U.S. FDA Approves Bone Marrow Transplant Trial Using Proprietary Cell Therapy

Mesoblast Limited announced the commencement of a Phase I/II clinical trial in the United States using patented allogeneic, or “off-the-shelf”, Mesenchymal Precursor Cells (MPCs) in up to 30 patients with hematologic malignancies undergoing bone marrow transplantation.

This follows clearance by the U.S. 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 U.S. 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 hematopoietic 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.

Umbilical cord blood is a preferred source of hematopoietic 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 hematopoietic 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, Prof 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, Prof Shpall showed that the proprietary allogeneic MPCs could be used to rapidly and significantly expand the number of hematopoietic progenitor cells present in cord blood by over 20 folds. Angioblast’s off-the-shelf MPCs provide a very reproducible and standardized product for these gravely ill patients, and are available for immediate use. In these time-critical procedures, Prof Shpall commented that these advantages will translate into faster and more effective bone marrow engraftment and improved patient outcomes.

About Mesoblast
Mesoblast Limited is an Australian biotechnology company that focuses on regenerative medicine and the development of novel treatments for orthopaedic conditions, including the rapid commercialization of a unique adult stem cell technology aimed at the regeneration and repair of bone and cartilage. 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).