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the regenerative medicine company

2014 Results & Corporate strategy

Focus on late stage product development

26 August 2014

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Significant achievements to date

- Successful translation of ***three*** cell-based technology platforms for multiple applications
- Robust IP portfolio of ***>60 patent families*** provides long-term commercial protection for mesenchymal lineage cells
- Portfolio of allogeneic regenerative medicine products, with ***five*** in Phase 3/Phase 3 ready
- Evidence-based mechanisms of action support clinical translation
- Established strategic partnerships enhance commercial success
- Capital market support and strong balance sheet

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2014 highlights

- FDA clears 1730 patient Mesenchymal Precursor Cell (MPC) Phase 3 trial in NYHA class II/III heart failure; actively recruiting across multiple North American sites
- NIH and MSB agree on 120 patient MPC trial in advanced / NYHA class IV heart failure
- End of Phase 2 meeting with FDA supports advancing to MPC Phase 3 trial in Chronic Discogenic Lower Back Pain (CDLBP)
- Acquired culture expanded Mesenchymal Stem Cell (MSC) assets from Osiris Therapeutics Inc.
- FDA discussions clarify pathway to accelerated US approval for GVHD
- Strategic relationship formed with Singapore Economic Development Board
- Positive type 2 diabetes trial results presented at 74th American Diabetes Association Annual Meeting
- Results of Type 2 diabetes trial provide support for the ongoing diabetic nephropathy trial

Financial Results

AUDm

	30 June 14	30 June 13	Change	%
Revenue from Continuing Operations	**26.0**	**28.8**	**(2.8)**	**(10)**
Commercialization revenue	16.4	18.3	(1.9)	(10)
Interest revenue	9.6	10.5	(0.9)	(9)
Other Income	**11.1**	**5.9**	**5.2**	**88**
Research & development tax incentive	8.6	5.9	2.7	46
Write back of unutilized provision	2.5	-	2.5	Nm
Expenses from Continuing Operations	**118.1**	**94.7**	**23.4**	**25**
Research & development	55.3	47.8	7.5	16
Manufacturing commercialization	27.6	23.2	4.4	19
Management & administration	26.6	22.8	3.8	17
Finance costs	4.3	-	4.3	Nm
Other expenses	4.3	0.9	3.3	Nm
Income tax expense	-	1.6	(1.6)	Nm
Loss After Tax	**81.0**	**61.6**	**19.3**	**31**

Nm = Not meaningful

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Operating Cash Burn Rate

AUDm

	30 June 2014	30 June 2013
Cash on hand *	**196.4**	**315.3**
Operating Cash Burn	**81.9**	**54.1**
Adjustments:		
Manufacturing timing of payments	(4.6)	4.6
One-off settlement of a provision	(5.9)	-
Corporate taxation refunds	6.8	3.3
Normalized Operating Cash Burn	**78.2**	**62.0**

Increased operating cash burn reflects:

- Increased spend on MPC clinical development and MSC clinical programs
- Investment in commercial manufacturing capability in Singapore and clinical supply
- Investment in employees supporting both MPC and MSC products

* Within the cash on hand movement investing cash flows include a one-off $35.5m for acquisition of MSCs assets from Osiris

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Corporate Strategic Imperatives

1. Clinically differentiated products
2. Focus on bringing late stage products to market
3. Enable manufacturing scale up to meet demands of portfolio
4. Leverage expanding talent base to continue to establish a culture of shared leadership and accountability
5. Continue to build strategic partnerships

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1. Clinically differentiated products

- Development of product-specific intellectual property
- Targeting of multiple indications based on sound science and unmet medical need
- Establishment of product specific pricing based on value based outcomes
- Parallel licensing/partnering strategies for each product

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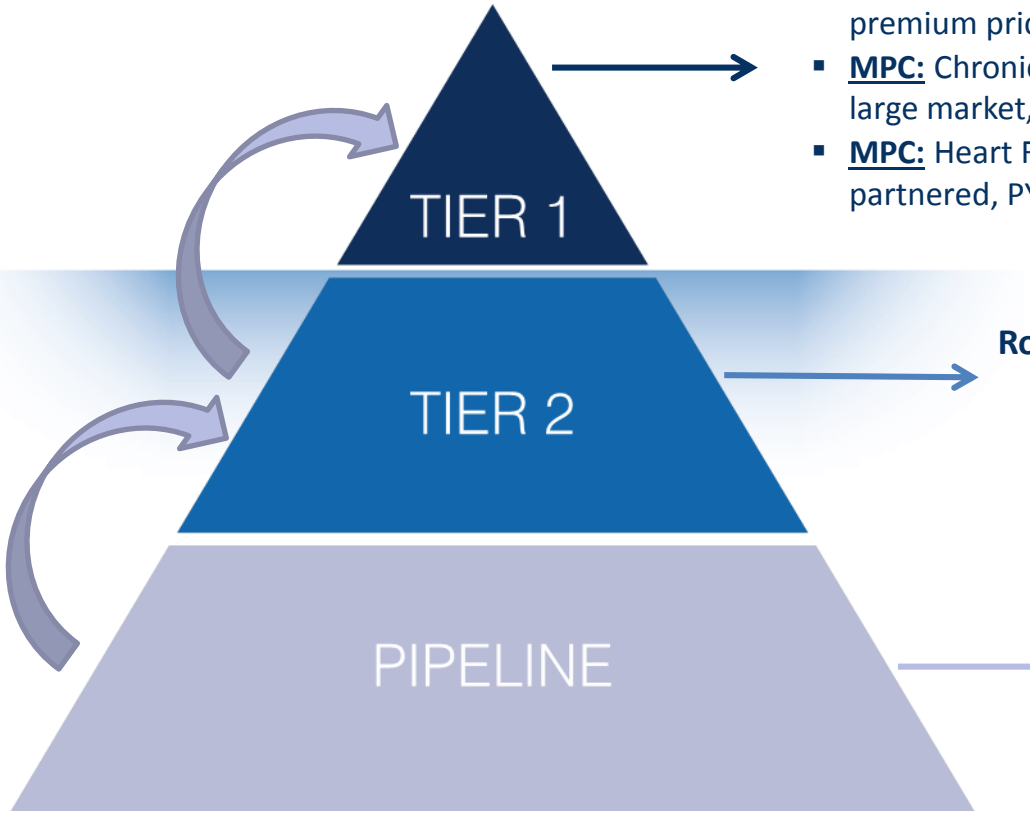
2. Focus on bringing late stage products to market



