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CHILDREN TREATED WITH REMESTEMCEL-L SHOW LONG-TERM SURVIVAL THROUGH FOUR YEARS IN STEROID-REFRACTORY ACUTE GRAFT VERSUS HOST DISEASE (SR-aGVHD)

Key Points:

- Long-term survival evident through 4 years in children treated with remestemcel-L in Phase 3 trial MSB-GVHD001
- Overall survival at 2 years was 51% in remestemcel-L treated children and 25-38% in recently published studies of children or adults with SR-aGVHD who received best available therapy (BAT) or the only approved agent in adults[1-4]
- These results reaffirm the potential significance of remestemcel-L as a life-saving therapy for children with SR-aGVHD
- These long-term survival outcomes are a key component of the Biologics License Application (BLA) resubmission to the United States Food and Drug Administration (FDA)

Melbourne, Australia; November 23 and New York, USA; November 22, 2022: Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in allogeneic cellular medicines for inflammatory diseases, today announced top-line long-term survival results for remestemcel-L from its pivotal Phase 3 trial (GVHD-001) in children with steroid-refractory acute graft-versus-host disease (SR-aGVHD). The results showed durable survival through 4 years of follow-up. These new long-term survival data are a key component of the company's BLA resubmission to the FDA for remestemcel-L in the treatment of children with SR-aGVHD, a life-threatening condition with no approved treatments for children under 12 years.

"These exciting long-term results provide further evidence of remestemcel-L's potential as a highly effective treatment for SR-aGVHD in children" said Dr. Joanne Kurtzberg, Jerome Harris Distinguished Professor of Pediatrics and Professor of Pathology, Duke University Medical Center, the Phase 3 trial's principal investigator. "Responses are durable, reducing mortality of this often lethal complication of hematopoietic stem cell transplantation."

A four-year observational cohort survival study was performed by the Center for International Blood and Marrow Transplant Research (CIBMTR) on 51 evaluable children with SR-aGVHD who were enrolled in Mesoblast's phase 3 clinical trial of remestemcel-L across 20 centers in the US.

"CIBMTR is proud that the high-quality comprehensive data included in our database supports critical clinical advances such as this to improve outcomes for cellular therapy patients" said Patricia Steinert, PhD, MBA, Executive Scientific Director, CIBMTR MCW Associate Professor, Department of Medicine Center for International Blood & Marrow.

Overall survival in the remestemcel-L cohort was 63% at 1 year, 51% at 2 years, and 49% at 4 years, with median survival of 2 to 3 years. In recently published studies of children or adults with SR-aGVHD who received best available therapy (BAT) or the only FDA-approved agent for adults, ruxolitinib, 1 year survival was 40-49% and 2 year survival was 25%-38%,[1-4] with median survival between 6.5 months and 11.1 months.[3]

Moreover, in the observational cohort study 88% of children treated with remestemcel-L had severe disease with highest mortality risk, defined by either IBMTR Grade C/D or Glucksberg Grade III/IV, whereas only 22% to 68% of patients in the other studies were considered to be severe.[1-4] These results reaffirm the potential significance of remestemcel-L as a life-saving therapy for children with SR-aGVHD.

Mesoblast Chief Executive Dr Silviu Itescu said: "These substantial and durable long-term survival outcomes seen in our Phase 3 trial with remestemcel-L are a cornerstone to our BLA resubmission."

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.[5,6] SR-aGVHD is associated with mortality as high as 90% and significant extended hospital stay costs.[7,8] There are currently no FDA-approved treatments in the US for children under 12 with SR-aGVHD.

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

About the CIBMTR
The Center for International Blood and Marrow Transplant Research is a nonprofit research collaboration between the National Marrow Donor Program (NMDP)/Be The Match, in Minneapolis, and the Medical College of Wisconsin, in Milwaukee. The CIBMTR collaborates with the global scientific community to increase survival and enrich quality of life for patients. The CIBMTR facilitates critical observational and interventional research through scientific and statistical expertise, a large network of centers, and a unique database of long-term clinical data for more than 600,000 people who have received hematopoietic cell transplantation and other cellular therapies. Learn more at cibmtr.org or follow the CIBMTR on Facebook, LinkedIn, or Twitter @CIBMTR.

References / Footnotes

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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 (including BLA resubmission), 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.

Release authorized by the Chief Executive.

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