



December 12, 2016



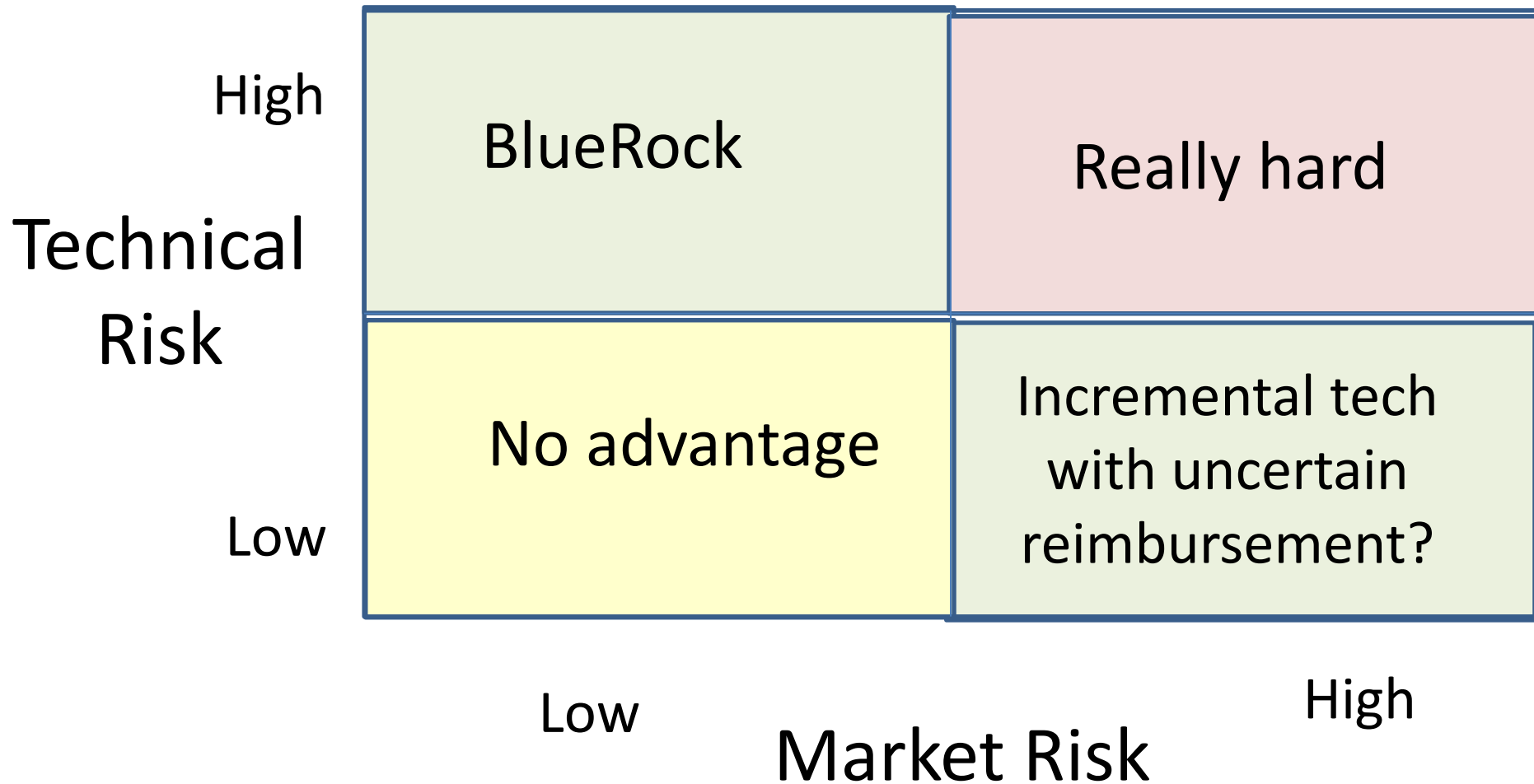
**Bayer & Versant Ventures
Join Forces to Launch Stem Cell Therapy
Company BlueRock Therapeutics with
US\$225 Million Series A Financing**



