mesoblast
the regenerative medicine company

asx announcement

Mesoblast Receives FDA Clearance for Phase 2 Clinical Trial of Proprietary Stem Cell Therapy in Type 2 Diabetes

Key Points:

- Phase 2 clinical trial of Mesoblast's proprietary allogeneic, or off-the-shelf, Mesenchymal Precursor Cells (MPCs) in Type 2 diabetes patients cleared to commence by United States Food and Drug Administration (FDA)
- Trial will be randomized, placebo-controlled to evaluate safety and effectiveness over 3 months of a single intravenous injection of one of three progressively increasing MPC doses in 60 Type 2 diabetes patients with elevated blood glucose levels
- MPC doses selected for clinical trial are those that have shown optimal beneficial effects on glucose lowering and on reducing markers of poor cardiac outcomes in non-human primates with obesity-related Type 2 diabetes
- Results in non-human primates have shown a dose-dependent sustained reduction in levels of fasting blood glucose and serum C-reactive protein (CRP), a major inflammatory marker which is highly predictive of risk for heart attack and cardiac death
- Results generated from this Phase 2 trial may enable Mesoblast to expand its therapeutic programs in Type 2 diabetes and metabolic diseases to address the large unmet medical needs of Type 2 diabetes patients with renal complications and the associated increase in cardiovascular morbidity and mortality.

Melbourne, Australia, and New York, USA: 31 January 2012: Mesoblast Limited (ASX: MSB) today announced that it has received clearance from the United States Food and Drug Administration (FDA) to begin a Phase 2 clinical trial of its proprietary adult stem cells for the treatment of Type 2 diabetes. Mesoblast's off-the-shelf, allogeneic, Mesenchymal Precursor Cell (MPC) product for Type 2 diabetes is the first of the company's suite of biologic therapies to be delivered via a simple intravenous injection.

The randomized, placebo-controlled Phase 2 trial will compare the effects of a single intravenous injection of one of three escalating doses of allogeneic MPCs compared with placebo in Type 2 diabetes patients with elevated blood glucose levels. The primary endpoint of the study will be to show safety of all three doses over three months of follow-up. Secondary endpoints include effects of each dose on blood glucose control over this period, changes in inflammatory markers and hormones that may be abnormal in patients with Type 2 diabetes, and effects on C-reactive protein (CRP), an established major predictor of heart attacks and death in patients with Type 2 diabetes.

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The MPC dose ranges which will be tested in the Phase 2 clinical trial are based on results from Mesoblast's preclinical studies in 17 non-human primates with obesity-related Type 2 diabetes. Key to guiding selection of optimal MPC doses for the FDA trial submission was evidence obtained over eight weeks that showed a single MPC injection reduced fasting blood glucose levels and serum CRP levels. CRP is a major inflammatory marker which is highly predictive of risk for heart attack and cardiac death when present at levels >3 mg/L in people with Type 2 diabetes.

Three-month updated results of this study, presented recently at the JP Morgan Healthcare Conference in San Francisco, showed a clear dose-dependent effect on fasting blood glucose levels following a single intravenous MPC injection. The most effective doses were 1 or 2 million MPC/kg which resulted in significant and sustained reductions in fasting blood glucose levels over three months compared to baseline, whereas no reductions in blood glucose levels were seen in controls.

The multi-center Phase 2 trial will enrol 60 patients with poorly controlled Type 2 diabetes, 45 who will be randomized to receive one of three escalating doses of 0.3, 1 or 2 million MPC/kg, and 15 to a placebo arm. Over the three-month trial period, patients will be evaluated for the primary endpoints associated with treatment safety and tolerability. Secondary endpoints of the study include assessment of glycemic control (fasting blood glucose, HbA1c, fasting insulin, C-peptide), assessment of CRP and other inflammatory markers, and possible effects on end-organ function such as kidneys and heart.

Type 2 diabetes accounts for 90-95 per cent of the 230 million people with diabetes in the industrialized world, with its prevalence increasing at an alarming rate. In the United States alone, according to data from the Centers for Disease Control and Prevention (CDC) there were 25.8 million people of all ages with diabetes in the United States in 2010 (8.3% of the United States population), of which 18.8 million people were diagnosed and 7 million were undiagnosed (Source: United States National Diabetes Fact Sheet, 2011). With a growth rate of 2.6% per year, this number is expected to increase to more than 35 million in 2020.

Despite various agents currently available for improved glucose control, patients with Type 2 diabetes continue to progress to end-stage kidney disease and need for dialysis, and remain at high risk of death from cardiovascular causes. Results generated from this Phase 2 trial may enable Mesoblast to expand its Type 2 diabetes and metabolism therapeutic programs to address the large unmet medical needs of Type 2 diabetes patients with renal complications and the associated increase in cardiovascular morbidity and mortality.

About Mesoblast Mesoblast Limited (ASX:MSB) is a world leader in commercialising biologic products for the broad field of regenerative medicine. Mesoblast has the worldwide exclusive rights for 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.

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