

## **Cynata Announces Ethics Approval and Expedited Regulatory Pathway for Phase 3 Osteoarthritis Clinical Trial**

**Melbourne, Australia; 18 June 2020:** Cynata Therapeutics Limited (ASX: “CYP”, “Cynata”, or the “Company”), a clinical-stage biotechnology company specialising in cell therapeutics, is pleased to report important progress towards the commencement of recruitment in the Phase 3 clinical trial of CYP-004, its Cymerus™ mesenchymal stem cell (MSC) product for osteoarthritis. This trial is sponsored by the University of Sydney, and funded by an Australian Government National Health and Medical Research Council (NHMRC) competitive Project Grant.

### **Key Highlights:**

- **Phase 3 clinical trial of CYP-004 approved by the University of Sydney HREC, a key milestone to trial commencement**
- **TGA confirms that the trial can be conducted under notification-only CTN scheme**
- **Led by the University of Sydney and funded by NHMRC, the 440-patient trial is expected to take place at study centres in Sydney and Tasmania**

The Phase 3 clinical trial of CYP-004 has now been formally approved by the University of Sydney Human Research Ethics Committee (HREC). The Company originally described this as a Phase 2 clinical trial, but it has since been determined that a Phase 3 trial will be performed, and that is reflected in the final HREC-approved version of the protocol.

Furthermore, the Company is pleased to announce that agreement has been reached on an expedited regulatory pathway for this trial. The Company previously announced (8 May 2020) that the trial sponsor, the University of Sydney, intended to utilise the Clinical Trial Exemption (CTX) clinical trial approval route for this trial. However, after further consultation between the Company, the University of Sydney and the Therapeutic Goods Administration (TGA), the TGA has advised that the trial can be conducted under the Clinical Trial Notification (CTN) scheme subject to Cynata supplying appropriate Good Manufacturing Compliance (GMP) compliance documentation to the trial sponsor. The CTN scheme requires only the submission of a notification to the TGA, as opposed to the formal review and approval process required under the CTX scheme.

The previously announced clinical trial restrictions imposed by the University of Sydney, which are aligned with Federal and state government recommendations in response to COVID-19, remain in place for now, but the Company expects patient recruitment to commence once those restrictions are lifted and final procedural and administrative arrangements are completed.

Dr Kilian Kelly, Cynata’s Chief Operating Officer, commented:

*“We are delighted to gain ethics approval for this important Phase 3 clinical trial in osteoarthritis patients. Approval by the HREC is a key milestone toward commencement of the trial. We look forward to providing further information around the timing of commencement of patient recruitment once more clarity is available regarding the lifting of COVID-19-related restrictions.”*

### **Osteoarthritis Clinical Trial Overview**

The aim of the Phase 3 clinical trial is to assess the effect of Cymerus MSCs compared to placebo on clinical outcomes and knee joint structure over a two-year period, in 440 patients with osteoarthritis

#### **Cynata Therapeutics Limited**

Level 3, 62 Lygon Street, Carlton, Victoria 3053, Australia  
PO Box 7165, Hawthorn North, Victoria 3122

T: + 613 9824 5254 F: + 613 9822 7735 E: [info@cynata.com](mailto:info@cynata.com)

ABN - 98 104 037 372

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of the knee. Preclinical research has shown that MSCs can exert a number of important effects that may improve outcomes in patients with osteoarthritis, including release of cytokines and growth factors that reduce inflammation and promote tissue repair, new blood vessel formation, and regeneration of compromised cartilage.

The trial will take place at study centres in Sydney and Tasmania, and be led by Professor David Hunter. Professor Hunter is the Florance and Cope Chair of Rheumatology and Professor of Medicine at the University of Sydney and has been Chief Investigator of numerous clinical trials in osteoarthritis. He has more than 450 publications in high-impact journals, including the *New England Journal of Medicine*, *Journal of the American Medical Association* and *British Medical Journal*.

The research team also includes Professor Changhai Ding (University of Tasmania), Professor Stefan Lohmander (Lund University, Sweden), Dr Rachel O'Connell (University of Sydney) and Dr Xia Wang (University of Sydney), as well as numerous associate investigators.

-ENDS-

Authorised for release by Dr Ross Macdonald, Managing Director & CEO

**CONTACTS:** Dr Ross Macdonald, CEO, Cynata Therapeutics, +61 (0)412 119343, [ross.macdonald@cynata.com](mailto:ross.macdonald@cynata.com)  
Claire LaCagnina, U.S. Media Contact, +1 315.765.1462, [clacagnina@6degreespr.com](mailto:clacagnina@6degreespr.com)

#### About the Phase 3 Clinical Trial in Osteoarthritis (The SCULPTOR trial)

The clinical trial, entitled *Stem Cells as a symptom- and strUcture-modifying Treatment for medial tibiofemoral OsteoaRthritis: a randomised placebo-controlled trial*, is funded by an Australian Government NHMRC Project Grant, in addition to in-kind contributions from participating institutions. Cynata will supply Cymerus™ MSCs for use in the trial and will not be required to contribute any cash to fund the project.

The trial will be a randomised, double-blind placebo-controlled trial, which will seek to enrol 440 patients with osteoarthritis of the knee. Participants will receive intra-articular injections of Cymerus MSCs or placebo on three occasions over a period of 1 year, and will be followed up for a total of two years from enrolment. The co-primary endpoints are: (i) the proportion of participants achieving patient-acceptable symptom state (PASS) for knee pain at 24 months; and (ii) central medial femorotibial (cMFT) cartilage loss from baseline to 24 months. Secondary outcome measures will include assessments of pain, other symptoms, physical function and quality of life.

#### 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 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 severe complications arising from COVID-19, GvHD, critical limb ischemia and into Phase 3 for osteoarthritis. In addition, Cynata has demonstrated utility of its Cymerus™ MSC technology in preclinical models of asthma, diabetic wounds, heart attack, sepsis, acute respiratory distress syndrome (ARDS) and cytokine release syndrome.

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