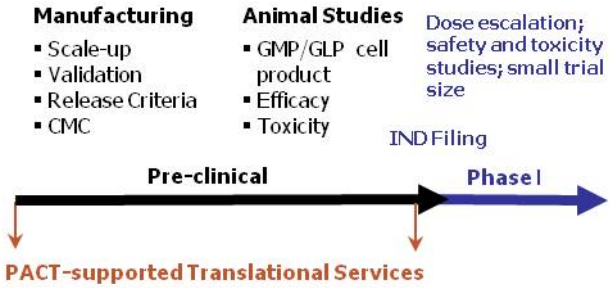
Production Assistance for Cellular Therapies (PACT)
Coordinating Center - The Emmes Corporation
401 N. Washington Street, Suite 700, Rockville, MD 20850

PACT Evaluation Criteria

Successful clinical cell therapies require transformation of laboratory based techniques into specific cell production processing with regulatory safety and efficacy standards. This product development model will be used when assessing application requests for research and clinical grade products.

Cell Therapy Product Development



The NHLBI-funded program, Production Assistance for Cellular Therapies (PACT) provides assistance for cellular therapy translational research and the manufacture of cellular therapy products that aid in the repair and regeneration of damaged/diseased tissues, organs, and biologic systems. Applications that fall under the interests of NHLBI which involve cardiovascular repair and disease, lung repair and disease, hematologic disease and hematopoietic cell transplantation, outside of primary treatment for malignancy will be considered by PACT.

Applications requesting services for cGMP/cGLP manufactured cellular products involving proof-of-principle, but IND-enabling translational research in heart, lung, blood and sleep indications will be considered by PACT. These services include optimizing manufacturing processes, SOP development, CMC section development and cell manufacturing for relevant animal model studies.

Areas of Shared Interest

NHLBI shares many areas of interest with other NIH Institutes. NHLBI's scientific interests lie with the development, structure and function of heart, lung and blood organ systems in normal and diseased states, as well as any disease or dysfunction of other closely related organs and systems where the problem is primarily cardiac, vascular, pulmonary or blood-related. Please refer the *PACT Scope Review Criteria* document located on the PACT website regarding shared interests.

1 Scope Review

The on-line Request for Services Application (RSA) is structured to allow NHLBI to conduct the PACT scope review using information provided by the applicant in the first section of the RSA. The entire RSA is not required to be completed to perform a PACT scope review. RSAs that do not meet the PACT scope criteria will be rejected and completion of the RSA will not be required.

2 RSA Submission

After the RSA is assessed and found to be within scope, the applicant is invited the complete the RSA. PACT considers assessment of clinical plans and objectives in evaluation of the project for PACT services and evaluates the following elements of a request for translational development research services in an RSA:

- Investigator information (Biosketch, CV, etc.)
- Product information
- NHLBI programmatic scope
- Current funding
- Manufacturing information

- Production methodology
- Product development
- Regulatory information
- Timeline
- Budget
- Publications
- Organization/institution contact information

2.1 Objective Evidence of Scientific Merit

PACT is interested in whether the proposed cell product has undergone peer review and received funding. If the translational product development has been supported through a peer reviewed grant process, details should be provided:

- NIH Peer Reviewed Grant
- Peer Reviewed Grant (Other Organizations)

3 Review Criteria

The RSA will be reviewed for the following:

- Relevance to the NHLBI scientific mission and programmatic scope
- Qualification of the applicant and the collaborative team to carry out the proposed project to a successful completion
- Evaluation of the strengths and weaknesses of preliminary data, manufacturing feasibility, significance to the field, degree of innovation, and overall scientific merit of the application
- Detailed translational development plan summary
- Product specifications (proposed release criteria and potency assay)
- Supplemental manufacturing funding
- Demonstration of an adequate plan for eventual use of the cell therapy product in a clinical trials
- Proposed work supports regulatory pathway for this product

An external, independent Scientific Review Board (SRB) comprised of experts in the field of cell therapy will review the completed RSA. The SRB reviews will be incorporated into NHLBI's overall assessment and final decision.

4 PACT CPF Designation/Final Steps to Initiate Product Manufacture

When a RSA is approved, the NHLBI will solicit proposals via a Task Order Request for Proposal (TORFP) from the PACT CPFs. The CPFs will competitively bid to perform the work by submitting a task order proposal to NHLBI. NHLBI will evaluate proposals against the requirements of the TORFP and the award will be issued to the CPF in accordance with the requirements stated in the TORFP. After award, technical and material transfer agreements between the designated PACT CPF and the investigator's institution/ organization will be negotiated. The investigator will communicate directly with the CPF to finalize the services and production timeline. When both parties reach agreement, the project may begin.