

C I R M

CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE

Grants Working Group Recommendations

PDEV Preclinical Awards

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Agenda

- ① PDEV Program Overview
- ② Review Process
- ③ Recommendations

Strategic Impact Goals

Accelerating Discovery & Translation	Cell & Gene Therapy Approvals	Accessibility & Affordability of Therapies	Diverse Workforce Development
<p>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</p> <p>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</p>	<p>Goal 3: Advance 4-7 rare disease projects to Biologics License Application (BLA)</p> <p>Goal 4: Propel 15-20 therapies targeting diseases affecting Californians to late-stage trials</p>	<p>Goal 5: Ensure that every BLA-ready program has a strategy for access and affordability</p>	<p>Goal 6: Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine</p>

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PDEV

- Accelerate the preclinical development incentivizing multidisciplinary collaborations and rapid progression to IND
- Incorporate **prioritization of innovative therapies for diseases that affect Californians**

PDEV Program

PDEV Program Objective

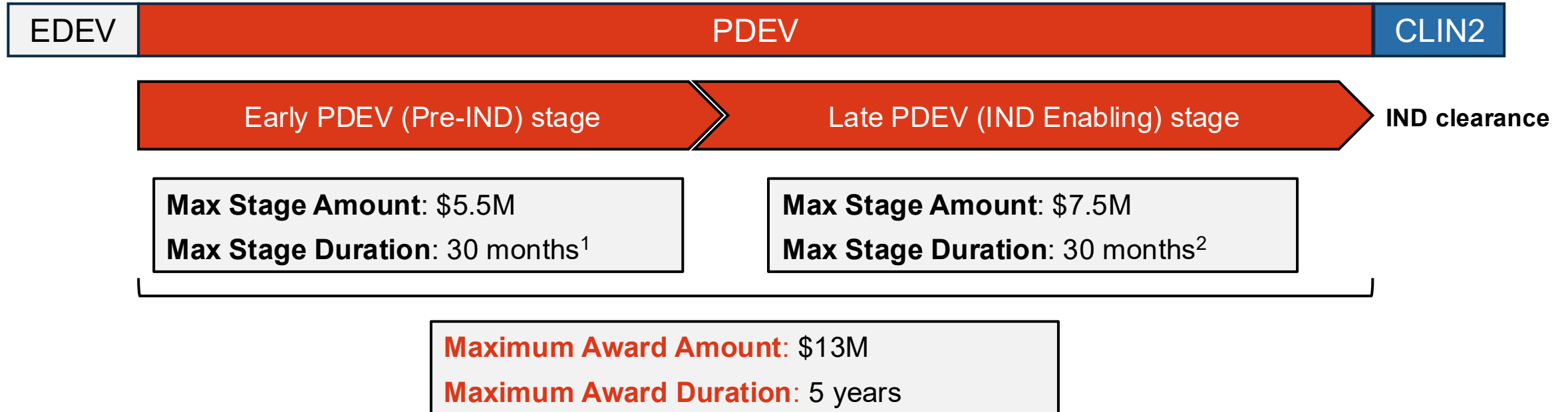
To accelerate completion
of preclinical
development, FDA IND
clearance, and clinical trial
startup for stem cell-based
and genetic therapies



PDEV Program Structure

	PDEV (Preclinical Development)
Recurrence	2x per year
Applicant	California organizations only
Co-funding	None (Unpartnered Non-Profit) 20% (Non-profit with For-Profit partner) 20% (For-Profit)
Maximum Award Amount	\$13 million
Maximum Award Duration	5 years
Awards/Year	12-21
Projection	7 Early (7x\$13M), 9 Late (9x\$7.5M)
Total Funds/Year	\$160M

