



# Standards Meeting Clinical Portfolio Update

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January 5, 2022



## OUR MISSION

Accelerating world class science  
to deliver transformative  
regenerative medicine treatments  
in an equitable manner to a  
diverse California and world

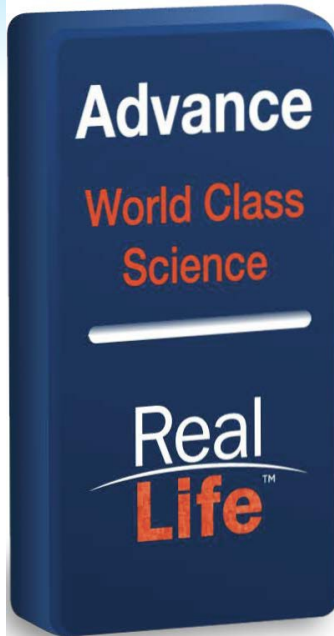


# Strategic Plan Principles

The new strategic plan is designed to **enhance**, **organize** and **interconnect** CIRM's proven funding model to achieve the overarching goals.



# 5-Year Strategic Summary



- Develop Competency Hubs
- Build Knowledge Networks



- **Advance therapies to marketing approval**
- Create a manufacturing partnership network
- Expand Alpha Clinics Network
- Create Community Care Centers of Excellence



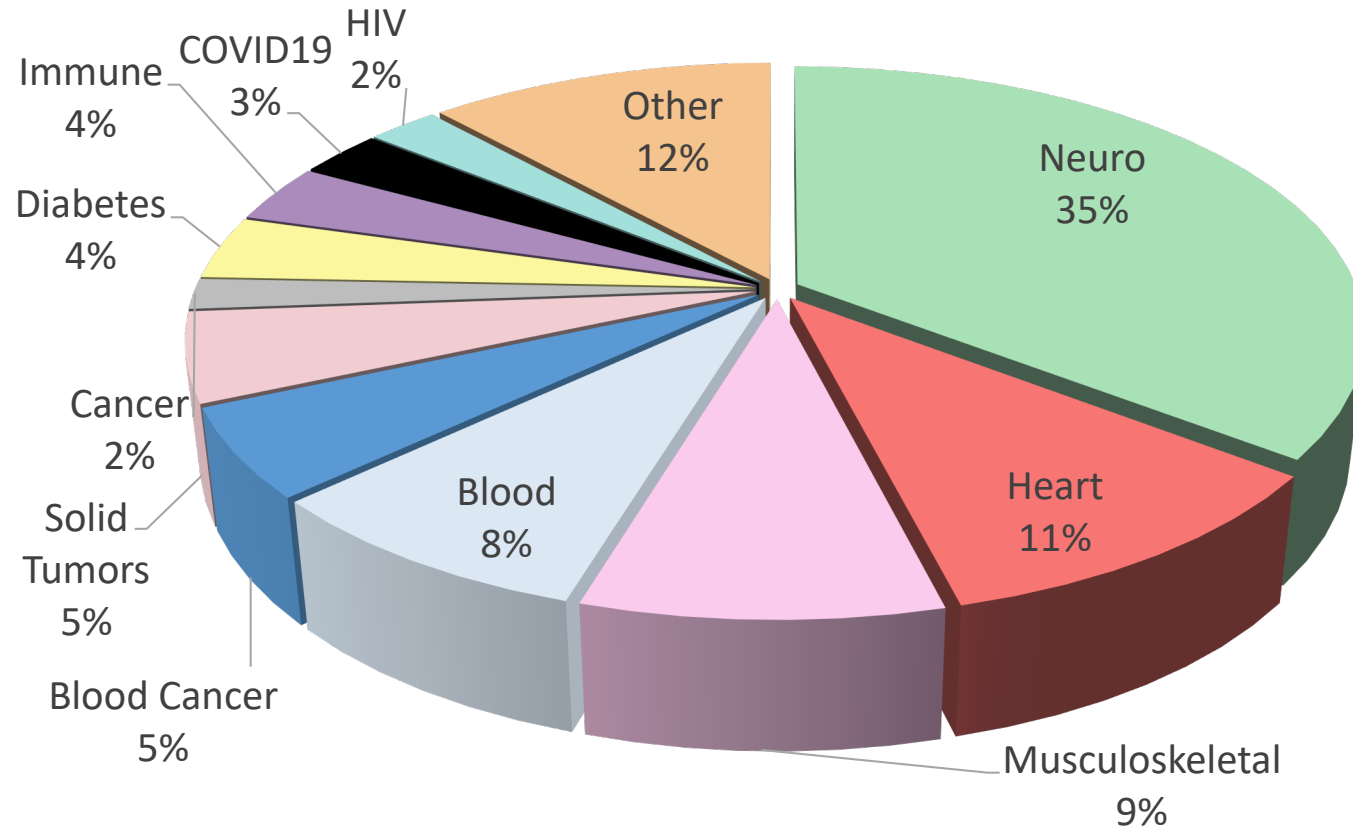
- Build a diverse and highly skilled workforce
- Deliver a roadmap for access and affordability



