# mesoblast the regenerative medicine company

**Acquisition of Osiris' Therapeutic Culture Expanded Stem Cell Business** 

11 October 2013

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# Mesoblast's corporate strategy is to:

- Leverage proprietary cell-based and complementary biologic technologies to develop products for unmet medical needs
- Bring multiple products to market within a parallel timeframe
- Underpin our future financial growth through investing in manufacturing operations; and
- Enhance the likelihood of commercial success through strategic partnerships



# Mesoblast acquires Osiris' cultured Mesenchymal Stem Cell (MSC) therapeutic business

### Strategic benefits:

- Firms up Mesoblast's leadership position in regenerative medicine
- Accelerates Mesoblast's first product launch
- Broadens market opportunities with new Phase 3 programs
- Facilitates leveraged build-out of infrastructure, skills and expertise needed for commercialising Mesoblast's Mesenchymal Precursor Cell (MPC) products
- Expands reach to Japan through collaboration with JCR Pharmaceuticals
- Acquisition of new intellectual property which is highly complementary to Mesoblast's existing patent estate

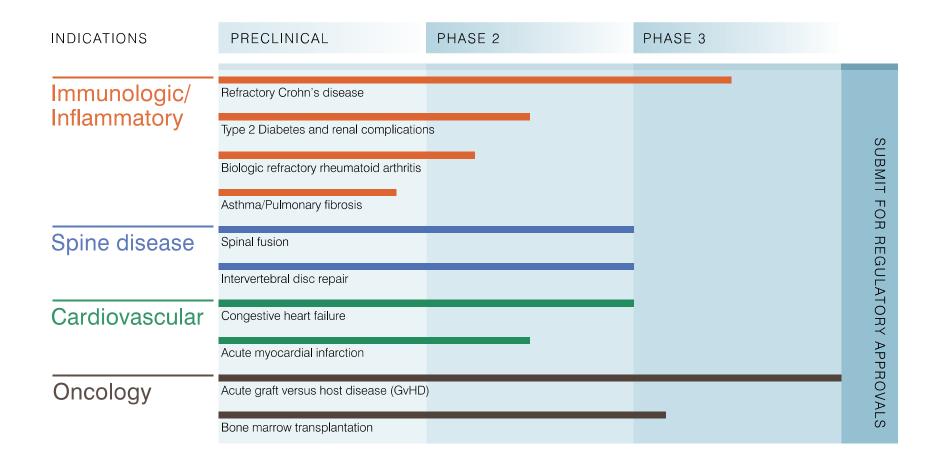


# Benefits to clinical development programs:

- A broadening of Mesoblast's Phase 3-ready products in two new indications:
  - Crohn's disease ongoing, 330-patient, Phase 3 trial in patients with Crohn's disease who have failed other biologic agents.
  - Acute Graft versus Host Disease (aGvHD) promising Phase 3 data in adults with GvHD at high risk of death due to liver or gut complications
- Plan to obtain United States Food and Drug Administration (FDA) approval for Prochymal®, the only stem cell therapeutic designated by the FDA as both an Orphan Drug and Fast Track product.
- Prochymal® has already received conditional approval in Canada and New Zealand for the treatment of children with acute GvHD, and is available in the United States under an Expanded Access Program for treatment of acute GvHD in both children and adults.
- Ownership of Osiris' extensive long-term clinical data from over 1,500 patients treated with cultured MSCs, including safety, efficacy and repeat dosing data.



## Six late-stage clinical trials across four core therapeutic areas





### Oncology product -Prochymal® for acute GvHD

- Allogeneic hematopoietic stem cell transplants (HSCT) are used for the treatment of diseases including hematological malignancies, certain forms of anemia, and immunological deficiencies.
- There are approximately 25,000 HSCTs globally per year
- GVHD is a potentially life threatening complication that arises in approximately 50% of all patients who receive an HSCT.
- GvHD affects the skin, gastrointestinal (GI) tract, and liver
- In patients with liver and gut complications, mortality can reach 85%
- In Phase 3 trials, Prochymal significantly improved overall responses in the adult subset with gut or liver GvHD and resulted in improved survival.
- In Phase 3 trials, Prochymal significantly improved overall responses and survival in children with severe GvHD.



# Inflammatory product - Prochymal® for refractory Crohn's disease

- Crohn's disease (CD) is a chronic inflammatory condition of the gastrointestinal tract
- Over 700,000 people in the US have Crohn's disease, with 20,000 new cases annually
- Approx. 60,000 patients in the US alone are intolerant, unresponsive or refractory to existing biologics (e.g. TNF alpha inhibitors)
- Prochymal<sup>®</sup> has shown benefit in early clinical trials of Crohn's disease
- Prochymal® is currently being evaluated in a 330-patient Phase 3 trial of Crohn's disease patients with moderate-to-severe treatment-refractory disease
- Following an interim analysis for futility, the best performing Prochymal<sup>®</sup> dose (based on the primary end-point of disease remission) was selected to complete this study **Pmeso**blast

# Mesoblast broadens intellectual property estate for mesenchymal lineage cells

The acquired patent portfolio is highly complementary and additive to Mesoblast's existing patent estate covering MPC composition-of-matter and uses in the US, Japan, Europe, China, and other jurisdictions

In addition to Mesoblast's 26 patent families, Mesoblast has acquired from Osiris all 35 additional patent families relating to culture-expanded MSC technology

Specifically, the newly acquired patents comprise –

- 110 granted patents globally comprising:
  - 48 granted US patents
  - 21 granted European patents
  - 9 granted Japanese patents
  - 32 granted patents ROW
- Granted patents through to 2025
- Patent applications out to 2031



### **Transaction terms**

#### Mesoblast pays-

- \$US 20m on transaction close
- \$US 15m in stock payable upon transfer of assigned assets
- \$US 15m six months after close

#### Osiris may receive -

- Up to \$US 50m in milestones contingent on successful achievement of future late-stage clinical or regulatory targets (e.g. US or European product approval)
- Mesoblast has discretion to pay for milestones with cash or stock
- Earnout on sales of acquired products ranging from low single digit to a 10% cap on annual sales > \$US 750m



# Impact of transaction on resources

- Existing cash reserves \$315 million June 2013
- Planned new hires to meet operational requirements
- Increased expenditure on clinical trial costs and manufacturing needs
- Existing funds are sufficient to meet all current and new product development plans
- Cost savings and synergies are expected across personnel, capital expenditure and manufacturing, and as a result there will only be a modest increase in operating cash burn



# Risk-managed business model

- Multiple products, parallel timeframes
- Products specifically target major medical conditions where proprietary technologies offers scientific and clinical advantages
- Strong cash position enables simultaneous development
- Commercial success enhanced via strategic partnerships and growth through profitable manufacturing operations
- Potential to deliver significant and sustainable revenues



# Key target areas for proprietary cell-based technologies

The Company's lead products will use its MPC or MSC platform technologies and continue to focus on four major and distinct areas:

- 1. Systemic diseases of inflammation and immunity
- II. Orthopedic diseases of the spine
- III. Cardiovascular diseases
- IV. Oncology conditions associated with bone marrow transplantation



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Leading the world in adult stem cell therapies