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the regenerative medicine company

MESOBLAST PHASE 3 TRIAL RESULTS FOR ACUTE GRAFT VERSUS HOST DISEASE PRESENTED AT 2018 INTERNATIONAL SOCIETY FOR CELL AND GENE THERAPY ANNUAL MEETING

New York, USA; and Melbourne, Australia; May 8, 2018: Mesoblast Limited (ASX:MSB; Nasdaq: MESO) today announced that results of the Phase 3 trial evaluating its product candidate MSC-100-IV (remestemcel-L) in children with steroid-refractory acute Graft versus Host Disease (aGVHD) were presented at the 2018 International Society for Cell and Gene Therapy (ISCT) plenary breakout session held in Boston last week. These results were presented by the trial's lead investigator Dr Joanne Kurtzberg, Jerome Harris Distinguished Professor of Pediatrics and Director of the Pediatric Blood and Marrow Transplant Program at Duke University Medical Center.

Dr Kurtzberg described the properties of remestemcel-L that are relevant to understanding proposed mechanisms of action (MOA) in aGVHD, including sensing of damaging inflammatory mediators and consequent reduction in activation of immune cells. These proposed MOAs provide the rationale for the potency assays developed for this product candidate. During the presentation, Dr Kurtzberg stressed the importance of Mesoblast's proprietary manufacturing processes for remestemcel-L, which have been shown to deliver a product with consistent batch-to-batch characterization and activity, critical to providing a reproducible clinical outcome.

As previously reported, the open label, single arm Phase 3 trial successfully met its pre-specified primary endpoint of Day 28 overall response rate, which is significantly increased (69%, p=0.0003) in children treated with remestemcel-L compared to the protocol's defined hypothesized control rate of 45%. Remestemcel-L was well tolerated in the Phase 3 trial with a safety profile consistent with prior controlled studies in greater than 1,000 patient exposures.

This Phase 3 trial is being conducted under a United States Food and Drug Administration (FDA) Investigational New Drug Application (NCT#02336230). Key secondary endpoints include survival at 100 days, the results of which are expected to be read out shortly, and safety and survival through 180 days.

There are currently no products approved in the United States for treatment of steroid-refractory aGVHD. Given the serious nature of this condition, in 2017 the United States Food and Drug Administration (FDA) granted Mesoblast Fast Track designation for the use of remestemcel-L to achieve improved overall response rate in children with aGVHD. If successful, remestemcel-L will be the first approved therapy for this devastating condition in the United States. Japan is the only jurisdiction where this therapy is available, through Mesoblast's licensee JCR Pharmaceuticals Co. Ltd.

About Graft Versus Host Disease
Mesoblast is developing MSC-100-IV for the treatment of aGVHD following an allogeneic bone marrow transplant (BMT). In patients who have received a BMT, donor cells may attack the recipient (the person receiving the transplant), causing aGVHD, resulting in activation of pro-inflammatory T-cells and tissue damage in the skin, gut and liver. This condition, when severe and unresponsive to initial steroid therapy, is often fatal. According to the Center for International Blood and Marrow Transplant Research, there are approximately 30,000 allogeneic BMTs globally per year for diseases including hematological cancers, with approximately 20%[1] of all cases in the pediatric population. Approximately 50% of all allogeneic BMT patients develop aGVHD. Liver or gastrointestinal involvement occur in up to 60%[2] of all patients with aGVHD and are associated with the greatest risk of death, with mortality rates of up to 85%.

1. CIBMTR 2015 Volume Data Set
2. Jagasia, M., Arora, M., Flowers, M. (2012) Risk Factors for acute GVHD and Survival after Hematopoietic Cell Transplantation. Blood, 5 January (119):296-307

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About Mesoblast

Mesoblast Limited (Nasdaq:MESO; ASX:MSB) is a global leader in developing innovative cell-based medicines. Through a proprietary process, Mesoblast selects highly purified 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.

The Company has leveraged its proprietary technology platform to establish a broad portfolio of late-stage product candidates. Mesoblast's allogeneic, 'off-the-shelf' cell product candidates are being evaluated in their ability to target advanced stages of diseases with high, unmet medical needs including cardiovascular conditions, orthopedic disorders, immunologic and inflammatory disorders and oncologic/hematologic conditions.

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; ability to successfully produce and distribute product sufficient to support market demand; 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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