

A Next Generation Stem Cell Company

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September 2017



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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

What we said 12 months ago at this conference

What we have achieved in last 12 months

We will ...

We will monetise our technology through partnering and licensing

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

FUJIFILM

 **apceth**
BIOPHARMA

We will prove out our platform in pre-clinical and clinical testing

- 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

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Global regenerative medicine market was worth \$18.9 billion in 2016 and will grow to over \$53.7 billion by 2021¹

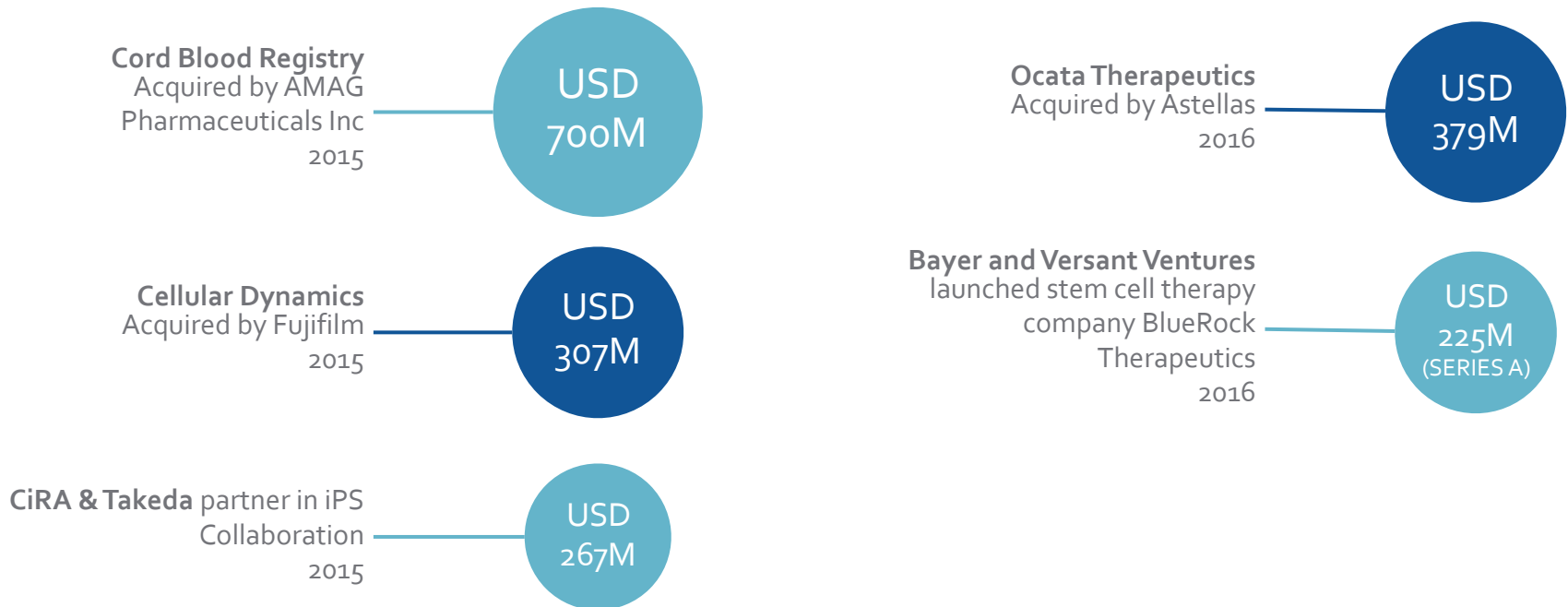
Stem cells are the cornerstone of contemporary regenerative medicine applications²

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

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

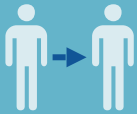
August 30: FDA approved Novartis' product, Kymriah, a CAR-T cell treatment for leukemia

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



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 2019¹








Pulmonary diseases - Pulmonary fibrosis/ scarring of the lungs expected to be **US\$3.2b** by 2025² and asthma that affects 1 in every 12 people reaching **U\$25b** by 2024³




