mesoblast
the regenerative medicine company

Global Leader in Allogeneic Cellular Medicines for Inflammatory Diseases

Financial and Operational Highlights for the Half Year Ended December 31, 2019

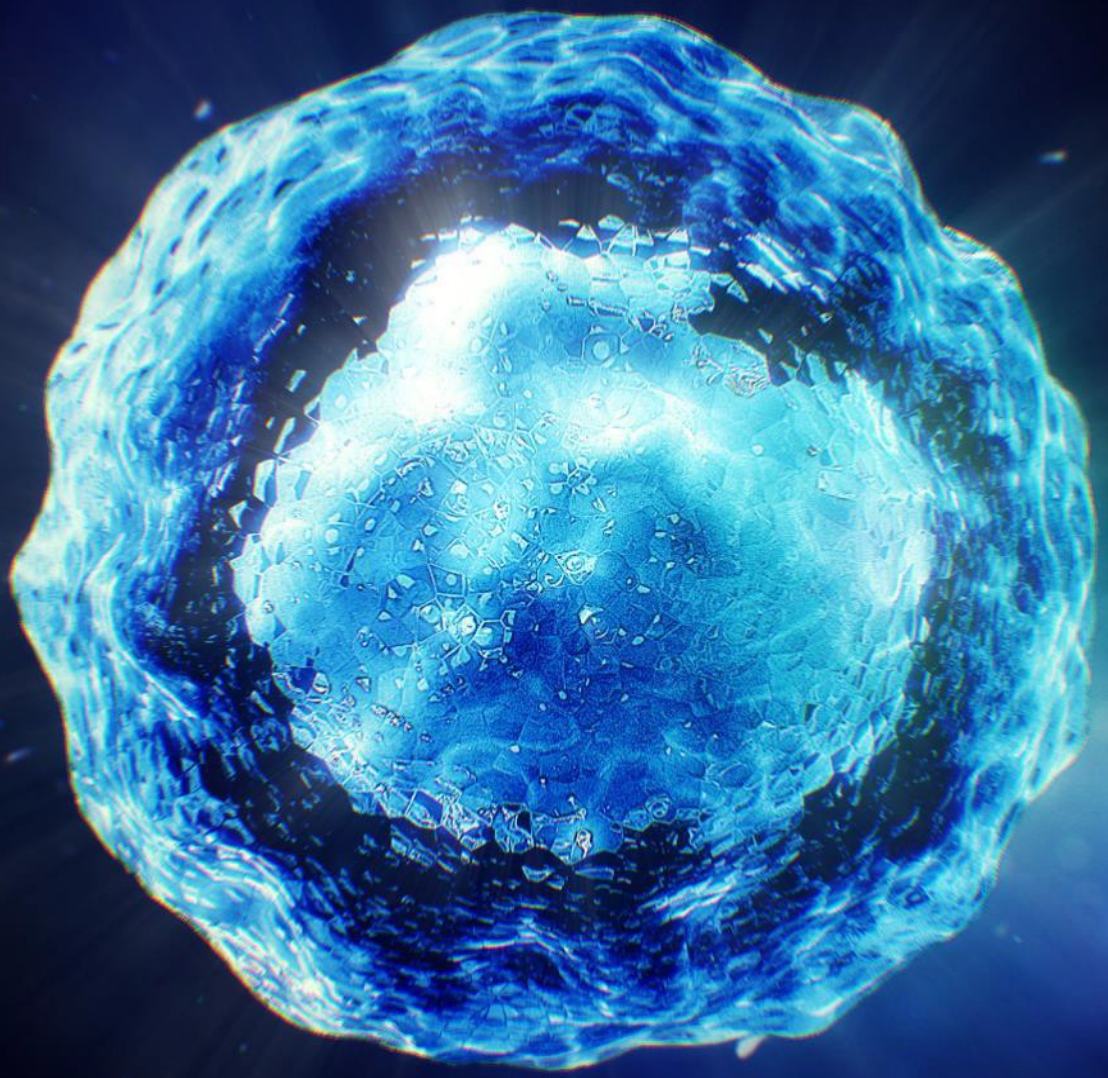
ASX: MSB; Nasdaq: MESO

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.

Our Mission

Mesoblast is committed to bringing to market innovative cellular medicines to treat serious and life-threatening illnesses

