

# *PACT*

## Production Assistance for Cellular Therapies



*University of Wisconsin*

*PACT-sponsored Workshop*

Developing Cellular Therapies: From Preclinical Safety To  
Clinical Evaluation



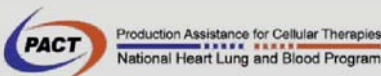
Tuesday, April 09, 2013  
Robert Lindblad, MD  
PACT Coordinating Center (EMMES Corporation)  
Rockville, Maryland

## PACT Program

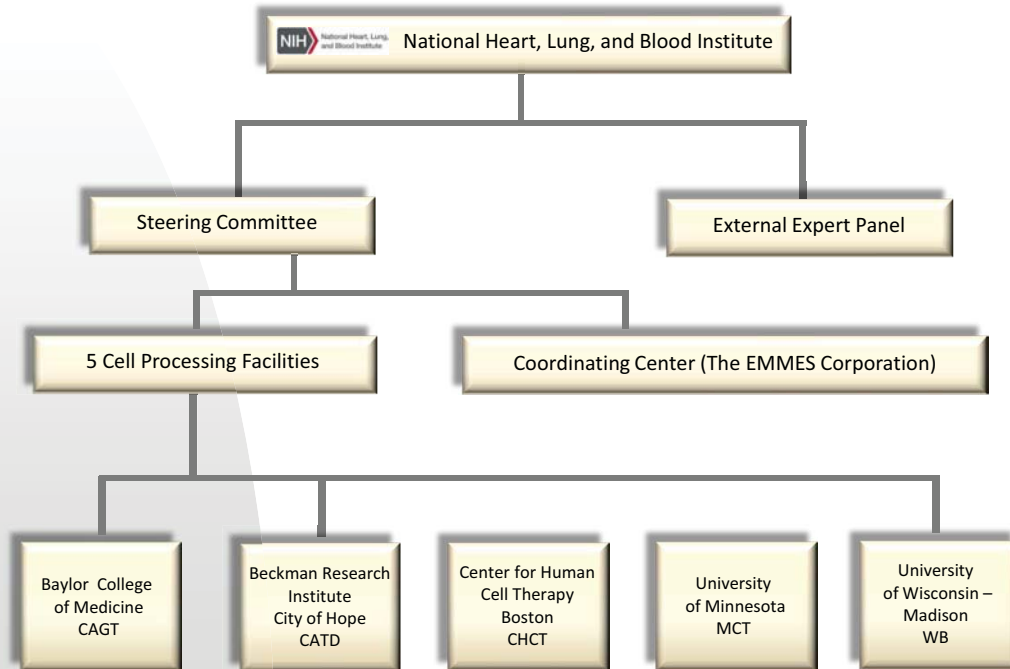
- NHLBI funded initiative
- Began in September 2003
- Renewed in January 2010
- # of facilities, scope, and interest expanded

### **Objective**

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



# PACT Organizational Structure



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## 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

