

September 2017 Quarterly Report

Melbourne, Australia; 27 October 2017: Australian stem cell and regenerative medicine company, Cynata Therapeutics Limited (ASX: CYP), has today released its Appendix 4C Report for the three-month period to 27 September 2017 and is pleased to provide a review of operational progress during the period.

KEY HIGHLIGHTS

- The Company continues to prioritise operational progress relating to its world first clinical trial of CYP-001 for the treatment of GvHD
- A total of seven recruiting sites are active with five hospitals in the U.K. and two in Australia
- Clear path determined for preclinical and clinical protocol requirements for CYP-001 product development in US and Canada with potential trials targeted
- Strengthened patent portfolio with additional patents under the U.S. Patent and Trademark Office (USPTO) and IP Australia
- Pre-clinical trials commenced to investigate Cymerus™ MSCs as a treatment for acute respiratory distress syndrome (ARDS)
- Positive results from second asthma study and analyses currently in progress that will potentially advance a Cymerus product towards clinical trials

Cynata Therapeutics CEO Dr Ross Macdonald commented:

"The Company is focused on progressing the world first GvHD trial and we are pleased with recruitment progress to date, especially given the small pool of potential trial participants suffering from this rare but devastating condition. Our progress clarifying the regulatory pathway with the US FDA is also positioning the Company for success as we move forward. We continue to maintain a long-term view, and have made strong progress in relation to MSCs as a treatment for acute respiratory distress syndrome (ARDS) and asthma."

OPERATIONAL UPDATE

World First Clinical Trial Approaching Half-Way Point Analysis

In May 2017, the first patient was dosed 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). This clinical trial also is the first trial in the world to study an iPSC-derived MSC product. Patients are being recruited at five major transplant centres in the UK and two in Australia and the Company expects to conduct the Data Safety Monitoring Board (DSMB) analysis in the first 8 patients in the near future. A total of 16 patients are expected to participate in the phase 1 trial.



The Company has been in discussions with the FDA and in July received written advice that has detailed the regulatory approval path under an Investigational New Drug (IND) for Cynata's CYP-001 product. The FDA confirmed that the scope and substance of Cynata's "Chemistry, Manufacturing and Controls" (CMC) dossier is commensurate with its expectations, which indicates that Cymerus MSC products are expected to be of suitable quality for clinical trial use in the US.

The FDA also clarified that Cynata may submit a request for "Regenerative Medicine Advanced Therapy" (RMAT) designation, which could lead to accelerated product approval under USA "21st Century Cures Act".

Furthermore, Cynata has met with Health Canada, the Canadian Government department for health, regarding the clinical development of its CYP-001 product. Health Canada agreed that the unique Cymerus process, including donor screening and testing, the creation of the MSCs from iPSCs (induced pluripotent stem cells) using the Cymerus platform, and the manufacture and testing of the final product, meets its expectations for a product entering clinical trials.

Cynata also received clarification from Health Canada on the design of preclinical studies required to support a Clinical Trial Application in Canada. Cynata is currently acting upon the advice from both Health Canada and the FDA in order to establish the preclinical trial activities to undertake ahead of filing applications to commence clinical trials in these jurisdictions.

Further Strengthened IP Portfolio

The U.S. Patent and Trademark Office (USPTO) has granted a patent for Cynata's proprietary Cymerus mesenchymal stem cell technology. The patent entitled "A method of making primate cells expressing apelin receptor that have mesangioblast potential" covers certain proprietary methods relating to the platform's ability to efficiently manufacture mesenchymal stem cells at scale. The patent has an expiration date of 1 February 2028.

Cynata has also filed a number of further patent applications with *IP Australia* covering certain novel and innovative applications of its proprietary Cymerus™ mesenchymal stem cell (MSC) technology and another that would expand the Company's patent portfolio to include immunotherapy.

Pulmonary Disease—New Study Commenced

The Company commenced its investigation into the use of its Cymerus MSCs as a treatment for **acute respiratory distress syndrome (ARDS)** with the *Critical Care Research Group* in association with the *Prince Charles Hospital*. The study is evaluating the effectiveness of Cymerus MSCs in a sheep model of ARDS involving a support treatment called extracorporeal membrane oxygenation (ECMO), which acts as an artificial lung to oxygenate the blood. A successful study would likely result in progression to a clinical trial in this very challenging condition that results in approximately 10% of all ICU admissions.



