

## June 2017 Quarterly Report

### Highlights:

- **World first clinical trial for the treatment of GvHD commenced: major milestone**
- **New product development collaboration to investigate Cymerus™ MSCs as a treatment for acute respiratory distress syndrome (ARDS)**
- **Pre-Investigational New Drug (IND) meeting with the US FDA Office of Cell, Tissue and Gene Therapy products with guidance on the regulatory approval path received and potential for accelerated approval under USA “21<sup>st</sup> Century Cures Act”**
- **Further patent applications filed with *IP Australia* and Notice of Allowance from the US Patent and Trademark Office further strengthening Cynata’s IP**

### Overview

In the field of stem cells and regenerative medicine, mesenchymal stem cells (MSCs) have emerged as one of the most promising candidates for mainstream medical use in a wide variety of economically important diseases and as such have very exciting commercial potential. Cynata’s business focus is the development and commercialisation of a novel, proprietary technology that addresses a critical shortcoming in existing methods of production of MSCs for therapeutic use, which is the ability to achieve economic manufacture at commercial scale. With its Cymerus™ process, which involves the use of induced pluripotent stem cells (iPSC’s) as starting material, Cynata is the only company in the world with technology for the manufacture of therapeutic allogeneic MSCs without reliance upon multiple stem cell donors.

### Operational Highlights

#### GvHD – World First Clinical Trial Commenced

Cynata achieved a major milestone with the commencement of its first clinical trial. The first patient was dosed with CYP-001, the Company’s lead MSC product, in its Phase 1 trial in patients with steroid-resistant acute graft versus host disease (GvHD). The commencement of the trial represents the first time in the world that a patient has been treated with an allogeneic, iPSC-derived therapeutic MSC product.

A total of 16 patients are expected to participate in the trial, each of whom 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.

Furthermore, the Company was granted a pre-Investigational New Drug (IND) meeting with the United States Food and Drug Administration (FDA) Office of Cell, Tissue and Gene Therapy products, to discuss the regulatory approval path for Cynata’s proprietary Cymerus mesenchymal stem cell (MSC) products in the USA. Written advice has since been received (see announcement dated 5 July 2017) which has detailed the regulatory approval path for Cynata’s CYP-001 product. 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 “21<sup>st</sup> Century Cures Act”.

#### Pulmonary – Positive Preliminary Data and New Study Added

The Company announced it is commencing an 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 will evaluate the effectiveness of Cymerus MSCs in sheep with ARDS who are currently being supported by a treatment called extracorporeal membrane oxygenation (ECMO), which acts as an artificial lung to oxygenate the blood.



ARDS commonly occurs in previously healthy individuals. It accounts for approximately 10% of all Intensive Care Unit admissions and almost 25% of patients requiring mechanical ventilation. If the study is successful, it is anticipated that the data would support progression to a clinical trial of Cymerus MSCs in humans with ARDS undergoing ECMO support.

Furthermore, a peer review of the preclinical study in asthma, completed in partnership with the Monash Lung Biology Network (part of a consortia that includes Monash University), has now completed and the study was published in the FASEB Journal, one of the world's most cited peer reviewed biology journals. The study examined Cymerus MSCs in a mouse model of chronic allergic airways disease and found that the MSCs may provide an efficacious and safe treatment for asthma. The study is paving the way for a clinical trial in asthma patients.

### **IP Strengthened with Additional Patents**

Cynata filed a number of further patent applications with IP Australia covering certain novel and innovative applications of its proprietary Cymerus MSC technology that expand the Company's patent portfolio to include immunotherapy.

The Company also received Notice of Allowance from the US Patent and Trademark Office for its Cymerus platform that will cover the 'method of making primate cells expressing apelin receptors that have mesangioblast potential,' further strengthening its intellectual property.

### **Outlook**

This quarter the Company achieved a major milestone with the commencement of a world first clinical trial for its lead program, GvHD, with patient recruitment for the trial actively underway at the study centres. The Company's strategic partnership with Fujifilm continues to progress well with ongoing interaction about future activities designed to expedite the commercial development of CYP-001.

Cynata is partnered with leading investigative institutions for the ongoing development and research of its Cymerus technology. The quality of its partners has provided strong support and validation of its ability and potential in the regenerative medicine sector and the Company is well positioned as it advances its preclinical trials across other indications.

