How industry, NIH, academia can collaborate to facilitate cellular therapy trials

Richard T Maziarz, MD
Oregon Health & Science University
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Personal History & Observations

- Pre-IND discussions with the FDA
  - Examples: immune reconstitution
- ISCT: Board participation and observations
- New ISCT strategic plan initiated
  - Mission statement: ISCT as a translational society is focused on EDUCATION & SERVICE
- ISCT meeting 2009
- Everyone wants platforms for dialogue and communication
ISCT Mission Statement

ISCT is a global association driving the translation of scientific research to deliver innovative cellular therapies to patients.

- Fosters international translational research
- Informs national and global regulatory framework development and harmonization
- Drives commercialization strategies
- Educates principal investigators, lab directors, technologists, regulators and commercial stakeholders
- Specifically focused on pre-clinical and translational aspects of developing cell therapy products
- As a consequence → ISCT helps the academic, government and biotech/pharma sectors transform research into practice and product.
2010 Membership Demographics

- USA: 59%
- Asia: 5%
- Australia: 7%
- Canada: 6%
- Europe: 19%
- Mexico: 1%
- Middle East and Africa: 1%
- South America: 2%

Bar chart showing:
- Regulatory: 8%
- Industry: 15%
- Research: 20%
- Laboratory: 24%
- Clinical: 32%
Fostering Translational Research

• Scientific cell and therapeutic committees
• Standards development; cell characterization, potency
• International and Regional educational events, including in the US, liaison meetings with the FDA since 2005
• Cytotherapy – the official journal of the ISCT
• Accomplishments: CD34, FACT, FDA guidelines, MSCs
Hypothesis: ISCT can drive Commercialization Strategies in partnership with Industry

- ISCT has the development experience and knowledge through its membership base to develop consensus on scientific standards and policy, and coordinate solutions to commercialization roadblocks to significantly advance the field of regenerative medicine.

- Therapeutic Areas
  - Risk/benefit analysis against current standard of care
  - Clinical endpoints for success

- Stem and Progenitor Cells
  - Standards of cell characterization for Regulatory agencies and physician selection

- Regulatory
  - Global patient safety
  - Clinical development strategies

- Industry
  - Development experience
  - Capital support of approval trials
2010: A New Vision for Partnership

ISCT Industry Task Force
Summary of Recommendations
Industry Task Force Objectives

1. Gather information and assess value and prioritize options

2. Define expectations and value in the relationship between industry, ISCT, and external societies

3. Evaluate the structure of ISCT sub-committees with recommendation for industry participation and function

4. An action plan and benchmarks to assess policy implementation

5. Create an appropriate role for industry input to the ISCT Executive Team
Feedback from Polling

• Education: cell characterization, cell processing and manufacturing standards

• Strong mandate to work with ISCT Cell and Therapeutic Area committees and identify commercialization bottlenecks and solutions

• Access to key opinion leaders (KOL) in therapeutic disease societies and stem cell basic sciences for open forum discussion of commercialization strategies
Feedback

• Priority
  – Clinical trial design in emerging therapeutic areas
  – Open dialogue around endpoints and dosing decisions

• ISCT can best engage industry (outside its membership roster) with:
  – Focused industry workshops at therapeutic disease societies
  – Hosting global workshops on emerging translational therapies
  – Annual meetings and webinars as platforms of choice for international dialogue
  – Cataloging global regulatory guidelines for cell therapy
Recommendations

• Re-charter Commercialization Committee and strengthen relationship to ISCT committees

• Build stronger relationship between industry and ISCT through Industry Community

• To identify an appropriate role for industry participation on the ISCT executive team
Centralizing Perspectives

Therapeutic Area Committee

1. Open development processes and education to clinical partners and regulators
2. Scalable and economic practices sufficient to meet payer expectations and gain acceptance as SOC

Commercialization Committee

Stem Cell Committee
## CC Subcommittees

<table>
<thead>
<tr>
<th>Process and Product Development Technology</th>
<th>Business Models, Reimbursement and COGS</th>
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<tbody>
<tr>
<td><em>To assess emerging cell processing technologies and forecast their impact on the commercialization process</em></td>
<td><em>To define economic aspects of manufacturing clinical cell products and tools influencing reimbursement and acceptance as standard of care</em></td>
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<th>Industry Education</th>
<th>Clinical Development and New Product Introduction</th>
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<tr>
<td><em>To construct educational platforms that drive commercialization objectives with external societies and industry community</em></td>
<td><em>To address unmet patient needs by connecting industry, academia, and global regulatory agencies</em></td>
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ISCT has launched a sponsorship based participation network for industry to:

- Provide a valuable peer to peer industry network.
- Offer access to key opinion leaders in cell therapy translation including regulatory, scientific, process development and clinical experts.
- Drive priorities on the development of resources and initiatives to remove key obstacles to commercialization.
- Members include:
  - large pharma, early phase Biotech, CMOs & services, academia and technologists, investment and regulatory consultants.
Use the society to create platforms and do it globally
Plans- Year 1

- Aggressive schedule for joint academic: industry educational forums (IBD, GVHD, COGS)
- Webinar re: manufacturing issues
- Industry forum in Cytotherapy
- Schedule one year Retro review- July 2011
Where are we today?
Landscape of cell therapy

- ASBMT, AABB, NMDP, FACT, ISCT, ISEH, ISSCR, ISCGT, AHA, ATS, JDRF, ASH
- Business groups: BIT, Terrapin, etc
- ICMS: >1000 members; goal??
- ARM- <2 yrs old
Definition of Regenerative Medicine

“A rapidly evolving interdisciplinary field that translates fundamental knowledge in biology, chemistry and physics into materials, devices, systems and therapeutic strategies, including cell-based therapies, which augment, repair, replace or regenerate organs and tissues.”

- Alliance for Regenerative Medicine
Alliance for Regenerative Medicine

Mission:
Advance regenerative medicine by representing and supporting the community of companies, academic research institutions, patient advocacy groups, foundations, and other organizations before the Congress, federal agencies and the general public.

History: Founded in 2009, 55+ members and growing

ARM Members:
• Companies
• Research Institutions
• Foundations
• Investors
• Patient Advocacy Groups
• Non-profits
# Alliance Members

## Foundations/Associations (7)
- Alliance for Aging Research
- Association of Clinical Research Organizations (ACRO)
- Foundation for Regenerative Medicine (Founding Member)
- Genetics Policy Institute
- Juvenile Diabetes Research Foundation
- New York Stem Cell Foundation
- Pittsburgh Tissue Engineering Initiative

## Research Institutions (6)
- Burnham Institute for Medical Research
- California Institute for Regenerative Medicine
- The Cleveland Clinic
- University of Louisville & Jewish Hospital Cardiovascular Innovation Institute
- University College London
- Wake Forest Institute for Regenerative Medicine

## Investors (4)
- Kleiner, Perkins, Caufield, Byers
- Proteus Venture Partners
- Safeguard Scientific
- Toucan Capital

## Companies (31)
- Aastrom Biosciences
- Advanced BioHealing
- Aldagen
- Allosource
- ATCC® The Global Bioresource Center™
- Athersys
- Celgene Cellular Therapeutics
- Cellerant Therapeutics
- Corning
- Cytori
- GE Healthcare
- Geron
- Fate Therapeutics
- International Stem Cell Corporation
- iPierian
- ISTO Technologies, Inc.
- Johnson & Johnson
- MaxCyte
- Millipore
- Organogenesis
- Organovo
- Roji Pharma
- Pervasis Therapeutics Inc.
- Pfizer Inc
- Regenesis Biomedical
- StemCells Inc.
- Theregen
- Tengion
- Tissue Genesis
- ViaCyte (formerly Novocell)
ARM Accomplishments

- The Regenerative Medicine Promotion Act
  - First of its kind legislation designed specifically to accelerate the commercialization of RM products and increase translational research in RM

- Engaged with key federal agencies (FDA, NIH, CMS)

- ARM International Regenerative Medicine Fly-In Day
  - First international advocacy effort for RM with participants in the U.S. and UK, September 21-22, 2010

- Development of economic data on the industry
  - Complete review of available economic data on the impact of RM
Why Advocacy?

Case study: *ES research funding in jeopardy*

- 08/23: Federal District Judge Royce Lamberth blocked President Obama’s 2009 executive order that expanded ES research because it violated a law banning federal funds for any procedure involving destruction of a human embryo.
- 08/24 Francis Collins, Director of NIH, announced that over $50 million in grants would be denied annual renewal.
- 08/26 NIH issued an order to stop all research on human ES cells.
- 09/07 Judge Lamberth rejected the government’s request to reconsider his ruling.
- 09/08 The U.S. Court of Appeals for the District of Columbia temporarily allowed federal funding to continue for research on embryonic stem cells by blocking Judge Lambeth’s ruling. This action may permit $78 million to be distributed to 44 sites.
Where do we go?