Products with mid term revenue streams

- **MSC:** GVHD – "Halo" niche product near to market, premium priced, PYS >$250m
- **MPC:** Chronic Discogenic Low Back Pain – low dose, large market, PYS USA >$2bn
- **MPC:** Heart Failure – large market, high value, partnered, PYS >$2bn

Robust Development Portfolio

- Progressing to next decision point
- Targeted for partnership
- Capable of acceleration

Emerging Pipeline

- Early stage development and pre-clinical work

PYS = Peak Year Sales ($US)

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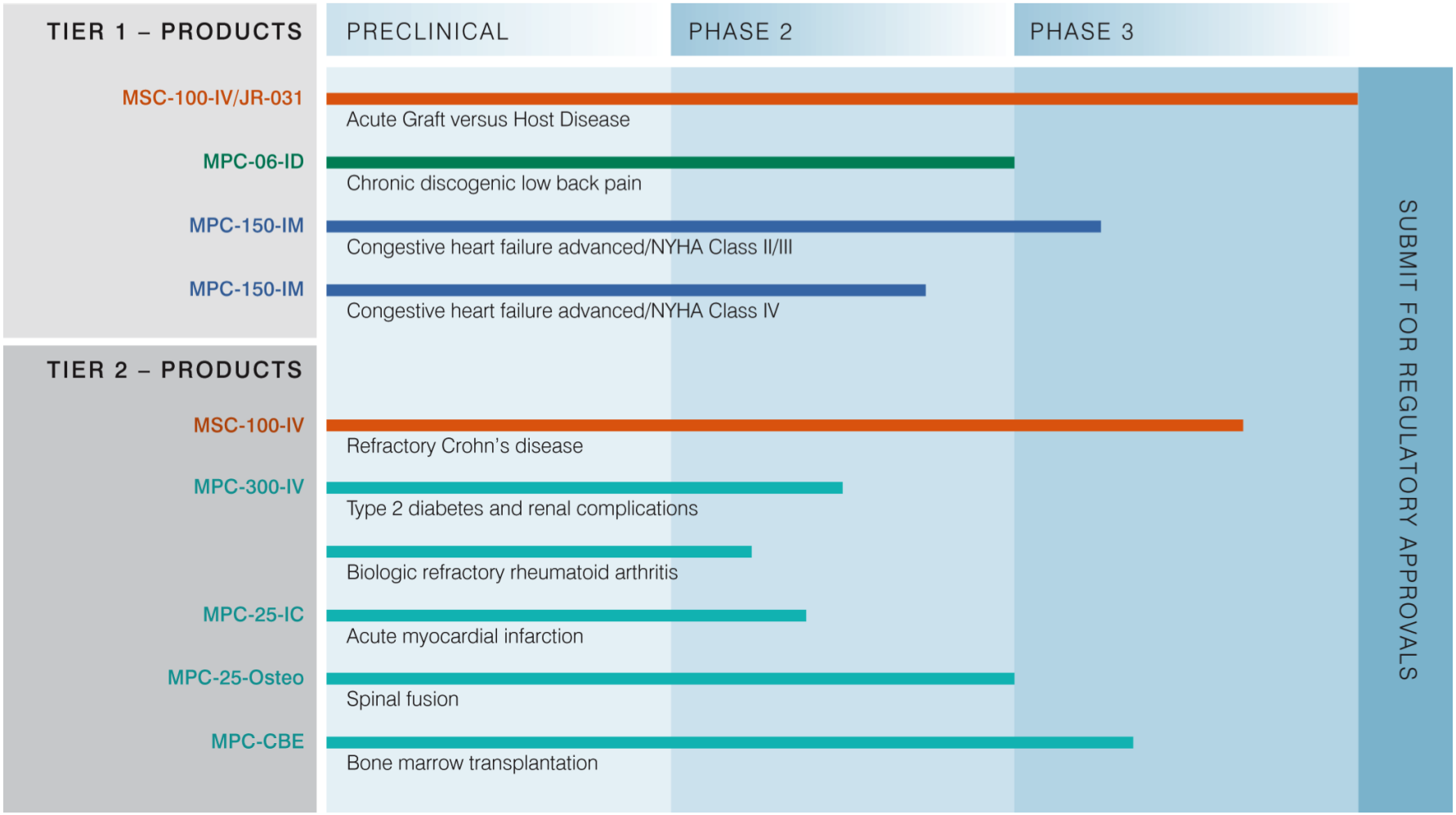
Robust Development Portfolio of Tier 1 and Tier 2 products



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MSC-100-IV: Graft vs Host Disease – Market Opportunity

MSC-100-IV is in development for pediatric and adult patients with acute Graft versus Host Disease (GVHD) following allogeneic hematopoietic stem cell transplant (HSCT) who have failed to respond to steroid treatment

Market Opportunity

- ~30,000 allogeneic HSCTs performed globally each year, 25% pediatric [1,2]
- ~40-50% of all patients develop GVHD (Grades II-IV) [3]
- ~50% acute GVHD steroid-refractory [4]
- Total one yr cost of multi risk factor transplant complications USD$845,000 - $1,000,000 [5]
- Ultra orphan indication with premium pricing potential

No Approved Treatment Options

- Mortality can reach 85% in patients with liver & gut complications
- No currently approved therapies for steroid refractory patients
- Off-label options have mixed efficacy with high toxicity
- Significant need for a new treatment with a favorable risk/benefit profile

Targeted physician population

- Highly targeted physician audience & commercial footprint for lead launch in pediatrics
- ~ 68 centers in the US conduct pediatric allogeneic HSCTs
- ~ 50% of all pediatric transplants concentrated in 12 centers & key metropolitan areas

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MSC-100-IV: Graft vs Host Disease – Evidence Of Efficacy