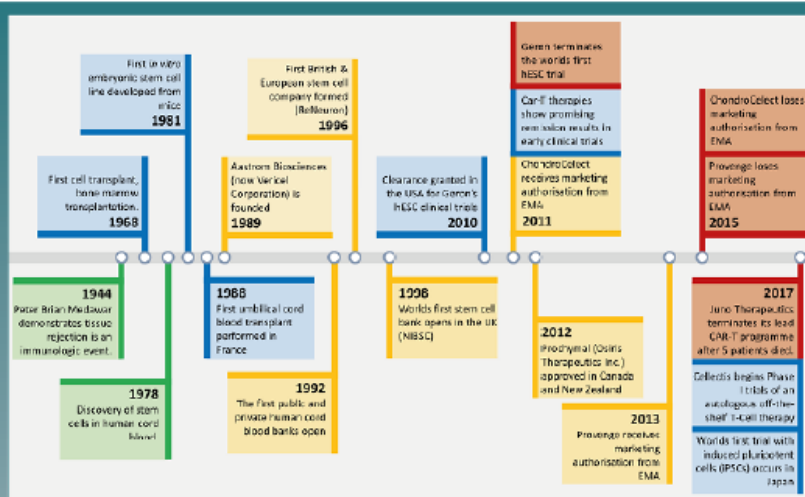
BlueRock
Therapeutics

Why was BlueRock able to attract this funding?

