SAF Update

Jon Thomas, PhD, JD

President & CEO

ICOC

September 25, 2025

CIRM
CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE

Purpose

Provide an update on the SAF status and budget forecast

Strategic Impact Goals

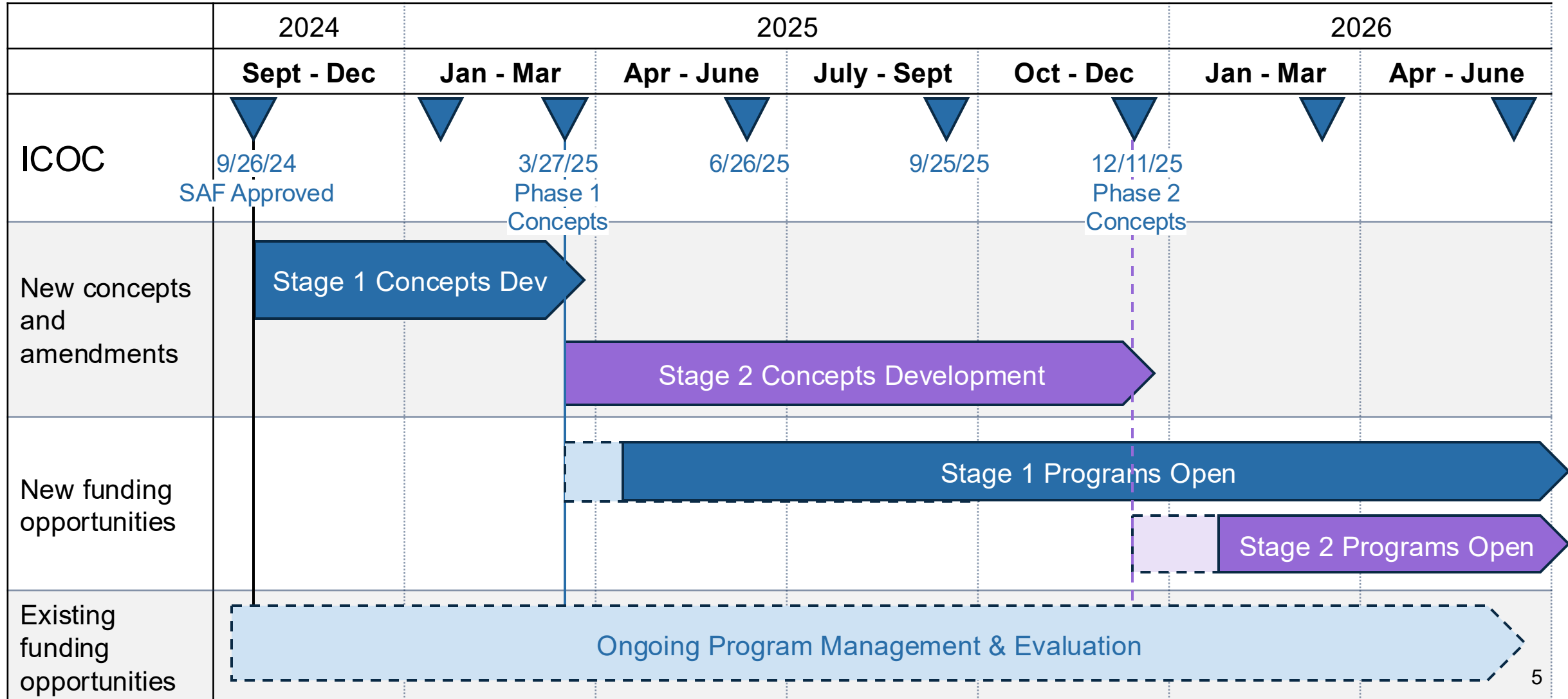| Accelerating Discovery & Translation	Cell & Gene Therapy Approvals	Accessibility & Affordability of Therapies	Diverse Workforce Development
Goal 1: Catalyze the identification and validation of at least four novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California	**Goal 3:** Advance 4-7 rare disease projects to Biologics License Application (BLA)	**Goal 5:** Ensure that every BLA-ready program has a strategy for access and affordability	**Goal 6:** Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine
Goal 2: Accelerate the development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies	**Goal 4:** Propel 15-20 therapies targeting diseases affecting Californians to late-stage trials		

