ASX ANNOUNCEMENT 31 January 2020

# Cynata Investor Presentation

**Melbourne, Australia; 31 January 2020:** Cynata Therapeutics Limited (ASX: CYP), a clinical-stage biotechnology company specialising in cell therapeutics, has today released a new investor presentation. Cynata will use this presentation to update shareholders, investors and other attendees at the Proactive CEO investor events in Sydney on Monday 3 February and in Melbourne on Tuesday 4 February, and for other upcoming investor events.

Further details of the Sydney and Melbourne Proactive events may be found at the Proactive Investors Australia website.

-ENDS-

Authorised for release by Dr Ross Macdonald, Managing Director & CEO

***CONTACTS:*** Dr Ross Macdonald, CEO, Cynata Therapeutics, +61 (0)412 119343, ross.macdonald@cynata.com
Claire LaCagnina, U.S. Media Contact, +1 315.765.1462, clacagnina@6degreespr.com

## About Cynata Therapeutics (ASX: CYP)

Cynata Therapeutics Limited (ASX: CYP) is an Australian clinical-stage stem cell and regenerative medicine company focused on the development of therapies based on Cymerus™, a proprietary therapeutic stem cell platform technology. Cymerus overcomes the challenges of other production methods by using induced pluripotent stem cells (iPSCs) and a precursor cell known as mesenchymoangioblast (MCA) to achieve economic manufacture of cell therapy products, including mesenchymal stem cells (MSCs), at commercial scale without the limitation of multiple donors.

Cynata's lead product candidate CYP-001 met all clinical endpoints and demonstrated positive safety and efficacy data for the treatment of steroid-resistant acute graft-versus-host disease (GvHD) in a Phase 1 trial. Cynata plans to advance its Cymerus™ MSCs into Phase 2 trials for GvHD, critical limb ischemia and osteoarthritis. In addition, Cynata has demonstrated utility of its Cymerus MSC technology in preclinical models of asthma, diabetic wounds, heart attack and cytokine release syndrome, a life-threatening condition stemming from cancer immunotherapy.

# A Next Generation Stem Cell Therapeutics Company

Proactive CEO Event - Investor Presentation: Cynata Therapeutics Limited
February 2020

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# Cynata Therapeutics is a Phase II-ready biotech with a highly scalable, proprietary platform for developing stem cell therapeutics

## Our focus

*Utilise our proprietary Cymerus™ platform technology to develop commercially scalable cellular therapeutic products to treat serious chronic disorders*

## Financial information

| | |
|---|---|
| Share price (27-Jan-20) | A$1.12 |
| Shares on issue | 102.8m |
| **Market capitalisation** | **A$115.2m** |
| Cash[1] | A$7.8m |
| Debt | - |
| **Enterprise value** | **A$107.4m** |

## Top shareholders

| | |
|---|---|
| Fidelity INTERNATIONAL | 9.3% |
| FUJIFILM | 7.9% |
| Board and management | 6.0% |



1. Cash incorporates A$5.92m as at 31 Dec 2019, adjusted for the A$1.89m R&D tax rebate received in January 2020

# Investment Highlights

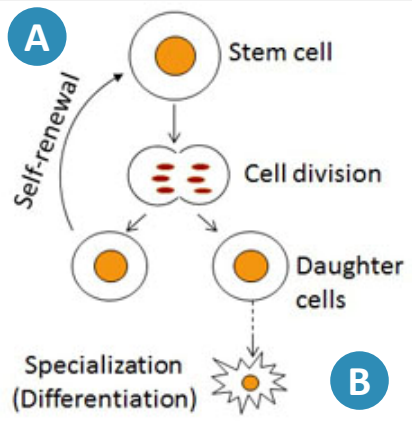
- **Outstandingly well positioned in regenerative medicine**
- **Scalable, globally applicable technology**
- **Successful clinical trial results: world leader**
- **Attractive and validated licensing business model**
- **Clear pipeline of high potential target areas**

# What are Mesenchymal Stem Cells (MSC's)?

## Stem Cells:

A have the ability to **divide and create an identical copy of themselves** (ie. 'self-renew')

B can **divide and differentiate to form mature cells** (e.g. skin cells, nerve cells, muscle cells etc)




## Mesenchymal Stem Cells:

MSCs are **multipotent** stem cells found in bone marrow, fat etc.

