

SOMETHING BETTER THAN HOPE

CIRM Scientific Strategy Advisory Panel



Maria T. Millan, M.D.
President & CEO
California Institute for Regenerative Medicine
February 22, 2021



Our Mission

Accelerate Stem Cell Treatments
To Patients with
Unmet medical needs.

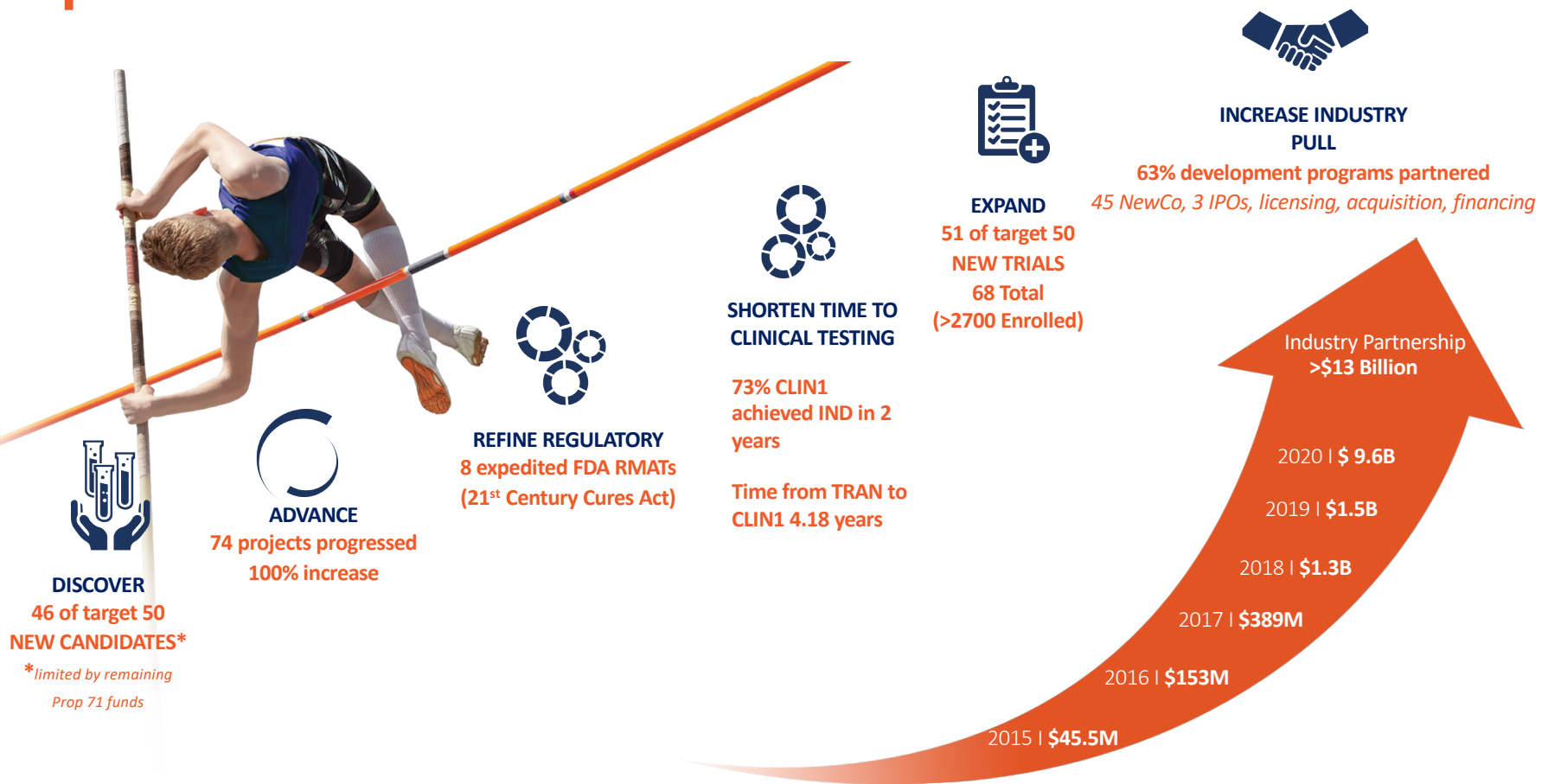
CIRM created by Patient Advocates and California Stakeholders
\$3B Bond under Proposition 71 (2004-2020)

