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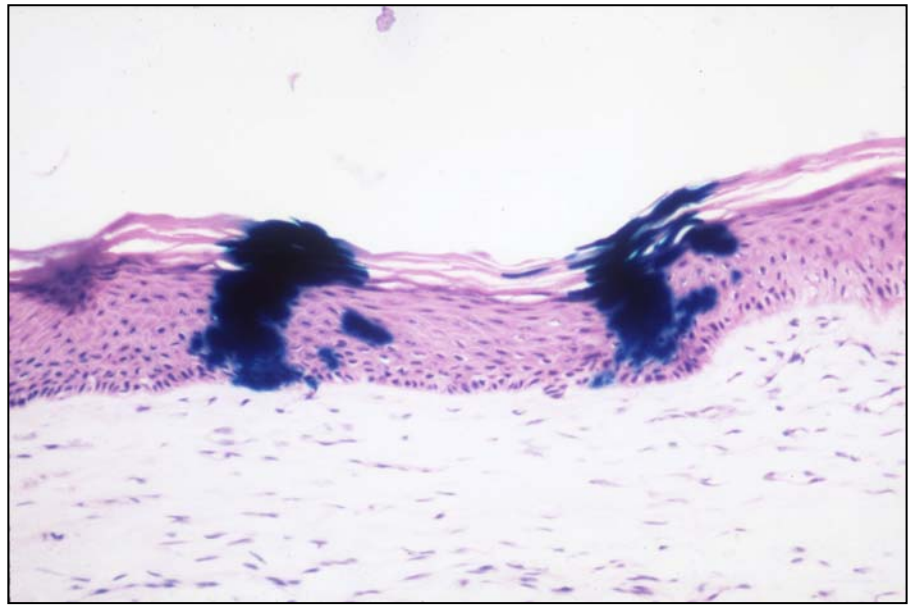


Sabbatical leave
2000 - 2001

Pharmaceutical Industry's View of Cell / Gene Therapies

Johnson & Johnson

Corporate Office of Science and Technology

COSAT

J&J

- $50.5B sales/2005
- >200 operating companies
- >50 countries
- Highly decentralized

COSAT

- 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

Personal insight

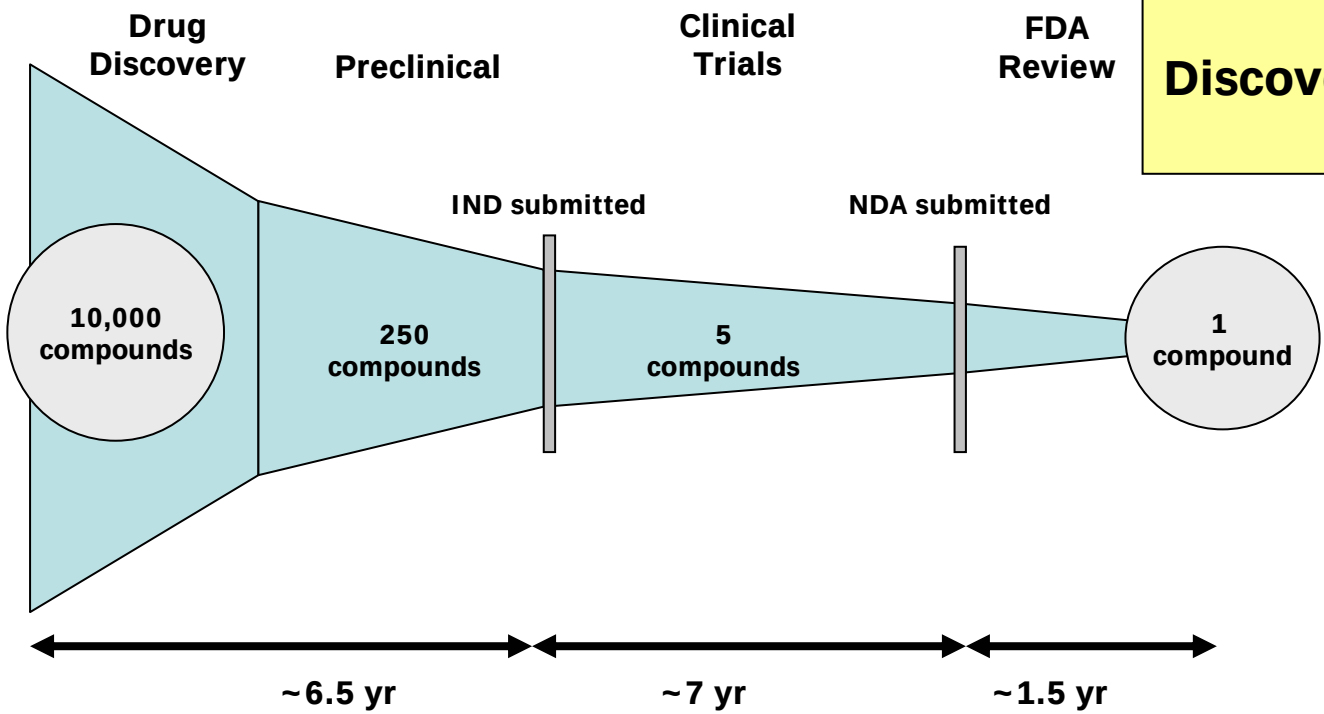
For the pharmaceutical industry,
cell/gene therapy is a round peg in
a square hole

