

CALIFORNIA'S STEM CELL AGENCY

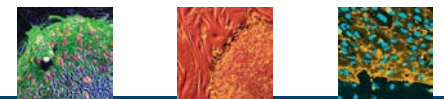
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

President's Report

C. Randal Mills, Ph.D.

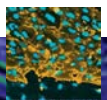
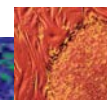
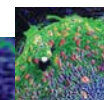
President and Chief Executive Officer
California Institute for Regenerative Medicine

October 23, 2014



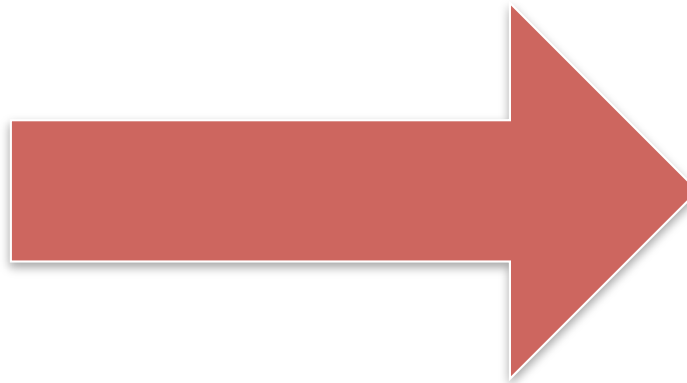
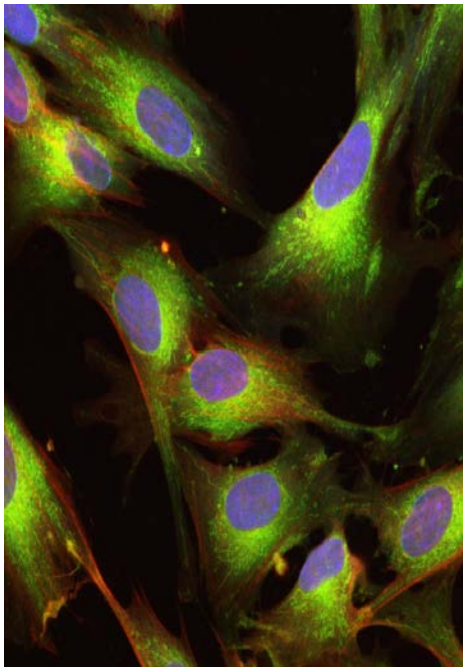
Topics

- CIRM Overview
- Budget Commentary
- **CIRM 2.0** – *Building a **better** stem cell agency*
- Scope Question for Future Awards



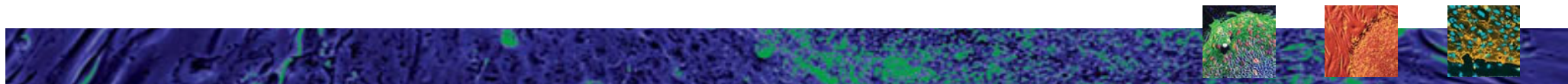
Our Mission

Accelerating stem cell treatments to patients with unmet medical needs.

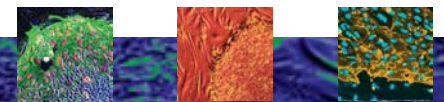
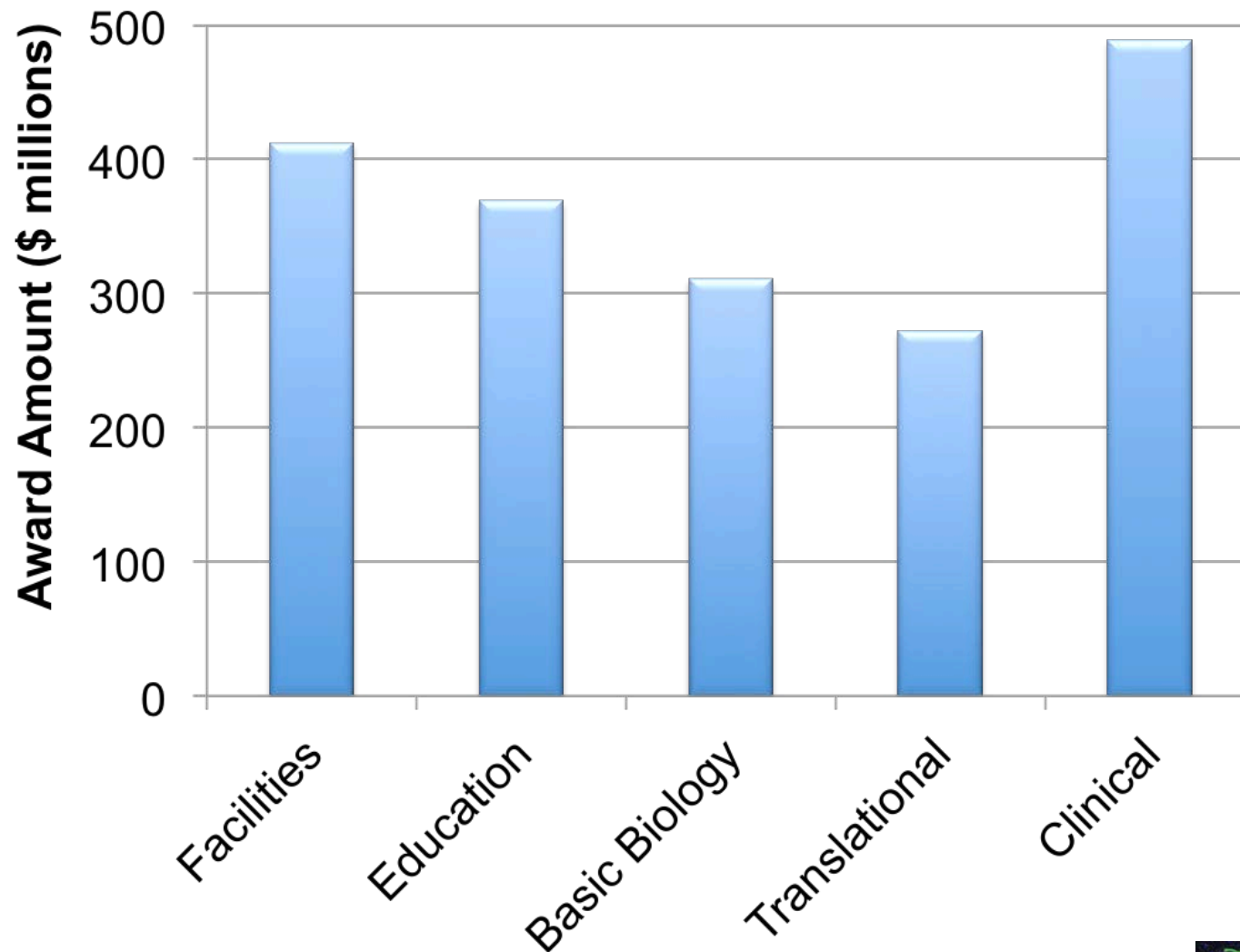


The Four Part Test

- Will it speed up the development of a stem cell treatment?
- Will it increase the likelihood of success?
- Will it fill an unmet medical need?
- Is it efficient?

