A Next Generation Stem Cell Company

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Cynata Therapeutics Overview

- **Australian Securities Exchange (ASX) listed** biotech company developing a novel therapeutic stem cell (MSC) technology: Cymerus™
- **Technology from** University of Wisconsin - Madison: "the home of stem cells"
- **World-first Phase I clinical trial commenced** in GvHD; sites in UK and Australia
- **Strategic partnership with Fujifilm Corporation**, leading Japanese regenerative medicine company
- **License option agreement with apceth GmbH & Co. KG** for several disease target areas
- **Strong balance sheet:** cash runway into 2019 based on current projections
- **Compelling preclinical data** from a range of animal proof-of-concept studies
- **Favorable regulatory environment** with Japan, US and EU fast tracking stem cell therapies
- **Broad commercial potential** in a range of diseases including stroke, heart disease and osteoarthritis

Cynata Key Facts

Cynata Therapeutics is an Australian clinical-stage biotechnology company developing disruptive regenerative medicines.

To build shareholder value through a commitment to commercialising and bringing to patients its proprietary Cymerus™ therapeutic stem cell technology.

ASX code	CYP
Commenced operations	November 2013
Market cap	A$ ~50m
Shares on issue	90m
Cash	$ 11.6m as at 31 March 2017 ($10m raised in Jan 2017 via placement and Fujifilm strategic partnership)
Number of shareholders	~2300; FUJIFILM ~9%

Dr Paul Wotton – Chairman

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

Dr Ross Macdonald – Managing Director and 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.

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.

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.

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.

Our Story

Cymerus™ MSC platform technology developed at Wisconsin Alumni Research Foundation, a technology transfer organisation serving the University of Wisconsin–Madison

WARF

Cymerus platform successfully validated as a GMP manufacturing process

Positive pre-clinical research in Graft vs. Host Disease, Asthma, Heart Attack and Brain Cancer paving the way for clinical trials

License option agreement with apceth

apceth BIOPHARMA

Approval for Phase I clinical trials in the UK and Australia for GvHD

Strategic partnership and $4m investment from FUJIFILM

FUJIFILM

Successful evaluation of Cymerus platform by apceth

NOW…
Patient dosing commenced in GvHD clinical trial….

WORLD FIRST

NEXT…
Exercise of license option agreement with FUJIFILM with US$3m fee PLUS ~A$60m in milestones PLUS double digit royalties thereafter

The Market

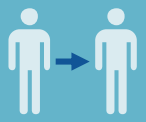
Global regenerative medicine market was worth $18.9 billion in 2016 and will grow to over $53.7 billion by 2021[1]

Stem cells are the cornerstone of contemporary regenerative medicine applications[2]

Sources: 1. Research and Markets - Global Regenerative Medicine Market Analysis & Forecast. 2. Orkin SH, Zon LI. Hematopoiesis: an evolving paradigm for stem cell biology. Cell. 2008

Disease Target Areas

Mesenchymal stem cells (MSCs) have broad therapeutic potential – Cynata is presently focussing on several exciting opportunities:



Graft v Host Disease (GvHD) – a common complication that can occur after bone marrow or organ transplants. A **half a billion dollar market** by 2021.

Cardiovascular disease (Heart Failure, Heart Attack and Acute Coronary Syndrome ACS) - The global market for Cardiovascular Disease (CVD) is expected to grow to **US$18.2 billion by 2019**[1]

Pulmonary diseases - Pulmonary fibrosis/scarring of the lungs expected to be US$3.2b by 2025[2] and asthma that affects 1 in every 12 people reaching **U$25b by 2024**[3]

Brain Cancer / Glioblastoma (engineered MSCs) – In 2012, 14 million new cases of cancer and about 8.2 million deaths were reported[5]. The market is estimated to be worth **US$773.1 million by 2025**[4]

Source: 1. GBI Research. 2. GlobalData 3. GrandViewResearch 4. GrandViewResearch 5. WHO

GvHD – A Growing Market

- Graft-versus-host disease (GvHD) occurs after a bone marrow or stem cell transplant from a donor
- This is an allogeneic transplant vs. an autologous transplant (when a patient receives their own stem cells)
- The transplanted cells regard the recipient's body as foreign and reject and attack the recipient's body
- There has been a large increase in prevalence and severity over the past two decades due to medical advances in stem cell therapies

