



ASX ANNOUNCEMENT

29 January 2019

## Results of Cynata's World-First GvHD Clinical Trial Accepted for Presentation at Major International Symposium

**Melbourne, Australia; 29 January 2019:** Cynata Therapeutics Limited (ASX: CYP), a clinical-stage biotechnology company specializing in cell therapeutics, is pleased to announce that results from the Phase 1 clinical trial of CYP-001 for the treatment of steroid-resistant acute graft versus host disease (GvHD) will be presented at the Transplantation & Cellular Therapy (TCT) Meetings of the American Society of Bone Marrow Transplantation (ASBMT) and the Center for International Blood and Marrow Transplantation Research (CIBMTR), which will take place in Houston, Texas, from 20-24 February 2019.

CYP-001 is Cynata's lead Cymerus™ mesenchymal stem cell (MSC) product candidate. The Phase 1 trial of CYP-001 represents the first time a clinical trial using induced pluripotent stem cell (iPSC)-derived MSCs has been completed.

Details of the poster are as follows:

**Title:** Results of the First Completed Clinical Trial of an iPSC-Derived Product: CYP-001 in Steroid-Resistant Acute GvHD

**Poster Number:** 362

**Date and Time:** Wednesday, February 20, 2019 from 6:45-7:45 PM

**Location:** George R. Brown Convention Center - Level 3 - Hall B

**Presenter:** Prof. John Rasko, (Professor, Sydney Medical School, University of Sydney; Head, Gene and Stem Cell Therapy Program, Centenary Institute; Head of Department, Cell & Molecular Therapies, Royal Prince Alfred Hospital, Sydney)

Dr Kilian Kelly, Cynata's Vice President of Product Development said "We are very pleased that data from our very successful Phase 1 clinical trial will be presented at this important medical meeting and that it continues to generate significant world-wide attention".

The TCT Meetings are the combined annual meetings of the ASBMT and the CIBMTR, focussed on haematopoietic cell transplantation and cellular therapy.

**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 percent.<sup>1</sup>



### **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 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.

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