CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Memorandum

To: Members of the ICOC
From: Rafael Aguirre-Sacasa, General Counsel
Re: CIRM Access Plan Requirements
Date: September 25, 2025

I. Introduction

This memorandum introduces and summarizes two key documents to be presented to the Accessibility and Affordability Working Group (AAWG):

- **Exhibit A –** an excerpt from California Institute for Regenerative Medicine's *Intellectual Property and Revenue Sharing Requirements for Non-Profit and For-Profit Grantees (Section VII. Access Requirements for Products Developed by Awardees)*, which sets forth the statutory and regulatory framework governing CIRM's Access Plan requirements for U.S. Food and Drug Administration (FDA)-approved products developed with CIRM funding (Exhibit A summarizes the legal obligations that apply to awardee Access Plans); and

  Exhibit B – a PowerPoint presentation, which outlines CIRM's Patient Assistance Program (PAP) benchmarking work, Access Plan requirements, and related case study content conducted by CIRM's consultants, Blue Ridge Life Sciences, who also consulted on the work done by the Program Team, and presented to the AAWG on April 30, 2025.

The presentation is intended to operationalize the statutory Access Plan requirements while also providing applied insights and best practices drawn from the Blue Ridge analysis.

II. Interaction with Program Team's Structured Review of Accessibility and Affordability

The Program Team's structured review and engagement process, as outlined in its April 30, 2025, memorandum and presentation to the AAWG, introduces access and affordability considerations at the earliest stages of CIRM funding. Applicants must address key checklist items—such as cost modeling, payer readiness, health economics, and scalability—during both the application and milestone phases.

This structured approach builds discipline in access planning, provides early visibility into potential barriers, and creates measurable commitments that can be tracked and refined over time. The Program Team's Accessibility & Affordability (A&A) toolkits, developed

with Blue Ridge Life Sciences, serve as optional resources to support awardees in developing their strategies.

These early planning requirements directly dovetail into the Access Plan obligations under Section VII of CIRM's Intellectual Property and Revenue Sharing Requirements for Non-Profit and For-Profit Grantees. By the time an awardee reaches U.S. Food and Drug Administration (FDA) approval, the core elements of their Access Plan—eligibility criteria, affordability safeguards, payer engagement, and implementation logistics—will already be in place.

This continuum ensures that awardees are well positioned to submit compliant Access Plans and deliver equitable access to CIRM-funded therapies.

III. Intellectual Property and Revenue Sharing Requirements for Non-Profit and For-Profit Grantees Excerpt – VII. Access Requirements for Products Developed by Awardees (Exhibit A)

At a high-level, Section VII (see excerpt) requires that:

- Commercializing Entities must submit an Access Plan to CIRM within 10 business days of FDA approval (extensions up to 30 business days).
- The Plan must ensure access for Californians with "no other means" to purchase the drug (defined as those without prescription drug benefits and with household income below 300% of the Federal Poverty Level).
- Plans must align with industry standards, account for company resources, and be approved by CIRM after a public hearing with public comment. Approval may not be unreasonably withheld.
- Entities are responsible only for providing the drug itself, not ancillary costs of administration or care.
- The ICOC may waive requirements if compliance would hinder delivery or if waiver benefits equal or exceed plan benefits.
- Drugs purchased with public funds must be sold at the benchmark price under California's Discount Prescription Drug Program or successor programs.

In accordance with Proposition 71, Proposition 14, and the California Public Records Act, any proprietary or confidential information included in an Access Plan must be clearly identified by the submitting entity at the time of submission, along with an explanation of why it qualifies as protected intellectual property or work product. CIRM will review the designated material and withhold it from public disclosure if it meets the applicable legal standards, and the Independent Citizens' Oversight Committee (ICOC) may review such proprietary materials in closed session. For all non-confidential portions, CIRM will post the Access Plan online and conduct a public hearing that includes at least a seven-business-day public comment period, after which the ICOC will render a decision within five business days.

IV. AAWG Meeting Presentation (Exhibit B)

The presentation integrates these statutory requirements into a framework for evaluating Access Plans and Patient Assistance Programs (PAPs) and also provides a summary of industry benchmarks for Access Plans in the context of cell and gene therapies (CGTx) as provided by CIRM's consultant's, Blue Ridge Life Sciences. Given the high cost, logistical complexity, and unique regulatory considerations associated with these therapies, PAPs have become a critical mechanism for ensuring patient access and adherence. By reviewing 14 established CGTx programs across multiple manufacturers, Blue Ridge's analysis identifies common elements and best practices that may guide CIRM's review and oversight of awardee access plans.

The benchmark research highlights key eligibility criteria, core attributes of a well-structured access plan, and the types of financial, logistical, and dedicated support services typically offered. In addition, this summary addresses common outsourcing practices, identifies gaps and open questions, and outlines a generalized timeline for program development. Together with CIRM's statutory requirements, these benchmark findings provide a practical framework for evaluating whether awardee PAPs align with CIRM's statutory requirements for Access Plans.

V. Conclusion

Patient Assistance Programs (PAPs) for CGTx represent an essential bridge between high-cost innovation and patient access. The statutory requirements, augmented by the Blue Ridge benchmarks summarized in this memorandum, provide CIRM with a structured lens through which to evaluate awardee proposals, ensuring that access plans are compliant, comprehensive and patient-centric. Key considerations include establishing transparent eligibility criteria, ensuring robust case management and logistical support, and leveraging hybrid models that balance manufacturer oversight with third-party expertise.

By aligning the Program Team's efforts with CIRM's statutory requirements and these industry benchmarks, CIRM intends to create a complementary system of safeguards to reduce barriers to treatment, promote equitable access, and help ensure that Californians benefit from the therapies their public investment has made possible. This framework is intended to guide CIRM in evaluating whether awardee Access Plans meet the statutory requirements and advance Proposition 14's goals of equitable access.

Exhibits to Memo:

Exhibit A – Excerpt from Intellectual Property and Revenue Sharing Requirements for Non-Profit and For-Profit Grantees applicable to grants made after September 4, 2018 – VII. Access Requirements for Products Developed by Awardees

Exhibit B - AAWG Meeting Presentation