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the regenerative medicine company

Building out a multi-product regenerative medicine company

Keynote presentation to Alliance for Regenerative Medicine Investor Day

New York, 26 March 2014

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Successful products built on traditional Pharma fundamentals

Commercialization strategy

1. **Proprietary platform technology**
   - adult vs embryonic vs iPS
   - autologous vs allogeneic
2. **Intellectual property**
   - protection of commercial interests
3. **Strong cash balance**
   - strategic needs, product development
4. **Strategic partnerships**
   - product development and commercialisation
5. **Development of multiple products**
   - target unmet medical need
   - technical/competitor advantages

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Manufacturing capabilities

1. **Defined regulatory path**
2. **Control of manufacturing**
3. **Product delineation for partners, reimbursement**
4. **Commercial scale-up**
5. **Reduction of COGS**

Product on market

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1 Proprietary platform technology – must carry inherent success factors to support product development and business model

- Mesoblast developing proprietary adult mesenchymal lineage cells, including Mesenchymal Precursor Cells (MPCs), Mesenchymal Stem Cells (MSCs), Dental Pulp cells and Adipose cells

  – IP protection for cell type, manufacturing process, indication
  – Adult stem cells not associated with ethical or safety issues of embryonic stem cells and iPS cells
  – excellent safety profile across multiple clinical indications
  – easy to expand in large numbers
    - low cost of goods, no supply constraints
    - high margin business model
  – relatively non-immunogenic, can be used from one donor for many recipients
    - “off the shelf”, classic pharmaceutical drug model
    - batch to batch consistency
    - clear, rapid regulatory pathway

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2 Intellectual property (IP) – protects commercial interests

- Mesoblast owns or has exclusive rights to more than 60 patent families covering mesenchymal lineage cells, which provide commercial advantages and long-term protection across its technology platforms
- Acquisition of Osiris culture-expanded MSCs business was complementary and additive to Mesoblast's existing patent position on MPCs, Dental Pulp and Adipose cells
- IP covers compositions-of-matter, cell extraction processes for purification and manufacturing scale-up, and a broad range of applications in key markets including the United States, Europe, Japan and China

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3 Strong cash position – allows multiple product development

Commercial success of platform technology developer is critically dependent on having access to substantial sources of funds, in order to

- ensure timely, optimal product clinical development
- enable multiple clinical development programs in parallel
- allow maximal exploitation of patent life
- facilitate establishment of more balanced partnering relationships

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4 Strategic partnerships – additional sources of funding, validation of technology, access to product commercialization and other areas of partners' expertise

- Mesoblast's alliances include:
  - cardiovascular and neurological MPC programs being developed in partnership with **Teva Pharmaceutical Industries Ltd**
  - Graft Versus Host Disease MSC programs in children and adults in Japan being developed in partnership with **JCR Pharmaceuticals**
  - strategic relationship with biologics manufacturer **Lonza** to ensure commercial scale-up and supply, product delineation, COGS reductions, and expansion of clinical manufacturing

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Manufacturing capabilities – define ability to commercialize cell-based products

- Manufacturing strategy must incorporate regulatory, commercial, and R&D strategies and consider:
  - Control of manufacturing
  - Regulatory compliance with best practice
  - Commercial scale-up with capacity for commercial product supply
  - Product delineation to support and separate partner markets and optimise reimbursement
  - Profitability variables including reduction of COGS
  - Management of product lifecycles and new product development
    - changes in formulation or dosage
    - products derived from different tissue sources (e.g. bone marrow, adipose, dental pulp),
    - combination therapies using different modes of delivery or devices, biologic modifications of cells

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5 Concurrent development of multiple products

- Increases risk-adjusted probability of success
- Leverages safety data across technology platform
- Allows re-focusing of clinical strategy as results become available
- Leverages IP protection maximally
- Early success may validate rest of pipeline