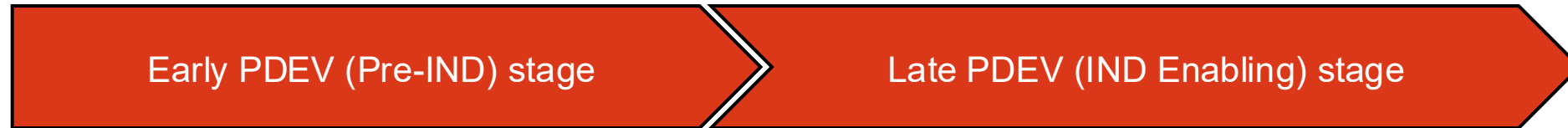
PDEV Award Amount & Duration Vary by Entry Points



Singular Expected Project Outcome: IND Clearance

1 - Inclusive of optional candidate optimization activity (max 6 months)
 2 - Inclusive of optional trial startup activity completion following IND clearance (max 6 months)

Applicants Requesting Funding for Both Early & Late PDEV Activities



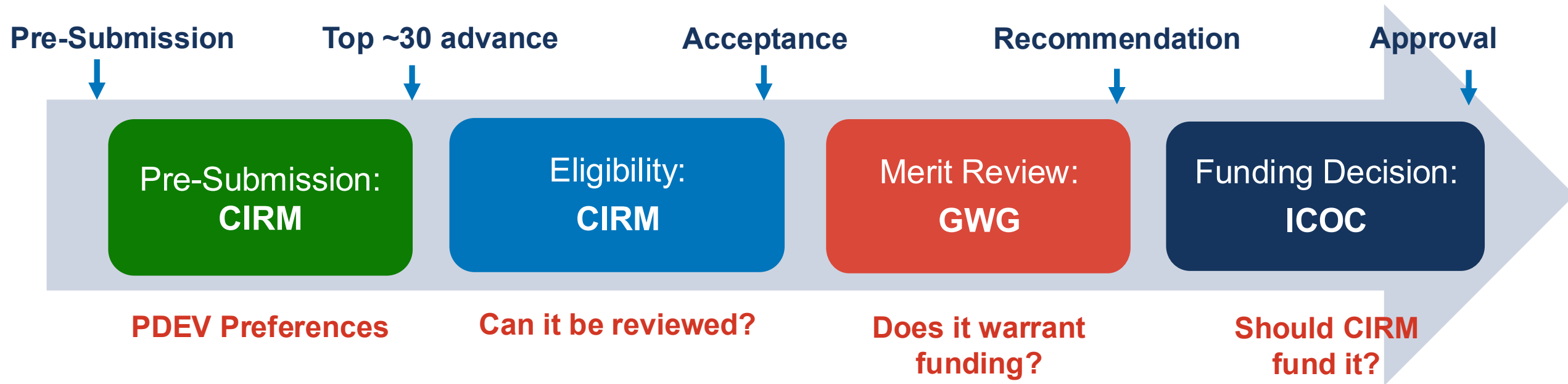
CIRM Award Management

- CIRM will review pre-IND strategy and participate in pre-IND meeting
- After pre-IND meeting, CIRM will review alignment of IND-enabling activities with FDA pre-IND feedback and may modify Operational Milestones & Award Disbursements

