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CIRM Patient Access Planning Requirements

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Patient Access

CIRM is committed to ensuring that therapies developed with CIRM funding can ultimately reach patients in California. Achieving this requires that applicants consider barriers to patient access during the development process. CIRM's role is to encourage strategies that make it more likely that a successful therapy can realistically be delivered to patients. Applicants to Preclinical (PDEV), Rare Disease Platform (RAPID), and Clinical (CLIN2) funding opportunities are required to provide stage-appropriate patient access plans that will be evaluated during scientific review and maintained as awards progress. Complementary activities to support commercialization and market access are also recommended to advance these therapies to patients. This guidance document is intended to help applicants understand and implement patient access planning, and to serve as a resource for recommended commercialization and market access activities.

Resources

CIRM has collaborated with external consultants and the Access and Affordability Working Group (AAWG) to identify activities supporting commercialization and market access. These resources are included as appendices to this document.

Appendix A: Patient access planning requirements by program stage

Appendix B: Description of patient access planning activities

Appendix C: Recommended stage-specific commercialization and market access activities

Descriptions in Appendix B are informational and should be read together with Appendix A, which controls whether an activity is required at application, required as an award milestone, or recommended only.

Key Terms

Patient access: the ability of individuals to obtain and use healthcare services, information and resources

Commercialization: the process of bringing a product to market, encompassing everything from R&D to manufacturing, marketing and distribution

Market access: ensuring the right patients receive the right treatments at the right time by effectively navigating healthcare payers, reimbursement policies, and pricing strategies

Program Requirements and Evaluation Overview

Application Stage Requirements:

The CIRM application requires the applicant team to describe stage-appropriate planning for patient access, market access and commercialization in the Patient Access and Commercialization Plan section of the proposal. Stage-appropriate patient access, market access and commercialization planning requirements are divided into two categories:

- **Required Patient Access Planning Activities:** Depending on project stage, applicants are required to demonstrate progress-to-date on required prerequisite ("Must Have at Entry") activities and to describe planned progress on any award-stage ("Award Milestone") activities over the course of the award project. Applicants must consult Appendix A in this guidance document to identify the prerequisite ("Must have at Entry") and award-stage ("Award Milestone") activities required for the project stage, and to follow further instructions in the template.
- **Recommended Market Access & Commercialization Activities:** Applicants are advised to describe any progress to date on recommended market access & commercialization activities and to describe any planned progress on these activities over the course of the award project.

Applicants must consult Appendix C in this guidance document for descriptions of recommended activities and strategies for completion.

It is the applicant's responsibility to adequately describe progress on any required prerequisite activities and to plan and budget for the required patient access planning activities over the course of the proposed award project. Any proposed patient access planning activities that will be conducted over the course of the award project must be described in the Project Plan section of the proposal. If CIRM funding is requested for these activities, the activities must be adequately budgeted and justified in the application as described below.

Application Stage Evaluation:

The Grants Working Group will evaluate progress on any prerequisite patient access planning activities as well as adequacy of proposed patient access planning activities as part of their overall assessment of value proposition and project plan and design. The Grants Working Group may consider any provided information on market access and commercialization planning activities as part of their overall assessment of the value proposition and project plan and design. The Grants Working Group review process and review criteria are described in the individual Program Announcements or Request for Applications.

Award Milestones:

Stage-appropriate required patient access planning activities will be incorporated into Operational Milestones as described in the individual Program Announcement or Request for Applications. CIRM may consult with external subject matter experts to assess progress towards completion of Operational Milestones.

Budget Justification Guidelines

Applicants should consider and justify any resources needed to complete proposed access, commercialization, and market access planning activities. If CIRM funds are requested for these activities, they must be included and justified in the submitted application as part of the overall Budget Justification for the project and incurred during the funded project period. Consistent with CIRM's Grants Administration Policy for Clinical Stage Projects, budget requests must not include infrastructure costs that are included in institutional overhead (e.g., awardee's facilities & indirect costs) or costs associated with the routine conduct of research. Costs may not be double charged, or inconsistently charged, as both direct and indirect costs. Resources to cover total cost of access, commercialization, and market access activities do not solely have to be funded by CIRM.