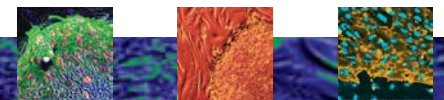
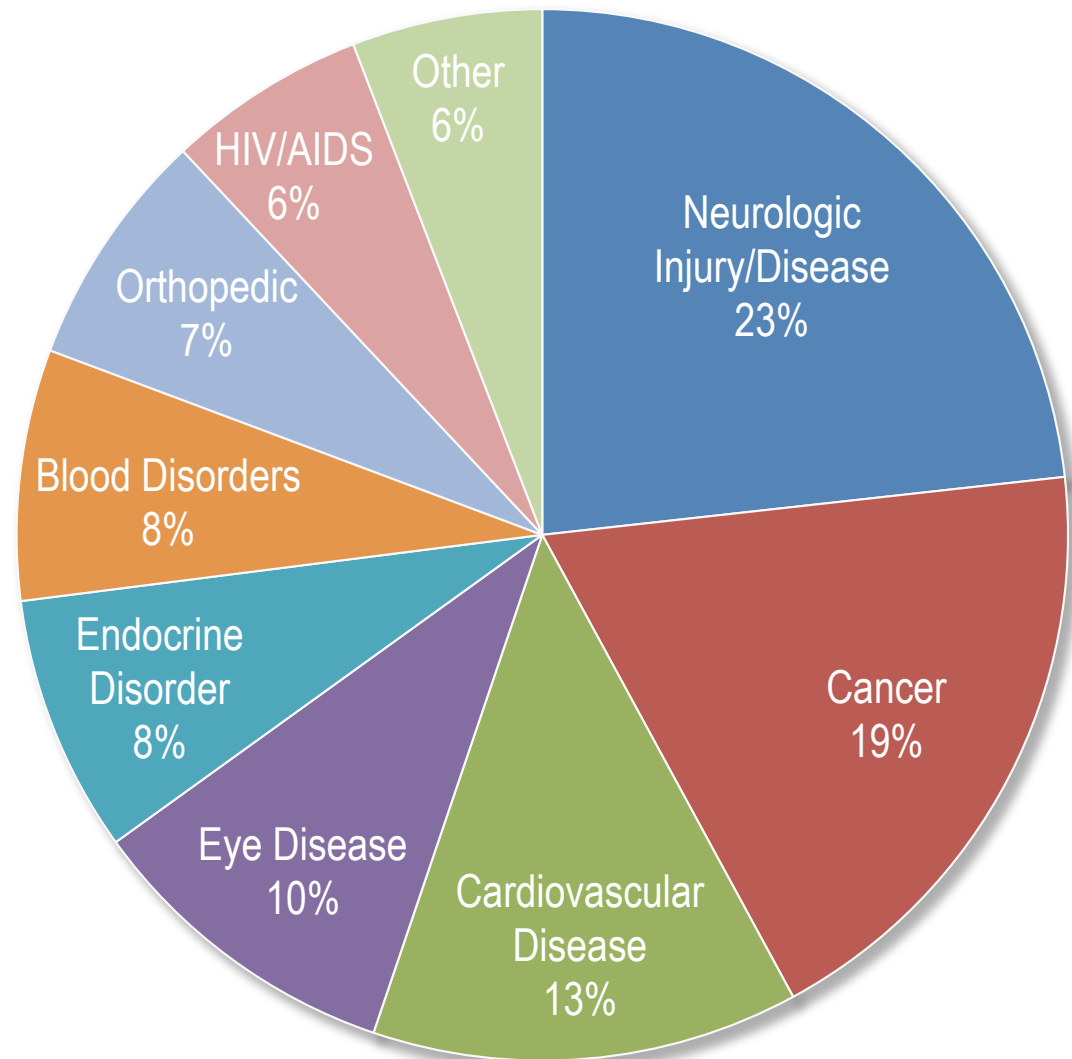


668 Awards Since Inception Totaling \$2 Billion

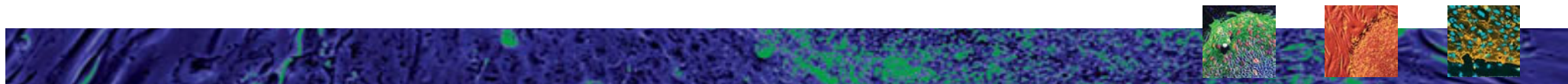
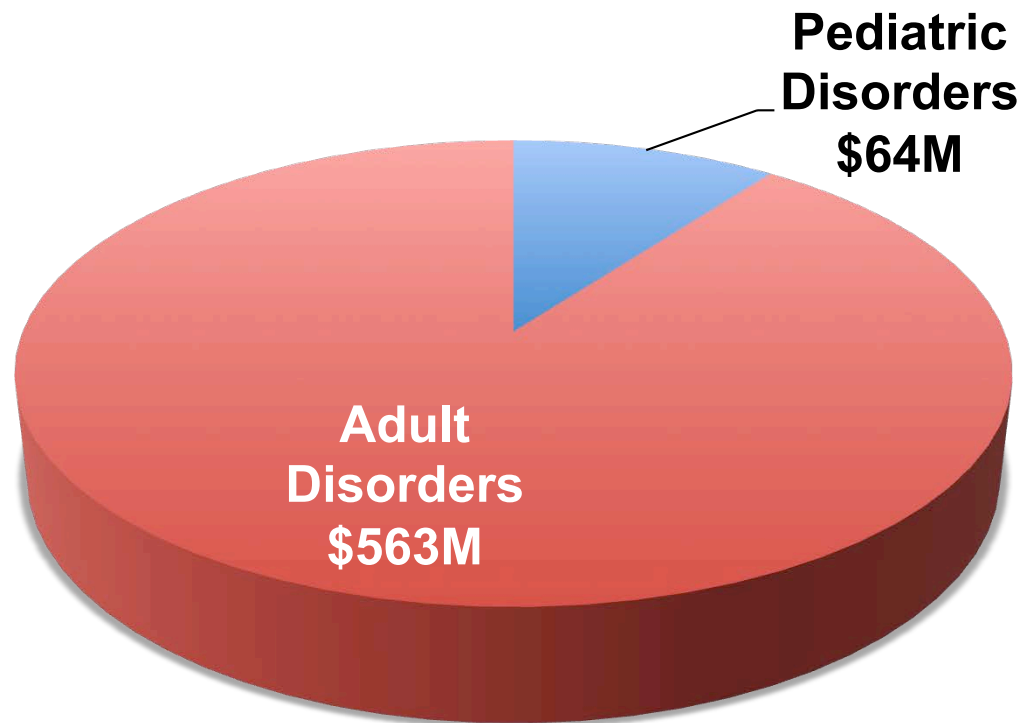


Our Translational and Clinical Programs are Targeting Treatments for 40+ Diseases

- 80 Active Programs
- \$627 million in Funding



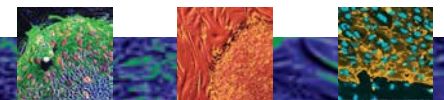
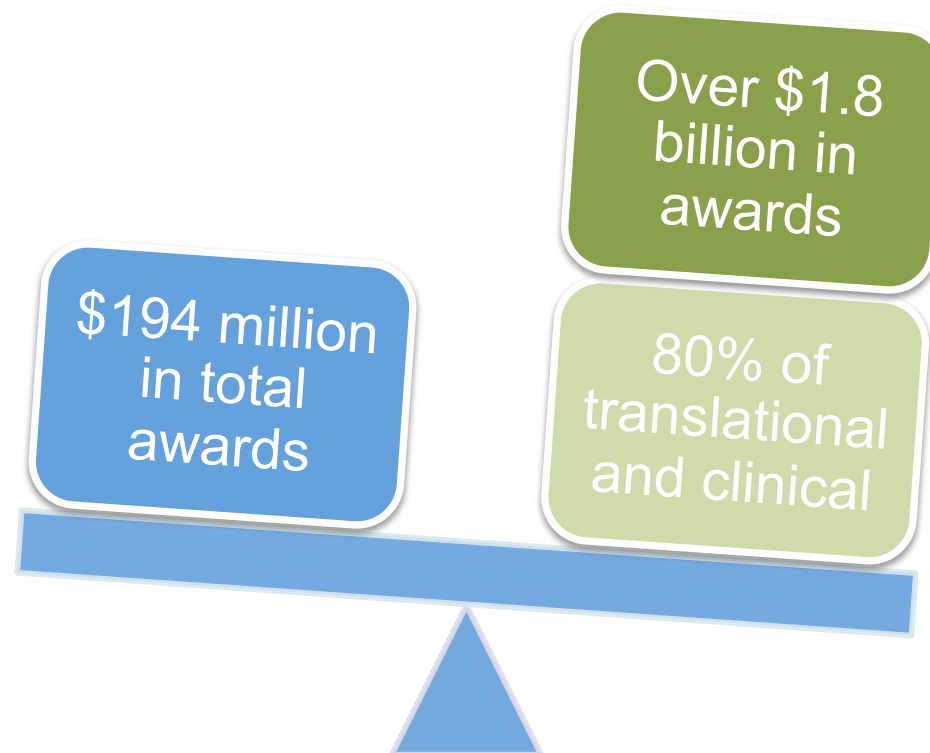
We have 14 Active Pediatric Indications in the Translational Portfolio