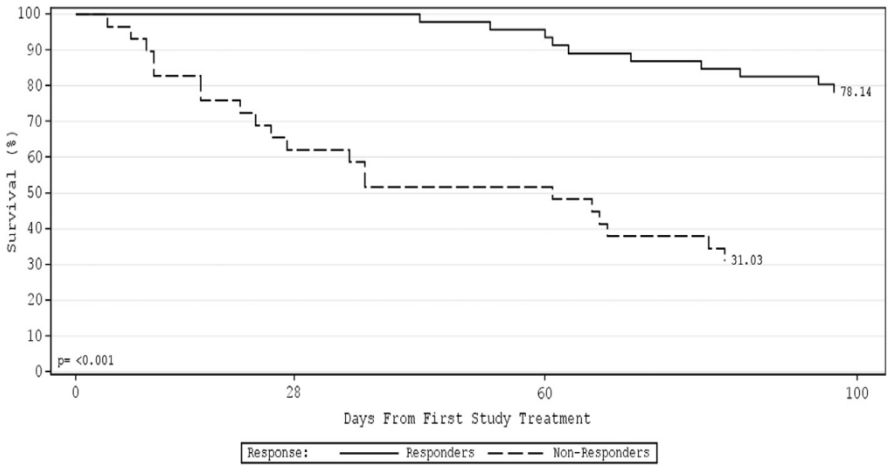
Pediatric Patients [6]

- Expanded Access Program in US has treated in excess of 200 patients
- In first 75 patients, response at day 28 to MSC-100-IV therapy was a significant predictor of improved day 100 survival ($p<0.001$)
- Day 100 survival was 76% in MSC-100-IV responders, compared to 28% in non-responders (p value <0.001, log rank test)

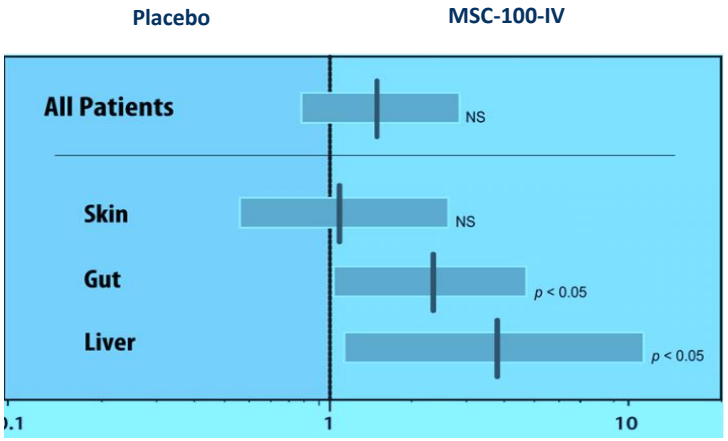
Adult Patients [7]

- In a randomized, placebo-controlled Phase 3 trial, MSC-100-IV significantly improved overall responses in the adult subset with gut or liver GVHD, and resulted in improved survival in this important subset.

Pediatric Day 100 survival [6]



Adult response day 28 by organ (Odds Ratio) [7]



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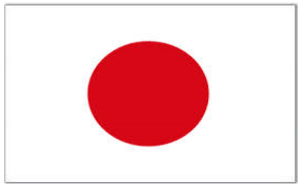
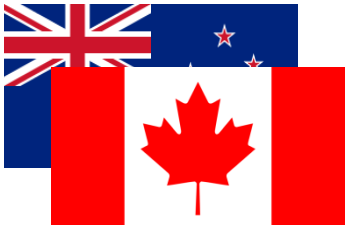
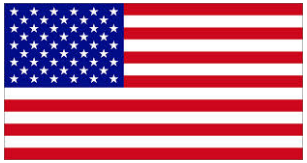
MSC-100-IV : Graft vs Host Disease – Product Launch



- JCR Pharmaceuticals plans to file for registration H2 2014
- Japanese launch 2015



- Canadian launch 2016
- New Zealand launch 2016



- Positive FDA meeting June 2014 - pathway for accelerated approval clarified
- 60 patient open-label phase 3 pediatric trial
- Confirmatory phase 3 adult trial in liver/gut subset
- BLA filing for pediatric registration 2016 / target launch 2017

MSC-100-IV has potential to be first allogeneic stem cell product approved in United States – “halo” effect for Mesoblast’s commercial go to market capability

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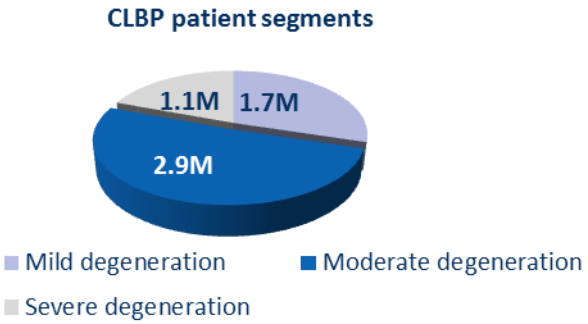
MPC-06-ID: Chronic Discogenic Low Back Pain (CDLBP) – Market Opportunity

MPC-06-ID is in development for the treatment of chronic low back pain (CLBP) lasting >6 months as a result of moderate degenerative intervertebral disc disease

Market Opportunity

- Prevalent CLBP population in the US to grow to 21.9m patients by 2022 [8]
- 55% of CLBP population seek treatment [8]
- ~ 40% of patients with CLBP have a discogenic cause



Gap in Treatment Options

- For patients who fail conservative treatment (rest, analgesia), and epidural steroids, treatment options are limited to highly invasive therapies such as vertebral disc fusion or artificial disc replacement
- Surgeons report that ~40% of patients ultimately fail back surgery [9]

Targeted Physician Population

- Specialists: Targeted physician audience & commercial footprint
  - Pain management specialists, and anaesthesiologist
  - Orthopedic / spine surgeons

MPC-06-ID is positioned to fill the significant treatment gap after conservative treatment options have failed