Positive Data from Second Preclinical Asthma Study

Highly promising data supporting the efficacy of Cymerus MSCs in a second preclinical asthma study was reported. The study focused on the effects of Cymerus MSCs in combination with or in comparison to the corticosteroid, dexamethasone, which is commonly used to treat exacerbations of asthma in human patients and found that Cymerus MSCs caused significantly greater reduction of airway hyperresponsiveness when compared to existing corticosteroid treatment. The data strongly suggested that Cymerus MSCs can be administered instead of or in combination with corticosteroids to reduce exacerbations of asthma. Further analyses on the effects of these cells on other features of the disease process including inflammation and airway remodelling are currently in progress and will advance the path towards clinical trials.

OUTLOOK

Cynata has been progressing towards a major milestone during the September quarter, with the advancement of its world first clinical trial for its lead priority, GvHD. The Company is approaching the half-way point (DSMB) analysis which will assess the welfare of the patients in the first study cohort, i.e. those patients receiving two doses of 1 million cells/kg, prior to commencing the second cohort of patients who will receive two doses of 2 million cells/kg. The DSMB analysis will be conducted 28 days after the eighth patient is dosed.

Cynata has a diverse portfolio of other targeted indications that it continues to progress through its partnerships with leading investigative institutions. The licence option agreement in place with FUJIFILM can be exercised any time up until 90 days after completion of the GvHD clinical trial and, should FUJIFILM choose to exercise the option, is expected to generate over \$60m in licence and milestone fees, in addition to royalty payments on eventual product sales. All future costs of developing and commercialising CYP-001 for GvHD will be met by FUJIFILM. Cynata is focused on securing additional partnerships with pharmaceutical companies and developers across its development pipeline in order to secure early revenue streams in the form of upfront licence and milestone payments, as it has done through its agreement with FUJIFILM.

Ends

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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.

Appendix 4C

Quarterly report for entities subject to Listing Rule 4.7B

Introduced 31/03/00 Amended 30/09/01, 24/10/05, 17/12/10, 01/09/16

Name of entity

Cynata Therapeutics Limited

ABN

98 104 037 372

Quarter ended ("current quarter")

30 September 2017

Consolidated statement of cash flows	Current quarter	Year to date
	\$A'000	(3 months)
		\$A'000
1. Cash flows from operating activities		
1.1 Receipts from customers	-	-
1.2 Payments for		
(a) research and development	(1,077)	(1,077)
(b) product manufacturing and operating costs	-	-
(c) advertising and marketing	(206)	(206)
(d) leased assets	-	-
(e) staff costs	(146)	(146)
(f) administration and corporate costs	(446)	(446)
1.3 Dividends received (see note 3)	-	-
1.4 Interest received	49	49
1.5 Interest and other costs of finance paid	-	-
1.6 Income taxes paid	-	-
1.7 Government grants and tax incentives	46	46
1.8 Other (provide details if material)	-	-
1.9 Net cash from / (used in) operating activities	(1,780)	(1,780)
2. Cash flows from investing activities		
2.1 Payments to acquire:		
(a) property, plant and equipment	-	-
(b) businesses (see item 10)	-	-
(c) investments	-	-

Consolidated statement of cash flows	Current quarter \$A'000	Year to date (3 months) \$A'000
(d) intellectual property	-	-
(e) other non-current assets	-	-
2.2 Proceeds from disposal of:		
(a) property, plant and equipment	-	-
(b) businesses (see item 10)	-	-
(c) investments	-	-
(d) intellectual property	-	-
(e) other non-current assets	-	-
2.3 Cash flows from loans to other entities	-	-
2.4 Dividends received (see note 3)	-	-
2.5 Other (provide details if material)	-	-
2.6 Net cash from / (used in) investing activities	-	-

3. Cash flows from financing activities		
3.1 Proceeds from issues of shares	-	-
3.2 Proceeds from issue of convertible notes	-	-
3.3 Proceeds from exercise of share options	-	-
3.4 Transaction costs related to issues of shares, convertible notes or options	-	-
3.5 Proceeds from borrowings	-	-
3.6 Repayment of borrowings	-	-
3.7 Transaction costs related to loans and borrowings	-	-
3.8 Dividends paid	-	-
3.9 Other (provide details if material)	-	-
3.10 Net cash from / (used in) financing activities	-	-

