

## MESOBLAST CLINICAL PROGRAM UPDATE FOR MPC-150-IM IN PATIENTS WITH CHRONIC HEART FAILURE

**New York, USA; and Melbourne, Australia; April 18, 2018:** Mesoblast Limited (ASX:MSB; Nasdaq:MESO) today provided an update on the Phase 3 and Phase 2b clinical trials of the Company's proprietary allogeneic mesenchymal precursor cell (MPC) product candidate MPC-150-IM in patients with moderate-to-severe and end-stage advanced chronic heart failure (CHF), respectively. These indications represent major unmet medical needs due to the high rates of morbidity and mortality despite existing therapies.

In the United States alone, there are more than 1.3 million patients with New York Heart Association (NYHA) Class III CHF, 250,000 patients with NYHA Class IV CHF, and 50,000 patients with end-stage CHF, representing a potential multi-billion dollar market opportunity for Mesoblast.

Positive clinical signals have been previously published in Phase 2 trials of Mesoblast's MPC therapy in advanced and end-stage heart failure,<sup>1,2</sup> supporting a common underlying mechanism of action (MOA). The MOA by which MPC-150-IM is thought to exert its effects in these patient populations, based on preclinical evidence, is through reduction of damaging inflammation, maturation of the vasculature, and cardiac repair.

### Phase 3 DREAM-HF Trial Targeting NYHA Class II/III Patients With Moderate-to-Advanced Chronic Heart Failure

- The objectives of this Phase 3 events-driven trial are to evaluate the ability of MPC-150-IM to reduce the primary endpoint of recurrent non-fatal heart failure-related major adverse cardiac events (HF-MACE) in patients with left ventricular dysfunction, as well as delay or prevent disease progression to end-stage HF and terminal cardiac events (TCE), defined as death, left ventricular assist device (LVAD) implantation, or cardiac transplant.
- As reported in April 2017, an interim futility analysis of the recurrent non-fatal HF-MACE primary efficacy endpoint in the DREAM-HF's first 270 patients, as performed by the trial's independent Data Monitoring Committee (DMC), was successful. According to the futility guidelines, the trial met the pre-specified threshold for continuation.
- In April 2018, the DMC for the Phase 3 trial undertook a scheduled review of available data from 465 randomized patients, including the primary and secondary endpoints of HF-MACE and TCEs, and all safety data. The DMC recommended continuation of the trial without modification. Enrollment of this Phase 3 trial is expected to be completed by the end of 2018.

### Phase 2b Trial Targeting End-State Heart Failure Patients with a Left Ventricular Assist Device (LVAD)

- End-stage heart failure is a major unmet medical need, with mortality approaching 50% at one year despite existing medical therapies. While LVAD implantation improves 12-month survival in these patients, fewer than 5,000 patients annually are given potentially life-saving LVADs due to the high risks of increased morbidity, recurrent hospitalizations, and inflammatory complications, including gastrointestinal bleeding, associated with these devices.
- Based on prior Phase 2 trial results which suggested that Mesoblast's MPCs improved native heart function, prolonged time to re-hospitalization and improved early survival in patients with an LVAD, in December 2017 the United States Food and Drug Administration (FDA) granted Mesoblast Regenerative Medicine Advanced Therapy (RMAT) designation for its novel MPC therapy in the treatment of heart failure patients with left ventricular systolic dysfunction and a LVAD.

Mesoblast Limited  
ABN 68 109 431 870  
www.mesoblast.com

Corporate Headquarters  
Level 38  
55 Collins Street  
Melbourne 3000  
Victoria Australia  
T +61 3 9639 6036  
F +61 3 9639 6030

United States Operations  
505 Fifth Avenue  
Third Floor  
New York, NY 10017  
USA  
T +1 212 880 2060  
F +1 212 880 2061

Asia  
20 Biopolis Way  
#05-01 Centros  
Biopreneur 3  
SINGAPORE 138668  
T +65 6570 0635  
F +65 6570 0176

- The RMAT designation under the 21st Century Cures Act aims to expedite the development of regenerative medicine therapies intended for the treatment of serious diseases and life-threatening conditions. The RMAT designation also offers eligibility for priority review. Once the biologics license application (BLA) for a product is approved, the FDA can require various post-approval confirmatory commitments.
- A 159-patient trial, funded by the National Institutes of Health (NIH) and the Canadian Institute of Health Research, and evaluating Mesoblast's investigational MPC cells as add-on therapy to LVADs has completed enrollment with a 12-month data read-out expected in the next quarter (Q3 CY18). The objectives of this Phase 2b trial are to confirm and extend the prior Phase 2 results.
- Mesoblast is actively pursuing a potential accelerated entry pathway for MPC-150-IM into the cardiovascular market that may be provided by RMAT designation.

