

Results of Cynata's World-First GvHD Clinical Trial Accepted for Presentation at American Society of Hematology Annual Meeting

Melbourne, Australia; 2 November 2018: Cynata Therapeutics Limited (ASX: CYP), a clinical-stage biotechnology company specializing in cell therapeutics, is pleased to announce that results from Cohort A of the Phase 1 clinical trial of CYP-001 for the treatment of steroid-resistant acute graft versus host disease (GvHD) will be presented in a poster at the American Society of Hematology (ASH) Annual Meeting, which will take place in San Diego, California, from 1-4 December 2018.

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.

Dr Kilian Kelly, Cynata's Vice President, Product Development, said, "Being selected to present at this prestigious meeting is further recognition of the potential of CYP-001 and the compelling results of our world-first clinical trial. We look forward to raising awareness of CYP-001 and the Cymerus technology more broadly, as we continue planning Phase 2 clinical trials."

Details of the poster are as follows:

Title: A Phase I Trial of iPSC-Derived MSCs (CYP-001) in Steroid-Resistant Acute GvHD

Abstract Number: 4562

Date and Time: Monday, December 3, 2018, 6:00 PM-8:00 PM

Location: Hall GH (San Diego Convention Center)

Presenter: Dr Adrian Bloor, The Christie Hospital, Manchester, UK Chief Investigator of the Phase 1 trial

The ASH Annual Meeting is the largest haematology conference worldwide, with an expected attendance of more than 25,000 haematology professionals, pharmaceutical company executives and investors.

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.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 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.

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