



A Next Generation Stem Cell Company

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Cynata Therapeutics Limited
AusBiotech Asian Investment Series, March 2018

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Investment overview

1

Unique, patented technology to efficiently mass-produce mesenchymal stem cells (MSCs), a highly promising type of therapeutic stem cell

2

World first clinical trial, with clear early efficacy and safety data received from 1st patient cohort & providing a springboard for further clinical studies

3

Large, active and growing market, with over 650 trials investigating the efficacy of MSCs in treating diseases including osteoarthritis, stroke & cardiovascular disease

4

Monetisation of the business model has already commenced, with Fujifilm license option and Cynata's largest shareholder with 9%

5

Strong balance sheet with cash runway into 2019 based on current projections

Corporate overview: A biotech company with a world-first clinical trial and leading technology platform

Company profile

- Cynata Therapeutics is an Australian stock exchange listed clinical-stage biotechnology company developing disruptive regenerative medicines. Cynata shows strong potential for 2018, with a strategic partnership and license option agreement in place with Fujifilm

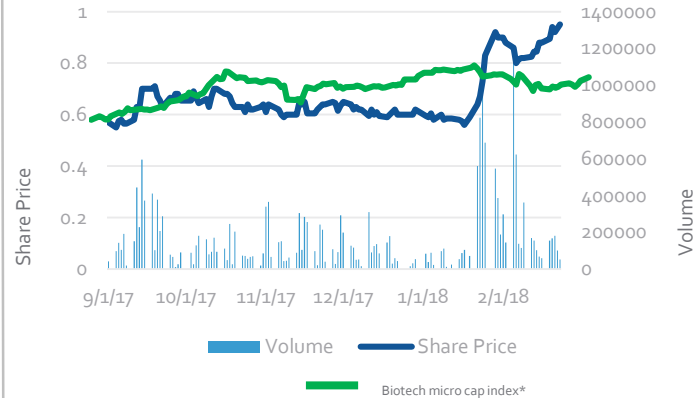
Financial information

Share price (8 March 18)	A\$1.23
52 week low / high	A\$1.35 / A\$0.37
Shares on issue ¹	90.3m
Market capitalisation	A\$111m
Cash (as at 31-Dec-17)	A\$8.8m
December quarter expected cash burn	~\$2.1m
Debt (as at 30-Jun-17)	-
Enterprise value	A\$102m

Source: IRESS

Notes: Excludes 10.4m unquoted options with exercise prices ranging from \$0.40 to \$1.022 and expiry dates between 27-Sep-2018 and 4-Aug-2020, and 750k unlisted incentive options with exercise price \$0.49 and expiring 16 December 2018 (500k subject to vesting conditions)

6 month share price performance



Top Shareholders

Shareholder	%
Fujifilm corporation	8.98
Board and Management	8.12%
Number of shareholders	~2300

Notes: ASX listed peers incorporates the average share price movements of MSB, RGS, CTE, OCC, LCT over the last 6 months

Why MSCs?

What are MSCs?

- Mesenchymal stem cells (MSCs) are highly potent adult stem cells found in bone marrow and certain other tissues.

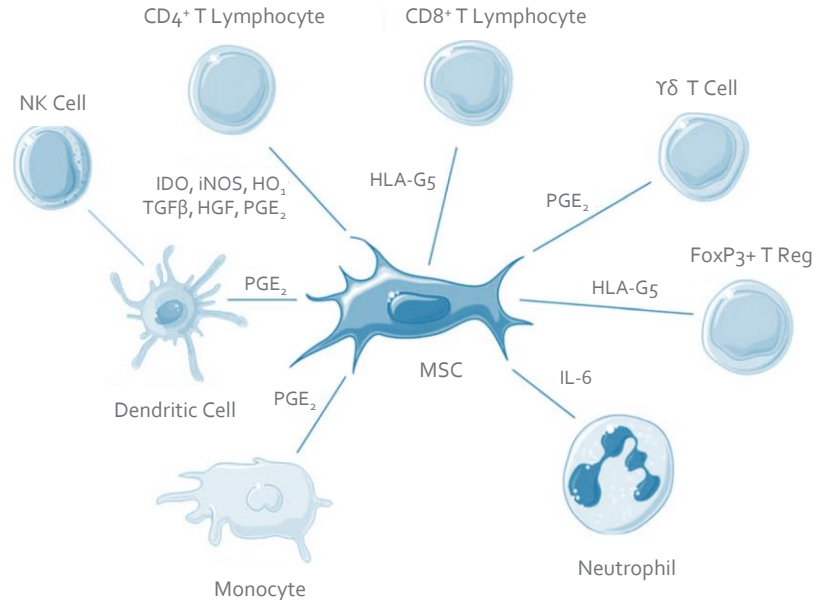
What do they do?

