A Next Generation Stem Cell Company

Cynata Therapeutics Limited (ASX:CYP) AGM – 2017

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A Year in Review

Key Milestones

Strategic Partnership and $4m Placement with FUJIFILM	**$6m Placement to institutional investors**	**Commenced world first clinical trial for GvHD: concept to clinic in <4 years**	**License option agreement with FUJIFILM for lead candidate product to treat GvHD**	**Strong GvHD pre-clinical data and progress across other indications**	**Additional target indications added to development pipeline**

Outperforming ASX listed microcap biotech stock index

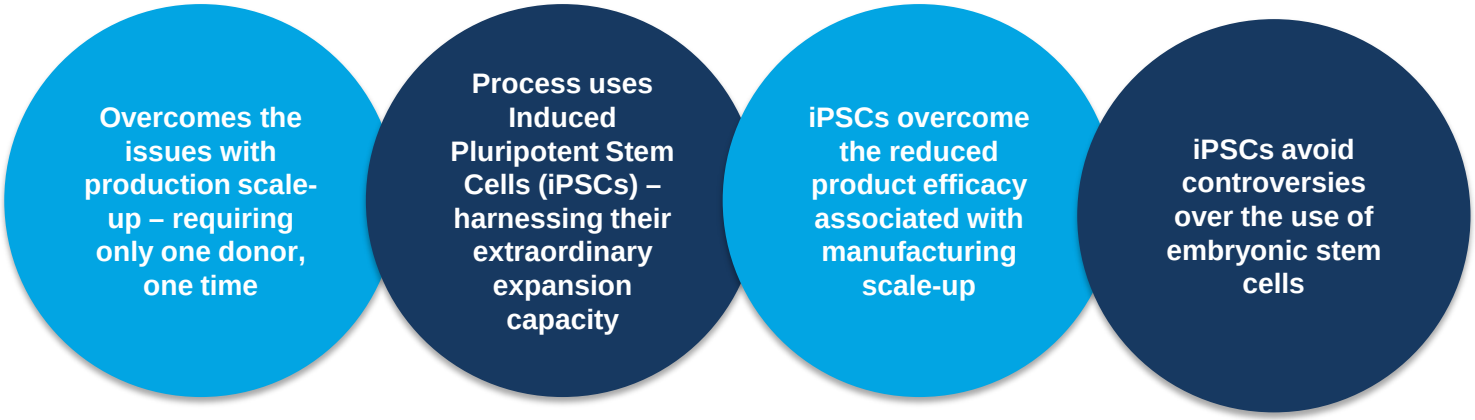
Custom index of 32 ASX biotech companies with an average market cap of $45m. Chart from Bloomberg.

ASX code	CYP
Commenced operations	November 2013
Market cap	A$ ~55m
Shares on issue	90m
Cash	A$8.7m as at 30 September 2017
Number of shareholders	~2300; FUJIFILM ~9%

Our Technology – Revolutionising Stem Cell Therapy

Cynata's proprietary Cymerus™ technology facilitates commercial-scale manufacture of a consistent, robust and premier grade therapeutic mesenchymal stem cell (MSC) product

The Cymerus platform uses induced pluripotent stem cells (iPSCs) that are derived from blood cells and have been reprogrammed back into an embryonic-like state enabling the development of an unlimited source of virtually any type of human cell.



