ASX ANNOUNCEMENT **28 March 2018**

FDA Grants Orphan Drug Designation to Cynata's Lead Cymerus™ MSC Product, CYP-001

Melbourne, Australia; 28 March 2018: Australian stem cell and regenerative medicine company, Cynata Therapeutics Limited (ASX: CYP) is pleased to announce that the United Stated Food and Drug Administration (FDA) has granted Cynata Orphan Drug Designation for CYP-001 for the treatment of acute graft versus host disease (GvHD). CYP-001 is the lead mesenchymal stem cell (MSC) product manufactured using Cynata's proprietary Cymerus platform manufacturing technology.

Key Highlights:

- Orphan Drug Designation means CYP-001 is eligible for important incentives, including an extended period of marketing exclusivity, tax credits and FDA fee waivers.
- Positions CYP-001 for cost-effective commercialisation in the USA – the world's largest healthcare market.

Orphan Drug Designation

An Orphan Drug is a therapeutic agent used for the prevention, diagnosis or treatment of a rare disease, which is defined as a disease or condition that affects fewer than 200,000 people in the USA.

Once a product has been granted Orphan Drug Designation by the FDA, the sponsor or manufacturer of that product can take advantage of three special and commercially significant incentives:

- Exclusivity – the potential to receive seven years of marketing exclusivity after FDA marketing approval is granted.
- Tax credit – half of the qualified clinical research costs for a designated orphan product may be claimed as tax credits in the USA.
- Waiver of FDA fees.

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% 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]

Cynata recently announced very positive data from the first cohort of patients in its ongoing Phase 1 trial of CYP-001 for the treatment of steroid-resistant acute GvHD. No treatment-related serious adverse events or safety concerns have been identified to date, while among the eight participants in Cohort A (who received the lower dose level of CYP-001), overall survival at Day 100 was 87.5%, the Overall Response rate by Day 100 was 100% (all eight participants showed an improvement in the severity of GvHD by at least one grade compared to baseline) and the Complete Response rate by Day

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100 was 50% (GvHD signs/symptoms completely resolved in four out of eight patients). Recruitment of the second (higher dose) cohort is currently progressing well.

Dr Kilian Kelly, Cynata's Vice President, Product Development, said, "We are delighted that the FDA has seen fit to grant Orphan Drug Designation to CYP-001, in recognition of the potential of this product to address the substantial unmet need associated with GvHD. This follows on from our successful pre-IND meeting with the FDA last year, and we look forward to building our productive relationship with the FDA over the coming years."

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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.*