Rare Disease Acceleration through Platform Innovation and Delivery (RAPID) Funding Opportunity for Preclinical Stage Projects

Concept Plan: January 29, 2026 ICOC Approval

Summary

OVERVIEW			
Objective		To create a scalable model that rapidly delivers transformative, platform-based genetic therapies to patients with rare diseases	
Scope		CIRM will support activities in the IND-enabling and clinical stages for RAPID Validation and in the pre-IND and IND-enabling stages for RAPID Innovation	
Recurrence		Annual (until RAPID program funds exhausted)	
AWARD DETAILS			
Stage-specific Amount and Duration	**Stage**	**RAPID Validation**	**RAPID Innovation**
	Amount	No maximum amount	No maximum amount
	Duration	6 years	3.5 years
ELIGIBILITY			
Applicant Organization		Only non-profit or for-profit organizations that meet CIRM's definition of a California Organization are eligible to apply	
Critical Roles		• A Principal Investigator, who must commit a minimum 15% effort • An experienced project manager at a minimum 50% effort	
Stage Readiness		**RAPID Validation:** pre-IND completed **RAPID Innovation:** INTERACT completed or requested at time of application	
SCHEDULES AND DEADLINES			
Application Due Date		Once per year	
GWG Review		Approximately 90 days after application submission deadline	
Award Approval		Approximately 150 days after application submission deadline	
Start Date		Must be ready to start award activities within 90 days of award approval	

Background

The mission of the California Institute for Regenerative Medicine (CIRM) is to accelerate world-class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world. In September 2024, CIRM's Governing Board, the Independent Citizens' Oversight Committee (ICOC), approved a Strategic Allocation Framework (SAF) to guide and optimize the impact of CIRM's current and future investments. A recommendation of the SAF was to implement a platform-based approach to accelerate therapy development for rare diseases.

Advances in platform-based genetic technologies, increasing regulatory receptiveness to master protocols and basket trial designs, and the persistent unmet needs of patients with rare disease together create a timely opportunity to accelerate development through a coordinated, platform-driven approach. By reducing per-indication development costs and enabling the efficient advancement of therapies for patients with rare diseases that are often not commercially viable under traditional models, platform-based approaches support broader access to transformative treatments.

To accelerate the development of genetic therapies for rare diseases[1], CIRM has launched the Rare-disease Acceleration through Platform Innovation and Delivery (RAPID) program. This initiative is designed to harness the power of platform-based development to enable the efficient advancement of multiple related therapies through shared technologies and infrastructure. CIRM defines platforms as a common set of technologies that are leveraged for accelerated and resource-efficient development, manufacture, clinical delivery, and regulatory review of multiple related therapies. By supporting platforms that integrate common components across therapeutic candidates, RAPID facilitates faster development timelines, more efficient use of resources, and a scalable model where each new candidate strengthens the overall platform. This approach allows for the parallel progression of multiple therapies with streamlined testing requirements, reduces capital and operational costs through shared development pathways, and builds a cumulative evidence base that enhances regulatory confidence and accelerates future approvals.

Through the RAPID program, CIRM is accelerating the preclinical and clinical development of platform-based genetic therapies for rare diseases by supporting innovative baskets of therapies through to clinical proof of concept in first-in-human (FIH) trials. This initiative directly contributes to the SAF goal of advancing 4–7 rare disease programs towards BLA. By investing in scalable, sustainable therapeutic platforms, RAPID not only drives scientific and regulatory innovation but also reduces key barriers to patient access and affordability—helping to expand the reach of transformative treatments to patients with rare diseases.

Objective

The objective of the RAPID program is to create a scalable model that rapidly delivers transformative, platform-based genetic therapies to patients with rare diseases.

Within the RAPID program, CIRM will serve not only as a funding agency but as an active partner, providing internal resources and leveraging a network of expert advisors to support awardees throughout development. A central feature of the program is the RAPID Awardee Knowledge Network, which will facilitate real-time sharing of technical, scientific, and regulatory insights across CIRM-funded teams. In addition, CIRM will promote external data sharing beyond its portfolio, with a focus on disseminating regulatory learnings to benefit the broader field.

