



asx announcement

MESOBLAST RECEIVES FDA CLEARANCE FOR PHASE 3 BONE MARROW TRANSPLANT TRIAL

Key points:

- Mesoblast's first Phase 3 trial submission cleared by United States Food and Drug Administration (FDA)
- Rapid clearance (within 30 days) further validation of Mesoblast's clinical, regulatory, and manufacturing capabilities
- Mesoblast's proprietary adult stem cells expand hematopoietic precursor cells in umbilical cord blood 40-fold, enabling rapid bone marrow reconstitution with lowered risk of life-threatening Graft Versus Host Disease (GVHD)
- Mesoblast's novel clinical approach could make the use of bone marrow transplantation available to all those in need of the procedure but who currently cannot find a donor, without the need for full matching
- Commercial opportunity based on potential to increase the total number of unrelated donor transplants performed globally by three- to four-fold
- This product may be the first of Mesoblast's revenue generating biologic therapies in both the US and Europe

Melbourne, Australia; 7 July 2011: Global regenerative medicine company Mesoblast Limited (ASX:MSB; OTC ADR: MBLTY) today announced that it has received clearance from the United States Food and Drug Administration (FDA) to begin a Phase 3 clinical trial for bone marrow regeneration in patients with blood cancers. FDA clearance was obtained within the 30-day minimum time period after Mesoblast filed its Phase 3 Investigational New Drug (IND) submission.

The Phase 3 trial will aim to reproduce the positive pilot trial results seen at the University of Texas MD Anderson Cancer, where accelerated neutrophil and platelet recoveries, together with excellent 100-day patient survival and low GVHD rates, occurred in patients receiving partially mismatched hematopoietic cells from umbilical cord blood expanded by Mesoblast's proprietary allogeneic or 'off-the-shelf' mesenchymal precursor cells (MPCs).

Mesoblast's off-the-shelf MPCs are being developed under an Orphan Drug Designation granted for the condition of insufficient hematopoietic stem cell production in patients with hematologic malignancies who have failed treatment with conventional chemotherapy. Such patients are in need of bone marrow transplantation using hematopoietic stem cells that are of either autologous (patient's own) or allogeneic (unrelated donor, e.g. blood from other adults or umbilical cord) origin. Mesoblast's MPCs may potentially be used for expansion of both autologous and allogeneic hematopoietic stem cells for subsequent transplantation.

According to the Center for International Blood and Marrow Transplant Research (CIBMTR), there are now over 60,000 autologous and allogeneic bone marrow transplants performed annually worldwide, a number projected to further increase due to the anticipated growth in incidence of hematologic malignancies associated with an aging population.



asx announcement

Of the total transplants performed annually worldwide, approximately 25,000 are allogeneic. This number represents less than 30 per cent of individuals who would otherwise be eligible to receive an unrelated donor bone marrow transplant because for the rest a fully matched donor cannot be found. Perfect matching is required for adult marrow transplants because of the very high risk of potentially life-threatening GVHD when unmatched transplants are performed. GVHD still occurs in as many as 60 per cent of patients who receive fully matched bone marrow transplants from unrelated adult donors.

In contrast, cord blood causes significantly less GVHD, and can be used as a partially mismatched donor source. However, the number of hematopoietic precursor cells in unexpanded cord blood is too few to enable sufficiently robust and predictable bone marrow engraftment.

Mesoblast's objective is to make available a source of unrelated donor hematopoietic precursor cells from cord blood which can be used without full matching to effect rapid bone marrow reconstitution with a low risk of GVHD. The Company believes that this would expand the use of allogeneic bone marrow transplantation to all those in need of the procedure but who currently cannot find a donor, with the potential to expand the total number of unrelated donor transplants performed by three- to four-fold.

Mesoblast's Phase 3 trial will be conducted across 50 centers in the United States, Europe and Australia, and will enrol 240 patients with hematologic malignancies undergoing unrelated donor bone marrow transplantation using matched or partially mismatched umbilical cord blood. Patients will be randomized to receive either non-expanded cord blood or cord blood expanded by Mesoblast's MPCs and containing 40-fold higher numbers of hematopoietic cells. The primary endpoint is a shortened time to neutrophil and platelet recovery in the treatment group.

Mesoblast Chief Executive, Professor Silviu Itescu, said the initiation of a Phase 3 trial was a landmark milestone for the company.

"This achievement again underscores the strength and robustness of Mesoblast's clinical, regulatory, and manufacturing capabilities. It is a significant step in bringing our broad range of adult stem cell therapeutics closer to global licensure, and to patients suffering from severe, debilitating diseases.

"We hope that this particular product will make bone marrow transplantation a more widely used and safer option for critically ill patients who undergo chemotherapy to potentially cure blood cancer.

"This product has the potential to be the first of our revenue generating biologic therapies in both the United States and Europe," Professor Itescu added.

The Phase 3 trial will be conducted together with Mesoblast's strategic alliance partner, Cephalon Inc., who will fund the trial.



asx announcement

About Orphan Drug Designation

Orphan drug designation is reserved for therapies which are being developed for conditions affecting up to 200,000 patients annually in the United States, and allows for an accelerated review process by the FDA, seven-year market exclusivity in the United States upon obtaining marketing authorization, tax benefits, and exemption from user fees.

Mesoblast Limited

Mesoblast Limited (ASX: MSB; OTC ADR: MBLTY) is a world leader in the development, manufacture, and commercialization of biologic products for the broad field of regenerative medicine. Mesoblast has the worldwide exclusive rights to a series of patents and technologies developed over more than 10 years relating to the identification, extraction, culture and uses of adult Mesenchymal Precursor Cells (MPCs). More information - www.mesoblast.com

For further information, please contact:

Julie Meldrum
Corporate Communications Director
T: + 61 (0) 3 9639 6036
E: julie.meldrum@mesoblast