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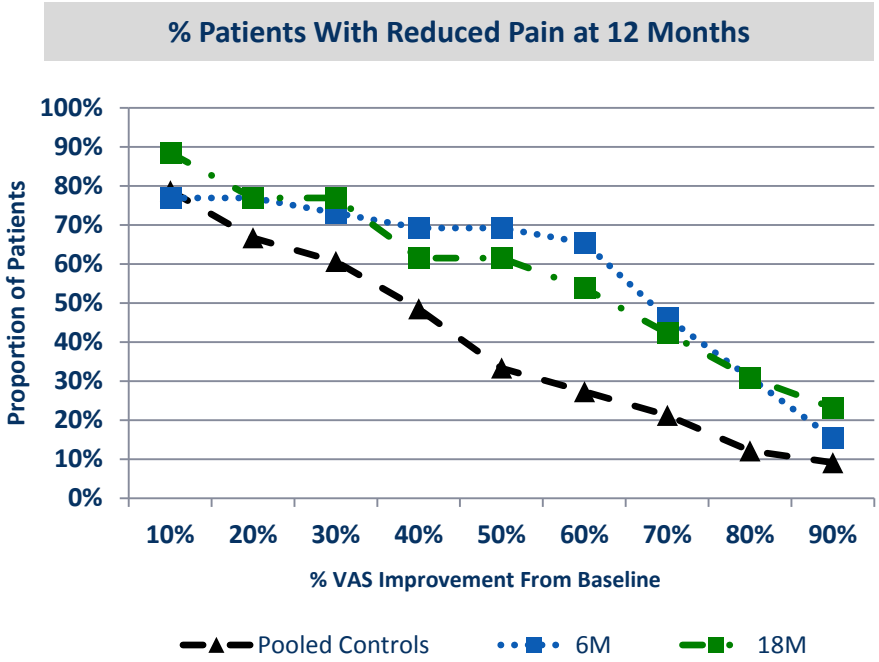
MPC-06-ID: CDLBP – Phase 2 Results

Phase 2 trial: 100 patients with > 6 months of discogenic low back pain failing other therapies were evaluated in a randomized, placebo controlled trial that compared saline, HA, HA+6M MPC, HA + 18M MPC's injected into culprit painful disc



- **52% of the 6M group and 42% of the 18M group** achieved a minimal residual back pain at 12 months compared with 18% for controls (p=0.01 and p=0.05 respectively)
- **69% of the 6M group and 62% of the 18M group** achieved >50% reduction in Visual Analog Score (VAS) for pain at 12 months compared with 33% of controls (p=0.009 and p=0.038 respectively)

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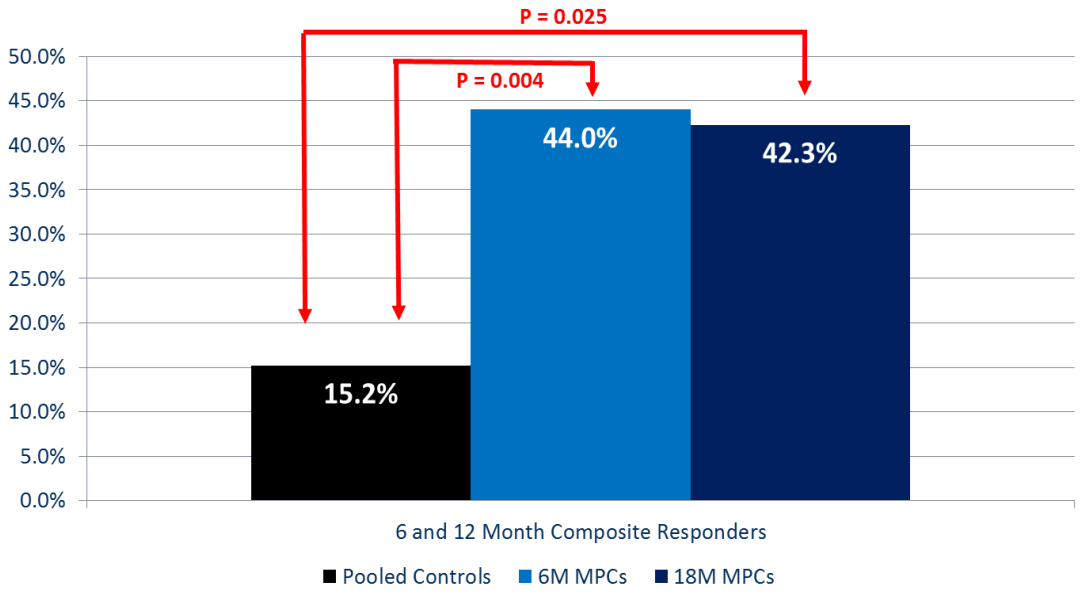
MPC-06-ID: CDLBP – Composite Endpoint for Phase 3

Treatment options for CDLBP must address both decreased pain and improved function, defined as Minimally Important Changes – at least 30% improvement in VAS and at least 10 point improvement in Oswestry Disability Index (ODI) [10]



% Responders positive at both 6 and 12 months for Composite of 50% VAS back pain reduction + 15 point ODI improvement + no intervention at the treated level

- Positive End of Phase 2 meeting with FDA supports progression into Phase 3
- Phase 3 study design will measure composite endpoint of decreased pain and improved function at 12 months
- Phase 3 trial initiation end of 2014

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MPC-150-IM: Congestive Heart Failure (CHF) – Market Opportunity

MPC-150-IM is in development for New York Heart Association (NYHA) class II to IV heart failure

Market Opportunity

- 5.1 million people in the US are diagnosed with CHF (2% of the population) [11]
- 825,000 new cases diagnosed each year – growing at 10% per annum [11]
- Mortality among CHF patients is 7% p.a.
- Targeting CHF patients with NYHA Class II/III with low ejection fraction (<35-40%): ~ 40% of all heart failure patients [12]

Gap in Treatment Options

- Despite standard of care using pharmacological treatments, Class II/III patients continue to be at high risk of repeated hospitalizations and mortality
- Heart transplant and mechanical support are the only options for advanced/NYHA Class IV patients

Targeted Physician Population

- Specialists: Targeted physician audience & commercial footprint
  - Heart Failure Specialists,
  - Interventional cardiologists
  - cardiac surgeons

