



# Global Leader in Allogeneic Cellular Medicines for Inflammatory Diseases

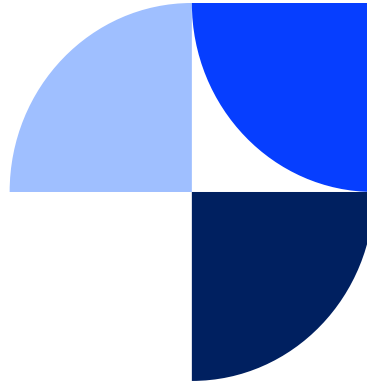
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Presentation at ISCT

Ryoncil® - The First FDA Approved Mesenchymal Stromal Cell Therapy

March 2025

ASX: MSB; Nasdaq: MESO



## CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This presentation 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. All statements other than statements of historical facts contained in this presentation are forward-looking statements. Words such as, but not limited to, “believe,” “expect,” “anticipate,” “estimate,” “intend,” “plan,” “targets,” “likely,” “will,” “would,” “could,” and similar expressions or phrases identify forward-looking statements. We have based these forward-looking statements largely on our current expectations and future events , recent changes in regulatory laws, and financial trends that we believe may affect our financial condition, results of operation, business strategy and financial needs. These statements may relate to, but are not limited to: expectations regarding the safety or efficacy of, or potential applications for, Mesoblast’s adult stem cell technologies; expectations regarding the strength of Mesoblast’s intellectual property, the timeline for Mesoblast’s regulatory approval process, and the scalability and efficiency of manufacturing processes; expectations about Mesoblast’s ability to grow its business and statements regarding its relationships with current and potential future business partners and future benefits of those relationships; statements concerning Mesoblast’s share price or potential market capitalization; and statements concerning Mesoblast’s capital requirements and ability to raise future capital, among others. 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 presentation together with our financial statements and the notes related thereto, as well as the 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, include, without limitation: risks inherent in the development and commercialization of potential products; uncertainty of clinical trial results or regulatory approvals or clearances; government regulation; the need for future capital; dependence upon collaborators; and protection of our intellectual property rights, among others. 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.

*Mesoblast is committed to bringing to market innovative off-the-shelf allogeneic cellular medicines to treat serious and life-threatening inflammatory illnesses*

## Our Mission



# Global leader in allogeneic cellular medicines for inflammatory diseases

- ✓ World leader in developing allogeneic (off-the-shelf) cellular medicines for the treatment of severe and life-threatening inflammatory conditions
- ✓ Locations in Australia, the United States and Singapore
- ✓ Listed on the ASX (MSB) and NASDAQ (MESO)
- ✓ Developing product candidates for distinct indications based on its remestemcel-L and rexlemestrocel-L stromal cell technology platforms
- ✓ Extensive global intellectual property portfolio with protection extending through to at least 2041 in all major markets
- ✓ FDA-inspected commercial scale manufacturing process and facilities

