A Next Generation Stem Cell Company

Dr. Ross Macdonald, CEO
Cynata Therapeutics Limited
September 2017
Important Information

This presentation has been prepared by Cynata Therapeutics Limited. ("Cynata" or the "Company") based on information available to it as at the date of this presentation. The information in this presentation is provided in summary form and does not contain all information necessary to make an investment decision.

This presentation does not constitute an offer, invitation, solicitation or recommendation with respect to the purchase or sale of any security in Cynata Therapeutics, nor does it constitute financial product advice or take into account any individual’s investment objectives, taxation situation, financial situation or needs. An investor must not act on the basis of any matter contained in this presentation but must make its own assessment of Cynata Therapeutics and conduct its own investigations. Before making an investment decision, investors should consider the appropriateness of the information having regard to their own objectives, financial situation and needs, and seek legal, taxation and financial advice appropriate to their jurisdiction and circumstances. Cynata Therapeutics is not licensed to provide financial product advice in respect of its securities or any other financial products. Cooling off rights do not apply to the acquisition of Cynata Therapeutics securities.

Although reasonable care has been taken to ensure that the facts stated in this presentation are accurate and that the opinions expressed are fair and reasonable, no representation or warranty, express or implied, is made as to the fairness, accuracy, completeness or correctness of the information, opinions and conclusions contained in this presentation. To the maximum extent permitted by law, none of Cynata Therapeutics, its officers, directors, employees and agents, nor any other person, accepts any responsibility and liability for the content of this presentation including, without limitation, any liability arising from fault or negligence, for any loss arising from the use of or reliance on any of the information contained in this presentation or otherwise arising in connection with it.

The information presented in this presentation is subject to change without notice and Cynata Therapeutics does not have any responsibility or obligation to inform you of any matter arising or coming to their notice, after the date of this presentation, which may affect any matter referred to in this presentation.

The distribution of this presentation may be restricted by law and you should observe any such restrictions.

Forward looking statements

This presentation contains certain forward looking statements that are based on the Company’s management’s beliefs, assumptions and expectations and on information currently available to management. Such forward looking statements involve known and unknown risks, uncertainties, and other factors which may cause the actual results or performance of Cynata to be materially different from the results or performance expressed or implied by such forward looking statements. Such forward looking statements are based on numerous assumptions regarding the Company’s present and future business strategies and the political and economic environment in which Cynata will operate in the future, which are subject to change without notice. Past performance is not necessarily a guide to future performance and no representation or warranty is made as to the likelihood of achievement or reasonableness of any forward looking statements or other forecast. To the full extent permitted by law, Cynata and its directors, officers, employees, advisers, agents and intermediaries disclaim any obligation or undertaking to release any updates or revisions to information to reflect any change in any of the information contained in this presentation (including, but not limited to, any assumptions or expectations set out in the presentation).
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
R&R 2016 Redux

What we said 12 months ago at this conference

We will ...

What we have achieved in last 12 months

- Strategic partnership and investment from FUJIFILM
- Ongoing license option agreement with apceth

- Phase 1 clinical trial commenced in May 2017 in UK and Australia (GvHD)
- Compelling data in pre-clinical studies, e.g. asthma, CLI and heart attack

We will monetise our technology through partnering and licensing

We will prove out our platform in pre-clinical and clinical testing
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*\(^2\)

Market Activity

Cellular therapy is a key category and no longer an evolving market

**August 30:** FDA approved Novartis’ product, Kymriah, a CAR-T cell treatment for leukemia

**August 28:** Gilead to acquire Kite Pharma for US$11.9b

- **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)

**A significant number of licence agreements have also been secured over recent years**
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 **US$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. The market is estimated to be worth **US$773.1 million by 2025**⁴

