PACT - Collaboration

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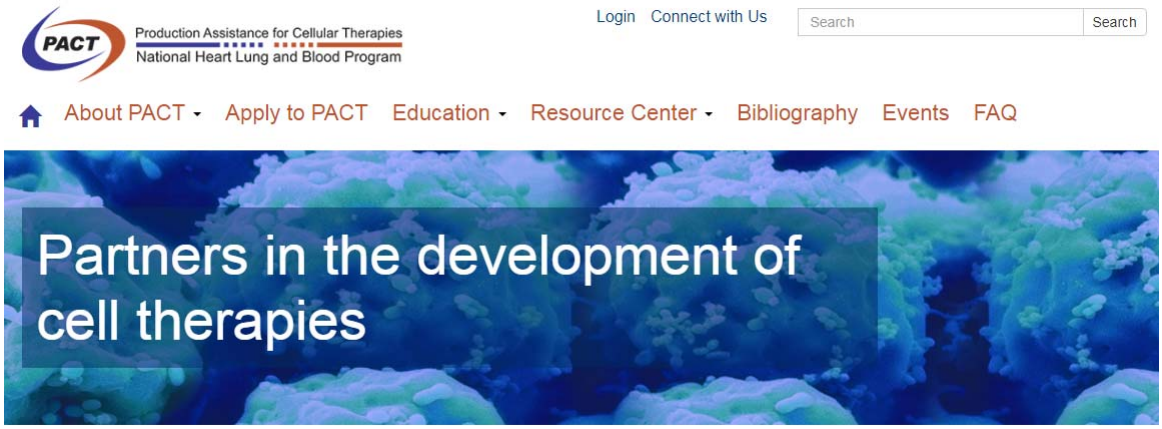


Collaboration

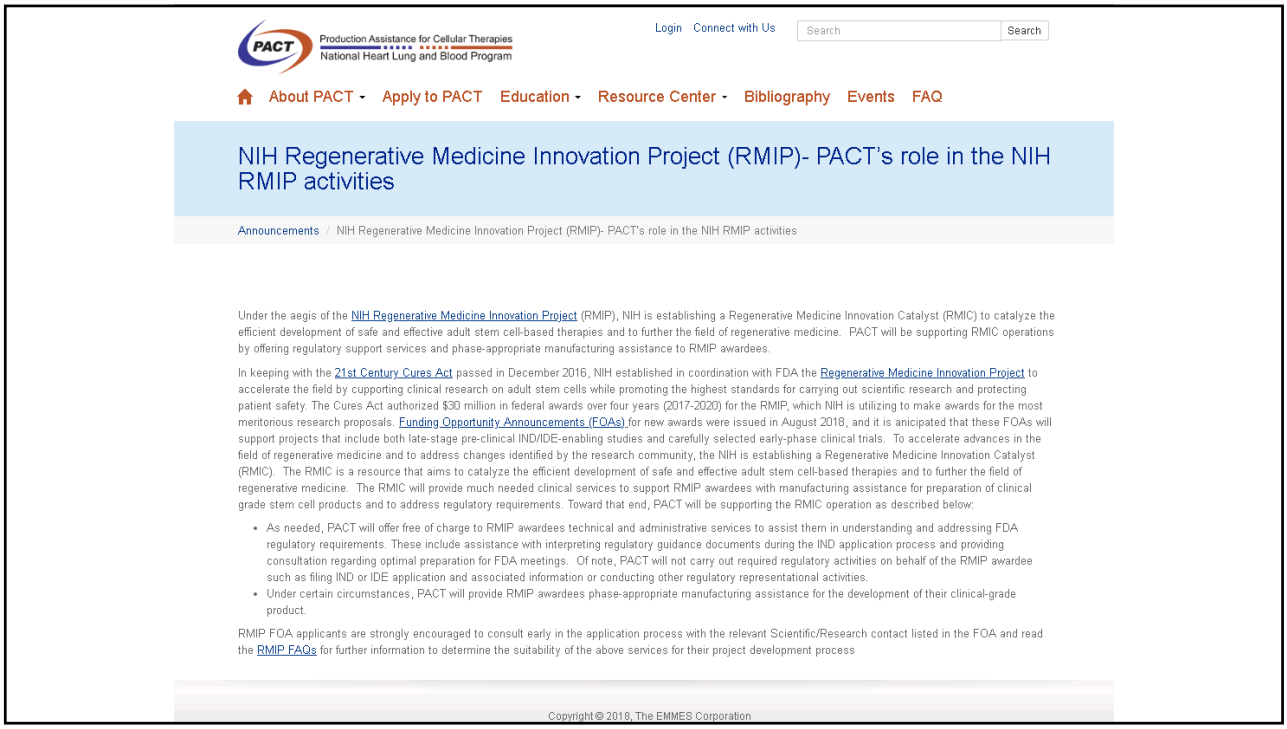
- NIH Regenerative Medicine Innovation Project (RMIP)
- NHLBI Cure Sickle Cell Initiative
- And with other NIH and NHLBI Programs

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

CURE SICKLE CELL.

