CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Memorandum

To: Members of the Accessibility and Affordability Working Group (AAWG)
From: Rafael Aguirre-Sacasa, General Counsel
Re: CIRM Access Plan Requirements
Date: September 5, 2025

I. Introduction

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

1. The first is an excerpt from the 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 FDA-approved products developed with CIRM funding; and

2. 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 Teams, and presented to the AAWG on April 30, 2025.

The PowerPoint presentation is intended to summarize and operationalize the Access Plan requirements under the "Intellectual Property and Revenue Sharing Requirements for Non-Profit and For-Profit Grantees, while also providing benchmarking data and applied insights from the Blue Ridge Life Science case study.

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

The Programs 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. By requiring applicants to address checklist items at both the application and milestone phases—including cost modeling, health economics, payer readiness, and scalability—the Programs Team ensures that awardees are actively incorporating patient access principles throughout the award's development lifecycle. This staged approach provides not only early visibility into potential barriers but also establishes measurable commitments that can be tracked and refined over time. In this way, applicants are guided toward building a feasible, equity-focused access strategy well before their therapy reaches commercialization. The A&A

toolkit(s) developed by the Programs Team with the help of Blue Ridge are intended to serve as resources for awardees, and not mandates, as they develop their A&A strategies and Access Plans.

These upstream activities directly support and "dovetail" into the Access Plan obligations required under the Intellectual Property and Revenue Sharing Requirements for Non-Profit and For-Profit Grantees at the time of FDA approval. By the time an awardee submits its Access Plan for CIRM approval, the foundational elements—eligibility criteria, affordability safeguards, payer engagement, and implementation logistics—will already have been developed and stress-tested through the Programs Team's oversight through the use of contracting milestones establishing stage-appropriate A&A activities. The result is a continuum of planning: early-stage programmatic requirements flowing naturally into a final Access Plan that is compliant, thereby ensuring that Californians, particularly those with no other means, have equitable access to the therapies funded by CIRM.

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 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.

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 established programs across multiple manufacturers, this

analysis identifies common elements and best practices that may guide CIRM's review and oversight of awardee access plans.

The benchmarks highlight 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, these findings provide a practical framework for evaluating whether awardee PAPs align with the requirements of Intellectual Property and Revenue Sharing Requirements for Non-Profit and For-Profit Grantees and industry standards.

Summary of Patient Assistance Program (PAP) Benchmarks

1) **Eligibility Criteria**
   a) U.S. citizenship or legal residency typically required
   b) Valid prescription; provider documentation often needed (prior authorization, diagnosis)
   c) Income thresholds: 200–600% of Federal Poverty Level (FPL)
   d) Many PAPs exclude Medicare, Medicaid, VA, or TRICARE patients
   e) Designed primarily for commercially insured or underinsured patients

2) **Eight Key Attributes of an Access Plan**
   a) Case manager/navigator oversight
   b) Benefit investigation and verification
   c) Appeals and prior authorization support
   d) Logistical coordination (cold chain, travel, lodging, childcare)
   e) Financial assistance (drug cost, copay, caregiver support)
   f) Ancillary wrap-around services (education, adherence, safety monitoring)
   g) Referral networks (foundations, mentoring, counseling)
   h) Ongoing compliance and post-treatment follow-up

3) **Dedicated Support**
   a) Access professionals aid with insurance verification, denials, and appeals
   b) Eligibility tools support providers in assessing patient fit
   c) Support extends beyond treatment, including adherence and milestone monitoring
   d) Programs coordinate limited authorized treatment centers for CGTx delivery

4) **Financial & Logistical Support**
   a) Direct costs: drug coverage, administration fees
   b) Indirect costs: travel, lodging, meals, childcare, lost wages when applicable

c) Wrap-around support: co-pay safety nets, caregiver expenses, case management
d) Logistics tailored to CGTx (cold-chain shipment, coordination with specialized centers)

5) Outsourcing to Third-Party Service Providers

a) Roughly 88% of manufacturers outsource some patient assistance services
b) Hybrid models (~80%) combine internal oversight with external hubs
c) Outsourcing addresses resource and compliance challenges, especially for smaller biotechs
d) Commonly outsourced functions include reimbursement support, financial assistance qualification, and travel/logistics

6) Gaps & Questions

a) Variation in administration models (in-house, hybrid, outsourced)
b) Transparency of eligibility criteria and scope of services
c) Inclusion of behavioral health, fertility, and non-IV therapies remains unclear
d) Limited provider and patient awareness: ~50% of patients unaware of PAPs
e) Ongoing challenge: providing comprehensive support while maintaining scalability and cost control

7) Generalized Timeline

a) 18–24 months pre-launch: Assess therapy profile, barriers; benchmark programs
b) 15–12 months: Design services and contract with hub providers
c) 12–9 months: Build IT, SOPs, patient resources; integrate logistics
d) 9–6 months: Train staff, educate providers, and initiate payer engagement
e) 6–0 months: Pilot workflows, refine processes based on feedback
f) Launch: Go live; monitor outcomes, satisfaction, and adapt services as needed

V. Conclusion

Patient Assistance Programs (PAPs) for cell and gene therapies represent an essential bridge between high-cost innovation and patient access. The benchmarks summarized in this memorandum provide CIRM with a structured lens through which to evaluate awardee proposals, ensuring that access plans are both 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 Programs Team efforts with these industry benchmarks and the Access Plan obligations under Intellectual Property and Revenue Sharing Requirements for Non-Profit and For-Profit Grantees, 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.

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