asx announcement

mesoblast
the regenerative medicine company

TYPE 2 DIABETES TRIAL RESULTS PUBLISHED IN DIABETES CARE

New York, USA; and Melbourne, Australia; 23 July 2015: Mesoblast Limited (ASX:MSB; USOTC: MBLTY) today announced that results of the Phase 2 trial of its intravenously-delivered Mesenchymal Precursor Cells (MPCs) for the treatment of type 2 diabetes have been published online ahead of full print in the peer-reviewed journal of the American Diabetes Association, *Diabetes Care*. The article is entitled *"Allogeneic Mesenchymal Precursor Cells in Type 2 Diabetes: A Randomized, Placebo-Controlled, Dose Escalation Safety and Tolerability Pilot Study"*.

Trial investigator and co-author, Dr Vivian A. Fonseca, Professor of Medicine and Chief of Endocrinology at Tulane University, and past President of Medicine and Science for the American Diabetes Association, said: "We are very encouraged by the safety profile and suggestion of efficacy of Mesoblast's cell therapy in patients with type 2 diabetes."

Type 2 diabetes is associated with aberrant immune activation and inflammation of various organs, including kidney, liver and fat tissues, resulting in resistance to the effects of insulin in the fat tissues, and poor glucose control. The most frequent end-organ complication involves the kidney, a condition termed diabetic nephropathy, which affects 40-50% of patients with type 2 diabetes.

Mesoblast's product candidate MPC-300-IV is being developed to treat systemic conditions of excessive inflammation, including type 2 diabetes and its complications, by down-regulating the production of pro-inflammatory cytokines and increasing the production of anti-inflammatory cytokines.

As a first step in developing an MPC-based immunomodulatory therapy for the treatment of type 2 diabetes and its complications, a study was performed which evaluated three escalating doses in patients with type 2 diabetes and poor glycemic control, without kidney disease or other organ involvement. The Phase 2 randomized, single-blind, placebo-controlled trial was conducted across 18 sites in the United States and evaluated the effects of a single intravenous infusion of 0.3, 1.0 or 2.0 million MPCs/kilogram (kg) or placebo over 12 weeks in 61 patients with a mean diabetes duration of 10 years.

The primary objective of the study was to assess the safety and tolerability of a single intravenous infusion of Mesoblast's MPC therapy (rexlemestrocel-L) in patients with type 2 diabetes. Secondary objectives included evaluation of treatment effect on glycemic control, defined by hemoglobin A1c (HbA1c) measurement, the gold-standard measure of long-term blood sugar control.

Key findings were:

Primary Endpoint of Safety

- A single MPC infusion ranging from 0.3 to 2.0 million MPCs/kg was safe and well tolerated, with no treatment-related adverse events, and no serious adverse events over the 12-week study period.
- There were no anti-human leukocyte antigen antibody immune responses against MPC donor antigens identified in any subject.

Secondary Efficacy Endpoints

- Following a single intravenous MPC infusion, there was an improvement in glycemic control as evidenced by a decrease at all timepoints after week 1 in HbA1c in MPC-treated patients compared with an increase in HbA1c in placebo-controlled patients.
- The highest dose (2.0 million MPCs/kg) showed the greatest overall reduction in HbA1c, with a peak decrease of 0.4% at 8 weeks compared with placebo ($p<0.05$).

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- In the less well-controlled subjects, as defined by a baseline HbA1c >8.0%, a 0.6% decrease in HbA1c was seen at 8 weeks in the high dose cohort compared with placebo.
- The clinical target of good glycemic control, HbA1c <7%, was achieved by 5/15 (33%) subjects in the MPC 2.0 million/kg group vs. 0/15 (0%) in the placebo group, ($p<0.05$).

Professor Paul Zimmet, Director Emeritus of the Baker IDI Heart and Diabetes Institute, and Foundation Director of the International Diabetes Institute, commented: "The treatment of type 2 diabetes with existing pharmaceutical therapies remains a major challenge to obtain optimal metabolic control for both wellbeing and reducing the risk of serious complications such as heart, kidney and eye disease, and amputations.

"This study is important and encouraging in the context of the search for better treatment for this ubiquitous and serious disorder. Given the pressing need for new developments in the treatment of type 2 diabetes, these findings are encouraging and support further studies on the possible use of adult stem cells for treating diabetes and its complications in future," he added.

Type 2 Diabetes
According to the International Diabetes Federation, there are more than 387 million people worldwide with diabetes and the number is likely to increase to 592 million in 2035. Global health spending to treat diabetes and manage its complications was estimated at 548 billion USD in 2013, projected to exceed 627 billion USD in 2035. The major individual, societal and economic burden of type 2 diabetes lies not only in hyperglycemia itself, but in the microvascular and macrovascular complications of type 2 diabetes which can lead to chronic kidney disease, cardiovascular disease, blindness and amputations in people with diabetes.

Mesoblast Limited
Mesoblast Limited (ASX: MSB; USOTC: MBLTY) is a global leader in regenerative medicine. 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 or 'off-the-shelf' cell product candidates target significantly advanced stages of diseases where there are highly unmet medical needs, including cardiovascular conditions, orthopedic disorders, immunologic/inflammatory disorders and oncology/hematology conditions. The lead therapeutic product candidates under investigation include MPC-150-IM for chronic congestive heart failure; MPC-06-ID for chronic discogenic low back pain; MSC-100-IV for acute graft versus host disease; and MPC-300-IV for biologic refractory rheumatoid arthritis, and diabetic nephropathy.

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