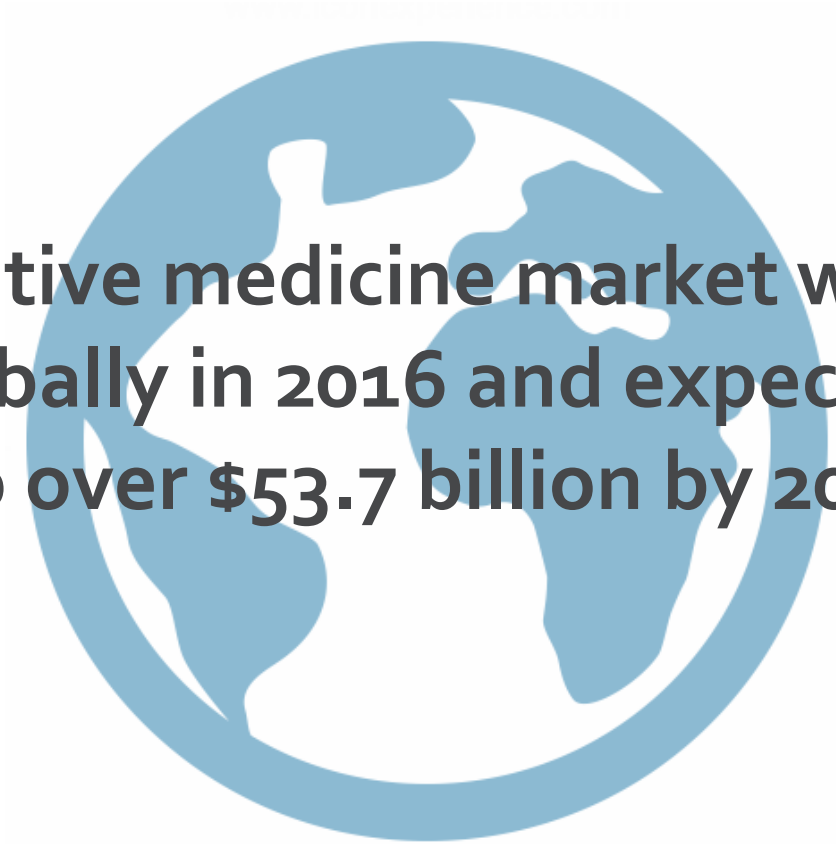
Why is stem cell therapy important and why do we use MSCs?

1. MSCs are specialised stem cells that may be used as therapeutics
2. Potential to regenerate and repair damaged tissue
3. MSCs play a key role in modulating inflammation and co-ordinating repair
4. immunosuppressive and immunoregulatory properties – giving them enormous therapeutic potential
5. ~650* clinical trials investigating the efficacy of MSCs

Multiple Sclerosis Macular Degeneration
Asthma Arthritis Spinal Cord Injury
Tissue Repair GvHD Bone and Cartilage Repair
Stroke Heart Disease Cancer Parkinson's Disease
Motor Neuron Disease Diabetes

*Clinicaltrials.gov.au

Regenerative Medicine is a Rapidly Expanding Sector and Cynata is Positioned at the Forefront



Regenerative medicine market worth $18.9 billion globally in 2016 and expected to grow to over $53.7 billion by 2021[1]

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

GvHD – Represents a Significant and Growing Market

- GvHD is a potentially fatal complication that can occur after a bone marrow transplant when the donor's immune cells attack the host (patient).
- Transplant market is expected to be worth +USD50 Billion by 2025[1]
- +120,000 transplants per year globally and growing[2]
- 70% of patients receiving a bone marrow transplant to treat blood cancer contract GvHD[3]
- GvHD is estimated to be a half a billion dollar market by 2021[4]
- Successful outcome will open many doors for Cynata to more economically important targets

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

1. Grand View Research - Transplantation Market Size http://www.grandviewresearch.com/press-release/global-transplantation-market
2. Global Observatory on Donation and Transplantation - http://www.transplant-observatory.org/
3. QIMR Berghofer Medical Research Institute - http://www.qimrberghofer.edu.au
4. Vision Gain - Global Graft Versus Host Disease (Gvhd) Market 2017-2027 - https://www.visiongain.com/Report/1794/Global-Graft-versus-Host-Disease-(GVHD)-Market-2017-2027

World First Clinical Trial Underway

- World-first Phase I clinical trial commenced in graft-versus-host disease (GvHD)
- Global industry focus on Cynata's progress
- 7 sites now recruiting patients in the UK and Australia at major cancer treatment centers
- 8 patients now dosed thereby completing enrolment of Cohort A
- Data Safety Monitoring Board will assess safety and tolerability after day 28 and before second stage (Cohort B) commences

Diverse Development Pipeline

MSCs have broad therapeutic potential and Cynata is focussing on several exciting opportunities