This collaborative model emphasizes scaling platform innovations, advancing regulatory science, expanding the safety and efficacy evidence base, and reducing the cost and time of development—ultimately creating a sustainable and knowledge-driven pathway for rare disease therapeutic development.

Scope and Structure

The RAPID program supports platform-based genetic therapy development via two complementary award types: RAPID Validation and RAPID Innovation. For both types of awards, platforms are expected to deliver accelerated and cost-effective development efficiencies through shared preclinical data and models, shared CMC processes and analytics, and streamlined clinical development approaches that support rapid expansion to additional candidates with reduced incremental burden. Within this shared platform framework, the RAPID program distinguishes between two award types based on stage of development, regulatory readiness and expected outcomes:

- **RAPID Validation** is intended for projects that have already engaged with the FDA and received preliminary regulatory alignment on their platform approach via a pre-IND meeting. Validation awards will support all activities required to initiate and complete a first-in-human (FIH) master protocol[2] clinical

[1] CIRM defines rare disease as a disease with a prevalence of <200,000 patients in the US

[2] A master protocol is a clinical trial protocol designed with multiple coordinated sub-studies to evaluate one or more investigational drugs for one or more diseases within the overall trial structure

trial for a basket of genetic therapy candidates. Applicants may propose a development plan that includes IND-enabling studies, submission and clearance of a master protocol IND, and execution of the FIH clinical trial. The expected outcome is the completion of the FIH trial and generation of clinical proof of concept (POC) for the platform, demonstrating its potential to support the future development of multiple therapeutic candidates for patients with rare disease.

- **RAPID Innovation** focuses on earlier-stage projects that are exploring novel or emerging platform technologies. These projects are expected to push the boundaries of what constitutes a platform, including innovations that could reduce testing requirements or expand applicability across multiple rare diseases. Applicants must have either completed an INTERACT meeting with the FDA or have submitted the INTERACT meeting request to the FDA at the time of application. Applicants may propose activities ranging from platform optimization, IND-enabling studies and submission of a master protocol IND. The expected outcome for Innovation awards is IND clearance, supported by early regulatory engagement, thereby de-risking the development path.

Program Funding Areas

As part of the RAPID program, CIRM intends to foster a diversified portfolio of projects that demonstrate the functionality, clinical validity, and scalability of platform-based in vivo genetic therapy approaches across multiple rare disease contexts, including the following:

- Projects that target single-gene defined rare diseases with high mutational frequency, where a platform approach enables rapid iteration across multiple genetic variants.
- Projects that target groups of related rare genetic disorders with a shared molecular mechanism or therapeutic target, where a single platform can address multiple indications through a common pathway or endpoint.

Program Activities

Applicants may request funds to cover costs for research activities conducted wholly in California and may also request costs for research activities conducted outside of California, provided that the California Organization exercises direction and control over the activities.

CIRM **will fund** the following activities under this opportunity:

TRACK	ALLOWABLE ACTIVITIES	EXPECTED OUTCOMES
RAPID Validation	• All activities required to achieve Master Protocol IND clearance • All activities required to conduct a Master Protocol clinical trial	First-in-human clinical trial completion
RAPID Innovation	• All activities required to achieve Master Protocol IND clearance	Master Protocol IND clearance

CIRM **will not fund** the following activities under this opportunity:

UNALLOWABLE ACTIVITIES ACROSS ALL STAGES	
✘	RAPID Innovation: The conduct of a clinical trial beyond start-up activities (patient recruitment, screening, or enrollment are not allowed)
✘	Activities already budgeted or paid for under a prior, existing or pending CIRM award, or which are already supported by another funder
✘	The costs of activities performed by a separate out-of-state organization that retains intellectual property or independent publication rights in any intellectual property (e.g., invention, technology, data) arising out of the CIRM-funded project
✘	Costs incurred on or before the date of ICOC approval

Award amount and duration

CIRM expects RAPID projects to advance quickly from preclinical development to master protocol IND clearance (RAPID Innovation) and FIH clinical trial completion (RAPID Validation) and will not accept applications with timelines exceeding program limits. RAPID Innovation applicants may request support for preclinical activities spanning pre-IND and IND-enabling stages, while RAPID Validation applicants must request support for activities spanning IND-enabling and Phase 1 clinical trial stages.