Financial Plan:

$3.5B* in new funding commitments over 6 years
   *Does not include funds for Accessibility & Affordability: (Community Care Centers Excellence Support & Patient Support Program)
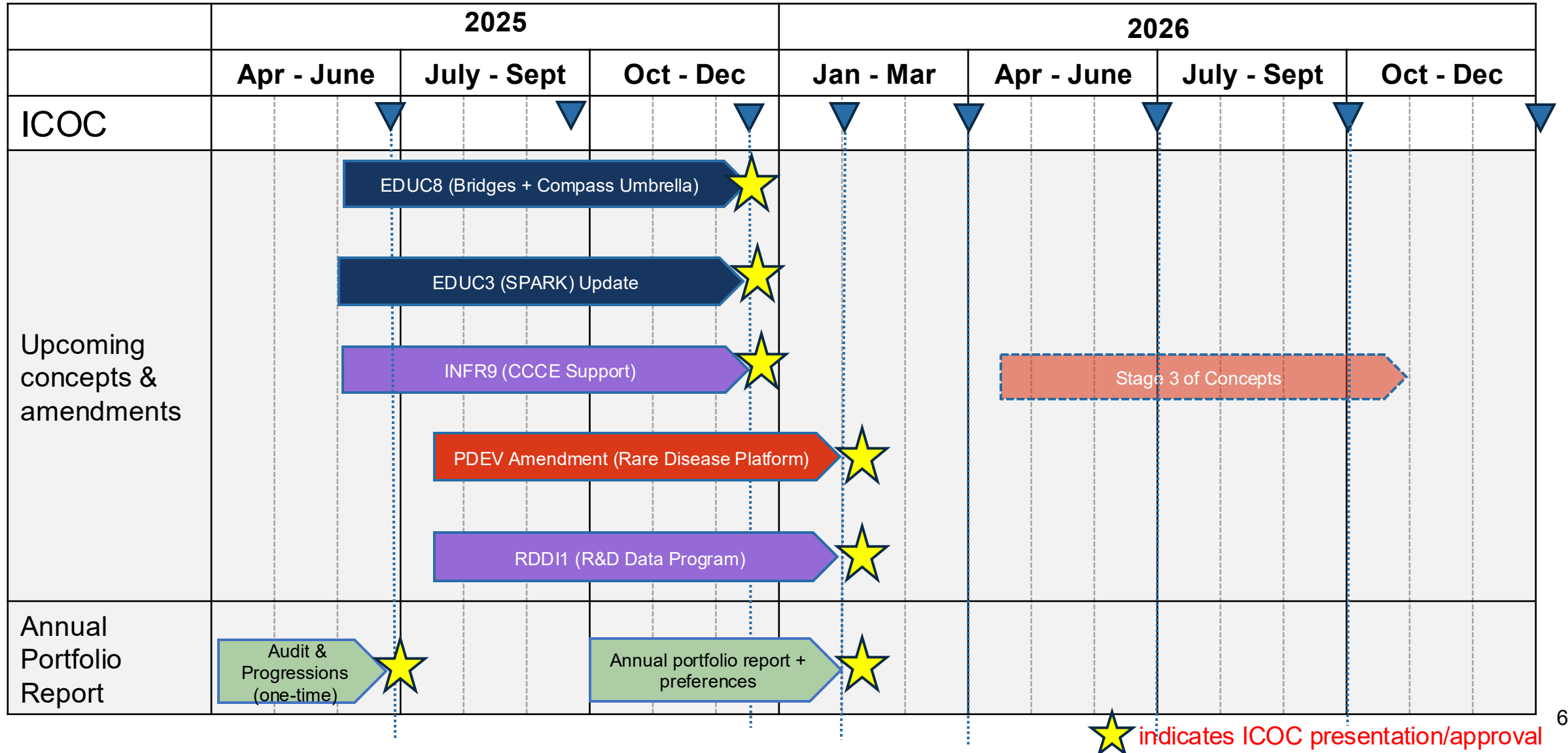
Pillar	2025/26	2026/27	2027/28	2028/29	2029/30	2030/31	Total
CLIN	$135	$135	$235	$135	$135	$235	$1,010
PDEV	$160	$230	$230	$170	$120	$90	$1,000
DISC	$158.2	$162	$162	$162	$162	$162	$968.2
EDUC	$1.5	$119.5	$182.5	$2.5	$2.5	$2.5	$311
INFR*	$36	$10	$40	$87	$10	$10	$193*
TOTAL	$490.7	$656.5	$849.5	$556.5	$429.5	$499.5	$3,482.2

