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MESOBLAST PARTNERS WITH BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK (BMT CTN) ON PIVOTAL TRIAL IN ADULTS WITH SR-aGVHD

Melbourne, Australia; November 22 and New York, USA; November 21, 2023: Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in allogeneic cellular medicines for inflammatory diseases, today announced that the Blood and Marrow Transplant Clinical Trials Network (BMT CTN), a body including centers responsible for approximately 80% of all US allogeneic BMTs, has entered into an agreement to develop a pivotal trial of Mesoblast's lead product candidate Ryoncil® (remestemcel-L) in the treatment of adults with steroid-refractory acute graft versus host disease (SR-aGvHD). The BMT CTN is funded by the United States National Institutes of Health (NIH).

Dr John Levine, Chair-Elect of the BMT CTN Steering Committee and Professor of Internal Medicine and Pediatrics, Icahn School of Medicine at Mount Sinai, New York said: "We are delighted to be partnering with Mesoblast in this pivotal Phase 3 trial of RYONCIL, a potentially life-saving treatment for adolescents and adults with the most severe form of aGVHD. The clinical data from children treated with RYONCIL which support this trial are very compelling."

In its September 2023 draft guidance to industry for development of agents to treat aGVHD,[1] the US Food and Drug Administration (FDA) stated that a marketing application might be supported by positive results from a single-arm trial in a population with refractory aGVHD where there are no available therapies. The trial to be developed and executed by the BMT CTN intends to evaluate RYONCIL in patients 12 and older who are refractory to both corticosteroids and a second line agent such as ruxolitinib, for whom there are no approved therapies. Prior to implementation, the clinical trial protocol will be reviewed by two independent National Heart, Lung, and Blood Institute (NHLBI)-appointed committees. Mesoblast will then submit the final protocol to FDA, as agreed at the Type A meeting with FDA in September.

Mesoblast also intends to provide FDA with additional potency assay data for RYONCIL product manufactured using the current FDA-inspected process, linking product which was used in the pediatric Phase 3 trial, which met its primary endpoint, with product which will be used in the proposed registration Phase 3 trial in adults. Showing that the product used in the pediatric and adult trials is standardized, together with data showing that future product is well characterized for commercial release, could support approval for the pediatric indication given the absence of any approved therapies for children.

Mesoblast Chief Executive Silviu Itescu said: "We are pleased to be partnering with the premier hematopoietic stem cell transplant network across the United States with the aim of having a product available for adults suffering from aGVHD and who have no other approved therapies."

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

About the Blood and Marrow Transplant Clinical Trials Network (BMT CTN)
The BMT CTN conducts rigorous multi-institutional clinical trials of high scientific merit, focused on improving survival for patients undergoing hematopoietic cell transplantation and/or receiving cellular therapies. The BMT CTN has completed accrual to 52 Phase II and III trials at more than 100 transplant centers and enrolled over 16,600 study participants. BMT CTN is funded by the National Institutes of Health (NIH,), and is a collaborative effort of 20 Core Transplant Centers/Consortia, The Center for International Blood and Marrow Transplant Research (CIBMTR), the National Marrow Donor Program (NMDP) and the Emmes Company, LLC, a clinical research organization. CIBMTR is a research collaboration between the NMDP/Be The Match and the Medical College of Wisconsin (MCW). Together, MCW, NMDP and Emmes have been providing research support to the BMT CTN since 2001, as the Network's data and coordinating center. More information about the BMT CTN can be found at www.bmtctn.net

About the National Marrow Donor Program® (NMDP)
The NMDP is the leading global partner working to save lives through cellular therapy. With 35 years of experience managing the most diverse registry of potential unrelated blood stem cell donors and cord blood units in the world, NMDP is a proven partner in providing cures to patients with life-threatening blood and marrow cancers and diseases. Through their global network, they connect centers and patients to their best cell therapy option—from blood stem cell transplant to a next-generation therapy—and collaborate with cell and gene therapy companies to support therapy development and delivery through Be The Match BioTherapies®. NMDP is a tireless advocate for the cell therapy community, working with hematologists/oncologists to remove barriers to consultation and treatment, and supporting patients through no-cost programs to eliminate non-medical obstacles to cell therapy. In addition, they are a global leader in research through the CIBMTR® (Center for International Blood and Marrow Transplant Research®)—a collaboration with Medical College of Wisconsin, investing in and managing research studies that improve patient outcomes and advance the future of care.

About the Medical College of Wisconsin (MCW)
With a history dating back to 1893, the MCW is dedicated to leadership and excellence in education, patient care, research, and community engagement. More than 1,500 students are enrolled in MCW's medical school and graduate school programs in Milwaukee, Green Bay, and Central Wisconsin. MCW's School of Pharmacy opened in 2017. A major national research center, MCW is the largest research institution in the Milwaukee metro area and second largest in Wisconsin. In the last 10 years, faculty received more than $1.5 billion in external support for research, teaching, training, and related purposes. This total includes highly competitive research and training awards from the National Institutes of Health (NIH). Annually, MCW faculty direct or collaborate on more than 3,100 research studies, including clinical trials. Additionally, more than 1,650 physicians provide care in virtually every specialty of medicine for more than 2.8 million patients annually. It has a long history in hematopoietic transplantation and cellular therapy, including operating an outcomes registry of transplantation and cellular therapy outcomes and facilitating related research since 1972.

About Emmes
Founded more than 45 years ago, Emmes is a global, full-service Clinical Research Organization dedicated to excellence in supporting the advancement of public health and biopharmaceutical innovation. The company's clients include numerous agencies and institutes of the U.S. federal government and a wide range of biotechnology, pharmaceutical and medical device companies throughout the world. To learn more about how our research is making a positive impact on human health, go to the Emmes website at www.emmes.com.

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References / Footnotes

1. United States Food & Drug Administration. Graft-versus-Host Diseases: Developing Drugs, Biological Products, and Certain Devices for Prevention or Treatment Guidance for Industry. Draft Guidance. September 2023

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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