**ONE**  
product FDA  
approved

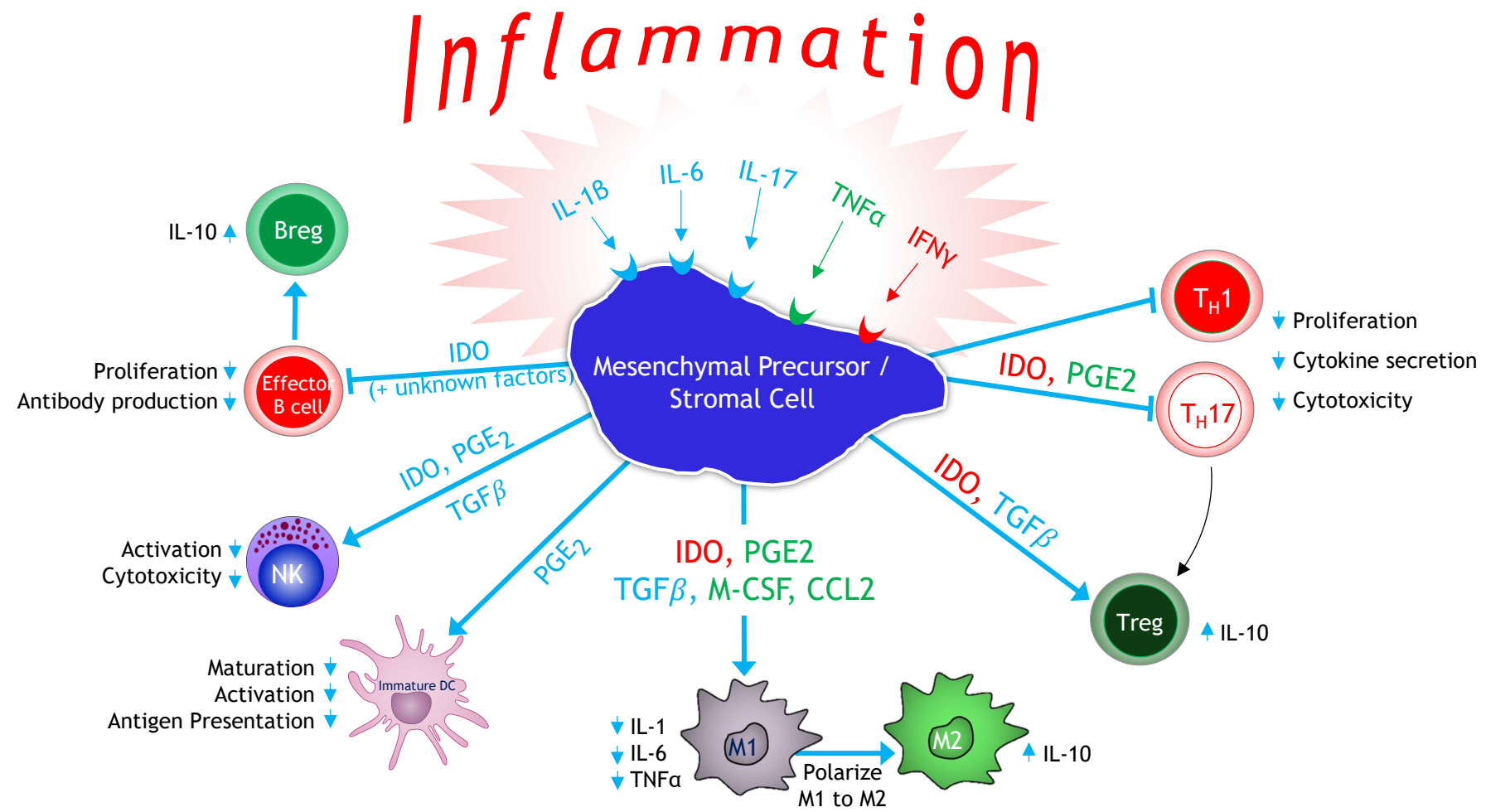
**Ryoncil**  
(remestemcel-L-rknd)

more than  
**1,100**  
patents &  
applications

Phase 3 trials  
in **TWO**  
major  
indications







# Platform technology: shared mechanism of action across our products

Our mesenchymal precursor/stromal cells respond to and are activated by multiple inflammatory cytokines through surface receptors, resulting in orchestration of an anti-inflammatory cascade



Source: data on file

# Mesoblast allogeneic Mesenchymal Precursor / Stromal Cell portfolio

Product	Indication	Phase 2	Phase 3	Regulatory Filing	Approved
<b>RYONCIL® remestemcel-L</b>	Pediatric SR-aGvHD				
	Adult SR-aGvHD				
<b>RYONCIL® remestemcel-L</b>	IBD / Crohn's				
<b>REVASCOR® rexlemestrocel-L (STRO3+)</b>	Pediatric HLHS				
	Adult HFrEF End-stage				
	Adult HFrEF Class II/III				
<b>Rexlemestrocel-L (STRO3+)</b>	CLBP				

SR-aGvHD = Steroid-Refractory Acute Graft versus Host Disease;  
IBD = Inflammatory Bowel Disease; HLHS = Hypoplastic Left Heart Syndrome  
HFrEF = Heart Failure with Reduced Ejection Fraction;  
CLBP = Chronic Low Back Pain;

*This chart is figurative and does not purport to show individual trial progress within a clinical program*

Notes:

- JCR Pharmaceuticals Co., Ltd. (JCR), has the right to develop mesenchymal stromal cells (MSCs) in certain fields for the Japanese market, including for the treatment of hematological malignancies, such as Graft vs Host Disease, and for hypoxic ischemic encephalopathy (HIE).
- Grünenthal has an exclusive license to develop and commercialize rexlemestrocel-L for chronic low back pain in Europe and Latin America/Caribbean.
- Tasly Pharmaceuticals has exclusive rights for rexlemestrocel-L for the treatment or prevention of chronic heart failure in China.

ersonal use only



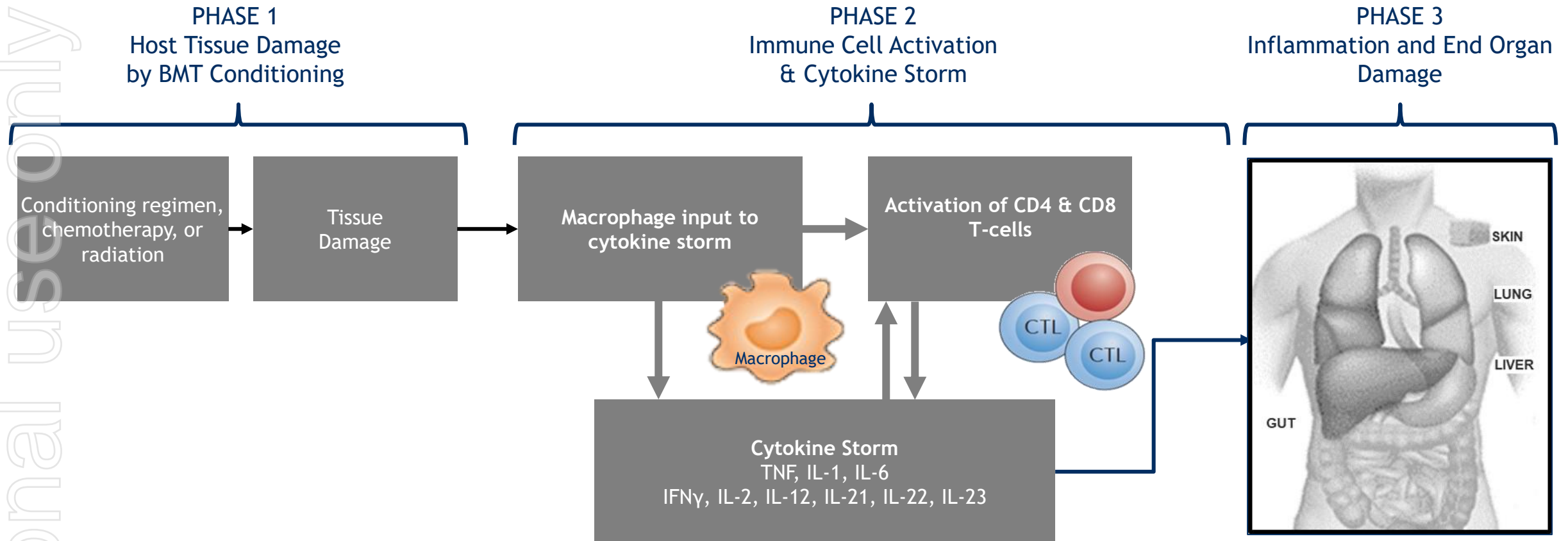
# Ryoncil<sup>®</sup>

(remestemcel-L-rknd)

**First mesenchymal  
stromal cell (MSC) therapy  
approved by FDA**



# Acute graft versus host disease (aGvHD) is a serious and potentially fatal complication of allogeneic bone marrow transplantation (BMT)





# Opportunity to address critical unmet need in children 2 months and older, including adolescents & teenagers with SR-aGVHD

~1,500

**Children & adolescents  
undergo allogeneic BMT  
in US annually**

>30,000 allogeneic BMTs performed globally (>20K US/EU) annually, ~20% pediatric<sup>2,3</sup>

Approx. 10,000 allogeneic BMTs performed in the US annually

Corticosteroids are first-line therapy for aGVHD

Acute GvHD occurs in ~50% of patients<sup>1</sup> with approx. half failing to respond to steroids

RYONCIL is the only approved therapy for SR-aGVHD in children 2 months and older

~50%

**Incidence of acute  
GvHD**

SR-aGVHD has high mortality<sup>1,4</sup> and significant extended hospital stay costs<sup>2</sup>

~375 pts/year  
with SR-aGVHD

1. Westin, J., Saliba, RM., Lima, M. (2011) Steroid-refractory acute GVHD: predictors and outcomes. Advances in Hematology. 2. 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. 3. HRSA Transplant Activity Report, CIBMTR, 2020 4. 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.

