mesoblast
the regenerative medicine company

Mesoblast Phase 3 Cell Therapy Trial for Acute Graft Versus Host Disease (aGVHD) Successfully Achieves Primary Endpoint

Presented at BMT/Tandem, February 21, 2018

22/23 February 2018

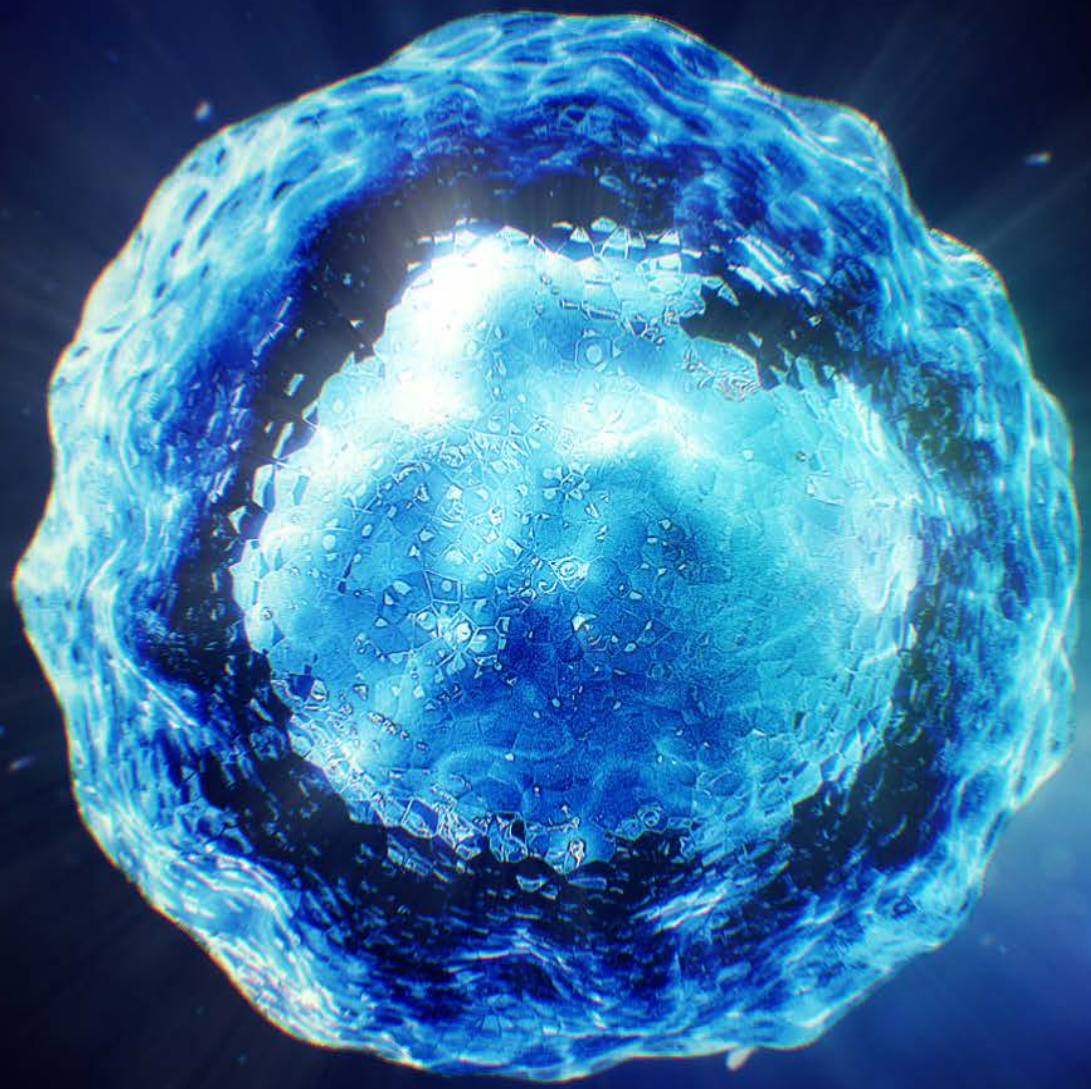
Nasdaq: MESO ASX: MSB

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 bring to market disruptive cellular medicines to treat serious and life-threatening illnesses



Investment Proposition:

Building a Leading Franchise of Cellular Medicines

- Disruptive Cellular Technology Platform
- Commercial Translation Capabilities
- Advanced Pipeline of Cellular Medicines
- Targeting Serious or Life-Threatening Conditions with Unmet Needs

Disruptive Cellular Medicine Platform[1-4]

- Mesenchymal Lineage Cells (MLCs) have unique receptors that respond to activating inflammatory and damaged-tissue signals
- In response to these signals, MLCs secrete a diverse variety of biomolecules responsible for immunomodulation and tissue repair
- The multi-modal mechanisms of action target multiple pathways
- STRO-1$^+$ Mesenchymal Precursor Cells (MPCs) are at the apex of the MLC hierarchy and their immuno-selection provides a homogeneous population of potent cells

1. Simmons PJ and Torok-Storb, B. Identification of stromal cell precursors in bone marrow by a novel monocloncal antibody, STRO-1. Blood. 1991;78:55-62.
2. Gronthos S, Zannettino AC, Hay SJ, et al. Molecular and cellular characterisation of highly purified stromal stem cells derived from human bone marrow. J Cell Sci. 2003;116(Pt 9):1827-35.
3. See F, Seki T, Psaltis PJ, et al.Therapeutic effects of human STRO-3-selected mesenchymal precursor cells and their soluble factors in experimental myocardial ischemia. J Cell Mol Med. 2011;15:2117-29.
4. Psaltis PJ, Paton S, See F, et al. Enrichment for STRO-1 expression enhances the cardiovascular paracrine activity of human bone marrow-derived mesenchymal cell populations. J Cell Physiol. 2010;223(2):530-40.