Numbers in the table are in millions of dollars ($000,000). Minor variances due to rounding

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SAF Implementation Timeline

	2024	2025				2026	
	Sept - Dec	**Jan - Mar**	**Apr - June**	**July - Sept**	**Oct - Dec**	**Jan - Mar**	**Apr - June**

ICOC

9/26/24 SAF Approved — 3/27/25 Phase 1 Concepts — 6/26/25 — 9/25/25 — 12/11/25 Phase 2 Concepts

New concepts and amendments

Stage 1 Concepts Dev

Stage 2 Concepts Development

New funding opportunities

Stage 1 Programs Open

Stage 2 Programs Open

Existing funding opportunities

Ongoing Program Management & Evaluation

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SAF Implementation Timeline *Concepts and Portfolio Reports*

	2025			2026			
	Apr - June	July - Sept	Oct - Dec	Jan - Mar	Apr - June	July - Sept	Oct - Dec
ICOC	▼	▼	▼	▼	▼	▼	▼	▼
Upcoming concepts & amendments	EDUC8 (Bridges + Compass Umbrella) ★						
	EDUC3 (SPARK) Update ★						
	INFR9 (CCCE Support) ★				Stage 3 of Concepts		
	PDEV Amendment (Rare Disease Platform) ★						
	RDDI1 (R&D Data Program) ★						
Annual Portfolio Report	Audit & Progressions (one-time) ★		Annual portfolio report + preferences ★				