MSCs can differentiate into specialised cells[1] and have very important biological properties, leading to intense investigation of their utility as therapeutic products

More than 1000 clinical trials of MSCs initiated around the world[2]



Note: There are many different types of stem cells, each with very different biological properties and potential to treat disease
1. Cells such as osteoblasts, chondrocytes & adipocytes; 2. www.clinicaltrials.gov (as at November 2019)

# Our patented Cymerus platform enables the production of MSC's from a single adult donor

To watch an animation of our Cymerus technology please click here:

https://www.cynata.com/#home

# Cynata's MSCs are high quality and commercially scalable

## Key advantages of the Cymerus process

**CONSISTENCY & SCALABILITY**

- ✓ **Consistent product quality –** single donor overcomes regulatory concerns
- ✓ **Bypasses complex and invasive surgeries** with a scalable and cost-effective process,
- ✓ **Lower cost of goods on a per cell basis** compared to conventional MSC products

**FEWER CELLS PER PATIENT**

**2 infusions per patient** in GvHD, compared to 8-12 for bone-marrow derived products

- ✓ **Greater convenience** for patients and hospitals
- ✓ **Lower costs** incurred by healthcare system

**Cynata has the only platform in the world able to produce commercial quantities of MSCs from a single source**

# Cynata is executing on a clear scientific and commercial vision and continually assesses pathways to optimise shareholder value

## Multiple options to create shareholder value

**Build value in platform independently**

***Cynata's current focus***

**License / partner with big Pharma to develop specific target areas**

**Strategic exit/merger**

## FUJIFILM case study

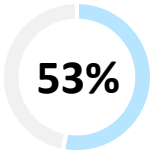
- ✓ Exclusive global licence in GvHD
- ✓ Multiple cash flow events:
  - US$3m equity @ 35% premium
  - US$3m upfront license fee received
  - US$40m in potential milestone payments
  - Double digit royalties (worth potentially >US$30m p.a.)
- ✓ Represents a major endoresement by Big Pharma
- ✓ Ongoing relationship with potential for further commercial agreements

**FUJIFILM transaction provides validation of the Cymerus platform and supports the licensing of additional target areas**

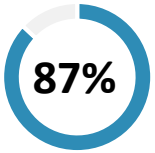
# Value inflection point following clear data

**Successful Phase 1 clinical trial data demonstrating efficacy of Cynata's technology platform**

- ✓ **Successful clinical trial results** with **all endpoints achieved**[1]