	Pre-Clinical	Phase 1	Evidence
GvHD	University of Massachusetts UMassAmherst		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. Clinical trial commenced in May 2017. A total of 16 patients to be enrolled to complete the trial.
Asthma	Monash University MONASH University		Cymerus MSCs demonstrated significant beneficial effects on three key components of asthma: airway hyper-responsiveness, inflammation and airway remodeling. Second study demonstrated greater reduction of hyper-responsiveness when compared to corticosteroid treatment. Study published in the FASEB Journal, one of the world's most cited peer-reviewed biology journals
Acute Respiratory Distress Syndrome (ARDS)	Critical Care Research Group Critical Care RESEARCH GROUP		Study to commence to evaluate the effectiveness of Cymerus MSCs in sheep with Acute respiratory distress syndrome (ARDS) in association with the *Prince Charles Hospital* in Brisbane.
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/ BWH		Research program in genetically modified MSCs in cancer. The collaboration involves modifying stem cells to target cancer.
Critical Limb Ischemia (CLI)	University of Wisconsin-Madison WISCONSIN		Pre-clinical study published in peer reviewed journal Cytotherapy, The Journal of Cell Therapy. Study found treatment with MSCs demonstrate beneficial impact on CLI.

Other Disease Target Areas – Our Markets

Significant markets with a real opportunity to use regenerative medicine to improve the lives of millions of people globally

Heart Attack – Over 50,000+ Australian's per year suffer a heart attack[1]. Global heart failure market is expected to be worth US$16.1 billion by 2026[2]

Asthma- affects 1 in every 12 people and the market is expected to reach **U$25b by 2024**[3]

Acute Respiratory Distress Syndrome (ARDS) accounts for ~10% of all ICU admissions and has high hospital mortality rates of 35%-46%.[4]

Brain Cancer / Glioblastoma ~250,000 people are diagnosed every year. [5] The leading cause of cancer related deaths in children.

The Critical Limb Ischemia (CLI) market is worth U**SD$12 billion.** Globally. 1.7 million patients suffer from this limb-threatening and life-threatening disease.[6]

Source: 1. The Heart Foundation. 2. GlobalData 3. GrandViewResearch 4. Bellani G, Laffey JG, Pham T, Fan E, Brochard L, Esteban A, et al. Epidemiology, Patterns of Care, and Mortality for Patients With Acute Respiratory Distress Syndrome in Intensive Care Units in 50 Countries. Jama. 2016;315(8):788. 5. World Cancer Report 2014 6. Ray Dirks Research

ARDS Preclinical Study In Progress

The Study

- Evaluating the effectiveness of Cymerus MSCs in sheep with Acute Respiratory Distress Syndrome (ARDS)
- In partnership with the *Critical Care Research Group* in association with the *Prince Charles Hospital* in Brisbane.

About ARDS

- ARDS is an inflammatory disease leading to build-up of fluid in the lungs and respiratory failure
- Existing treatment - ECMO circulates blood through an artificial lung and is used in patients whose lungs are unable to provide an adequate amount of oxygen to the blood..

Next Steps

- Successful results will pave the way towards clinical trials in humans.
- Results expected in Q1 CY2018.

Positive Preclinical Data in Asthma Studies

Study #1	• i.v. and intranasal delivery of Cymerus MSCs in well-established chronic allergic airways disease model of asthma at *Monash University*: successfully completed
Study #2	• Second study in asthma model at *Monash* involving Cymerus MSCs administered alone or in combination with corticosteroids
Study Results	• Cymerus MSCs have a positive impact on all three components of asthma: hyper-responsiveness, inflammation and airway remodelling. • Data published in FASEB Journal, a leading peer-reviewed scientific journal • Ongoing study has reported positive preliminary results with significant superiority of Cymerus MSCs alone versus corticosteroids.
Next Steps	• The compelling data from these studies is paving the way for a potential future clinical trial in asthma patients.

Positive Preclinical Data in Heart Attack

The Study

- Preliminary study of the impact of Cymerus MSCs for heart attack conducted in an experimental rat model at the *Westmead Institute for Medical Research*

Study Results

- Early results show Cynata's MSCs have the potential to restore cardiac function and reduce scar size after a heart attack
- The study involved an assessment of cardiac function and scar size over a 28 period after an induced heart attack in a total of 11 rats.
  - 4 treated with Cymerus MSCs
  - 3 treated with bone-marrow derived MSCs
  - 4 treated with a placebo control

Next Steps

- Studies continuing using larger subject pool and additional assessments to strengthen initial findings and investigate effect of the treatment on ventricular arrhythmia (a potentially fatal abnormal heart rhythm that often develops after a heart attack).
- Results expected in Q1 2018

Positive Preclinical Data in Critical Limb Ischemia

The Study

- Study at University of Wisconsin School of Medicine and Public Health, investigating the potential of Cymerus MSCs to treat critical limb ischemia (CLI) in mice.

