

Access and Affordability Program Implementation – Update



Shyam Patel, Associate Vice President Preclinical Development
Joseph Gold, Senior Director Clinical Development
ICOC Meeting
June 26th, 2025

Overview

Context

- Prop 14, SAF Goal 5, and Aug 2023 AAWG feedback

Our Process

- Tool development
 - A&A Checklist
 - Evaluation Rubric
 - Draft Guidance Document
- Tool Integration into Preclinical and Clinical programs
- AAWG Input

Updates since 04/30/25 AAWG meeting

Background – Why A&A Planning Must Be Embedded Early

Strategic Imperative from Proposition 14

- Ensure that therapies developed with public funds **are accessible and affordable for all Californians**, particularly underserved populations

Feedback from AAWG (August 2024)

- Consensus: A&A operationalize @ both programmatic and project levels
- Broad agreement that access strategies must align with the *stage of development*
- Need for **consistent expectations** and clearer guidelines across CIRM funding stages

AAWG Toolkit Review and Approval (April 2025)

- Approved A&A Toolkit and Guidance Documents for implementation of A&A in CIRM Funding Programs

CIRM's Impact Goals

Accelerating Discovery & Translation

1. **Catalyze** the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California
2. **Accelerate** development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies

Cell & Gene Therapy Approvals

3. **Advance** 4-7 rare disease projects to BLA
4. **Propel** 15-20 therapies targeting diseases affecting Californians to late-stage trials

Accessibility & Affordability of CIRM-Funded Cell & Gene Therapies

5. **Ensure** that every BLA-ready program has a strategy for access and affordability

Diverse Workforce Development

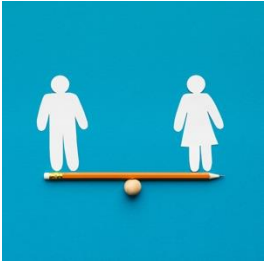
6. **Bolster** CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

Framing the Challenge – Key Questions and Considerations

Guiding Questions Raised by the AAWG

- What *stage-appropriate* A&A activities should be required, and when?
- How can CIRM leverage its infrastructure to *incentivize planning early* while ensuring feasibility and proportionality?
- What mechanisms and metrics are needed to *track and support* execution of A&A strategies?

Access and Affordability Definitions



Accessibility

The ability of individuals to obtain and use healthcare services, information, and resources



Affordability

An individual's ability to afford treatment, testing and other health care costs



Commercialization

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



Market Access

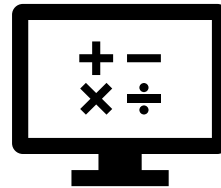
Ensuring the right patients receive the right treatments at the right time by effectively navigating healthcare payers, reimbursement policies, and pricing strategies

A&A tools and resources being developed with BlueRidge



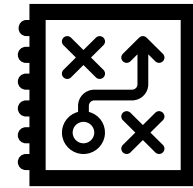
Checklist

- Interactive list of phase-appropriate A&A activities as subset of commercialization activities
- Allows PDEV and CDEV funding mechanisms to create A&A review criteria



Evaluation Rubric

- Excel tool to calculate a composite score for an applicant's A&A activities
- Embedded in review processes for CDEV funding mechanisms



