RAPID Funding Opportunity: Concept Overview

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CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

***R**are-disease **A**cceleration through **P**latform **I**nnovation and **D**elivery*

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

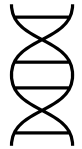
Pilot Platform-Based Rare Disease Therapy Development

Problem Statement

10,000+ Unique Rare Diseases

1 in 10 Americans
~30M people

> 50% are
childhood diseases



80%
genetic diseases

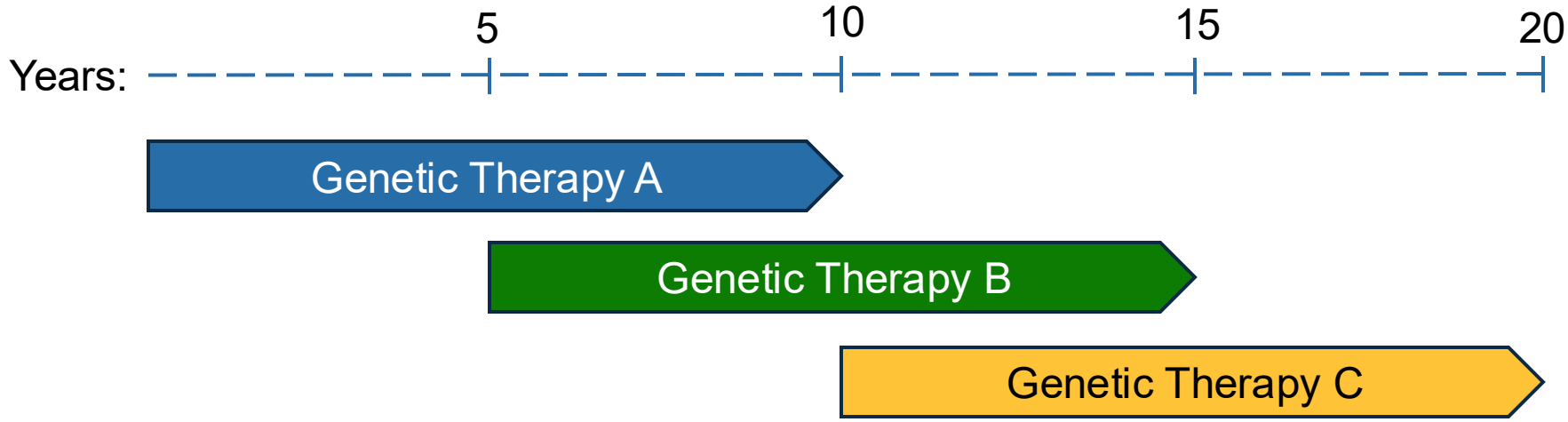
95% of rare diseases have **no approved therapy**