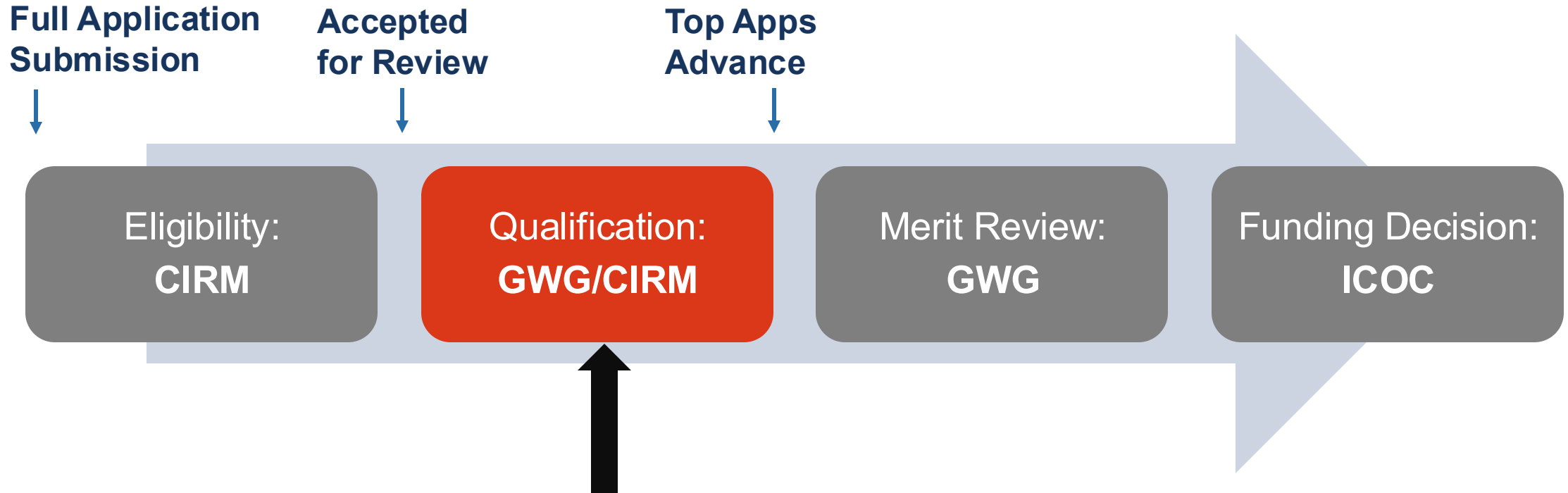★ indicates ICOC presentation/approval

New Program Reviews
Status Update

Gil Sambrano, PhD
Vice President, Portfolio Development & Review
September 25, 2025
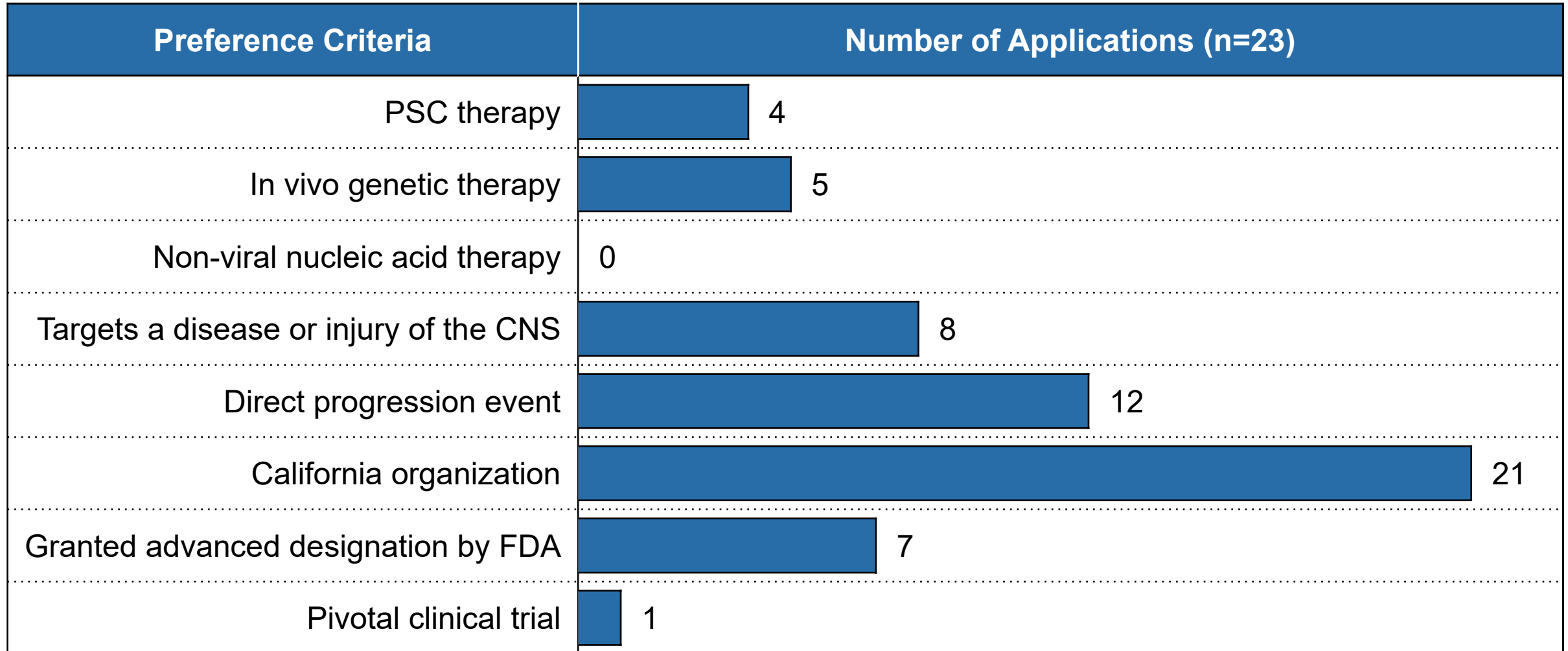
Qualification Process for CLIN2

Full Application Submission

Accepted for Review

Top Apps Advance

GWG Recommendation

Final Approval

Eligibility: **CIRM**	Qualification: **GWG/CIRM**	Merit Review: **GWG**	Funding Decision: **ICOC**

Can the application be reviewed?