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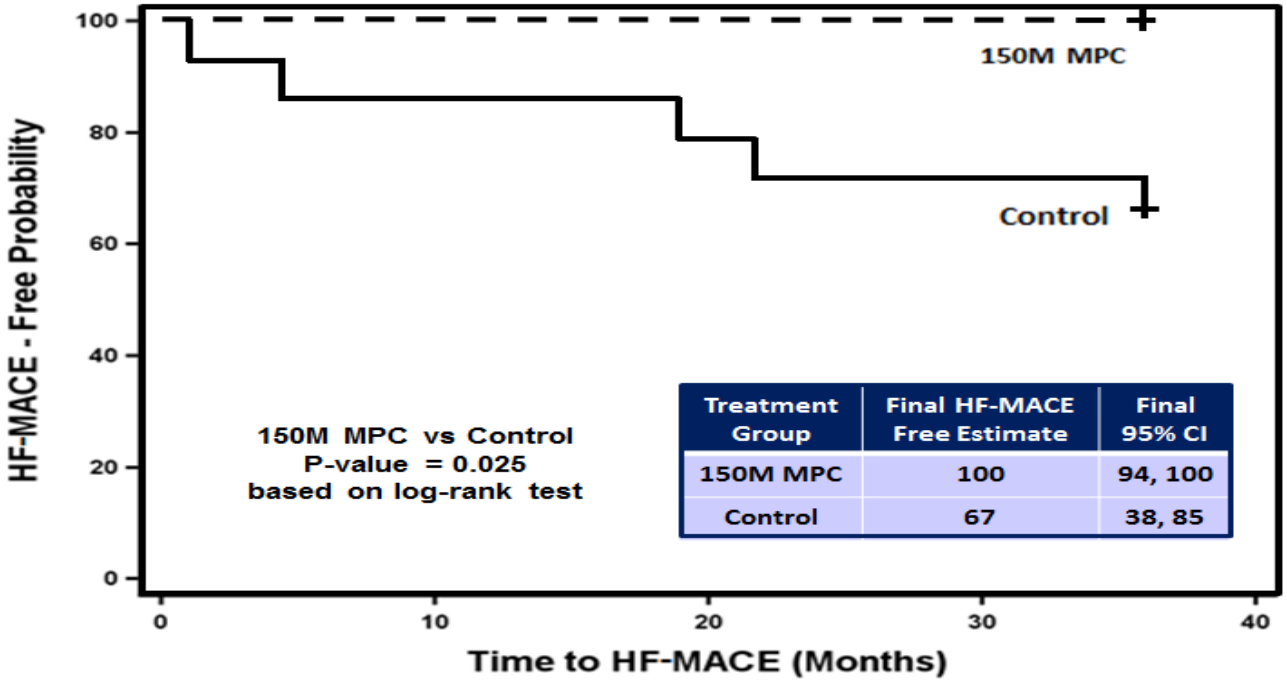
MPC-150-IM: Congestive Heart Failure (CHF) – Phase 2 Results

The MPC dose for the Phase 3 trial was chosen on the basis of results from a 60-patient Phase 2 trial



Treatment Group	Final HF-MACE Free Estimate	Final 95% CI
150M MPC	100	94, 100
Control	67	38, 85

Kaplan-Meier Analysis for Time-to-First HF-MACE 150M vs Control Treatments (36 Months Follow-up)

Phase 3 primary efficacy endpoint: time-to-first event analysis of heart failure-related major adverse cardiac events (HF- MACE)

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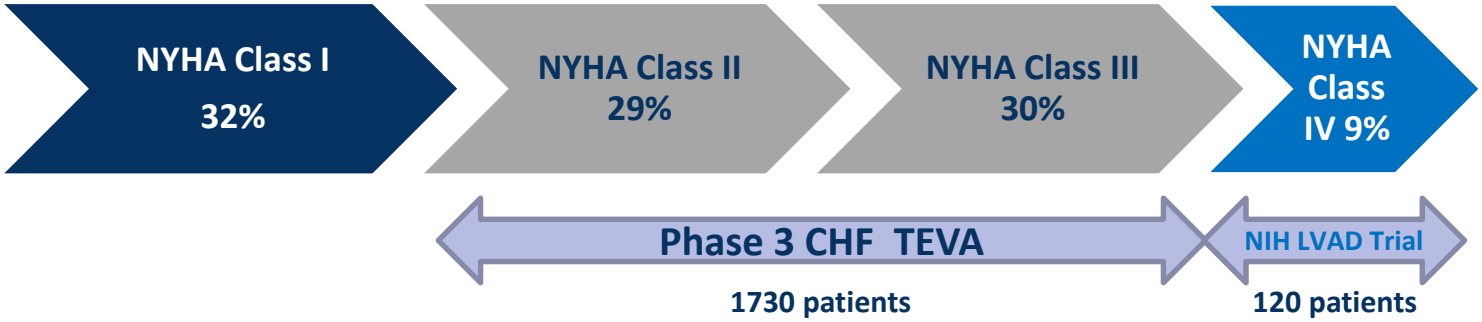
MPC-150-IM: Congestive Heart Failure (CHF) – Trials underway

New York Heart Association (NYHA) classification of heart failure



- NYHA Class II/III Phase 3 Program up and running with targeted completion in 2018. Options for acceleration being discussed with Teva Pharmaceutical Industries and regulatory authorities
- The National Institutes of Health (NIH) is conducting a 120 patient study in advanced/NHYA Class IV patients with full trial results expected in H2 2016

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3. Objectives of commercial scale manufacturing

