

A Next Generation Stem Cell Company

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Cynata Therapeutics Ltd Key Facts

CYP - Daily Line Chart [Close]

CYP - Volume (with MA) [200]

- 0.700

0.650

0.600

0.450

0.400

= 30000000

20000000

Feb

December

2014

ASX: **CYP** (prev ECQ)

Market Cap (10 Feb 14): \$21.9m

Shares on Issue*: 55.0m

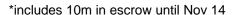
Options (10 Feb 14, \$0.2): 11.11m

Cash (31 Dec 13): \$6.2m

Number of

shareholders: ~1121

Business focus: Stem cells + regenerative medicine



Major holders:	Mr Ian Dixon	4.34%
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Prof Igor Slukvin 4.34%

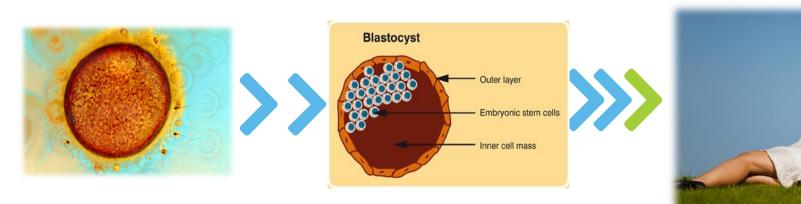
Celtic Capital Pte Ltd 3.64%

JK Nominees Pty Ltd 3.64%

^{2,500,000 27/9/18} unlisted \$0.40 restricted options issued to each of RM and SW, vesting upon attainment of performance hurdles

Cell Replication and Differentiation

The extraordinary capacity of cells to multiply and differentiate:



1 fertilized egg cell

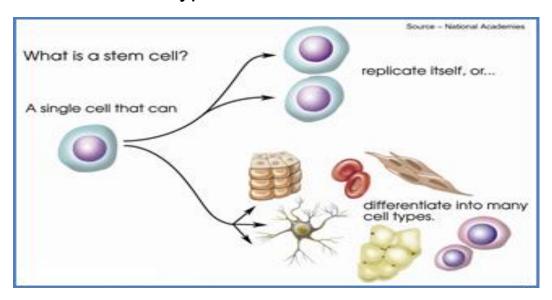
~100 cells "pluripotent"

>10¹³ cells*



What is a Stem Cell?

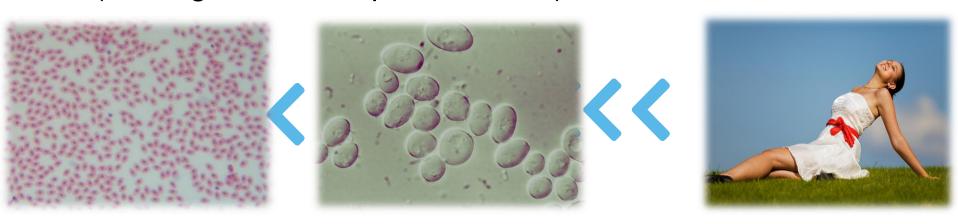
• Stem cells are unspecialised (undifferentiated) cells in the body that give rise to all functional cell types: blood, nerves, bone, muscle......



- Derived from embryos and also certain adult tissues, eg bone marrow
- Stem cells <u>may also</u> assist in the body's own ability to repair or replace tissue that is damaged or destroyed by injury or disease:
 - Physical reconstruction of tissue (or causing it to happen)
 - Immune modulation, i.e. anti-inflammatory mesenchymal stem cells ("MSCs")

Cell De-Differentiation & Reprogramming

 The seminal discovery that cells can be "re-programmed" (leading to a Nobel prize in 2012):



MSCs (or other functional cell types)

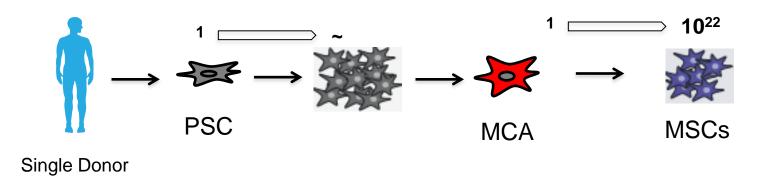
"pluripotent" stem cells (iPSCs)

1 x tissue sample

- Eliminates need for embryos or multiple donors
- Reproducible, consistent source material



Cynata's Cymerus™ Technology Facilitates Commercial-Scale Manufacture



- Patent-protected
- Single donor → unlimited production of uniform, pharmaceutical grade MSCs lacking contaminating immune cells
- Easier manufacturing; easier regulatory route
- Greater clinical predictability



Cynata's Cymerus™: Outstanding Pedigree

2007

- Inventors include James Thomson who derived the first human embryonic stem (ES) cell line in 1998 and human induced pluripotent stem cells (iPSCs) in
- Scientific leadership: Prof Igor Slukvin (UW), co-founder and author of >70 publications in the stem cell field
- WARF: US\$2 billion endowment built from licensing and investment
- In-licensed intellectual property includes several issued US patents as well as a broad estate of issued and pending patents

(12)	United	States	Patent
	Vodvanyk	et al.	