- They have the ability to self renew.
- They secrete bioactive molecules and have immunosuppressive and immunoregulatory properties – giving them enormous therapeutic potential.

How much commercial interest is there?

Over 650 clinical trials investigating the efficacy of MSCs in treating diseases have been initiated.¹


Promising results have been shown in conditions such as heart attack, stroke, GvHD, Crohn's disease, multiple sclerosis, osteoarthritis and diabetes complications



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Excessive culture expansion: “the most egregious divergence between [commercial and academic MSC products] is the scale of product expansion”

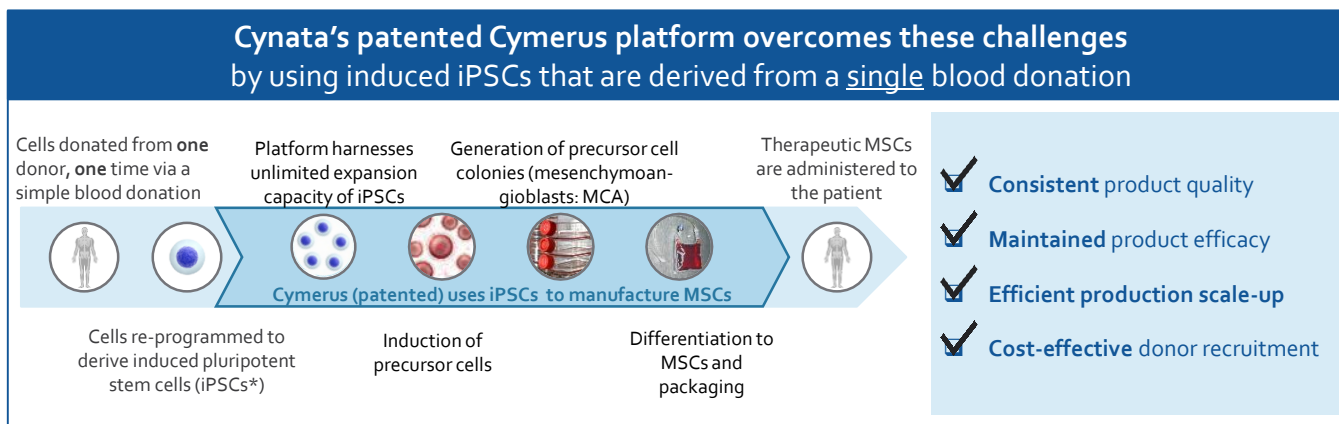
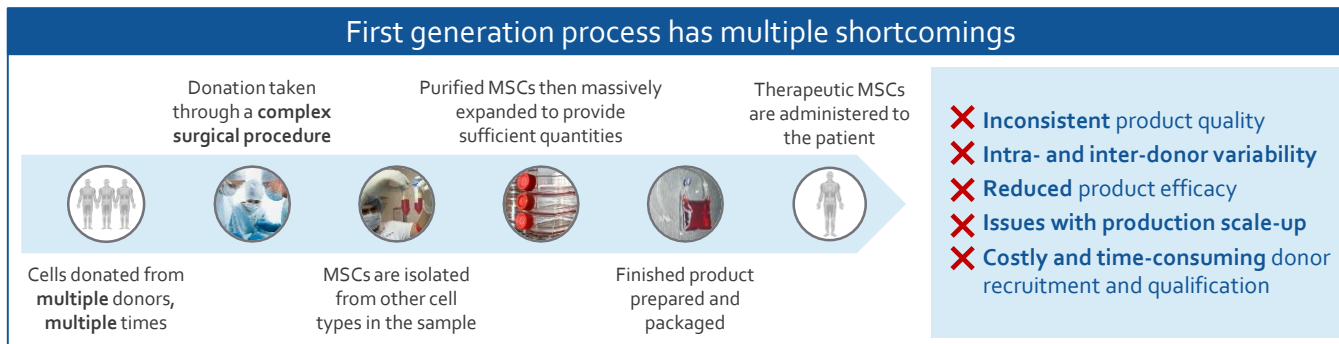
Cytotherapy, 2013; 15: 2–8

International Society for Cellular Therapy
ISCT 

The mesenchymal stromal cells dilemma—does a negative phase III trial of random donor mesenchymal stromal cells in steroid-resistant graft-versus-host disease represent a death knell or a bump in the road?

