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REMESTEMCEL-L CLINICAL AND POTENCY STUDIES PRESENTED AT PREMIER MEETING OF TRANSPLANTATION RESEARCH AND THERAPY

Melbourne, Australia; February 17 and New York, USA; February 16, 2023: Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in allogeneic cellular medicines for inflammatory diseases, today announced that two studies on the remestemcel-L development program for the treatment of children with steroid-refractory acute graft versus host disease (SR-aGVHD) were selected by peer review to be presented at the 2023 Tandem Meetings of the American Society for Transplantation and Cellular Therapy (ASTCT) and the Center for Blood and Marrow Transplant Research (CIBMTR), taking place February 15-19 in Orlando, Florida.

The studies are titled "The Immunomodulatory Activity of Remestemcel-L on T Cell Activation in vitro is a Direct Measure of Product Potency and Correlates with Clinical Outcomes in Pediatric Patients with Steroid-Refractory Acute GVHD" and "Long-Term Survival in Children Treated with Remestemcel-L for SR-aGVHD". The lead author of each study is Dr Joanne Kurtzberg, Jerome Harris Distinguished Professor of Pediatrics and Professor of Pathology, Duke University Medical Center. The data from both studies formed key components of Mesoblast's recent resubmission of its remestemcel-L Biologics License Application (BLA) to U.S. Food and Drug Administration (FDA) for children with SR-aGVHD.

Survival outcomes have not improved over the past two decades for the most severe forms of SR-aGVHD, a life-threatening complication of an allogeneic bone marrow transplant following treatment for blood cancers and other conditions.[1-3] The lack of any approved treatments for children under 12 means that there is an urgent need for a therapy that improves the dismal survival outcomes. If remestemcel-L receives FDA approval, it will be the first allogeneic "off-the-shelf" cellular medicine to be approved in the United States, and the first therapy for children under 12 years old with SR-aGVHD.

About Steroid-Refractory Acute Graft Versus Host Disease
Acute GVHD occurs in approximately 50% of patients who receive an allogeneic bone marrow transplant (BMT). Over 30,000 patients worldwide undergo an allogeneic BMT annually, primarily during treatment for blood cancers, including about 20% in pediatric patients.[4,5] SR-aGVHD is associated with mortality as high as 90% and significant extended hospital stay costs.[6,7] There are currently no FDA-approved treatments in the US for children under 12 with SR-aGVHD.

About Remestemcel-L
Mesoblast's lead product candidate, Remestemcel-L, is an investigational therapy comprising culture expanded mesenchymal stromal 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 SR-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.

The original BLA submission contained clinical outcomes of 309 children with SR-aGVHD treated with remestemcel-L showing consistent treatment responses and survival across three separate trials. The data were reviewed by the agency's panel of the Oncologic Drugs Advisory Committee (ODAC) which voted in favor 9:1 that the available data support the efficacy of remestemcel-L in pediatric patients with SR-aGVHD.

The BLA resubmission now contains additional clinical and biomarker data, including from a propensity-matched study of children with high-risk disease, based on the validated MAP biomarker score, comparing outcomes in 25 children from Mesoblast's Phase 3 trial and 27 control children treated with various biologics, including ruxolitinib, from the Mount Sinai Acute GvHD International Consortium (MAGIC) database. The study showed that 67% of high-risk children treated with remestemcel responded positively to treatment within 28 days and were alive after 180 days compared to just 10% in both categories in the MAGIC group.

The BLA resubmission also contains results of a 4-year survival study performed by the Center for International Blood and Marrow Transplant Research (CIBMTR) on 51 evaluable patients with SR-aGVHD who were enrolled in the Phase 3 trial. The results demonstrated durability of the early day 180 survival benefits, with 63% survival at 1 year and 51% at 2 years in a group of children with predominantly grade C/D disease (89%) and with expected 2 year survival of just 25-38% using best available therapy.[1,8-9]

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 is developing product candidates for distinct indications based on its remestemcel-L and rexlemestrocel-L allogeneic stromal cell technology platforms. Remestemcel-L is being developed for inflammatory diseases in children and adults including steroid refractory acute graft versus host disease, biologic-resistant inflammatory bowel disease, and acute respiratory distress syndrome. Rexlemestrocel-L is in development for advanced chronic heart failure and chronic low back pain. 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

References / Footnotes

1. Rashidi A et al. Outcomes and predictors of response in steroid-refractory acute graft-versus-host disease: single-center results from a cohort of 203 patients. *Biol Blood Bone Marrow Transplant* 2019; 25(11):2297-2302.
2. Berger M, Pessolano R, Carraro F, Saglio F, Vassallo E, Fagioli F. Steroid-refractory acute graft-versus-host disease graded III-IV in pediatric patients. A mono-institutional experience with a long-term follow-up. *Pediatric Transplantation*. 2020; 24(7):e13806
3. Biavasco F, Ihorst G, Wasch R, Wehr C, Bertz H, Finke J, Zeiser R. Therapy response of glucocorticoid-refractory acute GVHD of the lower intestinal tract. *Bone Marrow Transplantation.* 2022
4. 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.
5. HRSA Transplant Activity Report, CIBMTR, 2019
6. Westin, J., Saliba, RM., Lima, M. (2011) Steroid-refractory acute GVHD: predictors and outcomes. *Advances in Hematology*.
7. 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*.
8. MacMillan ML et al. Pediatric acute GVHD: clinical phenotype and response to upfront steroids. *Bone Marrow Transplant* 2020; 55(1): 165-171
9. Zeiser R et al. Ruxolitinib for Glucocorticoid-Refractory Acute Graft-versus-Host Disease. *N Engl J Med* 2020;382:1800-10.

Forward-Looking Statements

This press release 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 initiation, timing, progress and results of Mesoblast's preclinical and clinical studies, and Mesoblast's research and development programs; Mesoblast's ability to advance product candidates into, enroll and successfully complete, clinical studies, including multi-national clinical trials; Mesoblast's ability to advance its manufacturing capabilities; the timing or likelihood of regulatory filings and approvals, manufacturing activities and product marketing activities, if any; the commercialization of Mesoblast's product candidates, if approved; regulatory or public perceptions and market acceptance surrounding the use of stem-cell based therapies; the potential for Mesoblast's product candidates, if any are approved, to be withdrawn from the market due to patient adverse events or deaths; the potential benefits of strategic collaboration agreements and Mesoblast's ability to enter into and maintain established strategic collaborations; 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; the scope of protection Mesoblast is able to establish and maintain for intellectual property rights covering its product candidates and technology; estimates of Mesoblast's expenses, future revenues, capital requirements and its needs for additional financing; Mesoblast's financial performance; developments relating to Mesoblast's competitors and industry; 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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