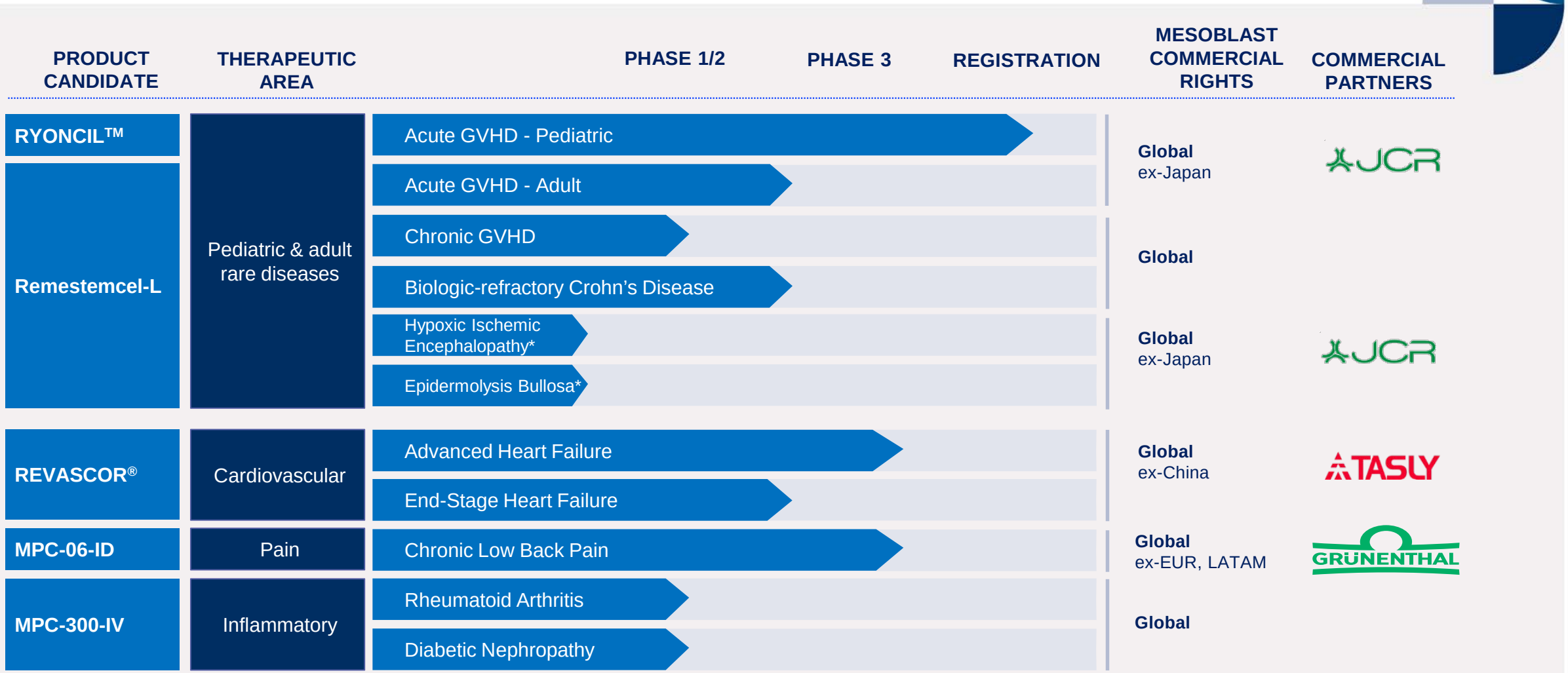


Allogeneic Cellular Medicines for Inflammatory Diseases

Innovative Technology Platform	Lead Product Candidate	Late Stage Product Pipeline
▪ Allogeneic mesenchymal precursor cells (MPCs) and their progeny (MSCs) ▪ Well characterized immunomodulatory mechanisms of action ▪ Targeting severe and life threatening inflammatory conditions ▪ Underpinned by extensive, global intellectual property estate	▪ RYONCIL™ (remestemcel-L) BLA filed with US FDA for pediatric steroid-refractory acute GVHD ▪ Targeted US commercial team for potential launch ▪ If approved, launch planned for 2020 ▪ Industrial-scale manufacturing in place to meet commercial demand ▪ Continued growth in royalty revenues from Japan sales of licensee product for acute GVHD[1]	▪ Lifecycle expansion of remestemcel-L for pediatric and adult rare diseases ▪ Two additional product candidates in Phase 3 trials, heart failure and back pain, with near-term US readouts ▪ Back pain product candidate partnered in Europe & Latin America with Grünenthal ▪ Heart failure product candidate partnered in China with Tasly

1. Licensee JCR Pharmaceuticals Co., Ltd. received the first full PMDA approval for an allogeneic cellular medicine in Japan and markets this product under its trademark, TEMCELL® Hs Inj.

Mesoblast Pipeline



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

* Mesoblast has the right to use data generated by JCR Pharmaceuticals Co Ltd in Japan to support its development and commercialization plans for remestemcel-L in the US and other major healthcare markets, including for GVHD, EB, and HIE

Partnerships and License Agreements

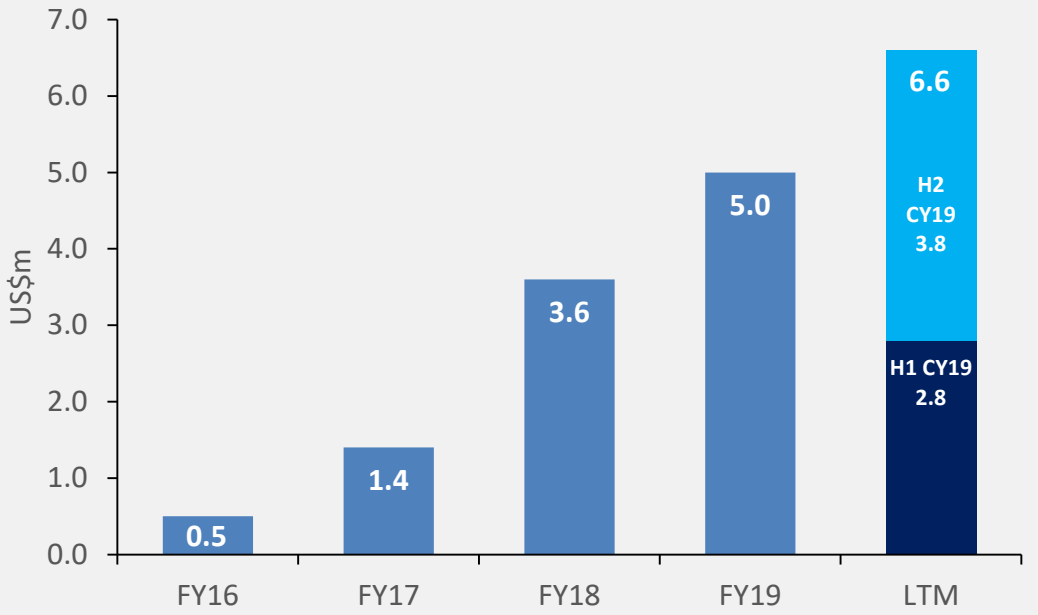
Commercial products

TEMCELL®

ACUTE GRAFT VERSUS HOST DISEASE + OTHER INDICATIONS

- Rights to use MSC technology for acute GVHD in Japan
- US$6.6 million royalties received in last 12 months
- Product adoption and reimbursement informs Mesoblast US commercial strategy for RYONCIL in acute GVHD
- US addressable market for acute GVHD in children and adults is ~ eight-fold larger than Japan due to greater patient numbers, incidence and pharmacoeconomics
- License expanded to cover:
  - Epidermolysis bullosa (EB), a highly debilitating and sometimes lethal skin disease; and
  - Hypoxic ischemic encephalopathy (HIE) in newborns, a disease with a high frequency of mortality

ANNUAL REVENUE FROM TEMCELL ROYALTIES IN JAPAN



Success of TEMCELL by Mesoblast Licensee JCR Informs Potential US Market for RYONCIL

TEMCELL® Hs. Inj. is a registered trademark of JCR Pharmaceuticals Co Ltd.