- 1027 Awards in 5 Pillars:
Education, Infrastructure, Discovery, Translational, Clinical

- 68 Clinical Trials (most first in human)

\$5.5B Bond Initiative under Proposition 14 passed November 2020

Outcome of CIRM 5-yr Strategic Goals (2016-2020)



CIRM Investments



INFRASTRUCTURE

\$480M



EDUCATION

\$220M



DISCOVERY

\$900M



TRANSLATION

\$360M



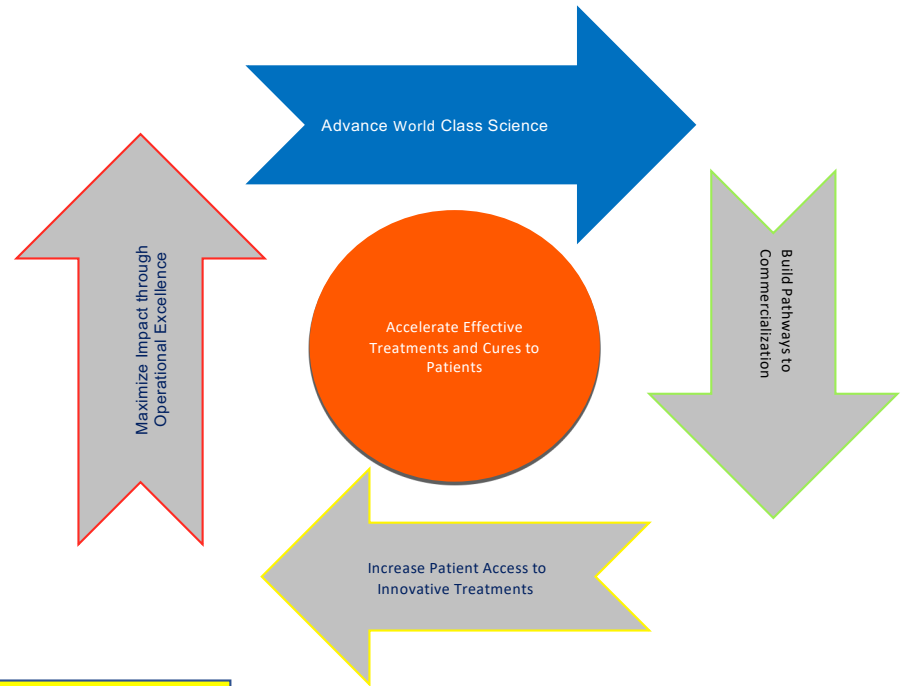
CLINICAL

\$740M

Strategic Themes for CIRM under Proposition 14



Leverage & Build on Established Funding Model



Advance World Class Science

Accelerating Scientific Advancements	<ul style="list-style-type: none"> • Consortium approach • “Team Science” and built-in collaborations • Shared technology cores and infrastructure • Data & Knowledge Networks • DEI principles to address the “real world”
Clinical Paradigm	Next generation trial design (long-term studies, Real World Evidence, Patient Centric endpoints, consortia models, post-marketing)
Strategic Partnerships	Tangible deliverables from recently implemented demonstration cases: <ul style="list-style-type: none"> • NHLBI for Cure Sickle Cell • CZI for COVID genomics
Training Future Scientists and Workforce	“on-ramps” along educational and career stages, incorporate DEI & integrated into other CIRM pillars <i>(e.g. hands-on experience in CIRM funded research labs and infrastructure programs such as clinical research exposure Alpha Clinics Network and internships in future manufacturing initiatives)</i>

CIRM Acceleration Model and Funding Pillars well suited to support Consortia:

Future Consortia: Knowledge Network, Built-in Efficiencies, Partnership Hub & Platform

Special Calls e.g. emergency COVID-19 RFA's

Clinical Infrastructure: Alpha Clinics Network

Genomics Program *supports CIRM researchers & collab., e.g. CZI MOU*

Sickle Cell
example. NHLBI MOU



Education



Infrastructure



Discovery



Translation



Clinical

Meeting Format:

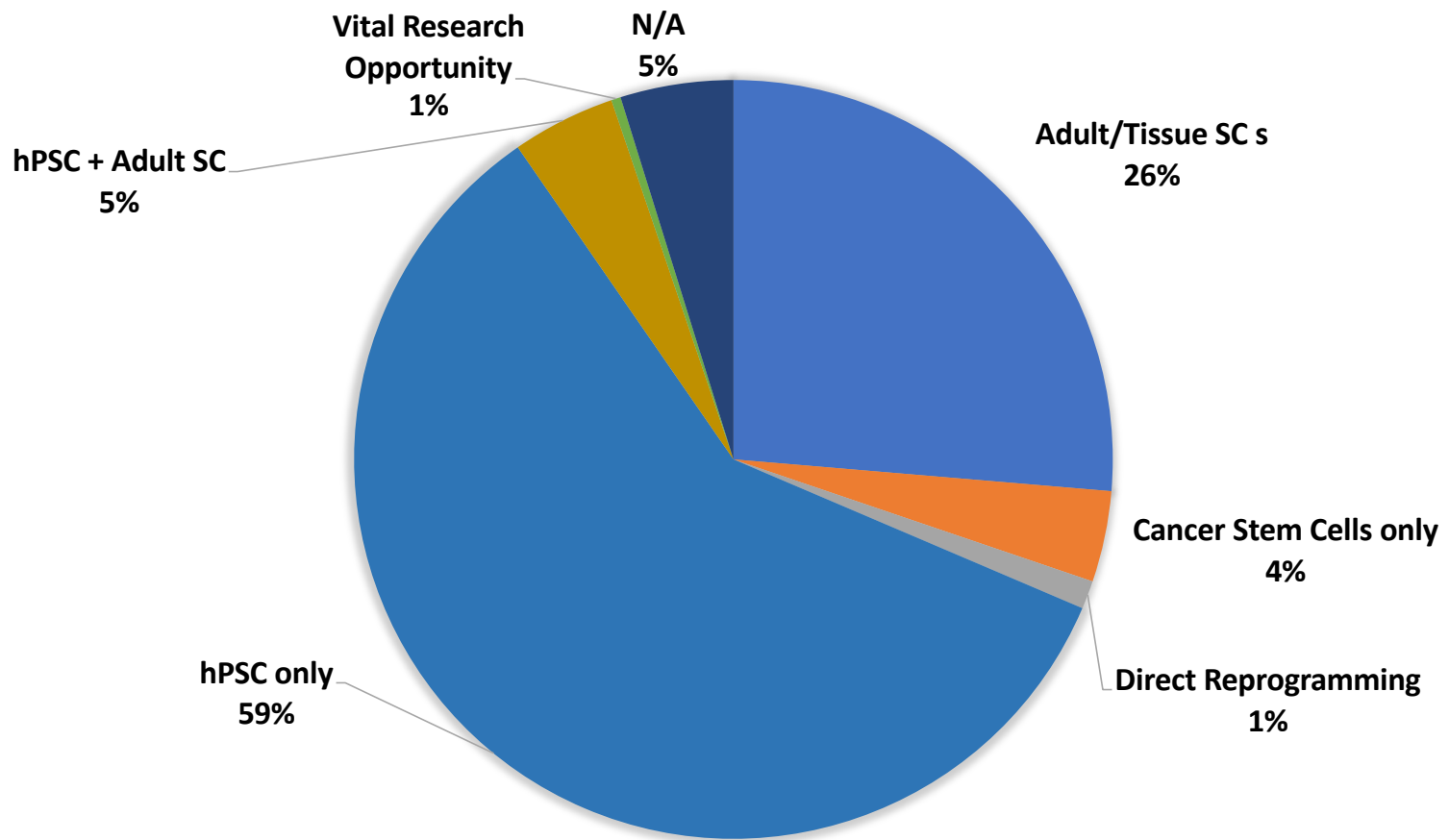
Anchoring Questions:

- What is the greatest impact that CIRM could make in the next 10 years for stem cell research
- What types of vital research opportunities are in need of funding within the field of stem cell biology, genomics, gene therapy, particularly in the neuroscience field. Are there vital research opportunities that fall outside of these categories?
- Advantages and disadvantages of consortia
- What is the largest gap in stem cell research – in basic and translational research
- What key scientific & clinical research infrastructure gaps are there in the field? *(in addition to manufacturing)*

Representatives from the CIRM scientific community and Grants Working Group will introduce a variety of topic areas- 10-minute talks followed by 15 minutes of discussion (MM will moderate). No specific project proposals will be presented.

