

March 2018 Quarterly Report

Melbourne, Australia; 27 April 2018: Australian stem cell and regenerative medicine company, Cynata Therapeutics Limited (ASX: CYP or “the Company”), has today released its Appendix 4C Report for the three-month period to 31 March 2018 and is pleased to provide a review of operational progress during the period.

Highlights

- Encouraging early safety and efficacy data in the ongoing Phase 1 clinical trial, with all eight participants in the first cohort demonstrating at least a Partial Response, 50% demonstrating a Complete Response, and a survival rate of 87.5%
- DSMB recommended progression onto the second cohort of patients at higher dosage and recruitment is now well underway
- MoU signed with Celularity, Inc. for the evaluation of potential commercial opportunities for the Cymerus™ platform and Celularity’s leading cell therapy assets
- FDA grants Orphan Drug Designation for CYP-001, making it eligible for a number of incentives and positioning it for cost-effective commercialisation in the US

Operational update

Encouraging data received from first patient cohort and recruitment of the second cohort underway

The Company’s world first clinical trial for the treatment of GvHD (graft-versus-host disease), in which patients are treated with an allogeneic, induced pluripotent stem cell (iPSC)-derived therapeutic mesenchymal (MSC) product, reported encouraging efficacy and safety data following the Data Safety Monitoring Board (DSMB) review in January.

Importantly, at the conclusion of the Primary Evaluation Period (100 days) the overall response rate (being an improvement in the severity of GvHD by at least one grade compared to baseline) was 100% and the complete response rate was 50% (being complete absence of symptoms of GvHD). This is an astounding result, given that GvHD is a life-threatening disease. The survival rate was 87.5%; one patient died as a result of developing pneumonia, which is a common finding and not considered to be a treatment-related illness.

Participants enrolled in Cohort A received a dose of CYP-001 that was anticipated to be at the lower end of the effective dose range (one million cells per kilogram of bodyweight, up to a maximum of 100 million cells per infusion).

No treatment-related serious adverse effects or safety concerns were identified and the DSMB recommended that the clinical trial progress, with the first of a further eight patients beginning treatment in the second cohort (Cohort B) at the end of January 2018.

Recruitment continues at the seven major transplant centres in the UK and Australia. Patients in Cohort B will receive two infusions of CYP-001 one week apart, each at a dose of two million cells/kg, up to a maximum dose of 200 million cells. This is twice (2x) the dose level received by patients in Cohort A. Commencement of Cohort B signifies advancement toward completion of the trial, expected later in 2018.

MoU signed with Celularity for commercial evaluation

In January, Cynata signed a non-binding MoU with Celularity Inc., to assess how the Cymerus technology can enhance Celularity's ability to produce stem cells in large quantities. Pursuant to the MoU, Cynata's proprietary Cymerus platform will be evaluated in conjunction with Celularity's cell therapy assets, and the successful evaluation could lead to the development of new products and cell therapies in the regenerative medicine field.

Celularity boasts a portfolio of innovative assets, developed from the human placenta. This partnership would provide Cynata with the opportunity to expand its target disease areas further into the broad range of degenerative and immunological diseases that Celularity's portfolio targets.

CYP Engineered MSC study yields promising results

The first stage of an ongoing study has shown that Cynata's Cymerus MSCs can be successfully engineered to express diagnostic and therapeutic proteins, using unique expression promoters developed by Dr Khalid Shah, Director of the Center for Stem Cell Therapeutics and Imaging (CSTI) and Vice Chairman, Department of Neurosurgery, Brigham and Women's Hospital, Harvard Medical School. Moreover, this expression is stable during continued culture (growth) of the modified MSCs in the lab, and the modified MSCs persist in vivo for a sufficient duration to facilitate a therapeutic effect.

The results not only provide the potential for Cymerus MSCs to be used in oncology treatments, but the cellular engineering opens up additional applications for the Cymerus platform in cell manufacturing.

FDA Grants CYP-001 Orphan Drug Designation

The United States Food and Drug Administration (FDA) has granted Cynata Orphan Drug Designation for its CYP-001, used for the treatment of GvHD. Receipt of this certification means CYP-001 is eligible for important incentives, including an extended period of marketing exclusivity (up to seven years), tax credits and FDA fee waivers. It also better positions the treatment for cost-effective commercialisation in the US.

