Therapeutics Development Hunting Strategy

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ICOC Meeting
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Our Mission

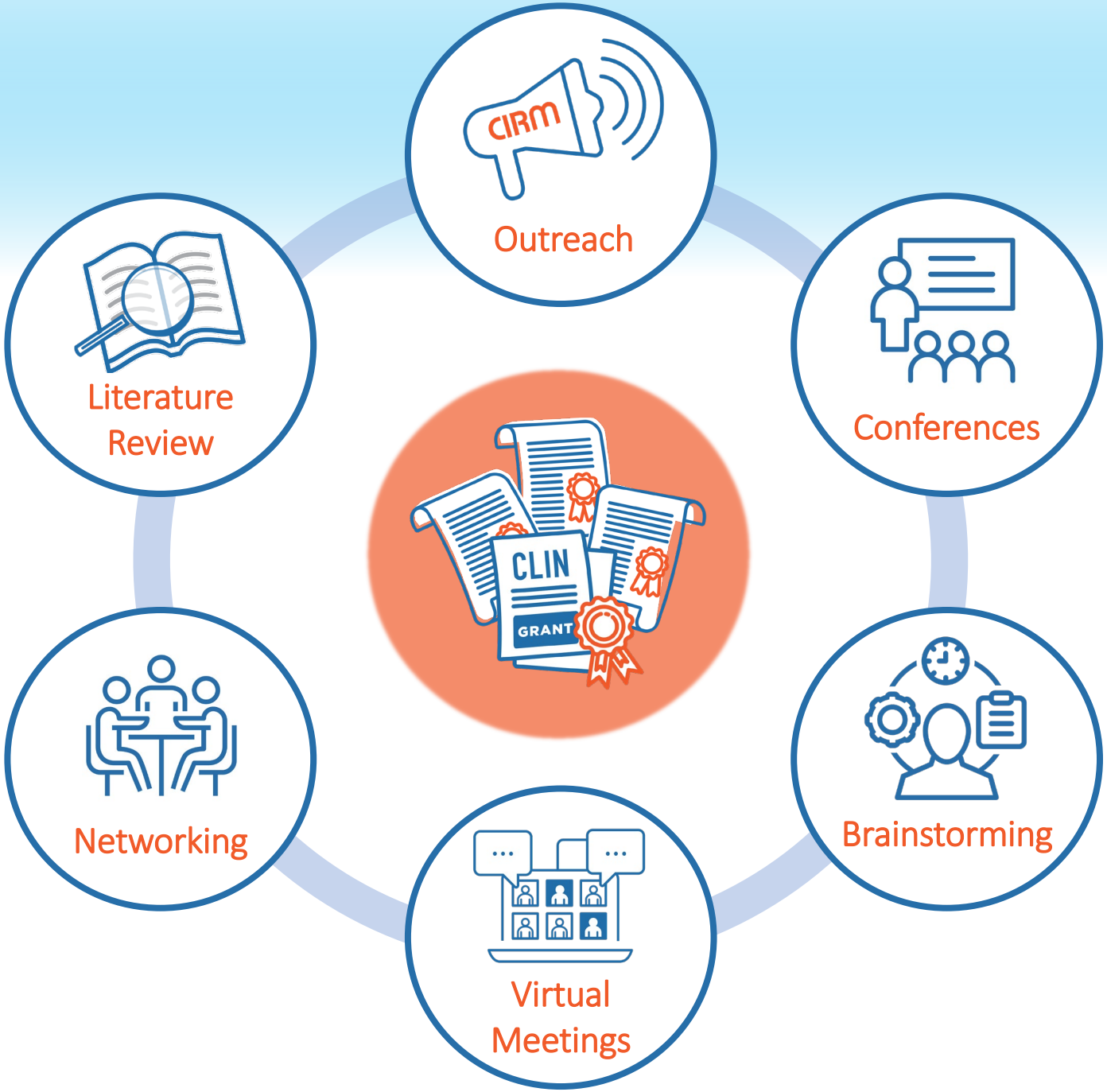
OUR MISSION

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

What is Hunting?