# Ryoncil® for treating pediatric patients with SR-aGvHD

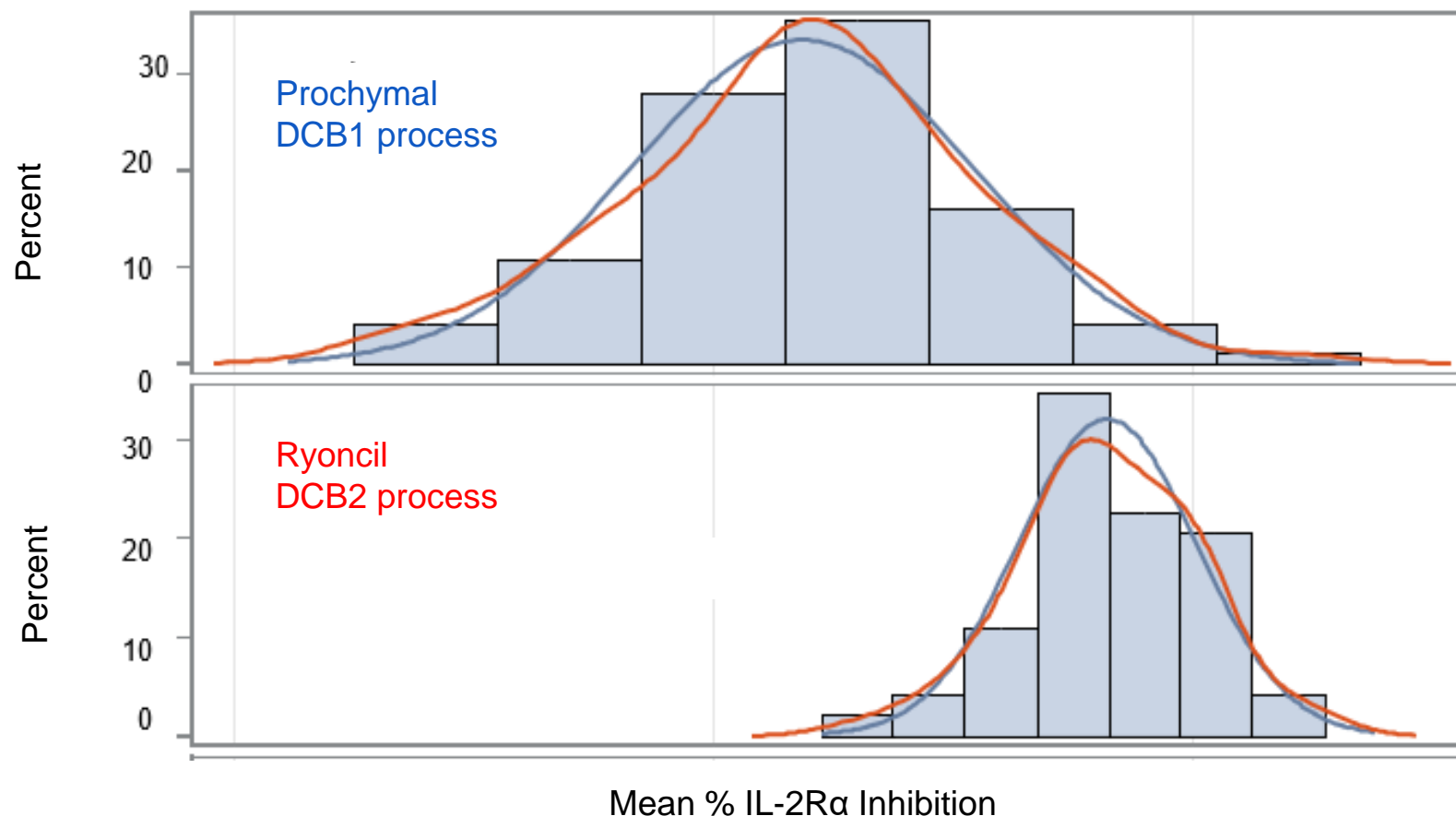
The recommended dosage of Ryoncil® for treatment of pediatric SR-aGvHD is  $2 \times 10^6$  MSC/kg body weight per intravenous infusion given twice per week for 4 consecutive weeks