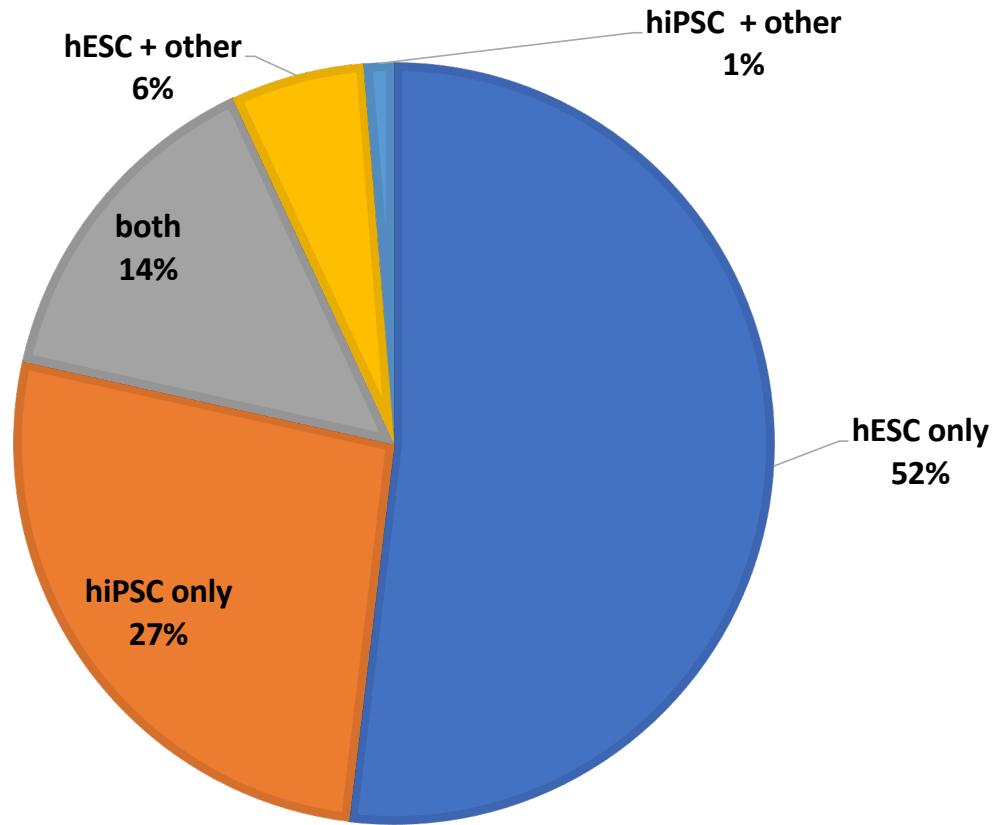
No project- or program- related feedback or funding recommendation is sought from the Panel

Neuroscience, specifically highlighted in Prop 14, will serve as an example for broader considerations in stem cell, genomics and regenerative medicine in non-neuroscience areas.