- Identifying & recruiting innovative cell & gene therapy projects
- Supporting applicants until grant submission

The Hunting Process



Building our Clinical Portfolio

Incoming Applications | Pre-Submission Activities | Review | Approved Grants

Progression Events

Hunted Applications

Unsolicited Applications

Pre-submission Eligibility

Discussion & Education

Proposal Feedback

Submission, Eligibility, Review, GMS, Legal

Grants Working Group

CIRM BOARD

“Wall” between Therapeutics Development and Review

Hunting Tools

Apply for Clinical Trial Funding

The **California Institute for Regenerative Medicine (CIRM)** funds the development of regenerative medicine approaches that address unmet medical needs, including progenitor and stem cell-based therapies, small molecules/biologics that involve stem cells, and genetic therapies.

Who can apply?

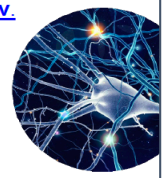
- **California organizations:** Funds may cover costs incurred both in CA and outside CA.
- **Non-California organizations:** Funds may cover costs incurred within CA and fully loaded costs of treating patients in CA. International organizations may also apply.

When should I apply?

Applications are accepted at **2pm PST** on the last business day of each month. A successful application can be funded within 90 days of submission. Pre-submission consultation with CIRM's Therapeutic Development team is encouraged. Reach the CIRM team at clinical@cirm.ca.gov.

Neuroscientists encouraged to apply!

CIRM allocated $1.5B in funding dedicated to the development of treatments for diseases affecting the Central Nervous System (CNS).



To learn more about CIRM clinical trial funding opportunities, scan the QR code or visit us at www.cirm.ca.gov

Living Our Mission

We work with **focus** and **urgency**, as though someone's life depends on it. **Because it does.**

CIRM CLINICAL TRIAL FUNDING — FAQS

What clinical trial activities can CIRM support?

CIRM's clinical trial funding programs support eligible projects conducting IND-enabling studies (**CLIN1**) and clinical trials (**CLIN2**). Supported activities include:

- **CLIN1:** IND-enabling studies, manufacturing to support IND filing or intended clinical trial, and clinical trial start-up activities.
- **CLIN2:** manufacturing to supply the proposed trial, conduct and completion of clinical trials, from first-in-human through registrational, and correlative studies associated with the proposed trial.

What is the award amount and duration?

CLIN1 up to $6M and 24 months to IND filing, **CLIN2** up to $15M and 48 months. Co-funding may be required, depending upon the stage and the applicant's for-profit status. See Program Announcement on our website for details.

How can my project get funded?

Applicants should propose compelling support for their approach's ability to impact unmet medical needs. Applications are reviewed based upon scientific merit, readiness, project plans, and plans to ensure adequate demographic diversity in the study population.

What is the application and award timeline?

A successful CLIN1 or CLIN2 application can be funded **within 90 days** of submission. There is no funding line, and post-review revision and re-submission is encouraged. See Program Announcement on our website for details.

To learn more about CIRM clinical trial funding, scan the QR code or contact us at clinical@cirm.ca.gov.

TRAN Hunting Process and Statistics

TRAN applicant engagement:

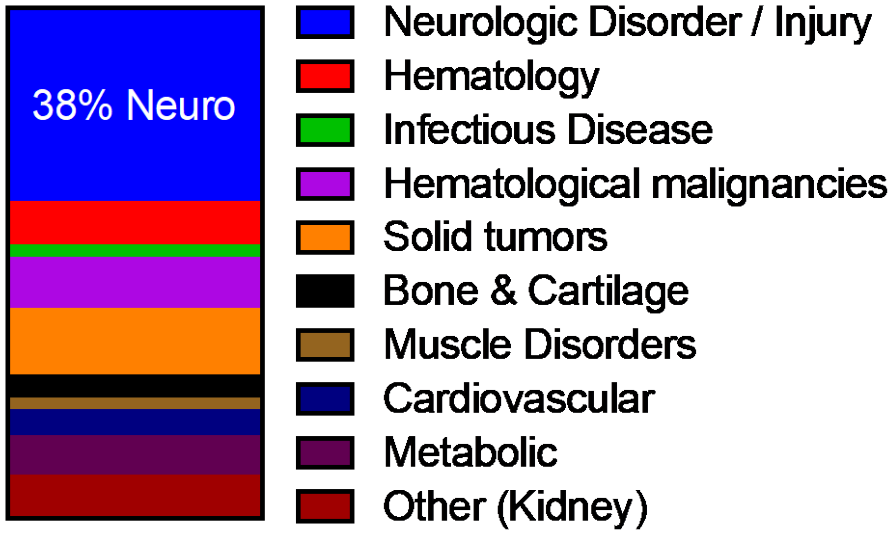
- Only CA organizations are eligible
- Applications offered twice a year
- All applicants are offered a pre-submission consultation during a one-month window in which application is available.
- In the last two rounds of the TRAN program, CIRM staff connected with ~80 applicants in total, 39 submitted applications.

CLIN1 and CLIN2 Hunting List

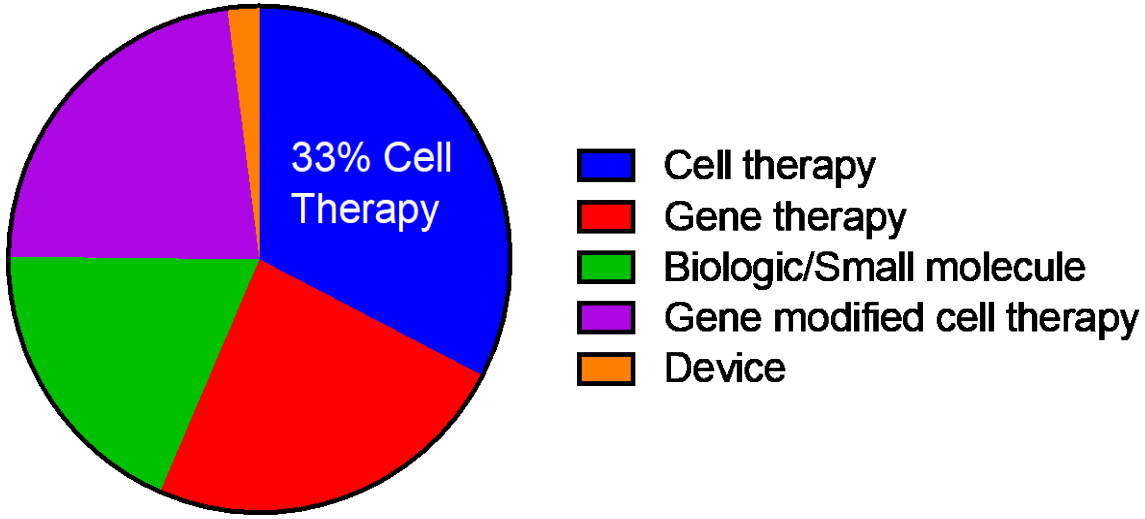
487 Entries- Hunting list (2016-2023)

- 63% CLIN2; 37% CLIN1
- 68% for-profit, 32% non-profit
- 38% in Neuro
- Mix of therapeutic approaches

Indication:



Therapeutic Approach:



Success Rate of CIRM CLIN applicants

Applicants who work with CIRM before submission	Applicants who don't work with CIRM before submission
66% (n=151)	9% (n=11)