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Appendix A: Patient Access Planning Requirements by Development Stage

Required Patient Access activity name	PDEV/RAPID		CLIN2/RAPID Phase 1 or First in Human		CLIN2 Phase 2		CLIN2 Phase 3/Pivotal	
	Must Have at Entry	**Award Milestone**	**Must Have at Entry**	**Award Milestone**	**Must Have at Entry**	**Award Milestone**	**Must Have at Entry**	**Award Milestone**
Market landscape assessment/market research		✓	✓		✓		✓	
Reimbursement and market access strategy*		✓		✓	✓		✓	
Early revenue and market forecast				✓	✓		✓	
Brand strategy and positioning						✓	✓	
Market segmentation*						✓	✓	
Establish (clinical and payer) advisory board						✓	✓	
Value message						✓	✓	
Quantitative pricing studies*						✓	✓	
Value communication dossiers						✓	✓	
Establish patient registry/Risk-evaluation strategy (post launch)*						✓	✓	
Patient journey*								✓
Pre-approval information exchange (PIE)								✓

Requirements per stage: A table outlining the different access activities expected to be complete at application ("Must have at Entry") and award milestone phases for PDEV, RAPID, and CLIN2 trial stages. For activities designated as required for award milestone phases, applicants must propose plans to

complete those activities during the award period. A * denotes activities with a strong impact supporting access.

Any CLIN2 applicant proposing a pivotal clinical trial must meet the prerequisite for Phase 3/Pivotal programs as specified in the table.

Appendix B: Patient Access Planning Activity Descriptions

Activity	Benefits	Strategy
Market landscape assessment/ market research: Conducting detailed market research to assess the potential demand, competition, pricing strategies, and market gaps.	Understanding the size of the market, potential customers, unmet needs, and potential barriers to entry.	Perform in-house research reaching out to providers/payers/advocacy groups, and other key stakeholders. Utilize free online industry reports to inform market assessment. Consider minimal investment options for acquiring therapeutic area/industry market reports.
Reimbursement and market access strategy: Planning for the product's reimbursement strategy by assessing the healthcare landscape, payer preferences, and pricing models.	Early understanding of the product's economic value to health systems and key stakeholders is important for successful adoption in the market. During clinical stages outline an evidence generation plan for a product to ensure the "right" data will be available for regulators and reimbursement authorities to justify value and pricing.	Ensure market research is executed highlighting where the product fits into the clinical paradigm. Conduct early pricing studies to understand the price corridor that might be acceptable from the perspective of payers and providers given the attributes of the Target Product Profile (TPP). Early engagement with payers to understand access hurdles (e.g., prior authorizations); understand impacts the new product might have on provider practice in order to facilitate adoption. Continue monitoring and refinement of strategy as the product goes through the development process.
Early revenue and market forecast: Develop early market and revenue forecasts using published evidence and qualitative primary research to understand the target population, peak market share, pricing and resulting revenues.	These analyses are refined throughout the development process and useful for negotiating potential licensing agreements.	Document key assumptions for early forecasts that are developed in house - these assumptions can be validated or refined during subsequent market research. Refine forecasts during the clinical development cycle by conducting robust secondary and primary market research using the TPP, understand where the product fits into the clinical paradigm, and gain insights from IDIs regarding how the product will be perceived, potential market share and price range.

		Work with a market access/strategy consulting firm with expertise in developing forecasts.
Brand strategy and positioning: Starting the process of brand development, including naming and positioning the drug in the marketplace. Understand how a product is differentiated in the market.	Lay the foundation for future value message development and what drivers of value are important to physicians, patients, payers and other stakeholders. Attract funding to support future development.	Ensure market research is executed highlighting where the product fits into the clinical paradigm; research with payers and providers will give insight into how the product will be perceived. Early in the development process, establish a clear vision for the product outlining the unmet need and how the product will meet the need. Later in the development process, develop a brand strategy that identifies the target patients/providers, define the brand by what messages are to be communicated, and lastly develop a positioning statement. This activity will require the value message development to be completed.
Market segmentation: Segregate potential customers (e.g., patients/providers/payers) into groups based on different characteristics (e.g., geography, demographics, behaviors, etc.).	Key benefits include the ability to optimize marketing effort, enhance adoption and brand competitiveness.	Identify datasets that allow for identification of site of service and healthcare provider (HCP) prescribing behaviors.
Establish (clinical and payer) advisory board: Identify key opinion leaders who could serve as advisers. Periodically seek feedback on clinical and economic evidence generation efforts. Periodically develop content for advisors to review and provide feedback to support the development process.	Clinical and payer key opinion leaders (KOLs) offer expert advice as the development program progresses through the clinical stages of development through commercial launch.	Develop logistics to follow relevant company and local rules and laws when organizing an advisory board meeting. Consider outsourcing the organization, logistics, agenda, content development and meeting out-put analysis/reporting to a contractor to assist unless in-house company support is available
Value message: Develop value messages that communicate key drivers of value, which is supported by evidence that helps differentiate a therapeutic in the marketplace.	Value messages help clearly communicate the benefits of a drug to various stakeholders, including regulators, payors, healthcare providers, and patients. This ensures that everyone understands the therapeutic and economic value of the drug.	Conduct early market research (i.e. pre-clinical or Phase 1) into the market landscape, unmet needs, and disease burden. Developing the TPP and evidence generation plan, early economic modeling will all contribute to charting the path to developing the right data required to support justifying the value and pricing of a product. Refine message as the product moves through the development stages.