JACQUES GALIPEAU

Only company in the world with a platform to mass-produce MSCs without multiple donors



*iPSCs are derived from e.g. blood cells and have been reprogrammed back into an embryonic-like state that enables the development of an unlimited source of virtually any type of human cell."

Cymerus Clinical Trial

Clinical trial now underway to investigate the safety and clinical efficacy of Cynata's Cymerus MSCs



Clinical Trial Protocol: CYP-GvHD-P1-01

- World's first allogeneic iPSC-derived cell therapy clinical trial
- Population: ~16 adults with steroid-resistant acute GvHD
- Clinical sites: 7 (UK and Australia)
- Endpoints:
 - Safety and tolerability (primary)
 - Complete/Partial Response by Day 28/Day 100
 - Complete response = absence of GvHD
 - Partial response = improvement by at least 1 grade
 - Overall survival at Day 28/Day 100
- Current status:
 - 1st cohort (8 patients) completed Nov 2017; 2nd cohort underway
 - Expect completion in 2Q18

GvHD Clinical Trial Cohort A Data



NO treatment related adverse effects



Overall Response rate: 100% (all eight participants showed an improvement in the severity of GvHD by at least one grade compared to baseline)



Overall Survival: 87.5% (one patient died of pneumonia, unrelated to treatment)








Complete Response rate: 50% (GvHD signs/symptoms completely resolved)

Conclusion: CYP-001 demonstrates highly promising clinical activity in GvHD; provides confidence for broader applications

Note: *prognosis for these patients is poor, with mortality rates in excess of 90 percent*³.

Indications Currently Being Targeted

Disease target area	External collaborations		Vigorous partner engagement		Further revenues	Key highlights
	Pre-clinical trials started	Proof of concept completed	Deal secured	Clinical trial started	Product in-market	
Graft vs Host Disease (vHD) Massachusetts 	✓	✓	✓	✓		<ul style="list-style-type: none"> Pre-clinical research with University of Massachusetts show Cymerus MSCs to be highly effective in GvHD Half a billion dollar market by 2021
Asthma University 	✓	✓				<ul style="list-style-type: none"> Cymerus MSCs demonstrated significant beneficial effects on three key components of asthma: airway hyper-responsiveness, inflammation and airway remodeling.
Critical care research group ARDS						
University of Sydney 	✓					<ul style="list-style-type: none"> Study to commence to evaluate effectiveness of Cymerus MSCs in sheep with ARDS in association with the Prince Charles Hospital in Brisbane.
Heart attack Harvard/BWH 	✓					<ul style="list-style-type: none"> Pre-clinical trials suggest Cymerus MSCs may have the potential to restore cardiac function and reduce scar size after a heart attack (US\$18.2 billion market by 2019¹)
Brain Cancer / Glioblastoma University of Wisconsin-Madison 	✓					<ul style="list-style-type: none"> Research collaboration in genetically modified MSCs in cancer: involves modifying stem cells to target cancer
Critical Limb Ischemia	✓	✓				<ul style="list-style-type: none"> Pre-clinical study published in peer reviewed journal Cytotherapy, The Journal of Cell Therapy. Study found treatment with MSCs demonstrate beneficial impact on CLI.

Note: ARDS – Acute Respiratory Distress Syndrome

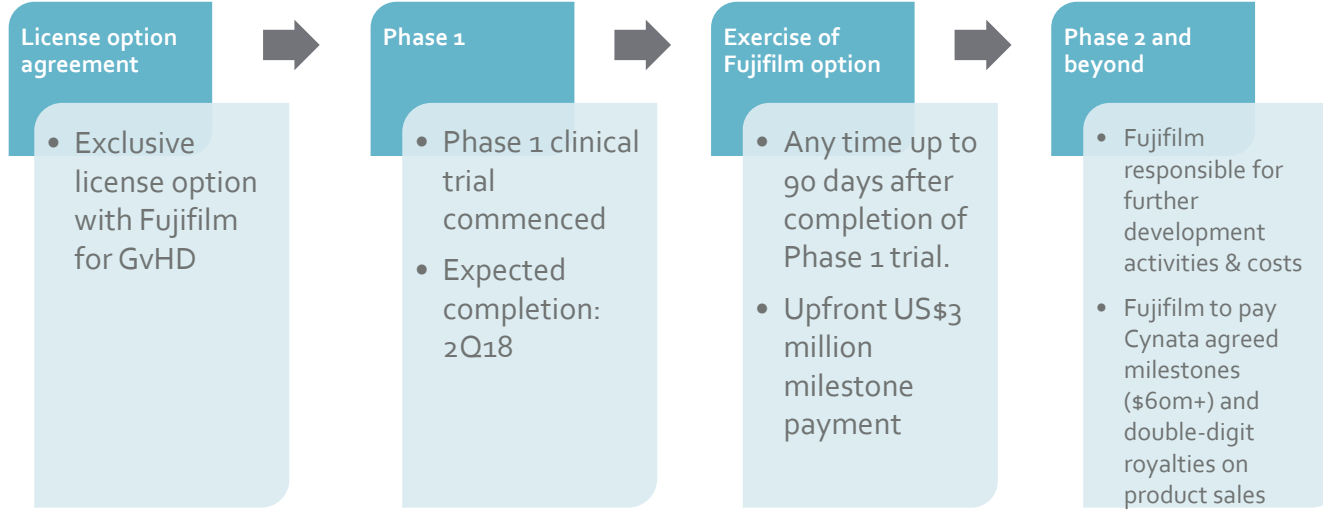
Source: 1. <http://gbi-research.com/media-center/press-releases/cardiovascular-disease-market-us-to-lead-modest-growth-forecasts-gbi-research>.

