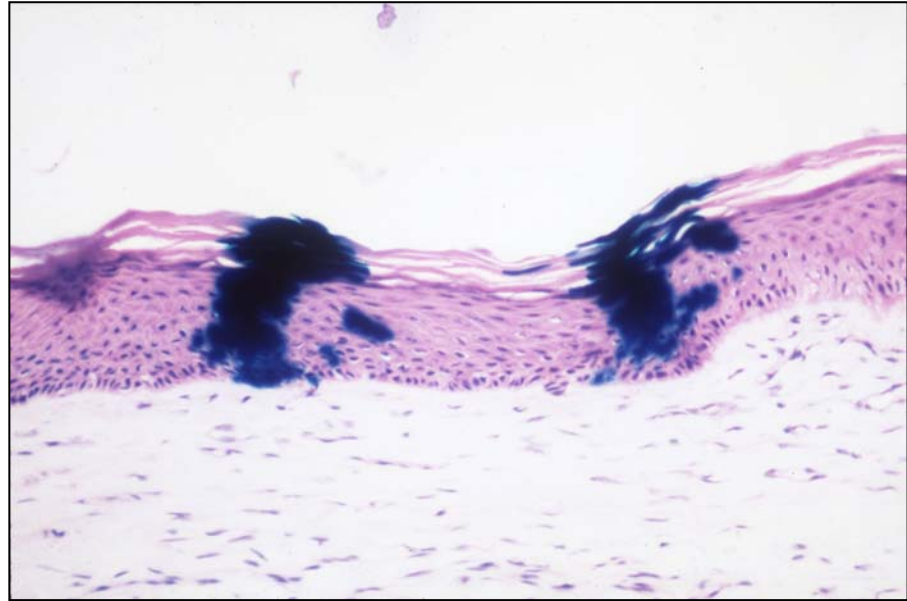


**Lorne
Taichman**



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

Johnson & Johnson





- 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

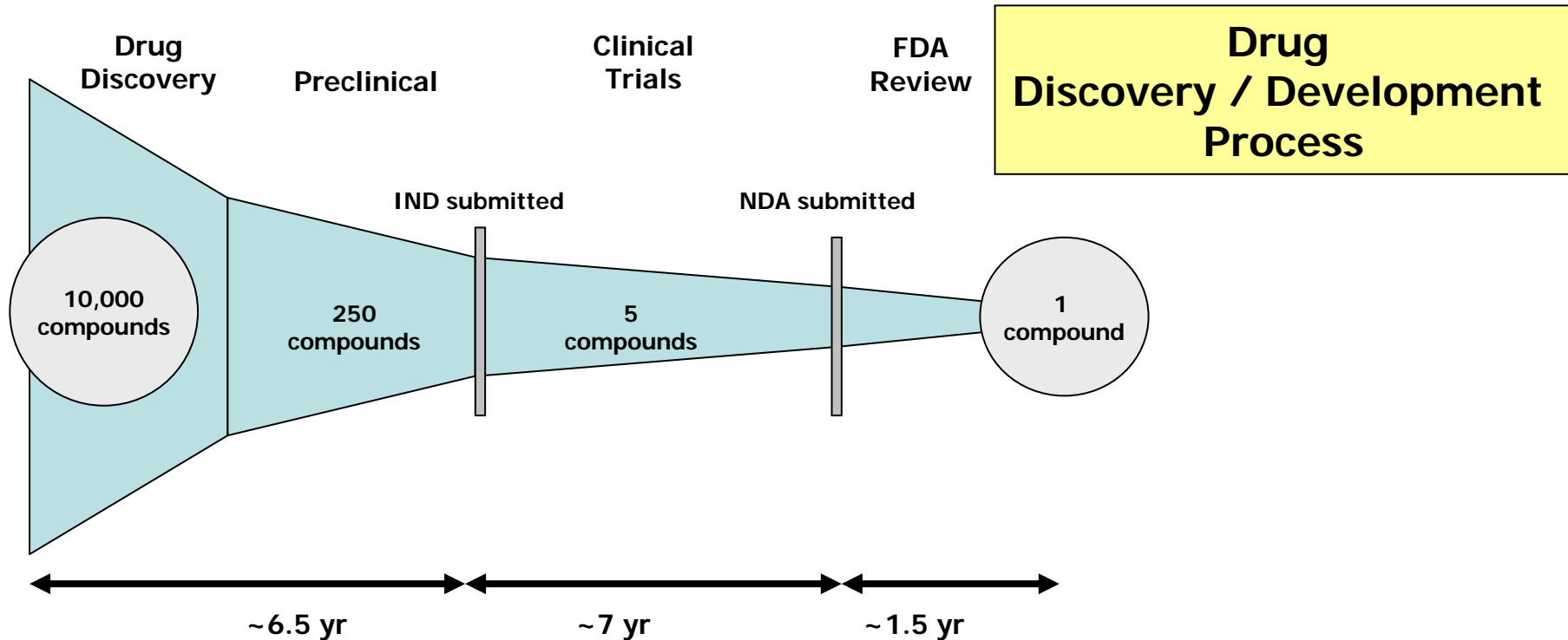
Why is this relevant ?

