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CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Rare Disease Acceleration Through Platform Innovation and Delivery (RAPID) Frequently Asked Questions (FAQ)

May 2026

FOREWORD

We strongly advise that all potential applicants carefully read the Request for Applications (RFA) and other documents linked in the section below. In addition, please open an application in the grants management system and review all of the uploads that are required for submission. Many questions can be answered with the information provided in those documents. If you have questions after review of these materials, you may email the Preclinical Development team at preclinical@cirm.ca.gov.

RESOURCES FOR APPLICANTS

- CIRM's Funding Opportunities: Common Requirements and Definitions
- CIRM's Data Sharing and Management Requirements
- CIRM's Patient Access Planning Requirements
- CIRM's Allowable Costs and Co-funding FAQ
- CIRM's Commercialization Rights Primer (IP, Revenue Sharing, Pricing, Access, March-in Rights)
- CIRM's Grants Administration Policy for Clinical Stage Projects

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PROJECT SCOPE

1. **What is a "platform" in RAPID; how many candidates must be included?**
   A platform in RAPID is a common set of technologies that can be leveraged for more resource-efficient development, manufacture, clinical delivery, and regulatory review across multiple related therapies. RAPID is intended to support programs where candidates share meaningful preclinical, CMC, and clinical development elements in a way that reduces redundant testing and supports scalable addition of new candidates. Platforms must comprise at least three in vivo genetic therapy candidates and maintain that minimum throughout the project.

   In practice, RAPID is not intended for a loose collection of related assets. Applicants will need to show that the candidates are connected through a robust development platform that creates time and cost efficiencies and supports expansion to additional candidates with reduced incremental burden.

2. **What is the difference between RAPID Innovation and RAPID Validation? How should applicants decide which type of award is the right fit?**
   Both award types are for platform-based in vivo genetic therapy programs for rare diseases, require a platform with at least three candidates, disease-modifying activity for at least one candidate, and at least a plausible biological mechanism for the others. Both also require platform-relevant FDA engagement covering all proposed candidates. The main differences are stage, regulatory readiness, supported activities, and expected outcome:

   - ***RAPID Validation** is for projects that have already completed a pre-IND meeting and have preliminary FDA alignment on the platform approach. Validation awards will support all activities required for master protocol IND clearance and to conduct a FIH master protocol clinical trial. The expected outcome is completion of the FIH clinical trial of at least three in vivo genetic therapy candidates that demonstrates clinical proof of concept (POC) for the platform.*
   - ***RAPID Innovation** is for earlier-stage platform projects and is intended to support novel genetic or delivery technologies, broader disease areas and/or novel testing strategies compared to existing approaches. Applicants must have completed or requested an INTERACT meeting at the time of application. Innovation awards will support activities from platform optimization through IND-enabling work and submission of a master protocol IND but not conduct of the clinical trial beyond startup activities. The expected outcome is Master Protocol IND clearance for an FIH trial of at least three in vivo genetic therapy candidates.*

3. **Is RAPID limited to in vivo gene editing approaches, or could other modalities be responsive? Is RAPID prioritizing specific technologies?**
   Only in vivo genetic therapies are eligible for RAPID funding. For the avoidance of doubt, ex-vivo genetic therapy approaches cannot be funded under this program. RAPID is not prioritizing any single genetic engineering technology, but rather looking for proposals that

use an in vivo genetic therapy platform that can be applied efficiently across multiple candidates.

4. **What activities can RAPID fund?**
   *RAPID can fund all activities required to achieve master protocol IND clearance. Funding for the conduct of a master protocol clinical trial is limited to RAPID Validation awards. RAPID **will not fund** activities already supported by another source or another CIRM award, costs incurred before ICOC approval, or certain out-of-state work where the outside organization retains IP or independent publication rights in project-generated IP/data.*

5. **Is there a preference for specific types of rare diseases?**
   RAPID does not prioritize specific disease areas. Instead, it is intended to support a diverse portfolio of rare diseases that are a strong fit for a platform-based approach. The key consideration is whether the disease(s) can be efficiently addressed using a shared platform, not the specific indication. Note: CIRM defines rare disease as a disease with a prevalence of <200,000 patients in the U.S.

