

# RAPID Funding Opportunity: Concept Overview

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*Rare-disease Acceleration through Platform Innovation  
and Delivery*

C I R M

CALIFORNIA INSTITUTE FOR  
REGENERATIVE MEDICINE

# RAPID | Outline

1. CIRM Impact Goal
2. Background
3. Objective
4. Scope
5. Structure
6. Timeline
7. Request for Approval

# SAF Goal 3 | Recommendations

## Goal 3 - Advance 4 – 7 rare disease projects to BLA

### Accelerate Current Rare Disease Therapy Pipeline

- **Increase and scale CLIN4 funding** to comprehensively address BLA readiness gaps in manufacturing, clinical/non-clinical research, and pre-commercialization\*

### Pilot Platform-Based Therapy Development

- **Implement pilot platform-based approach** for gene therapy development using life-threatening monogenic neurological disorders as a test case

\*Not exclusive to rare disease, but majority of programs near BLA in CIRM's portfolio are in rare disease

# SAF Goal 3 | Recommendations

**Pilot Platform-Based  
Rare Disease Therapy Development**

# Problem Statement

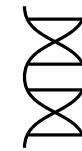
**10,000+ Unique Rare Diseases**



**1 in 10** Americans  
~30M people



**> 50%** are  
childhood diseases



**80%**  
genetic diseases

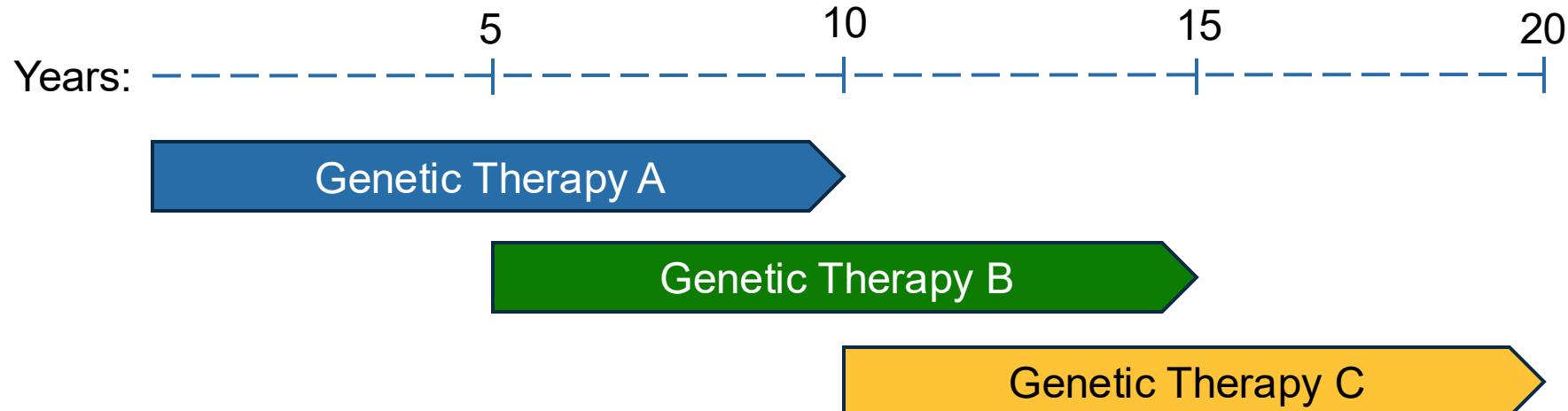
**95%** of rare diseases have **no approved therapy**

- Development of genetic therapies for rare diseases is **slow, expensive, and unsustainable**
- Current solutions are **fragmented, inefficient, leading to duplication and limited scalability**