1. Distinct manufacturing processes for each product

2. Commercial scale processes with batch-to-batch consistencies and reproducible release criteria

3. Ensure commercial product supply is aligned with projected market needs

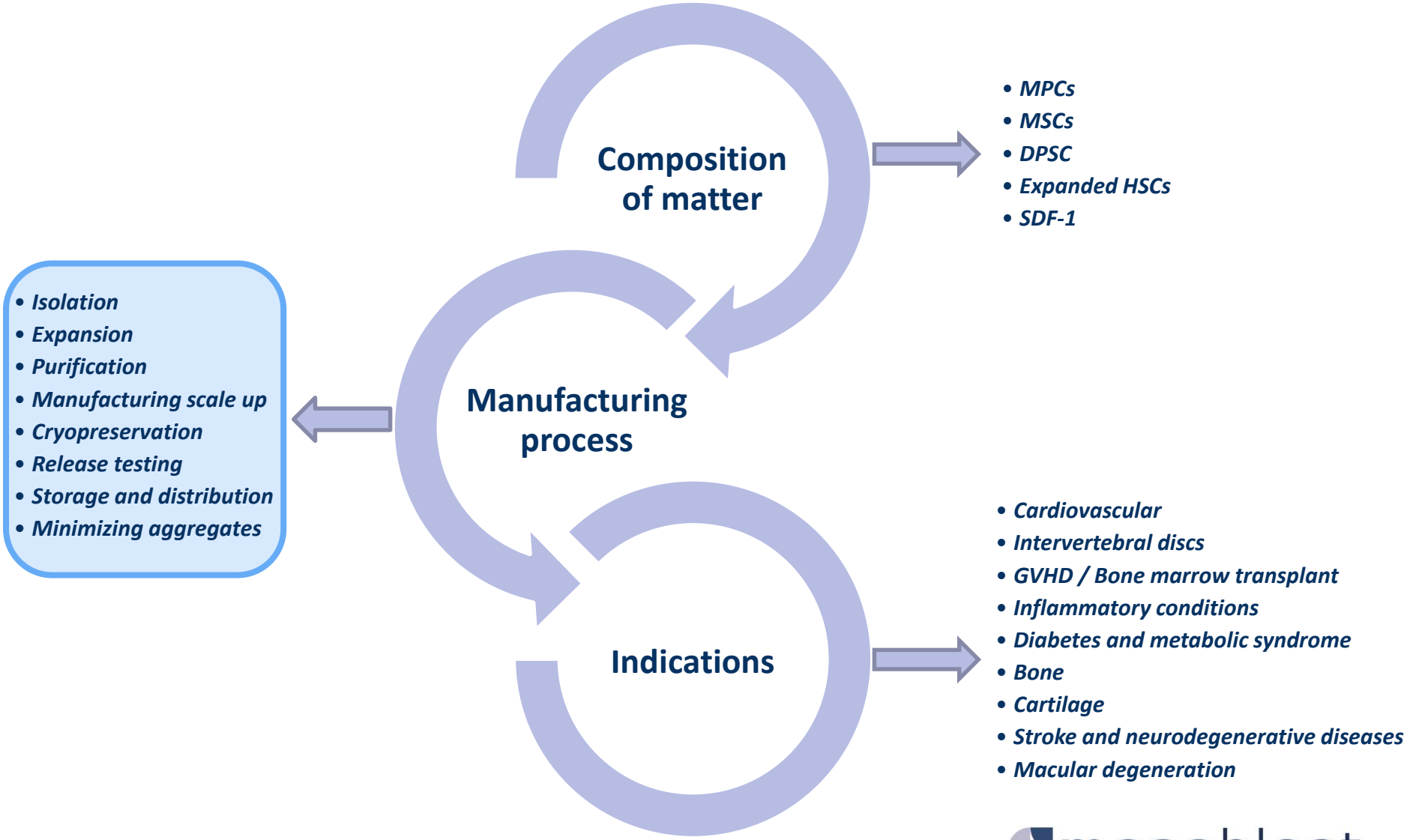
   – Production hub in Singapore

   – Scale up in 3D Bioreactors

   – Optimised cost of goods

Working with our expert partners Lonza and the Singapore Economic Development Board (EDB)

Manufacturing IP enhanced as well as IP for compositions and indications



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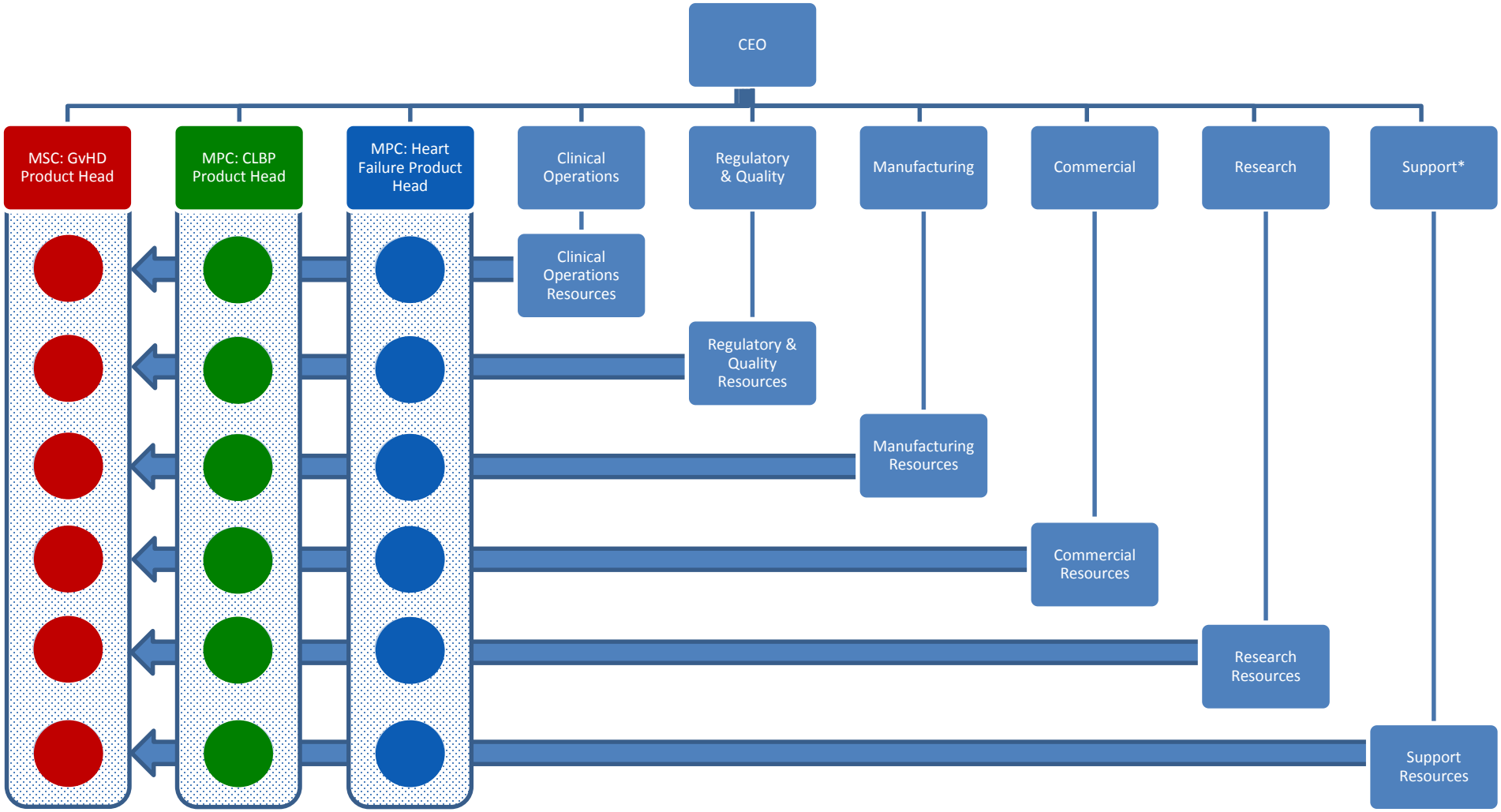
4. Create a product-focused organizational structure



