Stem Cell Center | QuintilesIMS

QuintilesIMS Stem Cell Center:
Core Services by CIRM Grant Types and Product Development Phases

I. TRAN: Candidate Translation

a. **Asset Development Strategy:** Technical due diligence of asset's clinical and technical benefits and risks, indication selection, biomarker strategy, clinical development plan (including TPP & protocol synopsis review and development).
b. **Regulatory Services:** Pre-IND readiness assessment, briefing package preparation and submission.
c. **Process Development, CMC, and Manufacturing:** Product Manufacturing (non-GMP), quality control assays (Development & Validation) and quality assurance.
d. **Preclinical // non-clinical:** Proof of concept, biodistribution and pilot studies, safety assessments, animal model development, immunohistochemistry // in vivo imaging.
e. **Commercial:** market sizing and forecasting

II. CLIN1: IND Enabling Research

a. **Asset Development Strategy:** Technical due diligence of asset's clinical and technical benefits and risks, protocol development, protocol review, clinical trial modeling and simulation.
b. **Regulatory Services:** IND readiness assessment, briefing package preparation, publication and maintenance.
c. **Process Development, CMC, and Manufacturing:** Process development, quality control assays (development & validation) and quality assurance product stability / shipping and technology transfer.
d. **Pre-clinical // Non-clinical Research:** Proof of Concept studies, safety assessments & distribution studies (GLP), immunochemistry, delivery mechanism evaluation and molecular pathology.
e. **Commercial:** Patient reported outcomes, pricing & market access, commercial assessment, evidence generation planning & design, and launch preparation.

III. CLIN2: Post-IND Submission / Approval

a. **Asset Development Strategy:** Technical due diligence of asset's clinical and technical benefits and risks, clinical trial modeling and simulation.
b. **Regulatory Services:** BLA preparation, generation, publication and maintenance
c. **Process Development, CMC, and Manufacturing:** Product manufacturing (GMP), process scale-up, product stability / shipping and technology transfer.
d. **Non-clinical:** Safety assessments & comparability studies (GLP).
e. Clinical Study Conduct: Complete set of CRO services
f. **Commercial:** same as section II e

IV. Other Services

a. **Integrated Project Management** (across all grant types)
b. Grant Application Services
    i. **Grant Evaluation:** Comprehensive evaluation of draft grant application and presentation of recommendations for addressing gaps and issues as well as translation of budgets into CIRM grant application format

ii. **Grant Co-Development:** Development of robust grant outline and application based on agreed strategy as well as translation of budgets into CIRM grant application format

Comprehensive list of SCC Services:

I. TRAN: Candidate Translation

a. Asset Development Strategy
   i. Technical Due Diligence
   ii. Indication Selection
   iii. Target Product Profile (Development & Evaluation)
   iv. Biomarker Strategy
   v. Regulatory Strategy
   vi. Clinical Development Plan
   vii. Protocol Synopsis Development
   viii. Protocol Synopsis Review

b. Regulatory Services
   ix. Pre-IND Readiness Assessment
   x. Pre-IND Meeting Briefing Package
   xi. Pre-IND Meeting Coordination

c. Process Development, CMC, and Manufacturing
   xii. Product Manufacturing (non-GMP)
      1. SOP / Batch Drafting
      2. Reagent Sourcing
      3. Cell Expansion, Harvest, Fill & Fish
   xiii. Quality Control Assays (Development & Validation)
   xiv. Quality Assurance

d. Preclinical // non-clinical
   xv. Mechanism of Action // Proof of Concept Studies (non-GLP_
   xvi. Pilot Studies (non-GLP)
   xvii. Safety Assessment (non-GLP)
      1. Toxicology Studies
      2. Tumorigenicity Studies
   xviii. Animal Model Development
   xix. Biodistribution Studies (non-GLP)
      1. Pharmacodynamics / Pharmacokinetics
   xx. Immunochemistry
   xxi. In Vivo Imaging / Cell Tracking

e. Commercial
   xxii. Market sizing and forecasting

II. CLIN1: IND Enabling Research

a. Asset Development Strategy
   i. Technical Due Diligence
   ii. Protocol Development
   iii. Protocol Review
   iv. Clinical Trial Modeling and Simulation

b. Regulatory Services
    i. IND Readiness Assessment
    ii. IND Submission
    iii. IND Generation
    iv. IND Publication
    v. IND Maintenance
c. Process Development, CMC, and Manufacturing
    i. Process Development
    ii. SOP / Batch Records Drafting
    iii. Reagent Sourcing
    iv. Cell Expansion, Harvest, Fill & Finish
    v. Quality Control Assays (Development & Validation)
    vi. Quality Assurance
    vii. Product Stability / Shipping
    viii. Technology Transfer
d. Pre-clinical // Non-clinical Research
    i. Mechanism of Action / Proof of Concept Studies (GLP)
    ii. Safety Assessment (GLP)
        1. Toxicology Studies
        2. Tumorigenicity Studies
        3. Comparability Studies
    iii. Biodistribution Studies (GLP)
        4. Pharmacodynamics / Pharmacokinetics
        5. Cell Fate
    iv. Delivery Mechanism Evaluation
    v. Immunochemistry
    vi. Molecular Pathology Studies
e. Commercial:
    i. Patient Reported Outcomes: Develop conceptual model and PRO PRO instruments to capture the patient perspective in clinical development to differentiate the product and optimize pricing and reimbursement
    ii. Pricing & Market Access: Conduct research to understand payer and provider landscape for optimal access and reimbursement for the therapy
    iii. Commercial Assessment: Data-driven evaluation framework to assess an asset's commercial potential to support launch and commercial planning, business development activities, and/or portfolio strategy
    iv. Evidence Generation Planning and Design: Define and develop evidence to demonstrate the added value of product over standard of care in economic terms
    v. Launch Preparation: Comprehensive understanding of patient journey, treatment algorithm, and buying process to identify critical patient and physician segments to target through customized launch and promotional strategies

III. CLIN2: Post-IND Submission / Approval

a. Asset Development Strategy
    i. Technical Due Diligence
    ii. Clinical Trial Modeling and Simulation

b. Regulatory Services
    i. Regulatory Meeting Support
        1. Pre-submission Briefing Package
        2. Pre0Submission Meeting Coordination
    ii. BLA Generation
    iii. BLA Publication
    iv. BLA Maintenance
c. Process Development, CMC, and Manufacturing
    i. Product Manufacturing (GMP)
    ii. Process Scale-up
    iii. Product Stability / Shipping
    iv. Technology Transfer
d. Non-clinical
    i. Safety Assessments & Comparability Studies (GLP)
e. Clinical Study Conduct
    i. Medical Writing
    ii. Site Selection & Management
    iii. Vendor Management
    iv. Data Management
    v. Biostatistics
    vi. Supply Chain
    vii. Medical Monitoring
    viii. Safety Monitoring
    ix. Quality Assurance
    x. Project Management
f. Commercial – same as section II e

IV. Other Services

a. Integrated Project Management across all grant types
b. Grant Application Services
    i. Grant Evaluation: Comprehensive evaluation of draft grant application and presentation of recommendations for addressing gaps and issues
    ii. Grant Co-Development: Development of robust grant outline and application based on agreed strategy