Partnerships and License Agreements

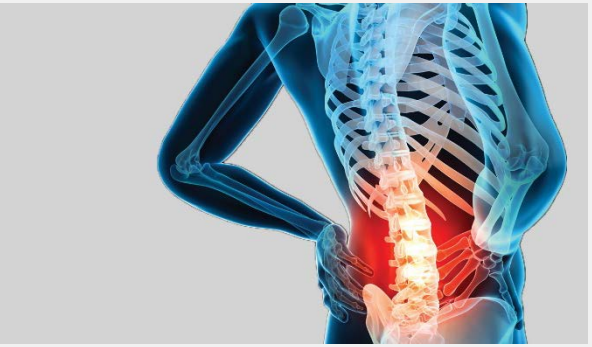
Products in development

MPC-06-ID

- Strategic partnership to develop and commercialize MPC-06-ID in Europe & Latin America
- Mesoblast will receive up to US$150 million in upfront and milestone payments prior to product launch
- Milestone payments could exceed US$1 billion depending on patient adoption
- Mesoblast will also receive tiered double digit royalties on product sales

CHRONIC LOW BACK PAIN - DEGENERATIVE DISC

PREVALENCE EUROPE
~7.0 MILLION

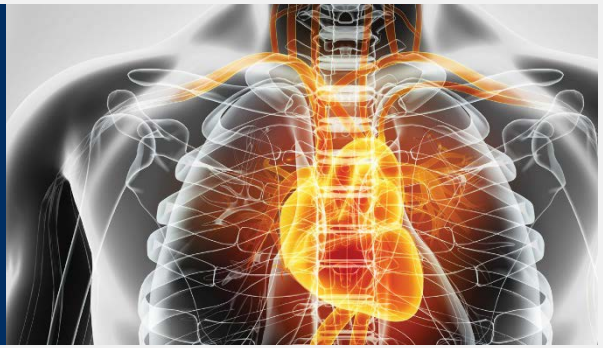


REVASCOR™

- Exclusive cardiovascular rights in China
- Mesoblast received US$40 million in an upfront payment and equity placement
- Eligible for additional milestones and royalties

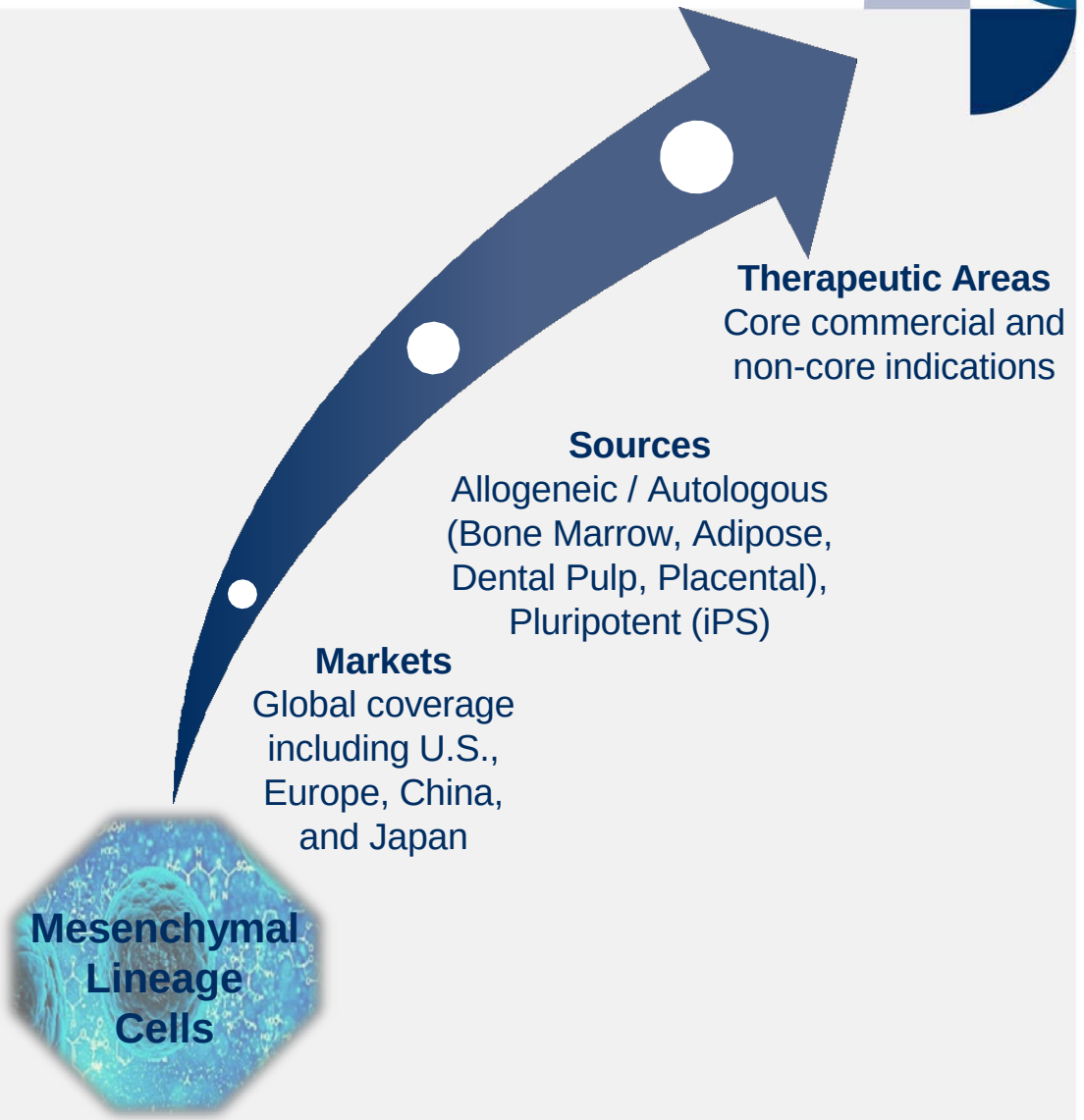
CARDIOVASCULAR – CHRONIC HEART FAILURE

PREVALENCE CHINA
~4.5 MILLION



Global IP Estate Provides Substantial Competitive Advantage

- ~1,000 patents and patent applications (68 patent families) across all major jurisdictions
- Covers composition of matter, manufacturing, and therapeutic applications of mesenchymal lineage cells
- Provides strong global protection in areas of our core commercial focus
- Grant rights to third parties who require access to our patent portfolio to commercialize their products, when outside our core commercial areas
- Mesoblast receives royalty income from its patent licensee TiGenix, S.A.U., a wholly owned subsidiary of Takeda, on its worldwide sales of its product Alofisel® for the treatment of complex perianal fistulas in adult patients with Crohn's disease, as well as milestone payments



Alofisel® is a registered trademark of TiGenix, S.A.U.