Guidance Documents

- Allows applicants, awardees, and reviewers to align on expectations

A&A Toolkit

Preclinical and Clinical Programs

A&A in the Context of Commercialization Planning

Color Key



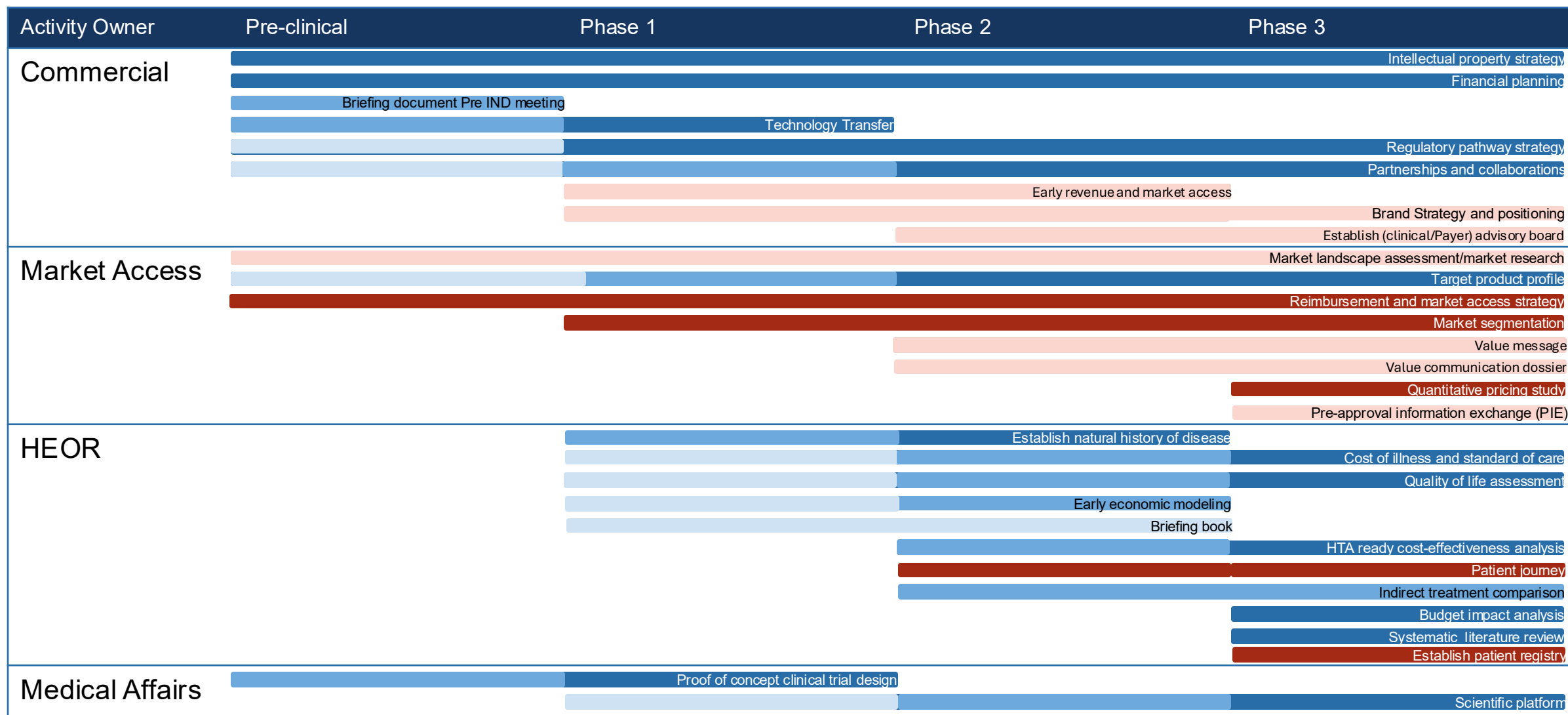
Nice to have



Should have



Must have



Designated as supporting access/affordability by BlueRidge

Designated as possibly supporting access/affordability

The Rest of the Presentation Will Focus on A&A Activities

Activity Owner	Pre-clinical	Phase 1	Phase 2	Phase 3
Commercial	Intellectual property strategy			
	Financial planning			
	Briefing document Pre IND meeting			
	Technology Transfer			
	Regulatory pathway strategy			
	Partnerships and collaborations			
	Early revenue and market access			
	Brand Strategy and positioning			
Market Access	Establish (clinical/Payer) advisory board			
	Market landscape assessment/market research			
	Target product profile			
	Reimbursement and market access strategy			
	Market segmentation			
	Value message			
	Value communication dossier			
	Quantitative pricing study			
HEOR	Pre-approval information exchange (PIE)			
	Establish natural history of disease			
	Cost of illness and standard of care			
	Quality of life assessment			
	Early economic modeling			
	Briefing book			
	HTA ready cost-effectiveness analysis			
	Patient journey			
Medical Affairs	Indirect treatment comparison			
	Budget impact analysis			
	Systematic literature review			
	Establish patient registry			
	Proof of concept clinical trial design			
	Scientific platform			

Designated as supporting access/affordability by BlueRidge

Designated as possibly supporting access/affordability

Checklist: Stage-Appropriate Access and Affordability Activities

	PDEV		CLIN2: Phase 1		CLIN2: Phase 2		CLIN2: Phase 3	
Activity name	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)								✓

*Activities with strong impact supporting A&A

A&A Implementation Overview – Preclinical

	PDEV
Application Requirements	<ul style="list-style-type: none">• Describe prior A&A activity progress to date• Describe plan for achievement of stage-appropriate A&A activities over course of PDEV award
Application Components	A&A checklist items incorporated into existing application proposal sections
Reviewer	Grants Working Group (GWG) scientific review
Scoring & Evaluation	Assessment of A&A activities incorporated into GWG review criteria (significance and project planning) and overall GWG score
Review Outcome	<ul style="list-style-type: none">• Informs GWG recommendation, CIRM team recommendation and ARS approval• GWG feedback provided to applicant• Informs CIRM management of approved awards