Brain Cancer / Glioblastoma (engineered MSCs) – In 2012, 14 million new cases of cancer and about 8.2 million deaths were reported⁵. The market is estimated to be worth **US\$773.1 million** by 2025⁴

Source: 1. GBI Research. 2. [GlobalData](#) 3. [GrandViewResearch](#) 4. [GrandViewResearch](#) 5. WHO

Development Progress

	Pre-Clinical	Phase 1	Phase 2	Phase 3	Evidence
GvHD					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					Cymerus™ MSCs demonstrated significant beneficial effects on three key components of asthma: airway hyper-responsiveness, inflammation and airway remodeling.
Heart Attack					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					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
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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

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 cancer¹

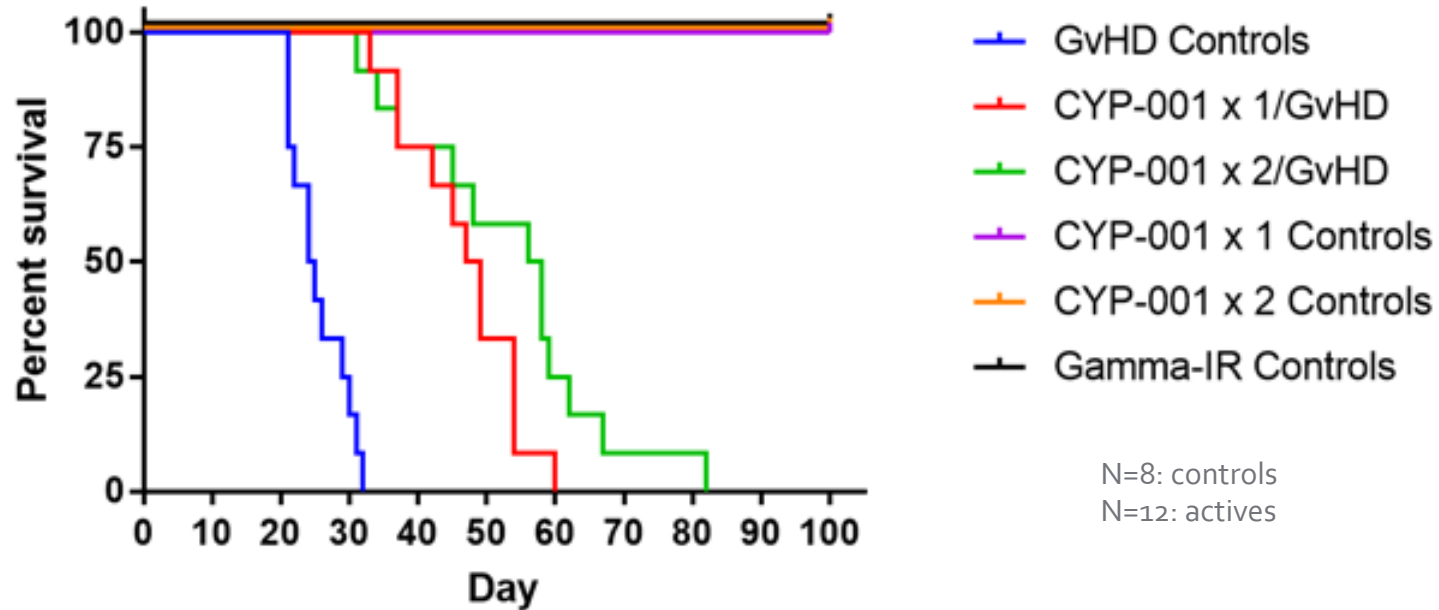
\$0.51bn

market value for the
treatment of GvHD²
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 \(WMDA\)](#)