Review Process

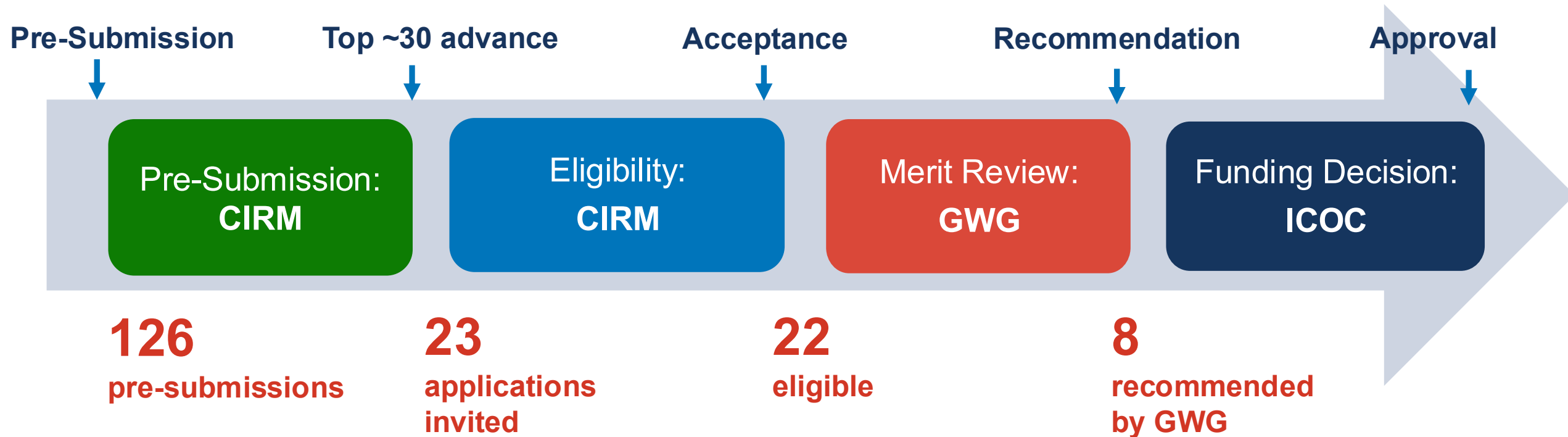


PDEV Application and Review Process



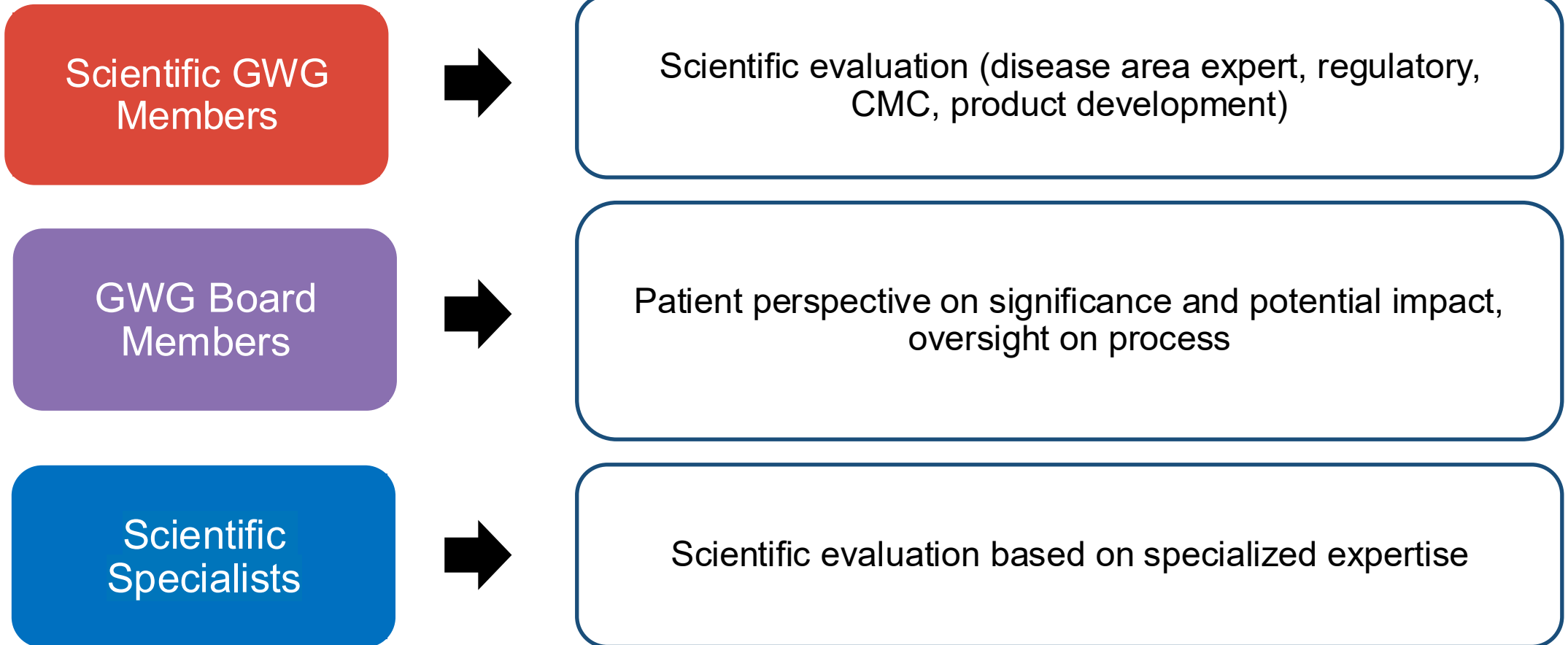
PDEV Application and Review Process

Round 2



PDEV GWG Panel Composition and Roles

Who participates and how they contribute



Scientific Scoring System

Using the 1 to 100 scale

- **Median score of 85-100:** Exceptional merit and warrants funding, if funds are available.
- **Median score of 1-84:** Not recommended for funding.
- Scoring is holistic and based upon all facets of the expert review.
- GWG are encouraged to make full use of the scoring range to signal their enthusiasm for each project.

PDEV Scientific Review Criteria

- **Value Proposition** - Evaluate the extent to which the therapy offers a compelling value proposition
- **Rationale** - Evaluate the scientific rationale for the proposed therapy and the strength of the supporting data
- **Project Plan and Design** - Evaluate the project's plan and design to achieve an active IND
- **Project Team and Resources** - Evaluate the expertise and resources that will be deployed to achieve the project deliverables
- **Population Impact** – Evaluate the extent to which the project considers the potential impact of the proposed therapy across affected populations

Recommendations

GWG Recommendations

Recommendation	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding	8	\$89,618,837	\$42,408,250
Not recommended for funding	14		

For each award, the final award amount shall not exceed the amount approved by the ICOC Application Review Subcommittee and may be reduced contingent on CIRM's assessment of allowable costs and activities.

Minority Reports