A&A Implementation Overview – Clinical

	CLIN2
Application Requirement	<ul style="list-style-type: none">• Describe completion of A&A checklist activities• Plan for achievement of stage-appropriate checklist activities
Application Components	Organized by A&A checklist for completed and planned activities for each trial phase
Initial CIRM review	Assess completeness of stage-specific checklist items in application
GWG Review	A&A checklist review conducted by expert specialist consultants
Scoring & Evaluation	<ul style="list-style-type: none">• Each activity scored for completeness and adequacy to yield a composite weighted score• Qualitative assessments of A&A activities and overall planning
Outcome	<ul style="list-style-type: none">• Informs GWG recommendation, CIRM team recommendation and ARS approval• Consultant feedback provided to applicant• Informs CIRM management of approved awards

Rubric

Composite score is a cumulative result of the overall status of A&A activities within 4 categories

A&A* activity score weighting

- A composite A&A activity score is calculated using the following formula:

- Composite A&A activity score:

$$\sum_{n=1}^n \left(\frac{(Priority\ Score)_{Activity\ n} \times (Raw\ Score)_{Activity\ n}}{5} \right)$$

- Note: the denominator is 5 so the top raw score assignment (raw score range 0-5) will be fully weighted



CIRM may use the composite scores to make funding decisions

An Excel tool was developed to facilitate calculating the composite score for A&A activities

Cell and Gene Therapy (CGT) Checklist for Commercialization Activities and Scoring Tool										Priority Level Key				Status/Notes				Raw Score				Weighted Score			
										Low (Green)				Medium (Yellow)				High (Red)				Not Started (Grey)			
										Pre-clinical				Phase 1				Phase 2				Phase 3			
Development Stage	Phase 3	Activity Owner	Timing	Activity name	Activity Owner	Activity description	Benefits of undertaking activity	Barriers to executing activity	Overcoming barriers	Supports Access/Adopt	Pre-clinical	Phase 1	Phase 2	Phase 3	Raw Score	Raw Score Weight	Level of Priority	Weighted Score							
4	Pre-clinical/1/2/3	Market landscape assessment/market research	Market Access	Conducting detailed market research to assess the potential demand, competition, pricing strategies, and market gaps.	Market Access	Understanding the size of the market, potential customers, unmet needs, and potential barriers to entry.	The cost of preclinical market research can be perceived as prohibitively expensive and very time consuming for small companies or startups and often there is only one person in the company with access to reliable evidence including real-world data to inform pricing and reimbursement decisions can be challenging.	Perform in-house research reaching out to providers/payers/advisory groups, and other key stakeholders.	Possible						2	0.5	3	1.5							
10	Pre-clinical/1/2/3	Reimbursement and market access strategy	Market Access	Planning for the product's reimbursement strategy by assessing the healthcare landscape, payer preferences, and pricing models.	Market Access	Early understanding of the product's economic value to health systems and key stakeholders is important for successful adoption in the market.	These analyses are refined throughout the development process and useful for negotiating potential licensing agreements.	Utilize free online industry reports to inform reimbursement where the product fits into the clinical paradigm.	Yes						2	0.5	3	1.5							
12	Phase 1/2/3	Early revenue and market forecast	Commercialization	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.	Commercialization	Early forecasts will be rudimentary, and largely based on crude hypothetical assumptions.	Document key assumptions for early forecasts that are developed in-house - these assumptions can be validated or refined during subsequent market research.	Possible							2	0.5	3	1.5							
13	Phase 1/2/3	Brand strategy and positioning	Commercialization	Starting the process of brand development, including naming and positioning the drug in the marketplace.	Commercialization	Lay the foundation for future value message development and what drivers of value are important to physicians, patients, payers and other stakeholders.	Early product positioning will be largely hypothesis driven given the lack of available/relevant evidence.	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.	Possible						2	0.5	3	1.5							
14	Phase 1/2/3	Market segmentation	Market Access	Understand how a product is differentiated from other potential competitors (e.g., patients/providers/payers) into groups based on different characteristics (e.g., geography, demographics, behaviors, etc.).	Market Access	Key benefits include the ability to optimize marketing effort, enhance adoption and brand competitiveness.	Not understanding the product's place in the market or lack of data to be able to look at geography, demographics, and behavior of the different stakeholders.	Identify datasets that allow for identification of site of service and HCP prescribing behaviors.	Yes						2	0.5	3	1.5							
20	Phase 2/3	Establish (clinical and payer) advisory board	Commercialization	Identify key opinion leaders who could serve as advisors. Periodically seek feedback on clinical and economic evidence generation efforts.	Commercialization	Clinical and payer KOLs offer expert advice as the development program progresses through the clinical stages of development through commercial launch.	Identifying KOLs and logistics of identifying availability of multiple advisors with busy schedules.	Develop logistics to follow relevant clinical and local rules and laws when organizing an advisory board meeting.	Possible						2	0.5	3	1.5							
21	Phase 2/3	Value message	Market Access	Develop value messages that communicate key drivers of value, which is supported by evidence that help differentiate a therapeutic in the marketplace.	Market Access	Value messages help clearly communicate the benefits of a drug to various stakeholders, including regulators, payers, healthcare providers, and patients. This ensures that everyone understands the benefits of a drug.	KOLs will need independent contractor input early in the evidence generation process (i.e. Phase 1) so that Phase 2 and pivotal trials contain the right data to support a strong value proposition.	Consider outsourcing the organization. Conduct early market research (e.g. pre-clinical or Phase 1) into the market landscape, unmet needs, and disease burden.	Possible						2	0.5	3	1.5							
23	Phase 2/3	Patient journey	HEOR	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.	HEOR	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 experience.	Integrating and accessing data from various sources, such as electronic health records, patient surveys, and clinical trials, can be difficult.	Engage with experts with experience in integrating data from diverse sources to streamline the process.	Yes						2	0.5	2	1							
25	Phase 2/3	Value communication dossier	Market Access	Development of AMCP dossier for the US (pre-approval/ approved versions) and HTA submission dossiers for Ex-US markets.	Market Access	As part of the technology assessment US payers typically request an AMCP dossier for new interventions.	Sensitivity to sharing pre-approval information (e.g. clinical study report, final label and launch pricing).	Ensure available evidence is collected (e.g. market research, SLR, TIC, model reports, clinical study report, pre-launch label, value messages).	Possible						2	0.5	3	1.5							
27	Phase 3	Quantitative pricing studies	Market Access	Conduct a robust conjoint analysis to understand the impact of product attributes have on price from the perspective of payers and other key stakeholders.	Market Access	Understand the market acceptable price given demonstrated product attributes. Ability to optimize price and market share to maximize revenues.	Quantitative pricing studies require a large sample size of participants and can be very costly to execute due to development and programming of a pricing and attribute survey. Also in late diseases it may not align on the variables and outcomes to collect.	In real-world disease areas leverage other research studies (e.g. CEA, BMA, advisory boards, qualitative pricing research) to inform pricing decisions.	Yes						2										
29	Phase 3/Registration	Establish patient registry/Risk-evaluation strategy (post-launch)	HEOR	Develop a registry for patients to collect long-term, real-world data on the safety, efficacy, and outcomes of CGT or best supportive care or usual care.	HEOR	Enable monitoring of patients treated (or not treated) with a cell or gene therapy over the long-term. Enables quantification and validation of long-term treatment effects including duration of effect on other outcomes.	Costly to design, implement and maintain, which for CGT therapies could be a significant barrier.	Develop a study plan, protocol and project plan that outlines the scope, purpose/objectives, timeline, patient populations, interventions, outcomes.	Yes						2										
30	Phase 2/3	Pre-approval information exchange (PIE)	Market Access	Develop a presentation to proactively share and educate payers in the US about certain health care economic and scientific information about a new emerging products.	Market Access	Market readiness tool that is used to facilitate early market access conversations with payers before Food and Drug Administration (FDA) approval.	Requires internal process for regulatory review and approval of materials to be shared externally with payers and other HCEI audiences.	Ensure there is redundancy support for the HCEI to comply FDA regulations and socialization of best practices on sharing	Possible						2										
Activity Count										2				5				9				12			
Total CIRM Commercialization Score for Application																									