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

Source: Global Genes, NIH

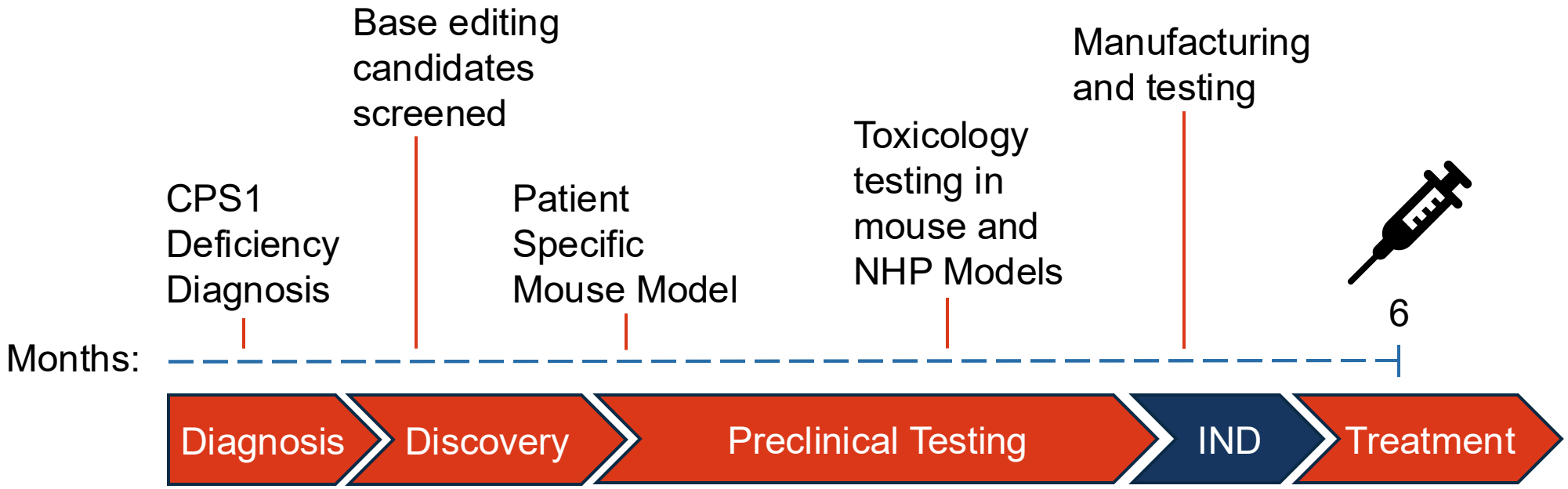
Therapeutic development is a long and costly process

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**

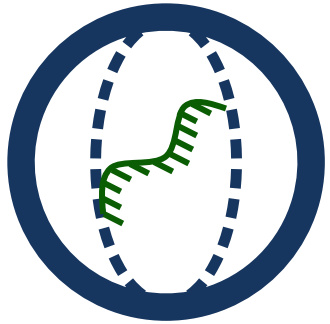
What if you could leverage common aspects of therapies?

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

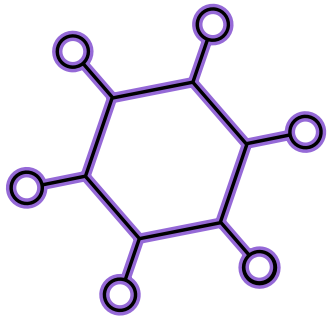
Illustrative Examples

Non-Viral Gene Editing

RNA-based Therapies



AAV Gene Delivery



Platform-Based Therapies Can Reach More Patients

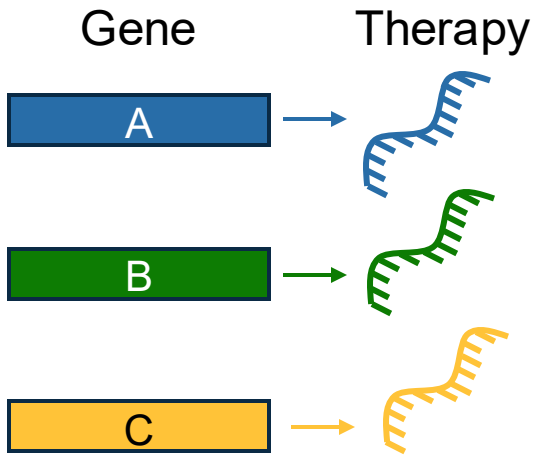
Rapidly identify therapies for multiple mutations

