

## **INDEPENDENT DATA MONITORING COMMITTEE INITIATES PROCESS FOR INTERIM ANALYSIS OF MESOBLAST'S PHASE 3 CHRONIC HEART FAILURE TRIAL**

**New York, USA; and Melbourne, Australia; March 31, 2017:** Mesoblast Limited (ASX: MSB; Nasdaq: MESO) today announced that the Independent Data Monitoring Committee (IDMC) for the ongoing Phase 3 trial in chronic heart failure (CHF) has initiated the process for the pre-specified interim futility analysis of the trial's efficacy endpoint.

The interim analysis dataset has been locked and will be analyzed and reviewed by the trial's independent statisticians. Throughout this review process, Mesoblast will remain blinded to individual treatment allocation as well as grouped safety and efficacy data.

The dataset for the interim analysis includes non-fatal and terminal cardiac events from the first 270 of the anticipated 600 patients to be included in this ongoing trial. The IDMC will review and interpret the results of the interim analysis and provide recommendations shortly.

The trial's efficacy endpoint is a comparison of recurrent heart failure-related major adverse cardiac events (HF-MACE) in moderate to advanced CHF patients receiving either MPC-150-IM by catheter injection into the damaged left ventricular heart muscle or sham control. Enrollment in the 1:1 randomized Phase 3 trial is ongoing across multiple study sites in the United States and Canada.

### **About MPC-150-IM and the Unmet Need of Chronic Heart Failure**

MPC-150-IM is Mesoblast's lead allogeneic, cell-based product candidate for the treatment of moderate to advanced chronic heart failure (CHF) due to left ventricular systolic dysfunction. In 2016, more than 15 million patients in the seven major global pharmaceutical markets are estimated to have been diagnosed with CHF.<sup>1</sup> Prevalence is expected to grow 46% by 2030 in the United States alone, affecting more than 8 million Americans.<sup>2</sup>

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 New York Heart Association (NYHA) and ranges from Class I (mild) to Class IV or end stage (severe). Approximately half of people who develop heart failure die within 5 years of diagnosis.<sup>3</sup> Patients with late NYHA Class II or Class III CHF continue to represent a significant unmet medical need despite recent advances in new therapies. 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.<sup>4</sup>

### **About Mesoblast's Phase 3 CHF Trial**

Mesoblast's Phase 3 trial in CHF is a multicenter, double-blind, randomized, scripted sham procedure-controlled, parallel-group study to evaluate the efficacy and safety of MPC-150-IM cell therapy (human bone marrow-derived adult allogeneic mesenchymal precursor cells) in patients with moderate to advanced CHF due to left ventricular systolic dysfunction of either ischemic or non-ischemic etiology who have received optimal medical and coronary revascularization therapy.

<sup>1</sup>GlobalData-PharmaPoint (2016):Heart Failure-Global Drug Forecast and Market Analysis to 2025

<sup>2</sup>AHA Statistical Update – Heart Disease and Stroke Statistics-(2017). Circulation. 2017;131:00-00. DOI: 10.1161/CIR.0000000000000485

<sup>3</sup> 1.Mozzafarian D, Benjamin EJ, Go AS, et al. on behalf of the American Heart Association Statistics Committee and Stroke Statistics Subcommittee. Heart disease and stroke statistics—2016 update: a report from the American Heart Association. Circulation. 2016;133:e38-e360

<sup>4</sup> 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)

## **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 diseases, immune-mediated and inflammatory disorders, orthopedic 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. 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:*

### **CORPORATE COMMUNICATIONS**

Julie Meldrum

T: +61 3 9639 6036

E: [julie.meldrum@mesoblast.com](mailto:julie.meldrum@mesoblast.com)

### **INVESTOR RELATIONS**

Schond Greenway

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