Production Assistance for Cellular Therapies (PACT) Program
NHLBI

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PI PACT Coordinating Center

Objectives

- **What is PACT?**
- **Why contact PACT?**
- **How do I contact PACT?**

Email: pactgroup@emmes.com

Website: www.pactgroup.net

Outline

- **PACT PROGRAM**
  - Program, cell types, application for manufacturing assistance
  - Education
  - Technical projects
- **REGULATORY SUPPORT**
  - Gap analysis
  - FDA meeting support

PACT Program

NHLBI-funded initiative NIH National Heart, Lung, and Blood Institute

- PACT 1: Began in September 2003-2009 (1 year no cost extension)
  - 3 facilities/1 Coordinating Center. Scope - clinical product support
- PACT 2: Renewed in January 2010-2015
  - 5 facilities/1 Coordinating Center. Scope - clinical/translational
- 1 ½ year hiatus
- PACT 3: Re-awarded in July 2016
  - 5 facilities/1 Coordinating Center. Scope - preclinical/translational

Current Mission

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

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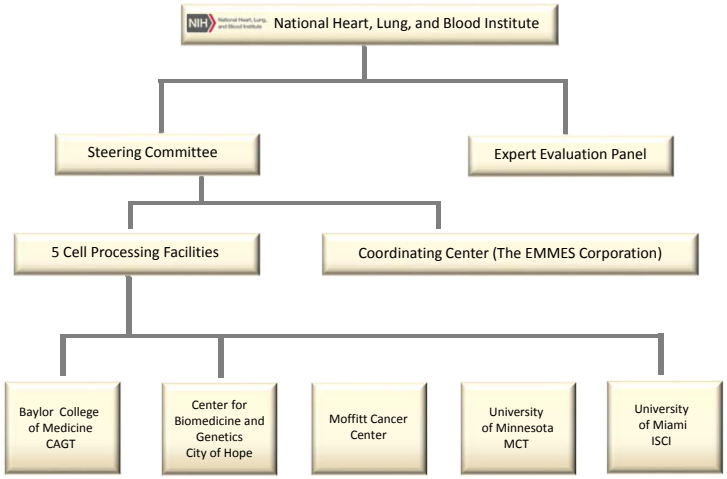
PACT Organizational Structure