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NHLBI



- 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

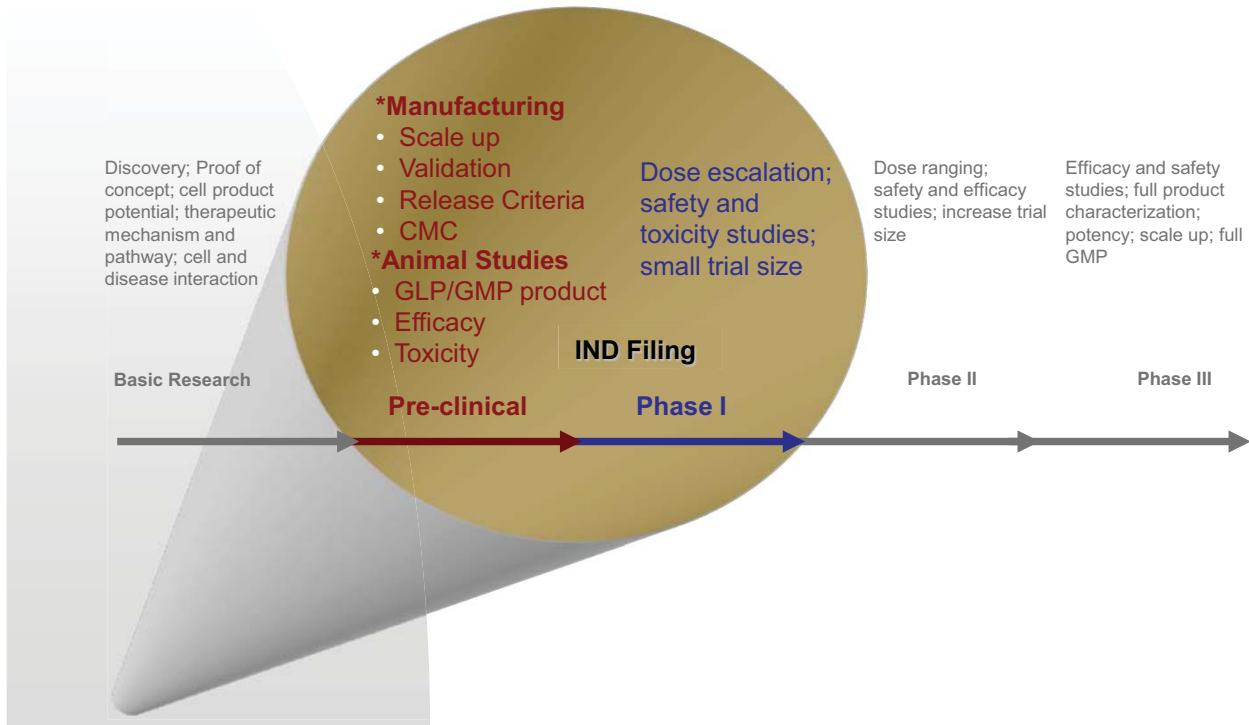


## Expanded Interest



- Support for translational work
  - All translational work will be evaluated
- Specific support for GMP translational work not funded through standard NIH/NHLBI grant mechanisms

# PACT's Role in Supporting Preclinical Work and Phase I Clinical Trials



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## PACT Program Status - Clinical

- 47 clinical projects approved
  - 27 completed
  - 4 closed; 3 withdrawn/hold
  - 13 ongoing clinical projects
    - 8 currently delivering clinical product
      - CTLs for GVHD
      - CTLs (multi-virus, LMP1/2-specific and gene modified) for post transplant viral infections
      - NK cells for hematological malignancies and GVHD
      - CD 133 cells for critical limb ischemia
      - CD 34 cells for X-linked severe combined immunodeficiency [SCID-X1])

*Over 600 PACT-manufactured cell therapy products have been administered for a range of illnesses and diseases such as stroke, acute myocardial infarction, sickle cell disease, diabetes, hematologic malignancies, and graft versus host disease.*

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# PACT Program Status - Translational

- 46 translational projects approved
  - 18 completed
  - 2 closed; 6 withdrawn/hold
  - 20 ongoing translational projects
    - Pre-clinical animal studies using hESCs and MSCs for cardiac and lung indications
    - MSCs for treatment for vocal fold scarring, emphysema, and human tissue engineered lung transplant
    - T-regs for GVHD
    - HIV-1-specific CTL therapy
    - Corneal and epithelial stem cells for corneal transplantation
    - Fibroblasts for wound healing

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## PACT and INDs

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



# Animal Model Studies



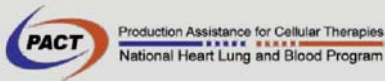
- Indication - Acute Lung Injury
- FDA request
- Pre-clinical animal model work
  - Provided cells for large animal studies
    - Allogeneic human MSCs were manufactured by the UMN-MCT. MCT developed CMC section
    - Investigator submitted IND in Dec 2012. No additional questions from FDA
    - Phase 1 dose escalation trial to commence late 2013

# Animal Model Studies

- Indication - Bronchiolitis Obliterans
- FDA request
- Pre-clinical animal model work
  - Provided cells for animal (rodent) studies
    - Allogeneic human MSCs were manufactured by the University of Wisconsin-Madison
    - Developed animal model and preclinical study protocol
    - Animal study near completion

# 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



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## Education Initiative: Webinars

- 20 Webinars
  - 15 in PACT 1
    - Attendance - Average 170
  - 5 in PACT 2
    - Attendance - Average 240
  - Variety of cell therapy topics
  - 1-3 speakers (internal and external to PACT, investigators, FDA)
  - Interactive Q&A session



Slide presentations, webinar recordings, and Q&A transcripts available on the PACT website [www.pactgroup.net](http://www.pactgroup.net)

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# Education Initiative: Workshops



- 7 facility-hosted GMP workshops
  - 3 PACT 1; 4 PACT 2 (WB today!)
- 2 national workshops (NIH campus)
  - PACT 1 - *Converging Concepts in Cell Therapy*; April 23-24, 2009  
*Stroncek D, et al. Developments in Clinical Cell Therapy. Cytotherapy. 2010;12: 425–428.*
  - PACT 2 - *Cell Therapy for Pediatric Diseases: A Growing Frontier*; September 14-15, 2011  
*(Manuscript submitted to Pediatrics in 2012)*
- Planned
  - *Off-the-Shelf Cell Therapies*; October 28-29, 2013

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## 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)