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Agenda Item #7
Consideration of Recommendations from the Grants Working Group on the Preclinical Development Award Applications

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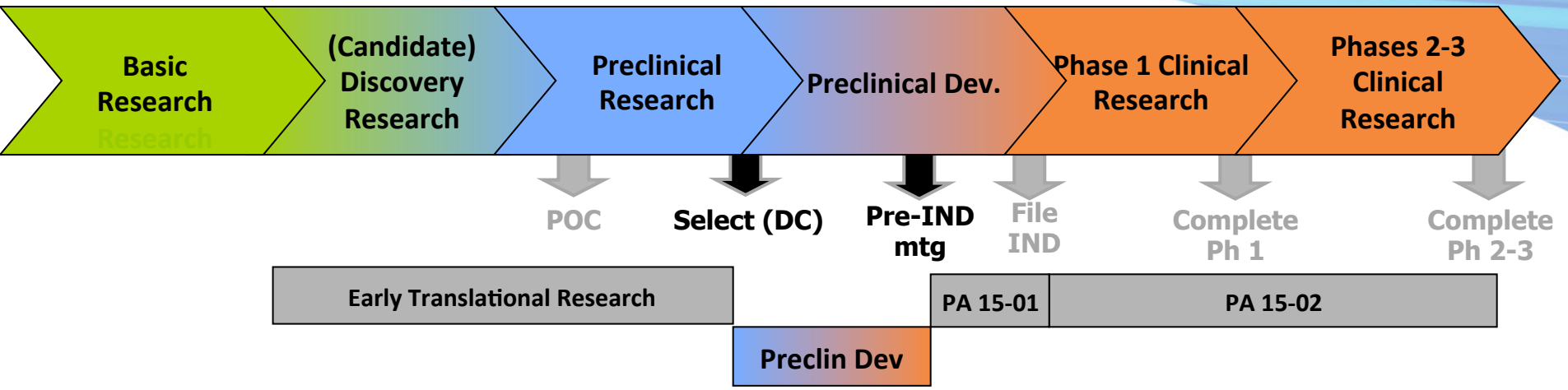
PCD Awards: Purpose



- Fund early preclinical development of successful stem-cell based translational projects.
- Upon successful completion of award: ready for PA 15-01, Late-Stage Preclinical Development.
- Final RFA issued under the “1.0” process.

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PCD Awards: Eligibility

Two key scientific eligibility criteria:

Development Candidate (DC)

- ✓ A single DC that derives from or targets stem cells.

Readiness

- ✓ Convincing, reproducible disease-modifying activity in relevant models
- ✓ Preliminary assessments of safety, mechanism-of-action
- ✓ Reproducible, scalable research-grade production of candidate

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PCD Awards: Objective and Scope

Objective: Carry out activities needed to conduct a well-prepared pre-IND meeting with the FDA at the end of the award

In-Scope Activities:

- GMP manufacturing process development
- Dose, regimen, route of administration studies
- Pharmacokinetic, pilot safety and mechanism of action studies
- Selection of target indication
- Preparation of clinical development plan, draft protocol
- Conduct of pre-IND meeting with FDA

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PCD Awards: Funding Information

Award Information

- Up to $40M total, 5 to 8 awards
- 30 months, up to $5-8M justifiable total project costs

Co-Funding

- 1:1 matching funding required if DC not identified with prior CIRM funding

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PCD Awards: Review Criteria Highlights

1) **Should the proposed therapeutic be developed?**
   - Significance: Competitive with standard of care
   - Scientific Rationale: Potential for clinical benefit in targeted indication

2) **Can the proposed plan achieve the RFA Objective?**
   - Readiness: Convincing preclinical efficacy, preliminary safety and mechanism-of-action, reproducible production of DC
   - Design and Feasibility: Project plan complete, timeline realistic
   - Assets, Collaborations, Environment: MTAs, patents, contracts, equipment and facilities.

3) **Is this the right team to execute the plan?**
   - PI and Team: Experience in team leadership, preclinical development

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Scoring by Grants Working Group (GWG)

- Tier 1 (scores ≥ 75): recommended for funding
- Tier 2 (scores 65-74): moderate quality or consensus on merit not reached
- Tier 3 (scores ≤ 64): not recommended for funding

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PCD Priorities for Programmatic Consideration

The following priority areas were called out in the RFA:

- Cell therapies, especially if derived from pluripotent stem cells or directly reprogrammed cells
- Potentially transformative approaches to unmet medical needs
- 25% co-funding (if DC identified using CIRM funding)
- 1:1 industry co-funding (if DC not identified using CIRM funding)

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CIRM Recommendations and Rationale

Tier 1 Applications: Fund

- CIRM supports the GWG assessment
- Five awards, $15.8MM
- $3.25MM co-funding

Tier 2 and Tier 3 Applications: Do not fund

- Applicants may consider submitting improved proposals, addressing review comments
- Opportunities:
  - CIRM 2.0 Translational/Early Preclinical program announcement (anticipated for Summer 2015)
  - PA 15-01 Partnering Opportunity for Late Stage Preclinical Projects (Open Now)

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