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REMESTEMCEL-L REDUCES INFLAMMATORY BIOMARKERS PREDICTIVE OF HIGH MORTALITY IN ACUTE GRAFT VERSUS HOST DISEASE

Biomarker Study Results Presented at the 62nd American Society of Hematology Annual Meeting

Melbourne, Australia; December 7 and New York, USA; December 6, 2020: Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in allogeneic cellular medicines for inflammatory diseases, today announced results presented at the 62nd annual meeting American Society of Hematology (ASH), which provide *in vivo* biomarker evidence linking remestemcel-L's immunomodulatory activity to survival outcomes in children with steroid-refractory acute graft versus host disease (SR-aGVHD). The results were presented on December 6, 2020 by the Phase 3 trial's lead investigator and pediatric transplant physician, Dr Joanne Kurtzberg, the Jerome Harris Distinguished Professor of Pediatrics and Professor of Pathology, and Director, Pediatric Blood and Marrow Transplant Program at Duke University Medical Center.

Key conclusions were:

- Clinically meaningful overall responses and survival in children with SR-aGVHD treated with remestemcel-L were associated with significant reductions in certain biomarkers of inflammation which have been validated as predictors of mortality risk
- These biomarkers provide evidence of *in vivo* bioactivity of remestemcel-L in pediatric SR-aGVHD, where children under 12 are at high-risk for mortality, with no approved therapies in the United States
- The durable reductions in blood levels of certain biomarkers associated with inflammatory diseases of the gut suggest that these could be more generally reflective of remestemcel-L activity *in vivo* in other inflammatory bowel diseases such as Crohn's disease and ulcerative colitis

Blood levels of soluble suppression of tumorigenicity 2 (ST2)[1,2] and MAGIC Biomarker Score (MBS)[3,4], validated biomarkers that predict high mortality in SR-aGVHD and active gut inflammation more broadly, were measured at baseline and sequentially over 180 days in 40 of the 54 children with SR-aGVHD who received at least four weeks of remestemcel-L treatment in the single-arm Phase 3 trial. Both the elevated baseline levels of ST2 and MBS were significantly reduced after remestemcel-L treatment at Days 100, 160 and 180 (all timepoints $p<0.001$ for both markers). This was accompanied by significant reductions in activated circulating T cells. Day 100 survival was 74% in the 54 remestemcel-L children with SR-aGVHD (89% with Grade C/D disease), which compares very favourably with a mortality approaching 70-90% in children of similar severity treated with other therapies.

Dr Kurtzberg said: "These results support the bioactivity of remestemcel-L in treating the severe inflammation in children with acute graft versus host disease refractory to steroids and provide evidence linking the immunomodulatory properties of remestemcel-L with the excellent responses and survival we see when treating these desperately ill children."

About Remestemcel-L
Remestemcel-L is an investigational therapy comprising culture-expanded mesenchymal stromal cells derived from the bone marrow of an unrelated donor. Remestemcel-L is thought to have immunomodulatory properties to counteract the cytokine storms that are implicated in various inflammatory conditions by downregulating the production of pro-inflammatory cytokines, increasing production of anti-inflammatory cytokines, and enabling recruitment of naturally occurring anti-inflammatory cells to involved tissues.

About Mesoblast
Mesoblast Limited (ASX:MSB; Nasdaq:MESO) is a world leader in developing allogeneic (off-the-shelf) cellular medicines. The Company has leveraged its proprietary mesenchymal lineage cell therapy technology platform to establish a broad portfolio of commercial products and late-stage product candidates. Mesoblast has a strong and extensive global intellectual property portfolio with protection extending through to at least 2040 in all major markets. The Company's proprietary manufacturing processes yield industrial-scale, cryopreserved, off-the-shelf, cellular medicines. These cell therapies, with defined pharmaceutical release criteria, are planned to be readily available to patients worldwide. For more information, please see www.mesoblast.com, LinkedIn: Mesoblast Limited and Twitter: @Mesoblast

References
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4. Major-Monfried H et al. Blood. 2018;131(25):2846-2855.

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. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "likely," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions and variations thereof. 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. The risks, uncertainties and other factors that may impact our forward-looking statements include, but are not limited to: 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; 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. 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. Unless required by law, 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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