1 million

Stem cell transplants worldwide [3]

25 million

International Marrow Donor Registries and Potential Donors [4]

70%

GvHD occurs in up to 70 per cent of patients receiving stem cell transplant to treat blood cancer[1]

$0.51bn

market value for the treatment of GvHD[2] by 2021

FUJIFILM's projections for the GvHD market show peak revenues of US$300m p.a. which would result in >US$30m per year in royalties for Cynata

Sources: 1. QIMR Berghofer Medical Research Institute 2. Vision Gain 3. Leukaemia Foundation 4. Bone Marrow Donors Worldwide (BMDW) and the World Marrow Donor Association (WMDA)

About Stem Cells and the Platform

How the Cymerus MSC platform works and overcomes the inherent challenges facing MSC therapies today.

Sources: 1. Research and Markets -Global Regenerative Medicine Market Analysis & Forecast

Why MSCs?

What are MSCs?

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

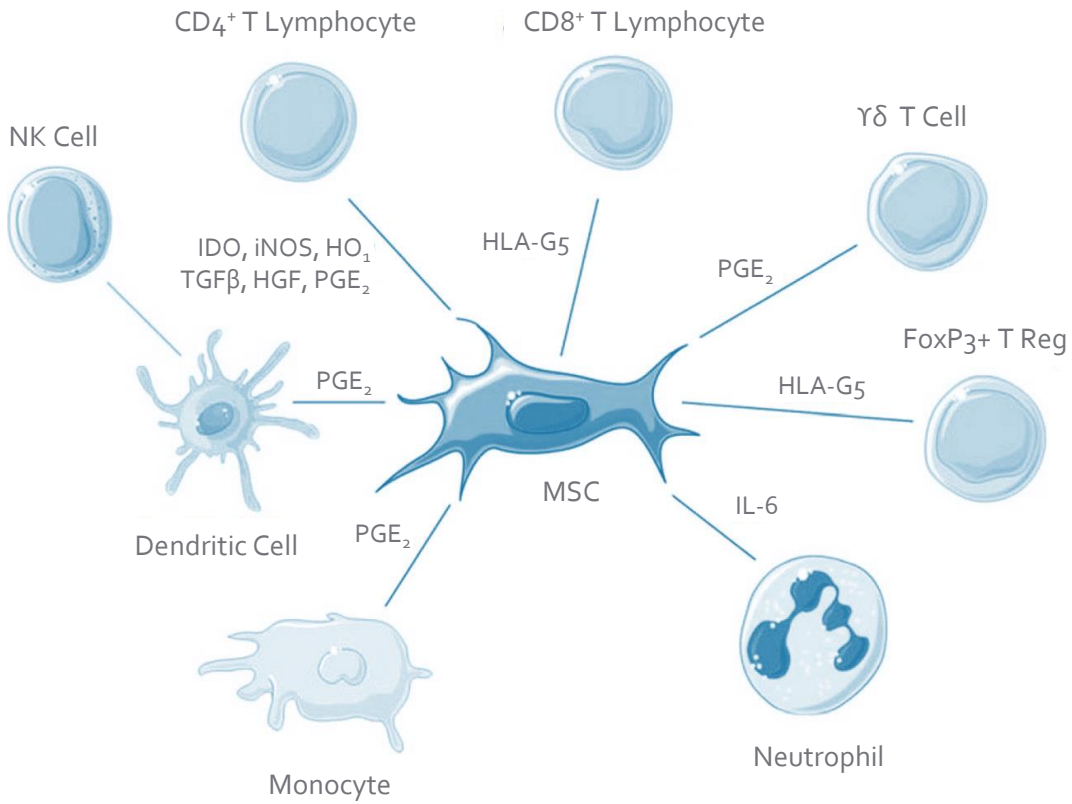
What do they do?