R&D Grants, 2007-2021; n = 748

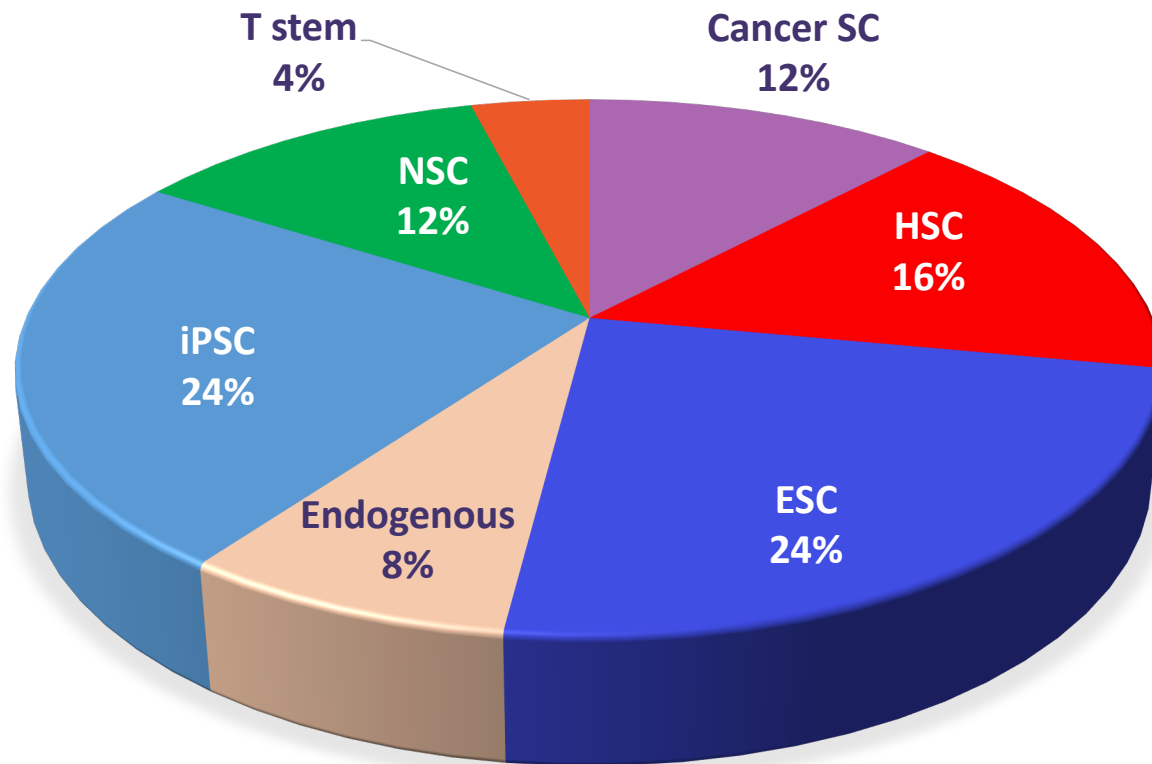
PSC BREAKDOWN



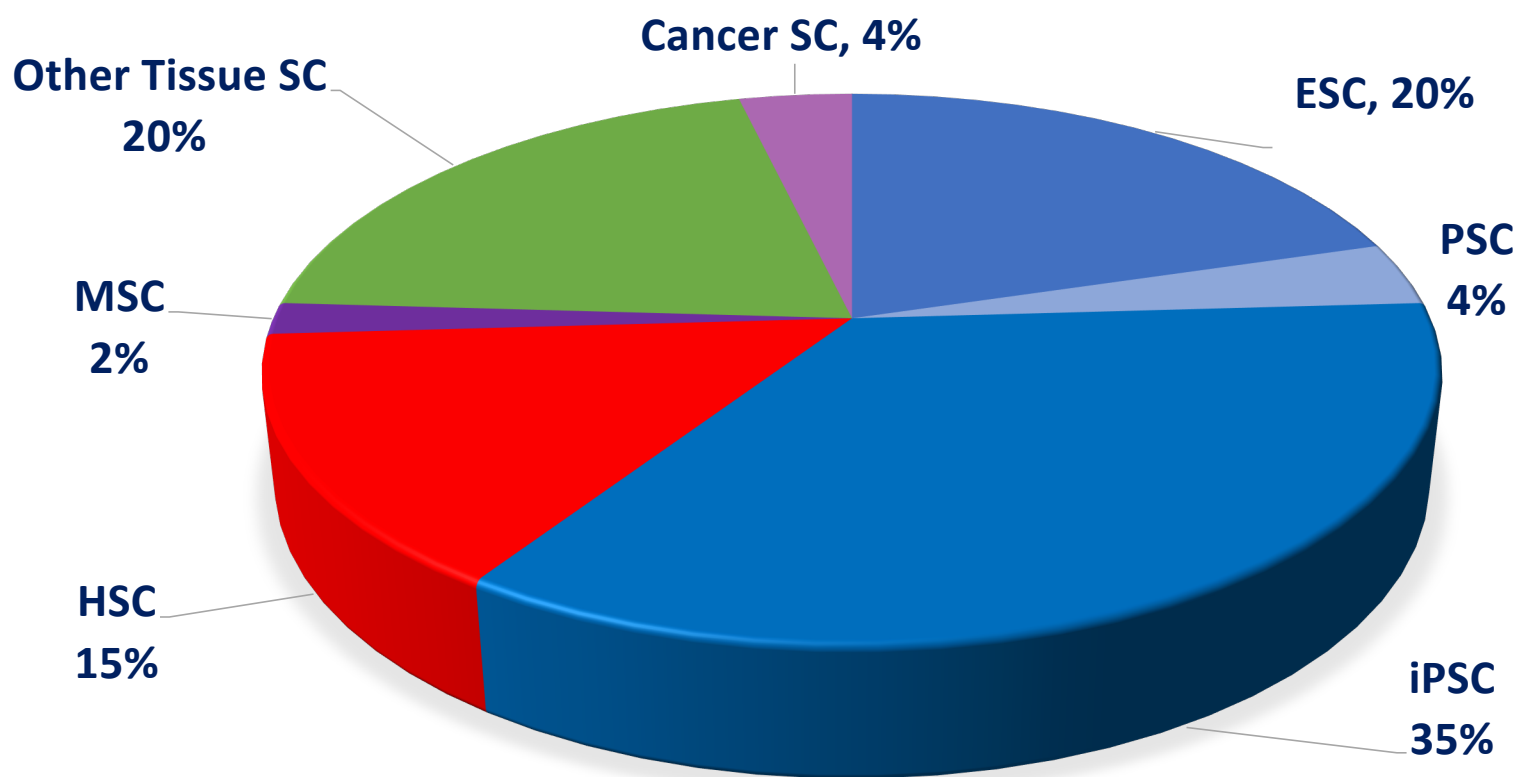
R&D Grants, 2007-2012; N = 474

CIRM's Active Translational Portfolio

Projects by Stem Cell Use or Target (n=25)