The Majority of our Funding has Gone to Academic Institutions

Industry

Academia



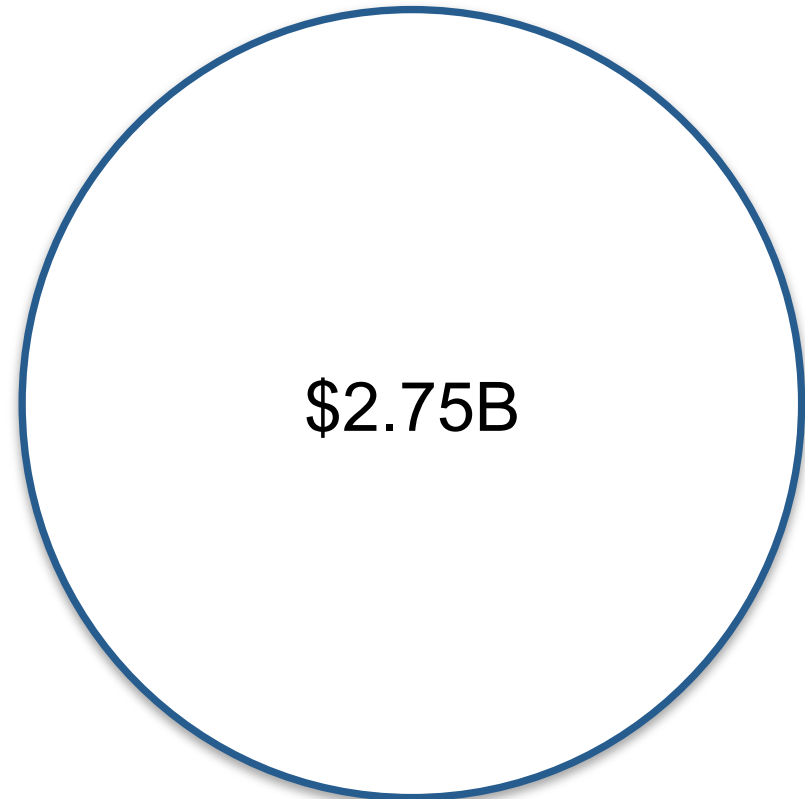
BUDGET REVIEW



Two Separate Funds



Administrative
Budget



Award Budget

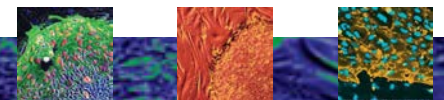


Grants Administration Budget

\$180M

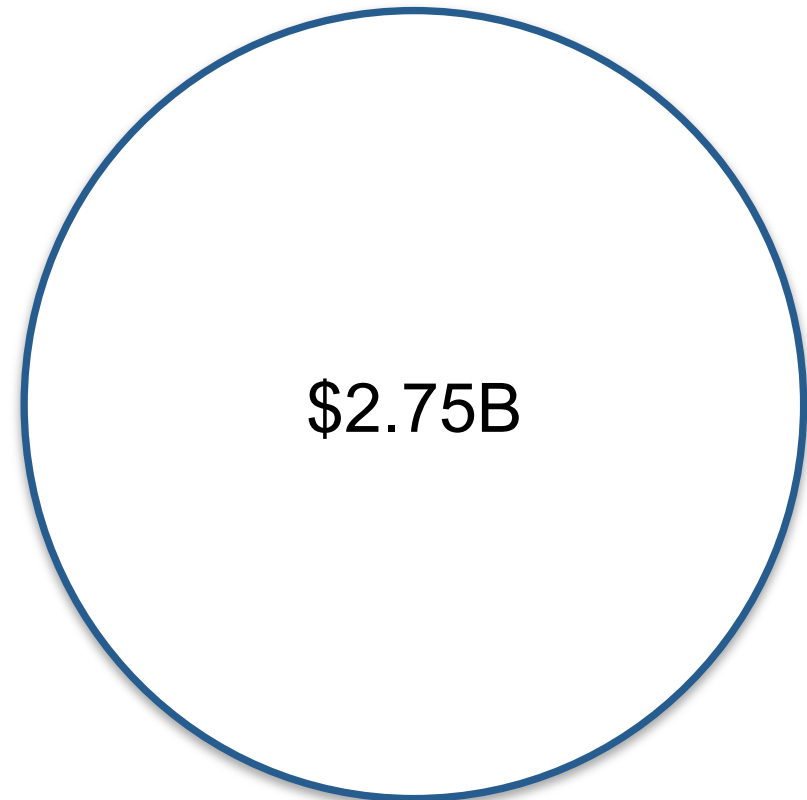
Administrative
Funding
Budget

- \$92 million spent
- \$88 million remaining
- Current spending rate is about \$13 million per year
- Funded into 2021 at current spend rate

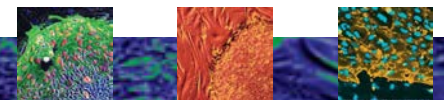


Award Budget

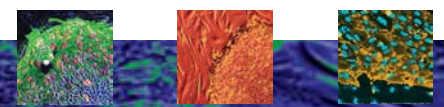
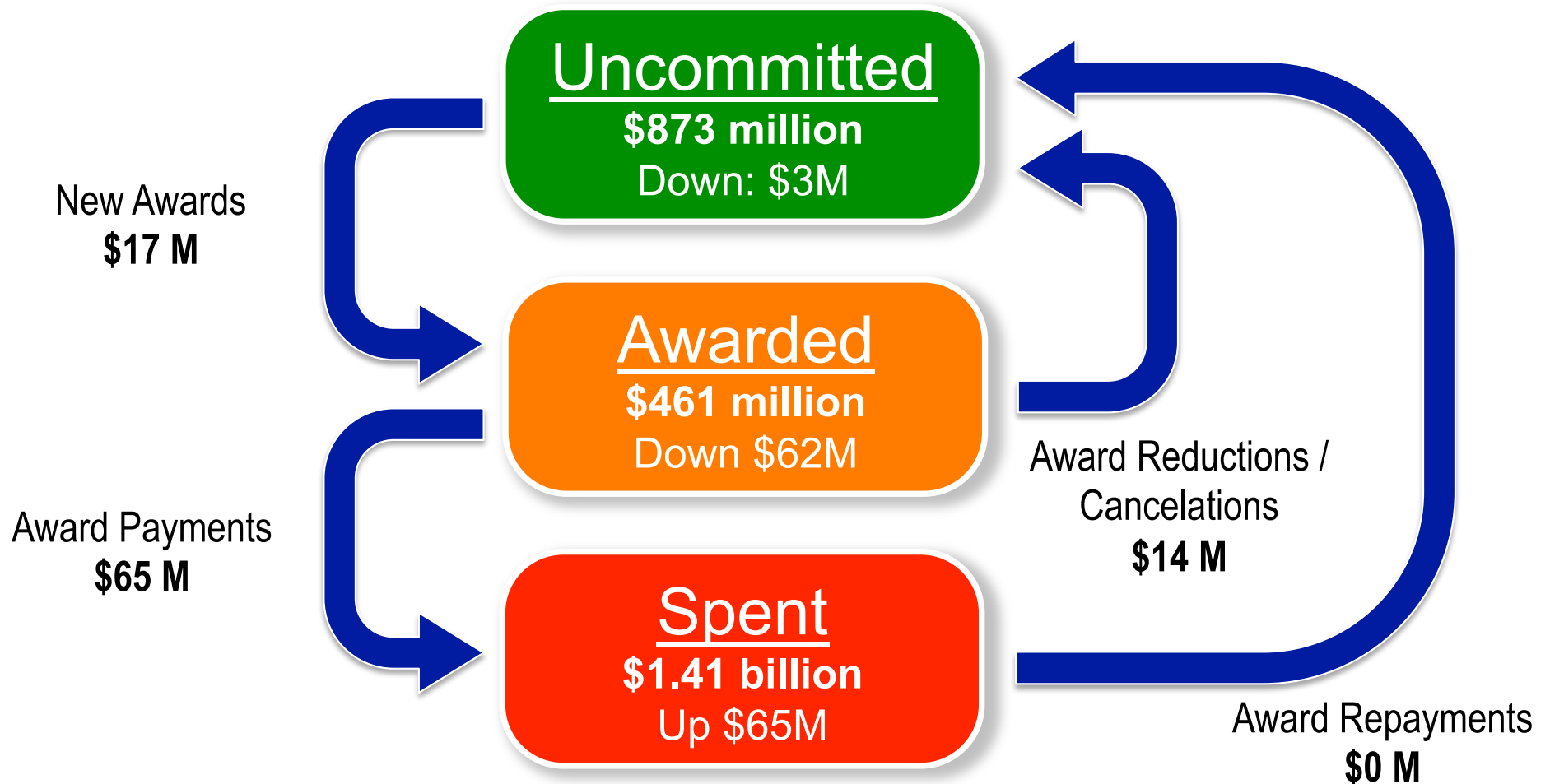
- \$1.9B net awarded (not all spent)
- Estimated \$100M will be recovered
- \$873M has **not** been awarded
- Leaves approximately \$1B available to award
- Sufficient funding at current rate until 2020