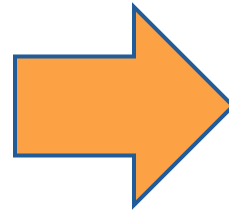
Award Management

	PDEV	CLIN2
Fundable Activities	Both programs will fund stage-appropriate allowable A&A activities	
Progress Reporting on all stage appropriate activities	Awardee will report progress on A&A activities over course of award	
Implementing milestones on A&A activities	<ul style="list-style-type: none">• A&A activities will be incorporated in award milestones• Inadequate progress on milestones could impact continuation of award	
Final Submission	Award will submit final strategic planning report as part of final operational milestone	

Implementing AAWG feedback

AAWG 4/30/25 Feedback

- **Broad support** for **early and consistent integration** of A&A planning
- **Scope** of activities **aligns with field standards** and **expectations** for comprehensive planning
- Execution will **require diverse expertise** across awardee teams
- **Recommend** CIRM provide **clear guidance** to applicants, reviewers, and the ICOC
- CIRM should **evaluate the completeness** of A&A plans during review and oversight



PDEV/CDEV Implementation

- CLIN2 & PDEV **PAs** posted with **A&A requirements** section
- A&A stage-appropriate activities **checklist** provided
- **Guidance Document** released with A&A activities defined and strategies to accomplish each listed
- **Wide range of consultants engaged**