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Bone Marrow Transplant Trial Using Proprietary Cell Therapy Receives United States FDA Clearance and Institutional Ethics Approval

Orphan drug designation enables faster product commercialisation

Key Points:

- US FDA clears submission for bone marrow transplant trial using proprietary stem cells
- Major US cancer centre approves ethics submission and commences patient recruitment
- Trial to be funded by US government grant
- Orphan drug designation for this "off-the-shelf" product may translate to earlier revenues
- Fourth IND cleared by FDA for proprietary stem cell technology platform

Melbourne, Australia; 5 November 2008: Mesoblast Limited (ASX: MSB; USOTC: MBLTY) today announced the commencement of a Phase I/II clinical trial in the United States using the patented allogeneic, or "off-the-shelf", Mesenchymal Precursor Cells (MPCs) in up to 30 patients with haematologic malignancies undergoing bone marrow transplantation.

This follows clearance by the United States Food and Drug Administration (FDA) of an Investigational New Drug (IND) submission, and ethics approval by the Institutional Review Board (IRB) at the University of Texas M. D. Anderson Cancer Center in Houston. The trial will be funded through a grant awarded by the United States National Institutes of Health (NIH).

The clinical trial will evaluate the safety and effectiveness of the proprietary MPCs to increase the rate and speed of bone marrow engraftment following transplantation of haematopoietic stem and progenitor cells. The trial's Principal Investigator is Professor Elizabeth J. Shpall, Director of the Cord Blood Bank and Medical Director, GMP Cellular Therapy Laboratory in the Department of Stem Cell Transplantation and Cellular Therapy.

The MPC product used in this trial will be developed under the FDA orphan drug designation recently granted to Mesoblast's United States-based sister company Angioblast Systems Inc. for treating patients with haematologic malignancies requiring increased haematopoietic stem and progenitor cell production. Orphan drug designation is reserved for therapies which are being developed for conditions affecting up to 200,000 patients annually in the US, and allows for an accelerated review process by the FDA, seven-year market exclusivity in the US upon obtaining marketing authorisation, tax benefits, and exemption from user fees.

For patients with haematologic malignancies, transplantation of haematopoietic stem and progenitor cells from the bone marrow of a healthy donor is a life-saving procedure as they rebuild bone marrow damaged and destroyed by cancer treatments. At present only about 30% of patients who could benefit from such a procedure have a genetically matched sibling, and for the rest receiving a bone marrow transplant from an unrelated donor carries a high risk of potentially fatal graft-versus-host disease.

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Umbilical cord blood is a preferred source of haematopoietic stem and progenitor cells because it has a reduced likelihood of causing graft-versus-host disease compared with bone marrow from an unrelated adult. However, the major limitation to cord blood use in adults is the limited number of haematopoietic stem and progenitor cells compared with bone marrow obtained from an adult. This often results in delay or inability to achieve satisfactory bone marrow reconstitution, resulting in increased rate of graft failure, infections, bleeding, and death.

For the past 10 years, Professor Shpall and her colleagues at the M.D. Anderson Cancer Center have been developing procedures for the ex vivo expansion of cord blood. In a recent collaborative study with Angioblast, Professor Shpall showed that the proprietary allogeneic MPCs could be used to rapidly and significantly expand the number of haematopoietic progenitor cells present in cord blood.

Professor Shpall said: "The most promising results we have generated to date are with Angioblast's proprietary off-the-shelf MPCs. Haematopoietic progenitor cells present in cord blood can be expanded by over 20-fold following simple culture with the company's proprietary MPCs.

"Angioblast's off-the-shelf MPCs provide a very reproducible and standardized product for these gravely ill patients. As important, since time is critical in these procedures, these allogeneic MPCs are available for immediate use. We hope that these advantages will translate into faster and more effective bone marrow engraftment and improved patient outcomes," added Professor Shpall.

About M. D. Anderson
The University of Texas M. D. Anderson Cancer Center in Houston ranks as one of the world's most respected centers focused on cancer patient care, research, education and prevention. M. D. Anderson is one of only 39 Comprehensive Cancer Centers designated by the United States National Cancer Institute. For five of the past eight years, M. D. Anderson has ranked No. 1 in cancer care in “America's Best Hospitals,” a survey published annually in U.S. News and World Report.

About Mesoblast
Mesoblast Limited (ASX:MSB; USOTC:MBLTY) is committed to the development of novel treatments for orthopaedic conditions, including the rapid commercialisation of a unique adult stem cell technology aimed at the regeneration and repair of bone and cartilage. Our focus is to progress through clinical trials and international regulatory processes necessary to commercialise the technology in as short a timeframe as possible. Mesoblast has the worldwide exclusive rights for a series of patents and technologies that have been developed over more than 10 years and which relate to the identification, extraction and culture of adult Mesenchymal Precursor Cells (MPCs). The Company has also acquired 39% of Angioblast Systems Inc., an American company developing the platform MPC technology for the treatment of cardiovascular diseases including repair and regeneration of blood vessels and heart muscle. Mesoblast and Angioblast are jointly funding and progressing the core technology. Mesoblast's strategy is to maximise shareholder value through both corporate partnerships and the rapid and successful completion of clinical milestones.

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