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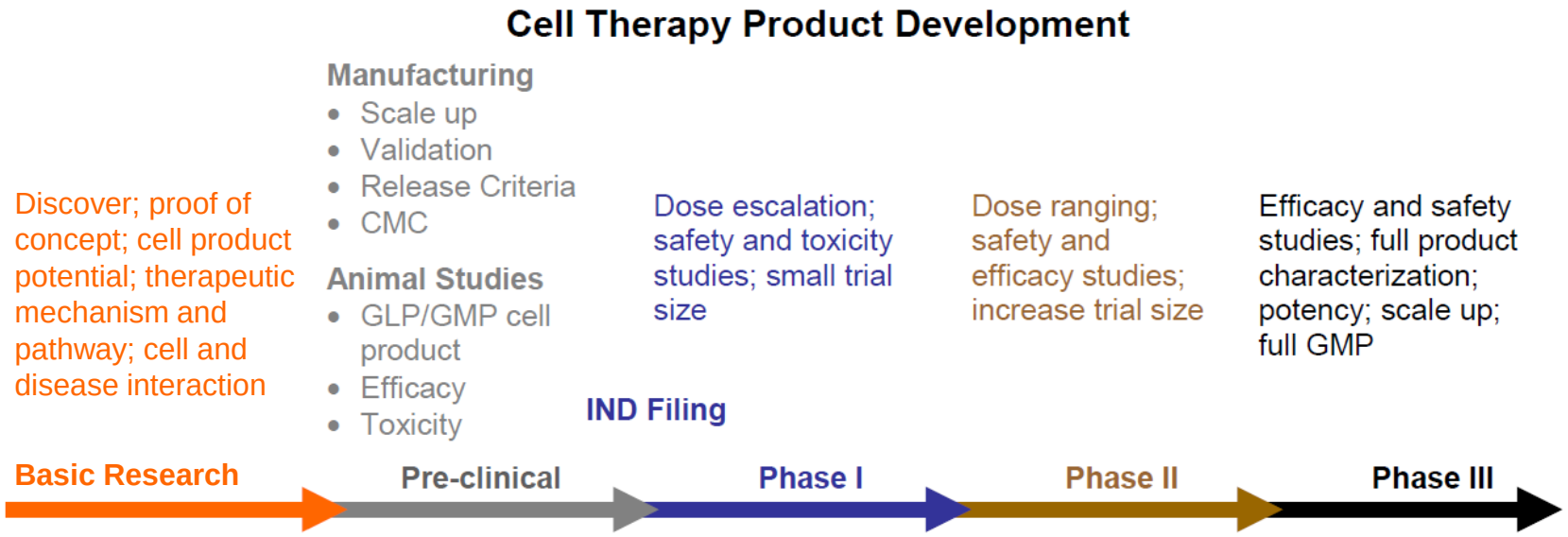
Defined product regulatory path – continues to evolve for cell-based products, combining traditional approval process with unique features



PACT Website, Accessed on March 25, 2014
https://secure.emmes.com/pactweb/system/files/evaluation_criteria_v3_0.pdf

