Access and Affordability Program Implementation – Update

CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

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ICOC Meeting
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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  - Draft Guidance Document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

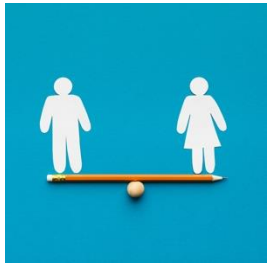
6. **Bolster** CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

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

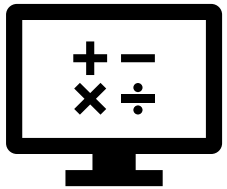
Market Access

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

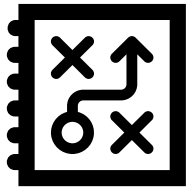
Checklist

- 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

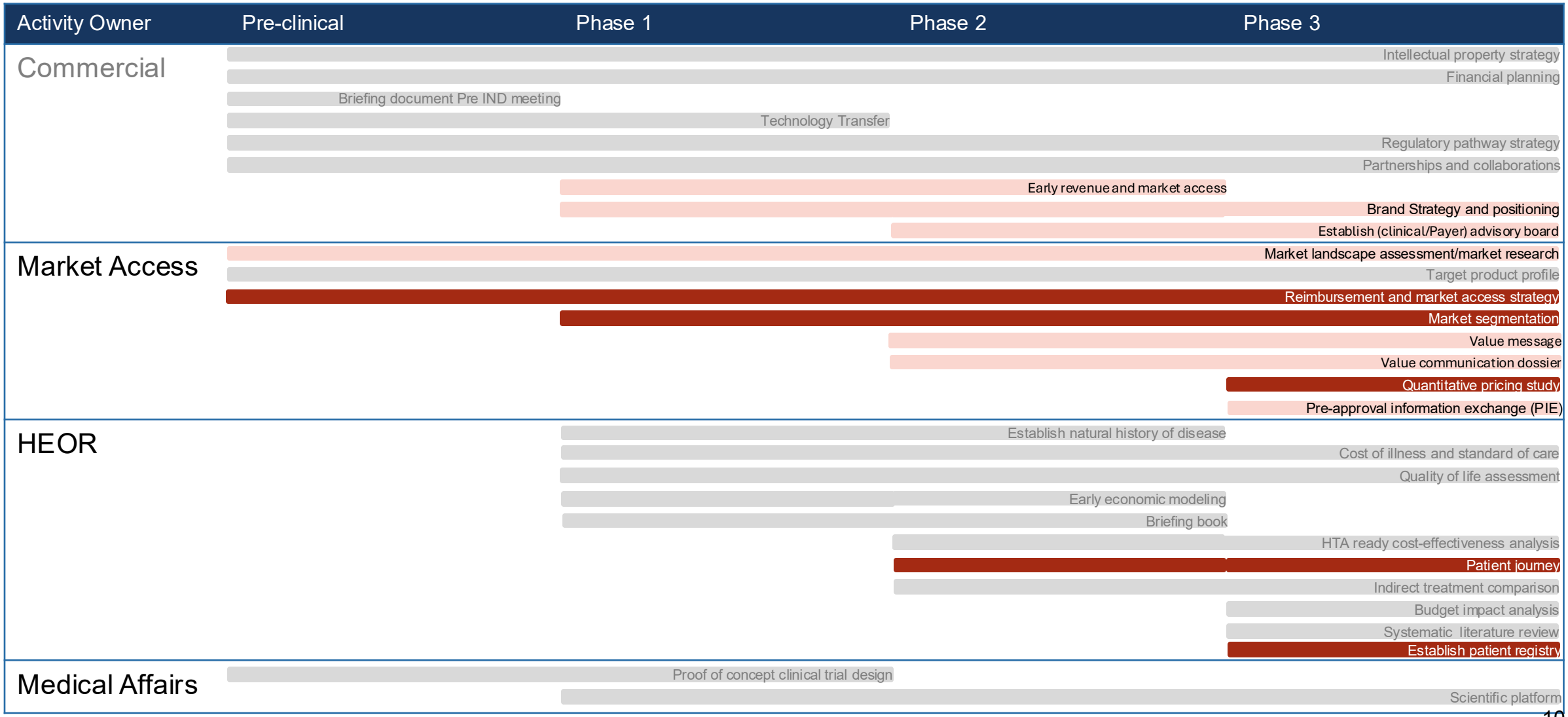
A&A in the Context of Commercialization Planning

