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Mesoblast Announces Successful Interim Futility Analysis In Phase 3 Trial For Acute Graft Versus Host Disease

New York, USA; and Melbourne, Australia; November 14, 2016: Mesoblast Limited (ASX: MSB; Nasdaq: MESO) today announced that the Phase 3 trial of its intravenous product candidate MSC-100-IV used as front-line therapy in children with steroid-resistant acute graft versus host disease (aGVHD) had been successful in a pre-specified interim futility analysis.

Enrollment in the 60-patient open label Phase 3 trial is ongoing across multiple sites in the United States, trial completion is expected in the first half of 2017, and commercial launch activities are underway.

The independent Data Safety Monitoring Board (DSMB) notified Mesoblast that an interim analysis showed that the predefined Bayesian futility rule used to determine the probability of the trial's success using the trial's primary endpoint of Day 28 overall response had been passed. The analysis method determined the likelihood of obtaining a statistically significant treatment effect at study completion, based on the data observed at this interim time point.

There are currently no products approved in the United States for this disease. Japan is the only jurisdiction where this therapy is available, through Mesoblast's licensee JCR Pharmaceuticals Co. Ltd. Based on guidance from the United States Food and Drug Administration (FDA), Mesoblast believes that positive data from this Phase 3 trial may be sufficient for filing for accelerated approval of MSC-100-IV in the United States. Mesoblast plans to broaden its use in adult patients with high-risk steroid-refractory aGVHD.

"We are pleased that Mesoblast has attained such an important milestone in making its product available for the potential treatment of steroid-refractory acute graft versus host disease, a serious and life threatening condition that has a very urgent need for effective therapies," said Dr Joanne Kurtzberg, the Jerome Harris Distinguished Professor of Pediatrics and Director of the Pediatric Blood and Marrow Transplant Program at Duke University Medical Center and the lead investigator on the ongoing Phase 3 trial.

"Mortality can reach 85% in patients with liver and gut complications and, outside of Japan, there are currently no approved therapies available. MSC-100-IV is on the cusp of becoming an important new treatment option for these patients," she said.

The successful outcome of the DSMB interim analysis using the trial's primary endpoint of Day 28 overall response is consistent with previously reported results in a pediatric Expanded Access Program (EAP) in children with steroid-refractory aGVHD. Results from this program, which evaluated MSC-100-IV in 241 children, were presented in February 2016 at the American Society of Blood and Marrow Transplantation annual meeting.

Key findings in the EAP program were:

- An overall response rate of 65% in all children at day 28 when MSC-100-IV was used either as last-line or front-line therapy after steroid failure
- An overall response rate of 81% at day 28 when MSC-100-IV was used as front-line therapy following steroid failure
- An overall response rate of 65% and 62%, respectively, in patients with gastrointestinal and liver disease, who have the highest mortality risk
- A significantly improved survival at day 100 in children who achieved overall response at day 28 (82% vs. 39%, log rank p-value <0.0001)

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

In February 2016, Mesoblast's licensee JCR launched TEMCELL® HS. Inj. in Japan, the first allogeneic cell-based product to receive full approval in the world's second-largest standalone healthcare market. A four-week, multi-dose treatment course of TEMCELL for an average adult is expected to be reimbursed by the Japanese Government's National Health Insurance body at a minimum of ¥13,898,880 (approximately US$130,000) or up to ¥20,848,320 (approximately US$195,000) if symptoms persist and additional dosing is required. Under its agreement with JCR, Mesoblast is entitled to receive royalties and other payments at predefined thresholds of cumulative net sales.

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.

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%.

Conference Call
Dr Kurtzberg and Mesoblast's Chief Medical Officer, Dr Donna Skerrett, are participating in a corporate update conference call beginning at 9:00 am Australian Eastern Daylight Time on Tuesday, November 15, 2016 / 5:00 pm Eastern Time on Monday, November 14, 2016.

To access the call, dial 1 800 558 698 (toll-free Australia), 1 855 881 1339 (toll-free US), or +61 2 9007 3187 (outside of the US and Australia).

The conference identification code is 912575.

The live webcast can be accessed via:
http://webcasting.boardroom.media/broadcast/58214386d5f1311b35bd36a6

There will be a slide presentation. Please log in approximately 15 minutes prior to the scheduled start time.

About Mesoblast
Mesoblast Limited (ASX:MSB; Nasdaq:MESO) is a global leader in developing innovative cell-based medicines. The Company has leveraged its proprietary technology platform, which is based on specialized cells known as mesenchymal lineage adult stem cells, to establish a broad portfolio of late-stage product candidates. Mesoblast's allogeneic, 'off-the-shelf' cell product candidates target advanced stages of diseases with high, unmet medical needs including cardiovascular diseases, immune-mediated and inflammatory disorders, orthopedic disorders, and oncologic/hematologic 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.

For further information, please contact:

Julie Meldrum
Global Head, Corporate Communications
Mesoblast Limited
T: +61 3 9639 6036
E: julie.meldrum@mesoblast.com

Schond Greenway
VP, Investor Relations
Mesoblast Limited
T: +1 212 880 2060
E: schond.Greenway@mesoblast.com