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REMESTEMCEL-L IMPROVES SURVIVAL OF CHILDREN WITH BIOMARKERS FOR HIGHEST MORTALITY IN STEROID REFRACTORY ACUTE GVHD

Melbourne, Australia; October 18, and New York, USA; October 17, 2021: Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in allogeneic cellular medicines for inflammatory diseases, today announced that results published in the latest issue of the peer-reviewed journal *Bone Marrow Transplantation*[1] showed that children with steroid-refractory acute graft versus host disease (SR-aGVHD) and biomarkers predictive for highest mortality had 64% survival when treated with remestemcel-L compared with only 10% survival when treated with other available therapies, including ruxolitinib or other biologics.

The study's senior author and expert in the predictive biomarkers, Dr. James Ferrara, Ward-Coleman Chair in Cancer Medicine, Professor and Director Hematologic Malignancies Translational Research Center at The Icahn School of Medicine at Mount Sinai Hospital, said: "The findings support and extend recent studies that children with severe, SR acute GVHD benefit from remestemcel-L therapy."

These data provide further support for the proposed anti-inflammatory mechanism of action of remestemcel-L and its immunomodulatory activity in patients with SR-aGVHD, resulting in improved survival outcomes. At its upcoming scheduled meeting with FDA's Office of Tissue and Advanced Therapies (OTAT), Mesoblast will address the appropriateness of potency assays related to remestemcel-L's proposed anti-inflammatory mechanism of action as well as the outstanding chemistry, manufacturing and controls (CMC) items which could support a resubmission of the current Biologics License Application (BLA) for remestemcel-L in the treatment of SR-aGVHD in children.

Summary of the study outcomes

The study compared outcomes in 25 children from Mesoblast's Phase 3 trial of remestemcel-L in SR-aGVHD with 27 closely matched children from the Mount Sinai Acute GVHD International Consortium (MAGIC)[2] who participated in a prospective natural history study and were matched for the Phase 3 trial entry criteria. The objective of the study was to evaluate whether outcomes differed according to treatment with remestemcel-L vs other therapies in children at highest risk of death, namely those with baseline MAGIC Algorithm Probability (MAP) biomarker levels ≥0.291, a level predictive of very high mortality and poor responses to therapy in SR-aGVHD. MAP combines the serum concentrations of two biomarkers, Reg3α and ST2, into a single value that predicts long-term outcomes and significant GI tract damage.

MAP levels ≥0.291 were present in 48% of remestemcel-L treated children (12/25) and 37% of the MAGIC cohort (10/27). Treatment with remestemcel-L resulted in 67% Day 28 Overall Response and 64% Day 180 overall survival compared with 10% Day 28 Overall Response and 10% Day 180 survival in the MAGIC cohort (both p=0.01) when treated with various biologics, including ruxolitinib. These results extend previous observations showing that children who achieved clinically meaningful responses and survival after treatment with remestemcel-L had significant reductions in the ST2 biomarker of inflammation, consistent with healing of the GI tract.[3]

About Steroid-Refractory Acute Graft Versus Host Disease (SR-aGVHD)
GVHD is a severe inflammation in the bloodstream caused by complications of bone marrow transplants. The disease occurs in up to 50% of the 30,000 patients who receive an allogeneic bone marrow transplant each year, primarily during treatment for blood cancers. In patients with the most severe form of GVHD, mortality can be as high as 90%. There are no therapies approved for treating SR-aGVHD in children under the age of 12.

About Mesoblast
Mesoblast is a world leader in developing allogeneic (off-the-shelf) cellular medicines for the treatment of severe and life-threatening inflammatory conditions. The Company has leveraged its proprietary mesenchymal lineage cell therapy technology platform to establish a broad portfolio of late-stage

product candidates which respond to severe inflammation by releasing anti-inflammatory factors that counter and modulate multiple effector arms of the immune system, resulting in significant reduction of the damaging inflammatory process.

Mesoblast has a strong and extensive global intellectual property portfolio with protection extending through to at least 2041 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.

Mesoblast has completed Phase 3 trials of rexlemestrocel-L for advanced chronic heart failure and chronic low back pain. Remestemcel-L is being developed for inflammatory diseases in children and adults including steroid refractory acute graft versus host disease and moderate to severe acute respiratory distress syndrome. 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

Footnotes

1. Kasikis S., et al. Mesenchymal stromal cell therapy induces high responses and survival in children with steroid refractory GVHD and poor risk. *Bone Marrow Transplantation* 2021; https://doi.org/10.1038/s41409-021-01442-3
2. Mount Sinai Acute GVHD International Consortium (MAGIC) - a group of ten BMT centers throughout the US and Europe whose purpose is to conduct ground-breaking clinical trials in GVHD, including developing informative biorepositories that assist in developing treatments that can guide GVHD therapy
3. Presented at the annual meeting of the American Society of Hematology (ASH) 2020

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, including our intention to discuss a regulatory pathway with the FDA, 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; whether the FDA agrees to a regulatory pathway; 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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