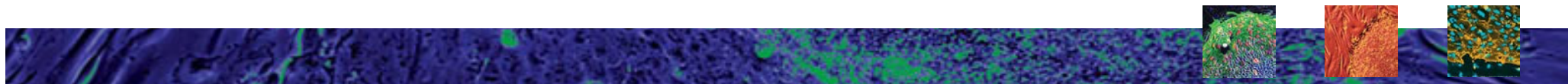
Award Budget



CIRM's \$2.75 Billion Award Budget 1Q15 Activity



*Building a **better** stem cell agency*
CIRM 2.0



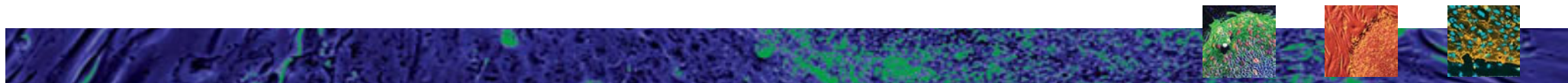
CIRM 2.0: Purpose



To create a process at CIRM for attracting, awarding and administering grants that better serves our mission

If successful, this should lead to:

- More high quality applications
- Reduced cycle time
- Accelerated progression of funded projects
- Clarity

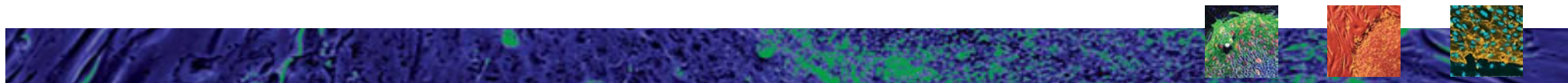


The Current Process from 30,000 ft.

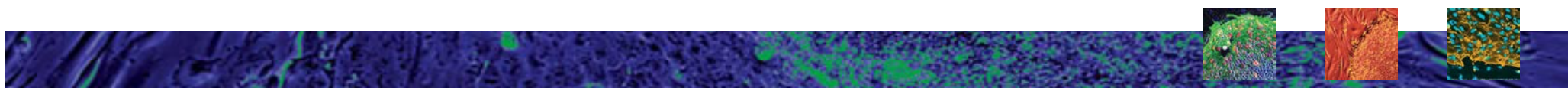
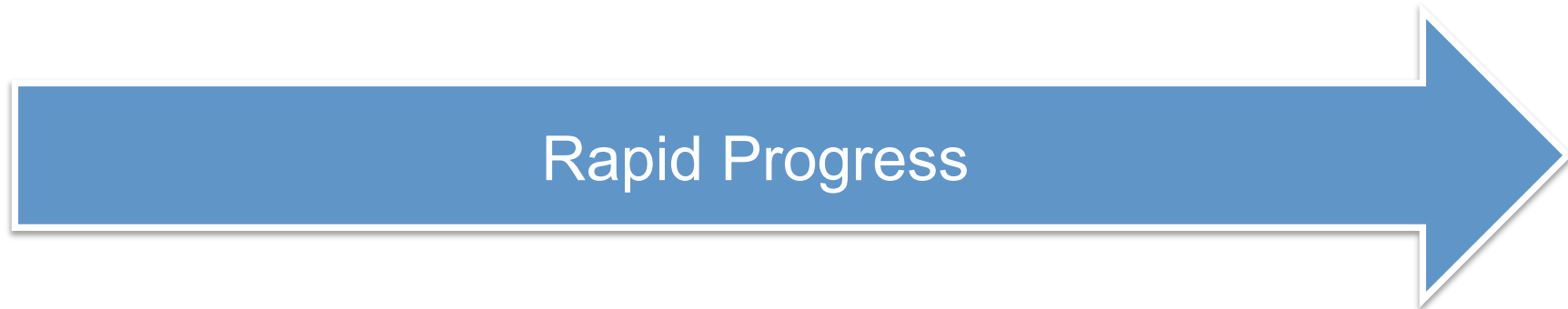


Up to 21 years

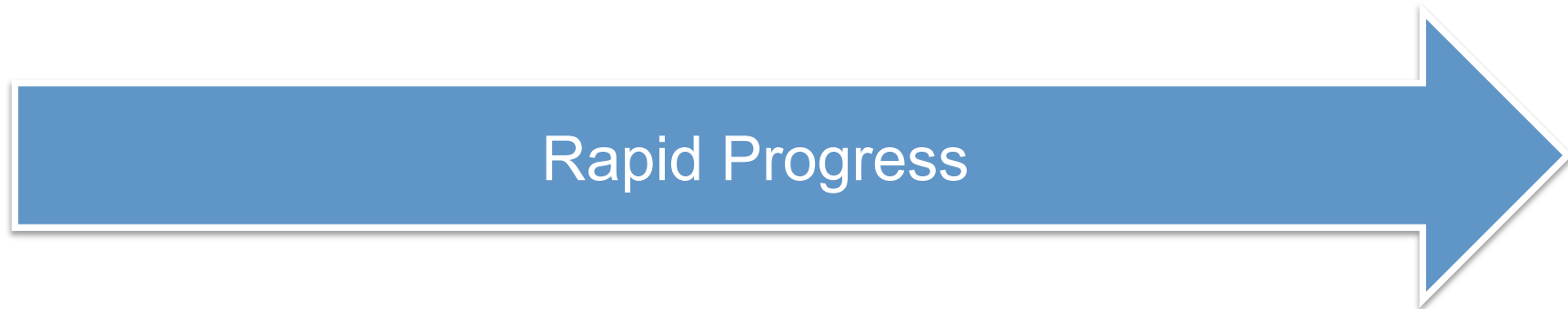
Opportunities open sporadically



What do we want out of the process?



What do we want out of the process?



