PACT
Production Assistance for Cellular Therapies

14th Annual ASGCT Meeting
Translational Science Training Course
-Resources-

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PACT Program

- NHLBI funded initiative
- Began in September 2003
- Renewed in January 2010
- # of facilities, scope, and interest expanded

**Objective**

Provide assistance with cellular therapy translational research and the manufacture of cellular therapy products
PACT Organizational Structure

National Heart Lung and Blood Institute

Steering Committee

External Expert Panel

5 GMP Cell Processing Facilities

Coordinating Center (The EMMES Corporation)

- Baylor College of Medicine
- Beckman Research Institute
- Center for Human Cell Therapy
- University of Minnesota
- University of Wisconsin - Madison

- CAGT
- City of Hope CATD
- Boston
- Les Silberstein
- MCT
- John Wagner
- WCBF
- Derek Hei
- Adrian Gee
Application Requests

Products and Services

- Products that aid in the repair and regeneration of damaged/diseased tissues, organs, and biologic systems
- Preclinical/translational studies including animal model work
- Products of programmatic interest to the Institute and associated with a funded clinical study
- Proposals possessing procedural advancements to further foster and standardize cell therapies
NHLBI Scope

- Cardiac repair and disease
- Lung repair and disease
- Complications of malignancy treatment
  - GVHD
  - Post transplant viral infections
- Hematologic disease outside of primary treatment for malignancy
Expanded Interest

- Support for translational work
  - All translational work will be evaluated
- Specific support for GMP translational work not funded through standard NHLBI grant mechanisms
PACT’s Role in Supporting Pre-Clinical Work and Phase I Clinical Trials

**Discovery**
- Proof of concept; cell Product potential; therapeutic mechanism and pathway; cell and disease interaction

**Basic Research**
- Proof of concept; cell Product potential; therapeutic mechanism and pathway; cell and disease interaction

**Pre-clinical**
- Dose escalation; safety and toxicity studies; small trial size
- IND Filing
  - Dose ranging; safety and efficacy studies; increase trial size
  - GLP/GMP product
  - Efficacy
  - Toxicity

**Manufacturing**
- Scale up
- Validation
- Release Criteria
- CMC

**Animal Studies**
- GLP/GMP product
- Efficacy
- Toxicity

**Phase I**
- Dose escalation; safety and toxicity studies; small trial size

**Phase II**
- Efficacy and safety studies; full product characterization; potency; scale up; full GMP

**Phase III**
- Manufacturing
  - Scale up
  - Validation
  - Release Criteria
  - CMC
  - Animal Studies
    - GLP/GMP product
    - Efficacy
    - Toxicity
PACT Manufacturing Models

- Centralized Manufacturing
  - Provide single or multiple sites without manufacturing capability with clinical product

- Distributive Manufacturing
  - Manufacturing occurring at multiple sites
PACT Program Status

- 40 ongoing projects
  - 23 - Clinical
    - 12 - Delivering clinical product (cardiac; GVHD; post transplant viral infections; hematological malignancies; X-linked severe combined immunodeficiency [SCID-X1])
  - 17 - Translational (pre-clinical animal studies for cardiac & lung indications; Wiskott Aldridge Syndrome Protein deficiency; stem cells for corneal transplantation)
PACT Application Process

- Web based Preliminary Application
- Concept Review by Steering Committee
- Full Application invited
- Web based Full Application
- Independent Reviews
- Facility and Technical Liaison Assignment
- Budget, Contract & Timeline
Contact Information

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