<u>Why is this relevant ?</u>
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

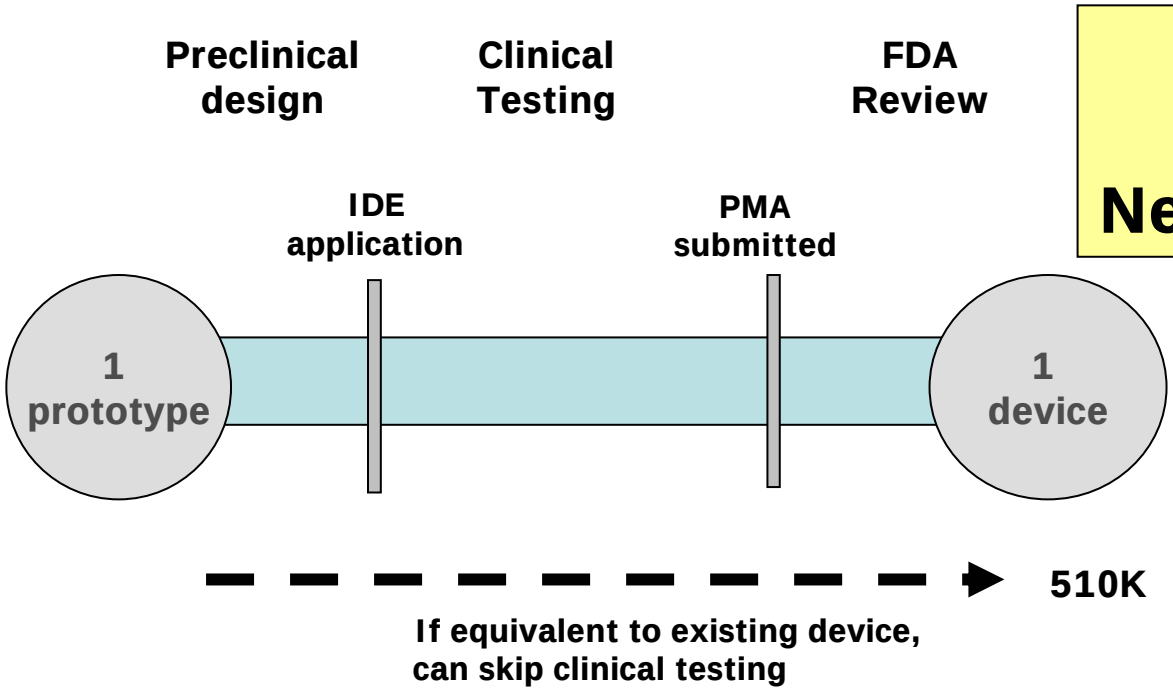
- Minimize risk
- Well defined product
- Clear path to commercialization
- No regulatory surprises
- Acceptable business models

Drug Discovery / Development Process



- 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



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

Problematic aspects of a cell or gene therapy

- **<u>Likely to involve a combination of drugs, biologics, pharmaceuticals and devices</u>**

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

- **<u>Product poorly defined</u>**

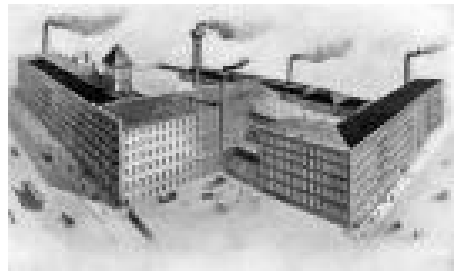
  - The product is defined by the method of cell recovery and processing