- 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

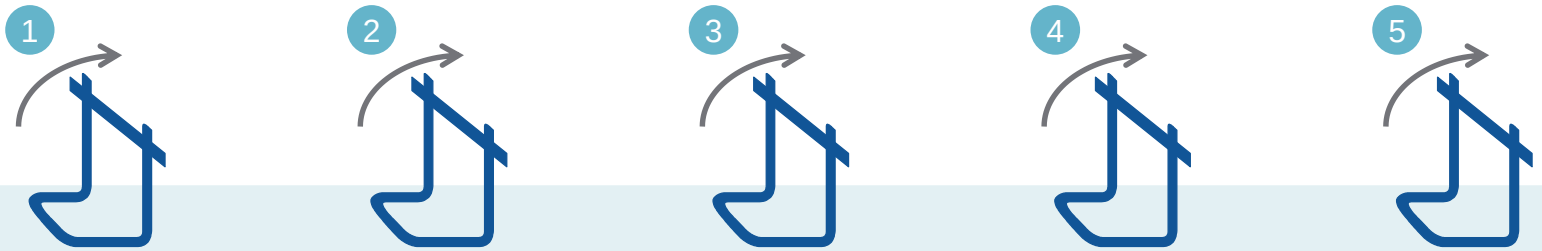


Source: 1. www.clinicaltrials.gov

How Are MSCs Manufactured?

First generation methods require many tissue donors and massive cell expansion (i.e., multiply) to manufacture sufficient product.

First generation methods pose a number of key challenges for the manufacture of MSC medicines….



1	2	3	4	5
Issues with production scale-up	Inconsistent product quality	Reduced product efficacy	Significant intra- and inter- donor variability	Recruitment and qualification of donors is costly and time consuming

Cynata's Cymerus platform overcomes each of these challenges
by using induced pluripotent stem cells (iPSCs) that are more easily derived from a single blood donation

Cynata's patented process uses iPSCs to manufacture MSCs

Source: 1. www.clinicaltrials.gov

Cymerus Platform vs First Generation Process

Cynata's Cymerus platform enables MSCs to be manufactured effectively and efficiently by eliminating the need to use multiple donors, multiple times.

First generation process for sourcing and manufacturing therapeutic MSCs

Cells donated from **multiple** donors, **multiple** times

Donation taken through a complex surgical procedure

MSCs are isolated from other cell types in the sample

Purified MSCs are then massively expanded to provide sufficient quantities

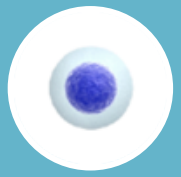
Finished product prepared and packaged

Therapeutic MSCs are administered to the patient

Cynata process for sourcing and manufacturing therapeutic MSCs

Cells donated from **one** donor, **one** time via a simple blood donation

Cells are re-programmed to derive induced pluripotent stem cells (iPSCs*)



Patented process uses iPSCs to manufacture MSCs

Cymerus™

Therapeutic MSCs are administered to the patient

Cymerus platform harnesses unlimited expansion capacity of iPSCs

Induction of precursor cells

Generation of precursor cell colonies (mesenchymoan-gioblasts) (MCA)

Differentiation to MSCs and packaging

*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."

Development Progress and Validation

Phase I Clinical trial in GvHD: patient dosing commenced.

Strategic partnership and license option agreement with FUJIFILM Corporation.

Sources: 1. Research and Markets -Global Regenerative Medicine Market Analysis & Forecast

Development Progress

	Pre-Clinical	Phase 1	Phase 2	Phase 3	Evidence
GvHD	University of Massachusetts / UMassAmherst The Commonwealth's Flagship Campus	Patient dosing commenced			Pre-clinical research with University of Massachusetts shown Cymerus™ MSCs to be highly effective in GvHD: CYP-001 treatment substantially prolonged survival in an animal model
Asthma	Monash University / MONASH University				Cymerus™ MSCs demonstrated significant beneficial effects on three key components of asthma: airway hyper-responsiveness, inflammation and airway remodeling.
Heart Attack	University of Sydney / THE UNIVERSITY OF SYDNEY				Preliminary results from pre-clinical trials suggests that Cymerus™ iPSC-generated MSCs may have the potential to restore cardiac function and reduce scar size after a heart attack.
Cancer / Glioblastoma	Harvard/ MGH / HARVARD				Research collaboration in genetically modified MSCs in cancer: involves modifying stem cells to target cancer