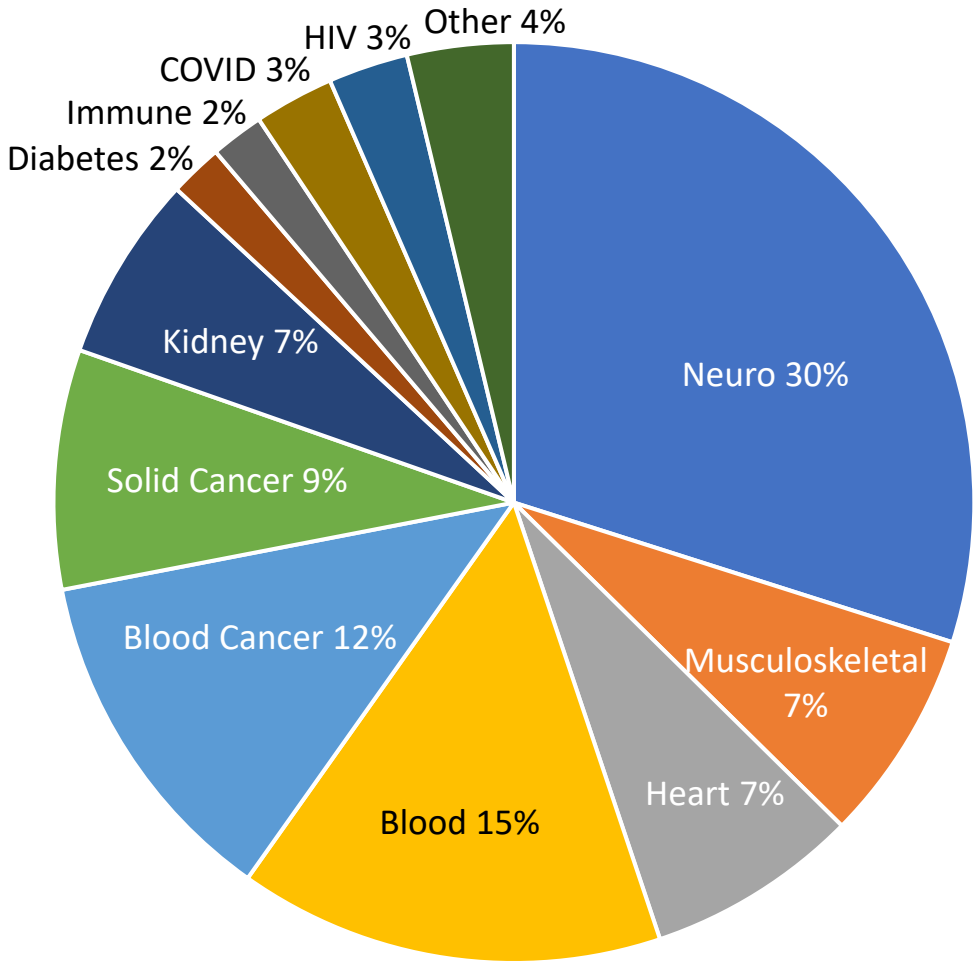
Hunting achievements

Current Translational and Clinical Portfolio

Pre-IND Meeting Prep	IND-Enabling	Early Clinical	Mid-Clinical	Pivotal-Late Clinical
33.6%	14.0%	46.7%	3.7%	1.9%