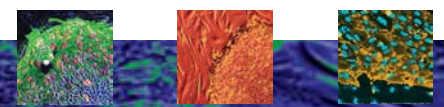
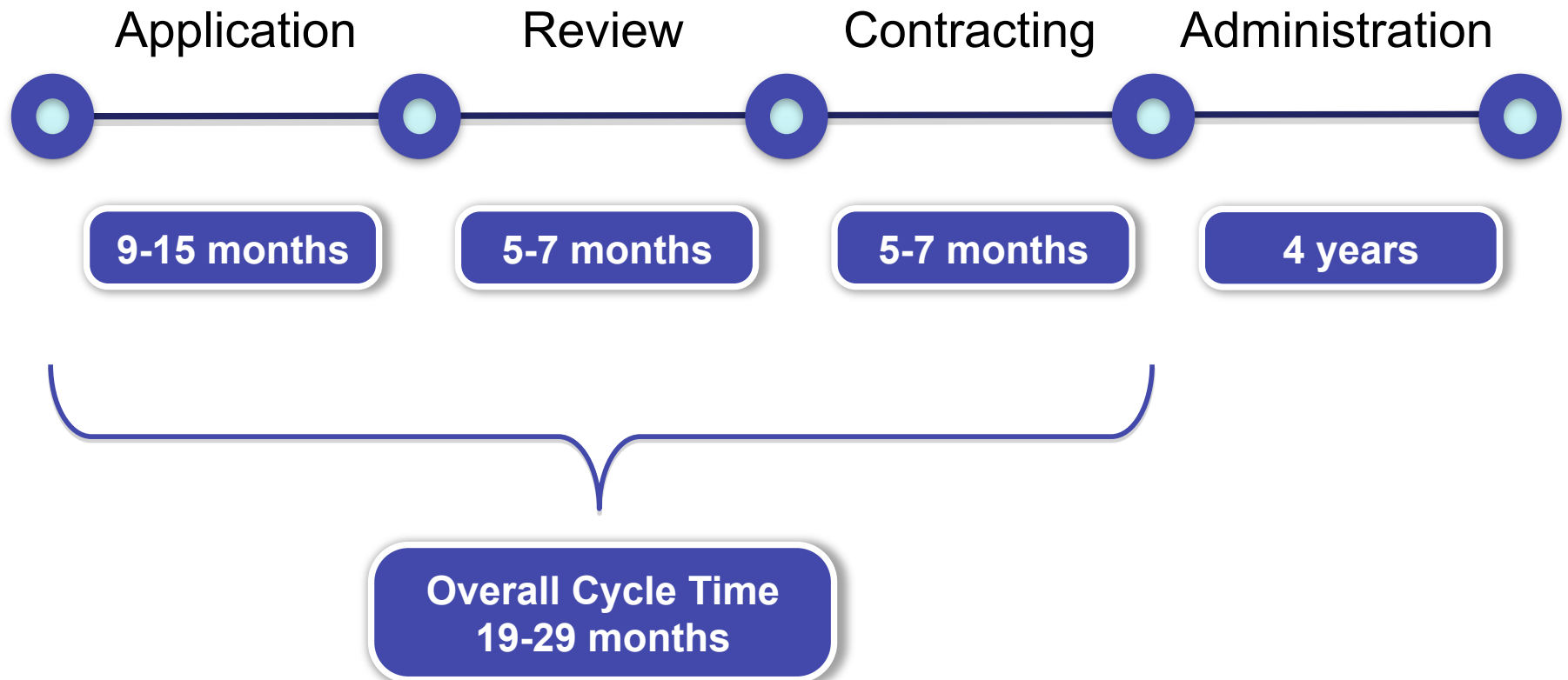
Discovery

Translation

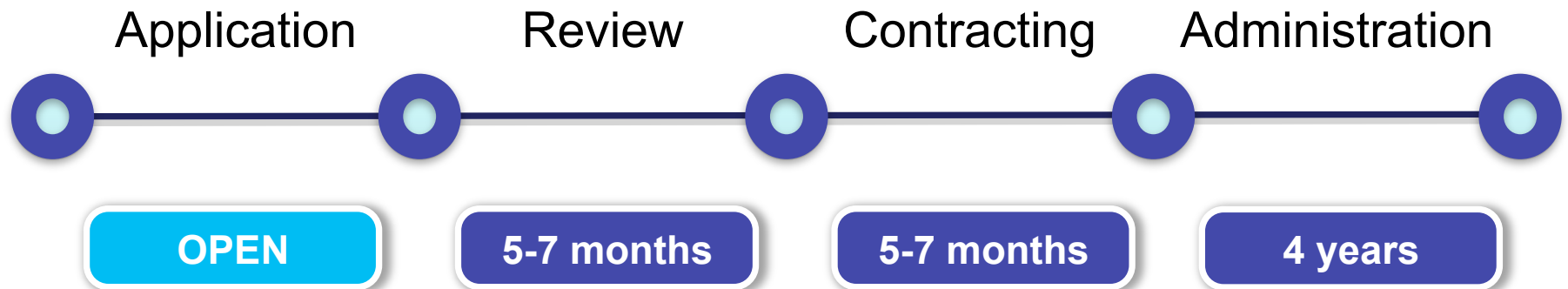
Clinical



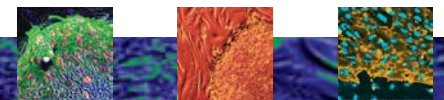
The Current Process



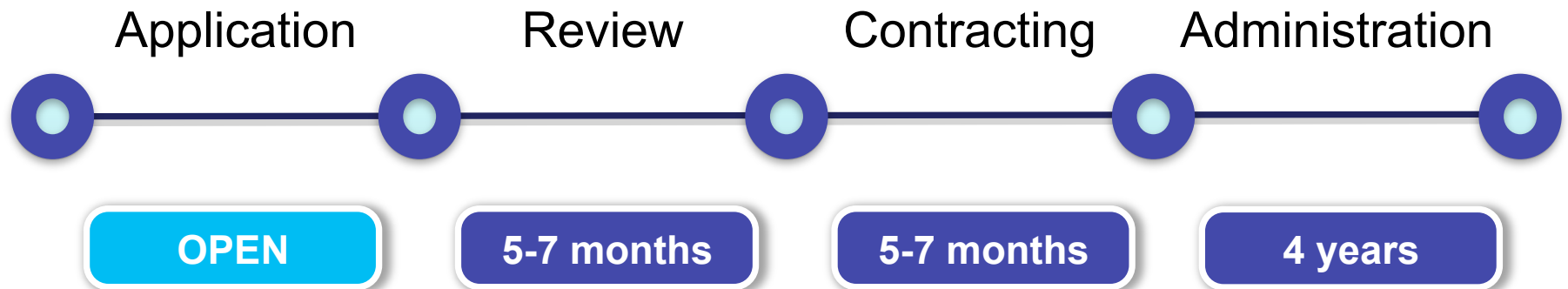
Application Process Improvement



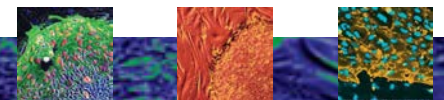
Open the RFA to rolling applications. Removes 9-15 months of delay.



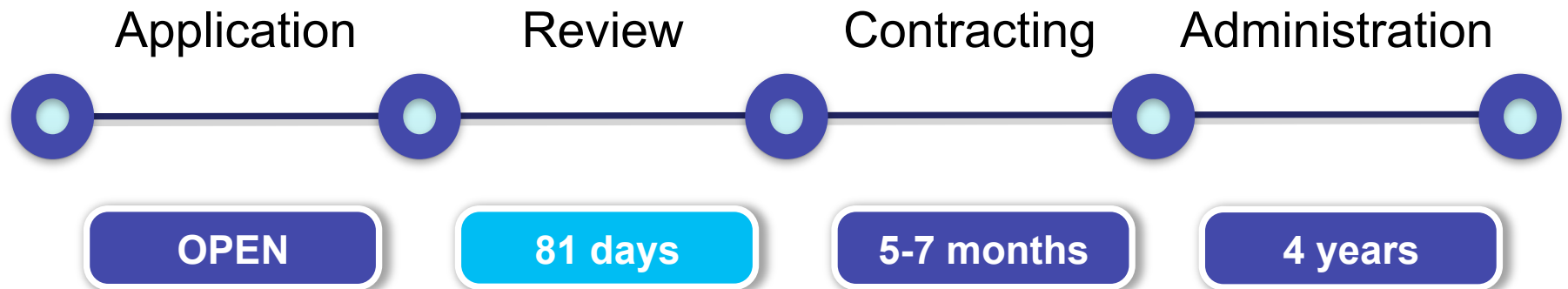
Apply When Ready



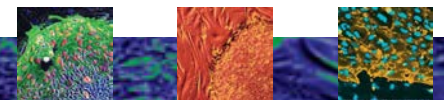
The open process also let's the applicant apply when they are in the best position to start the program



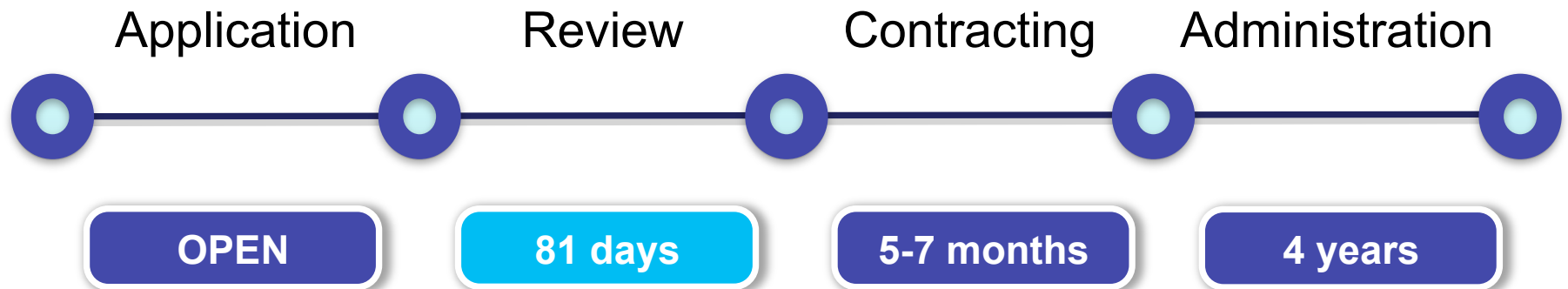
Review Process Improvement



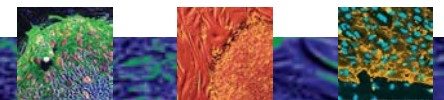
Open enrollment leads to smaller numbers of applications per review, thus quicker turnaround times.



