asx announcement

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the regenerative medicine company

# NATIONAL INSTITUTES OF HEALTH AND MESOBLAST ENTER INTO AGREEMENT FOR 120-PATIENT TRIAL IN END-STAGE HEART FAILURE

**New York, USA, and Melbourne, Australia; 7 August 2014:** Regenerative medicine company Mesoblast Limited (ASX: MSB; USOTC: MBLTY) today announced the signing of an agreement with the United States National Institutes of Health's (NIH) National Heart, Lung and Blood Institute to collaborate on a trial using Mesoblast's proprietary adult stem cell therapy to treat patients with advanced heart failure requiring an implantable left ventricular assist device (LVAD) to maintain circulatory support. The National Institute of Neurological Disorders and Stroke, part of the NIH, and the Canadian Institutes for Health Research are also supporting this trial.

The key objectives of using Mesoblast's Mesenchymal Precursor Cells (MPCs) in end-stage heart failure patients are to improve heart muscle function sufficiently to reduce the need for LVAD support, and to reduce the long-term complications of LVAD implantation which result in recurrent hospitalizations.

Mesoblast Chief Executive, Silviu Itescu, said: "We are pleased that the NIH has chosen to evaluate Mesoblast's cell-based therapy in patients with the most advanced stage of heart failure, a complementary area to our ongoing Phase 3 program in patients with earlier stage disease."

The 120-patient study, to be conducted by the NIH-funded Cardiothoracic Surgical Trials Network, will evaluate the effects of a single injection of 150 million allogeneic, or off-the-shelf, MPCs into the hearts of patients with advanced heart failure. This new study builds on the findings published in the June issue of the American Heart Association journal *Circulation* of a double blind study in 30 patients which showed the potential benefits of a single intra-cardiac injection of 25 million MPCs in advanced heart failure and LVAD implantation.

The trial is a double-blind, placebo-controlled, 2:1 randomized design that is being conducted in more than 20 sites across the United States. The primary efficacy endpoint of the study is the number of temporary weans from LVAD tolerated over 12 months. Additionally, the study will evaluate patient survival and re-hospitalization over 12 months.

The 150 million MPC dose selected for direct cardiac injection in this second study is the same dose that is currently being evaluated in an ongoing Phase 3 trial of approximately 1,700 patients with NYHA Class II-III heart failure. The Phase 3 trial, sponsored by Mesoblast's development and commercial partner, Teva Pharmaceutical Industries Ltd., is actively enrolling patients across multiple sites in the United States. The primary efficacy endpoint of that trial is a time-to-first event analysis of heart failure-related major adverse cardiac events (HF-MACE), defined as a composite of cardiac-related death or resuscitated cardiac death, or non-fatal decompensated heart failure events.

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

**Corporate Headquarters**
Level 38
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

**Congestive heart failure**
Congestive heart failure is a chronic condition characterized by an enlarged heart and insufficient blood flow to the organs and extremities of the body. According to the 2014 American Heart Association update on heart disease and stroke statistics, congestive heart failure affected about 5.1 million people 20 years or older in the United States in 2010, with 825,000 new cases diagnosed annually. As many as 50% of heart failure patients die within five years of diagnosis. About 30-40% of heart failure patients suffer from moderate/severe class II-III heart failure with low ejection fraction, and 10% have advanced or NYHA class IV heart failure. The only treatment options for end-stage or class IV heart failure are a heart transplant or mechanical support with an LVAD. Heart transplants cannot meet the large need due to limited donor availability, and permanent LVAD support is currently limited by clinical complications.

*Source: The American Heart Association (circ.ahajournals.org/content/129/3/e28.full.pdf)*

**Mesoblast Limited**
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 highly purified, immunoselected Stro-1/Stro-3 positive Mesenchymal Precursor Cells (MPCs), culture-expanded Mesenchymal Stem Cells (MSCs), Dental Pulp Stem Cells (DPSCs), and expanded Hematopoietic Stem Cells (HSCs). Mesoblast's protein technologies are based on factors derived from its proprietary cellular platforms, including Stromal Derived Factor-1 (SDF-1). Mesoblast's allogeneic or 'off-the-shelf' regenerative medicine products are being developed for the treatment of conditions with significant unmet medical needs. Product development focus is in four major and distinct areas - systemic diseases with an underlying inflammatory and immunologic etiology; cardiac and vascular diseases; orthopedic diseases of the spine; and improving outcomes of bone marrow transplantation associated with oncology or genetic conditions. www.mesoblast.com

*For further information, please contact:*

Julie Meldrum
Global Head of Corporate Communications
Mesoblast Limited
T: +61 (0) 3 9639 6036
E: julie.meldrum@mesoblast.com