Shyam Patel, Ph.D. & Sohel Talib, Ph.D.

INFR5 Phase 1 RFA Informational Webinar

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Email: bd@cirm.ca.gov

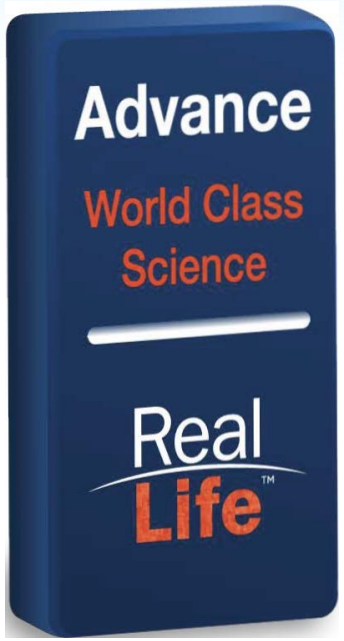
CIRM
CALIFORNIA'S STEM CELL AGENCY

Mission Statement

OUR MISSION

Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world

5-Year Strategic Summary



- Develop Competency Hubs
- Build Knowledge Networks

- Advance therapies to marketing approval
- **Create a manufacturing partnership network**
- Expand Alpha Clinics Network
- Create Community Care Centers of Excellence

- Build a diverse and highly skilled workforce
- Deliver a roadmap for access and affordability

California CGT Manufacturing Network

CIRM-Funded Academic GMP Facility Network

Industry Partners

- Manufacturing services
- Resources
- Investment and Partnerships

INDUSTRY ALLIANCE PROGRAM

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- **Accelerate**
  and de-risk path to commercialization
- **Advance**
  standards and quality by design
- **Build**
  manufacturing leadership and workforce

Potential Functions of the Cell & Gene Therapy Manufacturing Network

- World-class expertise across range of manufacturing and analytical technology platforms
- Support manufacturing of therapies for rare diseases
- Accelerate and de-risk late-stage and commercial manufacturing of therapies
- Establish standards for quality or accreditation of manufacturing facilities
- Build inclusive workforce entry and advancement opportunities in technical and leadership career pathways in partnership with EDUC programs and industry stakeholders

Funding Academic Facilities to Build Network

Bi-Phasic Funding Opportunity

Year 1 | Year 2 | Year 3 | Year 4 | Year 5 | Year 6 | Year 7

Phase 1

Program Budget: $20M
Applicant: Academic Cell/Gene Therapy GMP Manufacturing Facility
Collaborators: Optional
Goal: Individual facility enhancements

Phase 2

Program Budget: $60M
Applicant: Academic Cell/Gene Therapy GMP Manufacturing Facility
Collaborators: Required
Goal: Network-wide scaling of enhancements, specializations, and training programs

CIRM-Coordinated Steering Committee of Awardees & External Participants

CIRM-Funded Academic GMP Facility Network ⟷ **Industry Partners**

INFR5 Phase 1 RFA - Summary

INFR5 Phase 1 RFA Objective

- Fund California non-profit GMP manufacturing facilities to address cell and gene therapy manufacturing bottlenecks at their individual facilities; external partnerships encouraged.

INFR Phase 1 RFA Budget

- **$20M Total**

Phase 1 Awards

- **Max Award Amount & Duration:** $2 Million; 2 years
- **Co-Funding:** 20%
- **Allowable costs:** Direct Project Costs & Direct Facilities Costs

Who can apply?

- California non-profit organization with GMP manufacturing facility

INFR5 Phase 1 Application

- **Application Due Date:** January 24, 2023 at 2pm

- **Application Components:**
  - Online application (eligibility, budget, budget justification, etc.)
  - Project Proposal
  - Other Support
  - Letters of Support
  - Biosketches
  - Quotes & Other Budget Data
  - Licenses/MTAs

INFR5 Phase 1 RFA – Eligibility

Organization Eligibility

- California organization with cell and gene therapy GMP manufacturing facility
- Track record of performing GMP manufacturing activities for cell and gene therapy candidates
- Must demonstrate co-funding support

Program Director Eligibility

- Director of GMP facility or equivalent
- Commit 30% average effort on project
- Authorized to conduct the proposed activities, participate in steering committee

INFR5 Phase 1 RFA – Award Activities

Applications are expected to be responsive to all three categories of activities defined below but the scope of proposed activities in each category and the respective budget allocations are at the discretion of the applicant.

- Implement Quality-driven enhancements to de-risk manufacturing
- Demonstrate progress on specialization areas
- Develop inclusive workforce development programs for technical and leadership career pathways

Award Activities

Implementation of quality-driven enhancements that de-risk and accelerate early **and** late-stage process development and GMP manufacturing of cell and gene therapies.

