

MESOBLAST OUTLINES POTENTIAL PATHWAY TO ACCELERATED MARKET ENTRY FOR MPC-150-IM IN ADVANCED CHRONIC HEART FAILURE

- Advanced chronic heart failure (CHF) in patients with New York Heart Association (NYHA) Class III/IV is a major unmet medical need due to the high rates of morbidity and mortality despite existing therapies
- Mesoblast's proprietary allogeneic cell therapy MPC-150-IM is in late-stage clinical development in two randomized controlled trials which target, respectively, severe and end-stage advanced CHF
- Based on cumulative clinical results to date and the serious and life-threatening nature of this disease, we believe there is a pathway for accelerated entry of this product candidate into the market to provide a paradigm shift in treatment
- Top line results from the trial in end-stage advanced CHF patients are expected in Q1 2018
- There are more than 250,000 NYHA Class IV patients and 1.3 million NYHA Class III patients in the United States alone, representing a potential multi-billion dollar opportunity for Mesoblast

New York, USA; and Melbourne, Australia; August 23, 2017: Mesoblast Limited (ASX:MSB; Nasdaq: MESO) today announced plans to achieve an accelerated market entry of the Company's proprietary allogeneic mesenchymal precursor cell (MPC) product candidate MPC-150-IM in the treatment of patients with the most advanced stages of chronic heart failure (CHF), defined as New York Heart Association (NYHA) stages Class III and Class IV.

Patients with NYHA Class III/Class IV experience high mortality rates, recurrent hospitalizations, and incur substantial cost of care despite maximal existing therapies. We believe that under new regulatory frameworks that recognize the serious and life-threatening nature of advanced CHF, positive results from our ongoing Phase 2b/3 trials in these patients could support accelerated approval for MPC-150-IM and an opportunity to create a paradigm shift in this potential multi-billion dollar market.

MPC-150-IM is being evaluated in two ongoing randomized placebo-controlled Phase 2b/3 trials in patients with either severe or end-stage advanced CHF. The mechanism of action (MOA) by which MPC-150-IM is thought to exert its effects in these patient populations is through immunomodulation and cardiac repair. Positive clinical signals supporting a common underlying MOA have been previously published in Phase 2 trials of Mesoblast's allogeneic MPC therapy in moderate/severe and end-stage heart failure.^[1,2]

Specifically, the ongoing Phase 2b/3 trials in advanced CHF are:

- A Phase 2b multi-center study in 159 NYHA Class III/IV patients who have end-stage advanced CHF is being conducted in North America by a team of researchers within the National Institutes of Health (NIH)-funded Cardiothoracic Surgical Trials Network (CTSN). The trial is also supported by the National Institute of Neurological Disorders and Stroke and the Canadian Institutes for Health Research.

The trial is evaluating the safety and efficacy of MPC-150-IM injected into the native heart muscle of end-stage CHF patients whose circulation is being supported by a left ventricular assist device (LVAD). Given that high rates of mortality and recurrent hospitalizations continue to be seen in end-stage CHF patients even with LVAD implants, this trial has the potential to support an accelerated approval pathway for MPC-150-IM.

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The primary efficacy endpoint of the study is the number of temporary weans from LVAD tolerated over the 6 months post-randomization, indicating strengthening of the native heart muscle. Additional efficacy endpoints include patient survival, adverse events and rehospitalization rates over 12 months.

This trial is expected to complete enrollment shortly, with top-line results for the trial's primary endpoint expected in Q1 2018.

- A Phase 3 multi-center study targeting predominantly advanced CHF patients who have severe left ventricular systolic dysfunction is being conducted in North America in patients who have failed optimal medical care for their cardiac condition.

More than 400 of the anticipated approximately 600 NYHA Class II/III CHF patients have been randomized to date. The trial's primary efficacy endpoint is a comparison of recurrent non-fatal HF-related major adverse cardiac events (HF-MACE) between either MPC-treated patients or sham-treated controls.

In April 2017, Mesoblast announced that a pre-specified interim futility analysis of the efficacy endpoint in this Phase 3 trial was successful in the trial's first 270 patients. After notifying the Company of the interim analysis results, the trial's Independent Data Monitoring Committee stated that it had no safety concerns relating to MPC-150-IM and recommended that the trial should continue as planned. We believe that positive results from this Phase 3 trial in advanced CHF patients would serve to confirm results with MPC-150-IM obtained in end-stage heart failure patients.

About Advanced 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.^[3] Prevalence is expected to grow 46% by 2030 in the United States alone, affecting more than 8 million Americans.^[4] Approximately half of people who develop heart failure die within 5 years of diagnosis.^{[5][6]} 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%.^[7] 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.^[8] CHF causes severe economic, social, and personal costs. In the United States, it is estimated that CHF results in direct costs of \$60.2 billion annually when identified as a primary diagnosis and \$115 billion as part of a disease milieu.⁹

¹ Perin EC, Borow KM, Silva GV, et al. A phase II dose-escalation study of allogeneic mesenchymal precursor cells in patients with ischemic or non-ischemic heart failure. *Circulation Research*. July 2015.

² Ascheim DD, Gelijns AC, Goldstein D, et al. Mesenchymal Precursor Cells as adjunctive therapy in recipients of contemporary LVADs. *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 A Re-Evaluation of the Costs of Heart Failure and its Implications for Allocation of Health Resources in the United States. *Voigt J. Clinl. Cardiol.* 37, 5, 312-321 (2014).

⁷ Gustafsson G, Rogers J. (2017) Left ventricular assist device therapy in advanced heart failure: patient selection and outcomes. *European Journal of Heart Failure* 19, 595-602.

⁸ Agency for Healthcare Research and Quality: HCUPnet: ICD-9 principal procedure code 27.51 2014.

⁹ A Re-Evaluation of the Costs of Heart Failure and its Implications for Allocation of Health Resources in the United States. *Voigt J. Clinl. Cardiol.* 37, 5, 312-321 (2014).

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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 press release 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 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 initiation, timing, progress and results of Mesoblast's preclinical and clinical studies, and Mesoblast's research and development programs; Mesoblast's ability to advance product candidates into, enroll and successfully complete, clinical studies, including multi-national clinical trials; Mesoblast's ability to advance its manufacturing capabilities; the timing or likelihood of regulatory filings and approvals, manufacturing activities and product marketing activities, if any; the commercialization of Mesoblast's product candidates, if approved; regulatory or public perceptions and market acceptance surrounding the use of stem-cell based therapies; the potential for Mesoblast's product candidates, if any are approved, to be withdrawn from the market due to patient adverse events or deaths; the potential benefits of strategic collaboration agreements and Mesoblast's ability to enter into and maintain established strategic collaborations; Mesoblast's ability to establish and maintain intellectual property on its product candidates and Mesoblast's ability to successfully defend these in cases of alleged infringement; the scope of protection Mesoblast is able to establish and maintain for intellectual property rights covering its product candidates and technology; estimates of Mesoblast's expenses, future revenues, capital requirements and its needs for additional financing; Mesoblast's financial performance; developments relating to Mesoblast's competitors and industry; 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.

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