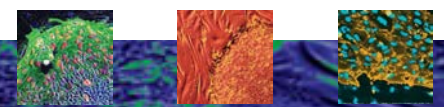
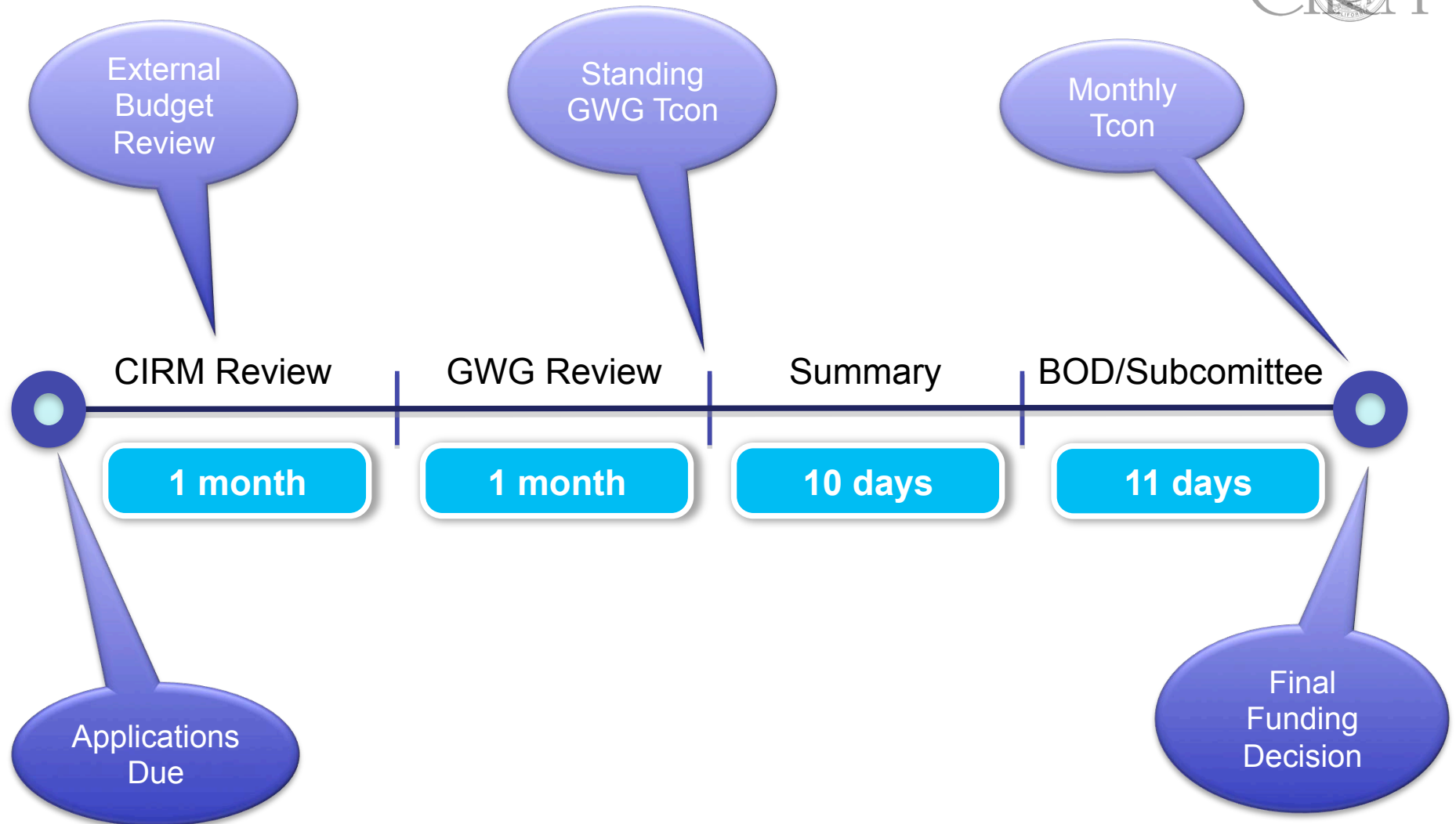
Review Process Improvement



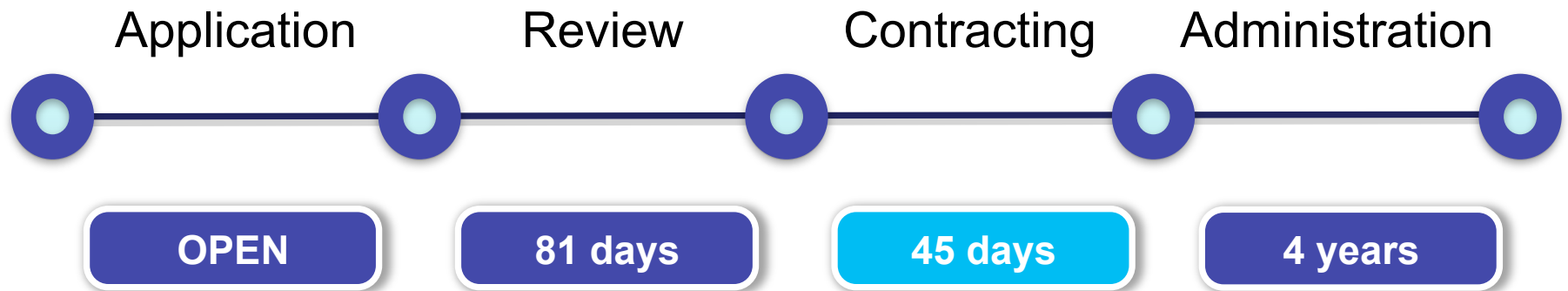
The impact of this change is huge!



