

Patient Treatment Commences in Cynata's World First Clinical Trial

- First patient with steroid resistant acute graft-versus-host disease (GvHD) treated in Cynata's phase 1 clinical study of its mesenchymal stem cell (MSC) product, CYP-001
- World first clinical trial using a MSC therapeutic product derived from an induced pluripotent stem cell sourced from a single blood donation from one donor
- Transition into active clinical trials cements Cynata's world leading position in the development and commercialisation of second generation stem cell products
- Partnership with Fujifilm for the commercialisation of CYP-001; trial commencement accelerates this target

Melbourne, Australia; 16 May 2017: Australian stem cell and regenerative medicine company, Cynata Therapeutics Limited (ASX: CYP), is pleased to announce that the first patient has been treated with CYP-001, the Company's first mesenchymal stem cell (MSC) product, in its Phase 1 clinical trial in patients with steroid-resistant acute graft versus host disease (GvHD).

Commencement of the trial represents a milestone for the Company and is the first time in the world that a patient has been treated with an allogeneic, induced pluripotent stem cell (iPSC)-derived therapeutic MSC product.

CYP-001 is manufactured in a scalable process using Cynata's Cymerus™ platform with iPSCs as the starting material. Cynata has sourced its iPSCs from Cellular Dynamics International, a Fujifilm subsidiary company. Cynata recently announced a strategic alliance with Fujifilm pursuant to which Fujifilm has taken an approximately 9% equity stake in Cynata making it the Company's largest shareholder.

These high quality clinical-grade iPSCs were derived from a single blood donation using a non-viral, non-integrating episomal reprogramming method, without genome modification. The Cymerus process overcomes both the need to source multiple donors and the inherent variability in products derived from multiple donations.

About Graft vs Host Disease

GvHD often follows a bone marrow transplant or similar procedure, and occurs when the immune cells in the donor material (the graft) attack the recipient's tissues (the host) as "foreign". Bone marrow transplants are used in the treatment of certain cancers including leukaemia. Corticosteroids are the current mainstay of GvHD treatment, but are often not effective. When steroid treatment fails, which is known as steroid-resistant GvHD, mortality rates can be as high as 80%.

Phase 1 Clinical Trial and Next Steps

A total of 16 patients are expected to participate in the phase 1 trial and participating patients will receive two infusions of CYP-001, with a week between doses. The trial has been opened for recruitment at several major transplant centres in the UK and Australia, and the first patient was treated at one of the UK centres.

The clinical study design includes a Data and Safety Monitoring Board review following the treatment of the eighth patient and the Company expects to provide an update at that time.



“Treatment of steroid-resistant GvHD is one of the major challenges in management of patients undergoing bone marrow transplantation,” said Dr Adrian Bloor, Consultant Haematologist at The Christie Hospital, Manchester, and the UK Chief Investigator for this trial.

“A number of previous studies have demonstrated that MSCs can be an effective treatment, but producing consistent MSCs in sufficient numbers for clinical use has been a major challenge until now. The Cymerus™ process offers a solution to this problem, by enabling the large-scale manufacture of a uniform MSC product derived from a single, one-time donor. I am delighted that patient dosing has now commenced, and I am optimistic that this technology will be of benefit to patients”, said Dr Bloor.

Cynata Managing Director and Chief Executive Officer Dr Ross Macdonald says the trial was another major milestone and value catalyst for the Company.

“The treatment of the first patient in this trial marks the beginning of an exciting new chapter in stem cell therapeutics and the future of regenerative medicine,” said Dr Macdonald.

“Our Cymerus™ technology eliminates the reliance upon multiple donors and the need to excessively expand MSCs derived from them. Cynata is truly at the forefront of the industry with a sustainable and robust manufacturing process for therapeutic MCS products.”

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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). The Cymerus™ platform provides a source of MSCs that is independent of donor limitations and provides 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.

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 will be enrolled in Cohort A and receive 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. There will be one week between the two CYP-001 infusions in each patient. The next eight participants will be enrolled into Cohort B and receive two infusions of CYP 001 at a dose of 2 million cells/kg, up to a maximum dose of 200 million cells. The primary objective of the trial is to assess safety and tolerability, 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 will conclude 100 days after the first dose in each patient. Efficacy will be 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 will enter a longer term non-interventional follow-up period, which will continue for up to two years after the initial dose.

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About Cellular Dynamics International

Cellular Dynamics International (CDI), a Fujifilm company, is a leading developer and manufacturer of human cells used in drug discovery, toxicity testing, stem cell banking, and cell therapy development. The Company partners with innovators from around the world to combine biologically relevant human cells with the newest technologies to drive advancements in medicine and healthier living. CDI's technology offers the potential to create induced pluripotent stem cells (iPSCs) from anyone, starting with a standard blood draw, and followed by the powerful capability to develop into virtually any cell type in the human body. Founded in 2004 by Dr. James Thomson, a pioneer in human pluripotent stem cell research, Cellular Dynamics is based in Madison, Wisconsin, with a second facility in Novato, California. For more information please visit www.cellulardynamics.com and follow on Twitter @CellDynamics."

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