Commercial Translation Capabilities:

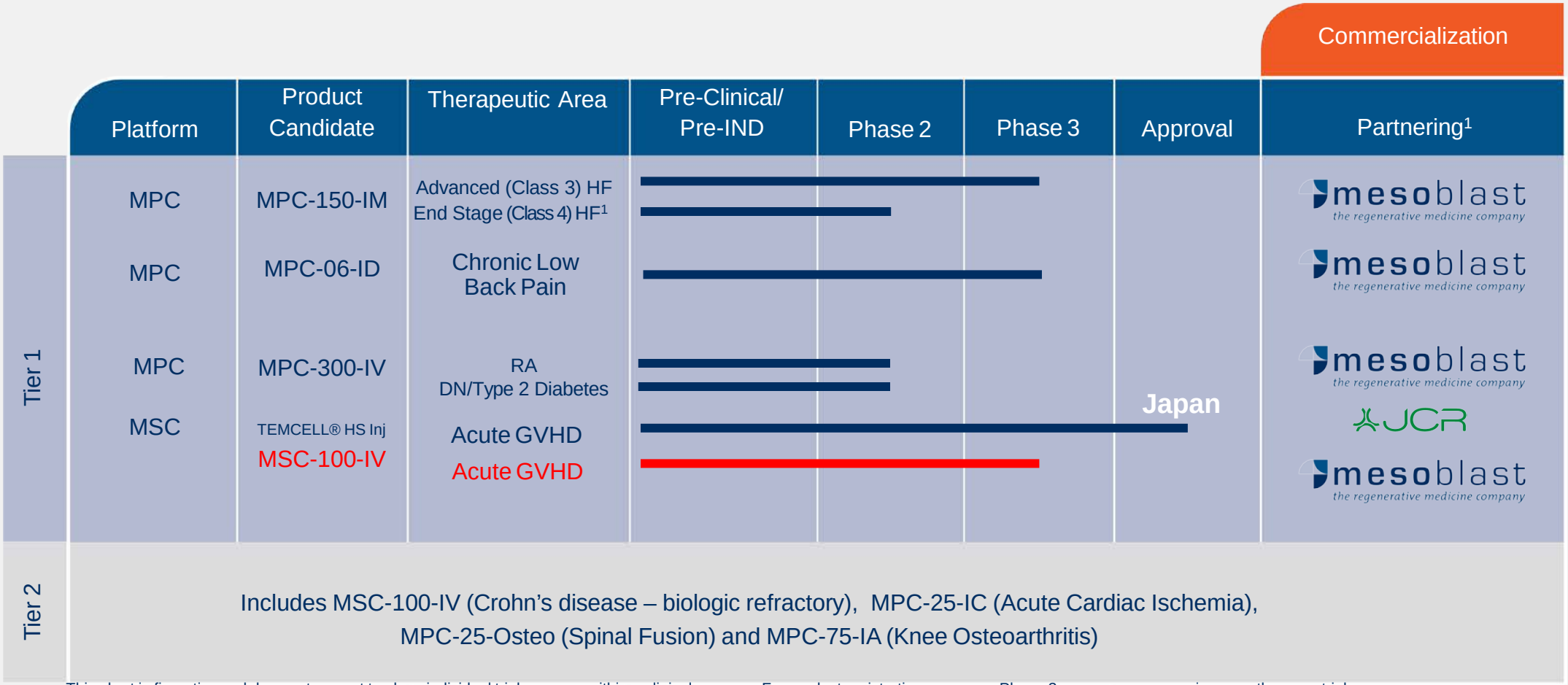
Technology Positioned for Scalable, Industrialized Manufacturing

- Immune privileged nature of MLCs enables allogeneic “off the shelf” product candidates
- Culture expansion scalable to produce commercial quantities of potent and reproducible therapeutic doses
- Specific formulations defined for product delineation
- Management know how in regulatory activities necessary for product approval and commercial launch
- TEMCELL® HS. Inj., first allogeneic cellular medicine received full approval in Japan and successfully launched for acute Graft vs Host Disease[1]
- MSC-100-IV (remestemcel-L) positioned to be first allogeneic MLC product launched in the USA

Lonza contract manufacturing facility in Singapore

[1] TEMCELL® HS. Inj. Is the registered trademark of JCR Pharmaceuticals Co. Ltd., Mesoblast’s Licensee.

Portfolio of Advanced Product Candidates:
Three Tier 1 Product Candidates in Phase 3



This chart is figurative and does not purport to show individual trial progress within a clinical program. For product registration purposes, Phase 3 programs may require more than one trial.
Tier 1 programs represent our lead programs where we focus the majority of our time and resources. Tier 2 programs are also in development and may advance to Tier 1 depending on the merit of newly generated data, market opportunity or partnering options.

1. Clinical trial is funded by the U.S. National Institutes of Health and the Canadian Health Research Institute.

Acute Graft vs Host Disease

Remestemcel-L (MSC-100-IV) for Steroid-Refractory aGVHD

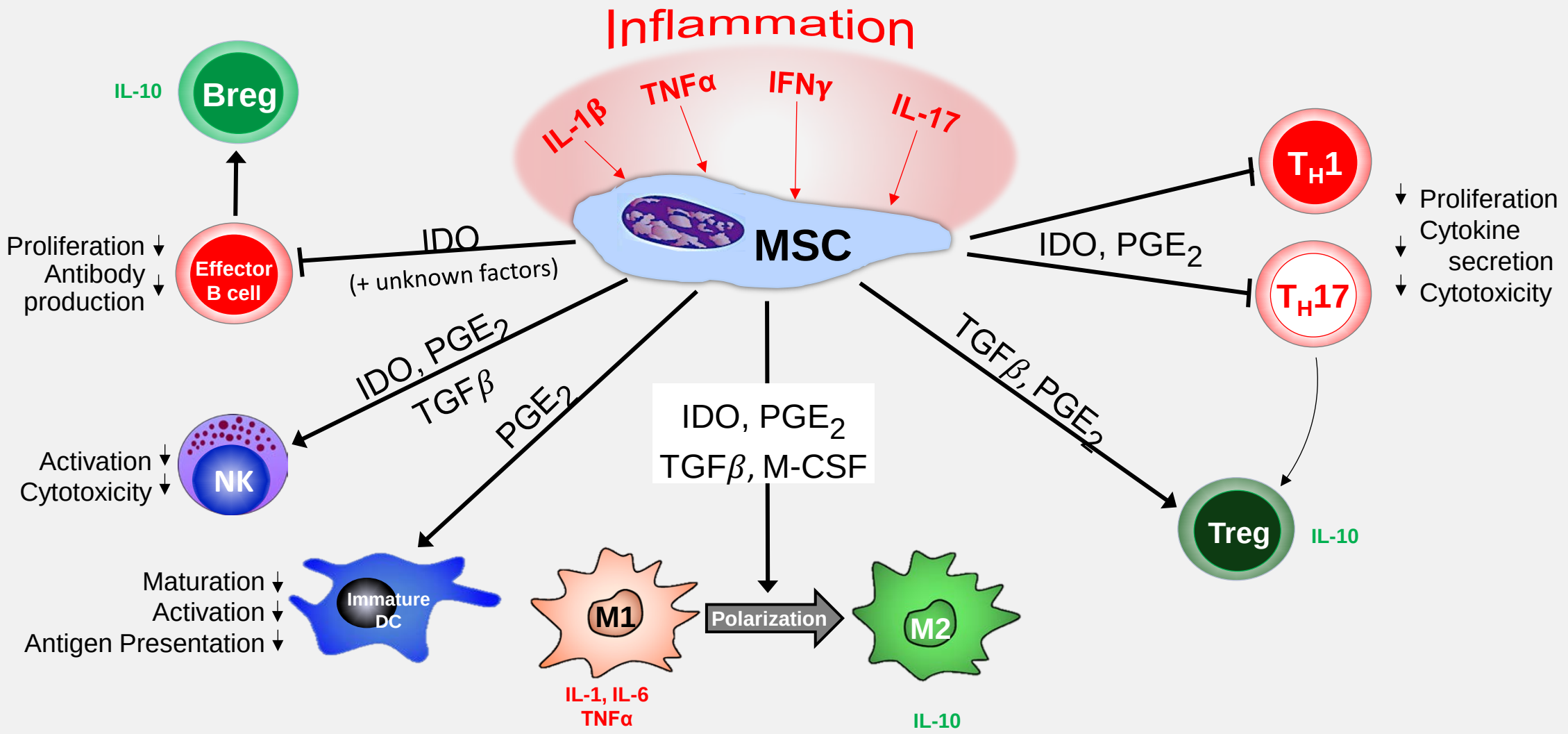
Acute graft-versus-host disease (aGVHD) Background