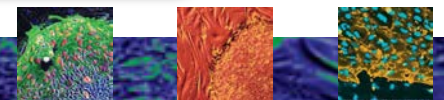
The Review Process



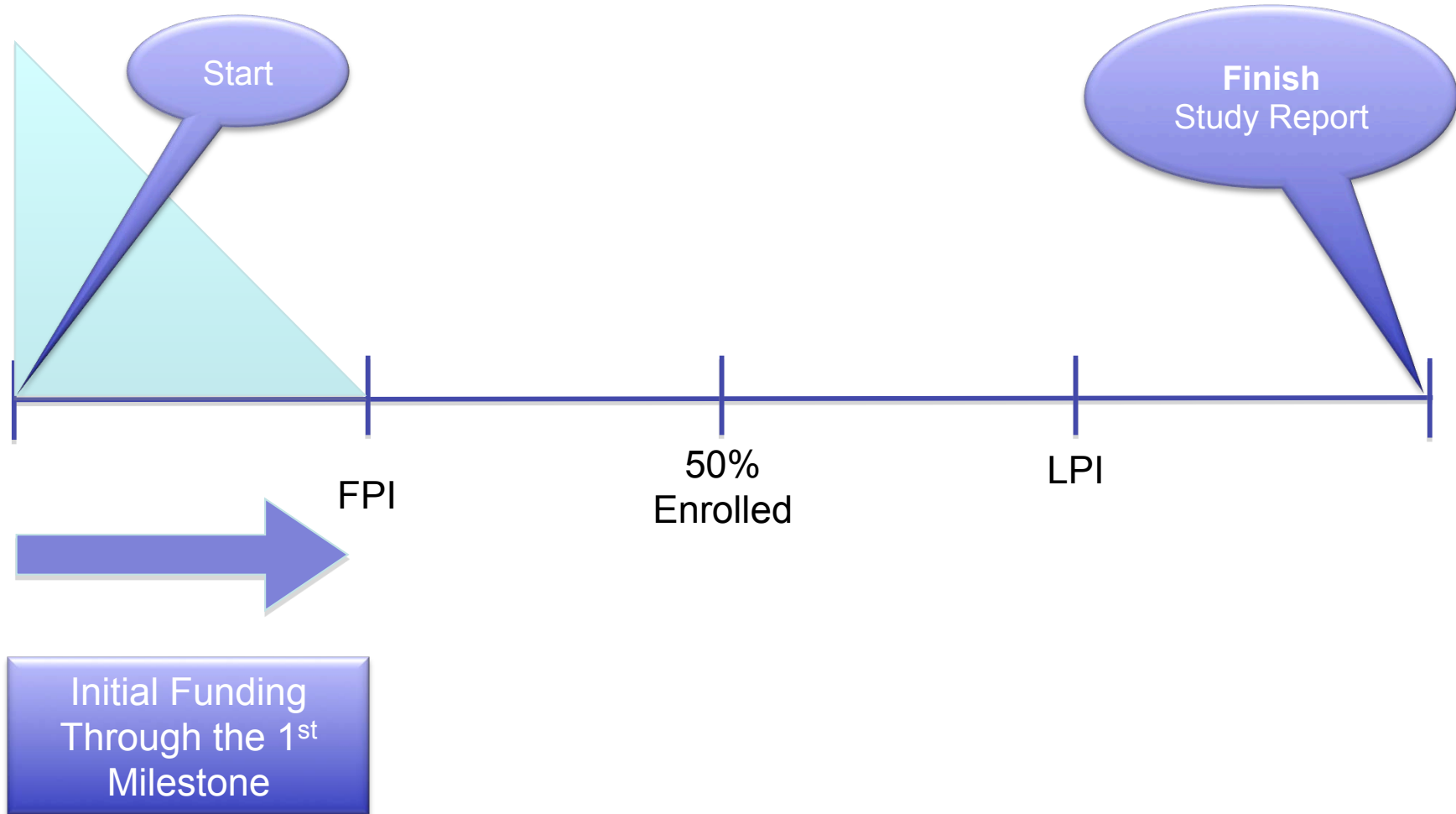
Contracting Process Improvement



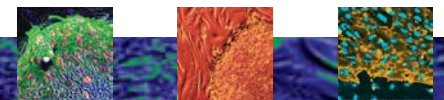
Contracting needs to be more standardized and focused on operational milestone delivery.