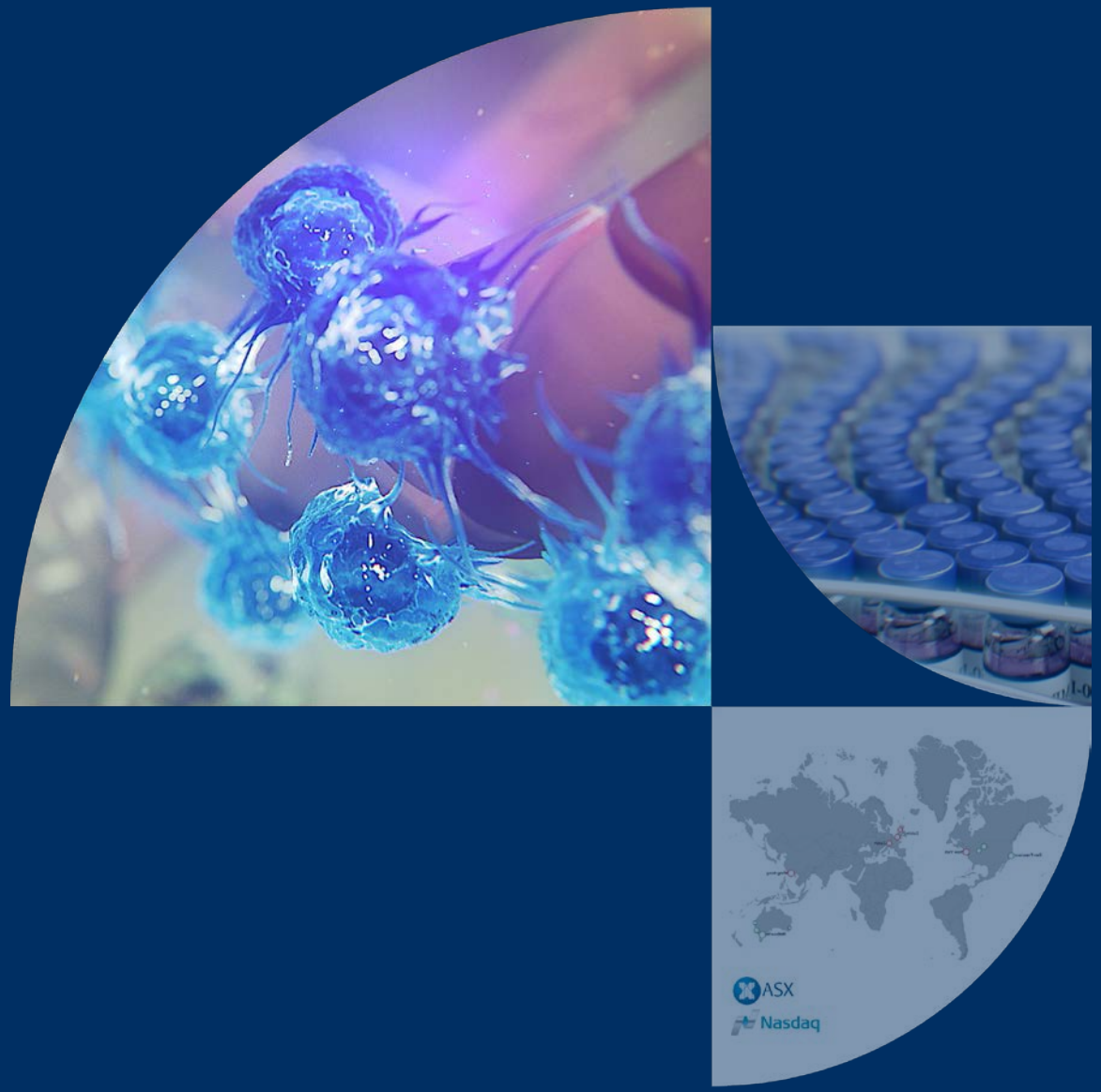
Commercial Scale Manufacturing Capability

- Scalable allogeneic “off-the-shelf” cellular platforms
- Manufacturing meets stringent criteria of international regulatory agencies
- Robust quality assurance processes ensure final product with batch-to-batch consistency and reproducibility
- Current capacity sufficient to meet near-term commercial projections

Lonza contract manufacturing facility in Singapore

- Proprietary xeno-free technologies will increase yields to meet long-term projected growth
- Next generation processes using 3D bioreactors to reduce labor and improve cost of goods

Financials



Financial Highlights

First Half FY2020 Compared to First Half FY2019

- 43% increase in total revenue to US$19.2m from US$13.5m
- 73% growth in commercialization revenue from sales of TEMCELL to US$3.8m from US$2.2m
- 36% increase in milestone revenues from strategic partnerships to US$15.0m from US$11.0m
- 32% (US$14.0m) reduction in loss after tax
- 22% (US$7.4m) decrease in R&D spend