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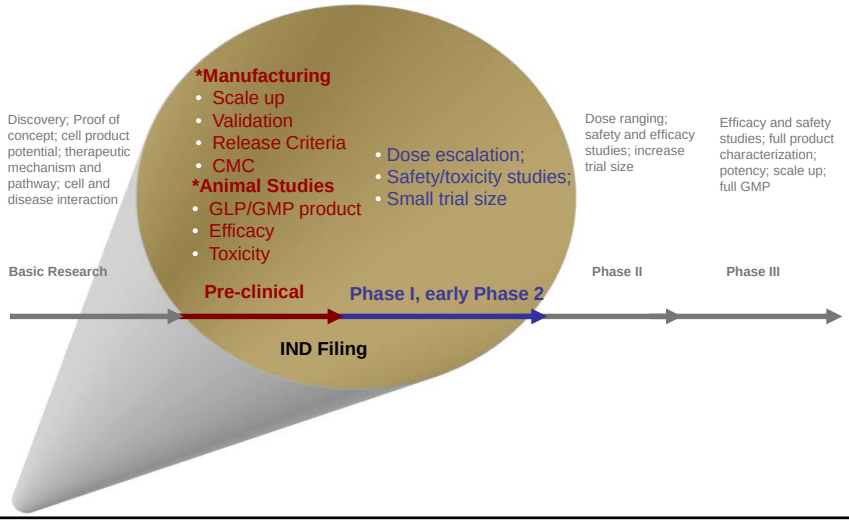
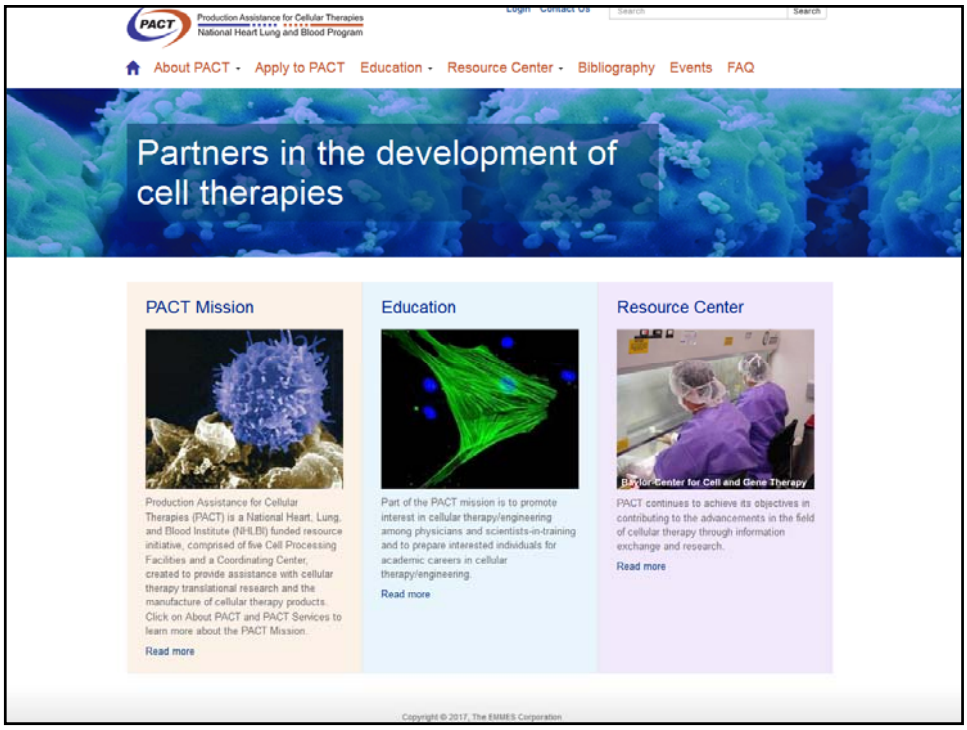
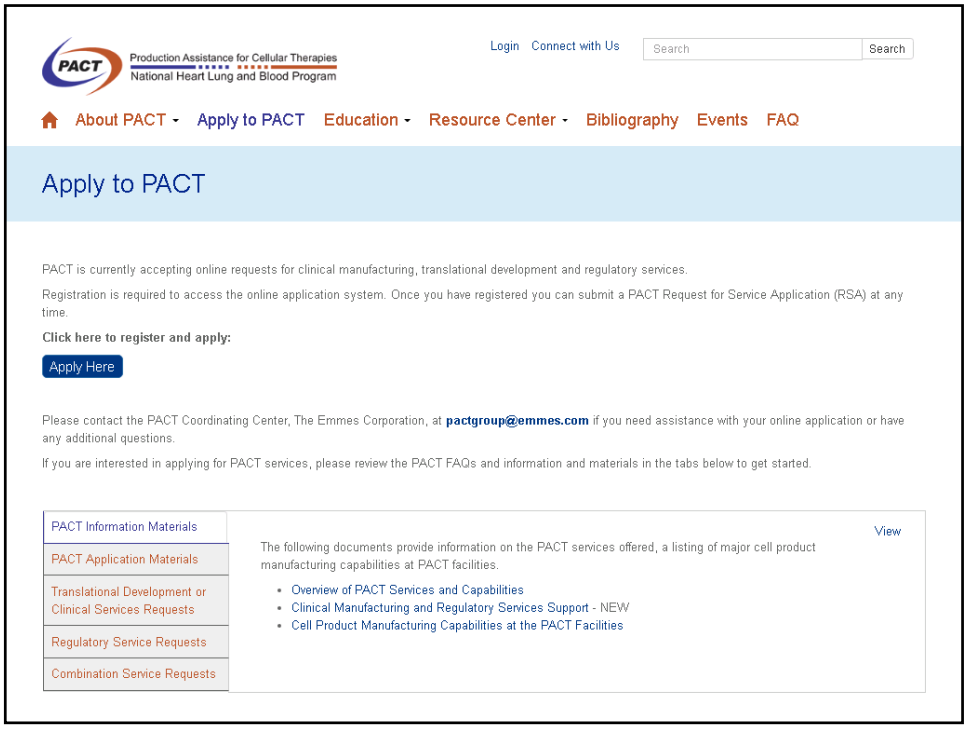
Scope