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



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	• 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	• 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	• 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	• Each activity scored for completeness and adequacy to yield a composite weighted score • Qualitative assessments of A&A activities and overall planning
Outcome	• Informs GWG recommendation, CIRM team recommendation and ARS approval • Consultant feedback provided to applicant • Informs CIRM management of approved awards

Rubric

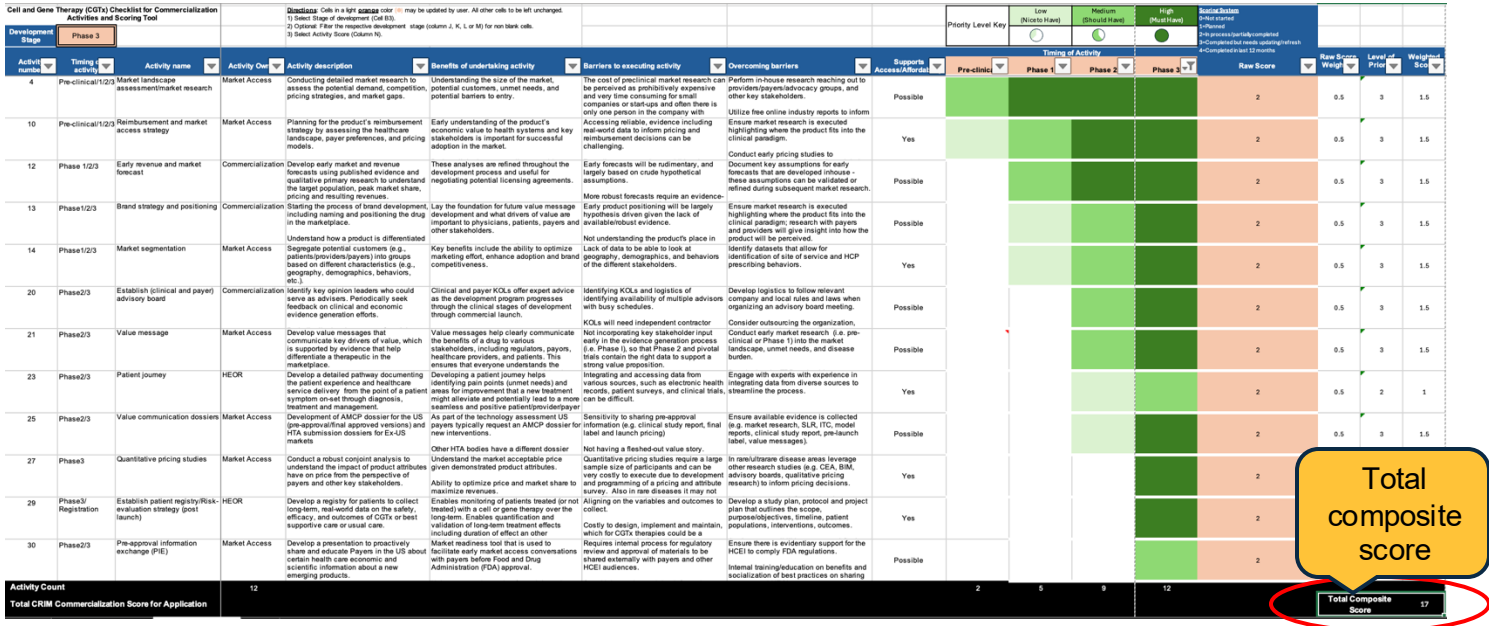
Composite score is a cumulative result of the overall status of A&A activities within 4 categories

A&A* activity score weighting

- A composite A&A activity score is calculated using the following formula:
- Composite A&A activity score:
- $$\sum_{1}^{n}\left(\frac{(Priority\ Score)_{Activity\ n}\times(Raw\ Score)_{Ativity\ \mathrm{n}}}{5}\right)$$
- 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



Total composite score

BlueRidge's Scoring Guidance

Scoring System
0=Not started
1=Reasonably planned
2=In process/partially completed
3=Completed but insufficient
4=Completed but needs updating
5=Completed in last 12 months

*This slide focuses on A&A activities, but all commercial planning activities will be scored

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 milest ones on A&A activities	• 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	PDEV/CDEV Implementation
• **Broad support** for **early and consistent integration** of A&A planning	• CLIN2 & PDEV **PAs** posted with **A&A requirements** section
• **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	• A&A stage-appropriate activities **checklist** provided • **Guidance Document** released with A&A activities defined and strategies to accomplish each listed
• CIRM should **evaluate the completeness** of A&A plans during review and oversight	• **Wide range of consultants engaged**