Cellular Therapy: Clinical Research Roadmap

Preclinical Studies

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Production Assistance for Cellular Therapies
National Heart Lung and Blood Program

Preclinical Studies

- Collect in vitro & in vivo data to demonstrate:
  - Potential clinical value in treatment of a specific disease
  - Safety
    - Lack of toxicity
    - Lack of adverse effects
  - Route and means of administration
  - Likely dose to be used initially in the clinical trial

Preclinical Studies – Help from PACT

- Help in the design of the pre-clinical study
- Review methods for preparing the product
- Selection of the appropriate animal model
- Selection of the appropriate in vitro tests
- Perform experiments to collect the data
- Help in selection of the product release criteria

Preclinical Studies

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Translational Development

- Preparation to manufacture the product under cGMP conditions
  - Coordination with Clinical Protocol
  - Selection of compliant reagents
  - Scale up of manufacturing
  - Selection of storage and shipment conditions
  - Generation of Standard Operating Procedures
  - Finalization of release tests
  - Training of manufacturing staff

Translational Development
Help from PACT

- Work with Investigators to determine product specifications
  - Dose, number of doses, volume, means of administration
- Develop manufacturing procedure using GMP compliant materials to meet these specifications
- Develop and test methods for storage and shipping
- Help with writing Chemistry, Manufacturing & Control (CMC) section of the IND

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Cell Product Validation

- Demonstrate that the manufacturing procedure reproducibly results in a product that meets release criteria
  - Criteria for acceptance are established before performing the validation
  - Normally 3 full scale runs are required
  - Performed as per SOP
  - All release criteria must be met
  - Contamination & cross-contamination must be prevented
  - Yields, purities etc must be within expected ranges

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Cell Product Validation
Help from PACT

- Assist in design of validation study
- Provide information/advice for pre-IND meetings with FDA
- Perform validation runs and release testing
- Perform validation of cell delivery system
- Provide validation report for CMC section

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With Free Help from PACT

- You can
  - Design and generate a pre-clinical data package for the IND submission
  - Obtain assistance in development of the product manufacturing and testing procedures
  - Generate a validation data section
  - Obtain a data package for the CMC section and assistance with preparation of the IND CMC

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