Production Assistance for Cellular Therapies - PACT

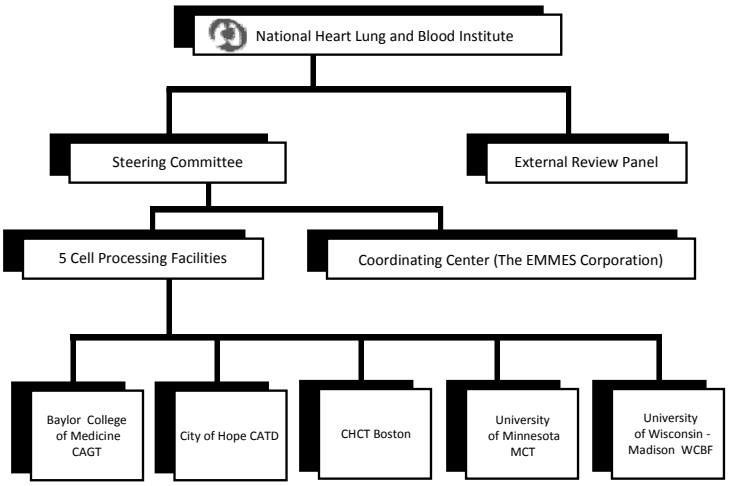
National Heart Lung and Blood Institute
People Science Health

Traci Heath Mondoro, PhD
NHLBI
Bethesda, Maryland

Renewed PACT Program

- Renewed January 15, 2010
- Scope and size expanded
- ↑ Cell Processing Facilities (CPFs) from 3 to 5
- Mission
  - Provide assistance for cellular therapy translational research and the manufacture of cellular therapy products

PACT Organizational Structure



NHLBI Expectations

- Continue to advance cellular therapy research in the areas of heart, lung and blood cellular therapy research
  - Provide consulting, manufacturing, preclinical study and early phase clinical trial study design, and administrative and regulatory expertise
  - Foster partnership of transfusion medicine and hematology with cell-based therapy
  - Collaborate with other NIH groups (CTSA) to streamline clinical and translational research
  - Provide educational leadership in the field of cell therapy

NHLBI Expectations

- Additional Services
  - Support for proof-of-principle animal and early translational research
  - Provide centralized services (i.e. functional assays)

Expanded Interest

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

Applications

- Scope:
  - Products and Services
    - Products that aid in the repair and regeneration of damaged/diseased tissues, organs, and biologic systems
    - Products of programmatic interest to the Institute and associated with a funded clinical study
    - Preclinical studies including basic and translational (animal models) work

Applications

- Sample NHLBI Scope:
  - Cardiac repair and disease
  - Lung repair and disease
  - Complications of malignancy treatment (GVHD)
  - Hematologic disease outside of primary treatment for malignancy

For more information on criteria for evaluation of PACT product requests visit www.pactgroup.net

Today's Discussion

- Facilities - Translational and Clinical projects that serve as examples of what PACT will support
- EMMES - Application Process

Contact Information

- Traci Heath Mondoro and Lisbeth Welniak

Phone: 301-435-0065
Email: mondorot@nhlbi.nih.gov
welniakla@nhlbi.nih.gov

Production Assistance for Cellular Therapies
National Heart Lung and Blood Program

May 4, 2010 PACT Webcast Overview

Production Assistance for Cellular Therapies (PACT) is an NHLBI-sponsored program. PACT is offering this web seminar to introduce the program to the cell therapy community and investigators potentially in need of cell products and cell therapy related services.

Web Seminar Description:

The purpose of the webcast is to describe the mission and goals of the PACT program and to answer participants' questions about the program's goals, the application process and criteria, and the details surrounding PACT product manufacturing assistance.

Intended Audience:

Clinical investigators, Scientists, Researchers, and Technologists specializing in cell therapy

I. NHLBI Presentation: Overview of the PACT program. Scope criteria used for accepting applications.

Slide set available on the PACT website at www.pactgroup.net.

The renewed PACT program continues to advance cellular therapy research in the area of immune based cell therapeutics, regenerative medicine and other treatments for diseases that do not currently have effective therapies. The program's scope is expansive supporting all translational research related to the development of novel cellular therapeutics including those that fall outside NHLBI scope. Requests for clinical product manufacturing, however, are restricted to those that fall within the programmatic interests of the NHLBI.

