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MESOBLAST FILES FOR ORPHAN DRUG AND PEDIATRIC RARE DISEASE DESIGNATIONS FOR REXLEMESTROCEL-L AS TREATMENT FOR SEVERE CONGENITAL HEART DISEASE

Melbourne, Australia; November 27 and New York, USA; November 26, 2023: Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in allogeneic cellular medicines for inflammatory diseases, today announced that it has filed for orphan drug designation (ODD) and rare pediatric disease designation (RPDD) with the United States Food and Drug Administration (FDA) for its allogeneic cell therapy Revascor® (rexlemestrocel-L) in the treatment of the congenital heart disease hypoplastic left heart syndrome (HLHS). The filings were based on results from a blinded, randomized, controlled prospective trial of REVASCOR conducted at a single center in the US in 19 children with HLHS and accepted for publication in an upcoming issue of the peer reviewed *The Journal of Thoracic and Cardiovascular Surgery Open (JTCVS Open).*[1]

HLHS is a severe congenital heart disease in which the left side of the heart does not fully develop and effective pumping of oxygenated blood by the left ventricle to the rest of the body is reduced. Without immediate surgery after birth, the prognosis is dismal with HLHS overall being responsible for 25% to 40% of all neonatal cardiac mortality.[2] In the longer term, surgery that creates a two-ventricle series circulation with the left ventricle (LV) pumping blood to the body and the right ventricle pumping blood to the lungs is the ideal anatomic repair. Unfortunately, achievement of this objective is limited by the inability in most patients for the left ventricle to grow sufficiently to support the circulation to the body.

REVASCOR is an allogeneic preparation of immunoselected and culture-expanded mesenchymal precursor cells which have been shown previously to have multiple mechanisms-of-action that may be beneficial to children with HLHS including neovascularization, anti-fibrosis, anti-apoptosis, immunomodulation, reduction in inflammation, and reversal of endothelial dysfunction. In the DREAM-HF randomized sham-placebo controlled prospective trial of REVASCOR in 565 adult patients with heart failure with low ejection fraction (HFrEF), a single intramyocardial administration of REVASCOR into the left ventricle resulted in significant improvement in LV ejection fraction at 12 months,[3] indicative of strengthened overall LV systolic function.

In the HLHS trial a single intramyocardial administration of REVASCOR at the time of staged surgery resulted in significantly increased LV systolic and diastolic volumes over 12 months compared with control. These changes are indicative of clinically important growth of the small left ventricle that can help facilitate a subsequent surgical correction allowing for a normal two ventricle circulation. Improvement in left ventricular functional outcomes with REVASCOR may encourage more widespread use of surgical procedures to create a functioning left ventricle in children with HLHS resulting in reduction in long-term morbidity and mortality compared with other medical and/or surgical approaches.

The FDA has authority to grant orphan drug (OD) designation to a drug or biological product to prevent, diagnose or treat a rare disease or condition, defined as any disease or condition that affects less than 200,000 persons in the United States. An orphan drug designation (ODD) qualifies sponsors for incentives including tax credits for qualified clinical trials, exemption from user fees, and the potential for seven years of market exclusivity after approval. A rare pediatric disease designation (RPDD) demonstrates that the disease is serious or life-threatening and the manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents, and that the disease is a rare disease or condition.

About Mesoblast

Mesoblast (the Company) is a world leader in developing allogeneic (off-the-shelf) cellular medicines for the treatment of severe and life-threatening inflammatory conditions. The Company has leveraged its proprietary mesenchymal lineage cell therapy technology platform to establish a broad portfolio of late-stage product candidates which respond to severe inflammation by releasing anti-inflammatory factors that counter and modulate multiple effector arms of the immune system, resulting in significant reduction of the damaging inflammatory process.

Mesoblast has a strong and extensive global intellectual property portfolio with protection extending through to at least 2041 in all major markets. The Company's proprietary manufacturing processes yield industrial-scale, cryopreserved, off-the-shelf, cellular medicines. These cell therapies, with defined pharmaceutical release criteria, are planned to be readily available to patients worldwide.

Mesoblast is developing product candidates for distinct indications based on its remestemcel-L and rexlemestrocel-L allogeneic stromal cell technology platforms. Remestemcel-L is being developed for inflammatory diseases in children and adults including steroid refractory acute graft versus host disease, biologic-resistant inflammatory bowel disease, and acute respiratory distress syndrome. Rexlemestrocel-L is in development for advanced chronic heart failure and chronic low back pain. Two products have been commercialized in Japan and Europe by Mesoblast's licensees, and the Company has established commercial partnerships in Europe and China for certain Phase 3 assets.

Mesoblast has locations in Australia, the United States and Singapore and is listed on the Australian Securities Exchange (MSB) and on the Nasdaq (MESO). For more information, please see www.mesoblast.com, LinkedIn: Mesoblast Limited and Twitter: @Mesoblast

References / Footnotes

1. Wittenberg RE, Gauvreau K, Leighton J, Moleon-Shea M, Borow KM, Marx GR, Emani SM, Prospective randomized controlled trial of the safety and feasibility of a novel mesenchymal precursor cell therapy in hypoplastic left heart syndrome, JTCVS Open (2023), doi: https://doi.org/10.1016/j.xjon.2023.09.031.
2. Kritzmire, S. M, et al. (2022). Hypoplastic left heart syndrome. https://www.ncbi.nlm.nih.gov/books/NBK554576/#
3. Perin EC, Borow KM, Henry TD, et al. Randomized Trial of Targeted Transendocardial Mesenchymal Precursor Cell Therapy in Patients With Heart Failure. Journal of the American College of Cardiology. 2023;81(9):849-863. doi:10.1016/j.jacc.2022.11.061

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. 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 (including any future decision that the FDA may make on the BLA for remestemcel-L for pediatric patients with SR-aGVHD), 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 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.

Release authorized by the Chief Executive.

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