Outlook

Cynata has already achieved a significant number of milestones during the first quarter of CY2018, and in accordance with outcomes thus far, the outlook for the remainder of 2018 is positive. The encouraging efficacy and safety data from the DSMB review of Cohort A provides strong confidence in the drug's potential. Recruitment of Cohort B is now well underway, and it is expected that enrolment will be completed soon.

Cynata has a diversified product pipeline, with pre-clinical results from studies in heart attack and ARDS (Acute Respiratory Distress Syndrome) expected in the coming months. Positive results from its two preclinical studies into asthma provide multiple opportunities to secure further partnerships, licencing agreements and clinical trials.

The Company is well positioned going forward, with its existing licence option agreement with FUJIFILM and the recent MoU with Celularity. The completion of the GvHD trial will trigger the 90-day expiry period in which FUJIFILM must exercise its option if it has not already done so. The licence option agreement is worth an upfront licencing fee of US\$3 million with over A\$60 million in milestone payments.

-ENDS-

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About Graft-versus-host-disease

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 percent 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%.

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 have subsequently been diagnosed with steroid-resistant Grade II-IV GvHD. The first eight participants were enrolled in Cohort A and received two infusions of CYP-001 at a dose of one million cells per kilogram of body weight (cells/kg), up to a maximum dose of 100 million cells. There was one week between the two CYP-001 infusions in each participant. The next eight participants will be enrolled into Cohort B and receive two infusions of CYP-001 at a dose of two million cells/kg, up to a maximum dose of 200 million cells. The trial's primary objective is to assess the safety and tolerability of CYP-001, 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 concludes 100 days after the first dose in each participant. Efficacy is 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 enter a longer-term, non-interventional follow-up period, which will continue for up to two years after the initial dose



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.

Cynata Therapeutics Limited

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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")

31 March 2018

Consolidated statement of cash flows	Current quarter	Year to date
	\$A'000	(9 months)
		\$A'000
1. Cash flows from operating activities		
1.1 Receipts from customers	-	-
1.2 Payments for		
(a) research and development	(826)	(2,755)
(b) product manufacturing and operating costs	-	-
(c) advertising and marketing	(131)	(401)
(d) leased assets	-	-
(e) staff costs	(108)	(363)
(f) administration and corporate costs	(161)	(802)
1.3 Dividends received (see note 3)	-	-
1.4 Interest received	39	124
1.5 Interest and other costs of finance paid	-	-
1.6 Income taxes paid	-	-
1.7 Government grants and tax incentives		
- Export Market Development Grant	-	46
- 2017 R&D Tax Incentive	-	1,329
1.8 Other (provide details if material)	-	-
1.9 Net cash from / (used in) operating activities	(1,187)	(2,822)

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

Consolidated statement of cash flows	Current quarter \$A'000	Year to date (9 months) \$A'000
(c) investments	-	-
(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	469	469
3.4 Transaction costs related to issues of shares, convertible notes or options	(4)	(4)
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 (<i>Shares yet to be issued following exercise of unquoted options. Shares were issued subsequent to end of the quarter</i>)	150	150
3.10 Net cash from / (used in) financing activities	615	615

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	8,839	10,350
4.2 Net cash from / (used in) operating activities (item 1.9 above)	(1,187)	(2,822)
4.3 Net cash from / (used in) investing activities (item 2.6 above)	-	-

Consolidated statement of cash flows		Current quarter	Year to date (9 months)
		\$A'000	\$A'000
4.4	Net cash from / (used in) financing activities (item 3.10 above)	615	615
4.5	Effect of movement in exchange rates on cash held	1	125
4.6	Cash and cash equivalents at end of quarter	8,268	8,268

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	3,768	3,339
5.2	Call deposits	4,500	5,500
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)	8,268	8,839

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

Current quarter
\$A'000

239

-

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

Current quarter
\$A'000

-

-

-

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	941
9.2 Product manufacturing and operating costs	-
9.3 Advertising and marketing	126
9.4 Leased assets	-
9.5 Staff costs	109
9.6 Administration and corporate costs	173
9.7 Other (provide details if material)	-
9.8 Total estimated cash outflows	1,349

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:


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Managing Director/CEO

Date: 27 April 2018

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.