

## HIGH ECONOMIC BURDEN IN CHILDREN WITH STEROID REFRACTORY ACUTE GRAFT VERSUS HOST DISEASE

### Mesoblast presents health economics and outcomes research data at 24<sup>th</sup> European Hematology Association Congress

**Melbourne, Australia; and New York, USA; June 18, 2019:** Mesoblast Limited (ASX:MSB; Nasdaq: MESO), global leader in cellular medicines for inflammatory diseases, presented health economics and outcomes research data for pediatric acute graft versus host disease (aGVHD) at the 24th European Hematology Association (EHA) Congress in Amsterdam, Netherlands. Key findings indicated that a steroid refractory state in aGVHD may result in significant deterioration in quality of life (QOL) and additional direct healthcare costs of up to \$500,000 on average per patient.

Acute GVHD is a potentially life-threatening complication of an allogeneic bone marrow transplant, with the most severe forms of the disease, Grades C/D or III/IV, frequently being refractory to steroid therapy and associated with mortality rates as high as 90%.<sup>1,2</sup>

In studies from the United States (US), children with aGVHD have longer hospitalizations (incremental 17.9 – 45.4 days) and increased costs (incremental \$114,698 - \$224,000) compared to recipients of allogeneic bone marrow transplants who did not develop aGVHD.<sup>3</sup> In steroid-refractory patients, the magnitude of the burden may be larger, with a preliminary US pediatric claims analysis suggesting additional direct healthcare costs of up to \$500,000 on average in steroid-refractory patients compared to those who responded to steroids.<sup>4</sup>

Mesoblast recently initiated a rolling Biologic License Application (BLA) to the United States Food and Drug Administration for its product candidate remestemcel-L in children with steroid-refractory aGVHD.

The BLA follows completion of Mesoblast's Phase 3 trial which recruited 55 children with aGVHD - 89% of whom had Grade C/D disease. The trial successfully met its primary endpoint of increased Day 28 Overall Response compared with a protocol-defined historical control rate (69% vs 45%, p=0.0003). Overall Response at Day 28 predicted highest survival at Day 100 and Day 180, 85% and 79%, respectively. 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. Remestemcel-L is administered to patients in a series of intravenous infusions of 60 minutes or less in duration over the course of 4-8 weeks. 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 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](http://www.mesoblast.com)

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1. Jagasia M, Arora M, Flowers ME, et al. Risk factors for acute GVHD and survival after hematopoietic cell transplantation. *Blood*. 2012;119(1):296.
2. 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*
3. European Hematology Association 2019 Congress Meeting: Abstract PF718, The economic and humanistic burden of graft-versus-host disease (GVHD) in pediatric patients: A systematic literature review (SLR)
4. Data on file: claim analysis with HealthCore Q42015

### 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.

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