World firsts:

Scalable manufacture of MSCs without reliance upon multiple donors

First clinical trial of an allogeneic, iPSC-derived MSC product

Next Steps with Fujifilm

FUJIFILM

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

License option agreement

- Exclusive license option with Fujifilm for GvHD

Phase 1

- Phase 1 clinical trial commenced
- Expected completion: end 2017

Exercise of Fujifilm option

- Any time up to 90 days after completion of Phase 1 trial.
- Upfront US$3 million milestone payment

Phase 2 and beyond

- Fujifilm responsible for further development activities and costs
- Fujifilm to pay Cynata agreed milestones ($60m+) and double-digit royalties on product sales

Business Model

External collaborations: preclinical PoC development of potential products for target diseases

- ✓ GvHD/transplantation
- ✓ Asthma/respiratory disease
- ✓ Heart Attack
- ✓ Cancer/Glioblastoma

Vigorous partner engagement to produce upfront payments: option/license agreements with pharma and biotech partners for clinical development (Phase 1, 2 & 3), registration and sale

FUJIFILM

- ✓ GvHD option license agreement with Fujifilm – Phase I trial now recruiting patients

apceth applied cell therapy

- ✓ Successful trial of Cymerus platform with apceth and license option agreement in place

Further revenues through milestone payments plus royalties on marketed products

Early Revenue Streams

Upfront Option/License payments
From pharma/biotech for licensing of Cymerus™ platform

Milestone payments
From partners as products progress through clinical trials and approval

Royalties
From partner revenue of marketed products

Validation and Outlook

- **Positive results from pre-clinical trials** in the treatment of GvHD, asthma, heart attack and limb ischaemia – further pre-clinical research in cancer and Acute Respiratory Distress Syndrome (ARDS)
- **Approval from the UK and Australia: Phase I clinical trial commenced** in GvHD (world first); safety data base will facilitate further disease targets
- **$4 million strategic investment from Fujifilm Corporation**, leading Japanese regenerative medicine company
- **License option agreement with Fujifilm** for GvHD to be exercised any time up until 90 days after the trial completion – worth up to $60m in license payments plus royalties
- **Successful evaluation of the Cymerus platform in apceth's systems** demonstrating ability to integrate Cymerus with other technologies giving a broader cell therapy applications for the platform
- **License option agreement with apceth** for several disease target areas
- **Favourable regulatory environment** with Japan, US and EU accelerating legislative changes to accelerate stem cell therapy research and uses

Market Activity and Investment Summary

Regenerative medicine and stem cell market highly active with a flurry of M&A and investment in recent years.

Sources: 1. Research and Markets -Global Regenerative Medicine Market Analysis & Forecast

Market Activity

Cord Blood Registry
Acquired by AMAG Pharmaceuticals Inc
2015

USD 700M

Ocata Therapeutics
(Acquired by Astellas
2016

USD 379M

Cellular Dynamics
Acquired by Fujifilm
2015

USD 307M

Bayer and Versant Ventures launched stem cell therapy company BlueRock Therapeutics
2016

USD 225M (SERIES A)

CiRA & Takeda partner in iPS Collaboration
2015

USD 267M

Novoheart Holdings
Woodrose Ventures to acquire
2017

USD 20M

A significant number of licence agreements have also been secured over recent years

Investment Summary

- **Only company** in the world with technology for mass-production of therapeutic MSCs of consistent quality and without reliance on multiple donors
- Cynata's Cymerus™ technology **overcomes the challenges inherent in first generation production** methods by industrialising the production of MSCs
- **Compelling data in pre-clinical studies** for the treatment of asthma, CLI, heart attack and GvHD
- **Regenerative medicine market expected to grow to US$170bn**[1] by 2020 and an active investment area for pharmaceutical companies, including Astellas, J&J and Fujifilm
- **License-driven business model** with license option agreements in place with Fujifilm and apceth producing early revenues
- **Experienced management** team
- **Strong** academic partnerships
- **Value-accretive news flow** expected in near term

Source: 1. Grand View Research Report published Sept 2015 http://www.grandviewresearch.com/industry-analysis/stem-cells-market

Thank you for your attention

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