ASX ANNOUNCEMENT **30 August 2018**

Cynata CYP-001 Stem Cell Therapy Meets All Safety and Efficacy Endpoints in Phase 1 Trial in GvHD

Melbourne, Australia; 30 August 2018: Australian stem cell and regenerative medicine company Cynata Therapeutics Limited (ASX: CYP) is pleased to announce that CYP-001, its lead Cymerus™ mesenchymal stem cell (MSC) product candidate, met all clinical endpoints in a Phase 1 trial for the treatment of steroid-resistant acute graft-versus-host disease (GvHD).

In a Day 100 analysis of patients in the high-dose Cohort B, CYP-001 demonstrated positive safety and efficacy data that is consistent with day 100 data from the lower-dose Cohort A and a day 28 evaluation of Cohort B. This data analysis marks the completion of the Primary Evaluation Period for all patients enrolled in the Phase 1 trial.

Key Highlights following Primary Evaluation Period (100 days) for all 15 patients

- **Overall Response rate by Day 100 was 93%**
  14 out of 15 patients showed an improvement in GvHD severity by at least one grade compared to baseline
- **Complete Response rate by Day 100 was 53%**
  GvHD signs and symptoms completely resolved in 8 out of 15 patients
- **Overall survival at Day 100 was at least 87%**
- **No treatment-related serious adverse events** or safety concerns were identified during the Primary Evaluation Period
- Efficacy data following the completion of the Primary Evaluation Period for Cohort B are consistent with results from earlier data analyses and support advancement of CYP-001 into a Phase 2 trial

Dr Kilian Kelly, Cynata's Vice President, Product Development, said, "We are pleased to announce that CYP-001 has met all clinical endpoints in the first trial of a product based on our Cymerus stem cell technology platform, which validates its potential to generate safe and effective iPSC-derived MSC therapies. Steroid-resistant GvHD is a devastating disease with a high rate of mortality. The clinical results from patients in Cohorts A and B are highly encouraging, as all of the patients had failed to respond to corticosteroid therapy, the only approved treatment for GvHD. We look forward to advancing our Cymerus MSCs into Phase 2 trials for GvHD and other indications."

Trial Results

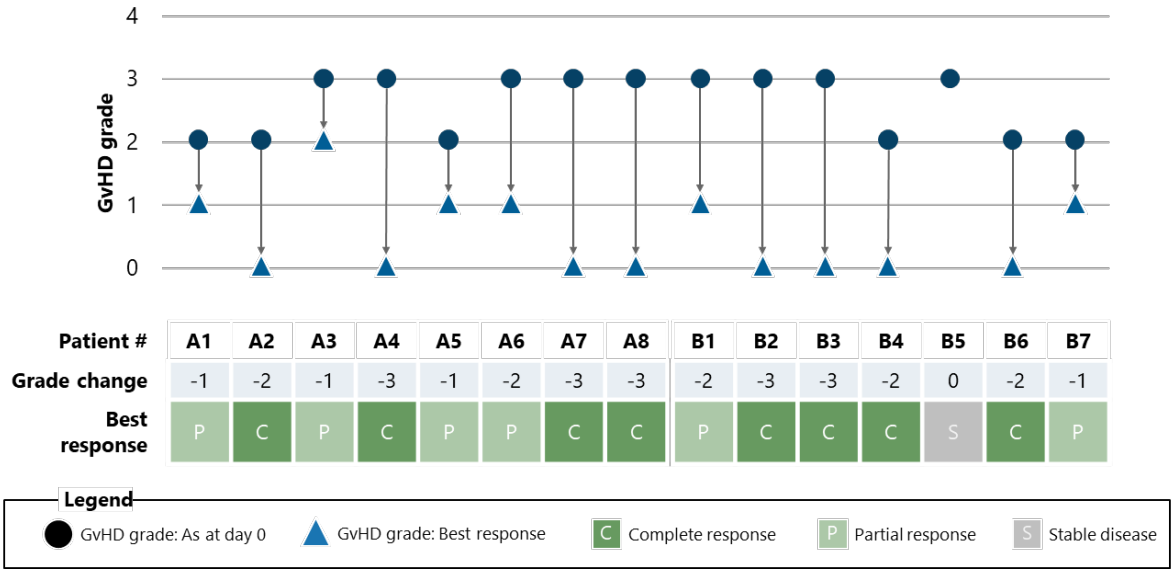
The Primary Evaluation Period for the Phase 1 trial comprised the first 100 days after the initiation of CYP-001 treatment. All treated patients received two infusions of CYP-001. Patients in Cohort A received a dose level of 1 million cells per kilogram of bodyweight, up to a maximum of 100 million cells per infusion. Patients in Cohort B received 2 million cells per kg of bodyweight, up to a maximum of 200 million cells per infusion.

