

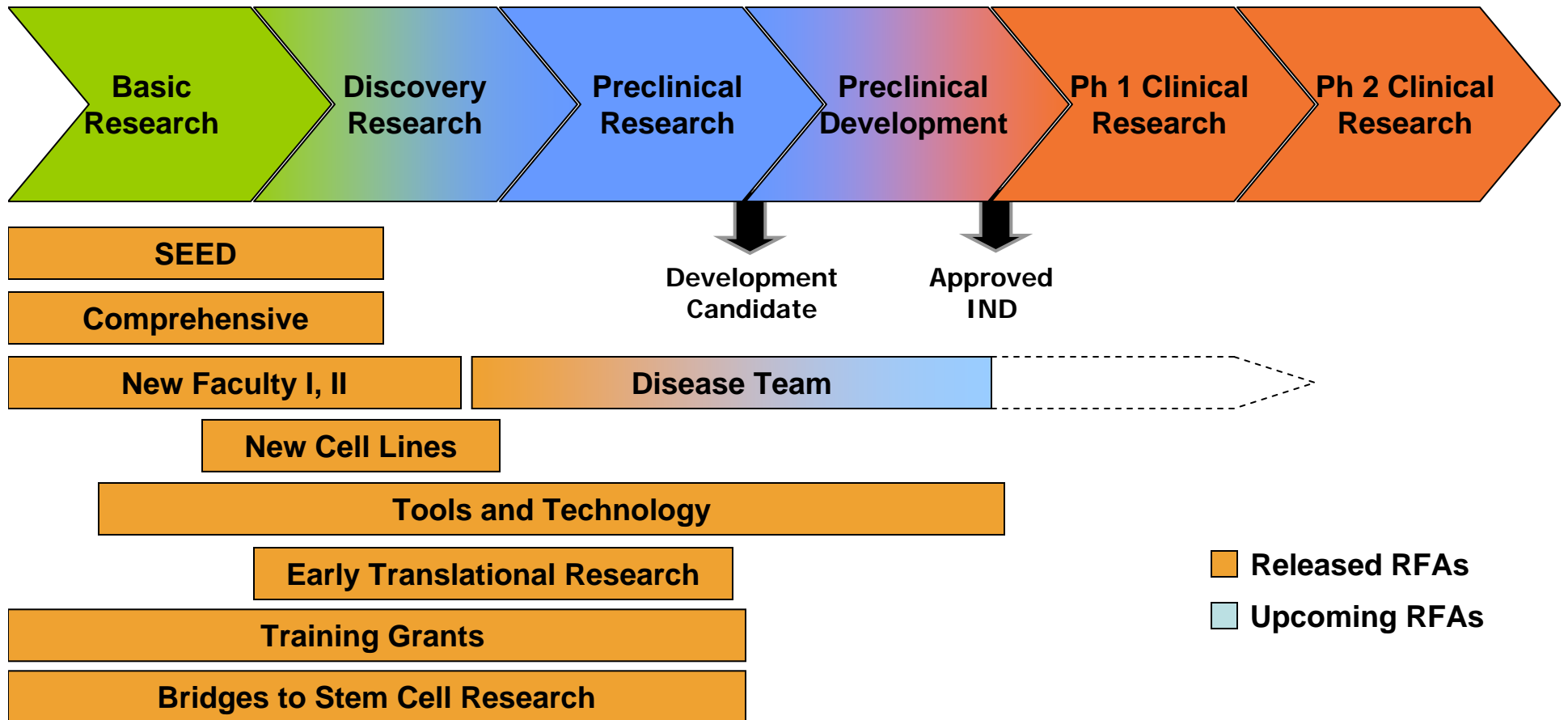
## **CIRM RFA Pipeline**

**Marie Csete MD, PhD**  
**Chief Scientific Officer**  
**[mcsete@cirm.ca.gov](mailto:mcsete@cirm.ca.gov)**

**Pat Olson PhD**  
**Director of Scientific Activities**  