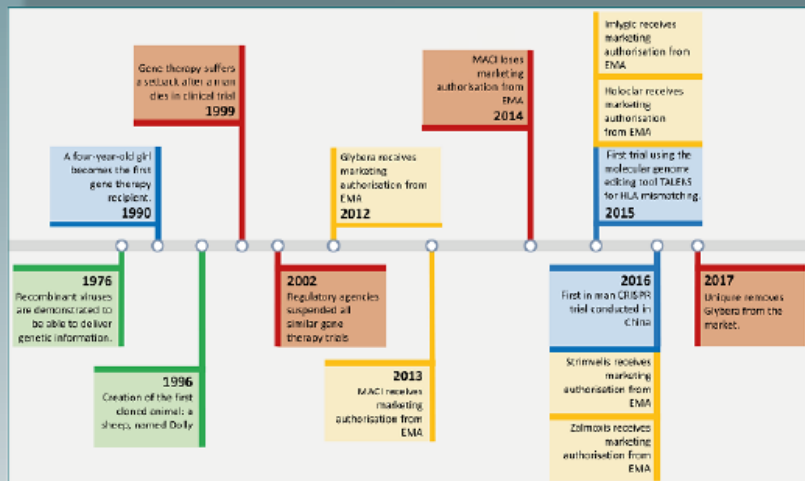
Market vs. Technical Risk



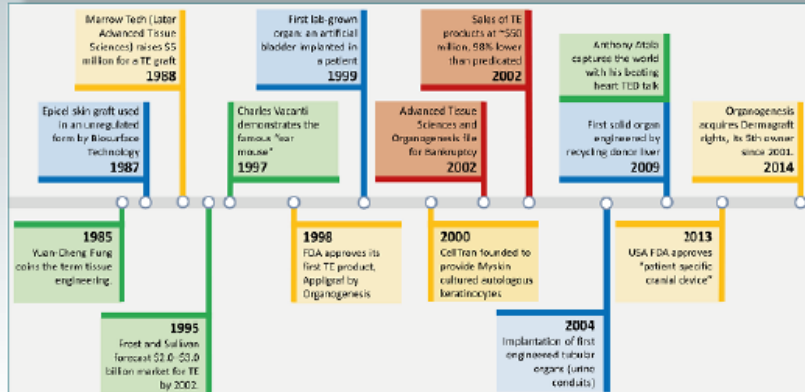
Cell therapy



Gene therapy



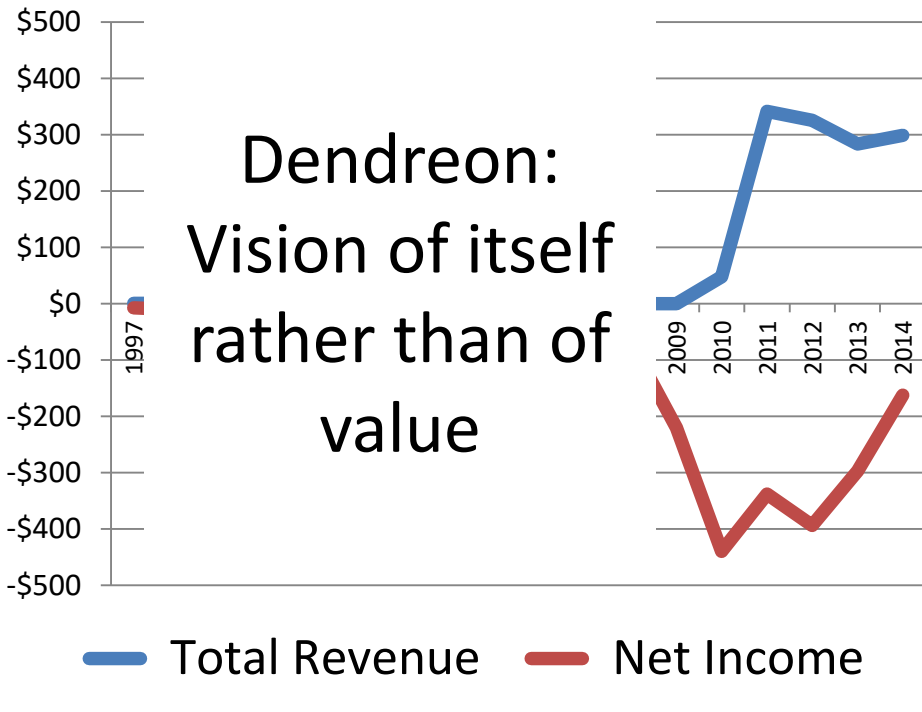
Tissue engineering



Harrison, Gracias, Mitchell (2018)

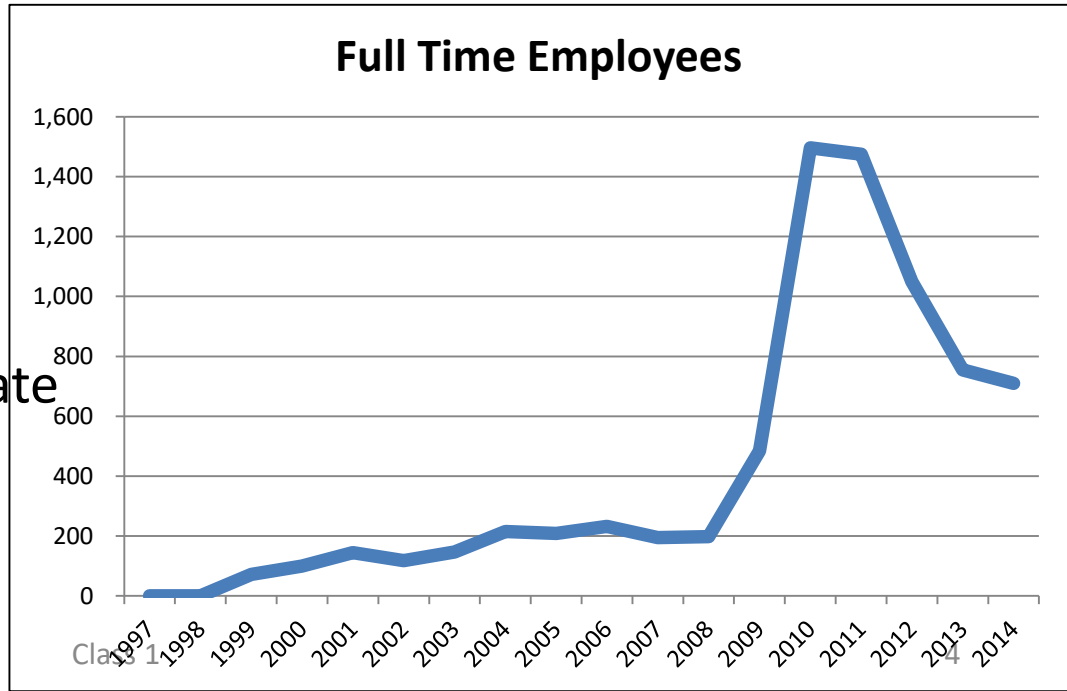
Translating Regenerative Medicine Science into Clinical Practice: The Local to Global Pivot (Richard Harrison, Aileen Gracias, Will Mitchell); Cell and Gene Therapy Insights, 2018. DOI: 10.18609/cgti.2018.043 Yy to Cell and Gene Therapy Insights 4/2018; conditional accept 5/2018; accept 5/2018.