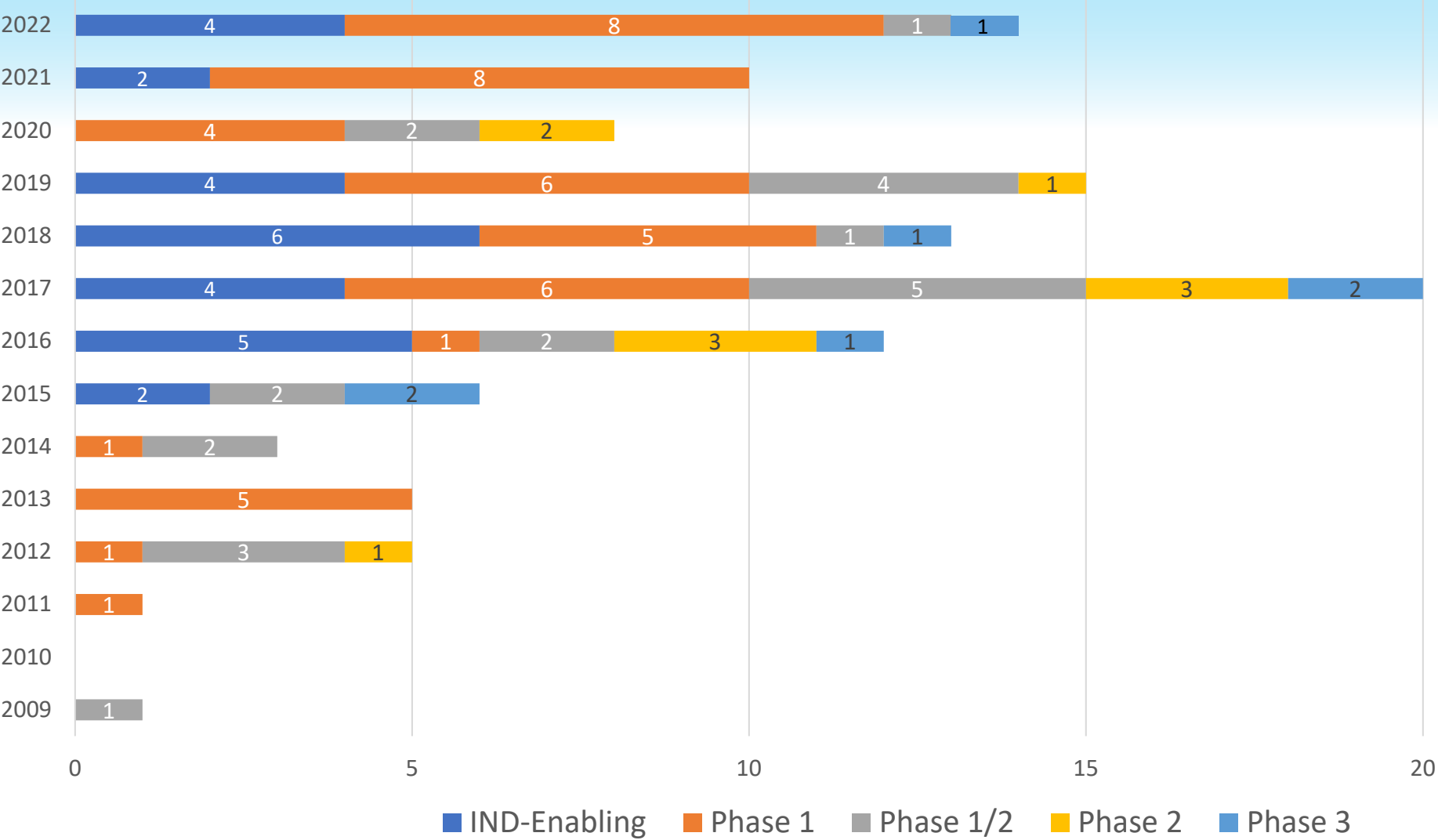
Pre-IND Meeting Prep	IND-Enabling	Early Clinical	Mid-Clinical	Pivotal-Late Clinical
35.5%	11.2%	34.6%	13.1%	5.6%

