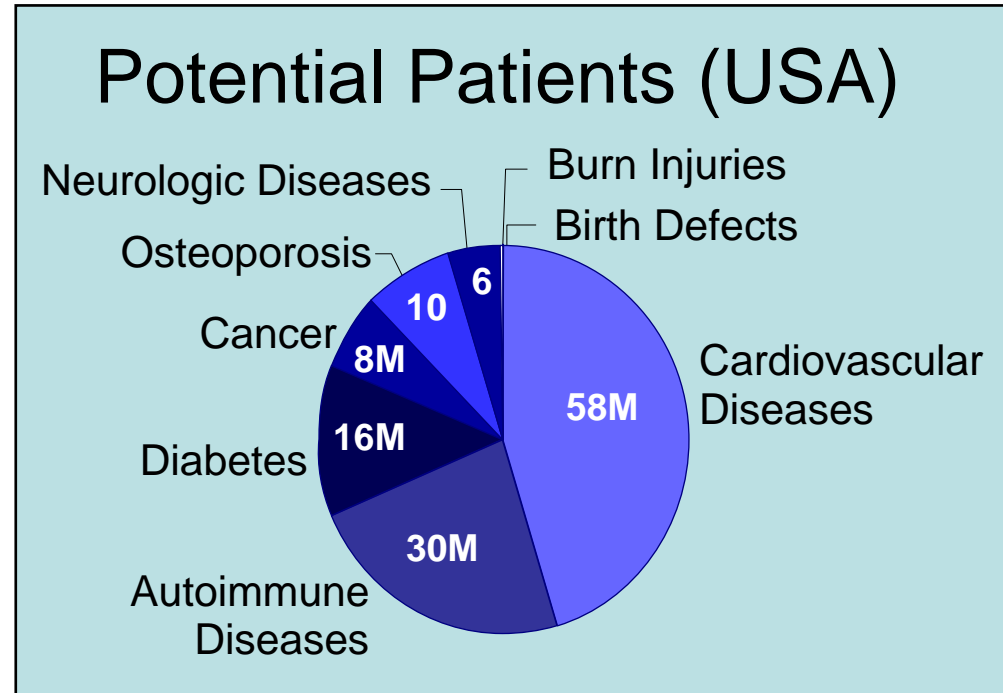


Manufacturing and Commercializing Cell Therapy Products

Scott R. Burger, MD
Advanced Cell & Gene Therapy

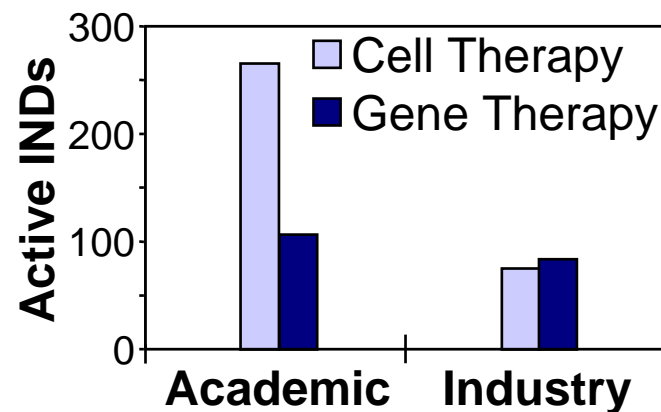
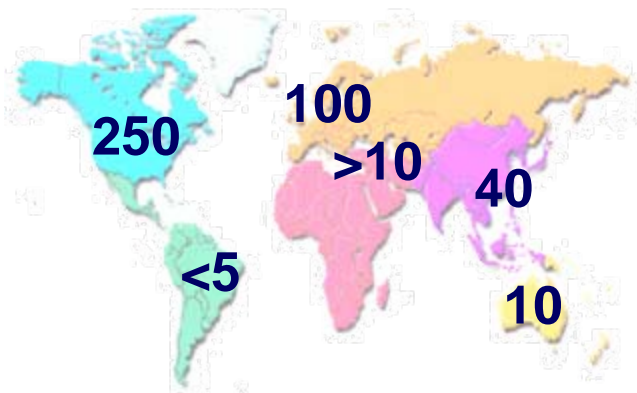
Applications and Opportunities in Cell Therapy

- Tissue repair, regeneration
- Immune, hematopoietic replacement
- Immune effector cell therapy
- Metabolic replacement, support
- Gene replacement, modification



Cell/Gene Therapy Industry, 2007

- Industry sponsoring 25% of cell therapy INDs
 - >400 cell/gene/tissue therapy companies
 - >500 cell/gene therapy INDs, >1000 gene therapy trials
- Increasing Big Pharma involvement
 - Internal ventures, external partnerships
- BLA filing (!), others expected



Manufacturing Cell Therapy Products

	<i>Biotechnology</i>	<i>Cell Therapy</i>
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

Cell Therapy Product Manufacturing Strategies

- Manufacturing process must protect product, patient
 - Focus on product characterization, process control
 - Controlled, consistent processes → controlled, consistent products
- High throughput, parallel processing achieves scale
 - Functionally-closed processing systems, automation
- Rigorous process development, characterization

Automated, Functionally-Closed Systems for Making a Nice Cup of Coffee

Individualized, cup-specific brewing of a variety of coffees, teas, cocoa, even *mochaccino with extra foam*.



Preloaded,
disposable,
individualized raw
material sets



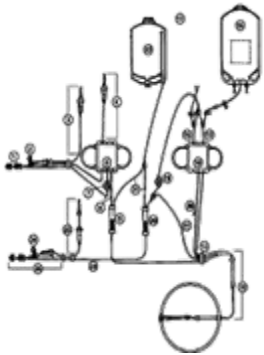
Separate
process
environment for
each product



Automated
processing
device

Automated, Functionally-Closed Systems for Manufacturing Cell Therapies

Individualized, patient-specific product manufacturing *at high throughput*



**Preloaded,
disposable,
individualized raw
material sets**



**Separate
process
environment for
each product**



**Automated
processing
devices**

Commercialization-Focused Development

- Target indications, patient population
 - Unmet/inadequately met medical need
 - Appropriate risk-benefit balance
- Product or service?
 - If product, where will service component come from?
- What happens *after* the BLA?
- Support infrastructure
 - Centralized or distributed manufacturing? Skills? Capacity?
 - Distribution, logistics and transport, tracking
- Pricing and reimbursement strategies