**[polson@cirm.ca.gov](mailto:polson@cirm.ca.gov)**

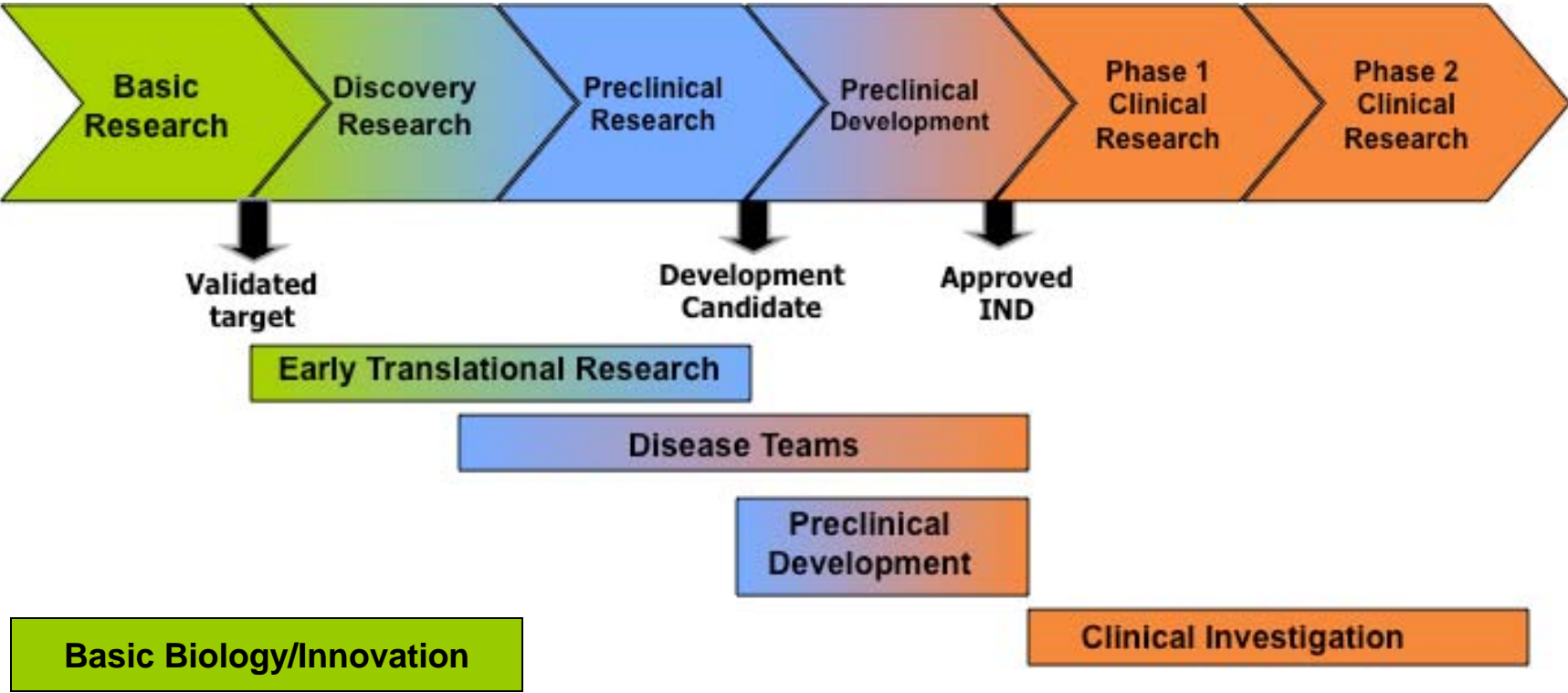
# Moving the Pipeline Forward

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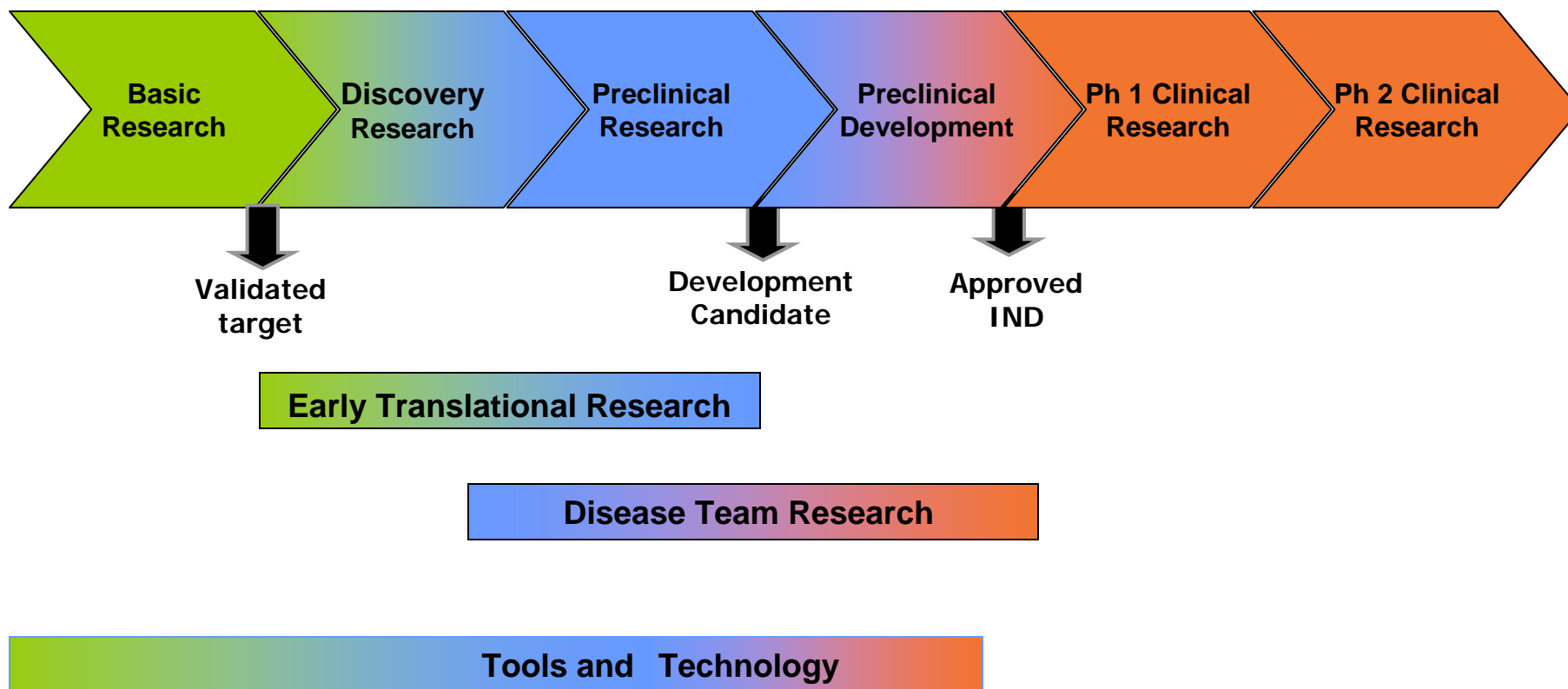
# Core RFA Program