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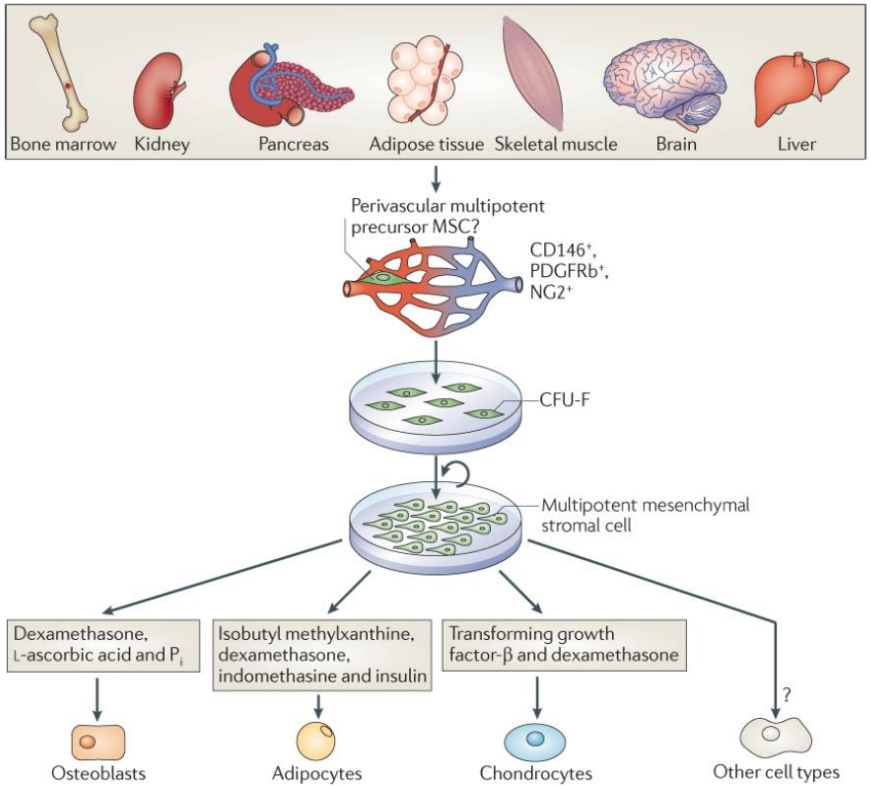
Cell variability drives regulatory paradigm

- Cell variability at a number of levels
- Donor - allogeneic/autologous, gender, age, health?
- Tissue source – bone marrow, adipose, cardiac, neural?
- How are the cells isolated/processed? Manual or device?
- Are the cells cultured? If so, how? Serum y/n? PD? Scale-up?
- Product characterization – surface markers for identity and purity?
- Potency (biological activity) assay candidates?
- Cryopreservation? Final formulation? Stability?
- Comparability between lots and between products?