94.4% of our current portfolio = de-risking projects

As of March15, 2023
% Total R&D awards



Updated: Mar 2023

Clinical Awards – Progression Events*

PI*	Institution	Indication	DISC	TRAN	CLIN1	CLIN2	CLIN2
Leslie Thompson	UC Irvine	Huntington's Disease					
Krys Bankiewicz	UCSF	Parkinson's Disease					
Ying Zhu	Ankasa	Spinal Fusion					
Ezra Cohen	UCSD	Blood Cancer ALL					
Clive Svendsen	Cedars-Sinai	Retinitis pigmentosum					
Diana Farmer	UC Davis	Spina bifida					
Gary Steinberg	Stanford	Stroke					
Saul Priceman	City of Hope	Breast cancer brain mets					
Cory Nicholas	Neurona Tx	Epilepsy					
Christine Brown	City of Hope	GBM					
Mort Cowan	UCSF	SCID-Artemis					
Wendy Pang	Jasper	SCID Transplant*					
Mark Chao	Forty Seven Inc	Blood Cancer AML*					
Sophie Deng	UCLA	Cornea LSC deficiency					
Toni Ribas	UCLA	Solid cancer					
Rosa Bacchetta	UCLA	Blood disorder IPEX					
Mark Walters	UCSF	Sickle Cell Disease					
Tippi Mackenzie	UCSF	Alpha Thalassemia					
John Zaia	City of Hope	HIV					
Kristen Johnson	Calibr	Osteoarthritis					
Joseph Wu	Stanford	Heart disease					
Clive Svendsen	Cedars-Sinai	ALS					
Stephanie Cherqui	UCSD	Kidney, cystinosis*					
Tom Kipps	UCSD	Blood Cancer ALL					
Mark Chao	Forty Seven Inc	Solid cancer*					
Nancy Lane	UC Davis	Osteonecrosis					
Mike Lewis	Cedars-Sinai	Lung PAH					
Manasi Jaiman	Viacyte	Type 1 Diabetes*					
Henry Klassen	jCyte	Retinitis pigmentosum*					
Linda Marban	Capricor	DMD					
Paul Finnegan	Angiocrine	Mucositis					

*2004-2023 * Partnered Grants

A Total of 126
CLIN 1 & CLIN2 Awards:

- 5 : DISC-TRAN-CLIN (Huntington's, Spinal Fusion, Artemis SCID, Osteoarthritis, Type 1 Diabetes)
- 12: TRAN- CLIN
- 21: CLIN1- CLIN2

- 25% (31 of 126) grants progressed to Clinical stage
- 19% (6 of 31) grants are partnered

Clinical Grant Awardees with FDA Accelerated Designations

	Clinical Phase	Disease Area	Investigator	Institution	FDA Designation
1	1	Blood disorder (Artemis SCID)	Cowan, Morton	UCSF	RMAT
2	1	Blood disorder (All SCID)	Pang, Wendy	Jasper Therapeutics	Fast-Track, Rare Pediatric Disease Priority Disease Voucher
3	1/2	LAD-1 Deficiency	Patel, Kinnari	Rocket Pharma	RMAT, Prime
4	1	Bone (infantile Malignant Osteopetrosis)	Rao, Gayatri	Rocket Pharma	Fast-Track
5	1/2	Blood disorder (ADA SCID)	Kohn, Donald	UCLA	Breakthrough, Rare Pediatric Disease Priority Disease Voucher
6	1/2	Blood disorder (X SCID)	Gottschalk, Stephen	St. Jude	RMAT
7	2	Kidney	Lawson, Jeffrey	Humacyte	RMAT
8	1/2	Spinal cord	Binette, Francois	Asterias (Lineage Cell Therapeutics)	RMAT
9	1/2	Retinitis pigmentosa	Klassen, Henry	jCyte	RMAT
10	3	Organ Transplants	Brennan, Daniel	Medeor Therapeutics	RMAT
11	2	Multiple myeloma	Belani, Rajesh	Poseida Therapeutics	RMAT

RMAT: Regenerative Medicine Advanced Therapies

Clinical Grant Awardees with FDA Accelerated Designations*

	Clinical Phase	Disease Area	Investigator	Institution	FDA Designation
12	3	Cancer	Finnegan, Paul	Angiocrine Bioscience	RMAT
13	2	Muscle (Duchenne Muscular Dystrophy)	Marban, Linda	Capricor Therapeutics	RMAT, Rare Pediatric Disease Priority Disease Voucher
14	1b	Blood cancer (AML, MDS)	Chao, Mark	Forty- Seven Inc.	Fast-Track, Breakthrough
15	1	Blood Cancer (AML, CMML)	Woodard, Paul	Immune-Onc Therapeutics	Fast-Track

*2016-2023 RMAT: Regenerative Medicine Advanced Therapies

21% (15 of 71)* Clinical Grantees attained FDA Accelerated Designations
Fast Track, RMAT, Breakthrough

Summary

- Hunting continues to be a resourceful Grants recruitment strategy
- Therapeutics Development welcomes your input to enhance, diversify, or potentially focus the clinical portfolio

Thank you

Therapeutics Development Team