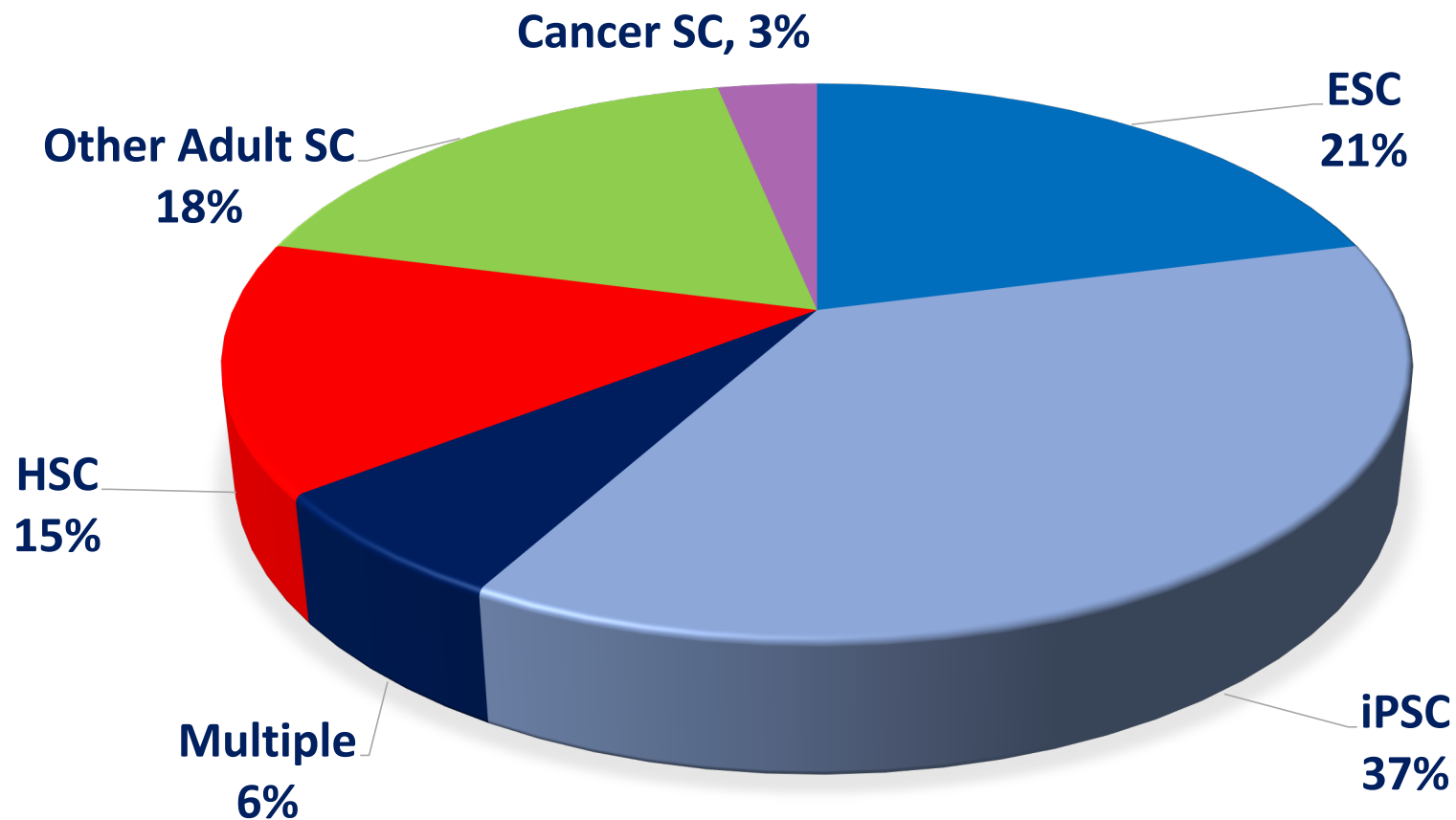


Discovery Awards: Therapeutics by SC Use or Target



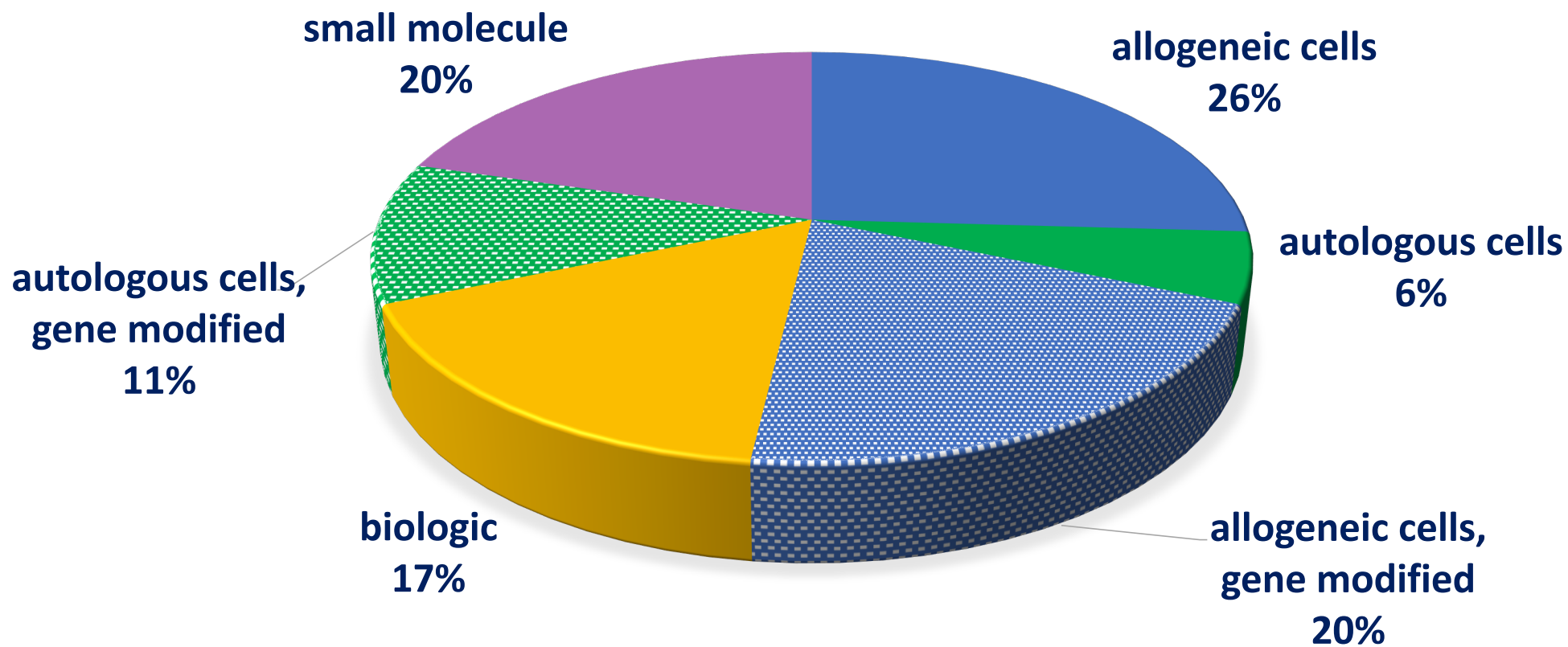
N=54

Discovery Awards: Stem Cell Use or Target



N=62

Discovery Awards: Therapeutics by Approach



N=54

CIRM's Active Translational Portfolio

Therapeutic Projects by Approach (n=24)

