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the regenerative medicine company

# CLINICALLY MEANINGFUL OUTCOMES USING REMESTEMCEL-L IN PATIENTS WITH CHRONIC GRAFT VERSUS HOST DISEASE

**Melbourne, Australia, February 20, 2020 and New York, USA, February 19, 2020:** Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in cellular medicines for inflammatory diseases, today announced that the investigator-initiated expanded access protocol using its cryopreserved allogeneic cell therapy product candidate remestemcel-L for steroid-refractory chronic graft versus host disease (chronic GVHD) has resulted in clinically meaningful outcomes in all three treated patients, two children and one adult, within 28 days after two infusions. On the basis of these outcomes, the investigator-initiated collaboration will be expanded to evaluate remestemcel-L in a pivotal trial for chronic GVHD.

Lead investigator Dr Joanne Kurtzberg, Jerome Harris Distinguished Professor of Pediatrics and Professor of Pathology, and Director, Pediatric Blood and Marrow Transplant Program at Duke University Medical Center, said: "We are delighted with these initial efficacy outcomes using remestemcel-L as first-line therapy in steroid-refractory chronic graft versus host disease, where there is a major unmet medical need for a safe and effective therapy."

Chronic GVHD occurs in 30-70% of recipients of an allogeneic bone marrow transplant.[1,2] Over 30,000 patients worldwide undergo an allogeneic bone marrow transplant annually, primarily during treatment for blood cancers, and these numbers are increasing.[3] In both the chronic and acute forms of GVHD, the donated bone marrow stem cells view the recipient's body as foreign, and attack the body causing significant morbidity and mortality. Acute GVHD usually manifests within 100 days following a transplant while chronic GVHD generally manifests later (>100 days), and the two may occur separately or within the same patient. The prevalence of chronic GVHD in the US is over 14,000 patients, with an estimated annual patient medical cost of approximately US$300,000.[4]

**About Remestemcel-L**

Remestemcel-L is an investigational therapy being developed for a range of rare diseases. The product candidate comprises culture-expanded mesenchymal stem cells derived from the bone marrow of an unrelated donor. Remestemcel-L is believed to have immunomodulatory properties to counteract the inflammatory processes that are implicated in steroid-refractory GVHD 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.

**References**

1. Arai et al. Biol Blood Marrow Transplant. 2015; 21(2): 266–274.
2. Grube et al. Biol Blood Marrow Transplant: 2016; 22 (10): 1781-179.
3. Niederwieser D, Baldomero H, Szer J. (2016) Hematopoietic stem cell transplantation activity worldwide in 2012 and a SWOT analysis of the Worldwide Network for Blood and Marrow Transplantation Group including the global survey.
4. Bachier C, Aggarwal S, Hennegan K (2019) Epidemiology and Real-World Treatment of Chronic Graft-Versus-Host Disease Post Allogeneic Hematopoietic Cell Transplantation: A US Claims Analysis. Blood 2019; 134:Supplement 1.

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**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 platforms to establish a broad portfolio of commercial products and late-stage product candidates. Mesoblast's proprietary manufacturing process yields 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.

Mesoblast has filed a Biologics License Application to the United States Food and Drug Administration (FDA) to seek approval of its product candidate Ryoncil™ (remestemcel-L) for steroid-refractory acute graft versus host disease (acute GvHD). Remestemcel-L is also being developed for other rare diseases. Mesoblast is completing Phase 3 trials for its rexlemestrocel product candidates for advanced heart failure and chronic low back pain. If approved, RYONCIL is expected to be launched in the United States in 2020 for pediatric steroid-refractory acute GVHD. Two products have been commercialized in Japan and Europe by Mesoblast's licensees, and the Company has established commercial partnerships in Europe and China for certain Phase 3 assets.

Mesoblast has locations in Australia, the United States and Singapore 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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