Challenges to Cellular Therapy Development

Cell Therapy Liaison Meeting
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Scientific

- Poor understanding of MOA
- Immunological barriers
- Formulation
  - » Cryopreservation science
- Non-predictive animal models
- Limited in vitro models
- Poor understanding of differentiation pathways

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Manufacturing/Quality

- Ancillary materials
- Characterization
  - Potency
  - Comparability
  - Stability
  - Reference standards
- Scale up/out
- Manufacturing site changes
- Process controls
- Product distribution
- Quality assurance at clinical sites

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Clinical

- Pharmacovigilance
- Phase 2
  - » Limited surrogate endpoints predictive of efficacy
  - » Imprecise dosing
- Pivotal
  - » Poor/no controls
  - » Underpowered studies
- Length of follow up

"Bummer of a birthmark, Hal."

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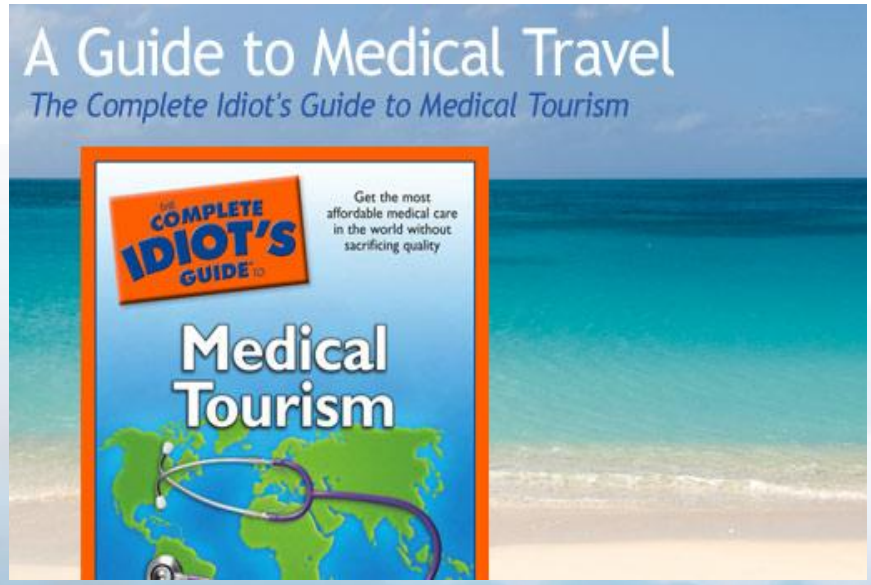
Policy

- Unrealistic public expectations
- Challenges to legitimacy
  - » Unethical medical tourism
- Regulatory
  - » Limited global harmonization
  - » Limited compendial standards



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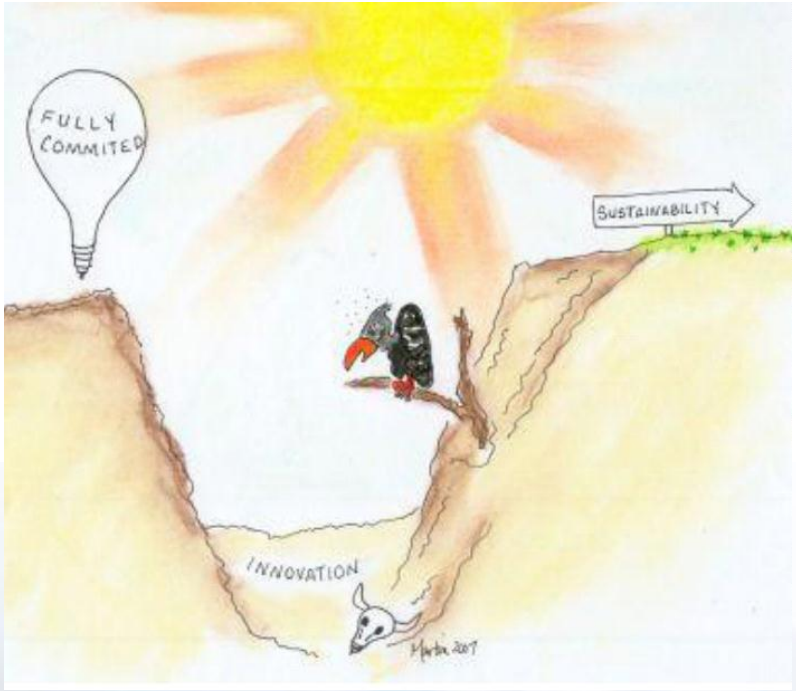
Financial/Business Models

- Access to capital (valley of death)
  - » Private
  - » Public
- High cost of goods
- Reimbursement
- Limited partnering opportunities
- Unconventional business models
  - » Autologous/patient specific



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Reasons for Optimism

- Unmet medical needs
  - » Population aging
- Proof of principle with HSCs
- Marketed cell therapy products
- Regulatory infrastructure in US and Europe
- Recent deals
  - » Genzyme/Osiris; Cephalon/Mesoblast/Teva; Pfizer/Athersys; Shire/ABH; Terumo/CaridianBCT/Harvest

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