- From RM science to clinical & market impact: **Really slow**
- Why slow?
 - Scientific challenges
 - Complementary technologies & services
 - Commercial mis-steps



Dendreon (kept Provenge) v. Vertex
(licensed non-US Incivek hep C to J&J)

- ## Dendreon: How to bankrupt a pharma company
- **Founded 1992** (Seattle), IPO ~2000, later secondary offers
 - **Main product:** Provenge (Prostate cancer immunotherapy)
 - **Chapter 11:** 12/2014 (2015: assets acquired by Valeant)



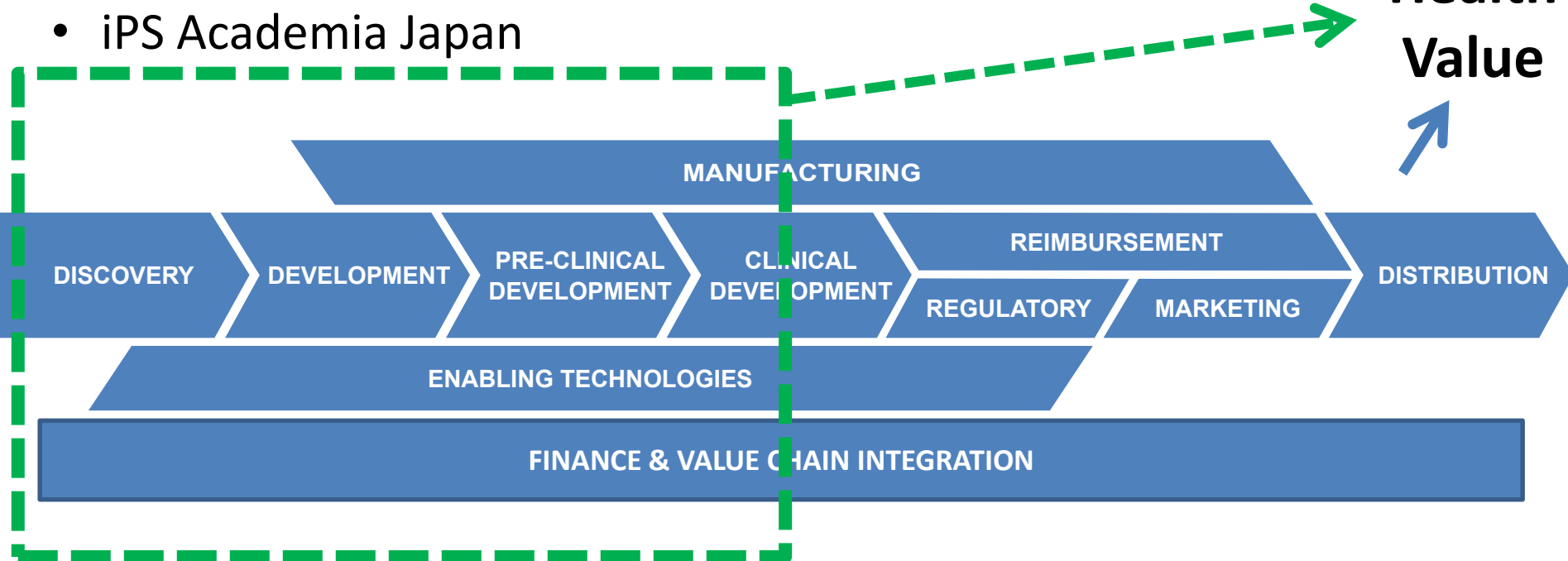
BlueRock in the Regenerative Medicine Value Chain

Discovery & development

- UHN
- Memorial Sloan Kettering
- iPS Academia Japan

- **Production: CCRM**
(value chain partner)

Health Value



Finance & value chain integration

- Bayer
- Versant

Regional ecosystems

- Toronto
- New York



Ex vivo lentiviral gene therapies for rare diseases (UHN spin-out, founded 2015; \$25 million Series A financing in 2016)

Disease	Proof-of-Concept	IND-Enabling	Phase 1/2	Pivotal	Worldwide Rights for AVROBIO
Fabry AVR-RD-01			3 patients		
Gaucher AVR-RD-02					
Pompe AVR-RD-03					
Cystinosis AVR-RD-04					



From Toronto to Massachusetts, with links back to Toronto

Cambridge, MA

One Kendall Square,
Building 300,
Suite 201
Cambridge, MA 02139

JLABS @ Toronto

MaRS Centre, West Tower
661 University Ave,
Suite 1300
Toronto, Ontario M5G 0B7

Key Components of Venture Creation

Which of these is most important?