Resources of the pharmaceutical industry are needed for clinical trials & FDA approval, and if the therapy is approved, for manufacturing, marketing & distribution.

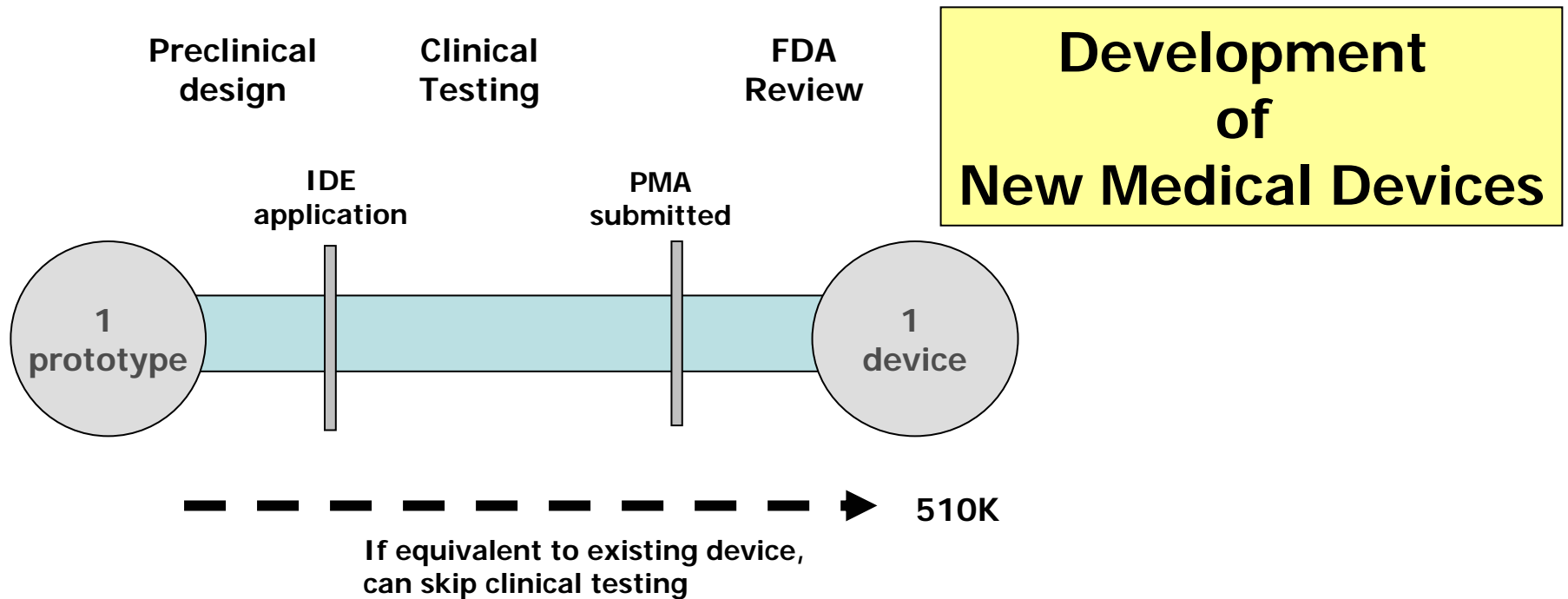


The Mind of Big Pharma

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



- 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



- **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

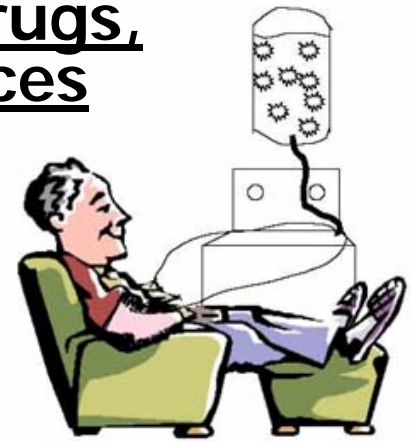
- **Likely to involve a combination of drugs, biologics, pharmaceuticals and devices**