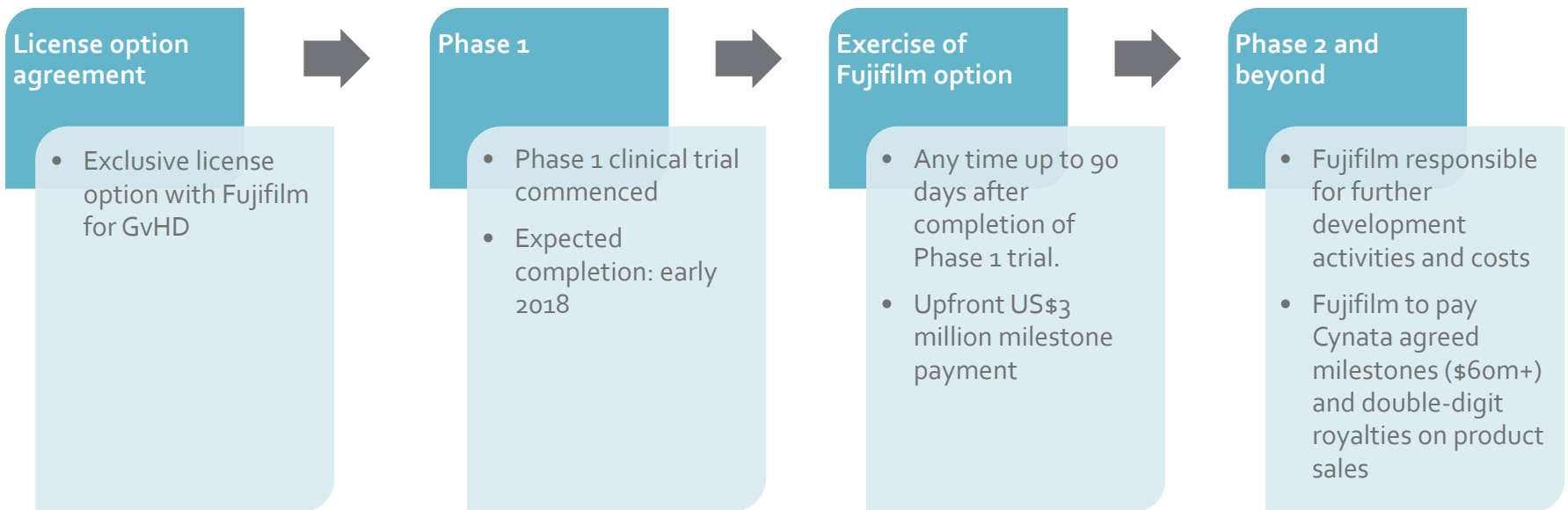
Cymerus iPSC-MSCs provide a significant survival benefit in a pre-clinical rodent model of Graft-vs-Host Disease:



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

FUJIFILM

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



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 spiked cell therapy

- ✓ 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

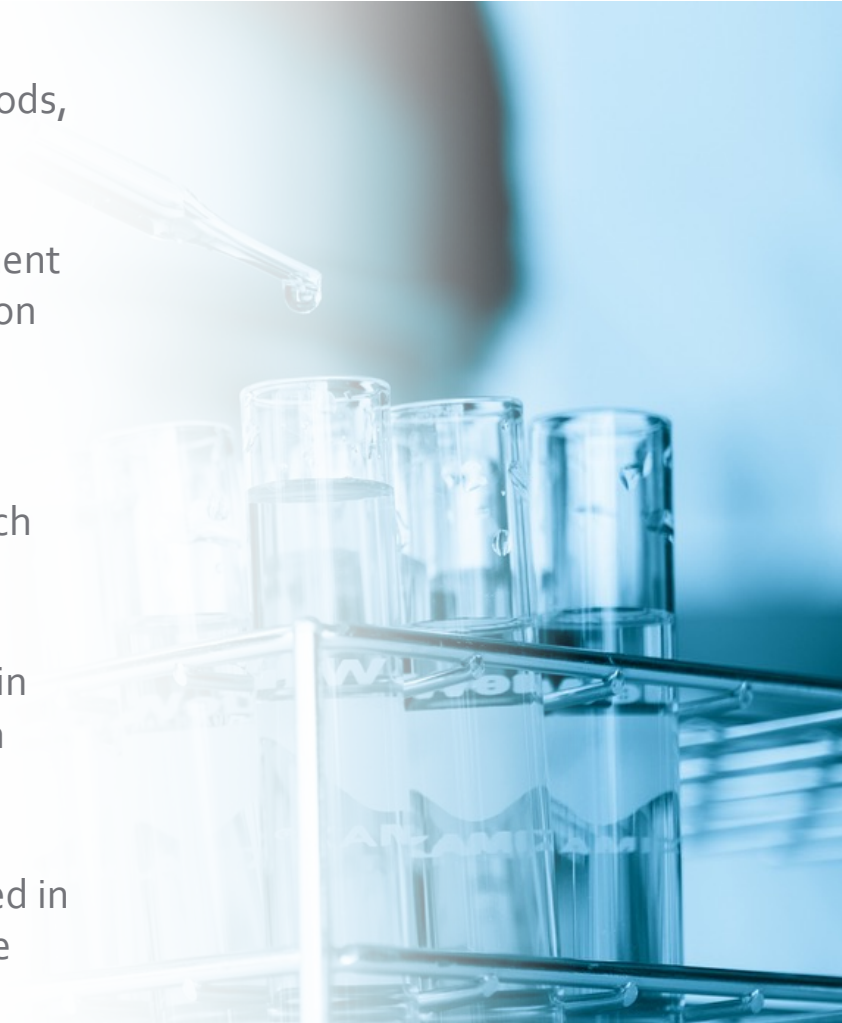
From partners as products progress through clinical trials and approval