***Complete Response[2] rate***




***Overall Response[3] rate***


***Overall survival[4] rate***

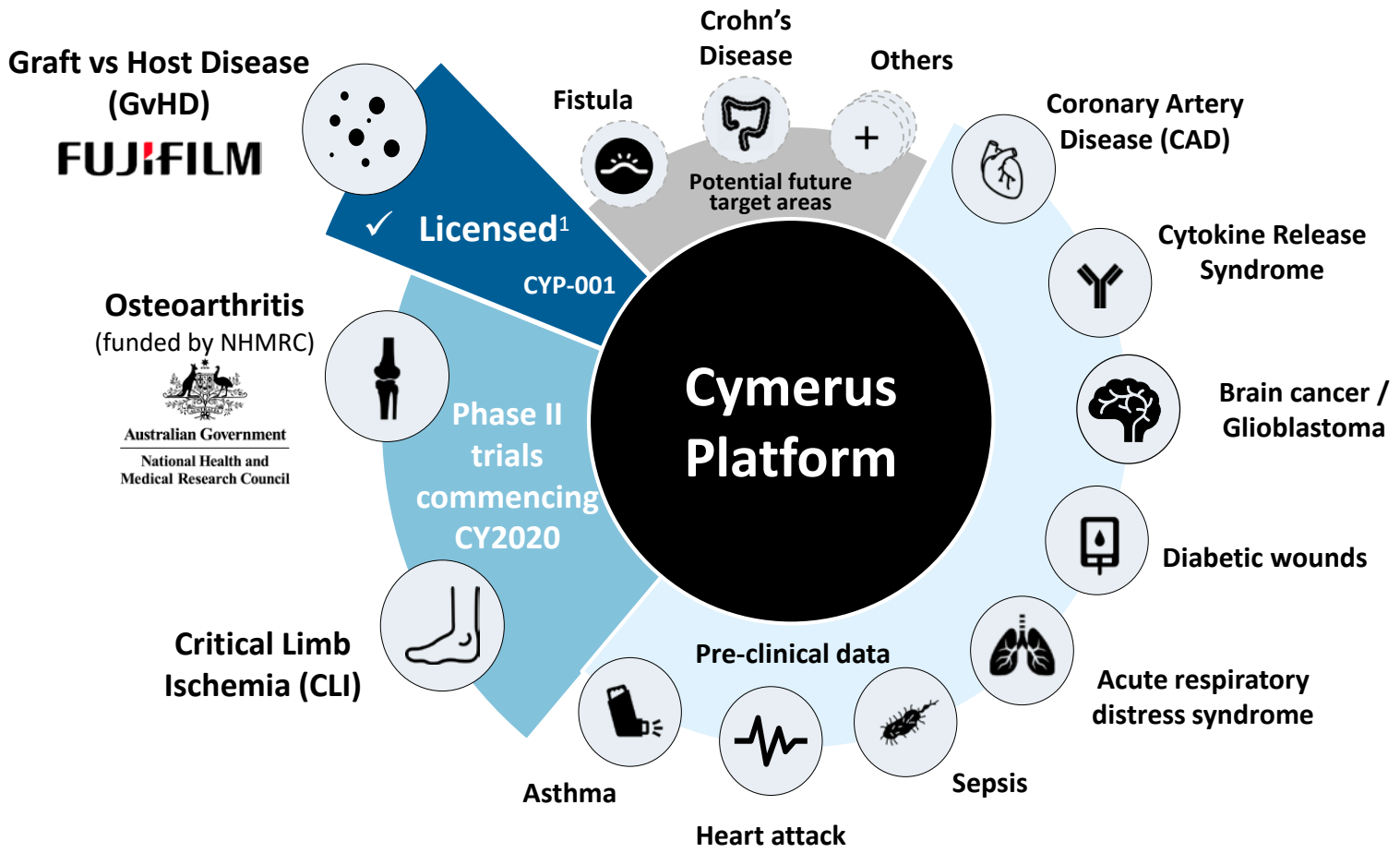
- ✓ **World-first allogeneic iPSC-derived cell therapy clinical trial** in steroid-resistant acute GvHD

- ✓ **Clinically meaningful findings** validate progress to multiple Phase II trials (ie. enables other indications to bypass Phase 1)



1. Pooled Cohort A/B results at 100 days. 2. Absence of GvHD. 3. Overall Response is either a Complete or Partial Response (improvement by 1+ grade).
4. One patient in Cohort A died of pneumonia (unrelated to treatment), one patient in cohort B withdrew from trial on Day 22 to commence palliative care.