About Critical Limb Ischemia

- CLI is a disease caused by poor blood supply and is commonly found in diabetic patients. It is caused by a narrowing or blockage of the arteries. It causes severe pain and disability, and can lead to amputation.

Results and Next Steps

- Mice injected with Cymerus MSCs demonstrated significantly improved blood flow and a substantially diminished impact from the ischemia.
- Study published in the prominent peer-reviewed journal Cytotherapy, The Journal of Cell Therapy
- Consider potential future clinical study

Progress in the US and Canada

- Received advice from the FDA Office of Cell, Tissue and Gene Therapy Products regarding the regulatory approval path for Cynata's CYP-001 product (for the treatment of GvHD) in the US.
  - Cymerus MSC products are expected to be of suitable quality for clinical trial use.
  - 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".
- Health Canada confirmed manufacturing process and testing meets expectations and preclinical expectations consistent with FDA advice.

Licensing Driven Business Model Driving Early Revenue Streams

Focus on developing early revenue streams through:

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 sales of marketed products

FUJIFILM

License option agreement signed with FUJIFILM for the commercialisation of Cynata's MSCs for GvHD

- Exercise any time up to 90 days after completion of Phase 1 trial – expected in 2018
- Upfront US$3 million milestone payment
- Fujifilm to pay Cynata agreed milestones ($60m+) and double-digit royalties on product sales + fund all development.

apceth BIOPHARMA

Successful evaluation of Cymerus platform with apceth GmbH & Co and license option agreement in place; next stage expected during 2018.

Cells as medicines - no longer a futurist therapy

August: FDA approved Novartis' product, Kymriah, a CAR-T cell treatment for leukemia

August: Gilead to acquire Kite Pharma for US$11.9b. Kite develops CAR-T cell products for cancer treatment

October: FDA approved Kite Pharma's product, Yescarta, a CAR-T treatment for leukemia

Cord Blood Registry
Acquired by AMAG Pharmaceuticals Inc
2015
USD 700M

Cellular Dynamics
Acquired by Fujifilm
2015
USD 307M

CiRA & Takeda partner in iPS Collaboration
2015
USD 267M

Ocata Therapeutics
Acquired by Astellas
2016
USD 379M

Bayer and Versant Ventures
launched stem cell therapy company BlueRock Therapeutics
2016
USD 225M (SERIES A)

+ multiple license agreements over recent years

What to Expect in FY18

- Completion of the Phase 1 clinical trial of CYP-001 in GvHD
- Progress in the Fujifilm strategic alliance
- Completion of pre-clinical programs in asthma, heart attack, glioblastoma
- Further development in apceth partnership
- Further corporate partnership activity
- Strengthened I.P. portfolio

Investment Highlights Summary

- **Scalable, robust technology:** Cymerus platform enables mass-production of therapeutic MSCs with multiple clinical targets
- **Clinical trials:** World first Phase I clinical trial underway in GvHD; Cohort A fully enrolled
- **Licensing-driven business model beginning to yield:** Partnership agreement with Fujifilm worth up to $60m plus royalties
- **Increasingly favorable regulatory environment**
- **Compelling preclinical data** from studies in asthma, heart attack and critical limb ischemia.
- **Value-accretive news flow** expected in near term, with completion of phase I GvHD trial
- **Strong balance sheet:** cash runway to 2019 based on current projections

Development and Corporate Partners

Expert Team

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.

Dr Killian Kelly – Vice President, Product Development

- +15 years' experience in pharmaceutical/biotechnology research and development
- Previous appointments include Senior Director, Drug Development at Biota Pharmaceuticals (NASDAQ: BOTA), Vice President, Regulatory and Clinical at Mesoblast Limited (ASX:MSB), positions with Kendle International, Amgen (NASDAQ: AMGN) and Astrazeneca (LSE: AZN).
- Masters in Pharmacy and a PhD in Pharmaceutical Sciences from Strathclyde University, Glasgow

Thank you for your attention

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