Ryoncil®  
(remestemcel-L-rknd)

# Improvements in manufacturing process give rise to - a product with greater potency

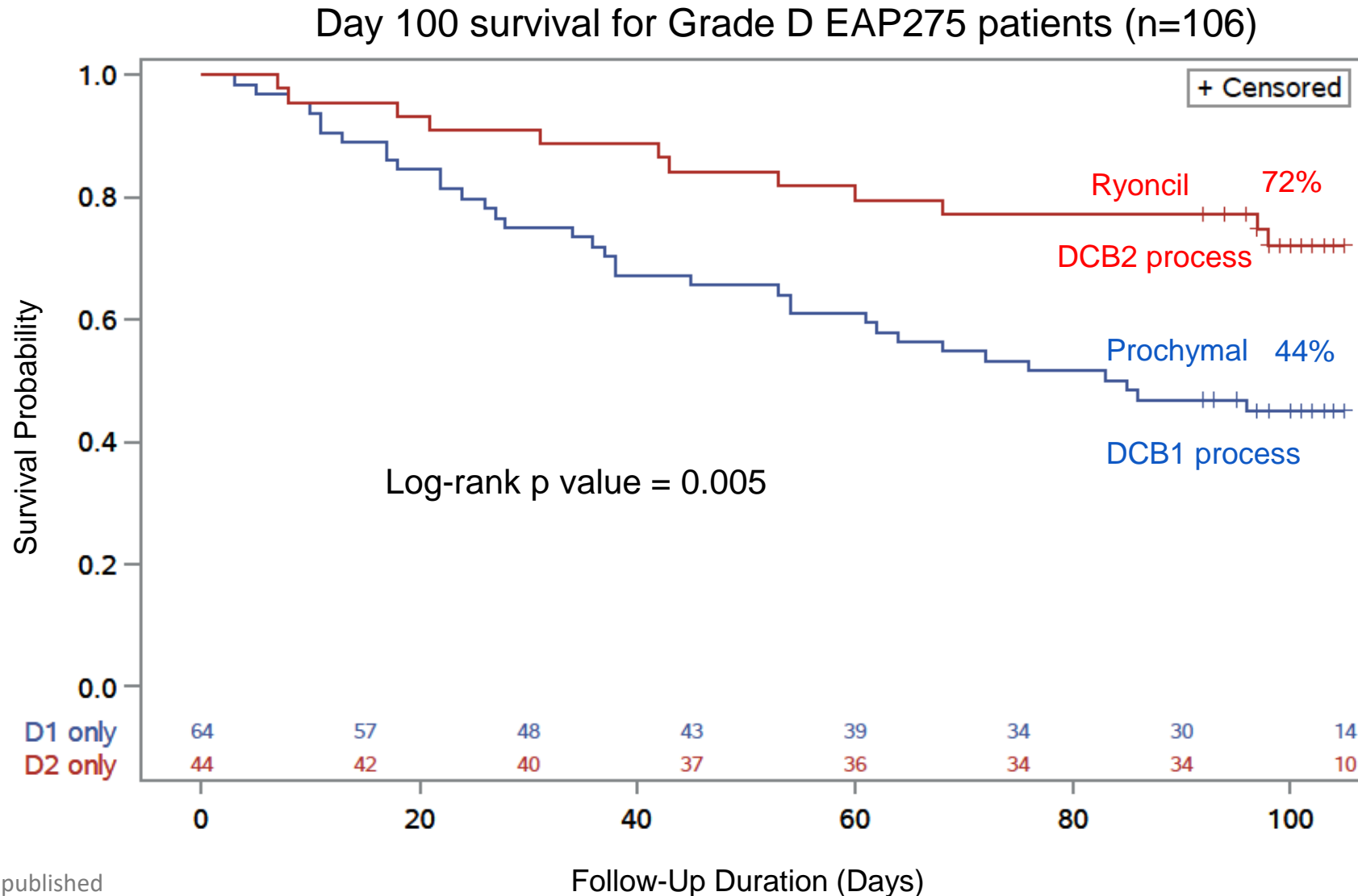
Potency for Drug Product Lots used in EAP275