**81.3%** of our current portfolio = de-risking projects

As of September 2022.  
% Total R&D awards



# Clinical Portfolio (CLIN1 and CLIN2)



- CIRM has enabled the funding of 86 clinical trials
- The clinical trials cover several therapeutic areas in various development phases
- Over 50% of CIRM-funded clinical projects are partnered with industry

2004-2016/17

2016 | 27

2017 | 42

2018 | 48

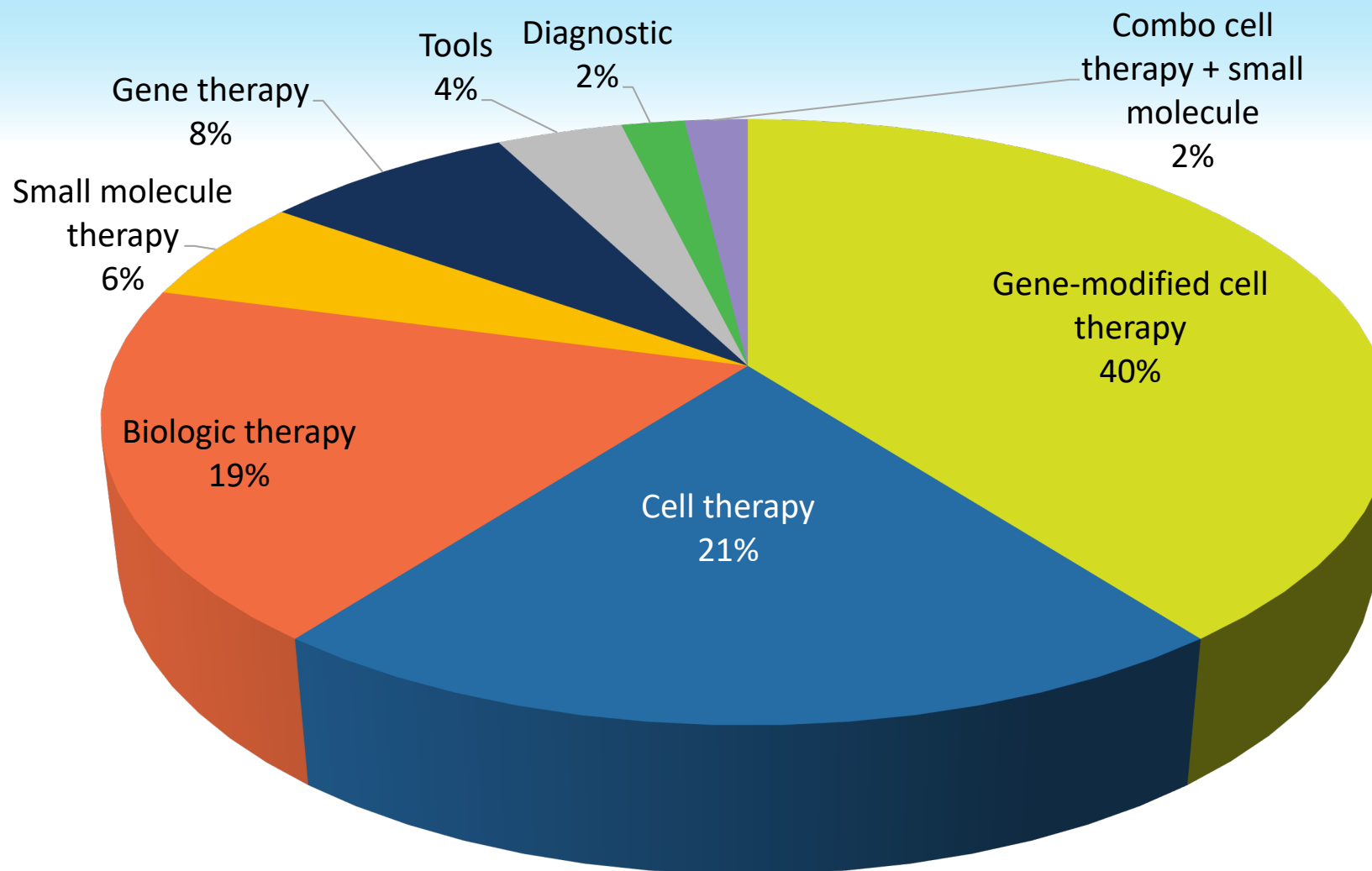
2019 | 57

2020 | 67

2021 | 74

2022 | 86 Clinical Trials

# Supporting Diverse Therapeutic Modalities





Disease Areas	Investigator	Organization	Trial Status	Target Enrollment
Blood disorder	Tippi MacKenzie	University of California, San Francisco	Enrolling by invitation	10
Neurobiology	Clive Svendsen	Cedars-Sinai Medical Center	Recruiting	16
Blood cancer	David Miklos	Stanford University	Recruiting	60
Blood cancer	Maria Grazia Roncarolo	Stanford University	Recruiting	22
Urinary disorder	James Yoo	Wake Forest University Health Sciences	Launching	10
Solid cancer	Christine Brown	City of Hope, Beckman Research Institute	Active, not recruiting	82
Solid cancer	Jana Portnow	City of Hope, Beckman Research Institute	Launching	36
Solid cancer	Saul Priceman	City of Hope, Beckman Research Institute	Recruiting	39
Solid cancer	Leo Wang	City of Hope, Beckman Research Institute	Recruiting	18
Solid cancer	Crystal Mackall	Stanford University	Recruiting	54
Ophthalmology	Sophie Deng	University of California, Los Angeles	Recruiting	20