### **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 developing therapies based on its proprietary Cymerus™ stem cell technology platform. Cymerus overcomes critical issues in the production of therapeutic mesenchymal stem cells (MSCs) by enabling the economical manufacture of commercial-scale MSCs, independent of multi-donor limitations. Cymerus' novel approach utilises induced pluripotent stem cells (iPSCs) derived from a single blood donation to generate mesenchymal stem cells (MSCs), a precursor that is used to manufacture an unlimited number of therapeutic MSCs. Cynata's unique "off-the-shelf" Cymerus platform has the potential to create a new standard in the development and manufacture of stem cell therapeutics.

## 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 June 2017

<b>Consolidated statement of cash flows</b>	<b>Current quarter</b>	<b>Year to date</b>
	<b>\$A'000</b>	<b>(12 months)</b>
		<b>\$A'000</b>
<b>1. Cash flows from operating activities</b>		
1.1 Receipts from customers		
1.2 Payments for		
(a) research and development	(826)	(3,833)
(b) product manufacturing and operating costs	-	-
(c) advertising and marketing	(232)	(554)
(d) leased assets	-	-
(e) staff costs	(104)	(570)
(f) administration and corporate costs	(191)	(857)
1.3 Dividends received (see note 3)	-	-
1.4 Interest received	11	68
1.5 Interest and other costs of finance paid	-	-
1.6 Income taxes paid	-	-
1.7 Government grants and tax incentives	-	1,749
1.8 Other (fees pursuant to license agreement)	-	(309)
<b>1.9 Net cash from / (used in) operating activities</b>	<b>(1,342)</b>	<b>(4,306)</b>

<b>2. Cash flows from investing activities</b>		
2.1 Payments to acquire:		
(a) property, plant and equipment	-	-
(b) businesses (see item 10)	-	-
(c) investments	-	-

Consolidated statement of cash flows	Current quarter	Year to date (12 months)
	\$A'000	\$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)	-	-
<b>2.6 Net cash from / (used in) investing activities</b>	<b>-</b>	<b>-</b>

<b>3. Cash flows from financing activities</b>		
3.1 Proceeds from issues of shares	-	9,972
3.2 Proceeds from issue of convertible notes	-	-
3.3 Proceeds from exercise of share options	-	30
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		
- Share issue costs	-	(386)
- Refund of option exercise monies	-	(30)
<b>3.10 Net cash from / (used in) financing activities</b>	<b>-</b>	<b>9,586</b>

<b>4. Net increase / (decrease) in cash and cash equivalents for the period</b>		
4.1 Cash and cash equivalents at beginning of quarter/year to date	11,586	4,866
4.2 Net cash from / (used in) operating activities (item 1.9 above)	(1,342)	(4,306)
4.3 Net cash from / (used in) investing activities (item 2.6 above)	-	-

<b>Consolidated statement of cash flows</b>		<b>Current quarter</b>	<b>Year to date (12 months)</b>
		<b>\$A'000</b>	<b>\$A'000</b>
4.4	Net cash from / (used in) financing activities (item 3.10 above)	-	9,586
4.5	Effect of movement in exchange rates on cash held	106	204
<b>4.6</b>	<b>Cash and cash equivalents at end of quarter</b>	<b>10,350</b>	<b>10,350</b>

<b>5. Reconciliation of cash and cash equivalents</b>	<b>Current quarter</b>	<b>Previous quarter</b>	
at the end of the quarter (as shown in the consolidated statement of cash flows) to the related items in the accounts	<b>\$A'000</b>	<b>\$A'000</b>	
5.1	Bank balances	10,350	11,586
5.2	Call deposits	-	-
5.3	Bank overdrafts	-	-
5.4	Other (provide details)	-	-
<b>5.5</b>	<b>Cash and cash equivalents at end of quarter (should equal item 4.6 above)</b>	<b>10,350</b>	<b>11,586</b>

**6. Payments to directors of the entity and their associates**

- 6.1 Aggregate amount of payments to these parties included in item 1.2
- 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

<b>Current quarter</b>
<b>\$A'000</b>
225
-

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**

- 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

<b>Current quarter</b>
<b>\$A'000</b>
-
-

-

8. <b>Financing facilities available</b> <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. <b>Estimated cash outflows for next quarter</b>	\$A'000
9.1 Research and development	2,384
9.2 Product manufacturing and operating costs	-
9.3 Advertising and marketing	161
9.4 Leased assets	-
9.5 Staff costs	168
9.6 Administration and corporate costs	358
9.7 Other (provide details if material)	-
<b>9.8 Total estimated cash outflows</b>	<b>3,071</b>

10. <b>Acquisitions and disposals of business entities (items 2.1(b) and 2.2(b) above)</b>	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:

  
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**Managing Director**

Date: 28 July 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.