# Cynata's Cymerus platform has potential applications across a wide range of diseases






1. FUJIFULM exercised license option in GvHD on 17 September 2019

# Cynata is well placed in a increasingly validated MSC market

## Clinical use of MSCs continues to grow

**~6 approved MSC therapies now on the market**[1]

**~30 Phase 3 trials** with MSC-based therapies currently active

**Over 1,000 clinical trials with MSCs** have been **initiated**[2]

**Growing evidence for the role of MSCs in repair & regeneration**

## Cynata is well placed in the MSC-based therapy market

Many ongoing Phase 3 trials involve very common conditions, representing **multi-billion** dollar market opportunities

Approvals in any of these indications will significantly **increase Big Pharma's interest** in MSCs

Demand for large quantities of product will focus attention on the current **major manufacturing challenges**[3]

**Cynata's uniquely scalable and consistent process is ideally placed to solve these manufacturing challenges**

1. Cupistem®; Queencell®; NeuroNata-R®; TEMCELL®; Stempeucel®; Alofisel®
2. www.clinicaltrials.gov (as at November 2019)
3. Major challenges associated with conventional production methods

# Significant upside potential based on Mesoblast market valuation

| | cynata therapeutics | mesoblast the adult stem cell company |
|---|---|---|
| **Market capitalisation (A$m)**[1] | 113 | 1,610 |
| **ASX listed** | ✓ | ✓ |
| **Focus** | • Regenerative and cellular medicine | • Regenerative and cellular medicine |
| **Products in market** | | • TEMCELL® (via licensee JCR Pharmaceuticals Co., Ltd)<br>• Alofisel®(via licensee Takeda) |
| **Development pipeline** | • Three Phase 2 ready indications (GvHD; CLI; Osteoarthritis)<br>• Broad development pipeline (>11 target areas) | • One product filing for FDA approval (aGvHD)<br>• Two Phase 3 product candidates (back pain; heart failure)<br>• Pipeline of emerging Phase 2 products (~4 target areas) |
| **Licensing** (for development pipeline) | • FUJIFILM (GvHD) | • Grünenthal (Back pain)<br>• Tasly (Heart failure) |

*"[The Fujifilm license] equates to significant validation of [Cynata's] platform … and is an indicator of likely success in other indications.'*

- Darren Vincent, Senior Analyst
Shaw and Partners

*"Success in GvHD alone, in our opinion, supports the current valuation of [Mesoblast]"*

- Jason Kolbert, Healthcare Research
Dawson James Securities



Source: Analyst Research Reports; Mesoblast Investor Presentation
1. As at 27-Jan-20