Infectious disease	John Zaia	City of Hope, Beckman Research Institute	Active, not recruiting	N/A
Neurobiology	Cory Nicholas	Neurona Therapeutics	Recruiting	40
Cardiovascular	Joseph Wu	Stanford University	Recruiting	18
Infectious disease	William Kennedy	Excision BioTherapeutics	Recruiting	9

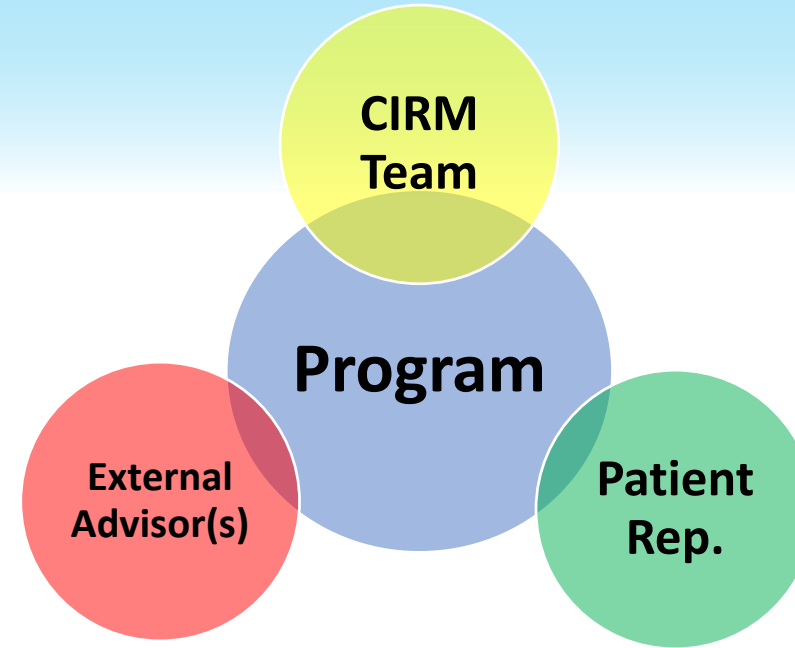
Disease Areas	Investigator	Organization	Trial Status	Target Enrollment
IPEX Syndrome	Rosa Bacchetta	Stanford University	Recruiting	36
Transplant	Robert Lowsky	Stanford University	Recruiting	15
Transplant	Everett Meyer	Stanford University	Recruiting	22
Blood cancer	Joseph Woodard	Immune-Onc Therapeutics	Recruiting	119
Solid cancer	Steven Dubinett	University of California, Los Angeles	Recruiting	24
Neurobiology	Krystof Bankiewicz	Brain Neurotherapy Bio	Recruiting	12
Blood disorder	Donald Kohn	University of California, Los Angeles	Recruiting	6
Ophthalmology	Clive Svendsen	Cedars-Sinai Medical Center	Recruiting	16
Solid cancer	Theodore Nowicki	University of California, Los Angeles	Recruiting	12
Blood disorder	Morton Cowan	University of California, San Francisco	Recruiting	25
Blood disorder	Joseph Rosenthal	City of Hope, Beckman Research Institute	Recruiting	6
Blood disorder	Mark Walters	University of California, San Francisco	Launching	9
Solid cancer	Pamela Contag	BioEclipse Therapeutics INC.	Recruiting	24
Neurobiology	Diana Farmer	University of California, Davis	Recruiting	55
Neurobiology	Gary Steinberg	Stanford University	Recruiting	30

