

## MESOBLAST'S CELL THERAPY INCREASES SURVIVAL IN CHILDREN WITH ACUTE GRAFT VERSUS HOST DISEASE

### *Results of 241 Patients Presented at Bone Marrow Transplant Tandem Meetings 2016*

**New York, USA, and Melbourne, Australia; 22 February 2016:** Mesoblast Limited (ASX:MSB; Nasdaq:MESO) today announced that results have been presented showing that use of its proprietary Tier 1 mesenchymal stem cell product candidate remestemcel-L (MSC-100-IV) demonstrated clinically meaningful responses and significantly increased survival in children with steroid-refractory acute Graft Versus Host Disease (aGVHD).

The data from 241 children treated in Mesoblast's Expanded Access Program, conducted across more than 50 sites in North America and globally, were presented at the tandem annual scientific meetings of the Center for International Blood & Marrow Transplant Research and the American Society of Blood and Marrow Transplantation in Hawaii on 20 February 2016.

The oral presentation was given by the study's independent lead investigator, Dr Joanne Kurtzberg, who is the Jerome Harris Distinguished Professor of Pediatrics and Director of the Pediatric Blood and Marrow Transplant Program at Duke University Medical Center.

Dr Kurtzberg said: "There is a critical and urgent need for an effective and well-tolerated treatment for the very ill children who develop this life-threatening complication after a bone marrow transplant.

"While historically there is a high mortality rate associated with this complication, we are now seeing the majority of children who receive Mesoblast's cell therapy respond and survive."

Key results in the 241 children with steroid-refractory aGVHD were:

- An overall response rate of 65% was seen at day 28 after treatment with MSC-100-IV
- A response rate of 81% was seen when MSC-100-IV was used as front-line therapy following steroid failure
- In patients with gastrointestinal and liver disease, who have the highest mortality risk, overall response rates were 65% and 62% respectively
- Children who achieved overall response at day 28 had significantly improved survival (82% vs 39%, log rank p-value <0.0001)
- Extending therapy beyond day 28 in children who had not achieved an overall response but had some improvement at day 28 resulted in significantly improved survival (72% vs 18%, log rank p-value 0.003).

To support filing of a biologic license application to the United States Food and Drug Administration for regulatory approval, Mesoblast is conducting a 60-patient, open label Phase 3 trial using MSC-100-IV as front-line therapy in children with steroid-refractory aGVHD.

Mesoblast Chief Executive Silviu Itescu said: "We are committed to making our cell therapy available to the many children suffering from this devastating disease."

### **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 which is often fatal.

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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 25% of all cases in the pediatric population. Nearly 50% of all allogeneic BMT patients develop aGVHD. Liver or gastrointestinal involvement occur in up to 40% of all patients with aGVHD and are associated with the greatest risk of death, with mortality rates of up to 85%.

Currently, there are no approved therapies for patients with acute steroid-refractory GVHD in the United States, and off-label options have demonstrated mixed efficacy with high toxicity.

### **About Mesoblast**

Mesoblast Limited (ASX: MSB; Nasdaq: MESO) is a world leader in developing innovative cellular medicines. We have established what we believe is the industry's most clinically advanced and diverse portfolio of cell-based products with five programs, two of which are partnered, in active Phase 3 clinical studies or Phase 3-ready, and four programs in Phase 2. All our clinical programs target significant, under-served therapeutic areas including cardiac diseases, spine orthopedic disorders, oncology and hematology diseases, and immune-mediated and inflammatory conditions.

### **Forward-Looking Statements**

This press release 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. 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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