# Therapeutic Development is a long and costly process

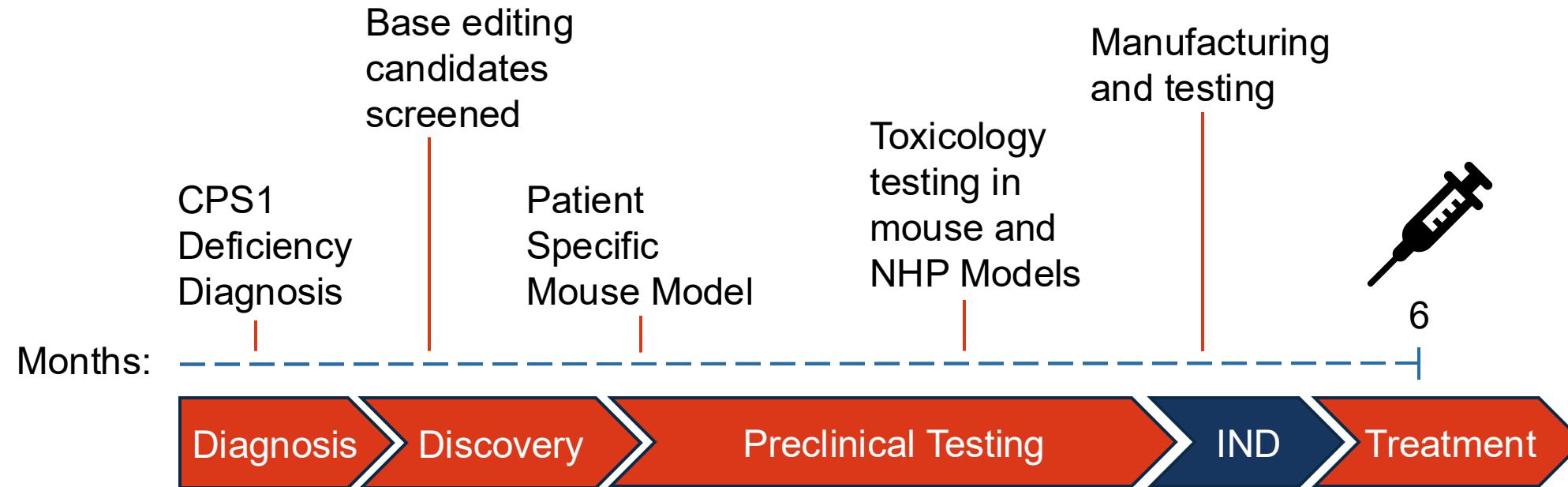


The next therapy in the pipeline takes roughly the same amount of time, resources and cost to develop.



# What if the development timeline is accelerated?

Baby KJ's gene editing therapy was developed and administered within 6 months of diagnosis



But a therapy for the **next infant** would need to **repeat this process**

# What if you could leverage common aspects of therapies?

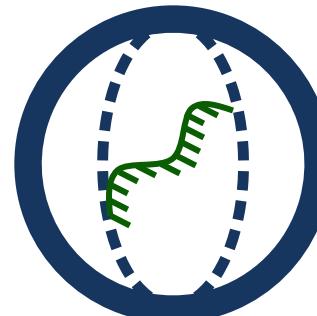
**Platform-based therapies:** Multiple related therapies for multiple indications that are rapidly advanced to patients by leveraging common components, technologies, data and resources