- Acute graft-versus-host disease (aGVHD) is associated with significant morbidity and is a leading cause of mortality after allogeneic hematopoietic stem cell transplantation
- Although the incidence of aGVHD varies across transplant type and regimen, severe aGVHD (determined by grade C/D, visceral organ and multi-organ involvement, or high risk stratification) has the highest risk of primary treatment failure and high transplant related mortality[1]
- Day 100 mortality can reach 70% in patients who fail to respond to initial steroid therapy[2-4], and 12 month mortality approaches 90%[5]
- Mesenchymal stem cells have anti-inflammatory and immunomodulatory biological activity that supports their investigational use in aGVHD[6]

1. Jaqasia M, Arora M, Flowers ME, et al. Risk factors for acute GVHD and survival after hematopoietic cell transplantation. Blood. 2012; 119 (1): 296-307.
2. MacMillan ML, DeFor TE, Weisdorf DJ. The best endpoint for acute GVHD treatment trials. Blood. 2010; 115 (26): 5412-5417.
3. MacMillan ML, Couriel D, Weisdorf DJ, et al. A phase 2/3 multicenter randomized clinical trial of ABX-CBL versus ATG as secondary therapy for steroid-resistant acute graft-versus-host disease. Blood. 2007; 109 (6): 2657-2662.
4. Pidala J, Kim J, Field T, et al. Infliximab for managing steroid-refractory acute graft-versus-host disease. Biol Blood Marrow Transplant. 2009; 15 (9): 1116-1121.
5. Arai S et al, Poor outcome in steroid refractory graft verses host disease with anti-thymocyte globulin treatment. Biol Blood Marrow Transplant. 2002; 8: 155-160.
6. Aggarwal S, Pittenger MF. Human mesenchymal stem cells modulate allogeneic immune responses. Blood. 2005; 105:1815-22.

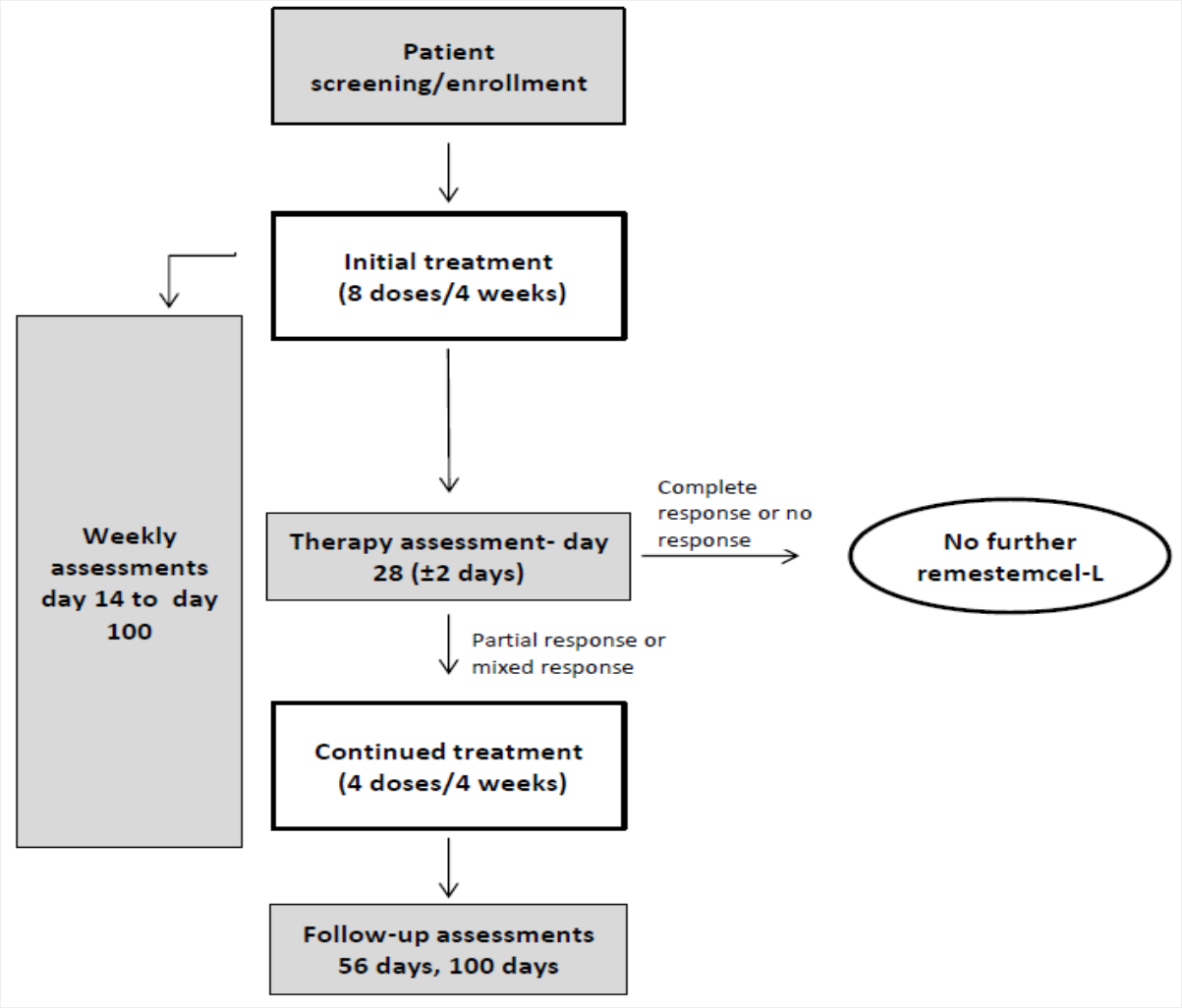
The Immunomodulatory Actions of MSCs are Triggered by their Response to Inflammatory cues:

Paracrine-mediated alteration of the function of multiple cellular constituents of the innate and adaptive immune systems



Remestemcel-L (MSC-100-IV): Phase 3 Pediatric Trial GVHD001 Completed Enrollment as First-line Therapy in aGVHD After Failing Steroids

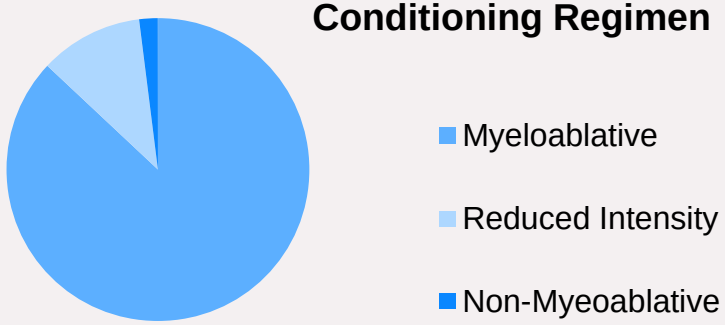
- Multi-center, Single-Arm, Open-Label to evaluate efficacy and safety to day 100 (GVHD001) and from day 100 to day 180 (GVHD002)
- 55 pediatric patients (2 months to 17 years)
- aGVHD following allogeneic HSCT failing systemic corticosteroid therapy
- Grade B aGVHD involving liver and/or GI tract with or without concomitant skin disease
- Grades C and D aGVHD involving skin, liver and/or GI tract
- Primary endpoint: **Overall response at Day 28**
- Key secondary endpoint: Survival at Day 100
- Interim futility analysis of primary endpoint successful Nov 2016



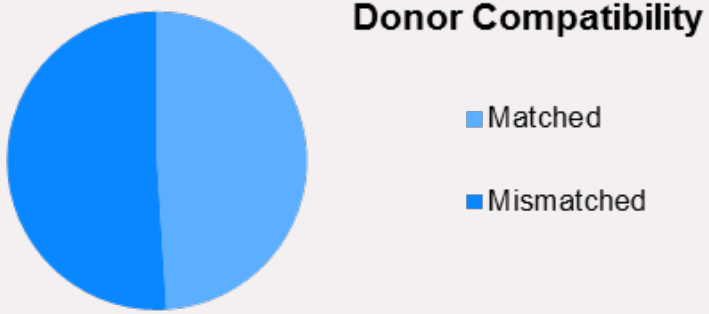
Protocol GVHD001: Demographics

Subjects Enrolled	55
Age (years)	
Mean (SD)	7.8 (5.44)
Median (minimum, maximum)	7.6 (0.6, 17.9)
Gender	
Male	35 (63.6%)
Female	20 (36.4%)
Underlying Disease	
AML	18 (32.7%)
ALL	12 (21.8%)
Anemia	5 (9.1%)
CML	4 (7.3%)
Sickle Cell	3 (5.5%)
JML	2 (3.6%)
MDS	2 (3.6%)
Other	9 (16.4%)

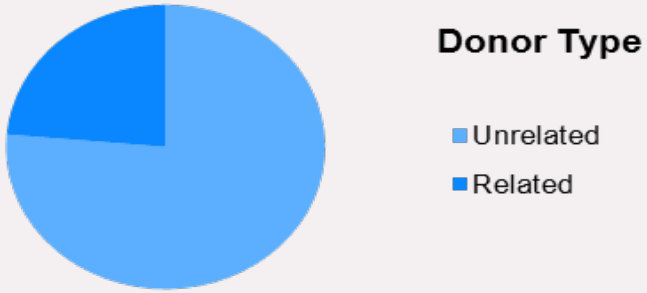
Protocol GVHD001: Transplant Characteristics reflect aGVHD risk factors



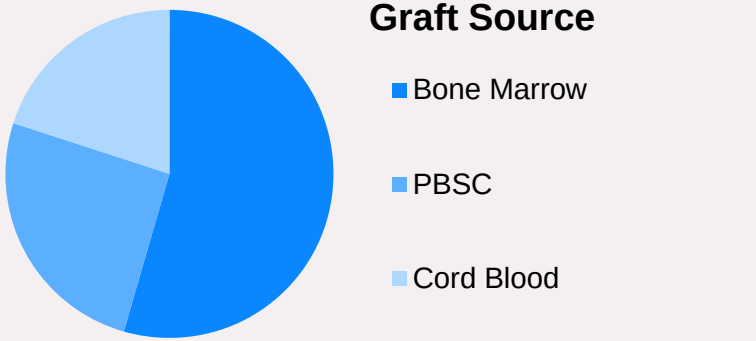
- 87% of subjects received myeloablative conditioning regimen



- 51% of subjects received an HLA-mismatched transplant



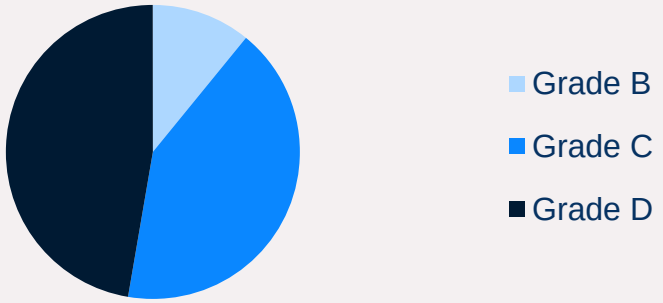
- 76% of subjects received an unrelated donor transplant



- 55% of subjects received a bone marrow transplant, 25% received PBSC, and 20% received CB

