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MESOBLAST RECEIVES IND CLEARANCE FROM FDA TO DIRECTLY PROCEED TO REGISTRATIONAL TRIAL FOR APPROVAL OF RYONCIL® IN DUCHENNE MUSCULAR DYSTROPHY

Partnering with Parent Project Muscular Dystrophy to ensure timely access to the trial for eligible patients

~15,000 children are living with DMD in the U.S.

New York, USA: April 7 and Melbourne, Australia: April 8, 2026: Mesoblast Limited (ASX:MSB; Nasdaq:MESO), global leader in allogeneic cellular medicines for inflammatory diseases, today announced that the United States Food and Drug Administration (FDA) has granted Investigational New Drug (IND) clearance to directly proceed for a registrational clinical trial evaluating Ryoncil® (remestemcel-L-rknd) in Duchenne muscular dystrophy (DMD), which affects approximately 15,000 children in the U.S.[1]

Ryoncil® is the first mesenchymal stromal cell (MSC) product approved by FDA, and the only product approved for children under age 12 with steroid-refractory acute graft-versus-host disease (SR-aGvHD)[2]. This new registrational trial builds on Ryoncil's® proven safety in children, evidence of efficacy in DMD preclinical models, and FDA-approved manufacturing process. Leveraging Ryoncil's® anti-inflammatory mechanism of action in SR-aGvHD, Mesoblast aims to reduce the inflammatory cascade characteristic of DMD, preserve muscle function, and slow disease progression.

The trial will randomize 76 patients aged 5 to 9 years to either Ryoncil® (7 infusions of 2 x 10^6 cells/kg over 9 months) or placebo, on top of standard of care. The trial's primary endpoint will be time-to-stand at nine months, a validated FDA endpoint for approval. To support the successful execution of this study, Mesoblast is collaborating with Parent Project Muscular Dystrophy (PPMD) to foster patient identification and trial awareness through proactive community engagement.

"This study represents an important step forward in potentially addressing the inflammatory component of DMD, a major driver of disease progression," said Aravindhan Veerapandiyan, MD, Director of the Comprehensive Neuromuscular Program at Arkansas Children's Hospital, and Principal Investigator of the study. "By leveraging the anti-inflammatory effects of Ryoncil, we aim to intervene at a stage where muscle tissue may still be preserved, potentially altering the trajectory of the disease."

"We are very pleased to have received clearance to proceed directly to a registrational study for DMD based on our preclinical data in DMD animal models and our extensive safety data in children with SR-aGvHD. Our experience with Ryoncil suggests that we may have a unique approach to help with this devastating disease in children," said Silviu Itescu, Chief Executive of Mesoblast

About Duchenne Muscular Dystrophy (DMD)

Duchenne Muscular Dystrophy (DMD) is a X-linked genetic disorder characterized by progressive muscle degeneration affecting the skeletal, respiratory, and cardiac muscles. It is caused by the absence of functional dystrophin, a key structural protein in muscle cells. DMD affects approximately 15,000 individuals in the United States and primarily impacts boys. Over time, deterioration of the muscle leads to loss of ambulation, respiratory failure and cardiomyopathy ultimately leading to death by the third decade.

While gene therapies that replace or increase the missing dystrophin protein are groundbreaking, they are not a complete cure. Chronic inflammation of skeletal and heart muscle remains a major underlying cause of progressive weakness, leading to loss of ambulation, reliance on wheelchair assistance and death in DMD. Corticosteroid use has improved survival but its effects plateau and long-term usage have serious side effects.

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
1114 Avenue of the Americas
4th Floor
New York, NY 10036
USA
T +1 212 880 2060
F +1 212 880 2061

Asia
21 Biopolis Road
#01-22 Nucleos (South Tower)
SINGAPORE 138567
T +65 6570 0635
F +65 6570 0176

About Parent Project Muscular Dystrophy
Parent Project Muscular Dystrophy (PPMD), a leading patient advocacy organization, to support patient identification, education, and trial awareness. The organization was founded in 1994: https://www.parentprojectmd.org/.

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 therapies from the Company's proprietary mesenchymal lineage cell therapy technology platform 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's Ryoncil® (remestemcel-L-rknd) for the treatment of steroid-refractory acute graft versus host disease (SR-aGvHD) in pediatric patients 2 months and older is the first FDA-approved mesenchymal stromal cell (MSC) therapy. Please see the full Prescribing Information at www.ryoncil.com.

Mesoblast is committed to developing additional cell therapies for distinct indications based on its remestemcel-L and rexlemestrocel-L allogeneic stromal cell technology platforms. Ryoncil® is being developed for additional inflammatory diseases including SR-aGvHD in adults and biologic-resistant inflammatory bowel disease. Rexlemestrocel-L is being developed for heart failure and chronic low back pain. The Company has established commercial partnerships in Japan, Europe and China.

About Mesoblast intellectual property: Mesoblast has a strong and extensive global intellectual property portfolio, with over 1,000 granted patents or patent applications covering mesenchymal stromal cell compositions of matter, methods of manufacturing and indications. These granted patents and patent applications provide commercial protection extending through to at least 2044 in all major markets.

About Mesoblast manufacturing: 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 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. https://cureduchenne.org/about/what-is-duchenne/#:~:text=Prevalence,Cardiac%20Dysfunction
2. Please see the full Prescribing Information at www.ryoncil.com

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, manufacturing activities and product marketing activities, if any; the commercialization of Mesoblast's RYONCIL for pediatric SR-aGVHD and any other 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.

For more information, please contact:

Corporate Communications / Investors
Paul Hughes
T: +61 3 9639 6036

Media – Global
Rubenstein
Caroline Nelson
T: +1 703 489 3037
E: cnelson@rubenstein.com

Media – Australia
BlueDot Media
Steve Dabkowski
T: +61 419 880 486
E: steve@bluedot.net.au