(54) GENERATION OF CLONAL MESENCHYMAL PROGENITORS AND MESENCHYMAL STEM CELL LINES UNDER SERUM-FREE CONDITIONS

(75) Inventors: Maksym A. Vodyanyk, Madison, WI (US); Junying Yu, Madison, WI (US); James A. Thomson, Madison, WI (US); Igor I. Slukvin, Verona, WI (US)

(73) Assignee: Wisconsin Alumni Research Foundation, Madison, WI (US)

US 7,615,374 B2 (10) Patent No.: (45) Date of Patent: Nov. 10, 2009

Olivier, Stem Cells, 2006, vol. 24, p. 1914-1922. Barberi T, et al. "Derivation of multipotent mesenchymal precursors from human embryonic stem cells," PLoS Med. 2: e161 (2005). Korhonen M. "Culture of human mesenchymal stem cells in serumfree conditions: no breakthroughs yet," Eur. J. Haematol. 77:167 (2007).

Meuleman N, et al., "Human marrow mesenchymal stem cell culture: serum-free medium allows better expansion than classical alphaminimal essential medium (MEM)," Eur. J. Haematol. 76:309-316

Meuleman N, et al., "Human marrow mesenchymal stem cell culture: serum-free medium allows better expansion than classical alphaminimal essential medium (MEM)," Eur. J. Haematol. 77:168 (2007).



Ageing: Major Economic and Medical Challenge

- Aging population demographic: 80% of aged Americans have a chronic degenerative disease (eg osteoarthritis, heart disease, diabetes); 50% have two
- By 2030, US healthcare costs will increase by 25% with the cost of providing health care for a person aged >65 being 3-5x higher than the cost for someone <65

Strong need for novel therapies to regenerate damaged tissues

n.b: recent fundamental change in regulatory environment for stem cell products in Japan







MSC Therapies Are Here and Now

- Tissue Engineering and Soft Tissue Repair
 - Reconstruction of tendons, bone, cartilage, bladder, trachea
- Cardiovascular Therapies
 - Pro-angiogenic properties
- Inflammatory Diseases, Transplant Rejection
 - Immunomodulatory properties

Translating to ~191 open clinical studies using MSCs to treat a variety of medical conditions*



Stem Cell Company Market Valuations

- Analogous to monoclonal antibody enabling technology: hybridoma technology developed in 1975 → therapeutic market value now in excess of US\$44.6b¹
- Most stem cell companies attractively priced based on forward estimates with many products now in Phase 2 and 3; M&A warming up

Company	Mkt cap	Development stage	Partners	Cash ⁺
Mesoblast (Aus)	\$1.8b	7 x Ph2 1 x Ph3 1 x approved	Teva JCR Pharm	\$250m
Medipost (S. Korea)	\$471m	Cartistem on sale 2 x Ph1	Dong-A	\$50m
Biotime (USA)	\$250m	2 x Ph1 (+ device in P1)	Teva (AMD)	\$7.5m
Cynata (Aus)	\$22m	Pre-clinical	pending	\$6.2m

Cymerus™ Product Development Program



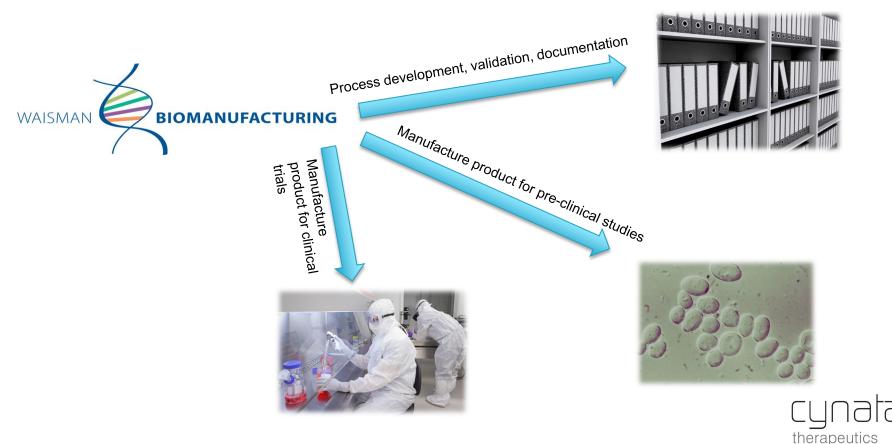
Cymerus™ Product Development

- Excellent data so far:
 - Identification and characterisation of Cymerus™ MCA-derived MSCs
 - Initial manufacturing process development
 - Proof-of-concept using Cymerus™ MSCs in model of critical limb ischemia
- Recruited VP, Product Development
 - Highly credentialed: ex Mesoblast, Biota
 - Multiple stem cell product experience
- Product manufacturing and process development underway (Waisman)
- Consultants engaged to chart regulatory roadmap
 - Findings mid-February
 - Essential guidance for preclinical program
- Further PoC study ready to commence
- Engaged with clinicians to conduct Phase 1 clinical study
 - Graft-versus-host disease