- Products and services of programmatic interest to the National Heart, lung and Blood Institute
- Products that aid in the repair and regeneration of damaged/diseased tissues, organs, and biologic systems
- Cell therapy manufacturing for preclinical studies including basic and translational work
- Cell therapy manufacturing for phase 1/2 clinical trials
- Regulatory support
- Proposals possessing procedural advancements to further foster and standardize cell therapies

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



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Apply to PACT
PACT is currently accepting online requests for clinical manufacturing, translational development and regulatory services.
Registration is required to access the online application system. Once you have registered you can submit a PACT Request for Service Application (RSA) at any time.
Click here to register and apply:
Apply Here
Please contact the PACT Coordinating Center, The Emmes Corporation, at pactgroup@emmes.com if you need assistance with your online application or have any additional questions.
If you are interested in applying for PACT services, please review the PACT FAQs and information and materials in the tabs below to get started.
PACT Information Materials
PACT Application Materials
Translational Development or Clinical Services Requests
Regulatory Service Requests
Combination Service Requests
View
The following documents provide information on the PACT services offered, a listing of major cell product manufacturing capabilities at PACT facilities.
Overview of PACT Services and Capabilities
Clinical Manufacturing and Regulatory Services Support - NEW
Cell Product Manufacturing Capabilities at the PACT Facilities