Balance Sheet

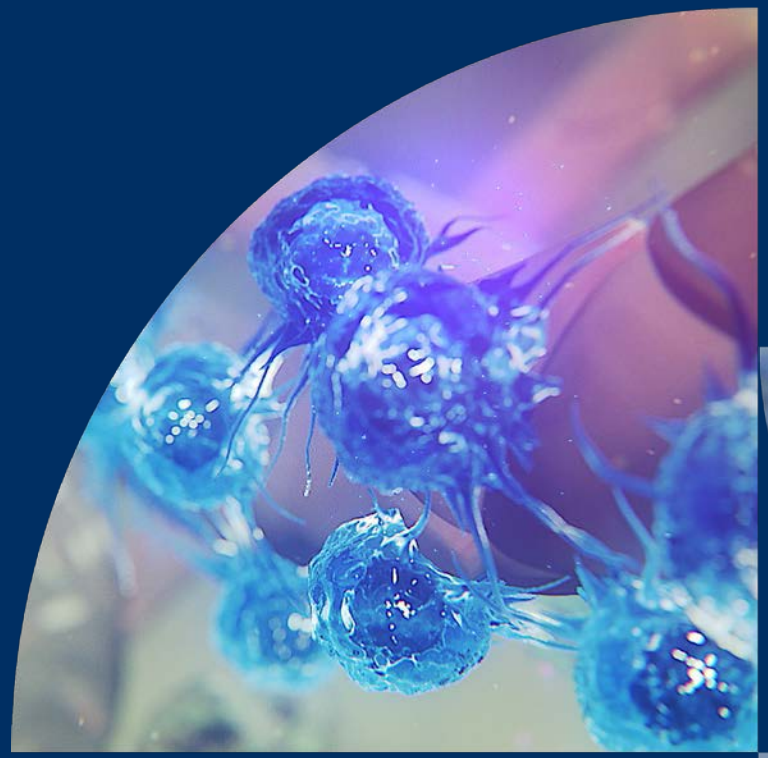
- Cash on hand US$81.3m as at December 31, 2019 compared to US$50.4m at June 30, 2019
    - Up to an additional US$62.5 million may be available through existing financing facilities and strategic partnerships over next 12 months

Figures are rounded

Substantial Increase in Revenues and Reduced Loss After Tax

Profit and Loss for the six months ending (US$m)	December 31, 2019	December 31, 2018
Commercialization revenue	3.8	2.2
Milestone revenue	15.0	11.0
Interest revenue	0.4	0.3
Total Revenue	**19.2**	**13.5**
Research and development	(26.6)	(34.0)
Manufacturing	(7.8)	(9.7)
Management & administration	(12.2)	(10.8)
Contingent consideration	(0.9)	(0.6)
Other operating income & expenses	0.4	(1.0)
Finance costs	(6.4)	(5.1)
Loss before tax	**(34.3)**	**(47.7)**
Income tax benefit	4.2	3.6
Loss after tax	**(30.1)**	**(44.1)**

Figures are rounded



Operational & Corporate Highlights

Acute Graft Versus Host Disease (aGVHD)

Significant market opportunity for RYONCIL

Burden of Illness

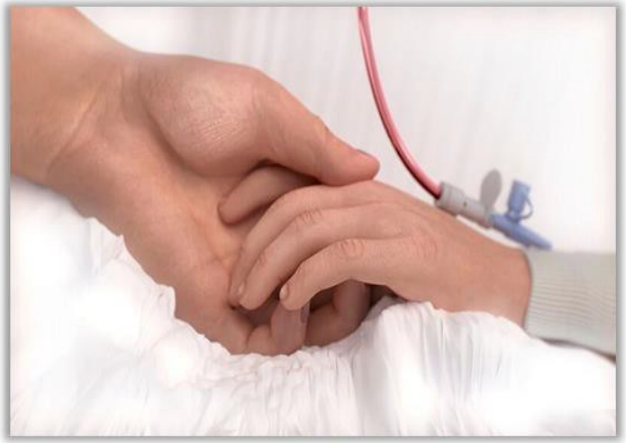
- aGVHD is a life-threatening complication that occurs in ~50% of patients receiving allogeneic bone marrow transplants (BMTs)[1]
- Steroid-refractory aGVHD is associated with **mortality rates as high as 90%[1,7] and significant extended hospital stay costs[2]**

Minimal Treatment Options

- There is only one approved treatment for SR-GVHD and **no approved treatment for children under 12 years old, outside Japan**
- In Japan, Mesoblast's licensee has received the only product approval for SR - aGVHD in both children and adults

Market Opportunity

- >30,000 allogeneic BMTs performed globally (>20K US/EU) annually, ~20% pediatric[3,4]
- Our licensee JCR Pharmaceuticals Co., Ltd launched TEMCELL® HS Inj.[5] in Japan for SR-aGVHD in 2016; reimbursed up to ~$USD195k[6]
- **SR-aGVHD represents $USD > 700m US/EU market opportunity[4,8]**