## Illustrative Examples

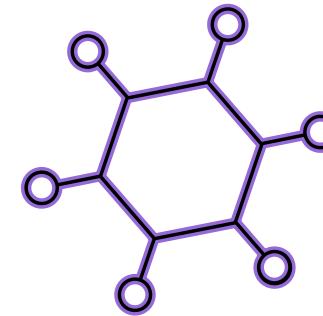
### Non-Viral Gene Editing



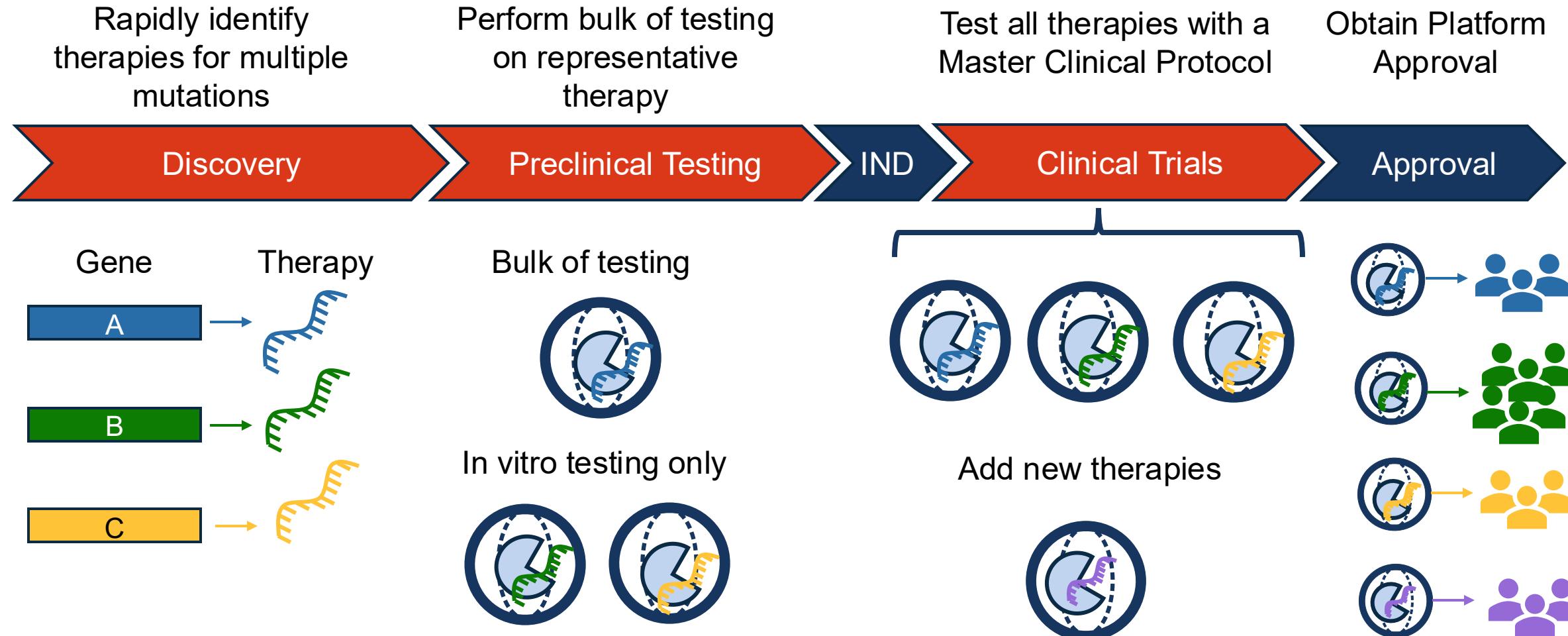
### RNA-based Therapies



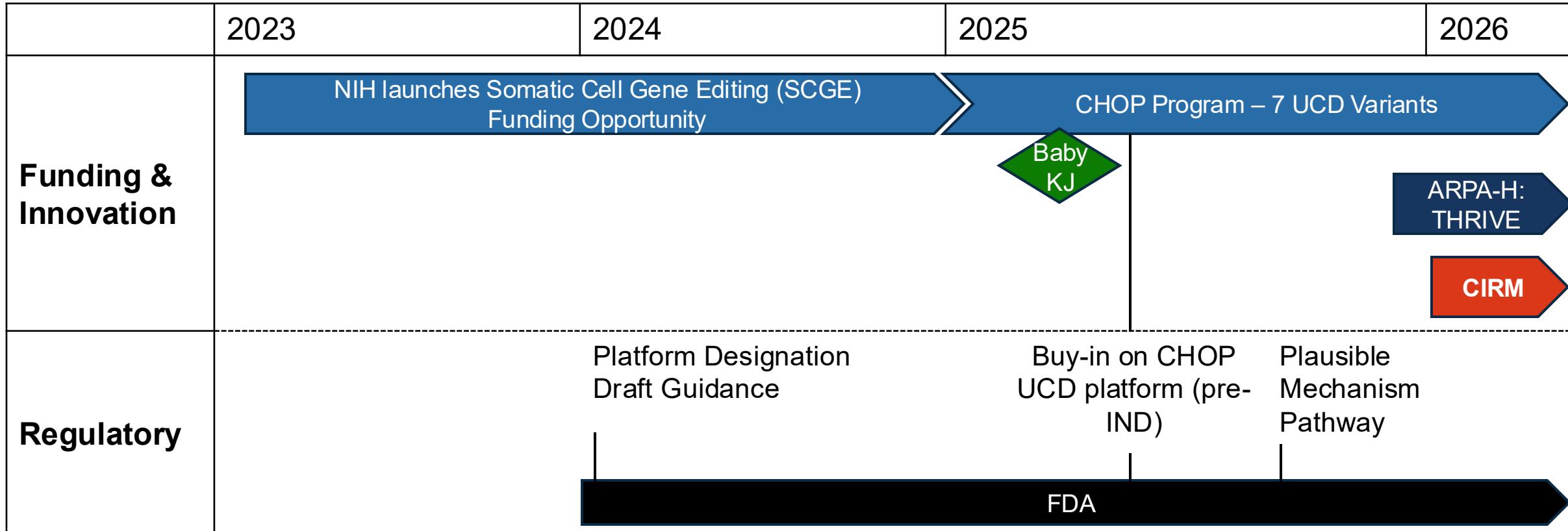
### AAV Gene Delivery



# Platform-Based Therapies Can Reach More Patients



# Paving the Way for Platform-Based Therapies



We can advance platform-based therapies from promise to practice by building robust evidence across rare diseases and technologies

