

CLINICAL TRIAL OF MESOBLAST'S CELL THERAPY IN END-STAGE HEART FAILURE PATIENTS COMPLETES ENROLLMENT

New York, USA; and Melbourne, Australia; September 28, 2017: Mesoblast Limited (ASX:MSB; Nasdaq:MESO) today announced that a multi-center team of researchers led by Icahn School of Medicine at Mount Sinai Hospital, New York, has completed enrollment of a 159-patient Phase 2b trial evaluating Mesoblast's novel allogeneic mesenchymal precursor cell (MPC) therapy for the treatment of end-stage heart failure. The trial is funded by the United States National Institutes of Health (NIH), and the Canadian Institute of Health Research (CIHR).

Due to the serious, life-threatening nature of end-stage heart failure, positive trial results using Mesoblast's product candidate MPC-150-IM in end-stage heart failure patients requiring left ventricular assist devices (LVADs) could provide support for an accelerated regulatory pathway.

There are approximately 50,000 end-stage heart failure patients in the United States and the one-year mortality rate on maximal medical therapy is over 50%^{1,2,3}. However, fewer than 5,000 of those 50,000 patients are given potentially life-saving LVADs due to the high risks of increased morbidity, recurrent hospitalizations, and inflammatory complications, including gastrointestinal bleeding, associated with these devices.

The primary efficacy endpoint of the 2:1 randomized, placebo-controlled trial will evaluate, over a six-month period, whether MPC-150-IM at a dose of 150 million cells can strengthen native heart muscle sufficiently to maintain circulation in end-stage heart failure patients once they have been weaned from an LVAD. Secondary efficacy endpoints will include rates of re-hospitalization, survival, and other quality of life measurements and will be measured over a 12-month period. If the trial's endpoints are met, MPC-150-IM therapy could facilitate far wider use of LVADs amongst end-stage heart failure patients.

Results from a 30-patient pilot study using MPC-150-IM at a substantially lower dose of 25 million cells, compared with 150 million cells used in the current Phase 2b study, have shown that the MPC-150-IM cell therapy improved native heart function, prolonged the time to first re-hospitalization following the implantation of an LVAD, and improved early survival rates in LVAD recipients⁴. These results are thought to be due to the ability of the MPCs to induce a mature blood vessel network in the ailing heart, and to reduce the damaging immune effects in both the native heart, and in its response to the presence of the LVAD.

"There is an urgent need to develop a clinical approach that could facilitate greater LVAD use in the 50,000 patients with end-stage heart failure in order to improve their dismal one-year survival rates on medical therapy alone," stated Dr Silviu Itescu, Chief Executive of Mesoblast.

"We believe that MPC-150-IM could substantially impact outcomes of patients with end-stage heart failure by reducing LVAD-related morbidity, reducing hospital re-admission rates, and improving survival. Importantly, if the native heart is strengthened sufficiently to facilitate early device explantation, this could create a bridge-to-recovery paradigm combining MPC-150-IM with temporary LVAD use."

Dr Annetine Gelijns, Chair of the Department of Population Health Science & Policy, and Edmund A. Guggenheim, Professor of Health Policy and Co-Director of InCHOIR at the Icahn School of Medicine, Mount Sinai Hospital, New York, commented, "We are very pleased to have completed the enrollment milestone in this study. We are also very excited at the potential for MPC-150-IM as an adjunctive therapy for an LVAD."

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The results of the MPC-150-IM 30-patient pilot study were published in the American Heart Association journal *Circulation*, and can be found [here](#).

About End-Stage Heart Failure

New York Heart Association Class IV heart failure affects more than 250,000 patients in the United States alone, with over 50,000 having end-stage disease. The number of end-stage heart failure patients is expected to rise in line with the 25% projected increase in total heart failure patients between 2010 and 2030^{5,6}. There are currently very few medical options for end-stage heart failure patients, as only around 2,000 heart transplants can be performed in the U.S. every year due to limited donor availability⁷. LVADs have significantly improved survival for end-stage heart failure patients, and are increasingly being used as a destination therapy^{8,9}. However, the 12-month mortality rates remain between 20% and 30% for patients implanted with LVADs, and repeated hospitalizations are very common. The complications arising from LVAD implantation have severely restricted their use by the vast majority of the end-stage heart failure population, as well as reducing its cost-effectiveness as a treatment.