6. **What regulatory readiness is required?**
   The required readiness depends on award type. For Validation awards, applicants must have completed an FDA pre-IND meeting for the platform. For Innovation awards, applicants must have completed or at least submitted an FDA INTERACT meeting request for the platform at the time of application.

7. **For in vivo validation, how much data is required to demonstrate disease modifying activity?**
   Applicants must demonstrate reproducible disease modifying activity for at least one candidate in relevant preclinical model(s). If no appropriate model exists, applicants should provide a justification and alternative evidence of biological or translational relevance. Additional candidates in the platform must be supported by a plausible biological mechanism of action, including a scientific rationale and supporting data if available.

   CIRM encourages reductions in animal testing and use of robust new approach methodologies (NAMs) where feasible.

8. **What happens if FDA does not accept the platform approach?**
   RAPID is designed to incorporate ongoing FDA engagement, and applicants are expected to adapt their development strategy based on FDA feedback. If substantial changes are required, continuation of the award will depend on whether the revised plan remains feasible, well-justified, and aligned with RAPID objectives. If a platform approach is ultimately not supported by the FDA, the award may be discontinued.

9. **If one candidate fails, does the entire platform fail?**

Not necessarily. RAPID is structured around a platform, not a single candidate. The rationale for the program is that shared platform components generate cumulative knowledge that can support multiple related candidates and strengthen future development. A single candidate setback would not automatically mean the platform fails, but the impact would depend on whether the issue undermines the broader platform and whether the three-candidate minimum can be maintained throughout the project. Additional candidates may be added during the project with prior CIRM approval and FDA alignment. Requests will be evaluated on a case-by-case basis.

APPLICANT ELIGIBILITY

10. Who is eligible to apply?

Only non-profit or for-profit organizations that meet CIRM's definition of a California Organization are eligible to apply. For-profit organizations must also demonstrate solvency, and applicants must meet CIRM requirements for good standing.

11. Are there any expectations or preferences regarding team size, composition or structure?

There is no minimum or maximum team size, and RAPID does not favor small or large multi-organization teams. Key considerations are whether the team is well-integrated, experienced, and has the expertise and capability to efficiently execute the shared preclinical, CMC, regulatory, and clinical activities required for a multi-candidate platform program and to deliver the proposed outcomes.

At minimum, teams must include a PI with at least 15% effort, an experienced Project Manager with at least 50% effort and an experienced Data Project Manager.

Please note that the intended IND sponsor (i.e., the entity to be named as the sponsor on the IND application to the FDA) must be the CIRM applicant organization if an organization sponsored IND or the CIRM PI if an investigator-sponsored IND.

12. Can work be performed outside California?

Yes, work may be conducted outside California, but the California Organization must exercise direction and control over those activities. Awardees must make good faith efforts to obtain at least 50% of goods and services from California suppliers, with justification when this is not feasible and documentation reported annually. There is no specific limit on out-of-state subcontracts; however, CIRM funds cannot support "research" conducted outside California. A subcontract is considered "research" if the third party retains intellectual property or independent publication rights. Such costs are allowable only if these rights are waived.

13. Can an investigator be included in more than one RAPID application?

The principal investigator may not apply for more than one RAPID award within the same funding cycle. The same individual may be named as key personnel on multiple RAPID applications, including those submitted within the same funding cycle.

APPLICATION AND REVIEW PROCESS

14. Are pre-application consultations required? How do I schedule one?

Yes. All prospective applicants must participate in a mandatory consultation with CIRM staff before submitting an application. The purpose is to ensure that proposed platform-based therapies meet RAPID eligibility, scope, and readiness requirements and to provide guidance on application expectations. Prior to the consultation, applicants are required to submit key information about their proposed project. This includes completion of the Eligibility Upload Document, where applicants should provide relevant data and supporting information demonstrating candidate and readiness eligibility. Please visit our program page to request a consultation.

15. How will my application be reviewed after submission?

Please refer to the Application Review Information section of the RAPID RFA for a detailed description of the review criteria and process.

KNOWLEDGE AND DATA SHARING AND MANAGEMENT

16. **What are RAPID's knowledge sharing expectations?**

Knowledge sharing is central to RAPID's goal of building a collective evidence base for platform-based in vivo genetic therapies to accelerate development, improve regulatory alignment, and reduce duplication across the field. Accordingly, RAPID includes enhanced data and knowledge sharing expectations beyond standard CIRM programs (see also Q.17 below for more information).