# CIRM Rare Disease Platform: Program Goals

## Goals

### Scientific & Regulatory Innovation

1. Demonstrate platform efficiencies for multiple genetic therapies in multiple disease areas
2. Continually advance innovative regulatory and technological solutions
3. Build an evidence-base for genetic therapy platform safety and efficacy

### Patient Impact

4. Ensure that supported therapies reach patients as quickly as possible
5. Model a path to scalable development so more patients have access to therapies

# Rare-disease Acceleration through Platform Innovation and Delivery: RAPID

## Objective

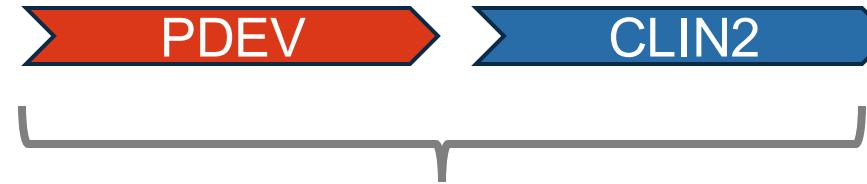
Create a **scalable model** to rapidly deliver transformative, **platform-based genetic therapies** to patients with rare disease

# Opportunities to Augment CIRM Rare Disease Portfolio

*TRAN / PDEV / CLIN1 / CLIN2*

CIRM's funding programs are **not currently designed to accelerate innovative high-risk platform approaches**

# Current Programs Not Designed for Platforms



Traditional PDEV to CLIN2	
<b>Therapy development</b>	<b>Single</b> candidate
<b>Disease impact</b>	<b>Single</b> indications
<b>Trial design</b>	Traditional trial design; <b>single</b> IND
<b>Non-clinical testing</b>	<b>Full</b> suite for candidate
<b>Manufacturing &amp; testing</b>	<b>Full</b> suite for candidate
<b>CIRM funding progression</b>	New CLIN2 application

# RAPID Accelerates Platform-based Trials to Clinic

*Linking PDEV & CLIN2 enables rapid transition from IND to FIH clinical trials*

RAPID	
<b>Therapy development</b>	<b>Multiple</b> candidates
<b>Disease impact</b>	<b>Multiple</b> related indications
<b>Trial design</b>	<b>Master protocol</b> trial
<b>Non-clinical testing</b>	Full suite for lead; <b>reduced</b> for basket
<b>Manufacturing &amp; testing</b>	Full suite for lead; <b>reduced</b> for basket
<b>CIRM funding progression</b>	<b>Accelerated</b> path to clinic

# RAPID Validation Awards

**Objective:** Accelerate platform-based therapies to clinical proof of concept

Pre-IND → “RAPID Validation” → Outcome: Trial completion

## Examples:

- Non-viral liver-targeted base editing therapies for metabolic disorders
- AAV-based gene delivery for neurodevelopmental diseases

## How these awards will achieve program objective:

- Demonstrate promise to practice for platform-based therapy development
- Build evidence base for safety and efficacy of platform-based therapies

# RAPID Validation Awards

**Objective:** Accelerate platform-based therapies to clinical proof of concept

Pre-IND

“RAPID Validation”

Outcome: Trial completion

<b>Candidates</b>	In vivo genetic therapies for rare genetic diseases
<b>Platform</b>	Demonstrates accelerated and resource-efficient development
<b>Entry Criteria</b>	Pre-IND conducted
<b>Outcome</b>	Completion of clinical trial for at least 3 candidates
<b>Max Duration</b>	6 years
<b>Award Cap</b>	Not specified
<b>Minimum Co-Funding</b>	Not required
<b>Applicant</b>	CA organizations

# RAPID Innovation Awards

**Objective:** Foster innovative regulatory and technology solutions toward clinical trials

Declared Candidates → “RAPID Innovation” → Outcome: IND clearance

## Examples:

- Non-viral gene delivery technologies for CNS
- Next generation gene editing therapies developed with novel in vitro models

## How these awards will advance platform goals:

- Accelerate novel genetic engineering and tissue-specific delivery technologies
- Advance regulatory strategies for emerging platforms

# RAPID Innovation Awards

**Objective:** Foster innovative regulatory and technology solutions toward clinical trials

Declared Candidates

“RAPID Innovation”

Outcome: IND clearance

<b>Candidates</b>	In vivo genetic therapies for rare genetic diseases
<b>Platform</b>	Demonstrates accelerated and resource-efficient development
<b>Entry Criteria</b>	FDA INTERACT requested or completed
<b>Outcome</b>	Master Protocol IND cleared for at least 3 candidates
<b>Max Duration</b>	3.5 years
<b>Award Cap</b>	Not specified
<b>Minimum Co-Funding</b>	Not required
<b>Applicant</b>	CA organizations