### About Chronic Heart Failure (CHF)

CHF is a progressive disease and is classified in relation to the severity of the symptoms experienced by the patient. The most commonly used classification system was established by the NYHA and ranges from Class I-II (mild to moderate) to Class III/IV (severe to end-stage). In 2016, more than 15 million patients in the seven major global pharmaceutical markets were estimated to have been diagnosed with CHF<sup>3</sup>. Prevalence is expected to grow 46% by 2030 in the United States alone, affecting more than 8 million Americans<sup>4</sup>. Approximately half of people who develop heart failure die within 5 years of diagnosis<sup>5,6</sup>. Patients with advanced CHF (NYHA Class III or Class IV) have the highest burden of disease, recurrent hospitalizations and mortality. In the United States alone, the NYHA Class III patient population is estimated at 1.3 million patients and the NYHA class IV population at 250,000 patients. There are approximately 50,000 patients with end-stage class IV heart failure who, despite optimal medical therapy, have a one-year mortality exceeding 50%<sup>7</sup>. The only options to increase survival in these patients are the use of LVADs or of heart transplants, the latter limited by donor availability to less than 3000 patients annually<sup>8</sup>. In the United States and worldwide, CHF results in severe economic, social, and personal costs to patients and their respective healthcare systems<sup>6</sup>.

#### References:

1. Perin EC, et al. *Circ Res* 2015;117:576-584
2. Ascheim DD, et al. *Circulation*. 2014;129:2287-2296
3. PharmaPoint: Heart Failure – Global Drug Forecast and Market Analysis to 2025.
4. GlobalData-PharmaPoint (2016): Heart Failure-Global Drug Forecast and Market Analysis to 2025.
5. AHA Statistical Update – Heart Disease and Stroke Statistics-(2017). *Circulation*. 2017;131.
6. Voight J, et al. *Clinl.Cardiol*. 2014;37:312-321
7. Gustafsson G, Rogers JG. *Eur J Heart Failure* 2017;19,595-602
8. Agency for Healthcare Research and Quality: HCUPnet: ICD-9 principal procedure code 27.51 2014.

### About Mesoblast

Mesoblast Limited (ASX:MSB; Nasdaq:MESO) is a global leader in developing innovative cell-based medicines. The Company has leveraged its proprietary technology platform, which is based on specialized cells known as mesenchymal lineage adult stem cells, to establish a broad portfolio of late-stage product candidates. Mesoblast's allogeneic, 'off-the-shelf' cell product candidates target advanced stages of diseases with high, unmet medical needs including cardiovascular conditions, orthopedic disorders, immunologic and inflammatory disorders and oncologic/hematologic conditions.

### Forward-Looking Statements

This announcement includes forward-looking statements that relate to future events or our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. Forward-looking statements should not be read as a guarantee of future performance or results, and actual

Mesoblast Limited  
ABN 68 109 431 870  
www.mesoblast.com

Corporate Headquarters  
Level 38  
55 Collins Street  
Melbourne 3000  
Victoria Australia  
  
T +61 3 9639 6036  
F +61 3 9639 6030

United States Operations  
505 Fifth Avenue  
Third Floor  
New York, NY 10017  
USA  
  
T +1 212 880 2060  
F +1 212 880 2061

Asia  
20 Biopolis Way  
#05-01 Centros  
Biopreneur 3  
SINGAPORE 138668  
  
T +65 6570 0635  
F +65 6570 0176

results may differ from the results anticipated in these forward-looking statements, and the differences may be material and adverse. Forward-looking statements include, but are not limited to, statements about the timing, progress and results of Mesoblast's preclinical and clinical studies; Mesoblast's ability to advance product candidates into, enroll and successfully complete, clinical studies; the timing or likelihood of regulatory filings and approvals; and the pricing and reimbursement of Mesoblast's product candidates, if approved. You should read this press release together with our risk factors, in our most recently filed reports with the SEC or on our website. Uncertainties and risks that may cause Mesoblast's actual results, performance or achievements to be materially different from those which may be expressed or implied by such statements, and accordingly, you should not place undue reliance on these forward-looking statements. We do not undertake any obligations to publicly update or revise any forward-looking statements, whether as a result of new information, future developments or otherwise.

*For further information, please contact:*

Julie Meldrum  
Corporate Communications  
T: +61 3 9639 6036  
E: [julie.meldrum@mesoblast.com](mailto:julie.meldrum@mesoblast.com)

Schond Greenway  
Investor Relations  
T: +1 212 880 2060  
E: [schond.greenway@mesoblast.com](mailto:schond.greenway@mesoblast.com)

**Mesoblast Limited**  
ABN 68 109 431 870  
[www.mesoblast.com](http://www.mesoblast.com)

**Corporate Headquarters**  
Level 38  
55 Collins Street  
Melbourne 3000  
Victoria Australia  
T +61 3 9639 6036  
F +61 3 9639 6030

**United States Operations**  
505 Fifth Avenue  
Third Floor  
New York, NY 10017  
USA  
T +1 212 880 2060  
F +1 212 880 2061

**Asia**  
20 Biopolis Way  
#05-01 Centros  
Biopreneur 3  
SINGAPORE 138668  
T +65 6570 0635  
F +65 6570 0176