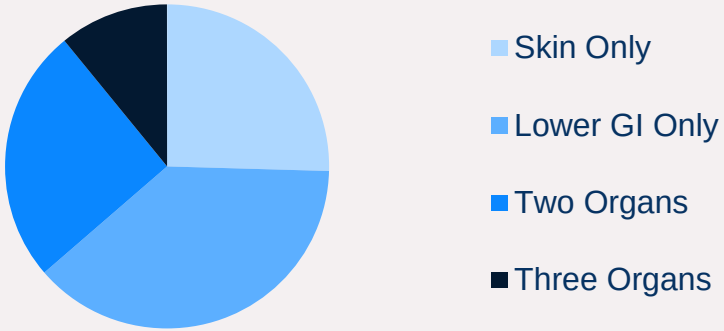
Protocol GVHD001: Disease Characteristics reflect aGVHD severity

GVHD Grade at Baseline



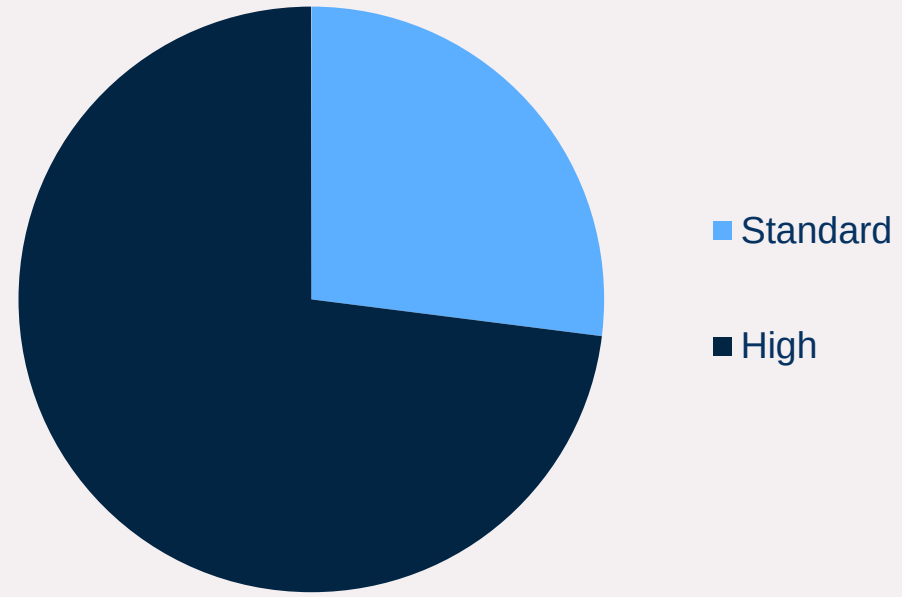
- 47% of subjects had Grade D disease at baseline
- 89% of subjects had Grade C/D disease at baseline

Baseline Organ Involvement



- 26% of subjects had Skin involvement only
  - all had stage 3 (n=10) or stage 4 (n=4) disease
- 38% of subjects had Lower GI involvement only
  - 16/21 had stage 3 (n=6) or stage 4 (n=10) disease
- 36% of subjects had multi-organ involvement, all with Lower GI
  - 6/20 had all three organs involved
  - 10/20 had Lower GI + Skin
  - 4/20 had Lower GI + Liver

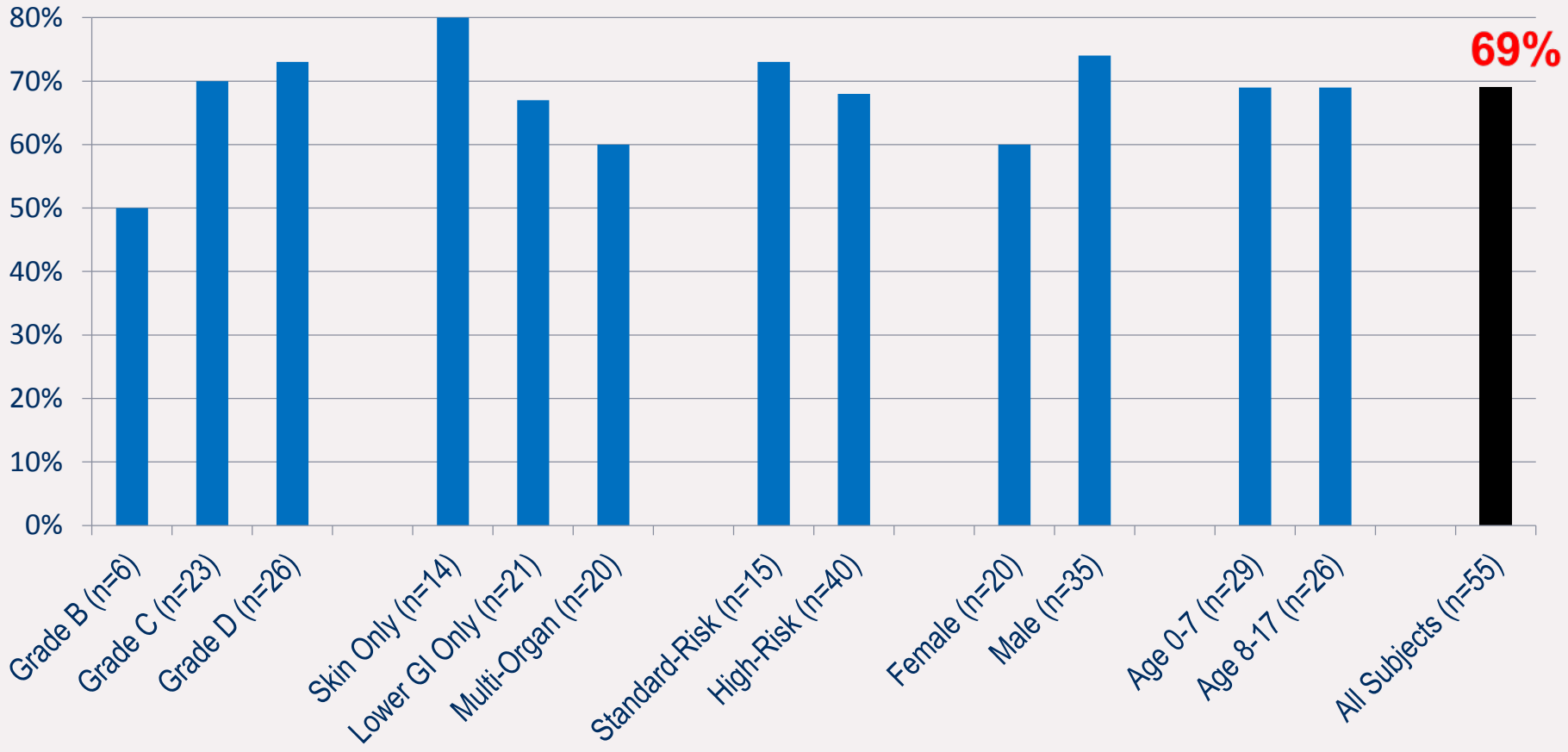
Protocol GVHD001: Disease Characteristics (risk)



