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MESOBLAST SUBMITS CLINICAL EFFICACY AND SAFETY DATA TO FDA IN ROLLING BIOLOGICS LICENSE APPLICATION FOR REMESTEMCEL-L

Melbourne, Australia; January 2, 2020; and New York, USA; January 1, 2020: Mesoblast Limited (ASX:MSB; Nasdaq: MESO), global leader in cellular medicines for inflammatory diseases, announced that the United States Food and Drug Administration (US FDA) has confirmed receipt of Mesoblast's filing of clinical efficacy and safety data for remestemcel-L in its rolling Biologics License Application (BLA) for the treatment of children with steroid-refractory acute graft versus host disease (SR-aGVHD). The final module will be filed during January, and Mesoblast will request an expedited FDA review of the BLA under the product candidate's existing Fast Track designation. If approved, remestemcel-L is planned to be launched in the US in 2020.

The clinical submission included analyses of 309 children with SR-aGVHD who have received remestemcel-L across three separate studies. In addition, Mesoblast provided new data in control pediatric subjects from the contemporaneous database of the Mount Sinai Acute GVHD International Consortium (MAGIC) to provide an unbiased and independent estimate of response rates and outcomes in matched pediatric control patients treated with institutional standard of care.

The results of the comparative analysis between Mesoblast's open-label Phase 3 study and contemporaneous controls receiving institutional standard of care demonstrate the effectiveness of remestemcel-L in this patient population, with particular efficacy and survival benefit in patients with the most severe forms of aGVHD. These conclusions are supported by prior results from an Expanded Access Program in 241 children where remestemcel-L was used as salvage therapy after failure of steroids and other agents.

Acute GVHD is a potentially life-threatening condition which occurs in about 50% of patients who receive an allogeneic bone marrow transplant. Over 30,000 patients worldwide receive an allogeneic bone marrow transplant, primarily during treatment for blood cancers, and 20-25% are children[1]. Mortality at 12 months is as high as 90% in those with aGVHD and Grade C/D or III/IV disease severity[2,3], and there are no approved treatments in the US for children under 12.

In Mesoblast's open-label Phase 3 trial of remestemcel-L in 55 children with SR-aGVHD, 89% of whom had Grade C/D disease, the primary endpoint of Day 28 Overall Response in those exposed to remestemcel-L was achieved in 70% and Day 100 survival was 75%. These outcomes were superior to those from a cohort of 30 pediatric patients with SR-aGVHD from the MAGIC consortium matched for inclusion criteria and disease severity (80% Grade C/D). In the MAGIC controls, Day 28 Overall Response was 43% and Day 100 survival was 57%.

Mesoblast Chief Medical Officer Dr Fred Grossman said: "We are pleased to have submitted to the FDA clinical efficacy and safety data for remestemcel-L, as well as comparative clinical outcome data from contemporaneous controls. We are working closely with the FDA to make our cellular medicine available and improve outcomes in children with this devastating condition."

About Remestemcel-L
Mesoblast's lead product candidate, remestemcel-L, is an investigational therapy comprising culture-expanded mesenchymal stem cells derived from the bone marrow of an unrelated donor. It is administered to patients in a series of intravenous infusions. Remestemcel-L is believed to have immunomodulatory properties to counteract the inflammatory processes that are implicated in aGVHD by down-regulating the production of pro-inflammatory cytokines, increasing production of anti-inflammatory cytokines, and enabling recruitment of naturally occurring anti-inflammatory cells to involved tissues.

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2. Westin, J., Saliba, RM., Lima, M. (2011) Steroid-refractory acute GVHD: predictors and outcomes. Advances in Hematology.
3. Axt L, Naumann A, Toennies J (2019) Retrospective single center analysis of outcome, risk factors and therapy in steroid refractory graft-versus-host disease after allogeneic hematopoietic cell transplantation. Bone Marrow Transplantation.

About Mesoblast
Mesoblast Limited (Nasdaq: MESO; ASX: MSB) is a world leader in developing allogeneic (off-the-shelf) cellular medicines. The Company has leveraged its proprietary cell therapy technology platform to establish a broad portfolio of commercial products and late-stage product candidates. Two products have been commercialized in Japan and Europe by its licensees, and it has established commercial partnerships in Europe and China for certain Phase 3 assets. In the United States, Mesoblast has initiated submission of a rolling Biologics License Application to the FDA to seek approval of its product candidate for acute graft versus host disease following a successful Phase 3 trial, and is completing Phase 3 trials for its advanced heart failure and chronic low back pain product candidates. Mesoblast's proprietary manufacturing process yields industrial-scale, frozen, off-the-shelf, cellular medicines based on its mesenchymal lineage cell platform technology. These cell therapies, with defined pharmaceutical release criteria, are planned to be readily available to patients worldwide. Mesoblast has locations in Melbourne, New York, Singapore and Texas and is listed on the Australian Securities Exchange (MSB) and on the Nasdaq (MESO). For more information, please see www.mesoblast.com, LinkedIn: Mesoblast Limited and Twitter: @Mesoblast

Mesoblast's 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.

Release authorized by the Chief Executive.

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