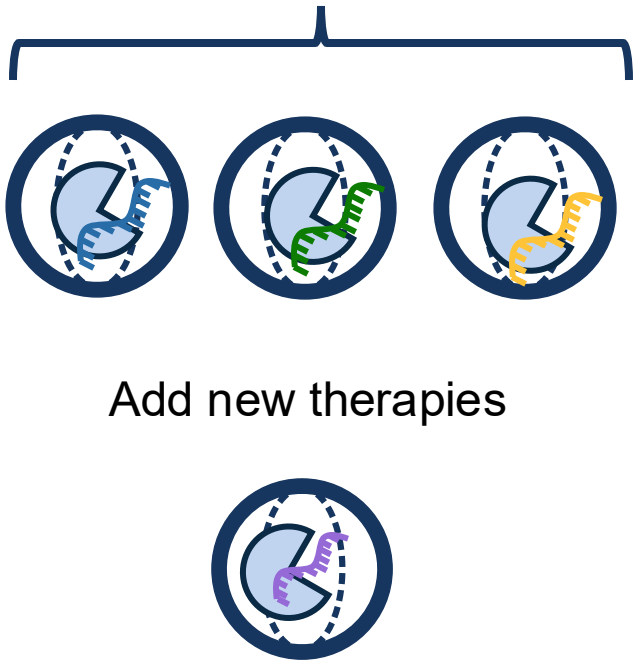
Perform bulk of testing on representative therapy

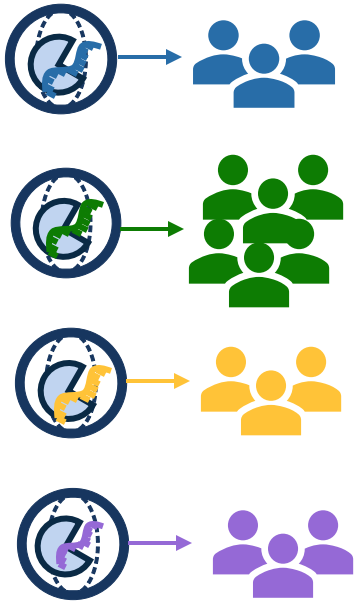
Test all therapies with a Master Clinical Protocol

Obtain Platform Approval

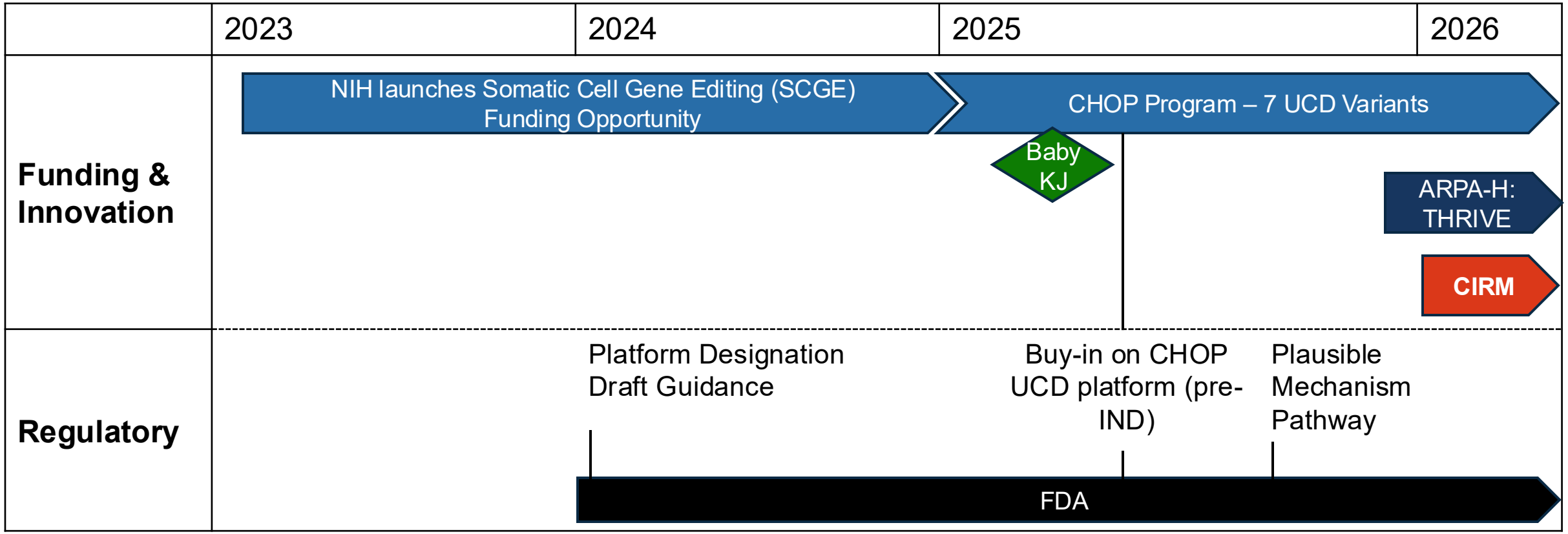
Discovery | Preclinical Testing | IND | Clinical Trials | Approval