- 73% of subjects had high-risk disease at baseline[10]
  - stage 3 (n=6) or stage 4 (n=10) Lower GI only
  - stage 4 Skin only (n=4)
  - any stage Lower GI + any stage Liver (n=4)
  - stage 2-4 Lower GI + any stage Skin (n=10)
  - all disease involving three organs (n=6)
- Standard risk disease was characterized by:
  - stage 3 (n=10) Skin only
  - Stage 1 (n=1) or stage 2 (n=4) Lower GI only

Protocol GVHD001: Primary Efficacy Outcome

Overall Response at Day 28 was 69%, p=0.0003



- 69% Overall Response rate at Day 28 (29% CR + 40% PR)
- p-value calculated from the binomial distribution, under the assumption of a 0.45 success rate under the null hypothesis

Protocol GVHD001: Results of Safety and Mortality

- Remestemcel-L (MSC-100-IV) infusions were well tolerated
- The incidence of adverse events in the trial was consistent with that expected from the underlying disease state and in line with previous use of Remestemcel-L (MSC-100-IV)
- Eleven subjects have died during the study (22% mortality through Day 100)
  - None of the deaths was reported to be related to remestemcel-L (MSC-100-IV) by the investigators
  - The underlying causes of death included HSCT-related causes in 9 subjects (8 due to infections and 1 due to GHVD progression), and primary cancer relapse in 2 subjects
- Four subjects have terminated participation in the study early (prior to Day 100)
  - 1 subject was not able to be dosed; 1 subject had a non-fatal AE (somnolence); 1 subject had parental consent withdrawn; and 1 subject was withdrawn by PI

Protocol GVHD001: Summary and Conclusions

- This Phase 3 study evaluated allogeneic mesenchymal stem cells (MSCs), Remestemcel-L (MSC-100-IV), for the treatment of steroid-refractory acute graft-versus-host disease intended to improve overall response rate in pediatric subjects

- Study successfully met the primary endpoint of improved Day 28 Overall Response in steroid-refractory pediatric subjects with severe disease
  - Day 28 OR was 69%
  - Day 28 OR was significantly improved (p=0.0003) compared to protocol-defined historical control rate of 45%

- Remestemcel-L (MSC-100-IV) was safe and the infusions were well tolerated. The incidence of adverse events in the trial was consistent with that expected from the underlying disease state and in line with previous use of Remestemcel-L (MSC-100-IV)[1]

- Among patients who received at least one treatment infusion and were followed up for 100 days (n=50), the mortality rate was 22%, an encouraging indicator of potential longer term benefit

- These findings are consistent with the overall response, safety, and survival in the previous report of remestemcel-L (MSC-100-IV) in a 241 subject expanded access protocol of pediatric subjects with SR-aGVHD who failed to respond to steroids as well as to multiple additional treatments[2]

1. Data on file from Protocol 280 Clinical Study Reports.
2. Kurtzberg J. et al. Effect of Human Mesenchymal Stem Cells (Remestemcel-L) on Clinical Response and Survival Confirmed in a Large Cohort of Pediatric Patients with Severe High-Risk Steroid-Refractory Acute Graft Versus Host Disease. BBMT. 2016; 22.

Protocol GVHD001: Authors

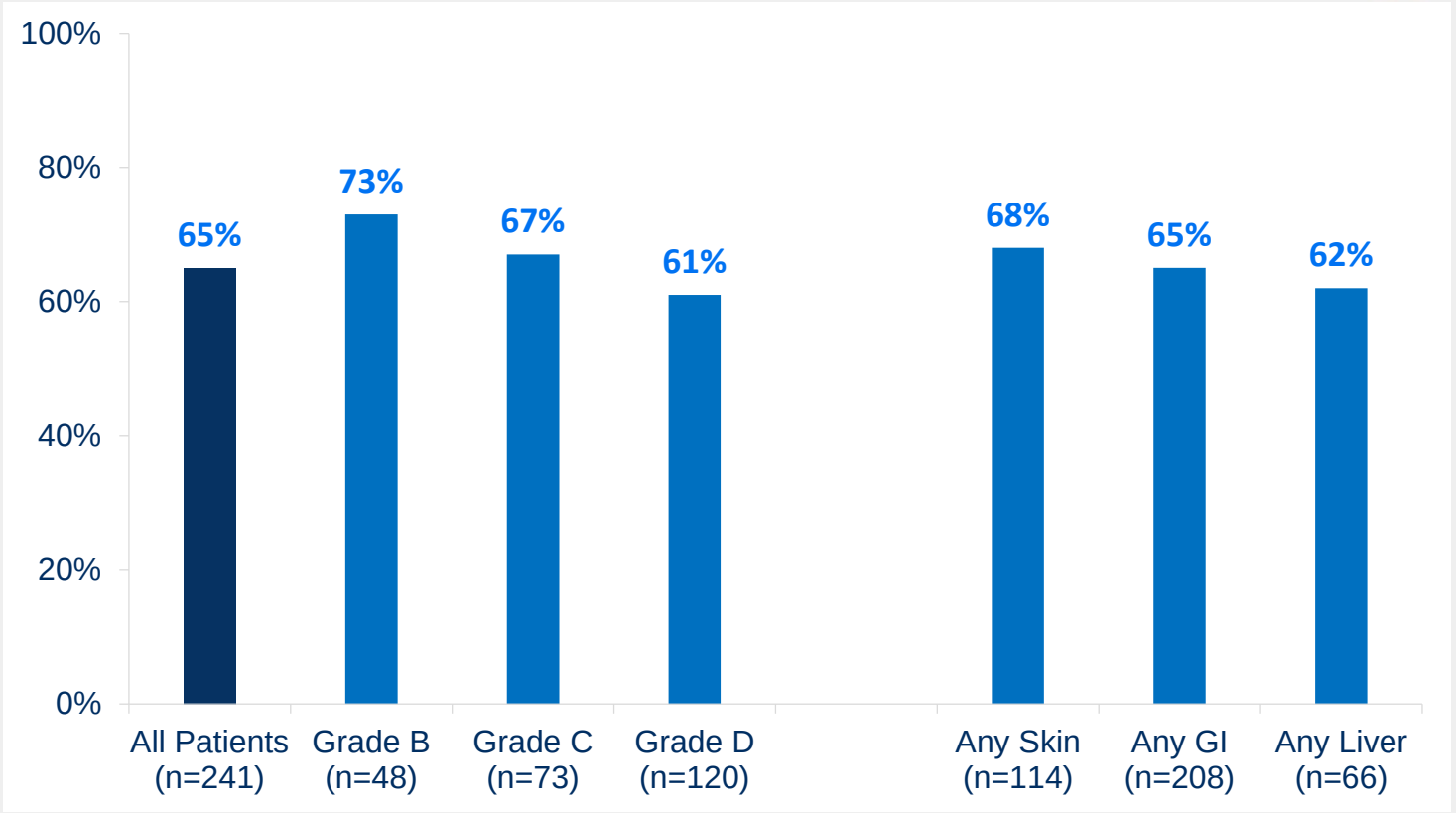
- Chaudhury S; Lurie Children's Hospital of Chicago, Chicago, IL
- Nemecek E; Oregon Health and Science University, Portland, OR
- Mahadeo K; MD Anderson Cancer Center, Houston, TX
- Prockop S; Memorial Sloan Kettering Cancer Center, New York, NY
- Horn B; University of Florida Health, Gainesville, FL
- Neudorf S; Children's Hospital of Orange County, Orange, CA
- Quigg T; Texas Transplant Institute, San Antonio, TX
- Carpenter P; Fred Hutchinson Cancer Center, Seattle, WA
- Hayes J, and Skerrett D; Mesoblast, Inc., New York, NY
- Kurtzberg J; Duke University Medical Center, Durham, NC