**Support = Finance, Legal, Communications, Project Management, Pharmacovigilance, Human Resources, Medical Affairs*

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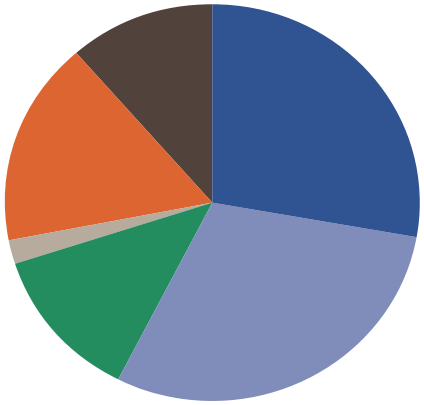
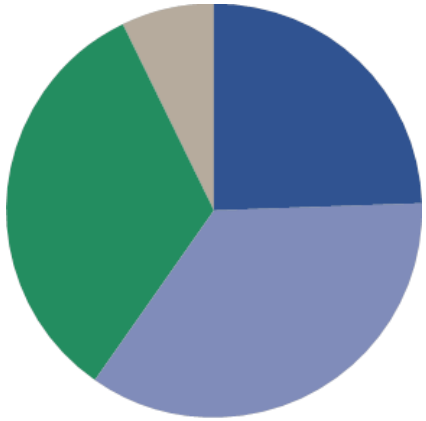
Build an experienced team for late-phase clinical execution and market launch

Industry Background



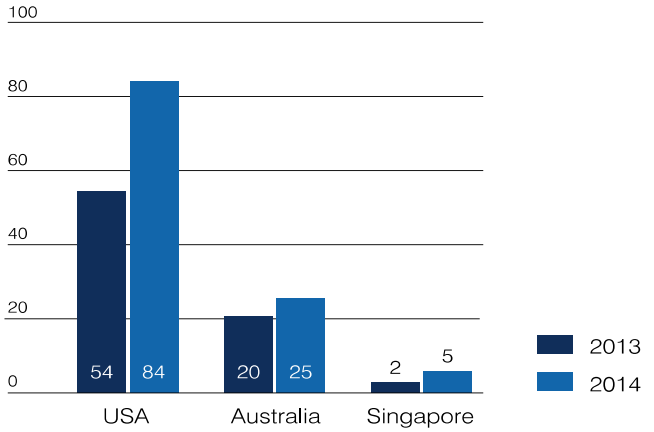
- Pharma – 27.8%
- Specialty Biotech – 29.9%
- Academia – 12.4%
- Regulatory Agencies – 2.1%
- Corporate/Professional – 16.5%
- Other – 11.3%

Qualification Level

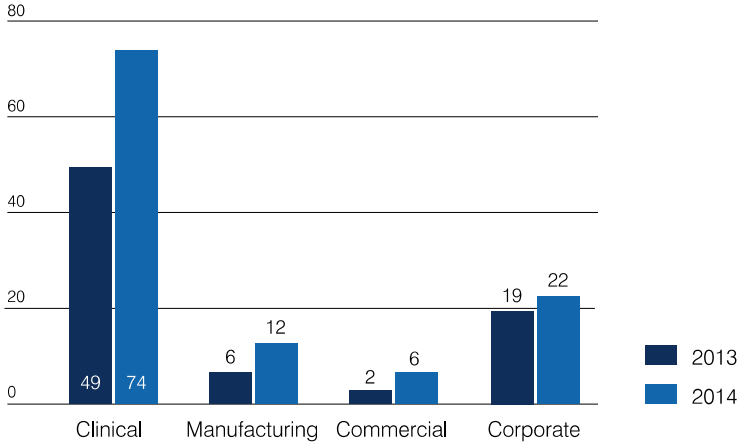


- PhD/MD – 24.7%
- Masters degree – 35.1%
- Bachelor degree– 33.0%
- Other – 7.2%