- **Demand side:** Customer value, market opportunity, competitors
- **Team:** Founders & network
- **Supply side:** Science, product/platform, IP status
- **Ecosystem:** Value chain -- inputs & potential exits

Components of a Venture

-
- A**
 - 1. **Demand side:** Market opportunity, customer value, competitors
 - 2. **Team:** Founders & network
 - B**
 - 3. **Supply side:** Science, product/platform, IP status
 - 4. **Ecosystem:** Value chain -- inputs & potential exits

Value chain integrator & VC partners



BlueRock
Therapeutics

Versant time line

- *2015*: Scan environment
- *2016*: Negotiations & contracts

- **Demand**: Cardiac & Parkinson's are blockbuster markets, limited current competition
- **Team**
 - Science dream teams: Studer, Keller, Laflamme
 - Leadership team: **TBD (Key: Versant & local networks)**
- **Supply side**
 - Science: Global reputation
 - Product: Parkinson's first to clinical trials, then cardiac, potential platform
 - IP status: Viable via multiple licenses
- **Ecosystem**: Local & global value chain
 - Bayer: Strategic investor
 - Production: CCRM / GE (**commercial scale up?**)
 - Clinical trials: UHN, MSK
 - Regulatory: Supportive environments
 - Reimbursement/market: **Uncertain**



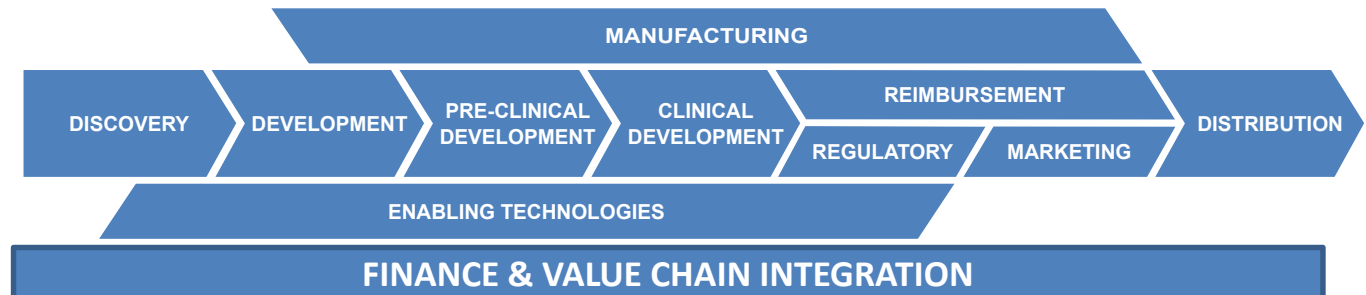
Initial Leadership Team: 2017

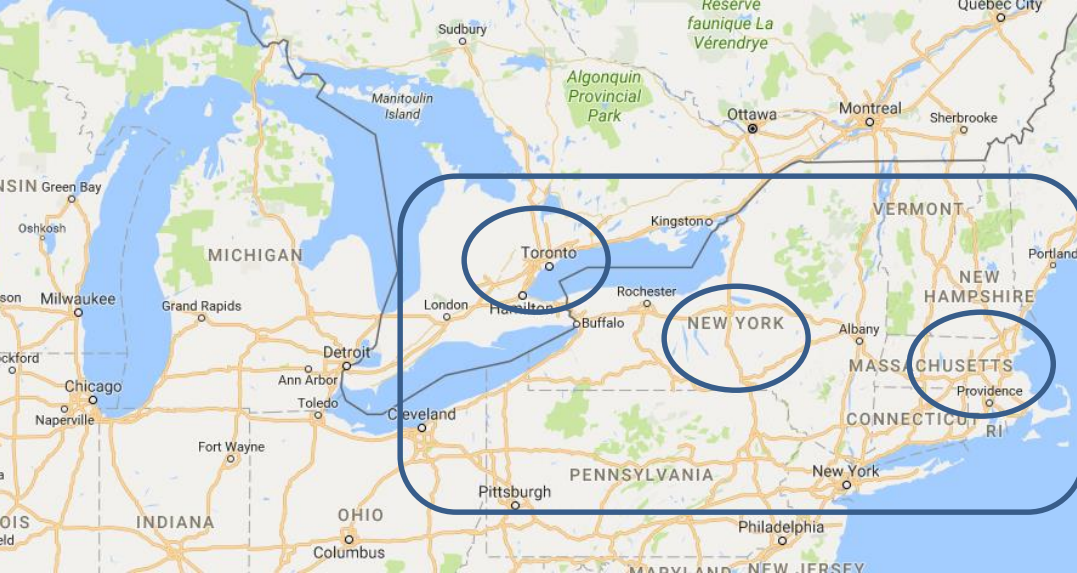
<https://www.linkedin.com/search/results/people/?facetCurrentCompany=%5B%2216175275%22%5D>