## Development Progress

<table>
<thead>
<tr>
<th>Condition</th>
<th>Pre-Clinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Evidence</th>
</tr>
</thead>
<tbody>
<tr>
<td>GvHD</td>
<td>University of Massachusetts</td>
<td>Patient dosing commenced</td>
<td></td>
<td></td>
<td>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</td>
</tr>
<tr>
<td>Asthma</td>
<td>Monash University</td>
<td></td>
<td></td>
<td></td>
<td>Cymerus™ MSCs demonstrated significant beneficial effects on three key components of asthma: airway hyper-responsiveness, inflammation and airway remodeling.</td>
</tr>
<tr>
<td>Heart Attack</td>
<td>University of Sydney</td>
<td></td>
<td></td>
<td></td>
<td>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.</td>
</tr>
<tr>
<td>Cancer / Glioblastoma</td>
<td>Harvard/ BWH</td>
<td></td>
<td></td>
<td></td>
<td>Research collaboration in genetically modified MSCs in cancer: involves modifying stem cells to target cancer</td>
</tr>
</tbody>
</table>

**World firsts:**
- Scalable manufacture of MSCs without reliance upon multiple donors
- First clinical trial of an allogeneic, iPSC-derived MSC product
Why GvHD?

- Graft-versus-host disease (GvHD) occurs after a bone marrow transplant from a donor (allogeneic).
- The transplanted cells regard the recipient's body as foreign and reject and attack the recipient’s tissues.
- MSCs shown to be effective.
- Quick trial: expected completion in early 2018.
- Successful Cynata trial outcome opens the door to multiple further indications.

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)
Efficacy of Cymerus MSCs in GvHD

Cymerus iPSC-MSCs provide a significant survival benefit in a pre-clinical rodent model of Graft-vs-Host Disease:

Comparison |  p  
-------------|------
GvHD/CYP-001 Single Dose vs GvHD Controls | <0.0001 
GvHD/CYP-001 Dual Dose vs GvHD Controls | <0.0001 
GvHD/CYP-001 Single Dose vs GvHD/CYP-001 Dual Dose | 0.0749 

N=8: controls  
N=12: actives
GvHD license option agreement with 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: early 2018

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
Our platform provides a scalable business model

External collaborations
Preclinical PoC development of potential products for target diseases

✓ GvHD/transplantation
✓ Asthma/respiratory disease
✓ Heart Attack
✓ Vascular disease
✓ 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
✓ Successful evaluation 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
Investment Summary

- **Scalable, world-first technology:** Cymerus platform overcomes inherent challenges of other production methods, and enables mass-production of therapeutic MSCs

- **Technology already being monetised:** Licensing agreement with Fujifilm, and apceth Biopharma. Fujifilm license option worth up to US$60m plus royalties

- **Clear regulatory path:** Japan, US and EU accelerating legislative changes to accelerate stem cell therapy research and uses

- **Clinical trials ongoing:** Phase I clinical trials commenced in UK and Australia in GvHD. License option agreement with apceth Biopharma for several other disease target areas

- **Near-term news flow:** Value-accretive news flow expected in near term, with a DSMB ‘halfway update’ expected for the phase I GvHD trial expected later in 2017
Thank you for your attention

Cynata Therapeutics Limited
Level 3
62 Lygon Street
Carlton
Victoria 3053
Australia

Contact details:
- ross.macdonald@cynata.com
- +61 (0) 412 119343
- www.cynata.com
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.

<table>
<thead>
<tr>
<th>ASX code</th>
<th>CYP</th>
</tr>
</thead>
<tbody>
<tr>
<td>Commenced operations</td>
<td>November 2013</td>
</tr>
<tr>
<td>Market cap</td>
<td>A$ ~50m</td>
</tr>
<tr>
<td>Shares on issue</td>
<td>90m</td>
</tr>
<tr>
<td>Cash</td>
<td>A$10.3m as at 30 June 2017 ($10m raised in Jan 2017 via placement and Fujifilm strategic partnership)</td>
</tr>
<tr>
<td>Number of shareholders</td>
<td>~2300; FUJIFILM ~9%</td>
</tr>
</tbody>
</table>

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

- Strategic partnership and $4m investment from FUJIFILM.
- 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.
- Approval for Phase I clinical trials in the UK and Australia for GvHD.

- 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.
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. Issues with production scale-up
2. Inconsistent product quality
3. Reduced product efficacy
4. Significant intra- and inter-donor variability
5. 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
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
- Therapeutic MSCs are administered to the patient

Cymerus platform harnesses unlimited expansion capacity of iPSCs
- Induction of precursor cells
- Generation of precursor cell colonies (mesenchymoangioblasts) (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.”