Supplement As Needed



# Recent/Upcoming RFAs: Commercial Sector Opportunities

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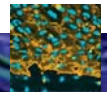
# NOW: Early Translational Research

## Development candidates

- A cell therapy derived from stem cells
- Biologic or small molecule where use of stem cells advantageous, required

## Bottlenecks

- Immunogenicity/tolerance
- Non-invasive assays for teratomas, tumorigenicity
- Methods to preserve chromosomal integrity in culture
- Methods to track migration/integration
- Pre-clinical development bottlenecks on later grants: (We say what we don't want)



# Eligibility: Early Translation Research

- PI Eligibility
  - M.D. and/or Ph.D. or equivalent degree
  - Authorized by the applicant institution to conduct the proposed research in California
  - Minimum 10 % effort
- PI may apply for an Early Translational Research Award OR a Disease Team Research Award



# What's coming up?

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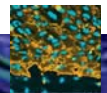
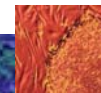
- Disease Team Research Awards
  - Major investment by our Board
  - Essential for success: Industry involvement
  - Milestone driven
  - Go/No-go Decisions
  - Project management
  - Oversight committees
  - Aggressive goal: IND filing



# In Development: Preclinical Development

- IND enabling toxicology
- IND enabling pharmacology, PK/PD
- Protocol development, clinical site recruiting, IRB approvals
- GMP Manufacturing processes
- IND preparation and filing

Potentially folded in to Disease Teams

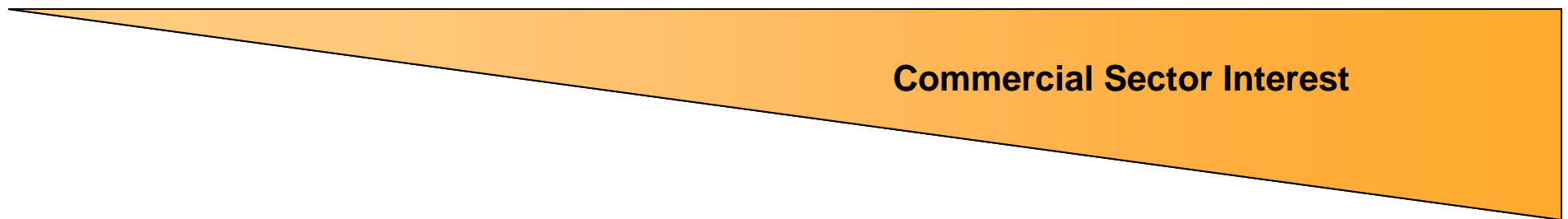
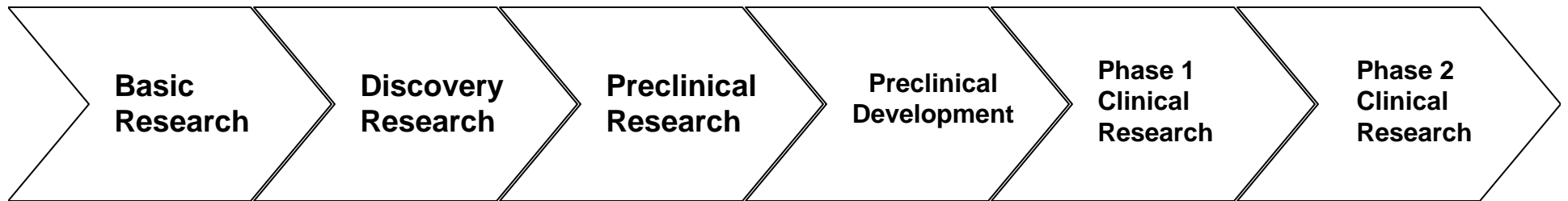




# Moving Discoveries into the Clinic: Engagement of the Commercial Sector

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“Valley of Death”



Grants (and Loans)