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

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Appendix

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	A\$10.3m as at 30 June 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.

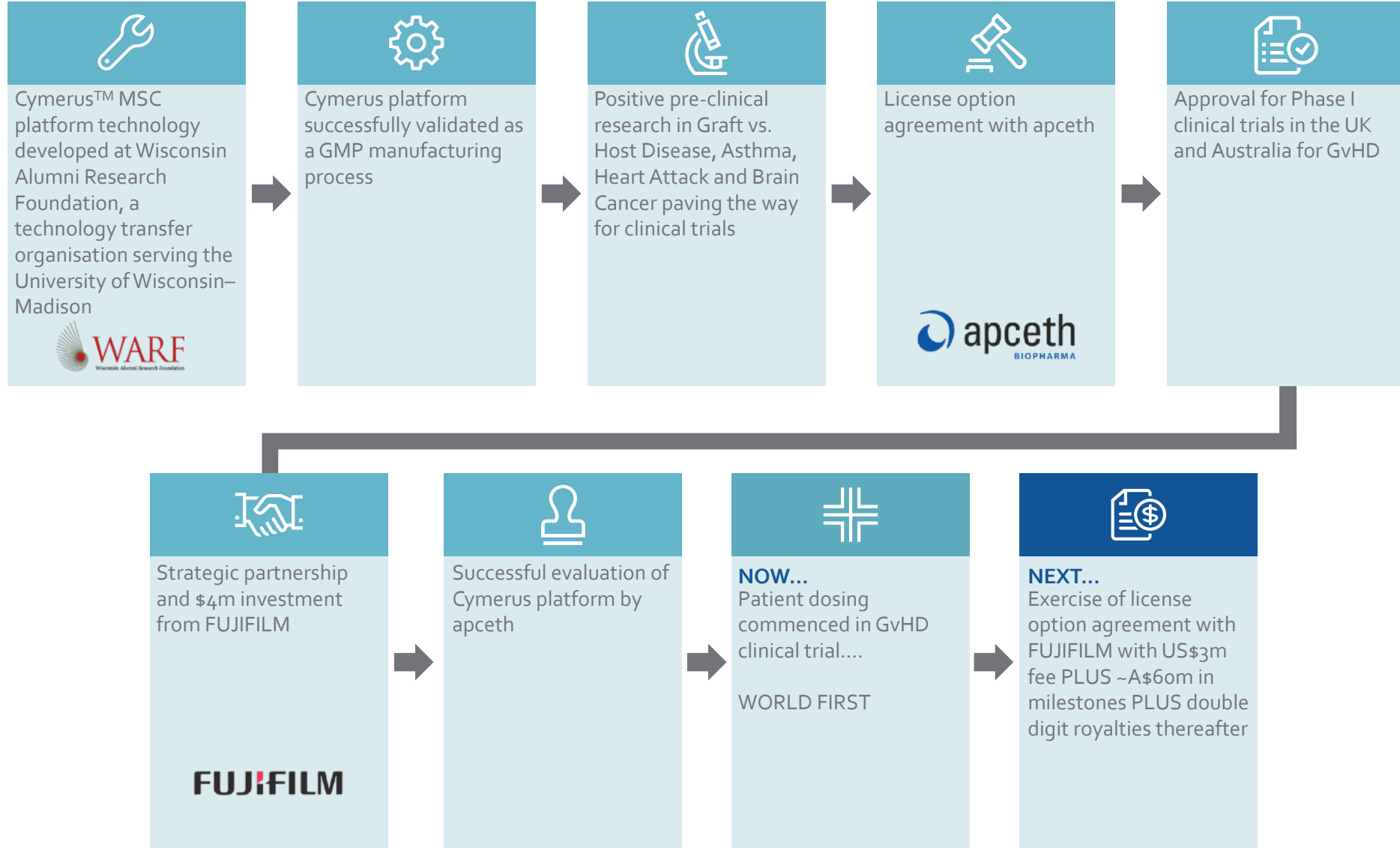
Dr John Chiplin – Non-Executive Director