100% of Ryoncil lots released meet FDA approved potency release criteria, compared with less than two thirds of Prochymal lots used in adult GvHD trial 280

# Improvements in manufacturing process give rise to Ryoncil<sup>®</sup> (remestemcel-L-rknd)

- a product associated with improved survival in SR-aGvHD



# Ryoncil® delivered high overall response rates at Day 28, a measure that predicts survival in aGvHD<sup>1</sup>

**MSB-GVHD001<sup>2,3</sup>**

(n=54)

Single-arm, multi-center  
Phase 3

**Overall Response Rate  
at Day 28**

95% CI 56.4, 82.0

**70%**

**SR-GvHD severity<sup>4</sup> at baseline  
in GVHD001:**

Grade B: 11%

Grade C: 43%

Grade D: 46%

**RYONCIL treatment was not discontinued or interrupted in any  
patient for any laboratory abnormality, and the full course was  
completed without interruption in more than 85% of patients**

Full Prescribing Information at [www.ryoncil.com](http://www.ryoncil.com)

1. MacMillan ML, Holtan SG, Rashidi A, et al. "Pediatric acute GVHD: clinical phenotype and response to upfront steroids." Bone marrow transplantation vol. 55,1 (2020): 165-171; 2.NCT02336230; 3. Kurtzberg, J. et al. A Phase 3, Single-Arm, Prospective Study of Remestemcel-L, Ex Vivo Culture-Expanded Adult Human Mesenchymal Stromal Cells for the Treatment of Pediatric Patients Who Failed to Respond to Steroid Treatment for Acute Graft-versus-Host Disease. Biol Blood Marrow Transplant 26 (2020) 845-854

<https://doi.org/10.1016/j.bbmt.2020.01.018>; 4. International Blood and Marrow Transplantation Registry Severity Index Criteria (IBMTR)

Abbreviations: CI = confidence interval

# Ryoncil® long-term survival free from aGvHD

Long-term follow-up of Ryoncil by the Center for International Blood and Marrow Transplant Research (CIBMTR)

Children from GVHD001

N=51

88% Grade C/D

Year 2 Survival:

