

## **FDA GRANTS FAST TRACK DESIGNATION FOR REMESTEMCEL-L IN THE TREATMENT OF ACUTE RESPIRATORY DISTRESS SYNDROME DUE TO COVID-19**

**Melbourne, Australia; December 2 and New York; USA; December 1, 2020:** Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in allogeneic cellular medicines for inflammatory diseases, today announced that the United States Food and Drug Administration (FDA) has granted Fast Track designation for remestemcel-L in the treatment of acute respiratory distress syndrome (ARDS) due to COVID-19 infection. Fast Track designation is granted if a therapy demonstrates the potential to address unmet medical needs for a serious or life-threatening disease.<sup>1</sup> ARDS is the primary cause of death in patients with COVID-19.

Fast Track designation by the FDA is intended to facilitate development and expedite review of therapies to treat serious and life-threatening conditions with no or limited treatment options so that an approved product can reach the market expeditiously.<sup>1</sup> Under Fast Track designation, a Biologic License Application (BLA) for remestemcel-L is eligible for both rolling submission and priority review.

Clinical data provided to the FDA supporting the potential of remestemcel-L to address the unmet medical need of COVID-19 ARDS included results from a pilot study of remestemcel-L under emergency compassionate use at New York's Mt Sinai Hospital in March-April this year. In this study, nine of 12 ventilator-dependent patients (75%) with moderate to severe COVID-19 ARDS were successfully discharged from hospital a median of 10 days after receiving two intravenous doses of remestemcel-L.

The ongoing randomized controlled Phase 3 trial of remestemcel-L in up to 300 ventilator-dependent patients with moderate to severe COVID-19 ARDS, under an FDA Investigational New Drug clearance, is approximately two-thirds enrolled. The trial's primary endpoint is overall mortality at Day 30, and the key secondary endpoint is days alive off ventilatory support through Day 60. Two interim analyses have been performed by the independent Data Safety Monitoring Board (DSMB), after 90 and 135 patients were enrolled, with recommendations to continue the trial as planned. A third and final interim analysis is planned to be performed by the DSMB when 180 patients have completed 30 days of follow-up.

Mesoblast recently entered into a license and collaboration agreement with Novartis for the development, manufacture and commercialization of remestemcel-L, with an initial focus on the treatment of ARDS, including COVID-19 ARDS. The closing of the agreement is subject to the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act and certain other conditions.

### **About COVID-19 Acute Respiratory Distress Syndrome (ARDS)**

ARDS is due to a dysregulated immune response in the lungs to various infectious agents including COVID-19. Deaths continue to increase in ventilator-dependent ARDS patients as COVID-19 cases continue to surge globally. Despite improved treatment and earlier intervention in hospitalized COVID-19 patients overall, the mortality rate in COVID-19 ARDS patients who are over 60 years old remains more than 60%.<sup>2</sup> These patients appear to be particularly refractory to corticosteroids such as dexamethasone<sup>3,4</sup> and have not responded to single cytokine antagonists, anti-virals, or anti-malaria agents.

### **About Remestemcel-L**

Remestemcel-L is an investigational therapy comprising culture-expanded mesenchymal stromal cells derived from the bone marrow of an unrelated donor. Remestemcel-L is thought to have immunomodulatory properties to counteract the cytokine storms that are implicated in various inflammatory conditions by downregulating the production of pro-inflammatory cytokines, increasing production of anti-inflammatory cytokines, and enabling recruitment of naturally occurring anti-inflammatory cells to involved tissues.

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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 platform to establish a broad portfolio of commercial products and late-stage product candidates. Mesoblast has a strong and extensive global intellectual property 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. For more information, please see [www.mesoblast.com](http://www.mesoblast.com), LinkedIn: Mesoblast Limited and Twitter: @Mesoblast

## References

1. <https://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm>
2. Rezoagli E, Fumagalli R, Bellani G. 2017. Definition and epidemiology of acute respiratory distress syndrome. Ann Transl Med. 5(14): 1- 12
3. Bellani G, Laffey J, Pham T. et. al. 2016. Epidemiology, patterns of care, and mortality for patients with acute respiratory distress syndrome in intensive care units in 50 countries. JAMA 315(8): 788-800
4. [https://www.hopkinsguides.com/hopkins/view/Johns\\_Hopkins\\_ABX\\_Guide/540747/all/Coronavirus\\_COVID\\_19\\_SARS\\_CoV\\_2](https://www.hopkinsguides.com/hopkins/view/Johns_Hopkins_ABX_Guide/540747/all/Coronavirus_COVID_19_SARS_CoV_2)

## 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 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; 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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