asx announcement

mesoblast
the regenerative medicine company

MESOBLAST'S CELL THERAPY STRENGTHENS NATIVE HEART FUNCTION IN PATIENTS WITH END-STAGE HEART FAILURE ON ASSISTED CIRCULATORY SUPPORT

Phase 2 Trial Results Presented at American Heart Association Scientific Sessions 2013

Melbourne, Australia; 19 November 2013: Patients with end-stage or New York Heart Association (NYHA) class IV heart failure who receive a surgically implanted left ventricular assist device (LVAD) heart pump to maintain circulation may obtain specific benefit to their native heart from a single dose of Mesoblast's proprietary mesenchymal precursor cells (MPCs) injected directly into their heart during surgery, a multicenter team of researchers within the United States National Institutes of Health (NIH)-funded Cardiothoracic Surgical Trials Network (CTSN), led by Icahn School of Medicine at Mount Sinai, New York, have found.

The researchers reported that a single injection into the native heart of 25 million allogeneic, or "off-the-shelf", MPCs resulted in an improvement in cardiac function at the pre-specified key efficacy endpoint of 90 days, as measured by the ability of the native heart to support the circulation with the LVAD temporarily turned down.

The results of the Phase 2 trial assessing the safety and efficacy of injecting a single dose of MPCs directly into the heart muscle of end-stage heart failure patients receiving an LVAD for either bridge-to-transplant or as a destination therapy were presented on 18 November at the American Heart Association Scientific Sessions 2013 being held in Dallas, Texas; (Abstract 19673): Intramyocardial Injection of Allogeneic Mesenchymal Precursor Cells in Left Ventricular Assist Device Patients.

The double-blind, placebo-controlled multicenter trial was performed across 11 sites in the United States, and randomized 30 end-stage heart failure patients 2:1 to receive either a single 25 million dose injection of MPCs or control media into the native heart at the time of LVAD implantation. LVAD weaning, defined as a transient reduction in pump speed for at least 20 minutes, was attempted in all patients at predetermined intervals to assess native myocardial function. Patients were followed for one year or until heart transplantation, whichever came first.

Key findings were:

1. Improved cardiac function at 90 days: at the trial's 90-day key efficacy endpoint, 50% MPC treated patients were able to tolerate being temporarily weaned from their LVADs compared with 20% controls.
2. Improved early survival: at the trial's 90-day primary endpoint, 0/20 MPC treated LVAD patients died compared with 3/10 (30%) controls.
3. Improved cardiac function was sustained over the 12-month follow-up period: 85% of MPC-treated LVAD patients were weaned successfully on multiple occasions compared with 40% of controls.
4. There were no cell-related serious adverse events noted.
5. There was no difference in human leukocyte antigen (HLA) sensitization between MPC treated and control groups.

Mesoblast Limited
ABN 68 109 431 870
www.mesoblast.com

Corporate Headquarters
Level 39
55 Collins Street
Melbourne 3000
Victoria Australia
T +61 3 9639 6036
F +61 3 9639 6030

United States Operations
505 Fifth Avenue
Third Floor
New York, NY 10017
USA
T +1 212 880 2060
F +1 212 880 2061

Asia
20 Biopolis Way
#05-01 Centros
Biopreneur 3
SINGAPORE 138668
T +65 6570 0635
F +65 6570 0176

"These clinical trial results provide important data about the safety and potential efficacy of a single MPC injection at the time of LVAD implantation," said the study's lead author Dr Deborah Ascheim, Associate Professor of Health Evidence and Policy and Cardiology at Icahn School of Medicine at Mount Sinai, who serves as Co-Director of the Data and Clinical Coordinating Center based at Mount Sinai for the NIH-sponsored CTSN who conducted the clinical trial study in collaboration with the Cardiovascular Cell Therapy Research Network.

"These mesenchymal precursor cells have shown their potential to safely facilitate early heart tissue repair in advanced heart failure. We look forward to investigating the benefits of this cell therapy further," Dr Ascheim commented.

According to the American Heart Association, 5.7 million Americans have chronic congestive heart failure, with about 10% of these with advanced or class IV heart failure.

Mesoblast Chief Executive Silviu Itescu said: "We are very encouraged by the data presented at the American Heart Association meeting by the independent researchers conducting the NIH-sponsored Phase 2 trial. NYHA class IV heart failure patients represent a substantial population for whom there are currently no alternatives other than assisted circulatory support by LVADs or heart transplantation.

"The results suggest that our MPCs may be effective in patients with advanced or NYHA class IV heart failure, and we intend to conduct further studies in this important group. This is in addition to patients with class II and III heart failure who have been shown to benefit from our MPCs in an earlier Phase 2 trial using catheter-based cell delivery."

Recently, the United States Food and Drug Administration cleared commencement of a Phase 3 trial of MPCs in patients with NYHA class II or III chronic congestive heart failure after filing of an Investigational New Drug application by Mesoblast's development and commercialization partner Teva Pharmaceutical Industries Ltd.

About Mesoblast

Mesoblast Limited (ASX:MSB;USOTC:MBLTY) is a world leader in the development of biologic products for the broad field of regenerative medicine. The Company's proprietary technologies include its Mesenchymal Precursor Cell and culture-expanded Mesenchymal Stem Cell technology platforms, Dental Pulp Stem Cells and expanded Hematopoietic Stem Cells. Mesoblast's allogeneic or 'off-the-shelf' regenerative medicine products are being developed for the treatment of conditions with significant unmet medical needs. The lead product candidates use its mesenchymal lineage cells in four major and distinct areas - systemic inflammatory conditions, cardiovascular diseases, orthopedic diseases of the spine, and oncology conditions. www.mesoblast.com

For further information, please contact:
Julie Meldrum
Corporate Communications
Mesoblast Limited
T: +61 (0) 3 9639 6036
E: julie.meldrum@mesoblast.com