The overall response rate by day 100 in Cohort B was six out seven (86%), and the Complete Response rate was four out of seven (57%). An Overall Response is defined as an improvement in the severity

of GvHD by at least one grade compared to baseline, while a Complete Response is defined as the absence of any GvHD signs/symptoms.

Overall and complete response rates by day 100 in the eight patients enrolled in Cohort A were 100% and 50%, respectively. Although the response rates by Day 100 were similar in Cohort A and B, patients in Cohort B demonstrated a more rapid response. By Day 28, the Complete Response rate was 57% in Cohort B, compared to 12.5% in Cohort A.

Phase I trial results



The overall survival rate across both cohorts at Day 100 was at least 13 out of 15 (87%). As previously reported, one patient in Cohort A died after developing pneumonia, which is a common finding in recipients of bone marrow transplants and similar procedures. This death was not considered to be treatment-related. One patient in Cohort B withdrew from the trial on Day 22 to commence palliative care, which meant it was not possible to collect any further data regarding that patient as part of this trial. All other patients remained alive at Day 100.

Next Steps:

A formal clinical study report (CSR) is being prepared to support further clinical development and commercialisation of CYP-001 in GvHD. The CSR will be provided to Fujifilm pursuant to the license option agreement between Cynata and Fujifilm. Upon receipt, Fujifilm will have 90 days to exercise its license option.

The safety and efficacy data from the Phase I trial in GvHD support the advancement of additional Cymerus MSC product candidates directly to Phase 2 trials in other indications. Cynata has begun planning for a Phase 2 trial in its next indication, critical limb ischemia (CLI).

Cynata will provide an update on ongoing partnering discussions and its proposed phase 2 trial in CLI at the appropriate time.

Dr Ross Macdonald, Cynata's Chief Executive Officer, said, "Cynata is the only company with the ability to produce allogeneic MSC therapies at commercial scale without the limitation of multiple donors. The positive data supports the potential of Cymerus to generate 'off-the-shelf' stem cell therapies for multiple diseases providing significant opportunities for monetisation and commercial partnerships.

The completion of this trial marks a major achievement not only for Cynata but also as a world-first for iPSC-derived therapeutic products."

Ends

CONTACTS: Dr Ross Macdonald, CEO, Cynata Therapeutics, +61 (0)412 119343, ross.macdonald@cynata.com
Alex Liddington-Cox , Australia Media Contact, +61 (0)474701469, alex.liddingtoncox@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.[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 were required to be adults who had 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 1 million cells per kilogram of body weight (cells/kg), up to a maximum dose of 100 million cells. Seven participants in Cohort B received two infusions of CYP-001 at a dose of 2 million cells/kg, up to a maximum dose of 200 million cells. There was one week between the two CYP-001 infusions in each participant.

The trial's primary objective was to assess the safety and tolerability of CYP-001, while the secondary objective was to evaluate the efficacy of two infusions of CYP-001 in adults with steroid-resistant GvHD. The primary evaluation period concluded 100 days after the first dose in each participant. Efficacy was 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 entered 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.