  - Product evolution during clinical trials

	<u>Amendments</u>
Cell therapy	**~15 / IND**
Gene therapy	**~ 20 / IND**

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

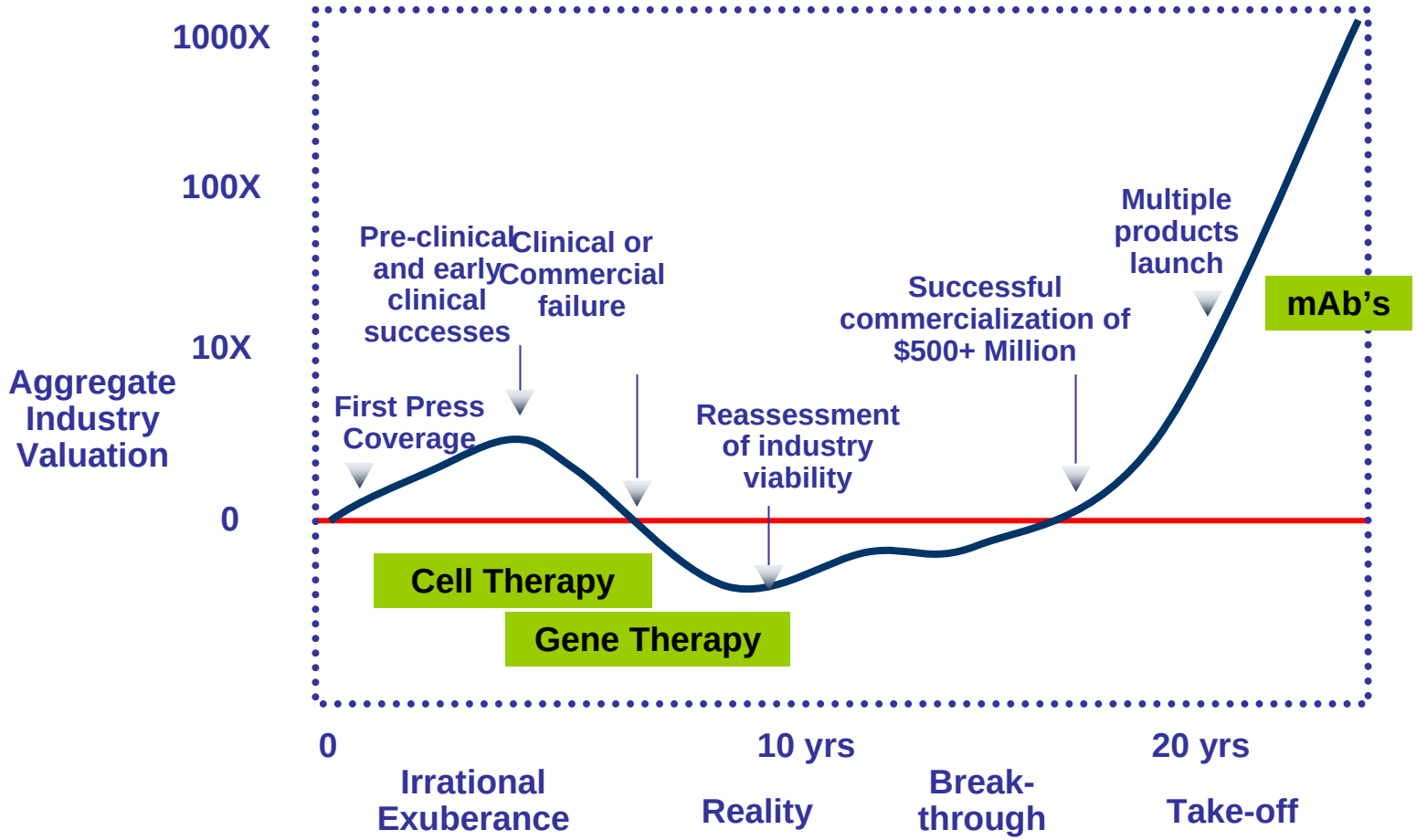
- **Problems of scale**

Problematic aspects of a cell or gene therapy

- **Broad and overlapping patent claims**

- **Neoantigens and host immune reactions**

Evolution of platform technologies



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