

MESOBLAST INITIATES ROLLING SUBMISSION OF BIOLOGICS LICENSE APPLICATION (BLA) TO U.S. FDA FOR REMESTEMCEL-L IN THE TREATMENT OF ACUTE GRAFT VERSUS HOST DISEASE

Melbourne, Australia; and New York, USA; May 30, 2019: Mesoblast Limited (ASX:MSB; Nasdaq: MESO), global leader in cellular medicines for inflammatory diseases, today announced that it has filed the first component of a rolling submission for a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for remestemcel-L in the treatment of children with steroid-refractory acute graft versus host disease (aGVHD), a life-threatening complication of an allogeneic bone marrow transplant.

The FDA has agreed to a rolling review of the BLA which enables individual components to be submitted and reviewed on an ongoing basis rather than waiting for all sections to be completed. The rolling process will provide opportunity for ongoing communication, and during this process the Company expects it will be able to adequately address any substantial matters raised by the FDA. Remestemcel-L has received Fast Track designation for aGVHD and under this designation Mesoblast intends to request a priority review once its BLA filing is completed and accepted by the FDA.

Mesoblast Chief Executive Dr Silviu Itescu stated: "Initiation of our rolling BLA submission to the FDA for potential United States approval of our first product is a major corporate milestone for the Company and an exciting moment in our history. We look forward to making this therapy available as soon as possible to children with this devastating disease."

There are more than 30,000 allogeneic bone marrow transplants performed annually worldwide¹, primarily in patients being treated for blood cancers, with up to 50% developing aGVHD. In the more severe forms of the disease, Grades C/D or III/IV, six-month mortality rates are as high as 90%.^{2,3}

In Mesoblast's Phase 3 trial of 55 children with aGVHD - 89% of whom had Grade C/D disease - treatment with remestemcel-L resulted in a six-month survival of 69%. In addition, achievement of an Overall Response at Day 28, which occurred in 69% of patients, predicted highest survival at Day 100 and Day 180, which was 85% and 79%, respectively. The trial successfully met its primary endpoint of increased Day 28 Overall Response compared with a protocol-defined historical control rate of 45% (p=0.0003). These data are consistent with prior results from an Expanded Access Program in 241 children where remestemcel-L was used as salvage therapy after failure of steroids and other agents.

About Remestemcel-L

Mesoblast's lead product candidate, remestemcel-L, is an investigational therapy comprising culture-expanded mesenchymal stromal cells derived from the bone marrow of an unrelated donor. It is administered to patients in a series of intravenous infusions. Remestemcel-L has demonstrated immunomodulatory properties to counteract the inflammatory processes that are implicated in 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.

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 technology platform to establish a broad portfolio of late-stage product candidates with three product candidates in Phase 3 trials – acute graft versus host disease, chronic heart failure and chronic low back pain due to degenerative disc disease. Through a proprietary process, Mesoblast selects rare mesenchymal lineage precursor and stem cells from the bone marrow of healthy adults and creates master cell banks, which can be industrially expanded to produce thousands of doses from each donor that meet stringent release criteria, have lot to lot consistency, and can be used off-the-shelf without the need for tissue matching. Mesoblast has facilities in Melbourne, New York, Singapore and Texas and is listed on the Australian Securities Exchange (MSB) and on the Nasdaq (MESO). www.mesoblast.com

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
20 Biopolis Way
#05-01 Centros
Biopreneur 3
SINGAPORE 138668

T +65 6570 0635
F +65 6570 0176

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*

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 in aGVHD; Mesoblast's ability to advance its aGVHD product candidate into, enroll and successfully complete, clinical studies; the timing or likelihood of regulatory filings and approvals for aGVHD; 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.

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

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
20 Biopolis Way
#05-01 Centros
Biopreneur 3
SINGAPORE 138668
T +65 6570 0635
F +65 6570 0176