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Sabbatical leave
2000 - 2001
Pharmaceutical Industry’s View of Cell / Gene Therapies

Corporate Office of Science and Technology

J & J
$50.5B sales/2005
>200 operating companies
>50 countries
Highly decentralized
• Technology & business assessments
• Evaluate startups & academic labs
• Advisory to upper management in entering new therapeutic/business areas
• Resource for J&J’s venture capital group (JJDC)
• Cell Therapy & Regenerative Medicine Task Force
A difficult transition
For the pharmaceutical industry, cell/gene therapy is a round peg in a square hole.

Why is this relevant?
Resources of the pharmaceutical industry are needed for clinical trials & FDA approval, and if the therapy is approved, for manufacturing, marketing & distribution.
The Mind of Big Pharma

• Minimize risk
• Well defined product
• Clear path to commercialization
• No regulatory surprises
• Acceptable business models
Drug Discovery / Development Process

Drug Discovery  Preclinical  Clinical Trials  FDA Review

10,000 compounds  250 compounds  5 compounds  1 compound

~6.5 yr  ~7 yr  ~1.5 yr

- High risk, high cost but extensive past experience
- Complex but familiar regulatory hurdles
- Product clearly defined at outset of trials
- Some information on toxicity and efficacy at the outset
- Scale and manufacturing issues well understood
- Path to market is familiar
Development of New Medical Devices

- 1 prototype
- Preclinical design
- Clinical Testing
- FDA Review
- IDE application
- PMA submitted
- 1 device

If equivalent to existing device, can skip clinical testing

- Less risks than pharmaceuticals
- Short development time
- Less complex regulatory path
- Scalability not an issue
- Short product life

510K
Problematic aspects of a cell or gene therapy

- Likely to involve a combination of drugs, biologics, pharmaceuticals and devices

  Example:

  Rx of hemoglobinopathy with corrective gene transfer
  - Biologics for inducing stem cell mobilization
  - Device for recovery of stem cells
  - Corrective gene and transfer vector

  → complex & unfamiliar development process
  → complex & unfamiliar regulatory pathway
Problematic aspects of a cell or gene therapy

- **Product poorly defined**
  
  - The product is defined by the method of cell recovery and processing
  
  - Product evolution during clinical trials

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<th>Amendments</th>
<th>Indication</th>
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<tbody>
<tr>
<td>Cell therapy</td>
<td>~15/IND</td>
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<tr>
<td>Gene therapy</td>
<td>~ 20/IND</td>
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Problematic aspects of a cell or gene therapy

• **Unfamiliar business models**

  **Autologous vs Allogeneic**
  (patient specific vs cells off-the-shelf)

  **Centralized vs Distributive Processing**

  **Unwanted Service Component**
Problematic aspects of a cell or gene therapy

- Problems of scale
Problematic aspects of a cell or gene therapy

- Broad and overlapping patent claims
- Neoantigens and host immune reactions
**Evolution of platform technologies**

- **Aggregate Industry Valuation**
- **Pre-clinical and early clinical successes**
- **Clinical or commercial failure**
- **Reassessment of industry viability**
- **Successful commercialization of $500+ Million**
- **Multiple products launch**
- **mAb’s**

- **First Press Coverage**
- **Cell Therapy**
- **Gene Therapy**

- **Irrational Exuberance**
- **Reality**
- **Breakthrough**
- **Take-off**
What drives this evolution

- Maturation of the science
- Successful well-run clinical trials
- Societal pressure
- Pharmaceutical industry seeking new sources of revenue
Overcoming Obstacles

• Approach armed with good science
• Deconvolute the cell processing
• Think like an investor not like a convert