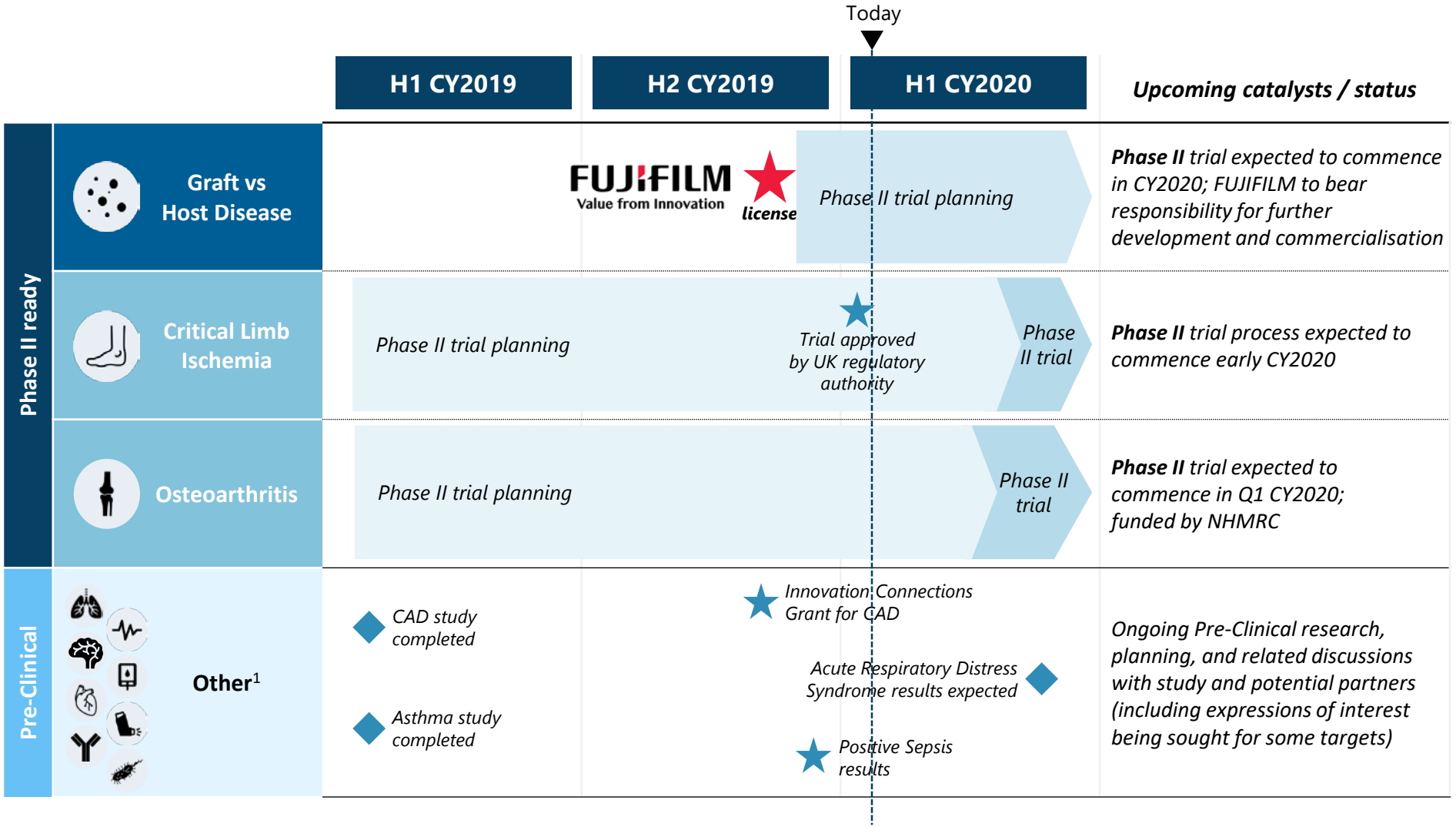
# Cynata is targeting significant market opportunities

| TARGET AREA | TRIAL PHASE | MARKET OPPORTUNITY |
|---|---|---|
| **Graft vs. Host Disease (GvHD)** | **Entering Clinical Phase II** | US $0.3bn[1] |
| **Critical limb ischemia (CLI)** | **Entering Clinical Phase II** | US $1.4bn[2] |
| **Osteoarthritis (OA)** | **Entering Clinical Phase II** | US $11.6 bn[3] |
| **Other** *Asthma, ARDS, Heart Attack, Coronary Artery Disease, Sepsis, Brain Cancer / Glioblastoma, Diabetic Wounds, CRS* | **Pre-Clinical** | |

1. Fujifilm's estimate of the peak annual global sales opportunity
2. ClearView's estimate of the peak annual global sales opportunity
3. Persistence Market Research 2018 research report: "Osteoarthritis Treatment Market: Global Industry Analysis (2012-2016) and Forecast (2017-2025)

## Outlook

# Cynata has a large pipeline of indications with upcoming catalysts




1. Other indications include Acute Respiratory Distress Syndrome; Heart Attack; Brain Cancer / Glioblastoma; Diabetic wounds; Coronary Artery Disease (CAD); Asthma; Cytokine Release Syndrome; Sepsis