4. Net increase / (decrease) in cash and cash equivalents for the period		
4.1 Cash and cash equivalents at beginning of quarter/year to date	10,350	10,350
4.2 Net cash from / (used in) operating activities (item 1.9 above)	(1,780)	(1,780)
4.3 Net cash from / (used in) investing activities (item 2.6 above)	-	-
4.4 Net cash from / (used in) financing activities (item 3.10 above)	-	-

Consolidated statement of cash flows		Current quarter	Year to date (3 months)
		\$A'000	\$A'000
4.5	Effect of movement in exchange rates on cash held	126	126
4.6	Cash and cash equivalents at end of quarter	8,696	8,696

5. Reconciliation of cash and cash equivalents	Current quarter	Previous quarter
at the end of the quarter (as shown in the consolidated statement of cash flows) to the related items in the accounts	\$A'000	\$A'000
5.1	Bank balances	10,350
5.2	Call deposits	-
5.3	Bank overdrafts	-
5.4	Other (provide details)	-
5.5	Cash and cash equivalents at end of quarter (should equal item 4.6 above)	8,696

6. Payments to directors of the entity and their associates	Current quarter	
	\$A'000	
6.1	Aggregate amount of payments to these parties included in item 1.2	369
6.2	Aggregate amount of cash flow from loans to these parties included in item 2.3	-
6.3	Include below any explanation necessary to understand the transactions included in items 6.1 and 6.2	

Directors' fees, salaries including superannuation benefits, and professional consultancy fees. All payments are on normal commercial terms.

7. Payments to related entities of the entity and their associates	Current quarter	
	\$A'000	
7.1	Aggregate amount of payments to these parties included in item 1.2	-
7.2	Aggregate amount of cash flow from loans to these parties included in item 2.3	-
7.3	Include below any explanation necessary to understand the transactions included in items 7.1 and 7.2	

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8. Financing facilities available <i>Add notes as necessary for an understanding of the position</i>	Total facility amount at quarter end \$A'000	Amount drawn at quarter end \$A'000
8.1 Loan facilities	-	-
8.2 Credit standby arrangements	-	-
8.3 Other (please specify)	-	-
8.4 Include below a description of each facility above, including the lender, interest rate and whether it is secured or unsecured. If any additional facilities have been entered into or are proposed to be entered into after quarter end, include details of those facilities as well.		

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9. Estimated cash outflows for next quarter	\$A'000
9.1 Research and development	(1,645)
9.2 Product manufacturing and operating costs	-
9.3 Advertising and marketing	(176)
9.4 Leased assets	-
9.5 Staff costs	(68)
9.6 Administration and corporate costs	(235)
9.7 Other (provide details if material)	-
9.8 Total estimated cash outflows	(2,125)

10. Acquisitions and disposals of business entities (items 2.1(b) and 2.2(b) above)	Acquisitions	Disposals
10.1 Name of entity	-	-
10.2 Place of incorporation or registration	-	-
10.3 Consideration for acquisition or disposal	-	-
10.4 Total net assets	-	-
10.5 Nature of business	-	-

Compliance statement

- 1 This statement has been prepared in accordance with accounting standards and policies which comply with Listing Rule 19.11A.
- 2 This statement gives a true and fair view of the matters disclosed.

Sign here: .....
Managing Director

Date: 27 October 2017

Print name: Dr Ross Macdonald

Notes

1. The quarterly report provides a basis for informing the market how the entity's activities have been financed for the past quarter and the effect on its cash position. An entity that wishes to disclose additional information is encouraged to do so, in a note or notes included in or attached to this report.
2. If this quarterly report has been prepared in accordance with Australian Accounting Standards, the definitions in, and provisions of, *AASB 107: Statement of Cash Flows* apply to this report. If this quarterly report has been prepared in accordance with other accounting standards agreed by ASX pursuant to Listing Rule 19.11A, the corresponding equivalent standard applies to this report.
3. Dividends received may be classified either as cash flows from operating activities or cash flows from investing activities, depending on the accounting policy of the entity.