There is no maximum award amount specified for this program. The amount of total project costs requested must be adequately justified and is subject to adjustments prior to issuance of an award based upon assessments of the Grants Working Group (GWG), the CIRM team, or by the Application Review Subcommittee (ARS) of CIRM's Governing Board.

Upon achievement of distinct project milestones (e.g., Validation awards at the clinical trial stage or Innovation awards following a pre-IND meeting), awardees may request supplemental funding to accelerate the project, subject to availability of RAPID program funds and CIRM approval. Supplements are not guaranteed and may only be requested at designated points in the award. Award supplements may be used for - but are not limited to - the addition of new candidates for RAPID Validation, and activities in response to FDA feedback received during early interactions or trial startup activities for RAPID Innovation.

AWARD TYPE	MAX AMOUNT	MAX DURATION
RAPID Validation	None	6 years
RAPID Innovation	None	3.5 years

Positive Selection

All prospective applicants to the RAPID Funding Opportunity will be required to participate in a mandatory consultation with CIRM staff prior to application submission to ensure that proposed platform-based therapies meet RAPID eligibility, scope and readiness requirements, and to provide guidance on application expectations.

When the number of applications received in a cycle exceeds the number that can be reviewed by the GWG panel, CIRM will conduct the review in two stages. In the first stage, a pre-review of applications (called "Positive Selection") will be conducted by members of the GWG to identify applications that are most responsive to the funding opportunity and hold the greatest potential for impact. The CIRM scientific team and CIRM President then determine whether any additional applications merit a full GWG review. The remaining non-selected applications are deemed to be denied.

Positively selected applications advance to the second stage of review, which involves assignment to specific reviewers on the panel, a full discussion at review meeting, and scoring by the GWG.

Provisional Timetable

The RAPID funding opportunity will recur once per year. The anticipated timeline of each funding cycle is as follows:

PROVISIONAL TIMETABLE	
Applications open	Once per year
Applications due	Approximately 60 days after applications open
Grants Working Group (GWG) review	Approximately 90 days after application due date
Application Review Subcommittee award approval	Approximately 60 days after GWG review
Award start	90 days after award approval

Eligibility

All the following requirements must be fully satisfied for an application to be accepted and considered for funding by CIRM. Requirements marked with a * incorporate by reference the requirements and definitions described in CIRM Funding Opportunities: Common Requirements and Definitions, which may be amended from time to time by CIRM.

ELIGIBILITY REQUIREMENTS	
1	**Outcome Eligibility:** Applications must propose studies to achieve the expected outcome of the Innovation or Validation award.
2	**Candidate Eligibility:** Applications must propose (and maintain throughout the project) a platform that includes at least three *in vivo* genetic therapy candidates that share preclinical, CMC and clinical development elements in a way that reduces redundant testing and supports scalable addition of new candidates.
3	**Readiness and Award Entry Eligibility:** Applicants must demonstrate disease modifying activity for at least one candidate at the time of application submission and at minimum must describe a plausible mechanism pathway for the other candidates. Applicants must also demonstrate regulatory readiness through completion of an FDA Pre-IND meeting for the platform (Validation awards) or submission of an FDA INTERACT meeting request for the platform (Innovation awards) covering all proposed candidates.
4	The PI must commit a minimum of 15% effort and adhere to CIRM's requirements*.
5	The project team must include an experienced project manager at a minimum 50% effort.
6	The project team must include data management experience.
7	The applicant must be ready to initiate work on the funded project within 90 days of approval.
8	The application must be complete and accurate.
9	The applicant organization must meet CIRM's definition of a California Organization*.
10	For-profit organizations must demonstrate solvency*.
11	The applicant must meet CIRM's requirements for "good standing" *.