Thank you for your attention

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

**Contact details:**

ross.macdonald@cynata.com

www.cynata.com


# Appendix

# Investment Summary

| | |
|---|---|
| **1 Scalable, globally applicable technology** | • Cymerus platform technology enables commercial-scale production of mesenchymal stem cells<br>• Fully patented process overcomes multiple issues with today's on-market solutions<br>• Value of platform to a range of diseases demonstrated across multiple clinical and pre-clinical studies |
| **2 Attractive licensing business model** | • A 'hub and spoke' model: intention to license Cymerus technology across a range of target areas<br>• Licence granted to FUJIFILM for GvHD on highly attractive terms, including US$3m upfront fee, >US$40m in milestone payments, double digit royalties on product sales and FUJIFILM responsible for all further product development activities and costs<br>• Cynata in active commercial discussions with multiple other parties |
| **3 Successful clinical trial results** | • First in-human trial of Cymerus MSCs in GvHD successfully completed in 2018<br>• All trial endpoints achieved: no safety concerns identified; highly encouraging efficacy<br>• Endorsement by FUJIFILM of Cynata's Cymerus platform supports the continued commercialisation of Cynata's cell therapeutic products in other indications |
| **4 Clear pipeline of high potential target areas** | • Phase II clinical trial in Critical Limb Ischemia (CLI) expected to commence in 2020, approved by UK regulators<br>• Phase II clinical trial in Osteoarthritis (OA) expected to commencie in 2020, funded by NHMRC<br>• Phase II clinical trial in graft-versus-host-disease (GvHD) expected to commene in 2020 (FUJIFILM)<br>• Compelling pre-clinical data in multiple other high-value target areas supports further clinical trials |
| **5 Well positioned in regenerative medicine** | • Cell therapeutics is an area of increasing interest from major pharmaceutical companies<br>• Global market opportunity of US$1.4bn for CLI and US$11.6bn for OA<br>• Over 1000 clinical trials investigating the efficacy of MSCs in treating diseases have been initiated globally |

# Globally experienced board and management team

**Dr Paul Wotton**
Chairman

- CEO, Obsidian Therapeutics
- Former CEO of Ocata Therapeutics (NASDAQ: OCAT) acquired 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; board member of Vericel Corp 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 / CEO

- 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-Exec Director

- 20+ years of CEO and Board experience in medical technology, biotech and agri-food companies
- Exec Chairman of Emerald Clinics, Chairman of Orthocell Ltd, Director of Botanix Ltd and Zelda Therapeutics Ltd
- 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 Geoff Brooke**
Non-Exec Director

- 30+ years venture capital experience
- Co-founded GBS Venture Partners
- Formerly President of Medvest, a US-based early-stage venture capital group he founded with Johnson & Johnson
- Other Board experience include non-executive director of Acrux Limited and Chairman of Actinogen Media Limited

**Extensive life sciences and financial expertise in US and Australia**

**Mr Peter Webse**
Non-Exec 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**
Chief Operating Officer

- 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)

**Extensive academic, commercial and management experience**

## GvHD clinical trial results

# Clinical trial design and key implications of clinical trial results

| What is GvHD? | Graft versus host disease (GVHD) is a condition where following a transplant the donor's immune cells in the transplant (graft) make antibodies against the patient's tissues (host) and attack vital organs. Organs most often affected include the skin, gastrointestinal (GI) tract and the liver. |
|---|---|

## Clinical trial design

**Screening criteria**
- Adults with steroid resistant acute GvHD
- Life expectancy of at least 1 month
- Other conditions screened out that may impact results

**Cohort A**
May-17 – Dec-17
n=8

$1x10^6$ cells/kg on Day 0 and Day 7[1]

28 day read-out ✓

100 day read-out ✓

Data and Safety Monitoring Board (DSMB) assessed Cohort A 28-day data and **approved commencement of Cohort B**

**Cohort B**
Jan-18 – May-18
n=7[3]

$2x10^6$ cells/kg on Day 0 and Day 7[2]

28 day read-out ✓

100 day read-out ✓

## Key implications of clinical trial results

**Endpoints**
- Endpoints in this trial were the **same as those required in a Phase 3 trial** (in contrast to early phase trials for some other conditions)

**Response rates**
- Response rates were **higher than what we expect would be required in Phase 3**, to support marketing approval

