

**PACT**  
Production Assistance for Cellular Therapies

---


*Baylor – PACT Workshop*

Monday, October 22, 2012  
Robert Lindblad, MD  
PACT Coordinating Center (EMMES Corporation)  
Rockville, Maryland

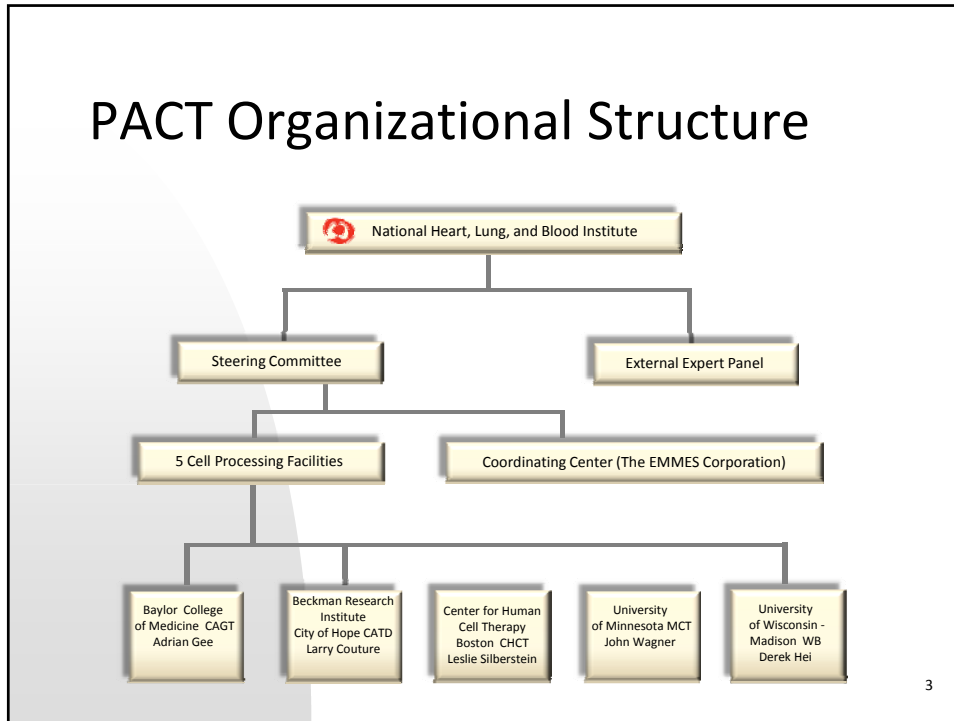
## PACT Program

- 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


 Production Assistance for Cellular Therapies  
National Heart Lung and Blood Program

2



## 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

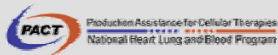


Production Assistance for Cellular Therapies  
 National Heart, Lung and Blood Program