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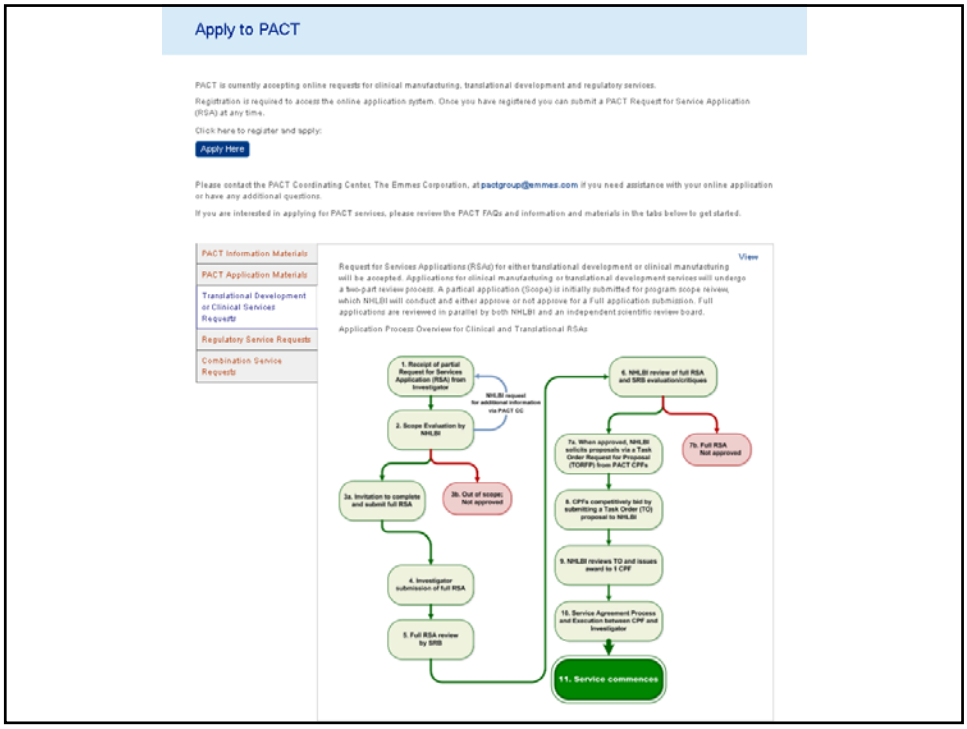
The following documents provide information on the criteria that NHLBI will take into consideration when performing the initial scope review as well as evaluating the full RSA review (full review for Translational or Clinical RSAs only).
PACT Application: Scope Criteria
PACT Evaluation Criteria
You can reference the PACT Application System Users Guide for questions on how to complete initial registration and how to navigate within the application system. You may contact pactgroup@emmes.com for technical assistance with submitting your application.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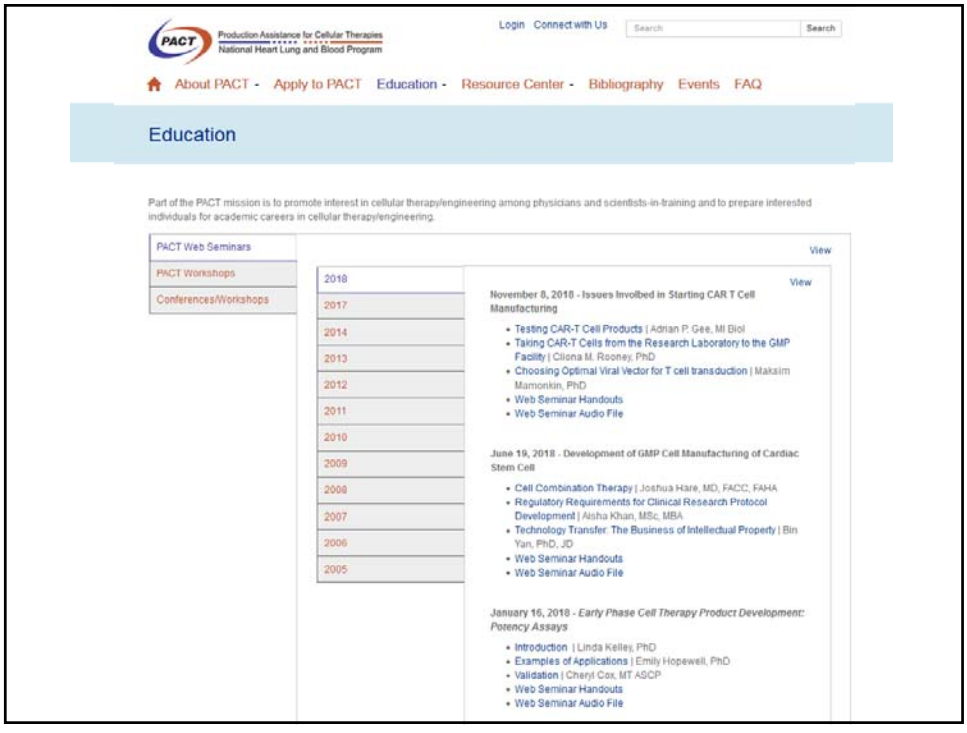
Request for Services Applications (RSAs) for either translational development or clinical manufacturing will be accepted. Applications for clinical manufacturing or translational development services will undergo a two-part review process. A partial application (Scope) is initially submitted for program scope review, which NHLBI will conduct and either approve or not approve for a Full application submission. Full applications are reviewed in parallel by both NHLBI and an independent scientific review board.
Application Process Overview for Clinical and Translational RSAs
1. Receipt of partial Request for Services Application (RSA) from Investigator
NHLBI request for additional information via PACT CC
2. Scope Evaluation by NHLBI
3a. Invitation to complete and submit full RSA
3b. Out of scope; Not approved
4. Investigator submission of full RSA
5. Full RSA review by SRB
6. NHLBI review of full RSA and SRB evaluation/critiques