NIH Regenerative Medicine Innovation Project (RMIP)

- 21st Century Cares Act passed in December 2016
- Support for clinical research on adult stem cells
- NIH released 4 funding opportunities, applications were due October 19, 2018
- PACT to offer RMIP awardees:
  - technical and administrative services to assist with FDA regulatory requirements
  - Phase-appropriate manufacturing assistance for the development of their clinical-grade product



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NIH Regenerative Medicine Innovation Project (RMIP)- PACT's role in the NIH RMIP activities

Announcements / NIH Regenerative Medicine Innovation Project (RMIP)- PACT's role in the NIH RMIP activities

Under the aegis of the NIH Regenerative Medicine Innovation Project (RMIP), NIH is establishing a Regenerative Medicine Innovation Catalyst (RMIC) to catalyze the efficient development of safe and effective adult stem cell-based therapies and to further the field of regenerative medicine. PACT will be supporting RMIC operations by offering regulatory support services and phase-appropriate manufacturing assistance to RMIP awardees.

In keeping with the 21st Century Cures Act passed in December 2016, NIH established in coordination with FDA the Regenerative Medicine Innovation Project to accelerate the field by supporting clinical research on adult stem cells while promoting the highest standards for carrying out scientific research and protecting patient safety. The Cures Act authorized $30 million in federal awards over four years (2017-2020) for the RMIP, which NIH is utilizing to make awards for the most meritorious research proposals. Funding Opportunity Announcements (FOAs) for new awards were issued in August 2018, and it is anicipated that these FOAs will support projects that include both late-stage pre-clinical IND/IDE-enabling studies and carefully selected early-phase clinical trials. To accelerate advances in the field of regenerative medicine and to address changes identified by the research community, the NIH is establishing a Regenerative Medicine Innovation Catalyst (RMIC). The RMIC is a resource that aims to catalyze the efficient development of safe and effective adult stem cell-based therapies and to further the field of regenerative medicine. The RMIC will provide much needed clinical services to support RMIP awardees with manufacturing assistance for preparation of clinical grade stem cell products and to address regulatory requirements. Toward that end, PACT will be supporting the RMIC operation as described below:

- As needed, PACT will offer free of charge to RMIP awardees technical and administrative services to assist them in understanding and addressing FDA regulatory requirements. These include assistance with interpreting regulatory guidance documents during the IND application process and providing consultation regarding optimal preparation for FDA meetings. Of note, PACT will not carry out required regulatory activities on behalf of the RMIP awardee such as filing IND or IDE application and associated information or conducting other regulatory representational activities.
- Under certain circumstances, PACT will provide RMIP awardees phase-appropriate manufacturing assistance for the development of their clinical-grade product.

RMIP FOA applicants are strongly encouraged to consult early in the application process with the relevant Scientific/Research contact listed in the FOA and read the RMIP FAQs for further information to determine the suitability of the above services for their project development process

Cure Sickle Cell Initiative

CURE SICKLE CELL.

- Launched in September 2018
- To accelerate the development of genetic therapies to cure sickle cell disease
- PACT will support the Cure Sickle Cell Initiative by enabling increased capacity to safely manufacture cellular therapy products through cell processing facilities capable of producing cGMP-grade genetically modified cells
- PACT currently providing regulatory support services to investigators
    - Pre-IND meeting package preparation
    - Pre-IND meeting support

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How is the Cure Sickle Cell Initiative contributing to scientific discoveries?

The Cure Sickle Cell Initiative builds on the legacy of NHLBI-supported research that has contributed to improving clinical care for patients who have sickle cell disease. It will also complement the Institute's broader sickle cell disease research investment, which includes basic, clinical, translational, and implementation science research.

The Initiative supports the following:

- **Enhanced clinical trial recruitment and establishment of transplant standards** to quickly and safety move clinical studies forward
- **Increased capacity to safely manufacture cellular therapy products** through the NHLBI Production Assistance for Cellular Therapies (PACT) program, which includes cell processing facilities to produce genetically modified cells so they can be safely used in patients

Collaboration

- PACT mission
  - to support the production and testing of novel cell therapies, particularly in relation to the strategic goals, objectives and research priorities of NHLBI https://www.nhlbi.nih.gov/about/strategic-vision

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CURE SICKLE CELL.