# RAPID | Program Budget

The RAPID Program will utilize a defined program budget of \$100M for 2 annual funding cycles with future opportunity to reinvest recovered funds in new projects

The RAPID Program will reserve an award supplement budget to further accelerate funded projects, subject to a CIRM request and approval process

## \$100M Program Budget

	FY 26-27	FY 27-28	Future Years
<b>Total Funds</b>	\$55M	\$45M	<i>Program may be reopened if RAPID funds are recovered, subject to ICOC Research Budget approval</i>
<b>New Awards</b>	Up to \$50M	Up to \$45M	
<b>Program Supplement</b>	\$5M	Additional funds TBD	
<b>Awards/Year</b>	2 – 3	2 – 3	

# RAPID | Application and Review

## **RAPID will require consultations prior to submission**

- Ensures applicants meet platform scope and readiness eligibility requirements
- Provides guidance on application requirements

## **RAPID will utilize existing application & GWG review processes**

- Requires submission of complete application
- If necessary, GWG conducts positive selection to filter application volume
- Numerical 1-100 GWG scoring system

# RAPID | Award Management

CIRM will utilize existing award management mechanisms to ensure RAPID projects adhere to the Program Objective and Expected Outcomes

## Proactive Award Management

- Quarterly reports and CIRM progress calls
- CIRM included in FDA meetings

## Performance Driven Operational Milestone (OM) Structure

- Funds disbursed upon meeting OM success criteria
- OM delays >4 months trigger evaluation and potential action
- Pre-defined suspension events halt funding until resolved

# RAPID | Knowledge & Data Sharing Requirements

## PDEV Awardee Knowledge Network

- Advance projects by facilitating real-time sharing of study designs, data, resources, and regulatory experience among CIRM awardees

## Public Data Sharing

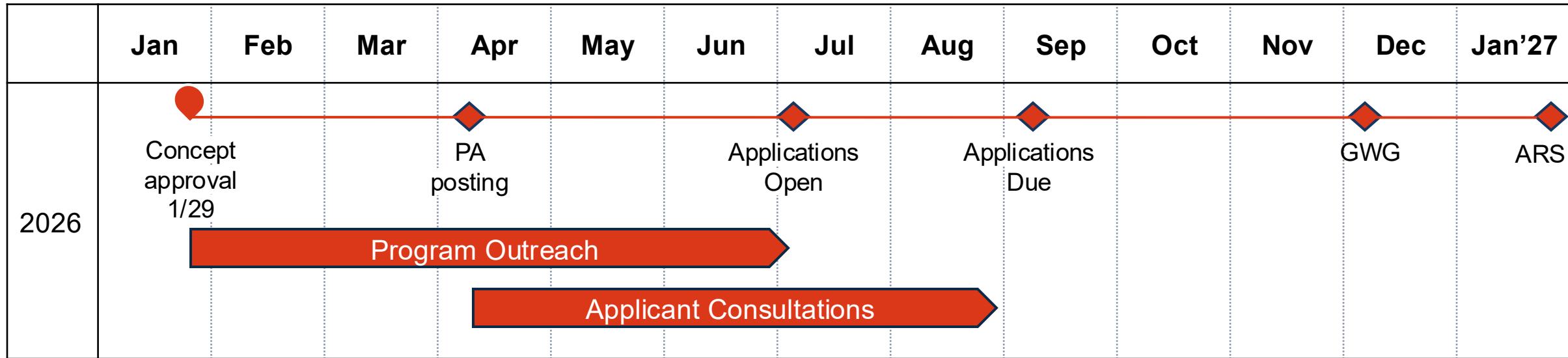
- Build evidence base and advance best practices for platform-based therapies by requiring public sharing of study designs, FDA interactions and applicable data

# Early Outreach to Applicants

- To **promote RAPID, respond to momentum in the field, and increase program-aligned submissions**, we propose focused outreach after the concept is approved and prior to PA and Application posting
- Outreach may include:
  - Early interactions with prospective applicants
  - Engaging with experts & patient advocacy organizations
  - Forums that convene key stakeholders

# Program Timeline

**Application open to award start ~ 9 months**  
**Awards start in April 2027**



# RAPID | Request for Motion

CIRM requests the Science Subcommittee endorse the proposed RAPID Program Concept for ICOC approval, with an initial allocation of **\$100M** in the first two funding cycles (FY2026-2027 and FY2027-2028)