Disease Areas	Investigator	Organization	Trial Status	Target Enrollment
Infectious disease	Michael Pulsipher	Children's Hospital of Los Angeles	Active, not recruiting	60
Infectious disease	William van der Touw	Cellularity Inc	Active, not recruiting	86
Cystinosis	Stephanie Cherqui	University of California, San Diego	Recruiting	6
Blood cancer	Mehrdad Abedi	University of California, Davis	Recruiting	18
Infectious disease	Steven Deeks	University of California, San Francisco	Recruiting	12
Blood disorder	Kinnari Patel	Rocket Pharmaceuticals, Inc.	Active, not recruiting	9
Blood disorder	Stephen Gottschalk	St. Jude Children's Research Hospital	Recruiting	28
Blood disorder	Donald Kohn	University of California, Los Angeles	Recruiting	16
Muscle	Peter Belafsky	University of California, Davis	Recruiting	62
Cardiovascular	Michael Lewis	Cedars-Sinai Medical Center	Active, not recruiting	26
Type 1 diabetes	Peter Stock	University of California, San Francisco	Recruiting	8
Type 1 diabetes	Manasi Jaiman	ViaCyte, Inc.	Active, not recruiting	75

Disease Areas	Investigator	Organization	Trial Status	Target Enrollment
Infectious disease	Michael Matthay	University of California, San Francisco	Recruiting	120
Ophthalmology	Henry Klassen	jCyte, Inc	Active, not recruiting	84
Ophthalmology	Henry Klassen	jCyte, Inc	Active, not recruiting	35
Blood disorder	David Williams	Boston Children's Hospital	Recruiting	10

Disease Areas	Investigator	Organization	Trial Status	Target Enrollment
Neurobiology	Ralph Kern	BrainStorm Cell Therapeutics	Active, not recruiting	263
Transplant	Daniel Brennan	Medeor Therapeutics, Inc.	Active, not recruiting	30
On Vascular Niche	Finnegan	Angiocrine	Active, recruiting	140

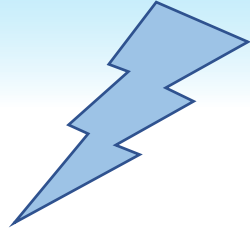
- **Purpose** of a **CAP** is to provide guidance and advice to the project team
- A **CAP** is assembled by CIRM for each Clinical Stage Award
- Multiple **CAP Meetings** occur over lifetime of a program award



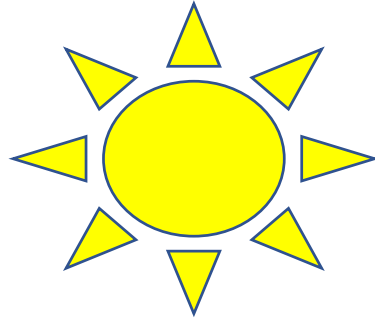
- 356 CAP meetings since 2016
- 91 External advisors
- 68 Patient representatives



# CAPs Make a Difference



- Resolving challenges
- Optimizing project execution
- Discovering critical information
- Strategic, Technical, Regulatory Guidance



## Outcomes:

- Manufacturing challenges overcome
- Clinical trial design optimized
- Enrollment enhanced
- Regulatory advice provided
- Partnering facilitated
- Development Path delineated

**CAP advice impacted >75% of CLIN awards**

- Translation & Clinical Portfolio is diverse, covers multiple Therapeutic areas
- Several of the clinical grants are in early development consistent with CIRM de-risking development
- Some grants progressed to later stage development working closely with CIRM
- The Translational and Clinical Advisory Panels resolved several technical, regulatory and strategic challenges and facilitated partnerships.

# Thank You!

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