- 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



Why MSCs?

What are MSCs?

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

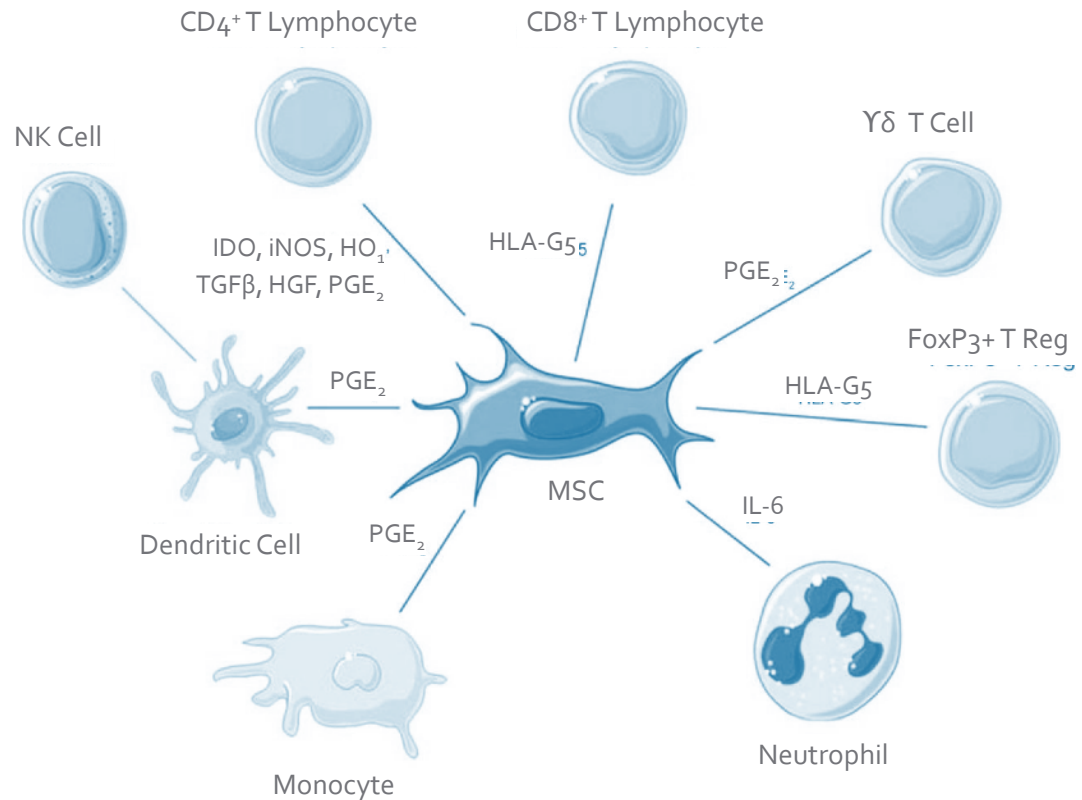
What do they do?

