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# EXPANDED ACCESS PROTOCOL INITIATED FOR COMPASSIONATE USE OF REMESTEMCEL-L IN CHILDREN WITH MULTISYSTEM INFLAMMATORY SYNDROME ASSOCIATED WITH COVID-19

**Melbourne, Australia; July 6, 2020; and New York, USA; July 5, 2020:** Mesoblast Limited (ASX:MSB; Nasdaq:MESO) today announced that an expanded access protocol (EAP) has been initiated in the United States for compassionate use of its allogeneic mesenchymal stem cell (MSC) product candidate remestemcel-L in the treatment of COVID-19 infected children with cardiovascular and other complications of multisystem inflammatory syndrome (MIS-C). Patients aged between two months and 17 years may receive one or two doses of remestemcel-L within five days of referral under the EAP.

The protocol was filed with the United States Food and Drug Administration (FDA) and provides physicians with access to remestemcel-L for an intermediate-size patient population[1] under Mesoblast's existing Investigational New Drug (IND) application. According to the FDA, expanded access is a potential pathway for a patient with an immediately life-threatening condition or serious disease or condition to gain access to an investigational medical product for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.

MIS-C is a life-threatening complication of COVID-19 in otherwise healthy children and adolescents that includes massive simultaneous inflammation of multiple critical organs and their vasculature. In approximately 50% of cases this inflammation is associated with significant cardiovascular complications that directly involve heart muscle and may result in decreased cardiac function. In addition, the virus can result in dilation of coronary arteries with unknown future consequences. Recent articles from Europe and the United States have described this disease in detail.[2-5]

Mesoblast Chief Medical Officer Dr Fred Grossman said: "The extensive body of safety and efficacy data generated to date using remestemcel-L in children with graft versus host disease suggest that our cellular therapy could provide a clinically important therapeutic benefit in MIS-C patients, especially if the heart is involved as a target organ for inflammation. Use of remestemcel-L in children with COVID-19 builds on and extends the potential application of this cell therapy in COVID-19 cytokine storm beyond the most severe adults with acute respiratory distress syndrome."

**Remestemcel-L**
Remestemcel-L is an investigational therapy comprising culture-expanded mesenchymal stem cells derived from the bone marrow of an unrelated donor and is administered in a series of intravenous infusions. Remestemcel-L is believed to have immunomodulatory properties to counteract the inflammatory processes that are implicated in several diseases 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.

1.www.clinicaltrials.gov; NCT04456439
2.Lancet2020; May 7. DOI: https://doi.org/10.1016/S0140-6736(20)31094-12
3.Lancet. 2020; (May 13) https://doi.org/10.1016/S0140-6736(20)31103-X
4.https://www.nejm.org/doi/full/10.1056/NEJMoa2021756
5.https://www.nejm.org/doi/full/10.1056/NEJMoa2021680

## 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 (IP) 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.

Mesoblast's Biologics License Application to seek approval of its product candidate RYONCIL™ (remestemcel-L) for pediatric steroid-refractory acute graft versus host disease (acute GVHD) has been accepted for priority review by the United States Food and Drug Administration (FDA), and if approved, product launch in the United States is expected in 2020. Remestemcel-L is also being developed for other inflammatory diseases in children and adults including moderate to severe acute respiratory distress syndrome. Mesoblast is completing Phase 3 trials for its product candidates for advanced 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

## 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; 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. 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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