7a. When approved, NHLBI solicits proposals via a Task Order Request for Proposal (TORFP) from PACT CPFs
7b. Full RSA Not approved
8. CPFs competitively bid by submitting a Task Order (TO) proposal to NHLBI
9. NHLBI reviews TO and issues award to 1 CPF
10. Service Agreement Process and Execution between CPF and Investigator
11. Service commences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Part of the PACT mission is to promote interest in cellular therapy/engineering among physicians and scientists-in-training and to prepare interested individuals for academic careers in cellular therapy/engineering.
PACT Web Seminars
PACT Workshops
Conferences/Workshops
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2018
2017
2014
2013
2012
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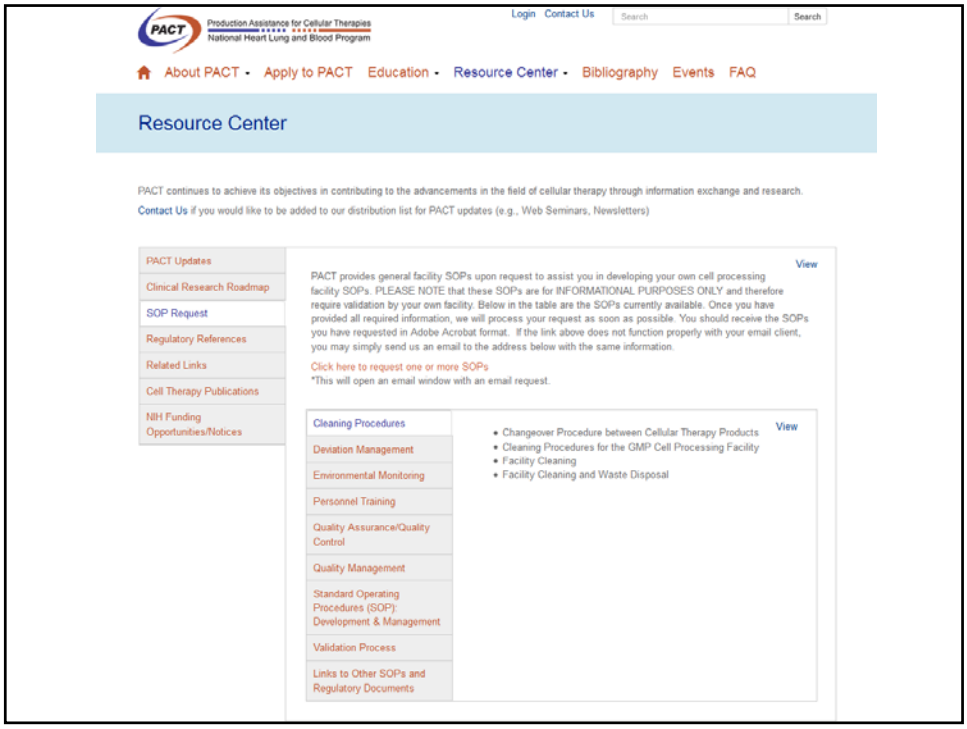
November 8, 2018 - Issues Involved in Starting CAR T Cell Manufacturing
Testing CAR-T Cell Products | Adrian P. Gee, MI Biol
Taking CAR-T Cells from the Research Laboratory to the GMP Facility | Cliona M. Rooney, PhD
Choosing Optimal Viral Vector for T cell transduction | Maksim Mamonkin, PhD
Web Seminar Handouts
Web Seminar Audio File
June 19, 2018 - Development of GMP Cell Manufacturing of Cardiac Stem Cell
Cell Combination Therapy | Joshua Hare, MD, FACC, FAHA
Regulatory Requirements for Clinical Research Protocol Development | Aisha Khan, MSc, MBA
Technology Transfer: The Business of Intellectual Property | Bin Yan, PhD, JD
Web Seminar Handouts
Web Seminar Audio File
January 16, 2018 - Early Phase Cell Therapy Product Development: Potency Assays
Introduction | Linda Kelley, PhD
Examples of Applications | Emily Hopewell, PhD
Validation | Cheryl Cox, MT ASCP
Web Seminar Handouts
Web Seminar Audio File