- 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



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

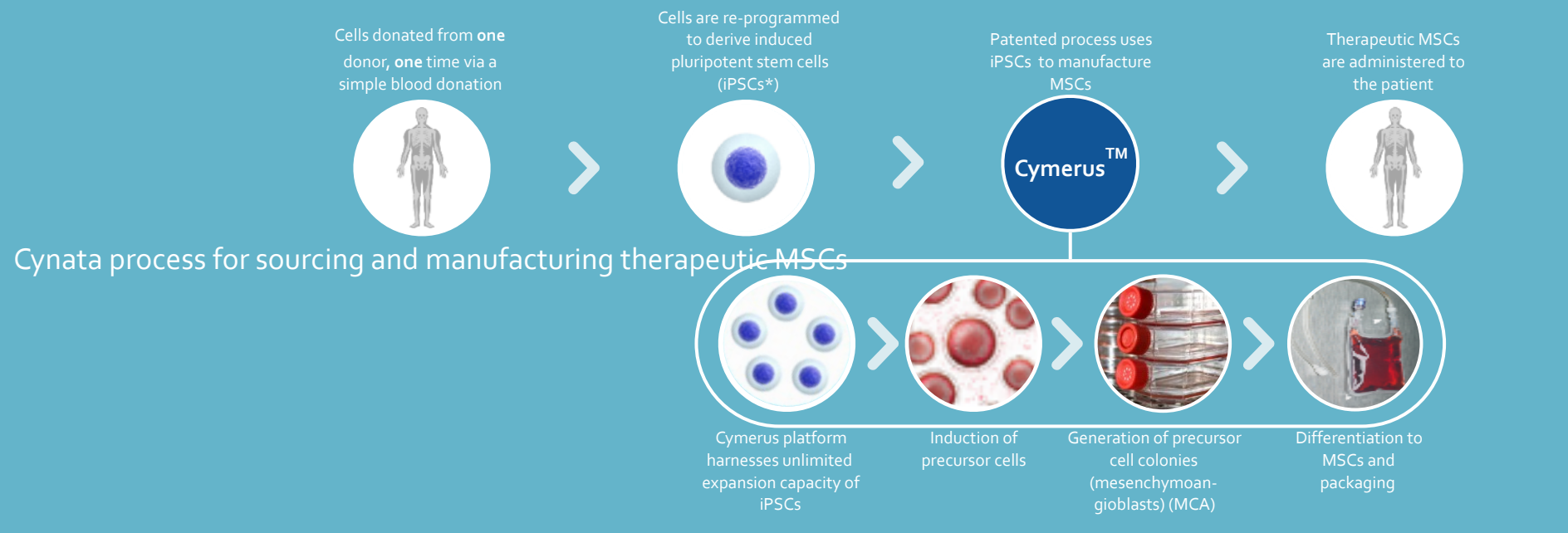
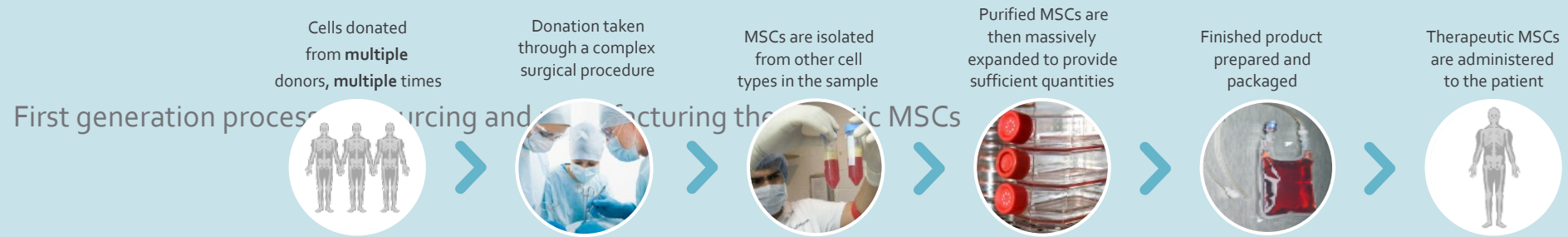
Cynata's Cymerus platform overcomes each of these challenges

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.



*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."