

KEY DAY 100 SURVIVAL OUTCOMES OF PHASE 3 TRIAL FOR ACUTE GRAFT VERSUS HOST DISEASE PRESENTED AT 2018 ISSCR ANNUAL MEETING

New York, USA; June 20, 2018; and Melbourne, Australia; June 21, 2018: Mesoblast Limited (ASX:MSB; Nasdaq:MESO) today announced key Day 100 survival outcomes of its Phase 3 trial for remestemcel-L, an allogeneic mesenchymal stem cell product candidate, in children with steroid refractory acute Graft Versus Host Disease (aGVHD). The results are being presented today at the 2018 annual meeting of the International Society for Stem Cell Research (ISSCR), being held in Melbourne from June 20-23.

The open-label Phase 3 trial enrolled 55 children with steroid-refractory aGVHD (aged between two months and 17 years) in 32 sites across the United States, with 89% of patients suffering from the most severe form, grade C/D aGVHD. The trial was performed under an United States Food and Drug Administration (FDA) Investigational New Drug Application (NCT#02336230).

This trial previously met the primary endpoint of Day 28 overall response rate (69% versus 45% historical control rate, p=0.0003). Top line Day 100 results demonstrated 87% survival rate for Day 28 responders to remestemcel-L treatment (33/38), and an overall survival rate of 75% (41/55). The multi-infusion regimen of remestemcel-L was well tolerated.

Acute GVHD is associated with significant morbidity and is a leading cause of mortality after allogeneic hematopoietic stem cell transplantation for blood cancers or other conditions. Severe aGVHD (determined by grade C/D, liver or gut involvement, or high risk stratification) has the highest risk of failure to first-line corticosteroids and high transplant-related mortality¹. Day 100 mortality can reach 70% in patients who fail to respond to initial steroid therapy²⁻⁴, and 12-month mortality approaches 90%⁵.

Mesenchymal stem cells have anti-inflammatory and immunomodulatory biological activity that supports their investigational use in aGVHD⁶. The immunomodulatory actions of these cells are triggered through receptor activation by inflammation, resulting in regulation of multiple cellular arms of the immune system that are central to aGVHD pathogenesis.

Based on interactions with the FDA, Mesoblast believes that successful results from the completed Phase 3 trial, together with Day 180 safety, survival and quality of life parameters in these patients, may provide sufficient clinical evidence to file for accelerated approval of remestemcel-L in the United States, where there are currently no approved products for steroid-refractory aGVHD.

Mesoblast Chief Executive Dr Silviu Itescu said, "It is wonderful to see that our unique cellular therapy has shown such promising survival rates at Day 100 in children suffering from this devastating disease. Our objective is to bring this new therapy to market, and make it available to patients who are in desperate need with this life-threatening complication of an allogeneic bone marrow transplant."

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2. MacMillan ML, DeFor TE, Weisdorf DJ. The best endpoint for acute GVHD treatment trials. *Blood*. 2010; 115 (26): 5412-5417.
3. 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*. 2007; 109 (6): 2657-2662.
4. 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.
5. Arai S et al, Poor outcome in steroid refractory graft versus host disease with anti-thymocyte globulin treatment. *Biol Blood Marrow Transplant*. 2002; 8: 155-160.
6. Aggarwal S, Pittenger MF. Human mesenchymal stem cells modulate allogeneic immune responses. *Blood*. 2005; 105:1815-22.

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About Mesoblast

Mesoblast Limited (Nasdaq:MESO; ASX:MSB) is a global leader in developing innovative cell-based medicines. Through a proprietary process, Mesoblast selects highly purified mesenchymal lineage precursor and stem cells from the bone marrow of healthy adults, and creates master cell banks which can be industrially expanded to produce thousands of doses from each donor that meet stringent release criteria, have lot to lot consistency, and can be used off the shelf without the need for tissue matching.

The Company has leveraged its proprietary technology platform to establish a broad portfolio of late-stage product candidates. Mesoblast's allogeneic, 'off-the-shelf' cell product candidates are being evaluated in their ability to 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 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.

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