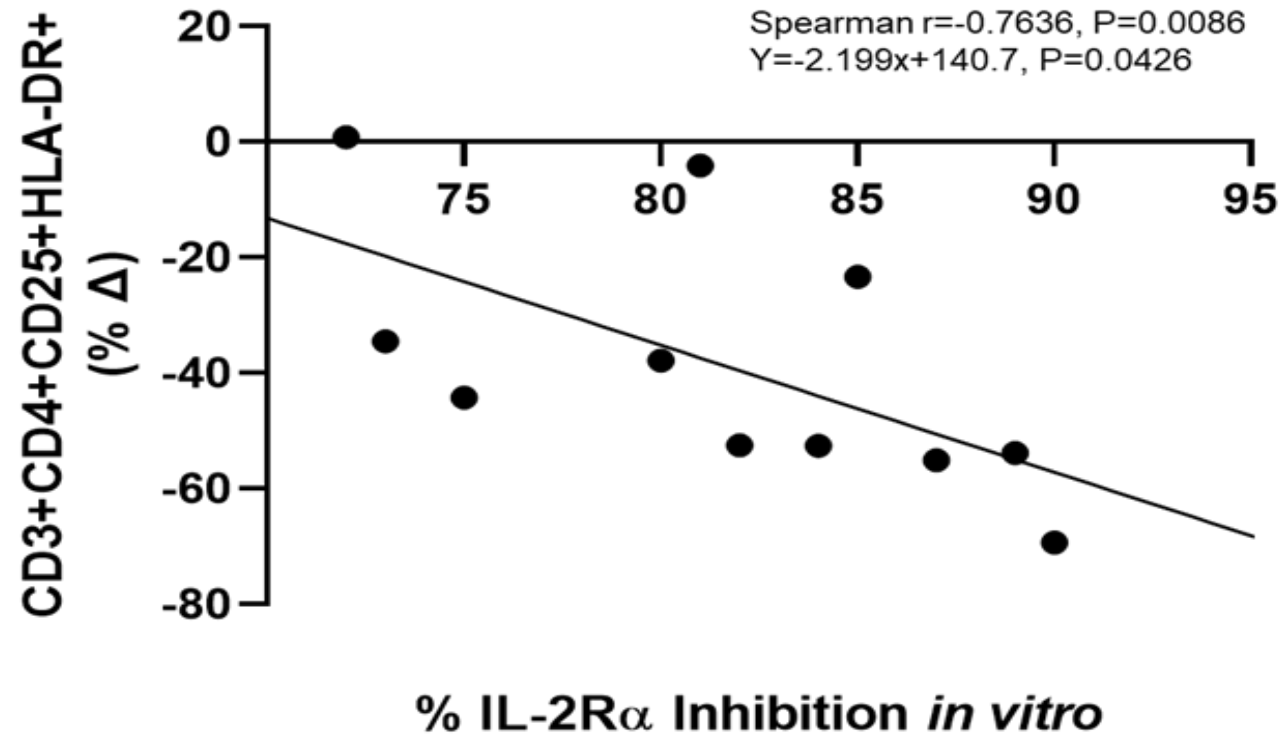
**51%**

Year 4 Survival:

**49%**

**Only 14% (N=7) died due to aGvHD through 4 years**

# Ryoncil® lot potency, measured by IL-2R $\alpha$ Inhibition Assay, correlates with reduction in circulating levels of activated T cells in patients with SR-aGVHD: evidence for immunomodulatory mechanism of action (MOA)



Supporting evidence for Ryoncil immunomodulatory MOA from pharmacodynamic analysis of blood samples in 40 subjects in GVHD001:

- Circulating levels of CD3+CD4+CD25+HLA-DR+ activated T cells were reduced by 64% at Day 180 as compared to baseline.
- Tumor necrosis factor receptor 1 (TNFR1) levels were reduced by 79% at Day 180 as compared to baseline.
- Suppressor of tumorigenesis 2 (ST2) levels, a biomarker of gut inflammation, were reduced by 75% at Day 180 as compared to baseline.



Ryoncil<sup>®</sup>  
(remestemcel-L-rknd)

## NOW APPROVED

Mesoblast is excited to announce that an innovative new treatment option is now approved for pediatric patients. Read the press release to learn more about this clinical advancement.

Visit [RYONCIL.com](http://RYONCIL.com)

**RYONCIL<sup>®</sup> is the first FDA-approved, off-the-shelf cell therapy for children aged 2 months and older, including adolescents and teenagers, with steroid-refractory acute graft versus host disease (SR-aGvHD), a life-threatening condition with high mortality rates.<sup>1</sup>**



Press Release available at [www.mesoblast.com](http://www.mesoblast.com)

1. Please see the full Prescribing Information at [www.ryoncil.com](http://www.ryoncil.com)

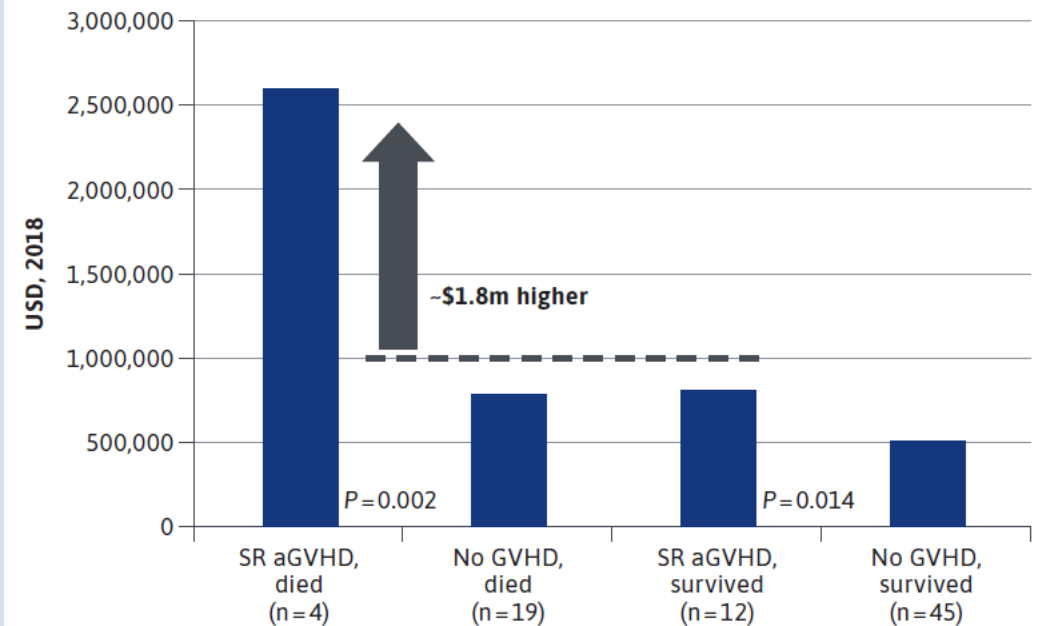
# High cost of treating child who dies from SR-aGvHD