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

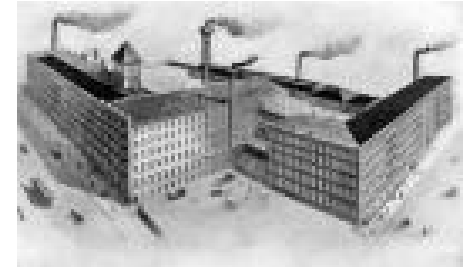
	<u>Amendments</u>
Cell therapy	~ 15/IND
Gene therapy	~ 20/IND

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

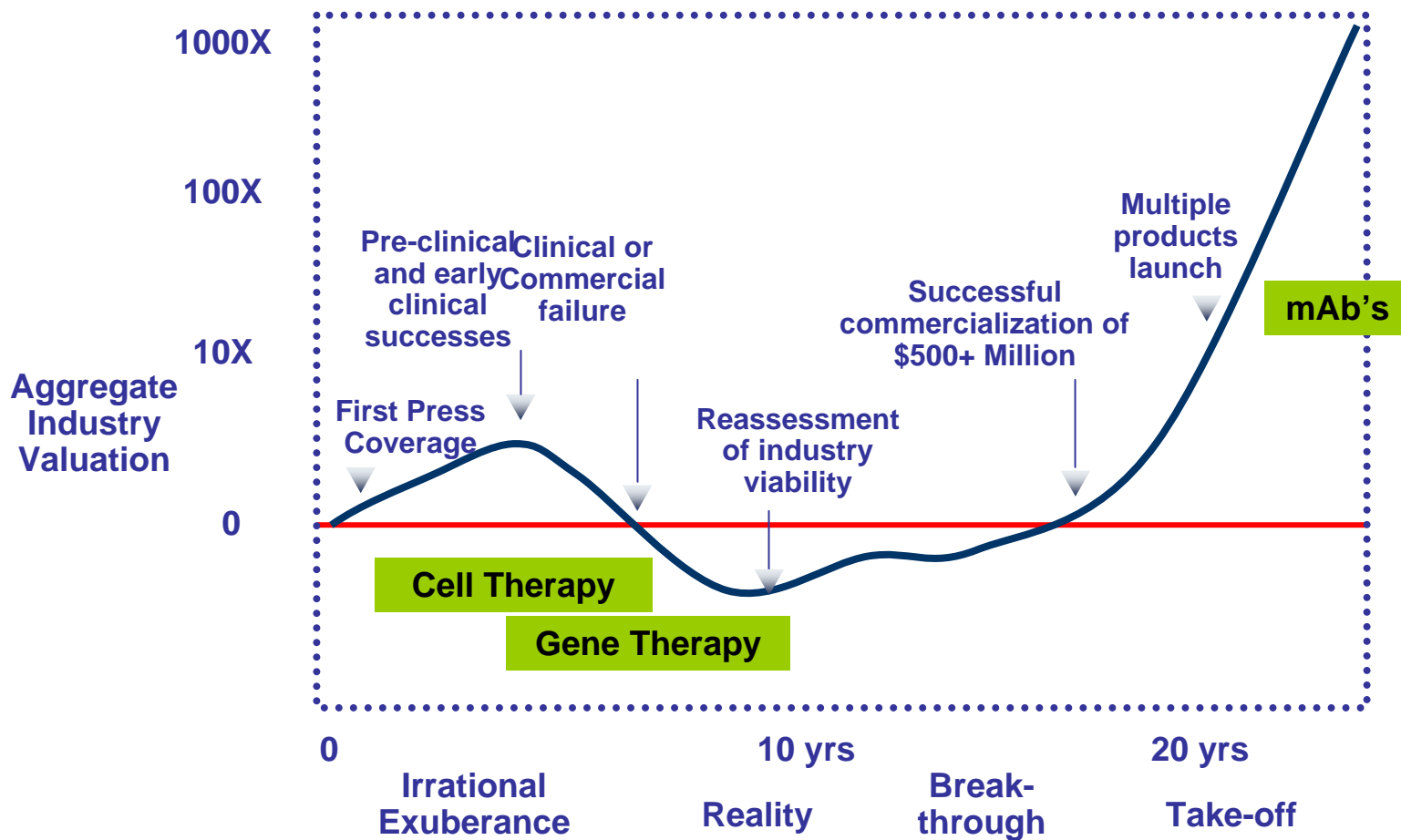
- Problems of scale



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