Surrogate endpoints in clinical trials of regenerative therapy

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Popular endpoints

• Contractile function
  - Global
  - Regional

• Vascular function
  - Perfusion

• Metabolic function
  - Glucose uptake

• Structural/morphological
  - Viable tissue vs scar
  - Chamber volumes
Considerations in assessing endpoints

• Predictive value for mortality and morbidity
• Insights into mechanism of action and/or pathophysiology
• Utility based on experience with drugs and devices

regeneration n. “Regrowth of lost or destroyed parts or organs.”


Criteria for regeneration

• growth of new healthy tissue
• improvement of function in regrown area
## Popular endpoints

<table>
<thead>
<tr>
<th>Functional Category</th>
<th>Predictive Value/MOA Insight</th>
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Emerging insights

• Relevant morphological and functional endpoints required to establish therapeutic regeneration
• “Regeneration” used loosely to date; some cells may work clinically without inducing regeneration
• Predictive utility in regenerative applications not yet established; new guiding principles needed
• If regeneration improves clinical outcomes, then endpoints indexing regeneration may deserve particular attention going forward