The cost of treating a child who dies of SR-aGVHD within 12 months of transplant is:

- Approximately \$2.5M
- \$1.8M higher than for those with SR aGvHD who remain alive<sup>1</sup>

**FIGURE 2**

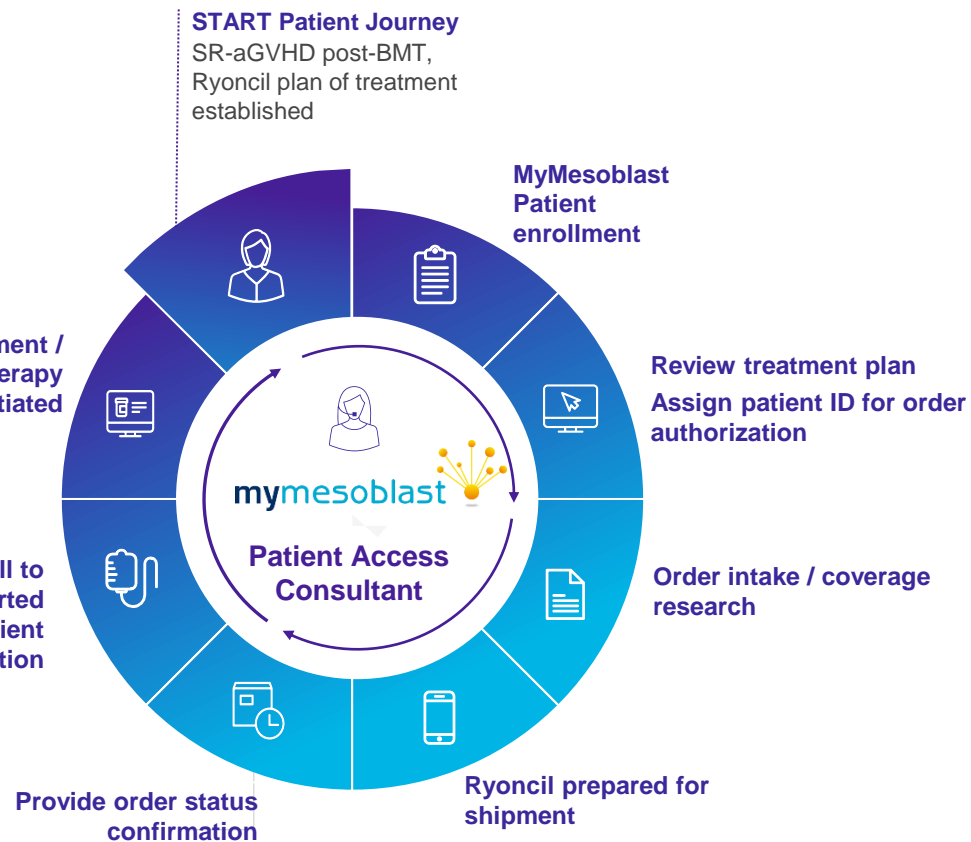
Mean Total Per-Patient Medical Costs for Patients With or Without All-Cause Mortality Within 12 Months



aGVHD = acute graft-versus-host disease; SR = steroid refractory.

# MyMesoblast Mandatory Hub

To assist patients with insurance coverage, financial assistance, and access programs, ensuring that no patient is left behind in receiving this potentially life-saving therapy, a comprehensive patient services hub has been established



# Availability of Ryoncil® for pediatric SR-aGvHD in the U.S. in March 2025

Staged approach  
based on transplant  
centers with highest  
volume and  
experience with  
Ryoncil® product

Targeted sales force  
with experience in bone  
marrow transplant  
centers

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15 highest volume  
centers account for  
~50% of patients

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Targeting 45 highest  
volume centers / 80% of  
patients



Thank You