PACT Facility Core Competencies

- Quality and Compliance
- Operations/Materials Management
- Control of Critical Systems
- Program Budgets/Forecasts/Cost Accounting
- Business Plan Development/Project and Resources Management
- Equipment Management
- Document Change Control
- Process Development, Qualification, Validation
- Regulatory Affairs – RAC, Pre-IND, IND support

II. PACT Cell Processing Facility (CPF) Speakers:

To illustrate types of preclinical and clinical projects, a speaker from each facility will provide examples of currently ongoing work at their Facility:

- Ann Leen, PhD – Baylor College of Medicine Center for Cell and Gene Therapy
- Larry Couture, PhD – City of Hope, Center for Applied Technology Development
- Myriam Armant, PhD – Center for Human Cell Therapy Boston
- Derek Hei, PhD – University of Wisconsin – Madison, Waisman Clinical BioManufacturing Facility
- John Wagner, MD – University of Minnesota Molecular and Cellular Therapeutics Facility

A. Ann Leen, PhD - Baylor College of Medicine Center for Cell and Gene Therapy (CAGT)

Email: amleen@txccc.org
Phone: (832) 824-4690

CAGT Facility Overview

- Translational Research Laboratories
- cGMP Manufacturing Facility
  - Quality Control Laboratory and Quality Assurance
- GMP Vector Production Facility

B. Larry Couture, PhD – City of Hope, Center for Applied Technology Development (CATD)

Email: lcouture@coh.org
Phone: (626) 256-8728

CATD Facility Overview

- Office of Project Management
- Office of Technology Licensing
- Center for Biomedicine and Genetics
- Office of Regulatory Affairs
- Office of Quality Systems

CATD *Active Projects (translational)*

- H1 hESC Master Cell bank
- Establishment of Cardiomyocyte Differentiation Process

C. Myriam Armant, PhD – Center for Human Cell Therapy (CHCT) Boston

Email: info@chct.org
Phone: (617) 919-2390

CHCT Facility Overview

- Translational Laboratory
  - Immune Disease Institute
  - Programs in Cellular and Molecular Medicine at Children's Hospital Boston
- Cell Manipulation Core Facility
  - Dana Farber Cancer Institute

CHCT *Active Projects (translational)*

Myocardial Regeneration using Cardiac Stem Cells Harvested from Right Atrial Appendages in Patients with Ischemic Cardiomyopathy.

D. Derek Hei, PhD – University of Wisconsin – Madison, Waisman Clinical BioManufacturing Facility (WCBF)

Email: hei@waisman.wisc.edu
Phone: (608) 263-5821

WCBF Facility Overview

- Waisman Clinical BioManufacturing Facility
- Clinical Stem Cell Collection and Processing Lab
- Pre-Clinical Animal Studies Core
- Regulatory Affairs Core

WCBF *Active Projects (translational)*

- Human ES Cell Banks
- Mesenchymal Stem Cell Banks

E. John Wagner, MD – University of Minnesota Molecular and Cellular Therapeutics (MCT)

Email: wagne002@umn.edu
Phone: (612) 626-2961

MCT Facility Overview

- Clinical Cell Therapy Laboratory, University of Minnesota Medical Center, Fairview
- Cancer Center/Regenerative Medicine Translational Laboratory
- Pancreatic Islet Transplant Program
- Active Pharmaceutical Ingredient Synthesis/Biotherapeutic Protein Production Facility

MCT *Active Projects (translational)*
UCB T Regulatory Cells to Prevent Acute Graft-versus-Host Disease

III. EMMES Corporation (PACT Coordinating Center)

Presentation: Overview of the PACT online application process. Access the online application using www.pactgroup.net

Slide set available on the PACT website at www.pactgroup.net

A Preliminary application is initially submitted and reviewed by the PACT Steering Committee (SC) to determine if the proposal is compatible with the NHLBI mission and objectives of supporting heast, lung and blood cellular therapy research. If appropriate, the applicant will be invited to submit a full application. The full application is needed to obtain the additional information required by the Steering Committee (SC) to evaluate the request, and if approved, to assign the work to the appropriate Cell Processing Facility (CPF). After verification that all requested information is included, the full application and associated materials will be provided to the PACT SC for review. *The Criteria for Evaluation and Product Requests* document is available on the PACT web site (www.pactgroup.net) to assist you completing your application.

APPLICATION PROCESS

Robert Lindblad, MD
EMMES Corporation
Rockville, Maryland

Production Assistance for Cellular Therapies
National Heart Lung and Blood Program

PACT Application Process

- Web-based Preliminary Application
- Concept Review by Steering Committee
- Full Application Invited
- Technical Liaison Assigned
- Web-based Full Application
- Peer Reviewed & SC Vote
- Budget, Contract & Timeline

2

Full Application Content

- Translational or Clinical
- SOPs and validation information
- Any manufacturing financial support
- Clinical trial financial support
- Details of amount of product required
- Clinical protocol and safety monitoring plan
- IND/regulatory status
- Proposed timeline and budget

3

Steering Committee Review Criteria

- Scientific merit & relevance to NHLBI
- Level of translation work
- Clinical trial design
- Funding
- Regulatory status
- Availability of expertise at PACT
- Capacity at PACT

4

Next Steps

- Budget
- Contract
  - Contract negotiations with designated facility
  - Draft contract developed
    - Intellectual property and/or indemnification
- Timeline
  - Development of production task order
  - Scheduling of manufacturing tasks
  - Scheduling of production

5

Website

- www.pactgroup.net
  - Application process information
  - Education Initiatives
  - FAQ and updates
  - Links to PACT facilities, regulatory references & other resources

6

Contact Information

- Robert Lindblad, Jamie Winestone or Debbie Wood

  Phone: 301-251-1161
  Email: pactinfo@pactgroup.net

7