

Symposium on Translation of Stem Cells into Clinical Stem Cell Therapeutics

Session I

Regulatory Issues Associated With Stem Cell Therapeutics

**National Academies Human Embryonic Stem Cell Research
Advisory Committee
20 November 2008**

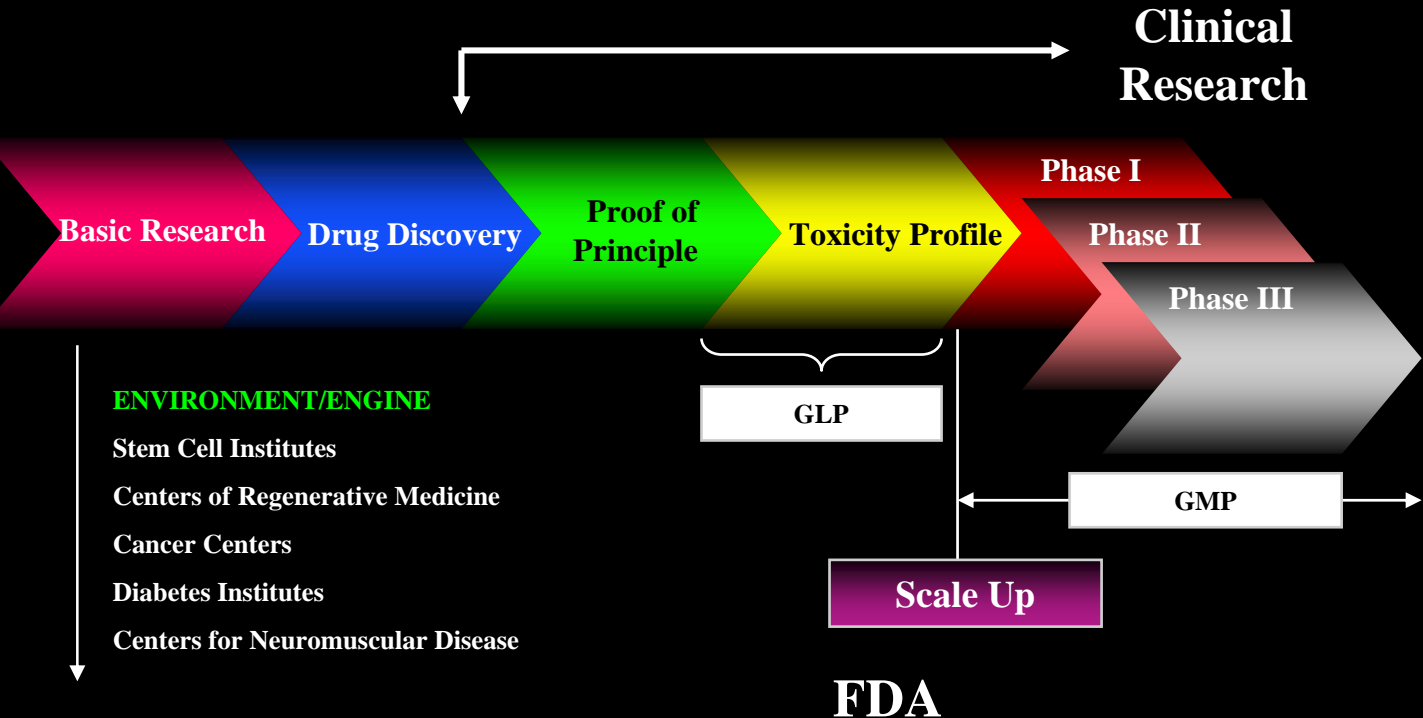
Bone Marrow Transplant 1968 - 2008

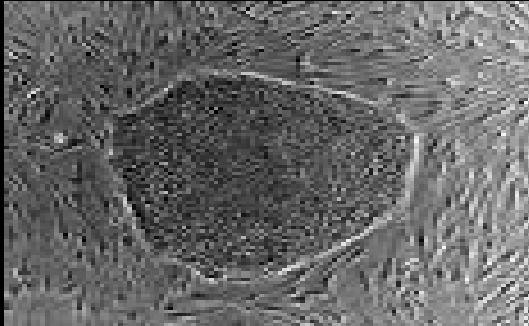
Cord Blood Transplant 1988 - 2008



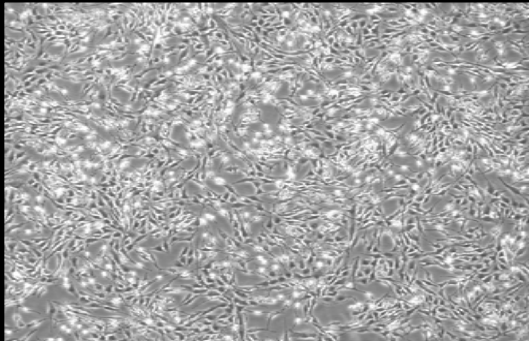
Proven Stem Cell
Therapy

'Bottleneck' of the Translational Pipeline





Embryonic Stem Cells

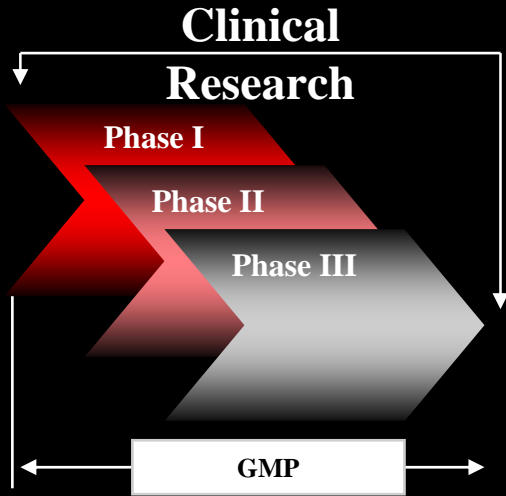


Adult Stem Cells

Phase I Clinical Trials

Goals and expectations

- **To document frequency and severity of side effects (safety profile)**
- **To identify the Maximal Tolerable Dose (MTD)**



Potential Risks

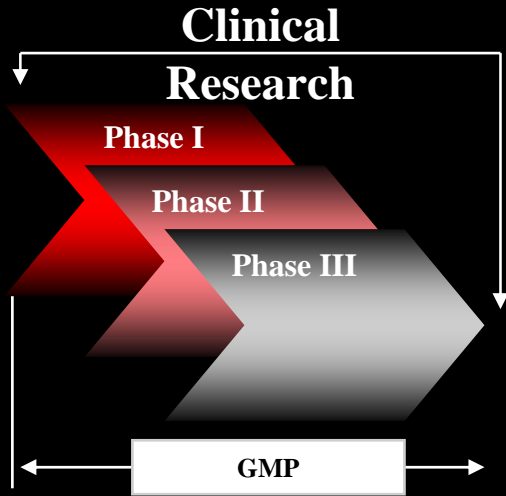
Teratoma

Cancer

Ectopic tissue formation

Infectious disease

Genetic disease



Other Questions

- Standards for animal models
- Methods of cell characterization
- Assessment of the immune response
- Potency assays

Goals

- **How do you determine the optimal animal model?**
 - **Proof of concept (efficacy)**
 - **Toxicology (risks)**
 - **Pharmacology (cell distribution/survival)**
- **What is needed to initiate clinical trials**
 - **Minimum standards for the Product**
 - **Clinical study designs to obtain early safety data**

Speakers

Deborah A. Hursh, Senior Investigator

**Division of Cellular and Gene Therapies for
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Darin J. Weber, Senior Consultant

**Cell and Gene Therapies
Biologics Consulting Group, Inc**