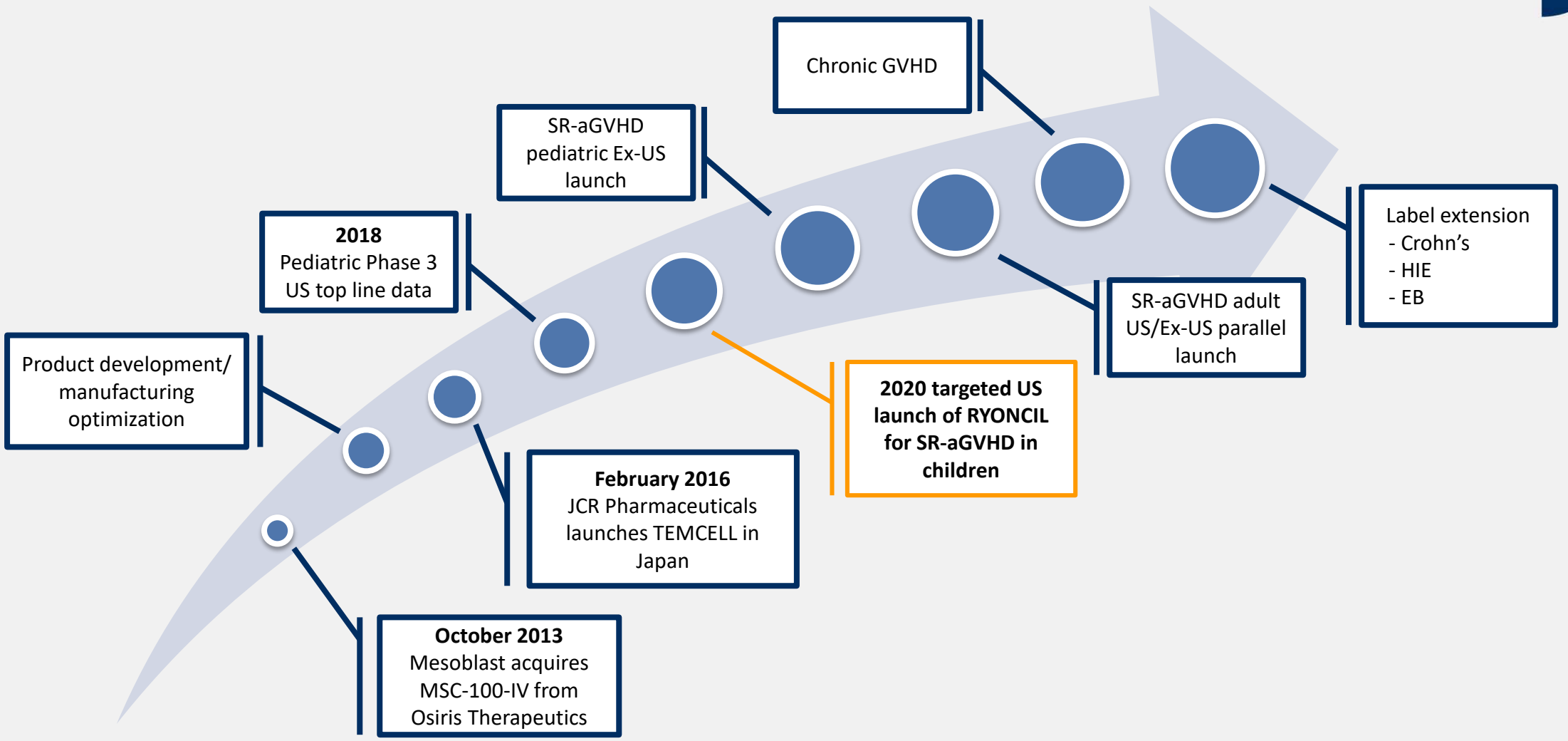


1. Westin, J., Saliba, RM., Lima, M. (2011) Steroid-refractory acute GVHD: predictors and outcomes. Advances in Hematology. **2**. Anthem-HealthCore/Mesoblast claims analysis (2016). Data on file **3.** Niederwieser D, Baldomero H, Szer J. (2016) Hematopoietic stem cell transplantation activity worldwide in 2012 and a SWOT analysis of the Worldwide Network for Blood and Marrow Transplantation Group including the global survey. **4.** Source: CIBMTR Current Uses and Outcomes of Hematopoietic Cell Transplantation 2017 Summary. Passweg JR, Baldomero, H (2016) Hematopoietic stem cell transplantation in Europe 2014: more than 40,000 transplants annually. **5.** TEMCELL is the registered trademark of JCR Pharmaceuticals Co. Ltd. **6.** Based on a ¥JPY = $USD 0.009375 spot exchange rate on market close on November 11, 2016. Amounts are rounded. Source: Bloomberg. 7. 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 transplantion. Bone Marrow Transplantation. 8.Data on file

RYONCIL: U.S. Regulatory and Commercial Strategy

- US strategy for RYONCIL informed by TEMCELL sales experience in Japan
- Fast Track designation provides eligibility for FDA priority review
- Commercialization strategy in place for product launch
- Ramp-up for inventory build
- Building out efficient, targeted sales force - 15 centers account for ~50% of patients
- Label extension planned for treatment of adult SR-aGVHD
- Lifecycle strategy

Remestemcel-L: Lifecycle Extension Strategy



RYONCIL: Recent Highlights and Key Milestones

Recent Highlights

- Filed BLA for RYONCIL with the US FDA for SR-aGVHD in children
- Consistent outcomes using RYONCIL as first line treatment or salvage therapy in 309 children with SR-aGVHD were presented at the annual meeting of the American Society for Transplantation Cellular Therapy & the Center for International Blood and Bone Marrow Transplant Research (TCT)
- Clinically meaningful outcomes using remestemcel-L in patients with chronic GVHD were reported in an investigator-initiated expanded access protocol
- Mesoblast entered into an agreement with Lonza for commercial product manufacture in line with the corporate strategy to facilitate appropriate inventory build ahead of the US planned launch of RYONCIL

Key Milestones

- Market will be updated on status of RYONCIL Priority Review and PDUFA date
- If approved, the US launch of RYONCIL is planned for 2020

Chronic Heart Failure: Rising Incidence & High Mortality Rates Highlight Large Clinical Unmet Need

Burden of Illness

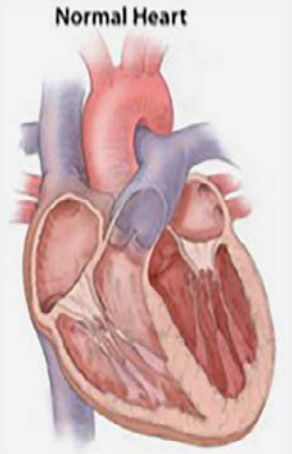
- More than 26 million people worldwide living with heart failure[1]
- 8.5 million patients in the US alone, expected to be living with heart failure by 2030[1]
- 17-45% of patients die within 1 year of hospital admission[1]

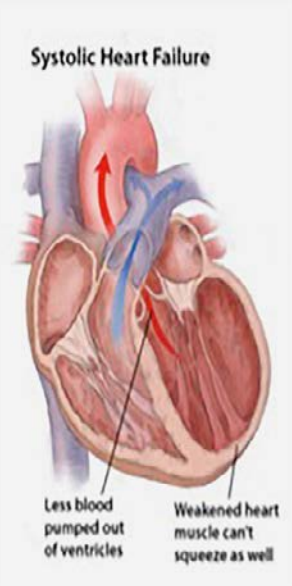
Limited Options / Unmet Need

- Advanced / End Stage heart failure defined as NYHA class III / IV and comprise ~30% of all heart failure patients[2,3]
- Despite new therapies for early-stage disease, there has been very little improvement in survival for patients with advanced heart failure[2,3]
- Majority of advanced heart failure patients die within 5 years[1]

Market Opportunity

- In the US, more hospital days are spent on the care of patients with heart failure than any other diagnosis[2,3]
- **Advanced heart failure has the highest hospital readmission rate of any diagnosis-related group, indicative of the limited treatment options when patients reach this stage[2,3]**
- **Large clinical unmet need with multi-billion dollar annual market opportunity in US[4,5]**