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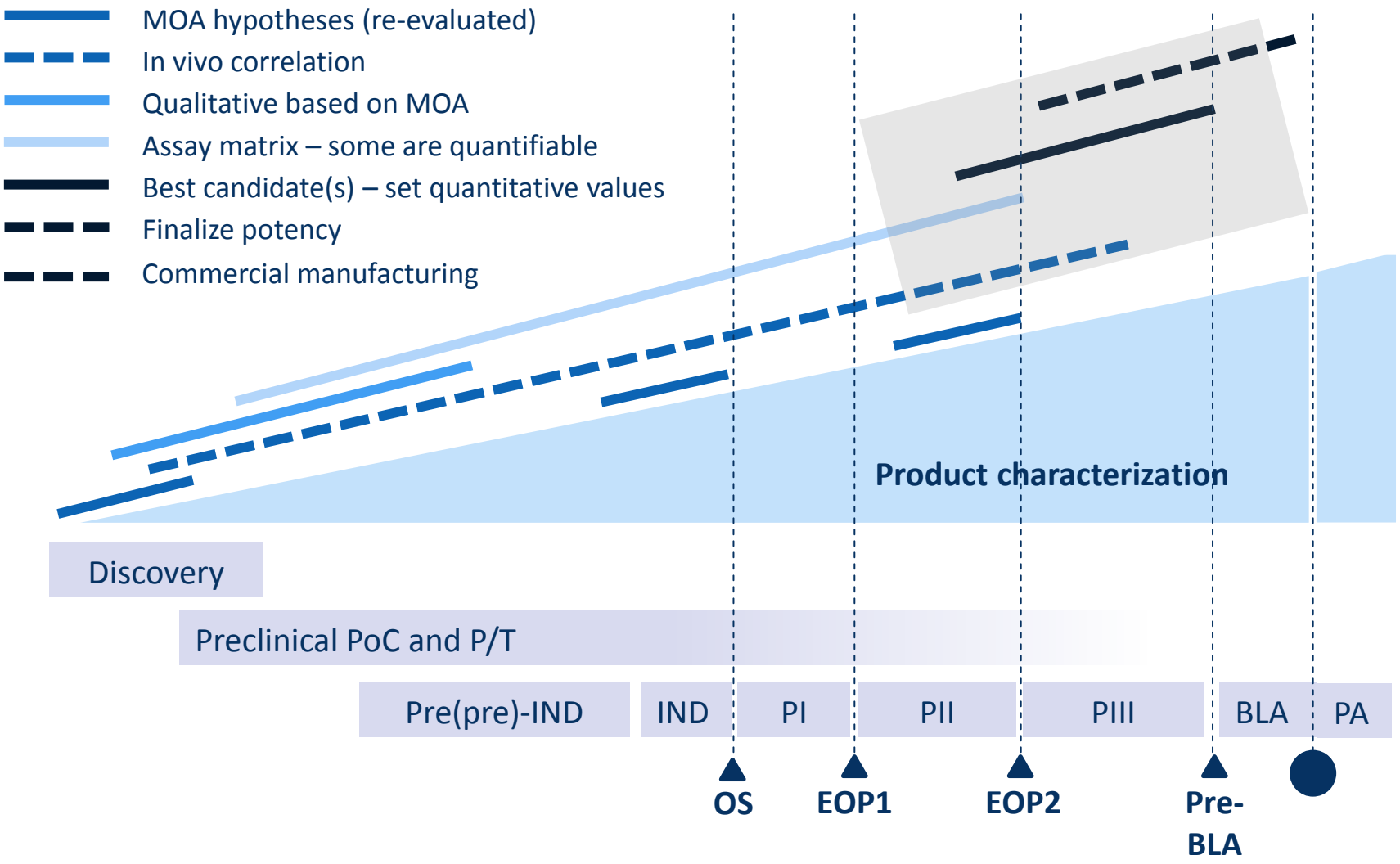
Ongoing regulatory interactions essential for successful product launch



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Mechanisms of Action (MOA) drive product development

- Must understand well the unique MOAs and scientific advantages attributable to the particular cell therapy being developed
- Is the proposed MOA replacement of damaged tissue by engraftment of differentiated or undifferentiated cells?
- Is the proposed MOA repair of endogenous tissue by secretion of paracrine factors?
- Product MOA must provide rationale for specific disease indication being targeted

