

## **FDA CLEARS HEART DISEASE TRIAL AT HARVARD'S BOSTON CHILDREN'S HOSPITAL USING MESOBLAST'S CELL THERAPY IN CHILDREN WITH CONGENITAL HEART DISEASE**

**New York, USA, April 3; and Melbourne, Australia; April 4, 2017:** Boston Children's Hospital, the pediatric teaching hospital of Harvard University, and Mesoblast Limited (ASX:MSB, Nasdaq:MESO) today announced that the United States Food and Drug Administration (FDA) has cleared the commencement of a 24-patient trial combining Mesoblast's proprietary allogeneic mesenchymal precursor cells (MPCs) with corrective heart surgery in children under the age of 5 with hypoplastic left heart syndrome (HLHS). The trial is sponsored and funded by the Boston Children's Hospital with support from Bulens and Capozzi Foundation and the Ethan Lindberg Foundation.

Dr. Sitaram Emani, Boston Children's Hospital's Director of the Complex Biventricular Repair Program, Associate Professor of Surgery at Harvard Medical School, and Principal Investigator of the study, commented: "Injecting Mesoblast's MPC cells into the hypoplastic left ventricle as an adjunct to surgical rehabilitation of the left heart has the potential to promote growth and regeneration of that ventricle and recruit it back into the circulation, so that the patient has a chance to regain a normal two-ventricle circulation with improved quality of life and longevity."

The normal heart contains left and right ventricles. Children with HLHS have a functioning right ventricle, but have a small left ventricle that is incapable of supporting the systemic circulation. If left untreated, the HLHS is uniformly fatal. Current treatment - called "single - ventricle palliation" - uses the right ventricle to support the entire circulation through a series of surgeries. However, the right ventricle eventually tires out, leading to nearly 50% mortality by adolescence. This is why Dr. Emani and his colleagues have been developing strategies to rehabilitate the left ventricle and perform biventricular conversion.

When successful, biventricular conversion gives the patient a normal circulation and can prevent complications associated with single ventricle circulation including renal failure, arrhythmias, and the need for a heart transplant. With surgical rehabilitation alone, only one third of patients are able to undergo biventricular conversion. The key to successful ventricular recruitment and biventricular conversion is cardiac muscle growth and regeneration. In the randomized controlled trial, Mesoblast's product candidate MPC-150-IM will be injected into the left ventricle of children with HLHS during surgical recruitment procedures of the small ventricle with the intent of improving ventricular mass and function leading to higher likelihood of biventricular conversion.

Mesoblast's product candidate MPC-150-IM is currently being evaluated in a Phase 3 trial in up to 600 patients with New York Heart Association (NYHA) Class II/III chronic heart failure (CHF) and in a United States National Institutes of Health (NIH)-funded Phase 2b trial of 159 patients with NYHA Class IV CHF in conjunction with implantation of a left ventricular assist device (LVAD). These advanced programs and the new study in children are built on a foundation of scientific data and prior clinical trial results which support the potential for Mesoblast's immunoselected and culture-expanded MPCs to release an array of biomolecules following intra-myocardial administration that act to both dampen damaging inflammatory processes and initiate reparative processes in the failing heart through new blood vessel formation, cardiac muscle cell growth, and reduction in fibrosis and scar. In Phase 2 results, a single injection of MPC-150-IM into the myocardium of Class II/III CHF patients prevented any heart failure related hospitalizations or cardiac deaths over three years of follow-up.

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Mesoblast's Senior Clinical Development Executive and Head of Cardiovascular Diseases, Dr. Kenneth Borow, a former Harvard Medical School faculty member and cardiologist with expertise in treating pediatric patients, said: "The objective of combining Boston Children Hospital's expertise in pioneering surgical approaches to treating hypoplastic heart syndrome with the regenerative potential of our lead cardiovascular product, MPC-150-IM, is to develop a highly innovative treatment for this complex congenital condition as well as other serious and life-threatening cardiac diseases in children."

### **About Boston Children's Hospital**

Boston Children's Hospital (BCH) is the primary pediatric program of Harvard Medical School. It is ranked #1 in Pediatrics: Cardiology & Heart Surgery by U.S. News & World Report. The Boston Children's Heart Center team consists of more than 500 individual experts, a size that allows its staff to offer customized care, in more specialties, for every patient.

### **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 diseases, immune-mediated and inflammatory disorders, orthopedic disorders, and oncologic/hematologic conditions.

### **Forward-Looking Statements**

This press release 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. 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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