

# Advanced Cell & Gene Therapy

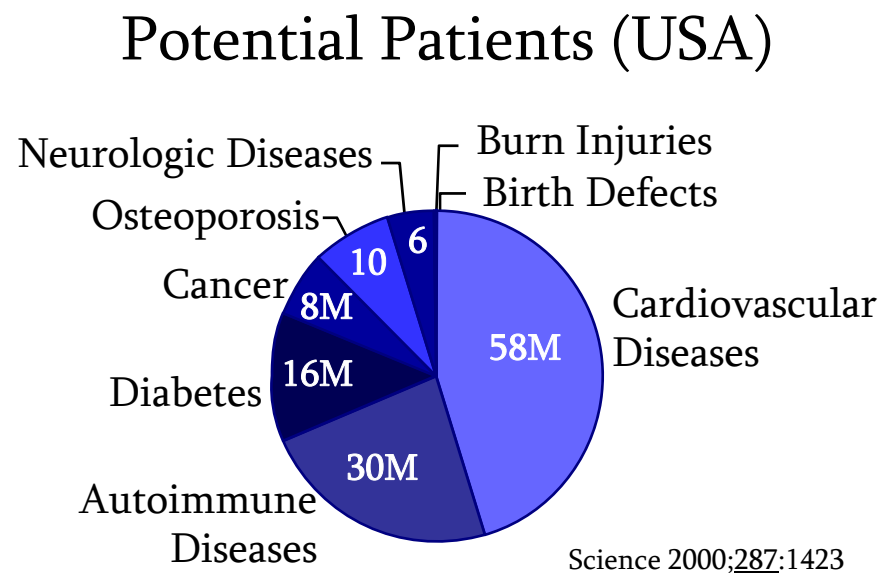
## Enabling Development and Commercialization of Cell and Gene Therapy Products

Scott R. Burger, MD

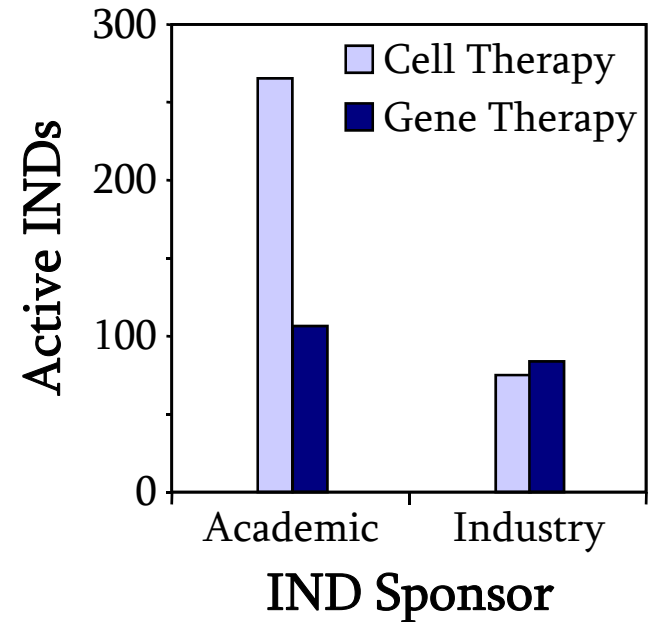
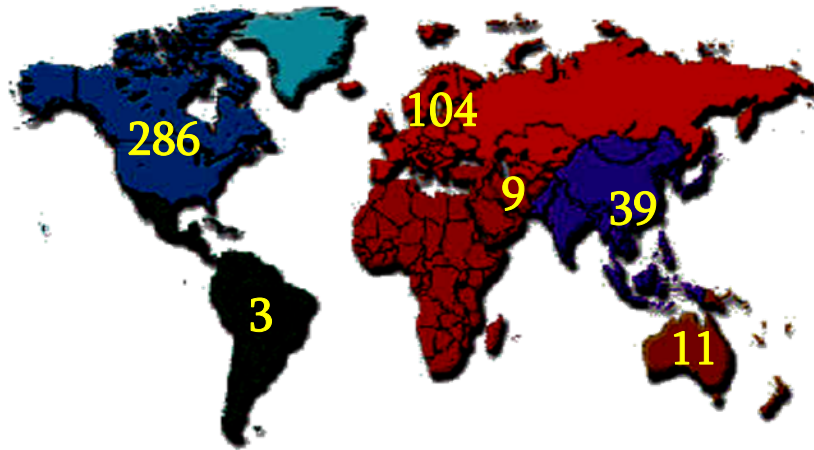
New Trends in Cell and Gene Therapy  
Industrial Technology Research Institute  
Hsinchu, Taiwan  
October 4, 2007

# Cell and Gene Therapies - Potential, Promise, and Plenty of Challenges

- Great opportunities, numerous potential applications, often for unmet needs.
- Unique challenges in development and commercialization
  - Evaluating products, IP, markets
  - Identifying potential partners, service providers
  - Commercializing individualized products
  - Regulations still evolving



# The Emerging Cell and Gene Therapy Industry



- Over 300 cell or gene therapy companies worldwide
- Over 500 cell or gene therapy products in clinical development
- Over 1,000 gene therapy clinical trials worldwide
- Industry sponsors 25% of cell therapy INDs

# Challenges of Cell-Based Therapy

## Biotechnology

## Cell Therapy

*Product*

Cultured cells generate product

Living cells *are* product

*Raw Material*

Seed cell lines

Unique, primary tissue

*Variability, Heterogeneity*

Limited

Substantial

*Product Definition*

Well-defined, definable products

Product defined through trials  
Full definition likely unattainable

*Process, Testing*

Established early

Evolve through trials

*Process Scale*

Bulk processes predominate

Patient-specific products common

# Advanced Cell & Gene Therapy, LLC

- Cell- and gene-based therapies offer unprecedented opportunities, but are complicated by unique challenges in development and commercialization.
- Advanced Cell & Gene Therapy serves biotechnology companies, clinical laboratories, and investors, leveraging extensive experience with cell, gene, and tissue therapies to provide practical, experienced guidance in key problem areas.
  - Commercialization-focused, clinical-scale process development
  - GMP/GTP manufacturing, quality assurance
  - Evolving regulatory requirements
  - Commercialization feasibility and strategy
  - Strategic partnership and investment opportunities

# Product Development

- Clinical development and commercialization strategies
- Materials and technology sourcing
- Manufacturing process analysis, optimization
  - Maximize product yield, function
  - High-throughput closed-systems
  - Enable process automation
- Product, process specifications
- Investigator and clinical site selection

# GMP/GTP Manufacturing, QA

- GMP manufacturing facility design
- GTP and GMP systems, quality programs
- Environmental control, monitoring
- Equipment selection, qualification
- Process and analytical method qualification, validation
- Laboratory operations
- Personnel training

# Regulatory Affairs

- Regulatory requirements and standards, applicability, compliance strategy
  - FDA-CBER Office of Cell, Tissues, and Gene Therapy (OCTGT), FDA-CDRH
  - EMEA, national regulatory agencies
  - NIH-RAC, AABB, AATB, Foundation for Accreditation of Cell Therapy (FACT)
- Regulatory submissions
  - Pre-IND, IND, IDE, RFD, BLA, 510K, PMA
- Audits and gap analysis

# Strategic Guidance

- Investment and partnership opportunities, identification of potential partners
- Due diligence, evaluation of technical validity, expertise, and resource sufficiency
- Market analysis

# Commercial Feasibility

- Analysis of potential market
- Pathways to regulatory approval
- Potential for high-throughput commercial manufacturing
- Potential product delivery issues, costs

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