4

## 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



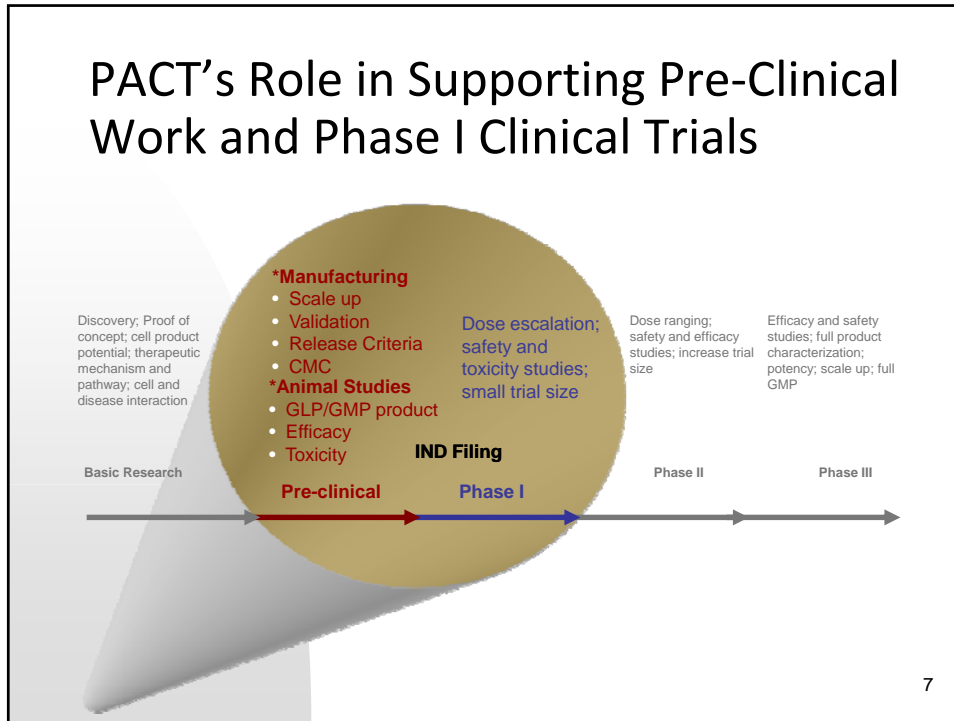
5


## 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



6



- ## PACT Manufacturing Models
- Centralized Manufacturing
    - Provide single or multiple sites without manufacturing capability with clinical product
  - Distributive Manufacturing
    - Manufacturing occurring at multiple sites
- 
- 8

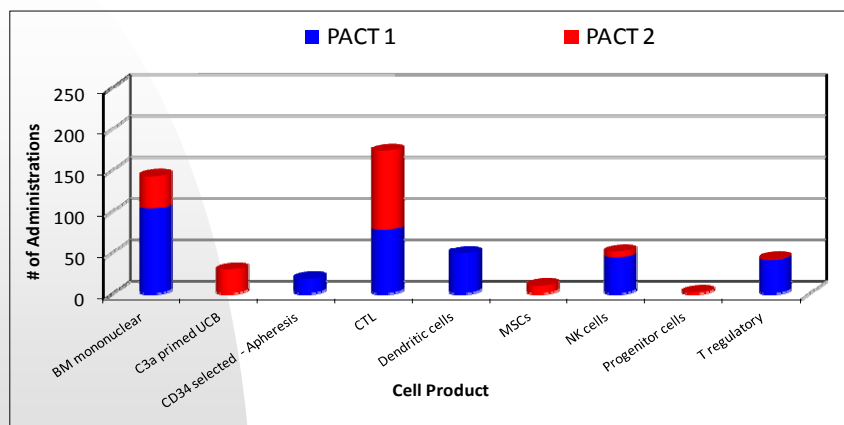
## PACT Program Status

- 27 Ongoing Projects
  - 13 - Clinical
    - 6 - Delivering clinical product (GVHD; post transplant viral infections; hematological malignancies; X-linked severe combined immunodeficiency [SCID-X1])
  - 14 - Translational (pre-clinical animal studies using hESCs and MSCs for cardiac and lung indications; transduced T cells for Wiskott-Aldridge Syndrome Protein deficiency; stem cells for corneal transplantation)