Remestemcel-L (MSC-100-IV): Expanded Access Program

Overall Day 28 Response in Pediatric aGVHD Patients Receiving Remestemcel-L (MSC-100-IV) as First-line or Salvage Therapy After Failing Steroids

Population: steroid-refractory aGVHD pediatric patients

- 241 pediatric patients undergoing HSCT were enrolled and treated at 50 sites in North America and Europe from 2007-2014
- Ages 2 months – 17 years
- Acute GvHD grades B-D (CIBMTR)
- Failed steroid treatment and multiple other agents
- aGVHD not improving after at least 3 days of methylprednisolone (at least 1 mg/kg/day or equivalent)



- Complete Response was 14%, Partial Response was 51%
- Responses were observed for all GVHD grades and did not differ by baseline organ involvement

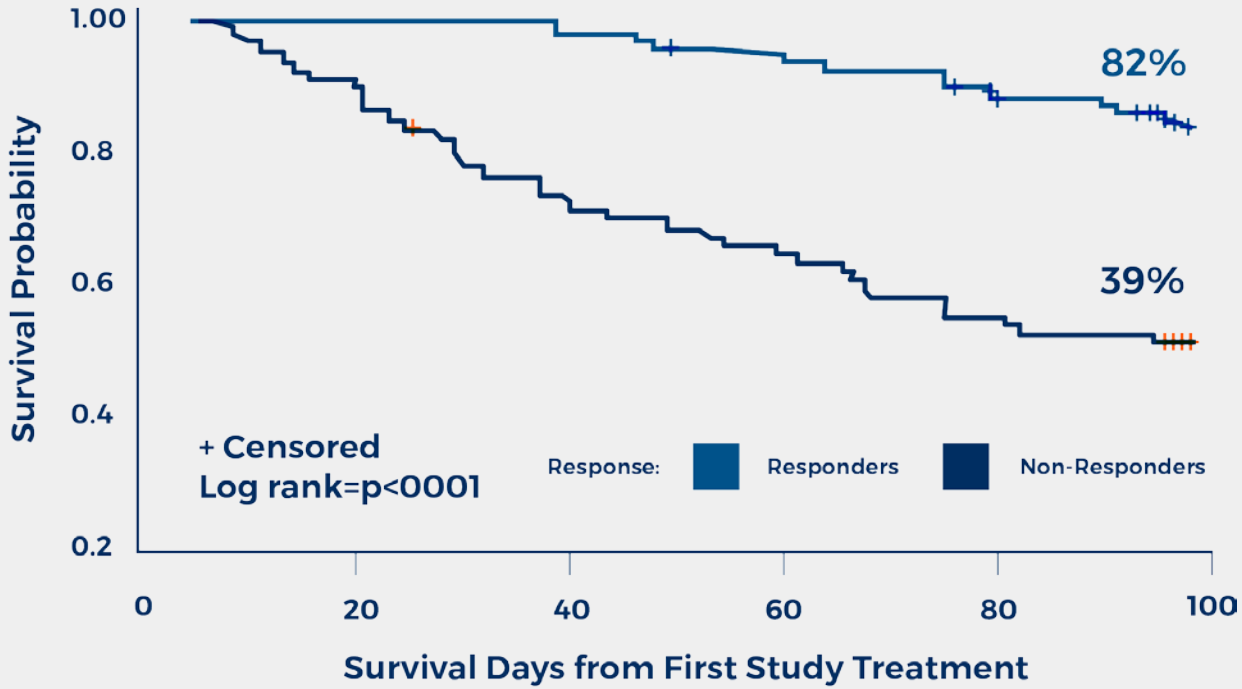
Kurtzberg et al: Presentation Tandem Feb 2016

Remestemcel-L (MSC-100-IV): Expanded Access Program

Correlation of Day 28 Overall Response with Day 100 Survival, Using Remestemcel-L (MSC-100-IV) as First-line or Salvage Therapy After Failing Steroids and/or Additional Treatments

Remestemcel-L (MSC-100-IV) in Children with SR-aGVHD who failed multiple other modalities

- Survival of Pediatric Patients Treated with MSC-100-IV 28-Day Responders vs Non-responders n=241



- In 241 Children under EAP, **Overall Response** (CR+PR) at Day 28 was **65%** (95% CI: 58.9%, 70.9%)
- **Day 100 survival** correlated with overall response, and was significantly improved in those who responded at Day 28 **(82% vs. 39%, p<0.0001)**

Kurtzberg et al: Presentation Tandem Feb 2016


Dublin
Zurich
New York
San Francisco
Hong Kong
Melbourne
ASX
Nasdaq

Remestemcel-L (MSC-100-IV): Market Opportunity for aGVHD

Burden of Illness

- Steroid-refractory aGVHD patients have mortality rates as high as 95%[1]
- Refractory aGVHD is associated with significant extended hospital stay costs[2]
- aGVHD - a severe immunological reaction occurring in BMT patients
- Is a major limitation in successful allogeneic hematopoietic stem cell transplants[1]

