asx announcement

mesoblast
the regenerative medicine company

# CONSISTENT OUTCOMES USING RYONCIL™ AS FIRST-LINE TREATMENT OR SALVAGE THERAPY IN 309 CHILDREN WITH STEROID-REFRACTORY ACUTE GVHD

**Melbourne, Australia; February 24, 2020; and New York, USA; February 23, 2020:** Mesoblast Limited (ASX:MSB; Nasdaq:MESO) today announced that aggregated results from 309 children treated with Ryoncil™ (remestemcel-L) were presented at the American Society for Transplantation Cellular Therapy and the Center for International Blood & Bone Marrow Transplant Research (TCT) meeting in Orlando, Florida on February 22. The data showed that treatment with RYONCIL across three separate trials resulted in consistent treatment responses and survival outcomes in children with steroid-refractory acute graft versus host disease (SR-aGVHD).

Key findings and conclusions were:

- Consistent safety and efficacy were observed across the continuum from first-line treatment after steroid failure through the most challenging patients who received RYONCIL as salvage after exhausting all other options.
- In the aggregated dataset, 204 of the 309 (66%) patients achieved an overall response at Day 28 following a four-week course of RYONCIL.
- Results were consistent across all grades of disease, including most severe (IBMTR Grade C/D or Glucksberg Grade 3/4).
- In the most severe patients (Grade C/D), who accounted for 82% of all treated patients, Day 28 overall response was 65%.
- Overall response at Day 28 was strongly predictive of survival at Day 100 and Day 180.
- Day 28 responders were more than twice as likely to survive as non-responders (84% vs 39% at Day 100, and 83% vs 38% at Day 180).
- RYONCIL was well tolerated with no infusion-related toxicity and no identified safety concerns.

Mesoblast Chief Medical Officer Dr Fred Grossman said: “These aggregated data from three studies demonstrate consistent efficacy and safety of RYONCIL in children suffering from steroid refractory acute graft versus host disease. If approved, RYONCIL has the potential to be an effective and safe therapy to improve survival outcomes in the most vulnerable population of children with severe forms of this disease who can have mortality rates as high as 90 percent.”

In January, Mesoblast filed a Biologics License Application (BLA) to the United States Food and Drug Administration (FDA) for RYONCIL for the treatment of children with steroid-refractory aGVHD. The Company has requested Priority Review of the BLA by the FDA under the product candidate’s existing Fast Track designation. If approved, RYONCIL is expected to be launched in the US in 2020.

**About Acute GVHD**

Acute GVHD occurs in approximately 50% of patients who receive an allogeneic bone marrow transplant (BMT). Over 30,000 patients worldwide undergo an allogeneic BMT annually, primarily during treatment for blood cancers, and these numbers are increasing.[1] In patients with the most severe form of acute GVHD (Grade C/D or III/IV) mortality is as high as 90% despite optimal institutional standard of care.[2,3]. There are currently no FDA-approved treatments in the US for children under 12 with SR-aGVHD.


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## About Ryoncil™

Mesoblast's lead product candidate, RYONCIL, is an investigational therapy comprising culture-expanded mesenchymal stem cells derived from the bone marrow of an unrelated donor. It is administered to patients in a series of intravenous infusions. RYONCIL is believed to have immunomodulatory properties to counteract the inflammatory processes that are implicated in SR-aGVHD by down-regulating the production of pro-inflammatory cytokines, increasing production of anti-inflammatory cytokines, and enabling recruitment of naturally occurring anti-inflammatory cells to involved tissues.

### References

1. Niederwieser D, Baldomero H, Szer J. (2016) Hematopoietic stem cell transplantation activity worldwide in 2012 and a SWOT analysis of the Worldwide Network for Blood and Marrow Transplantation Group including the global survey.
2. Westin, J., Saliba, RM., Lima, M. (2011) Steroid-refractory acute GVHD: predictors and outcomes. Advances in Hematology.
3. Axt L, Naumann A, Toennies J (2019) Retrospective single center analysis of outcome, risk factors and therapy in steroid refractory graft-versus-host disease after allogeneic hematopoietic cell transplantation. Bone Marrow Transplantation.

## About Mesoblast

Mesoblast Limited (ASX: MSB; Nasdaq: MESO) is a world leader in developing allogeneic (off-the-shelf) cellular medicines. The Company has leveraged its proprietary mesenchymal lineage cell therapy technology platforms to establish a broad portfolio of commercial products and late-stage product candidates. Mesoblast's proprietary manufacturing process yields 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 filed a Biologics License Application to the United States Food and Drug Administration (FDA) to seek approval of its product candidate Ryoncil™ (remestemcel-L) for steroid-refractory acute graft versus host disease (acute GvHD). Remestemcel-L is also being developed for other rare diseases. Mesoblast is completing Phase 3 trials for its rexlemestrocel product candidates for advanced heart failure and chronic low back pain. If approved, RYONCIL is expected to be launched in the United States in 2020 for pediatric steroid-refractory acute GVHD. 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

## Mesoblast's 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 timing, progress and results of Mesoblast's preclinical and clinical studies; Mesoblast's ability to advance product candidates into, enroll and successfully complete, clinical studies; the timing or likelihood of regulatory filings and approvals; 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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