		Conducting advisory board meetings are useful for testing what data and messages resonate most with key stakeholders in terms of communicating the value of a product.
Quantitative pricing studies: Conduct a robust conjoint analysis to understand the impact of product attributes have on price from the perspective of payers and other key stakeholders.	Understand the market acceptable price given demonstrated product attributes. Ability to optimize price and market share to maximize revenues.	In rare/ultrarare disease areas leverage other research studies (e.g. cost-effectiveness analysis, budget impact model, advisory boards, qualitative pricing research) to inform pricing decisions. In other cases, wait for the development program to reach Phase 3 and initial pivotal data becomes available. At this point the investment risk is minimized by the product reaching the end-stage of development. Work with a vendor with access to large numbers of HCPs and payers for recruitment, and with expertise of programming surveys for simple data collection. Build in safeguards such as screening questions as part of the survey design to minimize risk of unreliable data.
Value communication dossiers: Development of the Academy of Managed Care Pharmacy (ACMP) dossier for the US (pre-approval/final approved versions) and Health Technology Assessment (HTA) submission dossiers for Ex-US markets	As part of the technology assessment, US payers typically request an AMCP dossier for new interventions. Other HTA bodies have a different dossier format to communicate similar content, although it is usually more detailed.	Ensure available evidence is collected (e.g. market research, systemic literature review, indirect treatment comparison, model reports, clinical study report, pre-launch label, value messages). At minimum, have a draft/pre-launch version developed that can be quickly updated once the price is official (frequently this happens at launch).
Establish patient registry/Risk-evaluation strategy (post launch): Develop a registry for patients to collect long-term, real-world data on the safety, efficacy, and outcomes of the cell or gene therapy or best supportive care or usual care. This activity is required for eligible cell and gene therapy candidates and encouraged for all eligible regenerative medicine candidates.	Enables monitoring of patients treated (or not treated) with the cell or gene therapy over the long-term. Enables quantification and validation of long-term treatment effects including duration of effect and other unknowns at the time of launch. Regulatory authorities, such as the FDA and EMA, often mandate the use of patient registries to monitor post-authorization safety and efficacy of cell and gene therapy drugs. Results may affect	Develop a study plan, protocol and project plan that outline the scope, purpose/objectives, timeline, patient populations, interventions, outcomes. Assign responsibilities, personnel (incl advisory board), facilities, resource commitment, and contractors to execute and monitor the registry over time. Extract value from the registry from analysis of outcomes and publishing results.

	reimbursement decisions post-launch. Can be used to continuously further the evidence base of the disease area by publishing observed outcomes.	
Patient journey: Develop a detailed pathway documenting the patient experience and healthcare service delivery from the point of a patient symptom on-set through diagnosis, treatment and management.	Developing a patient journey helps identifying pain points (unmet needs) and areas for improvement that a new treatment might alleviate and potentially lead to a more seamless and positive patient/provider/payer experience.	Engage with experts with experience in integrating data from diverse sources to streamline the process.
Pre-approval information exchange (PIE): Develop a presentation to proactively share and educate Payers in the US about certain health care economic and scientific information about new emerging products.	Market readiness tool that is used to facilitate early market access conversations with payers before FDA approval. Helps prepare the market for anticipated place in therapy, pricing, clinical trial results, potential indication, and anticipated timeline for FDA approval.	Ensure there is evidentiary support for the Healthcare Economic Information (HCEI) to comply with FDA regulations. Internal training/education on benefits and socialization of best practices on sharing pre-approval HECI as a way to overcome sensitivities to keeping information confidential until launch.

