asx announcement

mesoblast
the regenerative medicine company

MD ANDERSON CANCER CENTER AND NATIONAL INSTITUTES OF HEALTH TO FUND CLINICAL TRIAL COMBINING TWO PROPRIETARY MESOBLAST TECHNOLOGIES FOR RAPID ENGRAFTMENT OF STEM CELL TRANSPLANTS IN CANCER PATIENTS

New York, USA; and Melbourne, Australia; December 6, 2016: Mesoblast Limited (ASX:MSB; Nasdaq:MESO) today announced that MD Anderson Cancer Center (MDACC) in Texas and the United States National Institutes of Health (NIH) will fund a clinical trial combining Mesoblast's two synergistic proprietary technologies, Mesenchymal Precursor Cell (MPC)-based expansion and ex-vivo fucosylation of hematopoietic stem cells (HSCs) for cord blood transplantation in cancer patients. The trial will provide clinical data on whether the combination of these two technologies synergistically facilitates more rapid cord blood HSC engraftment for bone marrow transplant patients than can be achieved by either technology alone.

The number of allogeneic bone marrow transplants performed globally each year could be substantially increased beyond the current 30,000, for cancer and non-cancer indications, if safe and effective alternative sources of allogeneic HSCs are available, such as cord blood, for patients who cannot find a matched donor. Unfortunately, cord blood transplants are associated with prolonged engraftment times due to insufficient numbers and inadequate homing capacity of cord blood HSCs, adversely impacting their clinical outcomes. Combining Mesoblast's proprietary technologies using MPC-based expansion plus ex-vivo fucosylation of cord blood HSCs aims to overcome the two key limitations to using cord blood for rapid, early engraftment and bone marrow reconstitution in adult bone marrow transplant patients. This novel clinical strategy has the potential to significantly increase the number of patients who can receive unrelated donor transplants.

Previously, Mesoblast conducted a Phase 2 clinical trial which demonstrated that transplantation of HSCs from MPC-expanded cord blood resulted in a reduced engraftment time, from a median of 24 days for placebo-treated cells to a median of 15 days for co-cultured cells.[i] Separately, another Phase 2 clinical study showed that transplantation of fucosylated, but non-expanded, cord blood HSCs also resulted in a reduced median engraftment time of 17 days.[ii] More recently, preclinical results from a group led by Dr Elizabeth J. Shpall, Director of the Cell Therapy Laboratory and a Professor in the Department of Stem Cell Transplantation at MDACC, where MPC-based expansion and ex vivo-fucosylation technologies were combined, showed a very rapid engraftment time of approximately seven days.

“Our data suggest that combining Mesoblast’s MPC-based HSC expansion and ex vivo fucosylation technologies may be the optimal clinical strategy for rapid engraftment of cord blood transplants, potentially making cord blood transplantation a real option for many desperate patients who cannot find a suitable alternative,” said Dr Shpall.

The new trial of up to 25 patients, entitled *‘Cord Blood Ex-vivo MPC Expansion Plus Fucosylation to Enhance Homing and Engraftment*’, is supported by a grant from the NIH National Cancer Institute (NCI Grant R01 CA061508-19) and will be led by Dr Amanda Olson, Assistant Professor, Department of Stem Cell Transplantation and Dr Shpall at MDACC. If the results of the combination study are positive, Mesoblast's proprietary ex vivo-fucosylation technology may be incorporated into the company's Phase 3 program of MPC-expanded HSCs.

About HSC Transplantation Treatment of Patients with Advanced Blood Cancers
Many patients with advanced blood cancers, such as acute myeloid leukemia, require a stem cell transplant to repopulate bone marrow HSCs after treatment with high dose chemotherapy. Patients typically undergo transplant with blood stem cells taken from the bone marrow or peripheral blood of a donor with a matched tissue type. However, it may be difficult to find a matched donor, especially for patients who are part of a racial or ethnic minority.

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

While transplants using cord blood-derived stem cells do not require the same degree of donor matching as blood and marrow, this approach has had limited success due to the low yield of stem cells in cord blood and their reduced ability to localize within the recipient's bone marrow.

About Ex-Vivo Fucosylation Technology
Ex vivo fucosylation is the addition of the sugar fucose to surface receptors on cells, including HSCs and mesenchymal lineage stem cells. This process modifies receptors on these cells by adding carbohydrate or sugar sequences which allows them to be recognized by and bound to their ligands present on endothelial cells lining blood vessels in inflamed tissues and in human bone marrow. As a result, such modified cells demonstrate enhanced homing properties to bone marrow or to tissues that are inflamed. Mesoblast has exclusively licensed the ex-vivo fucosylation technology, which was developed at the Harvard Medical School by Dr Robert Sackstein, for use with HSCs and with allogeneic mesenchymal lineage cells. This cell targeting technology resulted in engraftment of systemically infused human HSCs into mouse bone marrow at a rate ten times that of unmodified human HSCs.[iii]

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.

[i] N Engl J Med, 2012; 367:2305-15
[ii] Blood, 2015; 124(29): 2885-2892
[iii] Nature Medicine, 2008; 14: 181-187

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
Corporate Communications, Mesoblast
T: +61 3 9639 6036
E: julie.meldrum@mesoblast.com

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