¹ Jong P, et al. Effect of enalapril on 12-year survival and life expectancy in patients with left ventricular systolic dysfunction: a follow-up study. *Lancet* 2003; 361:1843-1848

² Swedberg K, et al. Long-term survival in severe heart failure in patients treated with enalapril. Ten-year follow-up of CONSENSUS I. *Eur Heart J* 1999; 20(2):136-139

³ Yancy CW, et al. 2017 ACC/AHA/HFSA Focused Update of the 2013 ACCF/AHA Guideline for the Management of Heart Failure. *Circulation*. 2017; e000-e000]

⁴ Ascheim D, et al. Mesenchymal Precursor Cells as Adjunctive Therapy in Recipients of Contemporary LVADs; *Circulation* 2014. 113.007412

⁵ Heidenreich PA, et al. Forecasting the future of cardiovascular disease in the United States: A policy statement from the American Heart Association. [Circulation 2011;123:933-944](#)

⁶ Heidenreich PA, et al. Forecasting the impact of heart failure in the United States: a policy statement from the American Heart Association. *Circ Heart Fail* 2013; 6:606-619

⁷ <http://healthresearchfunding.org/24-heart-transplant-waiting-list-statistics>

⁸ Miller LW, Guglin M. Patient selection for ventricular assist devices: a moving target. *J Am Coll Cardiol* 2013; 61: 1209-1221

⁹ Gustafsson G, Rogers JG. Left ventricular assist device therapy in advanced heart failure: patient selection and outcomes. *European Journal of Heart Failure* 2017; 19,595-602

About Mesoblast

Mesoblast Limited (ASX:MSB; Nasdaq:MESO) is a global leader in developing innovative cell-based medicines. 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, 'off-the-shelf' cell product candidates target advanced stages of diseases with high, unmet medical needs including cardiovascular conditions, orthopedic disorders, immunologic and inflammatory disorders and oncologic/hematologic conditions.

Forward-Looking Statements

This announcement includes forward-looking statements that relate to future events or our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. Forward-looking statements should not be read as a guarantee of future performance or results, and actual results may differ from the results anticipated in these forward-looking statements, and the differences may be material and adverse. Forward-looking statements include, but are not limited to, statements about: the initiation, timing, progress and results of Mesoblast's preclinical and clinical studies, and Mesoblast's research and development programs; Mesoblast's ability to advance product candidates into, enroll and successfully complete, clinical studies, including multi-national clinical trials; Mesoblast's ability to advance its manufacturing capabilities; the timing or likelihood of regulatory filings and approvals, manufacturing activities and product marketing activities, if any; the commercialization of Mesoblast's product candidates, if approved; regulatory or public perceptions and market acceptance surrounding the use of stem-cell based therapies; the potential for Mesoblast's product candidates, if any are approved, to be withdrawn from the market due to patient adverse events or deaths; the potential benefits of strategic collaboration agreements and Mesoblast's ability

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to enter into and maintain established strategic collaborations; Mesoblast's ability to establish and maintain intellectual property on its product candidates and Mesoblast's ability to successfully defend these in cases of alleged infringement; the scope of protection Mesoblast is able to establish and maintain for intellectual property rights covering its product candidates and technology; estimates of Mesoblast's expenses, future revenues, capital requirements and its needs for additional financing; Mesoblast's financial performance; developments relating to Mesoblast's competitors and industry; and the pricing and reimbursement of Mesoblast's product candidates, if approved. You should read this press release together with our risk factors, in our most recently filed reports with the SEC or on our website. Uncertainties and risks that may cause Mesoblast's actual results, performance or achievements to be materially different from those which may be expressed or implied by such statements, and accordingly, you should not place undue reliance on these forward-looking statements. We do not undertake any obligations to publicly update or revise any forward-looking statements, whether as a result of new information, future developments or otherwise.

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