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Appendix C: Recommended Stage-specific Commercialization and Market Access Planning Activities

Unless required as part of the online application or in instructions in the proposal template, these activities are recommended to support commercialization and market access of the proposed therapy. Unless otherwise directed, activity descriptions may be included in the Patient Access and Commercialization Plan section of the proposal.

Activity	Description	Stage Recommendation
Intellectual Property (IP) Strategy	Develop a robust IP strategy by securing patents or other forms of IP protection for the compound or technology	**PDEV/RAPID:** Recommended award activity **CLIN2 FIH/Phase 1, Phase 2, Phase 3/Pivotal**: Recommended at application
Financial Planning and Fundraising Strategy	Estimate the anticipated required funds for the full development process and initiate steps to prepare to secure funding.	**PDEV/RAPID:** Recommended award activity **CLIN2 FIH/Phase 1, Phase 2, Phase 3/Pivotal:** Recommended at application
Regulatory Pathway Strategy	Understand the options available by indication for regulatory approval. Are there special designations potentially available (i.e. fast track, orphan designation, breakthrough, and priority review) or 505(b)(2) approach? This activity may include consideration for ex-US markets.	**PDEV/RAPID:** Recommended award activity **CLIN2 FIH/Phase 1, Phase 2, Phase 3/Pivotal:** Recommended at application
CMC Planning	Ensure that IP is protected as patents. Develop plans for scaling processes and analytics (e.g. manufacturing, QC, supply chain) from pre-clinical development to later-stage clinical trials and eventual commercialization.	**PDEV/RAPID:** Recommended award activity **CLIN2 FIH/Phase 1, Phase 2, Phase 3/Pivotal:** Recommended at application
Partnerships and Collaborations	Identifying potential strategic partnerships, collaborations, or licensing deals with larger pharmaceutical companies, biotech firms, or research institutions. These partnerships may provide funding, technology, or expertise needed to advance the development program	**PDEV/RAPID:** Recommended award activity **CLIN2 FIH/Phase 1:** Recommended award activity **CLIN2 Phase 2, Phase 3/Pivotal:** Recommended at application
Establish Natural History of Disease	Track key outcomes that illustrate the natural course of the disease for the current standard of care paradigm.	**PDEV/RAPID:** N/A **CLIN2 FIH/Phase 1:** Recommended award activity **CLIN2 Phase 2, Phase 3/Pivotal:** Recommended at application
Cost of Illness and Standard of Care	Design and conduct studies using real-world data (RWD) to generate real-world evidence (RWE) evidence that supports product value	**PDEV/RAPID:** N/A **CLIN2 FIH/Phase 1:** Recommended award activity **CLIN2 Phase 2, Phase 3/Pivotal:** Recommended at application

Quality of Life (QoL) Assessment	Include Health-related QoL measures and utility weights in the pivotal clinical trial. May need to supplement with time tradeoff studies if clinical study sample size is not sufficiently large to assess QoL in the relevant health states.	**PDEV/RAPID:** N/A **CLIN2 FIH/Phase 1, Phase 2:** Recommended award activity **CLIN2 Phase 3/Pivotal:** Recommended at application
Early Economic Modeling	Develop an early cost-effectiveness model based on existing published literature and the Target Product Profile.	**PDEV/RAPID:** N/A **CLIN2 FIH/Phase 1, Phase 2:** Recommended award activity **CLIN2 Phase 3/Pivotal:** Recommended at application
Briefing Book	Obtaining early scientific advice from health technology assessment bodies on potential data gaps, trial design, and insights on approaches to demonstrating cost-effectiveness.	**PDEV/RAPID:** N/A **CLIN2 FIH/Phase 1, Phase 2:** Recommended award activity **CLIN2 Phase 3/Pivotal:** Recommended at application

Recent Document Revisions

Date	List of Changes
5/15/2026	• Inserted guidance and requirements for RAPID awards • Updated instructions related to describing patient access, commercialization, and market access activities in application proposals. • Updated review process descriptions to clarify that patient access activities will be evaluated by the Grants Working Group • Clarified requirements and recommendations for pivotal stage trials
4/3/2026	• Updated “Access and Affordability” to “Patient Access” throughout the guide • Removed CLIN2 evaluation rubric and replaced with description of qualitative assessment that will be returned to applicants