- Under Prop 14, any application that is not recommended for funding by the GWG, but which had 35% or more members score to fund the application must include a minority report.
- The minority report is included in the review summary and provides a brief synopsis of the opinion of reviewers that scored the application 85 or above.

Applications with Minority Report

The CIRM Team supports the majority position to not fund application PDEV-19728.

App Number	Title	Funds Requested	Score
PDEV-19728	AAV gene therapy for brain metastases	\$12,999,999	84

CIRM Team Recommendations

App #	Budget Request	GWG Score	Lo	Hi	Y	N	CIRM Recommendation
PDEV-19735	\$12,480,000	91	90	95	14	0	FUND
PDEV-19727	\$7,499,998	90	86	95	14	0	FUND
PDEV-19742	\$12,999,871	90	84	92	14	1	FUND
PDEV-19725	\$7,499,996	87	80	90	12	3	FUND
PDEV-19718	\$12,948,726	85	70	95	12	2	Do not fund
PDEV-19835	\$11,679,420	85	80	85	12	3	Do not fund
PDEV-19729	\$11,623,155	85	70	87	11	4	Do not fund
PDEV-19780	\$12,887,671	85	80	90	8	6	Do not fund
Total Applicant Request				Funds Available			
\$40,479,865				\$42,408,250			

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CIRM Team Recommendations Rationale

- The CIRM Team recommends funding the top four applications recommended by the GWG (Grants Working Group) and to *not* fund the bottom four applications recommended by GWG. The GWG evaluations showed a clear separation in score of the top four applications and the CIRM Team's examination of the competitive landscape and their fit within the CIRM portfolio supports this ranking. The CIRM Team did not find any compelling reason to advance funding of any lower ranked application.