, seven of the eight participants in Cohort A are alive. One participant died after developing pneumonia, which is a common finding in recipients of bone marrow transplants and similar procedures.[1] This death was not considered to be treatment-related.

Participants enrolled in Cohort A of the dose-escalation trial received a dose of CYP-001 that was anticipated to be at the lower end of the effective dose range (one million cells per kilogram of bodyweight, up to a maximum of 100 million cells per infusion). In Cohort B, a further eight participants

[1] *D'Souza A, Zhu X. Current Uses and Outcomes of Hematopoietic Cell Transplantation (HCT): Center for International Blood and Marrow Transplant Research (CIBMTR) Summary Slides, 2016.*

Cynata Therapeutics Limited
Level 3, 62 Lygon Street, Carlton, Victoria 3053, Australia
PO Box 7165, Hawthorn North, Victoria 3122
T: + 613 9824 5254 **F:** + 613 9822 7735 **E:** admin@cynata.com
ABN - 98 104 037 372

will receive two infusions of CYP-001 at a dose of two million cells per kilogram of bodyweight, up to a maximum of 200 million cells per infusion.

Dr Ross Macdonald, CEO of Cynata Therapeutics, said, "We are thrilled to report this encouraging early review of the Phase 1 trial of CYP-001, which marks the first time that patients have been treated with an allogeneic, induced pluripotent stem cell-derived therapeutic MSC product. The improvement in GvHD grade observed in 100% of these gravely ill people is very promising, especially given the low dose administered in Cohort A. The positive DSMB recommendation is an important milestone that enables us to begin enrolment in Cohort B, and advance toward our goal of completing the trial later this year. A successful outcome will support the application of CYP-001 in many medically and commercially significant targets where therapeutic MSCs have shown promising results."

Next Steps

Patient enrolment into Cohort B is now open at seven active sites across the U.K. and Australia. Cynata looks forward to providing further updates to the market as the study progresses.

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CONTACTS: Dr Ross Macdonald, CEO, Cynata Therapeutics, +61 (0)412 119343, ross.macdonald@cynata.com
Daniel Paproth, Australia Media Contact, +61 (0)421 858 982, daniel.paproth@mcpartners.com.au
Laura Bagby, U.S. Media Contact, 312-448-8098, lbagby@6degreespr.com

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 percent.[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 must be adults who have undergone an allogeneic haematopoietic stem cell transplant (HSCT) to treat a haematological (blood) disorder and 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

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

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 the Data Safety Monitoring Board Review

The Data Safety Monitoring Board Review (DSMB) is an expert advisory group, commissioned to ensure objective and independent review of participant safety during the conduct of the Phase 1 trial. In Cohort A, all participants received two infusions of CYP-001, each at a dose of one million cells per kilogram of bodyweight. The DSMB review was triggered when the eighth and final participant in Cohort A reached day 28 (28 days after the first CYP-001 infusion). As of that date, all participants had been followed for between 28 days and six months after their initial CYP-001 infusion.

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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