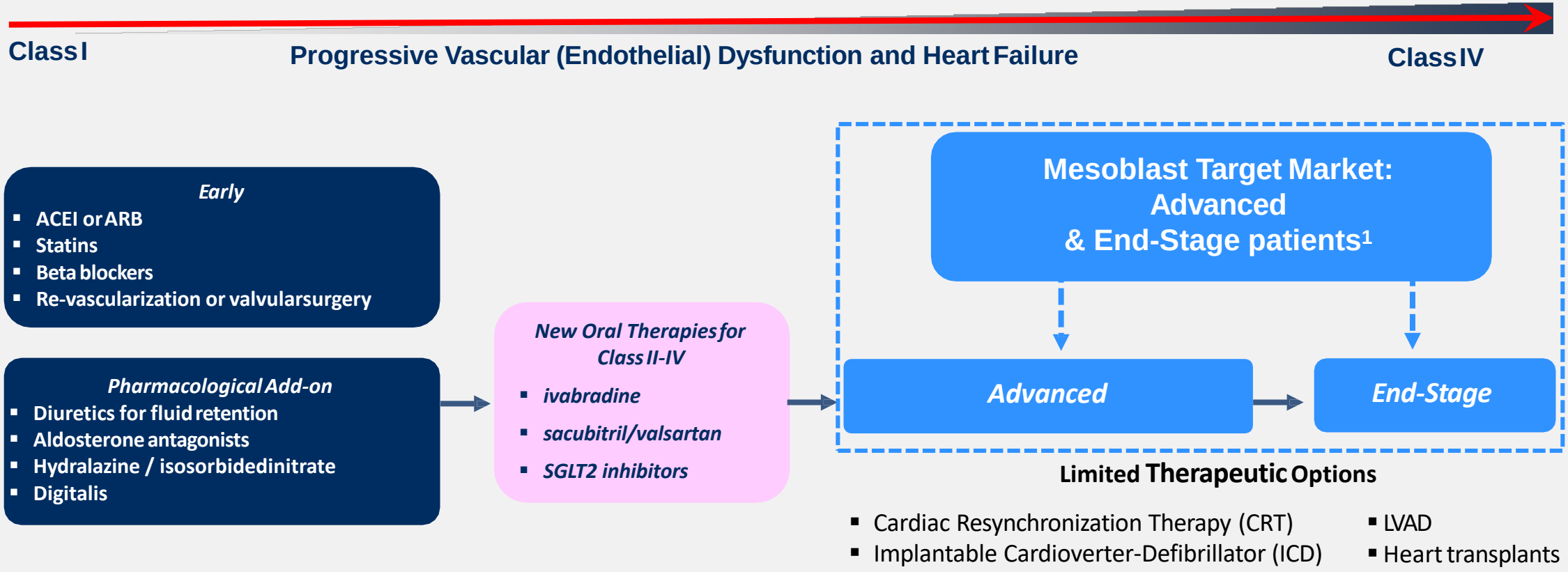




1. Heart Failure: Preventing disease and death worldwide – European Society of Cardiology 2014. 2. Leslie W.MillerMD, Maya GuglinMD, PhD. Patient Selection for Ventricular Assist Devices: A Moving Target. Journal of the American College of Cardiology. Volume 61, Issue 12, 26 March 2013, Pages 1209-1221, 3. Leslie W. Miller. Left Ventricular Assist Devices Are Underutilized. Circulation. 2011;123:1552–1558. 4.A Reevaluation of the Costs of Heart Failure and its Implications for Allocation of Health Resources in the United States. Voigt J. Clinl.Cardiol. 37, 5, 312- 321 (2014)., 5.The Medical and Socioeconomic Burden of Heart Failure: A Comparative Delineation with Cancer. Dimitrios, F. International Journal of Cardiology (2015), doi: 10.1016/j.ijard.2015.10.172.

REVASCOR for Advanced and End-Stage Heart Failure

Common Treatment Pathway in Progressive Heart Failure



1. GlobalData-PharmaPoint Heart Failure (2016); McMurray et al., 2012;Yancy et al., 2013, 2016 ACC/AHAHFSA Focused Update on New Pharmacological Therapy for Heart Failure: An Update of the 2013 ACCF/AHA Guideline for the Management of Heart Failure.

REVASCOR: Phase 3 trials for Advanced & End-Stage Heart Failure

Advanced Heart Failure

- Trial design: 1:1 randomized, controlled, double blinded; conducted over 55 sites across North America using 150 million cell dose vs control in 566 patients
- Target patient population enriched for LVESV>100ml, at highest risk for events
- Primary endpoint: reduction in recurrent heart failure-related major adverse cardiac events (HF-MACE)
- Secondary endpoint: reduction in terminal cardiac events
- Successful pre-specified interim futility analysis of the primary efficacy endpoint in the first 270 patients

End-Stage Heart Failure

- Mesoblast and the International Center for Health Outcomes Innovation Research (InCHOIR) at the Icahn School of Medicine at Mount Sinai in New York have agreed on a confirmatory Phase 3 trial of REVASCOR in patients with end-stage heart failure and a LVAD, in line with FDA guidance
- REVASCOR is being developed for these patients under existing FDA Regenerative Medicine Advanced Therapy (RMAT) and Orphan Drug designations

REVASCOR for Advanced and End-Stage Heart Failure

Recent Highlights

- In December 2019, the Phase 3 trial in advanced heart failure surpassed the number of primary endpoint events required for trial completion
- The independent DMC held its 10th and final scheduled meeting and recommended that the trial continue as planned. The DMC reviewed components of the trial's primary and secondary endpoints, and all safety data
- Final study visits have been initiated for all surviving patients in the Phase 3 trial in advanced heart failure

Key Milestones

- Data readout for Phase 3 trial in advanced chronic heart failure planned for mid-2020
  - ➢ Results may support regulatory approval in the US
- Mesoblast and InCHOIR will initiate a confirmatory Phase 3 trial of Revascor in end-stage heart failure patients with an LVAD

Disease Modifying Therapies Needed for Chronic Low Back Pain Due to Degenerative Disc Disease

Burden of Illness

- Back pain causes more disability than any other condition[1]
- Inflicts substantial direct and indirect costs on the healthcare system[1,2], including excessive use of opioids in this patient population