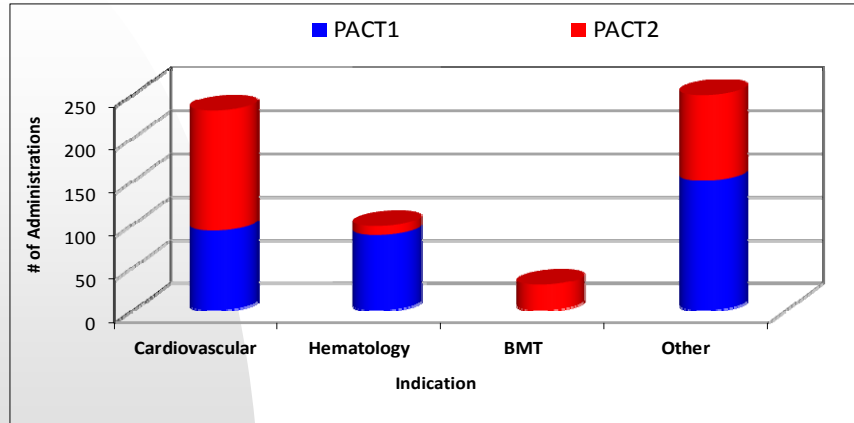
9

## Product Administrations By Cell Product

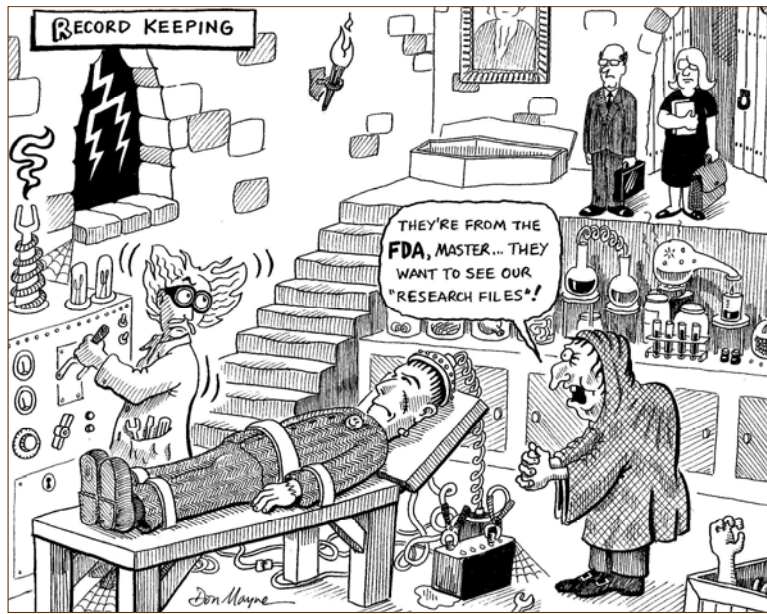


Total forms received = 616

## Product Administrations By Indication



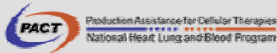
Total forms received = 616



12

## PACT and INDs

- Bench to Bedside
  - Process
    - Discovery
    - Animal studies
    - Scale up
    - IND submission
      - Pre-clinical efficacy/toxicity
      - Clinical protocol
      - CMC - manufacturing



13

## Manufacturing and Facilitating IND Submission...

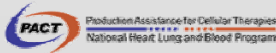
- Pre- Clinical work
  - Provide cells for animal studies
  - Animal models for preclinical studies
- Clinical study protocol development
  - Study product information
- Technology transfer



14

## Manufacturing and Facilitating IND Submission...

- Product development
  - Process Development/Product Scale-Up
  - Product SOPs Development/Batch Records
  - Process/Equipment Validation
  - Product Release Specifications and COA
  - Product Shipping to Clinical Sites



15

## CMC Section Development

- UMN MCT - Shipping Validation
- *Validation of Natural Killer Cell Activation During Long-Distance Shipping*
  - NK cells – not conducive to cryopreservation
  - Cells were incubated during shipping in IL-2
  - Temperature stabilizing packs to maintain approximate body temperature within the shipping container.
  - Following round trip flight to Columbus, OH (AirNet), cells were washed and re-suspended prior to QC/lot release testing.
- NK cells can be successfully shipped for use at distant clinical centers



16



## Animal Model Studies

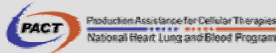
- Indication - Acute Lung Injury
- FDA request
- Pre-clinical animal model work
  - Provided cells for large animal studies
    - Allogeneic human mesenchymal stem cells were manufactured by the UMN-MCT

## Animal Model Studies

- Indication - Bronchiolitis Obliterans
- FDA request
- Pre-clinical animal model work
  - Provided cells for animal studies
    - Allogeneic human mesenchymal stem cells were manufactured by the University of Wisconsin-Madison
    - Developed animal model and preclinical study protocol
    - Study almost complete

## PACT Application Process

- Web based Preliminary Application
- Concept Review by Steering Committee
- Full Application invited (Clinical/Translational)
- Web based Full Application
- Independent Reviews
- Facility and Technical Liaison Assignment
- Budget, Contract & Timeline



19

## Contact Information

Debbie Wood or Jamie Winestone  
Phone: 301-251-1161  
Email: [pactinfo@pactgroup.net](mailto:pactinfo@pactgroup.net)

Website  
[www.pactgroup.net](http://www.pactgroup.net)



20