Waisman Biomanufacturing

- Specialist biological product manufacturer in Wisconsin, USA
- Particular experience in translating discoveries to products: developing scalable manufacturing, validation and quality control processes



Proposed Cymerus™ Phase 1 Clinical Trial

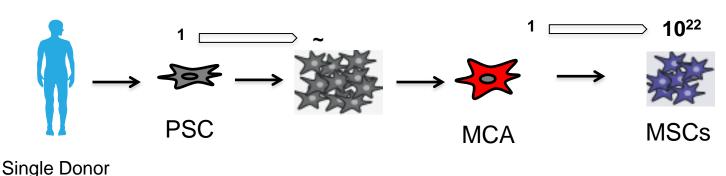
- Acute graft-versus-host disease (aGvHD)
 - Life threatening complication occurring in ~50% of unrelated-donor bone marrow transplant patients
 - Approximately 4,500 unrelated-donor bone marrow transplants/year in USA*
 - Standard-of-care (corticosteroids) effective in fewer than half of patients
 - Clinical evidence that MSCs are effective (one product approved in certain markets)
- Objective of Phase 1 studies is primarily safety
 - Secondary outcome will include measure of efficacy
- Successful outcome will confirm:
 - Validity of up-scaled manufacturing process
 - Clinical basis of activity
- Provides sound basis to expand to additional clinical indications



Commercialisation Roadblock for MSC Therapies

- Commercial-scale manufacture of current MSC products is a major practical & regulatory challenge
 - Limited expansion potential
 - Donor-to-donor and intra-population heterogeneity
 - The difficulties of obtaining pure MSC populations/immunogenicity

Cynata's Cymerus™ technology facilitates commercial-scale manufacture



Cynata's Commercial Goal

- Cynata will develop the proprietary Cymerus[™] technology into:
 - a scalable manufacturing process, and
 - commercial allogeneic stem cell therapeutic products
- Cynata's Cymerus™ MCA- derived MSC's are outstanding stem cell therapeutic candidates
 - Provide unlimited supply of well-defined drug-like quality cellular products for therapies
 - Easy to develop continuous manufacturing and quality control procedures to meet FDA criteria
 - High volume/low cost manufacturing

Timeline

Complete transaction

Regulatory engagement

Manufacturing

Safety (Animals)

Australian human trials

Further POC studies

Partner engagement and

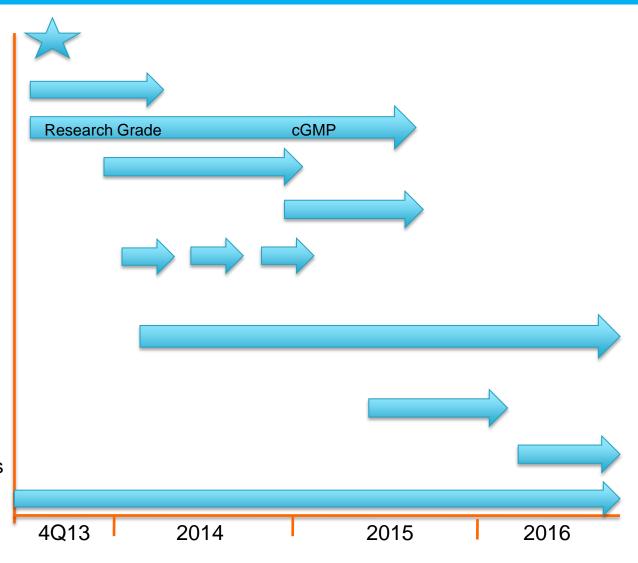
licensing

FDA pre IND meetings

and filing

IND Approval and US trials

IP Development



Potential revenue from Cymerus™ Technology

- Two potential revenue sources:
 - Clinical need: specific "off the shelf" therapeutic products derived from the Cymerus[™] technology
 - Manufacturing scalability: Cymerus[™] proprietary (enabling) method of commercial-scale manufacture → platform technology for partnering/licensing
- Partnership-driven business strategy: business development activities will be initiated upon completion of roll-up



Cynata Board and Management

Executive Chairman: Dr Stewart Washer

Managing Director & CEO: Dr Ross Macdonald

VP Product Development: Dr Kilian Kelly (from 3/14)

Non-executive Director

and Company Secretary: Mr Peter Webse

Executive Director: Mr Howard Digby

- A tight team with extensive industry, cell therapy and public company experience plus a track record of commercialising therapeutic products
- Additional resources to be considered as product development progresses

Why Invest in Cynata Therapeutics?

- Access to the vibrant and expanding field of stem cell medicine
- Innovative technology sourced from established and prestigious centre
- Cymerus[™] addresses a known shortcoming in the commercial model of existing stem cell companies
- Experienced management team
- Value-accretive news flow expected in near term
- Potential revenues from both specific therapeutic products and from enabling platform technology

Thank you for your attention

