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the regenerative medicine company

CHILDREN TREATED WITH REMESTEMCEL-L CONTINUE TO HAVE STRONG SURVIVAL OUTCOMES AT SIX MONTHS IN MESOBLAST'S PHASE 3 TRIAL FOR ACUTE GRAFT VERSUS HOST DISEASE

Preparations for Biologics License Application (BLA) Filing Underway

New York, USA; and Melbourne, Australia; September 20, 2018: Mesoblast Limited (ASX:MSB; Nasdaq:MESO) today announced continued strong survival outcomes through Day 180 in children with steroid refractory acute Graft Versus Host Disease (aGVHD) treated with Mesoblast's Phase 3 product candidate remestemcel-L, an allogeneic mesenchymal stem cell product candidate.

Mesoblast's open-label Phase 3 trial enrolled 55 children with steroid-refractory aGVHD (aged between six months and 17 years) at 32 sites across the United States, with the vast majority (89%) suffering from the most severe form of aGVHD (Grade C/D). This Phase 3 trial successfully met its primary endpoint of Day 28 Overall Response (OR) to remestemcel-L treatment, with 69% of patients achieving this endpoint compared to the protocol-defined historical control rate of 45% (p=0.0003).

In patients who had a positive OR to treatment with remestemcel-L at Day 28, survival was 87% at Day 100. At Day 180, survival in these patients was 79% (p=0.001 by Kaplan-Meier survival estimates compared to non-responders). Overall Day 180 survival for the entire remestemcel-L treated group was 69%. Historical survival rates in patients with Grade C/D disease and failure to respond to steroids have been only 10-30%[1-4].

These Phase 3 outcomes are consistent with previous results in 241 children with steroid-refractory aGVHD who failed to respond to multiple biologic agents and were treated under an expanded access program (EAP) that followed outcomes through 100 days. The multi-infusion regimen in both the EAP[5] and the Phase 3 trial was well tolerated[6].

In discussions with the Company, the United States Food and Drug Administration (FDA) advised that a successful Phase 3 trial should achieve both the primary endpoint of Day 28 OR and also demonstrate overall survival benefits through 180 days. Mesoblast is now working towards a pre-BLA meeting with the FDA in the next few months. Existing Fast Track designation from the FDA allows eligibility for priority review and a rolling BLA review process.

Mesoblast Chief Executive Dr Silviu Itescu stated: "Our goal is for remestemcel-L to be the first commercially manufactured allogeneic cellular medicine available in the United States. The commercial success of TEMCELL HS Inj.®[7] in Japan by Mesoblast's licensee, JCR Pharmaceuticals Co. Ltd, in the treatment of steroid-refractory acute GVHD is very encouraging in light of our launch plans in the United States, where there is a substantially larger unmet need."

The trial was performed under a United States Food and Drug Administration (FDA) Investigational New Drug Application (NCT#02336230).

About Acute Graft Versus Host Disease

Acute GVHD is associated with significant morbidity and is a leading cause of mortality after allogeneic hematopoietic stem cell transplantation for blood cancers or other conditions. Severe aGVHD (determined by grade C/D, liver or gut involvement, or high risk stratification) has the highest risk of failure to first-line corticosteroids and high transplant-related mortality[8]. Day 100 mortality can reach 70% in patients who fail to respond to initial steroid therapy[1-3], and 12-month mortality approaches 90%[4].

Mesenchymal stem cells have anti-inflammatory and immunomodulatory biological activity support their investigational use in aGVHD[9]. The immunomodulatory actions of these cells are triggered

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through receptor activation by inflammation, resulting in regulation of multiple cellular arms of the immune system that are central to aGVHD pathogenesis.

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5. Kurtzberg J. et al. Effect of Human Mesenchymal Stem Cells (remestemcel-L) on Clinical Response and Survival Confirmed in a Large Cohort of Pediatric Patients with Severe High-Risk Steroid-Refractory Acute Graft Versus Host Disease. BBMT. 2016; 22.
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7. TEMCELL® HS Inj. Is a registered trademark of JCR Pharmaceuticals Co. Ltd.
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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 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

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.

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