Potential enhancements could include but are not limited to*:

- Implementation of industry quality standards and quality-by-design principles
- Facilitation of project transition to manufacturing partners for late-stage manufacturing support
- Mitigation of capacity or expertise gaps at applicant facility
- Development of electronic records systems and technology transfer package templates
- Enhancement of analytical capabilities

*Refer to RFA and Application Proposal Template for additional examples

Award Activities

Specialization in one or more functional areas that overcome bottlenecks in the development and delivery of cell and gene therapies.

Specialization areas could include but are not limited to*:

- Innovative manufacturing models for cell and gene therapies for rare diseases
- Application of QbD in deep product characterization, identification of CQAs and CPPs, and process control strategies
- Development of data capture systems and analytics to support correlation of product characteristics to clinical outcomes
- Development of novel manufacturing process analytical technologies and manufacturing technologies

*Refer to RFA and Application Proposal Template for additional examples

Award Activities

Workforce development programs for technical and leadership positions, preferably in partnership with CIRM EDUC-funded programs, California cell and gene therapy industry stakeholders, and California academic institutions.

Workforce development activities include but are not limited to*:

- Development of paid training and/or certification programs for technical positions
- Recruitment, technical training and leadership mentoring programs for facility leadership positions

*Refer to RFA and Application Proposal Template for additional examples

CIRM Encourages Collaborative Proposals

- Proposals that leverage internal and external collaborations are strongly encouraged, particularly with California-based entities. Collaborators may include but are not limited to:
  - CIRM EDUC and INFR Awardees
  - INFR5 Applicants
  - Academic Institutions
  - Industry CDMOs, technology developers, supply chain solutions, consultants, biotechs, large biopharma, etc.
  - National Organizations (i.e. NIH, NIST, NIIMBL, ARMI, CMaT, ISCT, etc.)

CIRM does not require collaborations with any specific organizations

Budget Considerations – Unallowable Costs

- Costs associated with process development or manufacturing activities for individual projects
- Workforce development activities located outside of California
- Construction or renovation of physical facilities
- Activities already budgeted or paid for under a prior, existing or future CIRM award
- Equipment costs exceeding 5% of the direct project costs

Diversity, Equity, Inclusion

Addressing the Needs of Underserved Communities in CIRM-Funded Projects

- How the proposed project activities will improve access to cell and gene therapies by underserved and disproportionately affected populations
- Participation in workforce development programs by underserved populations
- Completed or proposed activities to bring diverse and inclusive perspectives and experience to the project

Knowledge Sharing

- Describe plan to capture and disseminate relevant know how, operational data, processes, expertise and guidance within network

- If planned, describe data management processes that will support CIRM TRAN/CLIN awardees to execute on their respective data management & sharing plans

Applications - GWG Review Criteria

Value Proposition of the Project

- Critical manufacturing/analytical bottlenecks addressed?
- Leveraging of collaboration opportunities?

Project Well Planned and Designed

- Are the proposed activities adequate (assessed by category)?

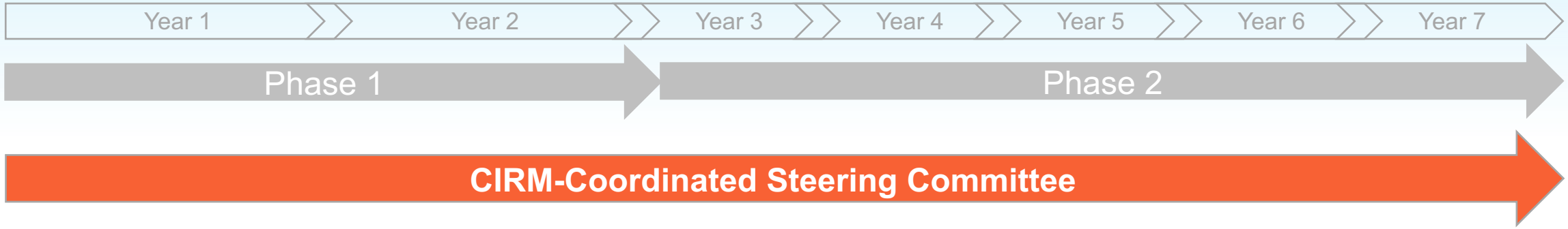
Project Feasibility

- Can the organization and team achieve the proposed project objectives?

Serving the needs of underserved and disproportionately affected communities

Refer to INFR5 RFA for complete list of review criteria

Steering Committee Drives Network Functions



CIRM will coordinate Steering Committee of awardees, California industry partners & national stakeholders to facilitate:

- Identification and adoption of standards, protocols and best practices across the network and potential criteria for facility accreditation
- Mitigation of capacity and expertise gaps across participating sites
- Collaborative planning for Phase 2 proposals
- Development of systems and processes for sharing information and resources between network participants
- Collaborative development and implementation of workforce training programs

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