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

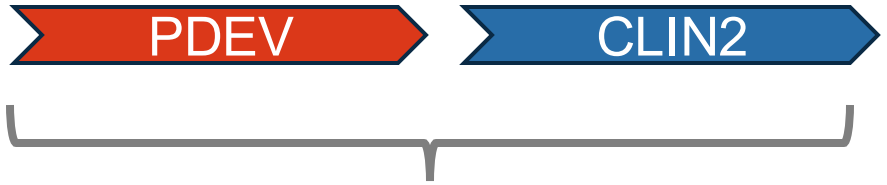
Create a **scalable model** to rapidly deliver transformative, **platform-based genetic therapies** to patients with rare disease

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
Therapy development	**Single** candidate
Disease impact	**Single** indications
Trial design	Traditional trial design; **single** IND
Non-clinical testing	**Full** suite for candidate
Manufacturing & testing	**Full** suite for candidate
CIRM funding progression	New CLIN2 application

IND: Investigational New Drug

RAPID Accelerates Platform-based Trials to Clinic

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

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

RAPID Validation Awards

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

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

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

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

INTERACT: Initial Targeted Engagement for Regulatory Advice on CBER/CDER Products

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
Total Funds	$55M	$45M	*Program may be reopened if RAPID funds are recovered, subject to ICOC Research Budget approval*
New Awards	Up to $50M	Up to $45M	
Program Supplement	$5M	Additional funds TBD	
Awards/Year	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

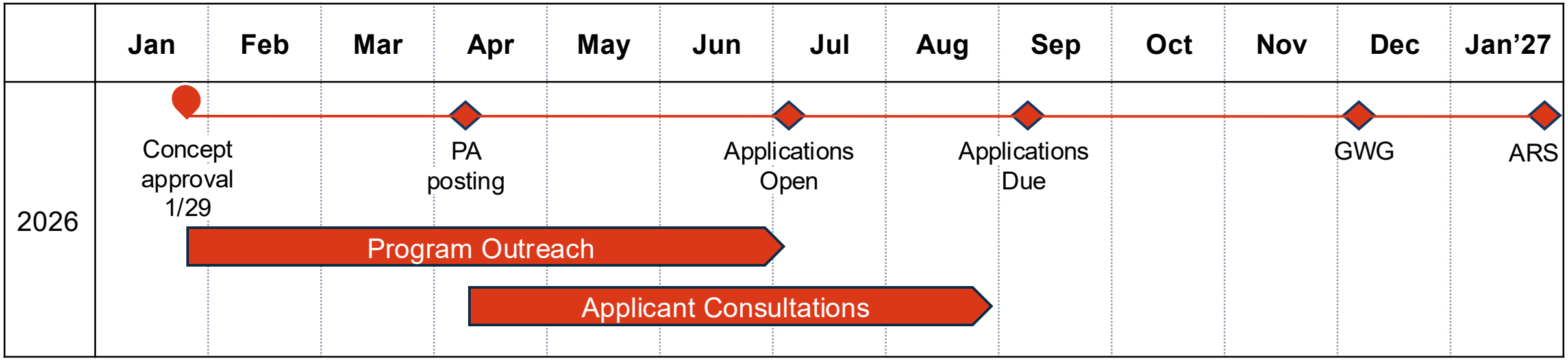
Early Outreach to Applicants

- To **promote RAPID**, **respond to momentum in the field**, and **increase program-aligned submissions**, we propose focused outreach will begin after the concept is approved

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