Minimal Treatment Options

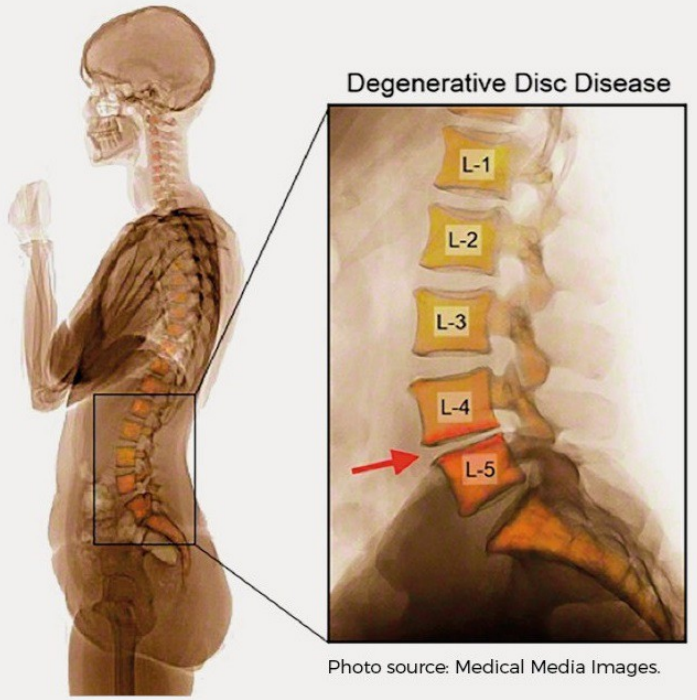
- Minimal treatment options for patients with chronic low back pain (CLBP) who fail conservative therapy include opioids and surgery
- 50% of opioid prescriptions are for CLBP

Unmet Need

- Disease modifying therapy for durable improvement in pain and function has potential to prevent progression to opioid use or surgical intervention

Market Opportunity

- Over 7m patients are estimated to suffer from CLBP due to degenerative disc disease (DDD) in each of the U.S. and E.U.5 [3-6]
- **MPC-06-ID development program targets over 3.2m patients in U.S. and 4m in E.U.5 with moderate to severe disease**



1. Williams, J., NG, Nawi, Pelzter, K. (2015) Risk factors and disability associated with low back pain in older adults in low-and middle-income countries. Results from the WHO Study on global ageing and adult health (SAGE). PloS One. 2015; 10(6): e0127880., 2. Simon, J., McAuliffe, M., Shamim, F. (2015) Discogenic Low Back Pain. Phys Med Rehabil Clin N Am 25 (2014)305–317., 3.Decision Resources: Chronic Pain December 2015., 4. LEK & NCI opinion leader interviews, and secondary analysis., 5. Navigant: Commercial Assessment for a Proprietary Cell-Based Therapy for DDD in the U.S. and the EU3 – August 2014., 6. HealthCare Utilization and Cost of Discogenic Lower Back Pain in the US – Anthem/HealthCore.

MPC-06-ID: Development Strategy for US & Europe

- Phase 3 trial in chronic low back pain completed enrolment in March 2018 with 404 patients randomized to receive MPC-06-ID or placebo
- Initiate confirmatory Phase 3 trial in Europe in partnership with Grünenthal
- Complete commercial manufacturing in partnership with Grünenthal
- Results of confirmatory Phase 3 clinical trials in US and Europe, together with commercial manufacturing, expected to support regulatory approval and commercial launches in both Europe and US for MPC-06-ID in chronic low back pain due to degenerative disc disease

MPC-06-ID for Chronic Low Back Pain

Recent Highlights

- Entered into strategic partnership with Grünenthal to develop and commercialize MPC-06-ID in Europe and Latin America

Key Milestones

- Last patient last visit at 24-months of follow up in the Phase 3 trial of MPC-06-ID for chronic low back pain H1 CY2020, with the primary endpoint being a composite outcome of pain and function at 12 and 24 months
- Obtain clearance from European regulatory authorities to begin European Phase 3 trial
- Results from the Phase 3 trials will be considered pivotal to support regulatory approval in the US, as well as Europe through the Grünenthal partnership

Major Operational Milestones for the Next 12 Months

Remestemcel-L for SR-aGVHD and Other Rare Diseases

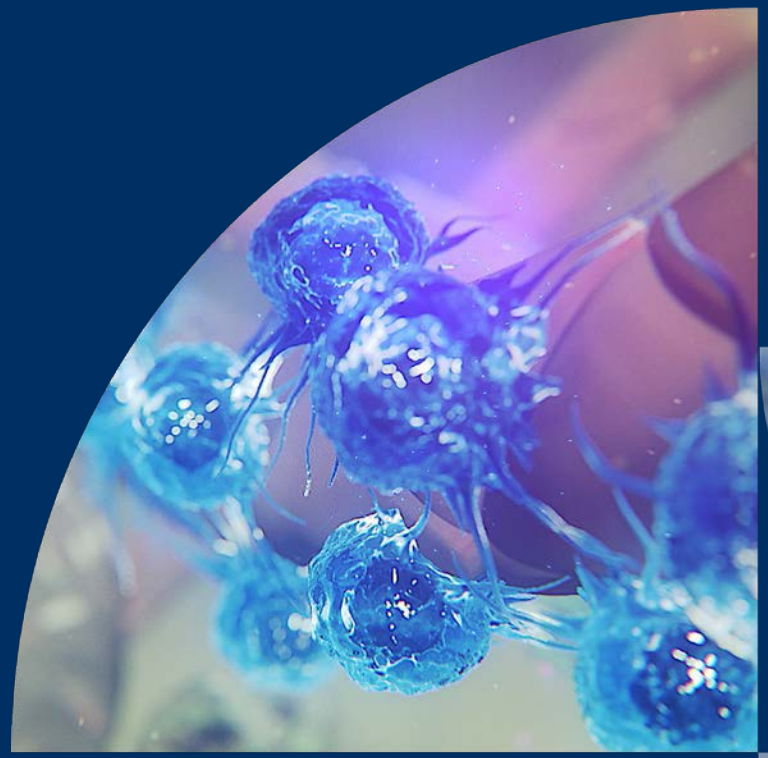
- Updates on RYONCIL Priority Review and Prescription Drug User Fee Act (PDUFA) date
- If approved, US launch of RYONCIL planned for 2020
- Expand investigator-initiated clinical trials for chronic GVHD and other indications

REVASCOR for Advanced and End-Stage Heart Failure

- Data readout for advanced chronic heart failure Phase 3 trial in mid-2020
- Initiate confirmatory trial in end-stage heart failure

MPC-06-ID for Chronic Low Back Pain

- Data readout for Phase 3 trial planned for mid-2020
- Obtain clearance from European regulatory authorities to begin European Phase 3 trial



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Thank You