Is proposal aligned with CIRM priorities?

Is the application scientifically meritorious?

Should CIRM fund this project?

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Qualification Process for CLIN2

Full Application Submission

Accepted for Review

Top Apps Advance

Eligibility: **CIRM**

Qualification: **GWG/CIRM**

Merit Review: **GWG**

Funding Decision: **ICOC**

The CIRM Review Team examines key information in the applications to score them against objective criteria defined in the PA. Goal is to advance up to 7 applications per cycle.

CLIN2 | Preferences for FY25/26
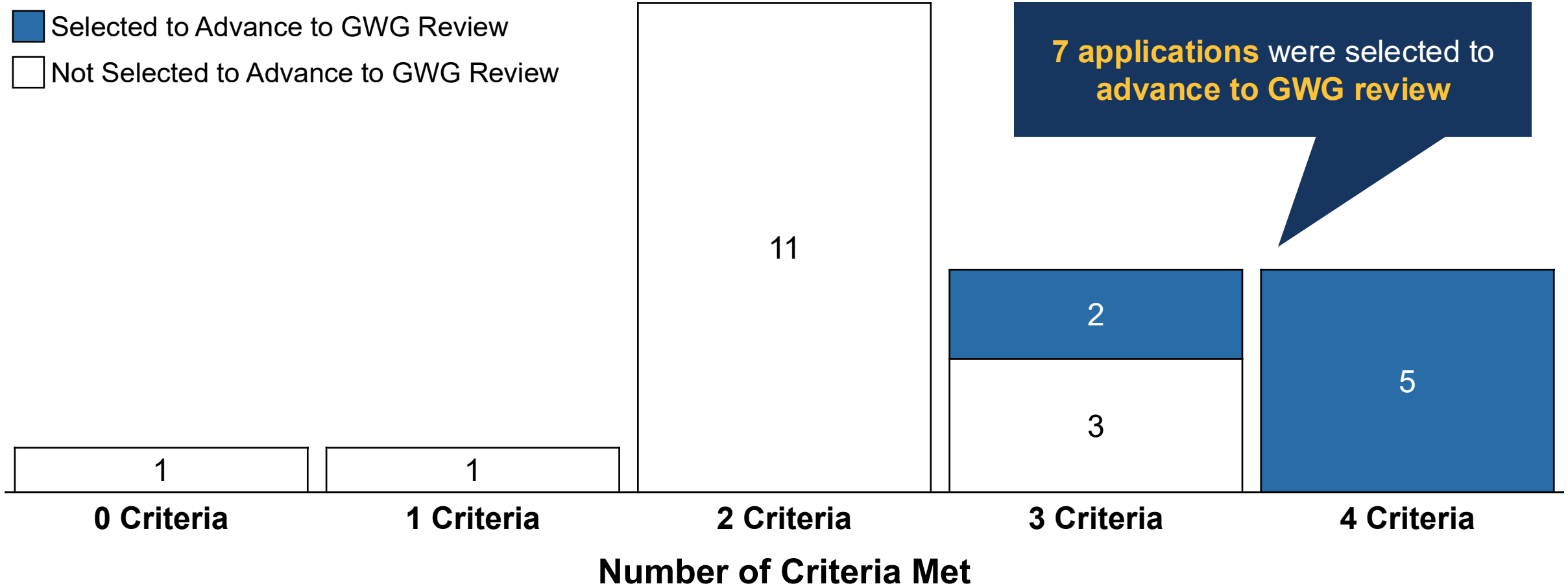
Preferences are factored in during Qualification and ARS review

Concept Preferences	Rationale
Pluripotent stem cell-derived therapies	• Propositions 71 and 14 • Potential to address patient access & affordability barriers
In vivo genetic therapies	• Potential to address patient access & affordability barriers
Non-viral nucleic acid delivery	• Potential to address patient access & affordability barriers
Diseases of the brain and CNS (Prop 14)	• Proposition 14 priority
CA organizations	• CA taxpayer-funded initiative
Progressions from IND-enabling or pipeline trial awards	• Advance CIRM-funded therapies
Fast Track, RMAT, or Breakthrough designations	• Leverage greater FDA access
Pivotal trials	• Fastest route to BLA