Educational Resources

- SOPs
  - 309 individual requests totaling >1275 individual SOP distributions
  - 113 US Institutions/sites and 22 international countries

✓ Most requested

Cleaning Procedures
Deviation Management
✓ Environmental Monitoring
Personnel Training
✓ Quality Assurance/Quality Control
✓ Quality Management
Standard Operating Procedures (SOP): Development & Management
✓ Validation Process

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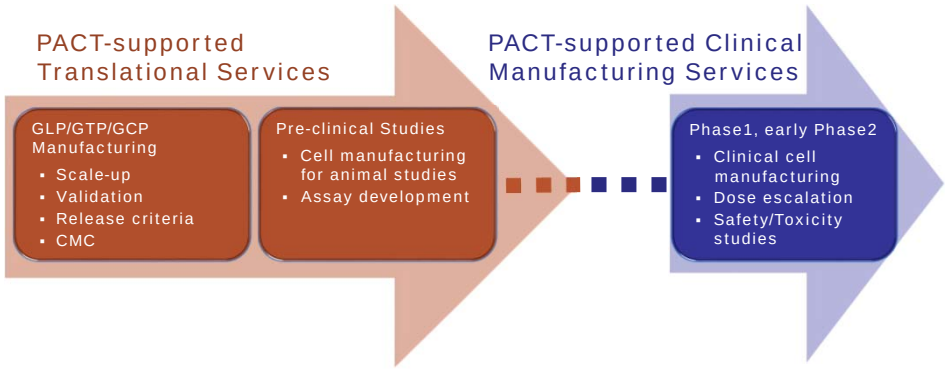
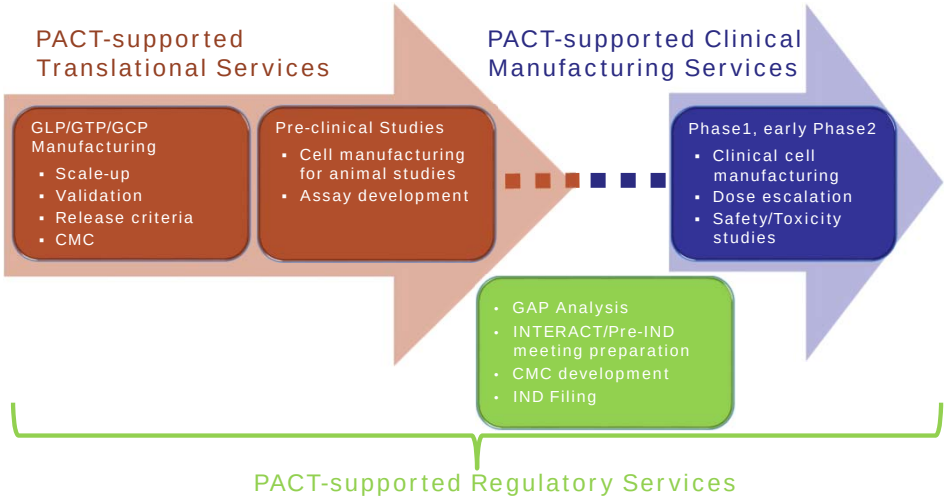
Regulatory Support

Cell Manufacturing for IND-enabling and Early Phase Clinical Studies



Cell Manufacturing for IND-enabling and Early Phase Clinical Studies



GAP Analysis

- Provides insight into what additional work may be needed to support an IND application
- Geared toward an understanding of how the data generated from Proof of Concept, non-clinical and CMC development map to the requirements of an IND application
- Recommendations for facilitating a successful a PACT Request for Service Application (RSA)

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FDA Meeting support

FDA Meeting Support

FDA communication and assistance:

- INTERACT and pre-IND meeting scheduling and planning
- Developed questions to ask the agency
  - Content
  - Format
- Quality of the CMC information
- Status of the Pre-clinical information
- Status of the general investigational plan and proposed clinical indication
- Adequate phase 1 synopsis, defined activity evaluation and protocol

Definitions for Acceleration of Review

- Serious or life threatening disease
  - Disease or condition associated with morbidity that has substantial impact on day-to-day functioning
- Unmet medical need
  - A condition whose treatment or diagnosis is not addressed adequately by available therapy

Other Regulatory Considerations

- Orphan Designation
- Special Protocol Assessment
- Accelerating Development
  - Fast Track
  - Breakthrough Designation
  - Priority Review
  - Accelerated Approval
- Regenerative Medicine Provisions
- Electronic submissions

Contact Information

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