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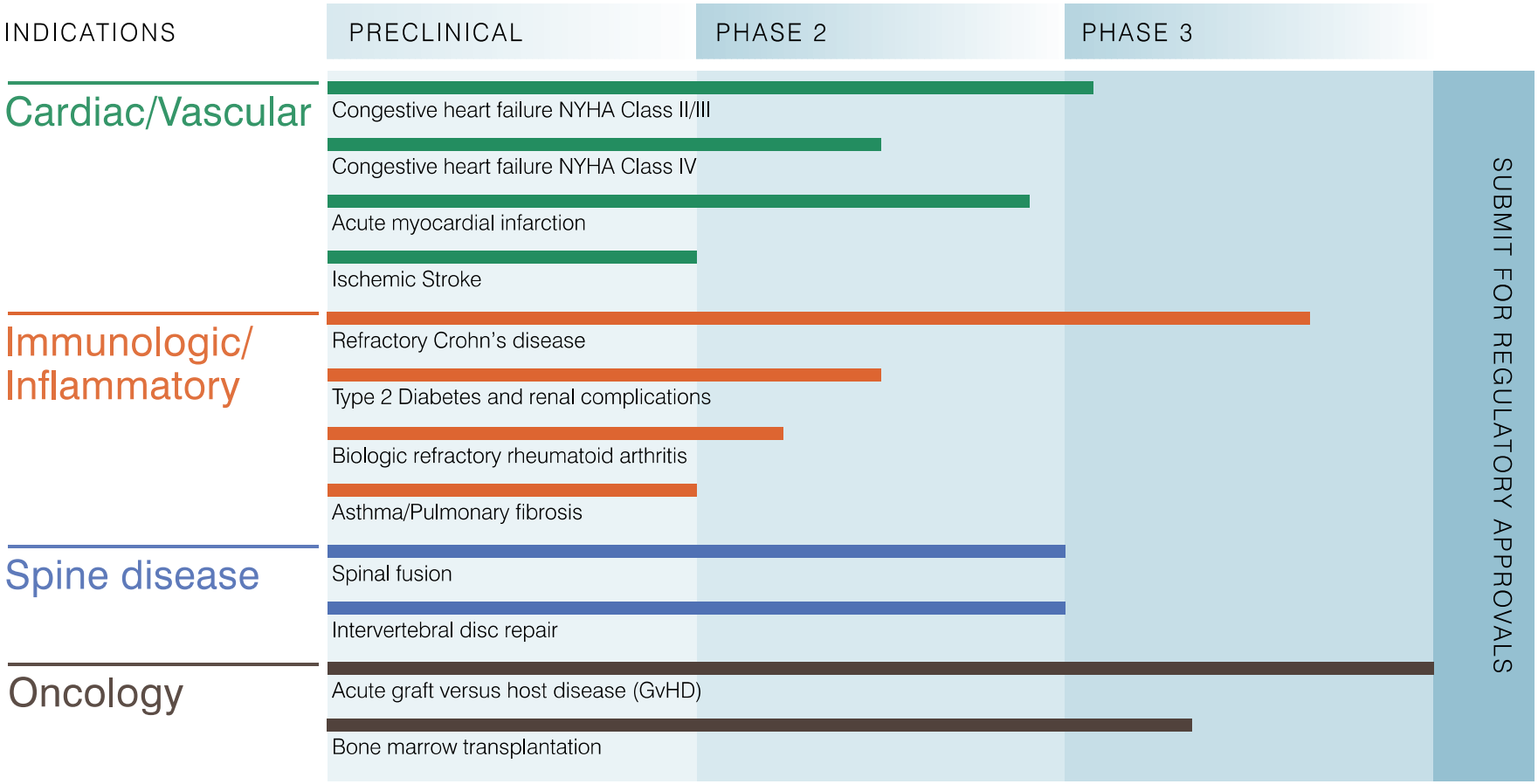
Mesenchymal lineage cells secrete multiple paracrine factors, giving rise to complex MOAs

Mesoblast's strategic product focus is guided by specific MOAs of our cellular therapies

The mesenchymal lineage stem cells respond to signals from inflammation and /or tissue damage by releasing a range of factors which act on endogenous tissues to

- stimulate blood vessel growth and maturation
- reverse endothelial dysfunction
- increase survival and improve function of various cell lineages, including cardiac muscle cells, cells of central nervous system, bone-forming cells, and cartilage-producing cells
- reduce scar formation and fibrosis
- induce polarization of pro-inflammatory monocytes to a non-inflammatory phenotype
- inhibit activated T cells and induce regulatory T cells

