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the regenerative medicine company

MESOBLAST SUBMITS COMPLETED BIOLOGICS LICENSE APPLICATION FOR RYONCIL™ TO US FDA

Melbourne, Australia; February 3, 2020; and New York, USA; February 2, 2020: Mesoblast Limited (ASX:MSB; Nasdaq: MESO), global leader in cellular medicines for inflammatory diseases, today announced that it has submitted its completed Biologics License Application (BLA) to the United States Food and Drug Administration (US FDA) for Ryoncil™ (remestemcel-L), its lead allogeneic cell therapy for the treatment of children with steroid-refractory acute graft versus host disease (SR-aGVHD).

Mesoblast filed the final module of the rolling BLA submission, covering quality control and manufacturing, with the FDA on January 31. The Company has requested Priority Review of the BLA by the FDA under the product candidate's existing Fast Track designation for SR-aGVHD. If approved, RYONCIL is expected to be launched in the US in 2020.

Mesoblast Chief Executive Dr Silviu Itescu stated: "This is a major corporate milestone for Mesoblast. We look forward to working closely with the FDA to potentially bring RYONCIL to market and providing our innovative biologic therapy to the many children with this life-threatening condition."

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, and these numbers are increasing[1]. In patients with the most severe form of acute GVHD (Grade C/D or III/IV) mortality is as high as 90% despite optimal institutional standard of care[2,3]. There are currently no FDA-approved treatments in the US for children under 12 with SR-aGVHD.

Mesoblast's product candidate RYONCIL has been used in 309 children with SR-aGVHD across three separate studies. RYONCIL was used as salvage therapy in an expanded access program in 241 children with SR-aGVHD (80% Grade C/D) who failed institutional standard of care. RYONCIL was also used as first-line therapy in Mesoblast's open-label Phase 3 trial in 55 children with SR-aGVHD, 89% of whom had Grade C/D disease.

About Ryoncil™
Mesoblast's lead product candidate, RYONCIL, is an investigational therapy comprising culture-expanded mesenchymal stem cells derived from the bone marrow of an unrelated donor. It is administered to patients in a series of intravenous infusions. RYONCIL 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.

References

1. 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.
2. Westin, J., Saliba, RM., Lima, M. (2011) Steroid-refractory acute GVHD: predictors and outcomes. Advances in Hematology.
3. 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.

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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 cell therapy technology platform to establish a broad portfolio of commercial products and late-stage product candidates. Mesoblast has filed a Biologics License Application to the United States Food and Drug Administration (FDA) to seek approval of its product candidate Ryoncil™ for steroid-refractory acute graft versus host disease, and is completing Phase 3 trials for its advanced heart failure and chronic low back pain product candidates. Two products have been commercialized in Japan and Europe by its licensees, and it has established commercial partnerships in Europe and China for certain Phase 3 assets. Mesoblast's proprietary manufacturing process yields industrial-scale, frozen, off-the-shelf, cellular medicines based on its mesenchymal lineage cell platform technology. These cell therapies, with defined pharmaceutical release criteria, are planned to be readily available to patients worldwide. Mesoblast has locations in Melbourne, New York, Singapore and Texas 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

Mesoblast's 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. 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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