**Number of patients**
- Although the Phase 1 trial involved just 15 treated subjects, **even late stage trials in this condition do not necessarily involve large numbers**
- For comparison, recently completed Phase 3 trials in Japan and US have involved just 25 and 55 patients, respectively

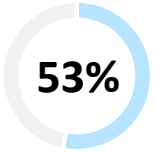


1. Max $1x10^8$ cells. 2. Max $2x10^8$ cells 3. One patient withdrew from trial prior to dosing; trial was intended to have 8 participants

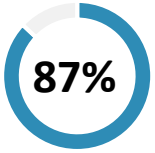
## GvHD clinical trial results

# Highly successful outcome, with majority of patients reporting a Complete Response from a devastating disease

### Phase I clinical trial data – all endpoints achieved[1]



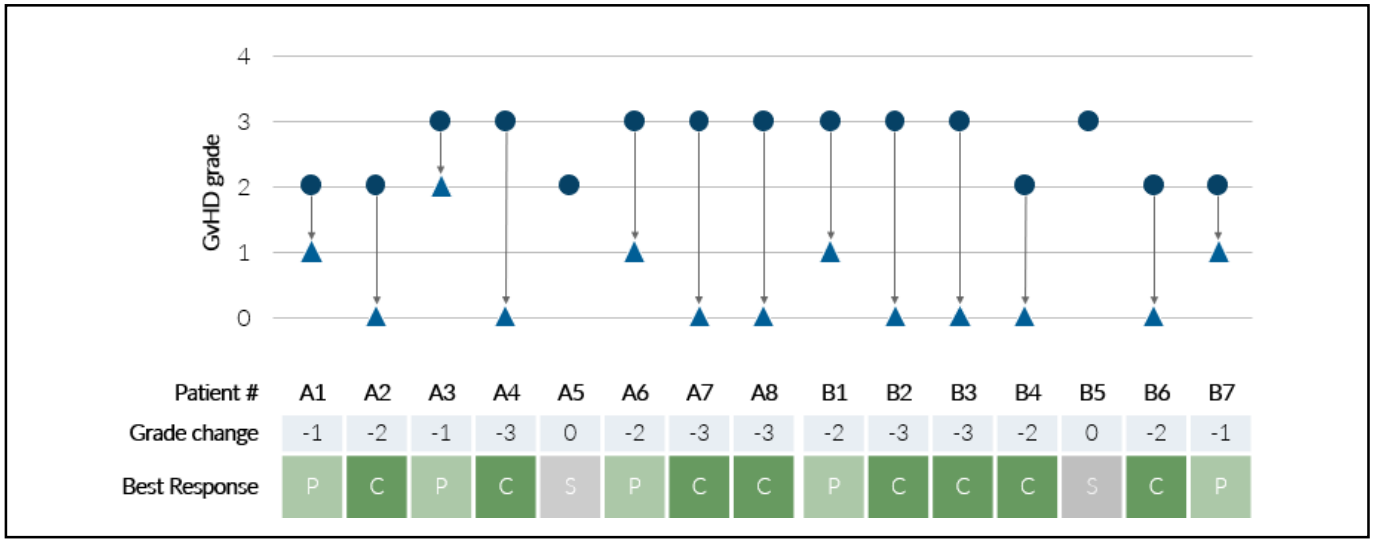

***Complete Response[2] rate***




***Overall Response[3] rate***


***Overall survival[4] rate***

### Patient data




**Legend**

**GvHD grade**

- GvHD grade: As at day 0
- GvHD grade: Best Response

**Patient response**

- C — Complete Response
- P — Partial Response
- S — Stable disease

No treatment-related serious adverse events or safety concerns were identified

1. Pooled Cohort A/B results at 100 days. 2. Absence of GvHD. 3. Overall Response is either a Complete or Partial Response (improvement by 1+ grade).
4. One patient in Cohort A died of pneumonia (unrelated to treatment), one patient in cohort B withdrew from trial on Day 22 to commence palliative care.