Successful outcomes open many other disease targets potentially benefiting from MSC treatment

Fujifilm has demonstrated confidence in Cynata's platform through a licensing agreement for GvHD



License option agreement for further development and commercialisation of Cynata's MSCs for GvHD



Board and management overview



Dr Paul Wotton
Chairman

- Former CEO of Ocata Therapeutics (NASDAQ: OCAT) managing it through a take-over by Astellas Pharma, in a US\$379m transaction
- Previous executive roles with Antares Pharma Inc. (NASDAQ: ATRS), Topigen Pharmaceuticals and SkyePharma
- Founding CEO, Sigilon Therapeutics; member of the boards of Vericel Corporation and Veloxis; past Chairman of the Emerging Companies Advisory Board of BIOTEC Canada

Expertise running and monetising Ocata Therapeutics, acquired by Astellas



Dr Ross Macdonald
Managing Director
Chief Executive
Officer

- 30 years' experience and a track record of success in pharmaceutical and biotechnology businesses
- Previous senior management positions with Hatchtech, Sinclair Pharmaceuticals, Connetics Corporation (Palo Alto, CA), and Stiefel Laboratories, the largest independent dermatology company in the world and acquired by GSK in 2009 for £2.25b

Track record of success in pharmaceutical and biotechnology businesses



Dr Stewart Washer
Non-Executive
Director

- 20+ years of CEO and Board experience in medical technology, biotech and agrifood companies
- Chairman of Orthocell Ltd and Minomic International.
- Previously CEO roles with Calzada (ASX:CZD), Phylogica (ASX:PYC) and Celentis and managed the commercialisation of intellectual property from AgResearch in New Zealand with 650 Scientists and \$130m revenues

Deep experience growing companies as CEO and on the Board



Dr John Chiplin
Non-Executive
Director

- Significant international experience in the life science and technology industries
- Recent transactions include US stem cell company Medistem (acquired by Intrexon), Arana (acquired by Cephalon), and Domantis (acquired by GSK)
- Was head of the \$300M ITI Life Sciences investment fund in the UK and his own investment vehicle, Newstar Ventures.

Overseen and managed a broad range of life sciences transactions



Mr Peter Webse
Non-Executive Director
Company Secretary

- +25 years' company secretarial experience
- Managing Director of Platinum Corporate Secretariat Pty Ltd, a company specialising in providing company secretarial, corporate governance and corporate advisory services

25+ years company secretarial and management experience



Dr Kilian Kelly
Vice President,
Product
Development

- 15 years' experience in pharmaceutical/ biotechnology research and development, in both commercial and academic settings
- Previous appointments include Senior Director, Drug Development at Biota Pharmaceuticals (NASDAQ: BOTA), Vice President, Regulatory and Clinical at Mesoblast Limited (ASX:MSB)

Academic and commercial excellence, extensive relevant management experience

Investment Summary

1

Unique technology to efficiently mass-produce mesenchymal stem cells (MSCs), a highly promising type of therapeutic stem cell

2

World first clinical trial in progress with clear early efficacy and safety data received from first patient cohort

3

Large, active and growing market, with over 650 trials investigating the efficacy of MSCs in treating medically and commercially important diseases

4

Scalable business model intended to target a **broad range of disease target areas** over time, and monetise these through licensing & partnerships

5

Cynata's initial target area is GvHD, intended to prove the quality of the MSC's produced by its patented Cymerus™ platform

6

Monetisation of the business model has already commenced, as license options have been entered with Fujifilm, who are Cynata's largest shareholder with 9%

7

Strong balance sheet with cash runway into 2019 based on current projections

Thank you for your attention

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