- **Boston**
 - Emile Nuwaysir, CEO (multiple life sciences)
 - Robert Deans, CTO (multiple life sciences)
 - Tracey Lodie, SVP Translational immunology (Syros, Genzyme)
 - Andrea Brener, HR consultant (life sciences)
- **California**
 - Michael Scott, SVP Product Dev. (ViaCyte)
 - Graham Walmsley, Board observer (Versant)
- **New York:** Eric Soller, VP Strategy (Versant)
- **Germany:** Jurg Eckhardt, Board member (Bayer)
- **Toronto**
 - Jennifer Moody: Sr. Director R&D (CCRM)
 - Nicke Timmins, VP Process Sciences (CCRM)
 - Ainslie Little: Director of IP (Torys LLP)
 - Sherif Sheta, CIO advisor (Bayshore Health)
 - Anne Koehler, Lab manager (U of T)
 - Kamar Green, Office manager (Tornado Spectral)

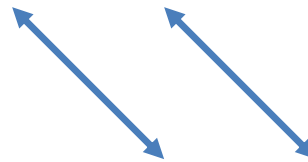
Lessons to Date

1. Urgency/pace
2. Critical: Value chain integrator
 - Scale
 - Scope & complexity
 - Logistics
3. Multiple ecosystems: Toronto, NY, ...





Regional & Global Ecosystem

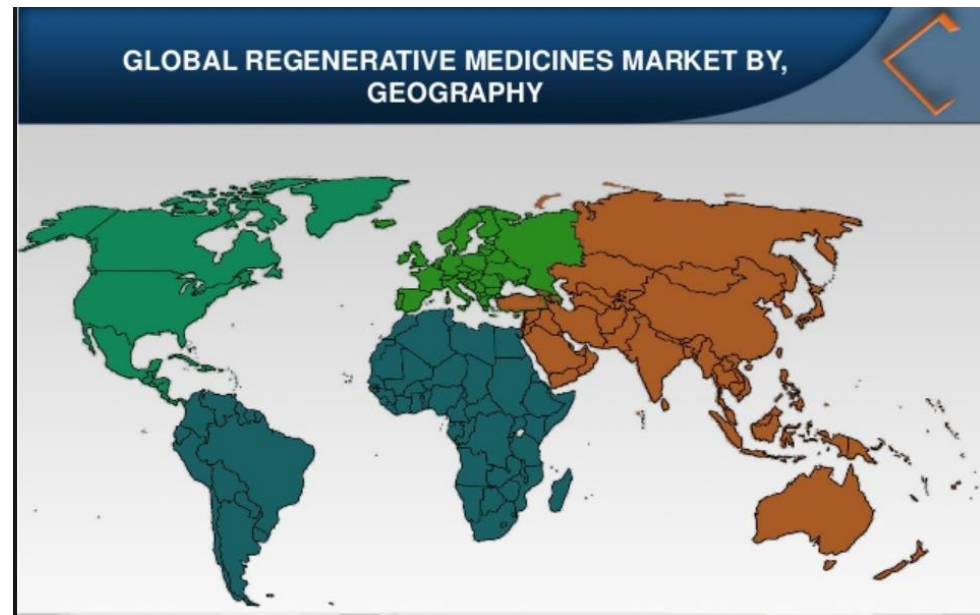


World Map



Regenerative Medicine Global Challenges

(Harrison, Gracias, Mitchell, 2018)



- Supply side challenges
 - Academic institutions
 - Funding gaps
 - Intellectual property
 - Commercial challenges
- Demand side challenges
 - Regulatory policy
 - Reimbursement & health technology assessment
 - Stakeholder engagement
 - Patient engagement

- Opportunities
 - Leverage regional expertise
 - Tap global infrastructure
 - Global logistics
 - International standards vs. differentiated advantage

Next Steps for Blue Rock?



- **Science:** What cells? What indications? Trials?
- **Operations**
 - Scale from lab to clinical trials?
 - Coordinate Parkinson's & cardiac programs?
 - Technology transfer among multiple partners?
 - Manage multiple regulatory environments?
- **Leadership**
 - Creating unified company culture?
 - Balance founders' scientific needs with commercial activity?
- **Multi-party governance**
 - Govern across multiple partners?
 - Lead integrators?
 - Board: Membership, responsibilities, committees, leaders?
 - Build trusted relationships to facilitate venture evolution?
- **Vision:** Evolution from scientific translation to market
- **Company future:** IPO, out-licensing, acquisition?