Mesoblast's pipeline in key therapeutic areas



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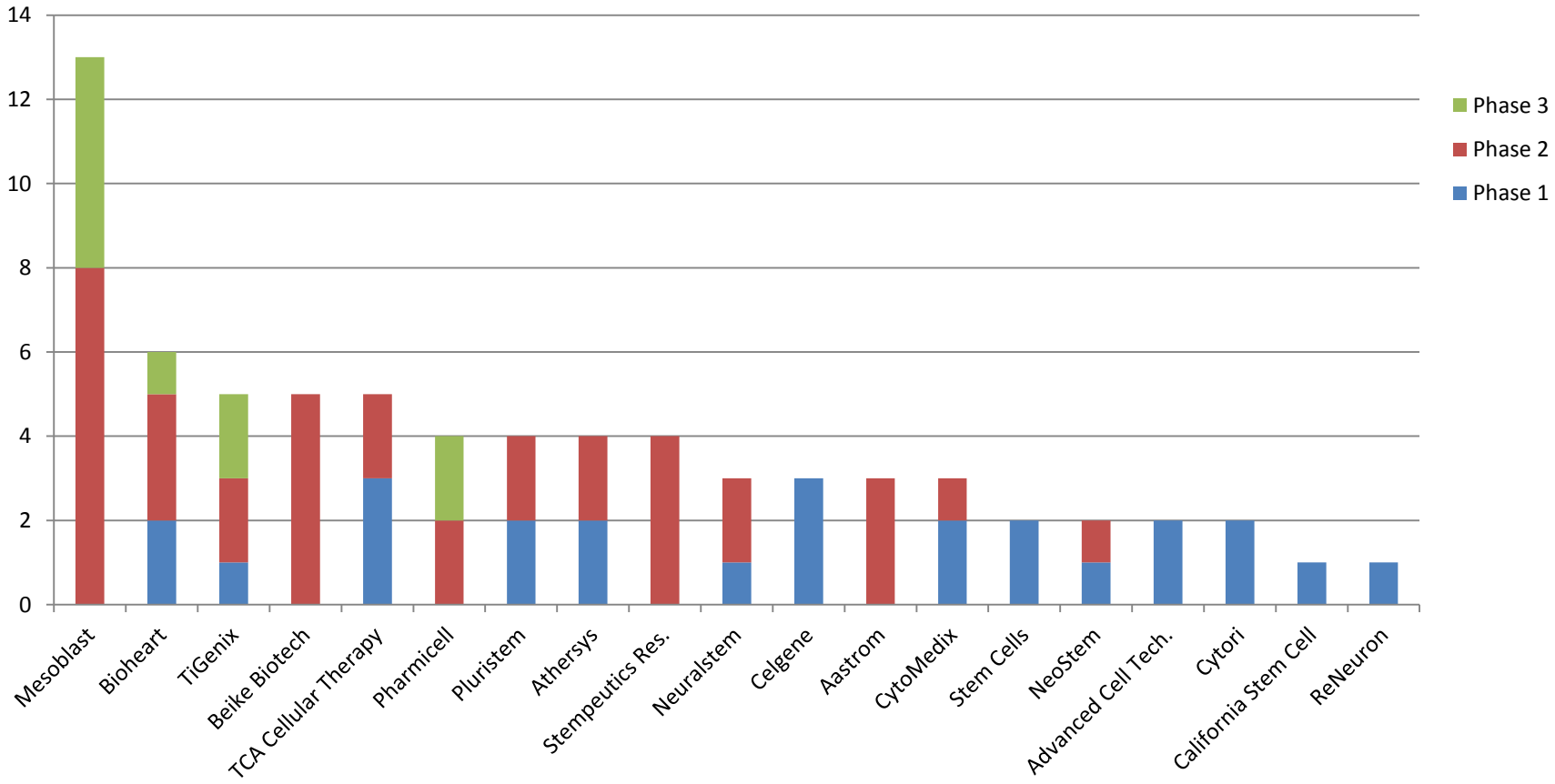
How is the regenerative medicine sector performing?

A sample of company sponsored stem cell products in clinical trials 2014



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Leveraging strengths

- Probability of success enhanced by:
  - proprietary technology offering unique scientific and clinical advantages
  - strong IP position
  - products specifically targeting major medical conditions
  - strong cash position enabling simultaneous product development where multiple products developed in parallel to increase probability of success
  - strategic partnerships providing additional sources of funding and expertise
  - manufacturing operations which are profitable and aligned to the commercial strategy

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Leading the development of adult stem cell therapies