Qualification Criteria for First Round of CLIN2

Preference Criteria	Number of Applications (n=23)
PSC therapy	4
In vivo genetic therapy	5
Non-viral nucleic acid therapy	0
Targets a disease or injury of the CNS	8
Direct progression event	12
California organization	21
Granted advanced designation by FDA	7
Pivotal clinical trial	1

PSC: Pluripotent Stem Cell; CNS: Central Nervous System
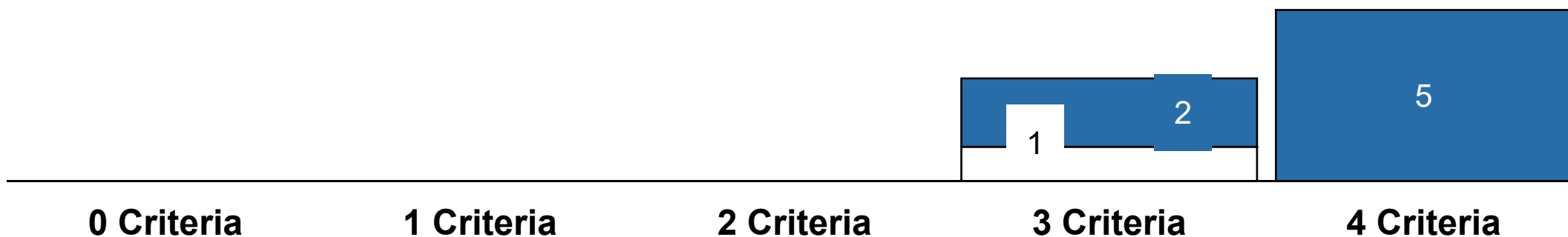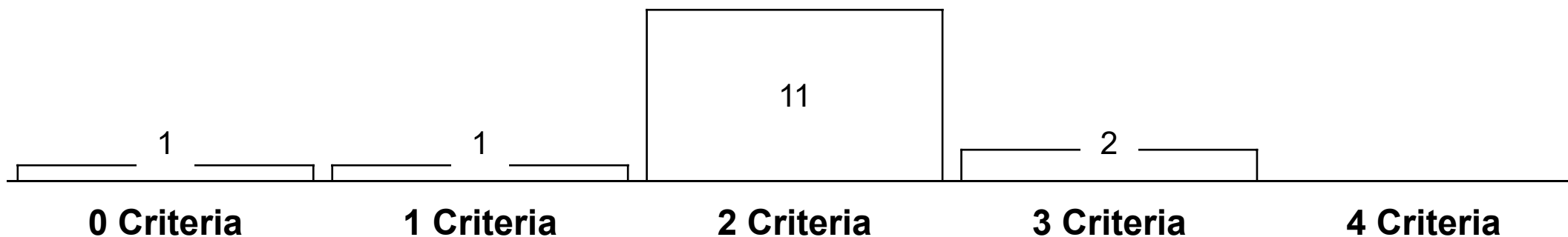
Qualification Process for CLIN2

All top 7 applications are from California organizations and therapies target CNS

- CNS Applications (8)
  - 6 are also pluripotent stem cell or in vivo gene therapies
  - 4 have an advanced designation

- Non-CNS Applications (15)
  - 3 are also pluripotent stem cell or in vivo gene therapies
  - 3 have an advanced designation

Although the top 7 applications are all targeting CNS, the fact that these applications also met additional concomitant preferences contributed to their selection

Summary

- CIRM is applying preferences in the selection of projects to ensure alignment with the SAF.

- Generally, projects require possessing multiple preferences to be selected.

- Although a preference for projects targeting diseases of the CNS is applied, projects addressing any disease area can be selected.

- As we have only completed one round, additional data on the use of preferences will continue to be collected and shared with the Board.

- Preferences are not static. CIRM will present an overview on the portfolio of funded projects each year to inform any changes to preferences that the Board may adopt.