

**PRIMARY ENDPOINT SUCCESSFULLY ACHIEVED IN MESOBLAST'S PHASE 3 CELL THERAPY TRIAL FOR ACUTE GRAFT VERSUS HOST DISEASE**

**New York, USA; and Melbourne, Australia; February 22, 2018:** Mesoblast Limited (ASX: MSB; Nasdaq: MESO) today announced that the Phase 3 trial of its allogeneic mesenchymal stem cell product candidate MSC-100-IV (remestemcel-L) in children with steroid refractory acute Graft versus Host Disease (aGVHD) has successfully met the primary endpoint of Day 28 overall response (OR, complete + partial response) rate.

In the 55 children enrolled in Mesoblast's open-label Phase 3 trial conducted across 32 sites in the United States, the Day 28 OR rate was 69%, a statistically significant increase compared to the protocol-defined historical control rate of 45% (p=0.0003).

Among patients who received at least one treatment infusion and were followed up for 100 days (n=50), the mortality rate was 22%. This is in contrast to Day 100 mortality rates as high as 70% in patients who fail to respond to initial steroid therapy<sup>1,2,3</sup>.

The treatment regimen of remestemcel-L was well tolerated and the incidence of adverse events was consistent with that expected from the underlying disease state and in line with previous remestemcel-L use.

These safety and efficacy results are consistent with Mesoblast's prior experience using remestemcel-L in 241 children treated under an expanded access protocol, where Day 28 OR correlated with Day 100 survival<sup>4</sup>.

The Phase 3 study results were presented today at the tandem annual scientific meetings of the Center for International Blood & Marrow Transplant Research (CIBMTR) and the American Society of Blood and Marrow Transplantation (ASBMT) being held in Salt Lake City from February 21-25, 2018. Full results for this ongoing trial will be provided in CY Q2 2018.

The Phase 3 trial's senior investigator, Dr Joanne Kurtzberg, Jerome Harris Distinguished Professor of Pediatrics and Director of the Pediatric Blood and Marrow Transplant Program at Duke University Medical Center, said: "These children are a very challenging patient population as they suffer from a particularly aggressive and life-threatening disease for which there are currently no available treatments. We are now seeing that children who receive remestemcel-L can have significant overall response rates and reduced early mortality.

"We are delighted that Mesoblast has attained such an important milestone towards delivering a potentially effective treatment for this very serious and life threatening condition."

There are currently no products approved in the United States for treatment of steroid-refractory aGVHD. Given the serious nature of this condition, in 2017 the United States Food and Drug Administration (FDA) granted Mesoblast Fast Track designation for the use of remestemcel-L to achieve improved overall response rate in children with aGVHD.

Based on interactions with the FDA, Mesoblast believes that successful results from the completed Phase 3 trial, together with Day 180 safety and quality of life parameters in these patients, may provide sufficient clinical evidence for filing for accelerated approval of remestemcel-L in the United States. The Phase 3 trial is being conducted under a FDA Investigational New Drug Application (NCT#02336230).

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Mesoblast Chief Executive Dr Silviu Itescu stated: "These are tremendous results that show the potential of our cell therapies to make a substantial difference in the treatment of patients with serious and life threatening diseases. They are a testament to the capabilities and expertise of the entire clinical, regulatory and manufacturing teams at Mesoblast."

### About Graft Versus Host Disease

Mesoblast is developing MSC-100-IV for the treatment of aGVHD following an allogeneic bone marrow transplant (BMT). In patients who have received a BMT, donor cells may attack the recipient (the person receiving the transplant), causing aGVHD, resulting in activation of pro-inflammatory T-cells and tissue damage in the skin, gut and liver. This condition, when severe and unresponsive to initial steroid therapy, is often fatal. According to the Center for International Blood and Marrow Transplant Research, there are approximately 30,000 allogeneic BMTs globally per year for diseases including hematological cancers, with approximately 20%<sup>5</sup> of all cases in the pediatric population. Approximately 50%<sup>6</sup> of all allogeneic BMT patients develop aGVHD. Liver or gastrointestinal involvement occur in up to 60%<sup>6</sup> of all patients with aGVHD and are associated with the greatest risk of death, with mortality rates of up to 85%.

<sup>1</sup> MacMillan ML, DeFor TE, Weisdorf DJ. The best endpoint for acute GVHD treatment trials. *Blood*. 2010; 115 (26): 5412-5417.

<sup>2</sup> MacMillan ML, Couriel D, Weisdorf DJ, et al. A phase 2/3 multicenter randomized clinical trial of ABX-CBL versus ATG as secondary therapy for steroid-resistant acute graft-versus-host disease. *Blood*. 2017; 109 (6): 2657-2662.

<sup>3</sup> Pidala J, Kim J, Field T, et al. Infliximab for managing steroid-refractory acute graft-versus-host disease. *Biol Blood Marrow Transplant*. 2009; 15 (9): 1116-1121.

<sup>4</sup> Kurtzberg J. et al. Effect of Human Mesenchymal Stem Cells (Remestemcel-L) on Clinical Response and Survival Confirmed in a Large Cohort of Pediatric Patients with Severe High-Risk Steroid-Refractory Acute Graft Versus Host Disease. *BBMT*. 2016; 22.

<sup>5</sup> CIBMTR 2015 Volume Data Set

<sup>6</sup> Jagasia, M., Arora, M., Flowers, M. (2012) Risk Factors for acute GVHD and Survival after Hematopoietic Cell Transplantation. *Blood*, 5 January (119):296-307

### Webcast

Mesoblast will host a webcast to discuss these results on Thursday, February 22 2018 at 4.30pm EST; Friday, February 23 2018 at 8.30am AEDT.

The live webcast can be accessed via

<http://webcasting.brrmedia.com/broadcast/5a84e2569677b06417edf8f5>

To access the call only, dial 1 855 881 1339 (U.S.), 1 800 558 698 (toll-free Australia) or +61 2 9007 3187 (outside of the U.S. and Australia). The conference identification code is 471187.

The archived webcast will be available on the Investor page of the Company's website – [www.mesoblast.com](http://www.mesoblast.com)

### 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 results may differ from the results anticipated in these forward-looking statements, and the

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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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