With a key objective of disseminating regulatory learnings and supporting documentation to advance regulatory innovation and inform future development, CIRM will facilitate the sharing of technical, scientific, and regulatory insights across awardees and where appropriate, with the broader scientific community. Knowledge sharing may be achieved through participation in the CIRM Awardee Knowledge Network and related forums, and through dissemination of lessons learned, best practices, and key development insights.

RAPID awardees are expected to publicly share regulatory interactions and learnings in a timely manner, including key documents such as regulatory submissions, FDA feedback, and correspondence (e.g., pre-IND meeting feedback within 90 days of receipt). These activities are core components of RAPID and will be incorporated as operational milestones. Awardees will contribute to cross-program learning through CIRM-facilitated discussions and, where appropriate, external knowledge-sharing efforts. CIRM will work with awardees to ensure that sensitive information is appropriately protected in any public disclosures.

17. What are RAPID's data sharing expectations?

The sharing of data and knowledge produced from CIRM-funded projects is key to advancing the field of regenerative medicine and accelerating the discovery, validation and development of treatments for patients. CIRM requires awardees to manage and preserve raw data, processed data, and metadata, and make Applicable Data and metadata available to the broader scientific community. CIRM also requires applicants to allocate funds in their proposed budget for personnel and/or activities related to managing and sharing data produced from the funded project. ***To ensure data processing steps can be replicated and data can be reused by other researchers****, CIRM requires sharing of data in accordance with FAIR and CARE data principles, using established repositories where possible. CIRM requires that applicants provide a* ***Data Sharing Overview*** *in their proposal, and awardees develop and execute a detailed* ***Data Sharing and Management Plan (DSMP)****. The data repositories selected and other information about deposited data must be reported to CIRM during and after the project period. To promote FAIR data sharing and open science, CIRM will share descriptions about CIRM-funded data, in the Data Explorer dashboard, including what types of data were generated and where data are deposited. RAPID awards have additional preclinical development program requirements and, if clinical activities are supported, additional clinical development program requirements to facilitate clinical trials data sharing. More information on data sharing requirements can be found at CIRM Data Sharing and Management.*

PATIENT ACCESS & COMMERCIALIZATION PLANNING

18. How are RAPID awardees expected to address patient access to the proposed therapies?

Patient access is a central rationale of the RAPID program. By supporting platform-based approaches, RAPID aims to reduce per-indication development costs, streamline development and manufacturing and enable scalability across multiple therapies. RAPID awardees are expected to make progress on stage-appropriate patient access strategic planning activities over the course of the CIRM RAPID awardees. Applicants are advised to review the Patient Access Planning Requirements document guidance for stage-appropriate activities. RAPID applicants are encouraged to budget for required patient access planning activities in the application.

19. Does RAPID require a commercialization plan?

Yes, a central premise of the RAPID program is that platform-based approaches are potentially more feasible to develop and commercially deliver to patients than current approaches. RAPID applicants are required to complete a commercialization plan section in the application. The commercialization plan section of the RAPID application requires the applicant to outline opportunities and challenges for commercial feasibility of their platform-based approach, any commercialization planning activities completed to date and any proposed planning activities over the course of the RAPID project.

AWARD DURATION, BUDGET, AND ADMINISTRATION

20. Is co-funding required?

No. Minimum co-funding is not required for RAPID. However, in-kind support and voluntary co-funding are strongly encouraged, and applicants must commit contingency funding for anticipated project risks.

21. Is there a maximum award amount or duration?

There is no maximum award amount for either RAPID Validation or RAPID Innovation. Budgets must be adequately justified, and will be adjusted by CIRM prior to issuance of an award based on review by Grants Working Group (GWG), CIRM staff, or the Application Review Subcommittee (ARS).

RAPID Validation awards are up to 6 years; RAPID Innovation awards are up to 3.5 years.

CONTACT

22. If I have questions about the RAPID program, who do I contact?

Please email us at preclinical@cirm.ca.gov

23. If I have questions about the review process, who do I contact?

Please email us at review@cirm.ca.gov

24. If I have questions about grants administration, who do I contact?

Please email us at GrantsManagement@cirm.ca.gov