Minimal Treatment Options

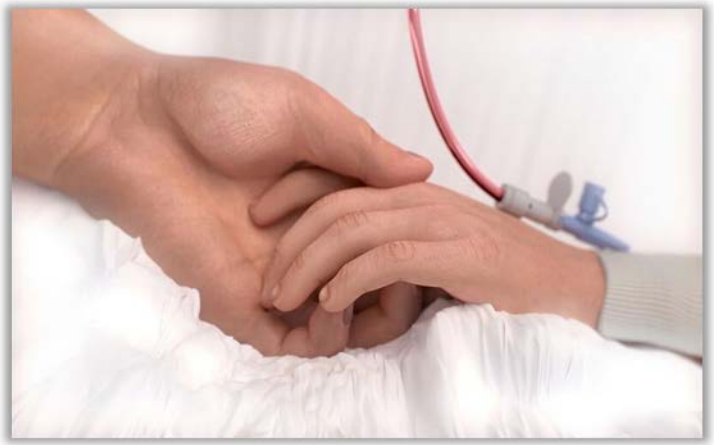
- No regulatory approved treatment for SR-aGVHD outside of Japan
- No broad consensus on off-label second-line agents

Targeting Unmet Need

- Pediatrics: first-line steroid refractory
- Adults: first-line steroid refractory in high-risk (liver/gut disease) patients

Market Opportunity

- ~30,000 allogeneic BMTs performed globally (~20K US/EU5) annually, ~20% pediatric[4,5]
- Our licensee JCR Pharmaceuticals Co., Ltd received full approval in Japan (TEMCELL® HS Inj.) for aGVHD in 2015; reimbursed up to ~$USD195k[3]



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).
3. Based on a ¥JPY = $USD 0.009375 spot exchange rate on as of the market close on November 11, 2016. Amounts are rounded. Source: Bloomberg.
4. Gratwohl A et al Quantitative and qualitative differences in use and trends of hematopoietic stem cell transplantation: a Global Observational Study. Haematologica. 2013 Aug;98(8):1282-90.
5. CIBMTR, Decision resources GVHD Epi Nov 2012.

Remestemcel-L for aGVHD: Product Development Strategy

1. Target ***pediatric*** patients with SR-aGVHD first
   - Extensive safety and efficacy data generated and published in children with SR-aGVHD[1]
   - High economic burden in treatment of children with SR-aGVHD
   - Fast-track designation provides pathway for priority review and rolling review process
   - Submit single, open-label Phase 3 trial for accelerated approval
2. Seek label extension for high-risk ***adult*** patients with SR-aGVHD
   - This adult subset has the highest mortality and greatest resistance to other treatment agents
   - High economic burden in treating this population subset
   - Remestemcel-L has shown efficacy signals in subgroup analyses of this population
3. Lifecycle potential in ***chronic*** GVHD (cGVHD)
   - Chronic GVHD represents a distinct GVHD patient population
   - Proof of concept data already published for MSC in cGVHD[2]

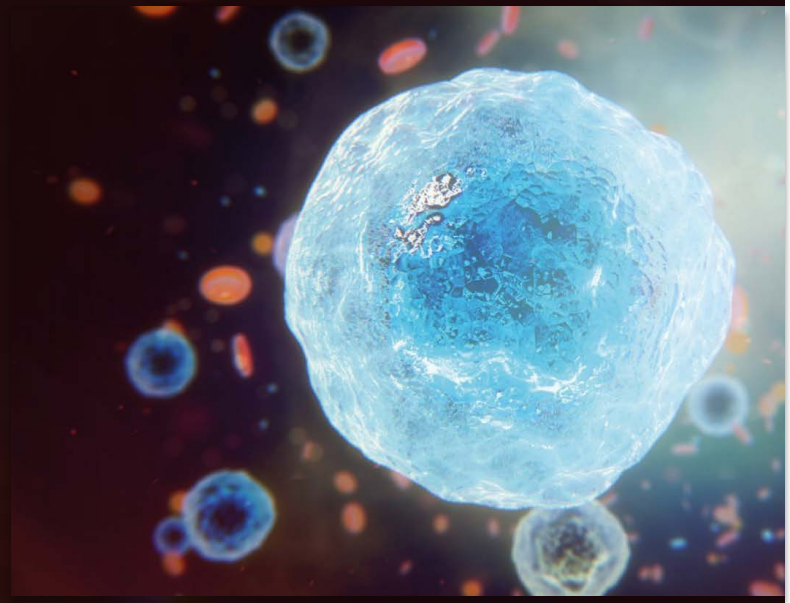
1. Allogeneic Human Mesenchymal Stem Cell Therapy (Remestemcel-L) as a Rescue Agent for Severe Refractory Acute Graft-versus-Host Disease in Pediatric Patients - Biology of Blood and Marrow Transplantation Journal, August 2013. 2. Khandelwal P, Teusink-Cross A, Davies S (2017) Ruxolitinib as Salvage Therapy in Steroid-Refractory Acute Graft-versus-Host Disease in Pediatric Hematopoietic Stem Cell Transplant Patients. Biol Blood Marrow Transplant 23; 1122-1127
2. Weng JY, Du X, Geng SX, Peng YW, Wang Z, Lu ZS et al. Mesenchymal stem cell as salvage treatment for refractory chronic GVHD. Bone Marrow Transplant 45: 1732-1740 (2010)


San Francisco
New York
Dublin
Zurich
Hong Kong
Melbourne
ASX
Nasdaq

Milestones

Targeted Upcoming Milestones and Catalysts

- **Remestemcel-L (MSC-100-IV): for Pediatric Acute GVHD**
  - Day 28 primary endpoint data read-out (Q1 CY18) **COMPLETE**
  - Day 100 survival data (Q2 CY18)
  - Day 180 safety data (Q3 CY18)
- **MPC-06-ID for Chronic Low Back Pain**
  - Phase 3 trial expected to complete enrollment (Q1 CY18)
- **MPC-150-IM for Advanced and End-Stage Heart Failure**
  - Phase 2B Class IV trial six-month primary endpoint reached (Q1 CY18)[1]
  - Phase 2B Class IV trial full data read-out (Q3 CY18)[1]
  - Phase 3 trial for Class II/III targeted enrollment completion (H2 CY18)
- **Potential Corporate Partnerships**



1. Study is funded by the United States National Institutes of Health (NIH) and the Canadian Health Research Institute (CHRI), and conducted by the NIH-funded Cardiothoracic Surgical Trials Network (CTSN).


San Francisco
New York
Dublin
Zurich
Hong Kong
Melbourne
ASX
Nasdaq

Questions?