Location



Function



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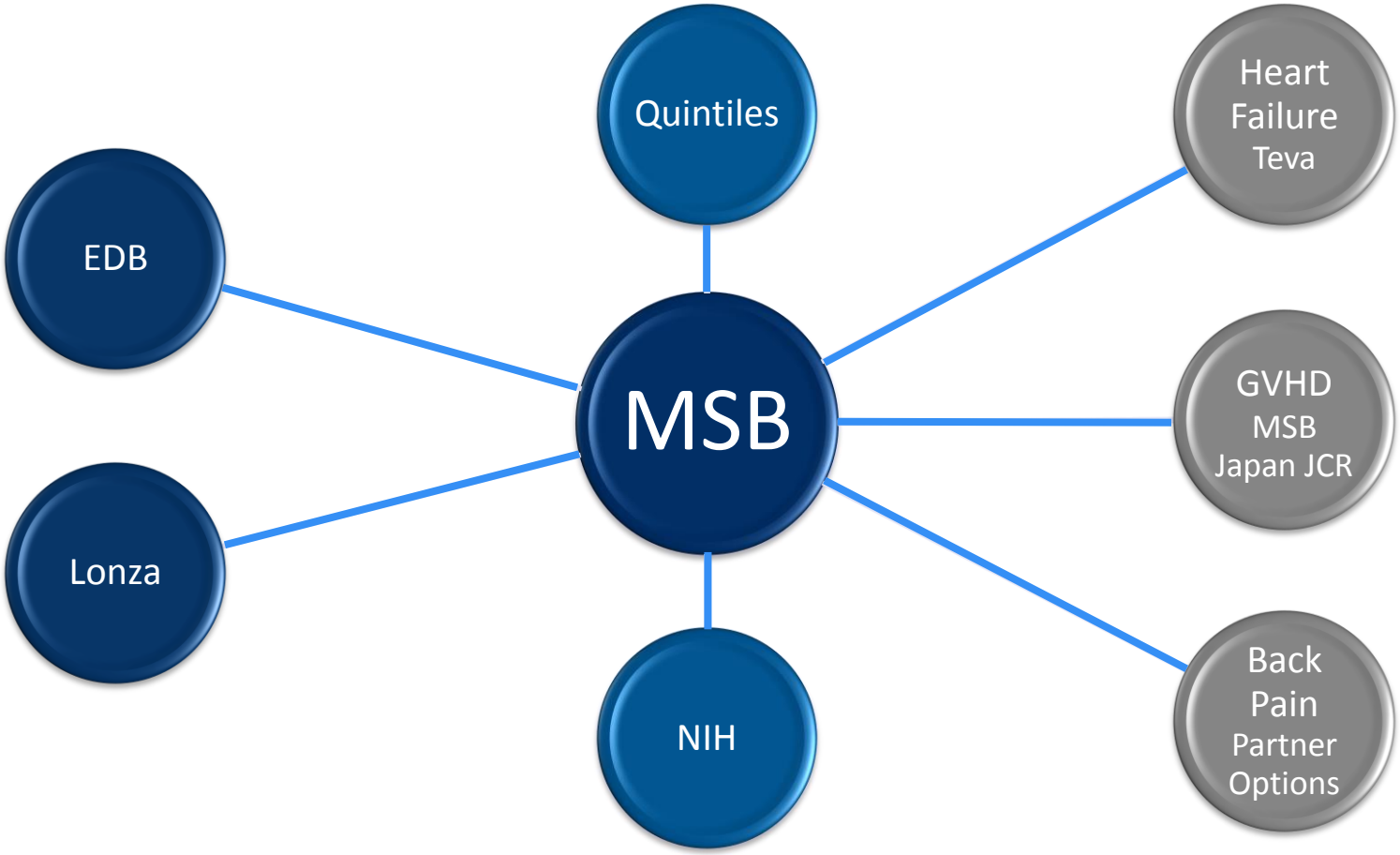
5. Build strategic partnerships

Manufacturing | **Development** | **Commercialisation**



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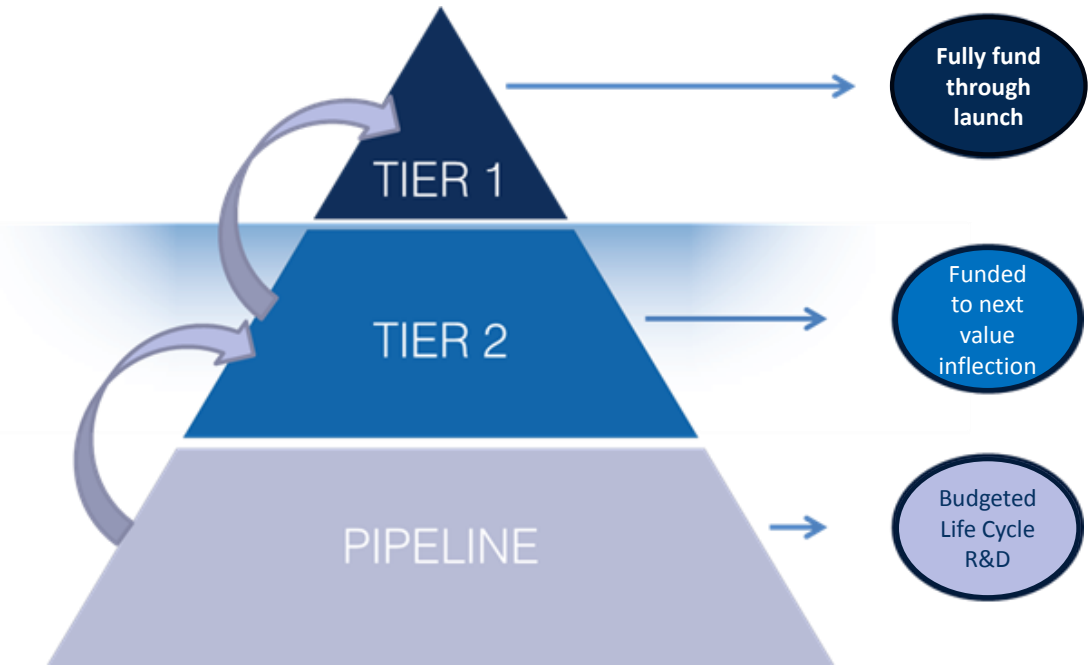
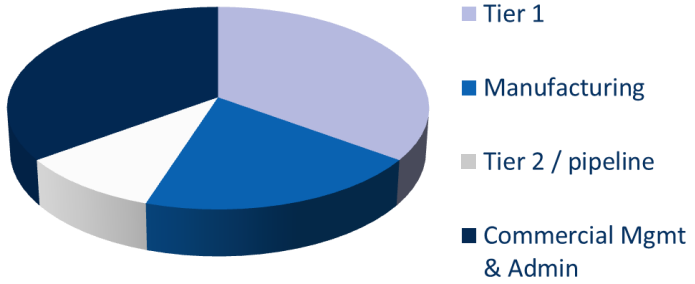
Use of financial resources



Allocation of Funds



Further sources of funds include:
Strategic Alliances, Government Partnering, Capital Markets and Product Revenues

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Outlook for next 12 months

GVHD – Unmet need with near term milestones

- Pediatric trial recruitment to support BLA filing for accelerated approval in US
- Parallel US trial in adults with liver/gut GVHD
- Commercial manufacturing to support product launches
- JCR partner to file for registration in Japan

Back pain – For patients who have few options

- Commence and actively recruit Phase 3 program in North America

CHF – Significant market opportunity

- Continue recruiting Phase 3 trial in NYHA class II/III heart failure across North American sites
- Interim analysis for safety/surrogate efficacy endpoints for HF-MACE
- Recruitment of NIH-funded trial in advanced NYHA class IV heart failure

Tier 2 products / pipeline

- Decision points for Type 2 diabetes/diabetic kidney disease, Crohn's Disease, Rheumatoid Arthritis

Realize Tier 1 investment potential

- Focus on strategic alliances and other funding sources to enhance commercial success

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Questions / Thank you

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