asx announcement

FDA ACCEPTS MESOBLAST'S BIOLOGICS LICENCE APPLICATION FOR RYONCIL™ AND AGREES TO PRIORITY REVIEW

Melbourne, Australia; April 1, 2020; and New York, USA; March 31, 2020: Mesoblast Limited (ASX:MSB; Nasdaq: MESO), global leader in cellular medicines for inflammatory diseases, today announced that the United States Food and Drug Administration (FDA) has accepted for priority review the Company's Biologics License Application (BLA) filing for RYONCIL™ (remestemcel-L), its allogeneic cell therapy for the treatment of children with steroid-refractory acute graft versus host disease (SR-aGVHD). The FDA has set a Prescription Drug User Fee Act (PDUFA) action date of September 30, 2020, and if approved, Mesoblast will make RYONCIL immediately available in the United States.

A Priority Review designation will direct overall attention and resources to the evaluation of applications for drugs that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications. The FDA has advised that they are planning to hold an Advisory Committee Meeting to discuss this application.

Mesoblast Chief Executive Dr Silviu Itescu stated: "There is a critical need to improve survival outcomes in children suffering from the more advanced stages of this devastating disease. The acceptance of the BLA represents an important milestone for the Company. Mesoblast is on track in its preparation for the potential launch of RYONCIL, including meeting its target inventory build and commercial team roll-out."

About Acute GVHD

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.

About RYONCIL™

Mesoblast's lead product candidate, RYONCIL (remestemcel-L), 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.

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'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 Limited
ABN 68 109 431 870

www.mesoblast.com

Corporate Headquarters
Level 38
55 Collins Street
Melbourne 3000
Victoria Australia

T +61 3 9639 6036
F +61 3 9639 6030

United States Operations
505 Fifth Avenue
Third Floor
New York, NY 10017
USA

T +1 212 880 2060
F +1 212 880 2061

Asia
21 Biopolis Road
#01-22 Nucleos (South Tower)
SINGAPORE 138567

T +65 6570 0635
F +65 6570 0176

Mesoblast has filed a Biologics License Application to the United States Food and Drug Administration (FDA) to seek approval of its product candidate RYONCIL™ (remestemcel-L) for steroid-refractory acute graft versus host disease (acute GvHD). Remestemcel-L is also being developed for other rare diseases. Mesoblast is completing Phase 3 trials for its product candidates for advanced heart failure and chronic low back pain. If approved, RYONCIL is expected to be launched in the United States in 2020 for pediatric steroid-refractory acute GVHD. 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 a strong and extensive global intellectual property (IP) portfolio with protection extending through to at least 2040 in all major markets. This IP position is expected to provide the Company with substantial commercial advantages as it develops its product candidates for these conditions.

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. 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.

For further information, please contact:

Julie Meldrum
Corporate Communications
T: +61 3 9639 6036
E: julie.meldrum@mesoblast.com

Schond Greenway
Investor Relations
T: +1 212 880 2060
E: schond.greenway@mesoblast.com