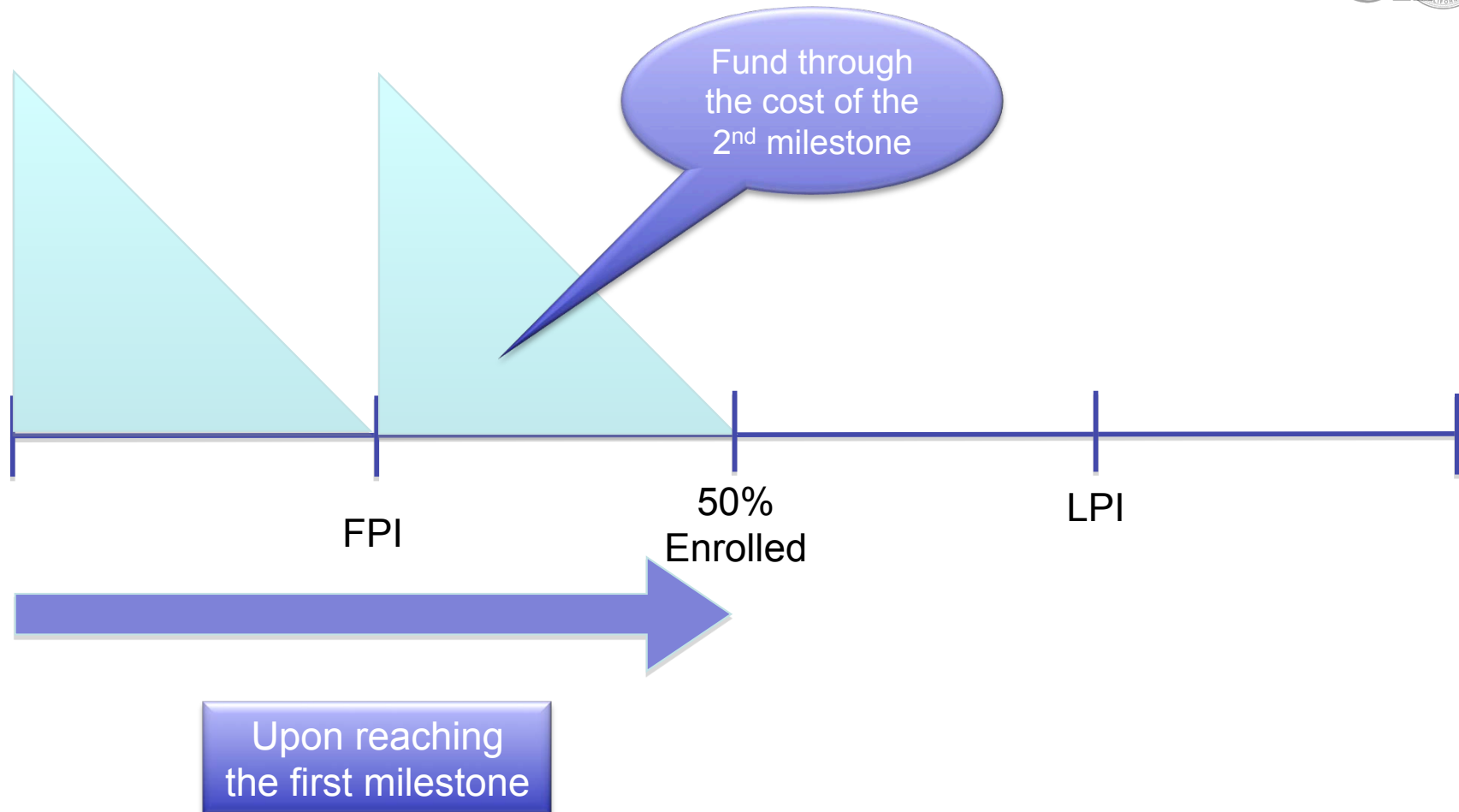
The Contracting Process



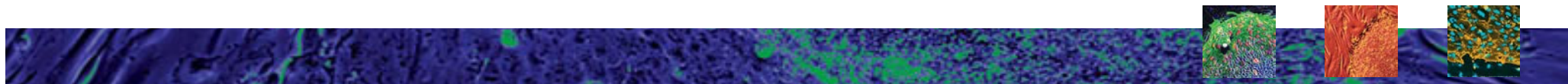
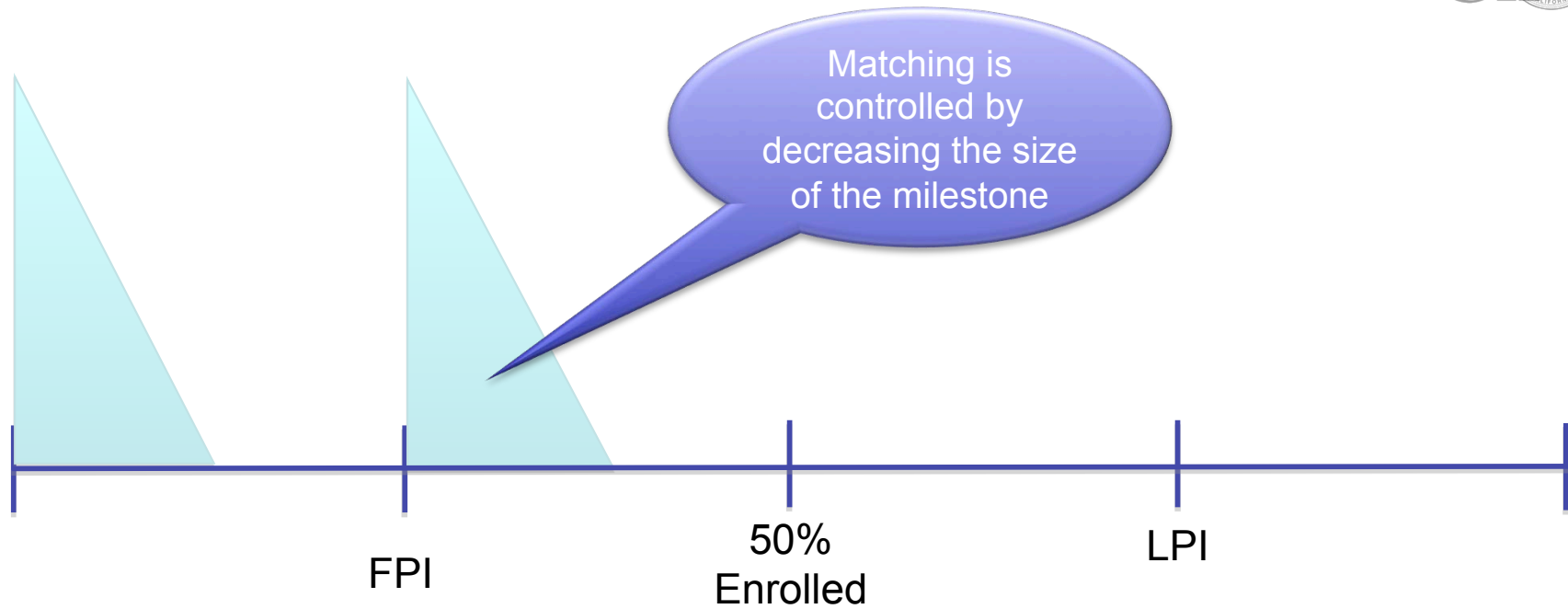
Initial Funding
Through the 1st
Milestone



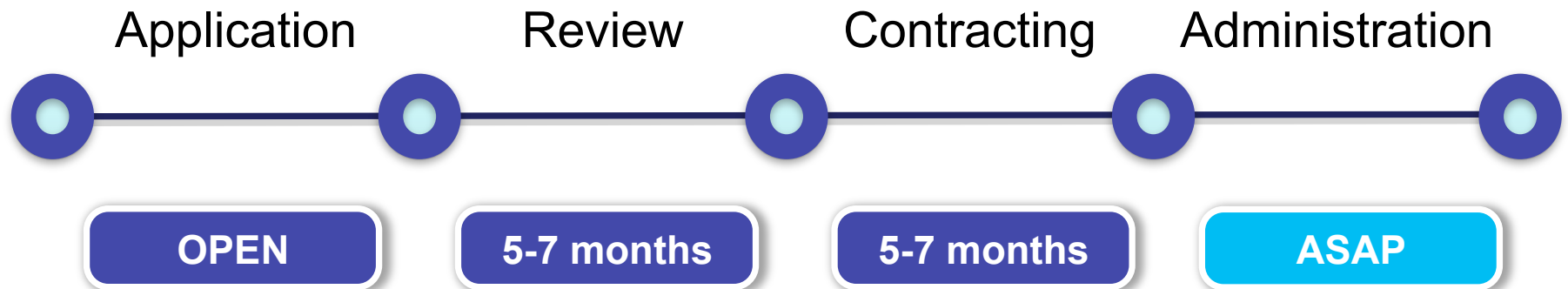
The Contracting Process



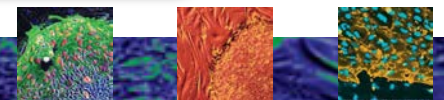
The Contracting Process



Administration Process Improvement

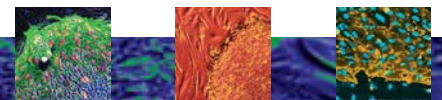


We need to eliminate the time component of the grant (fixed at 4 years) and provide more impactful help to awardees.



Advancing our programs

- How can we make CDAP work better?
- How do we combine the CDAP and Accelerated Development Pathway concepts?
 - Real-time course correction
 - All on the same team
 - Pushing more than evaluating
 - Seamless transitions



Clinical Advisory Panel

Standing “CAPs” for each project

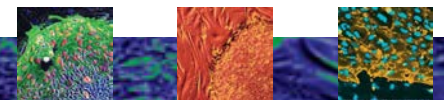
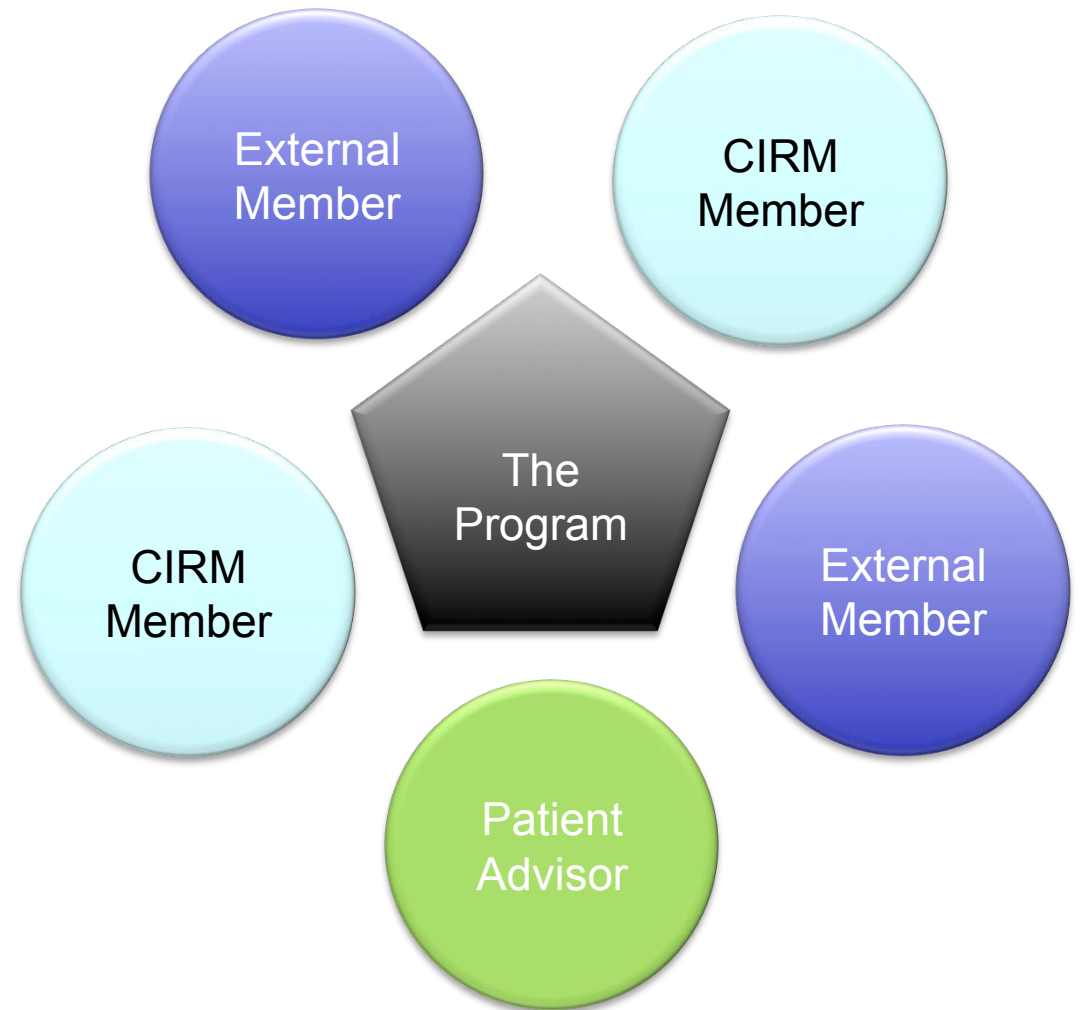
CIRM and external members

Tailored to the needs of the project

More frequent meetings

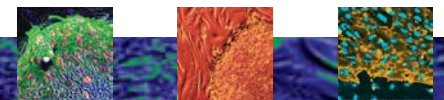
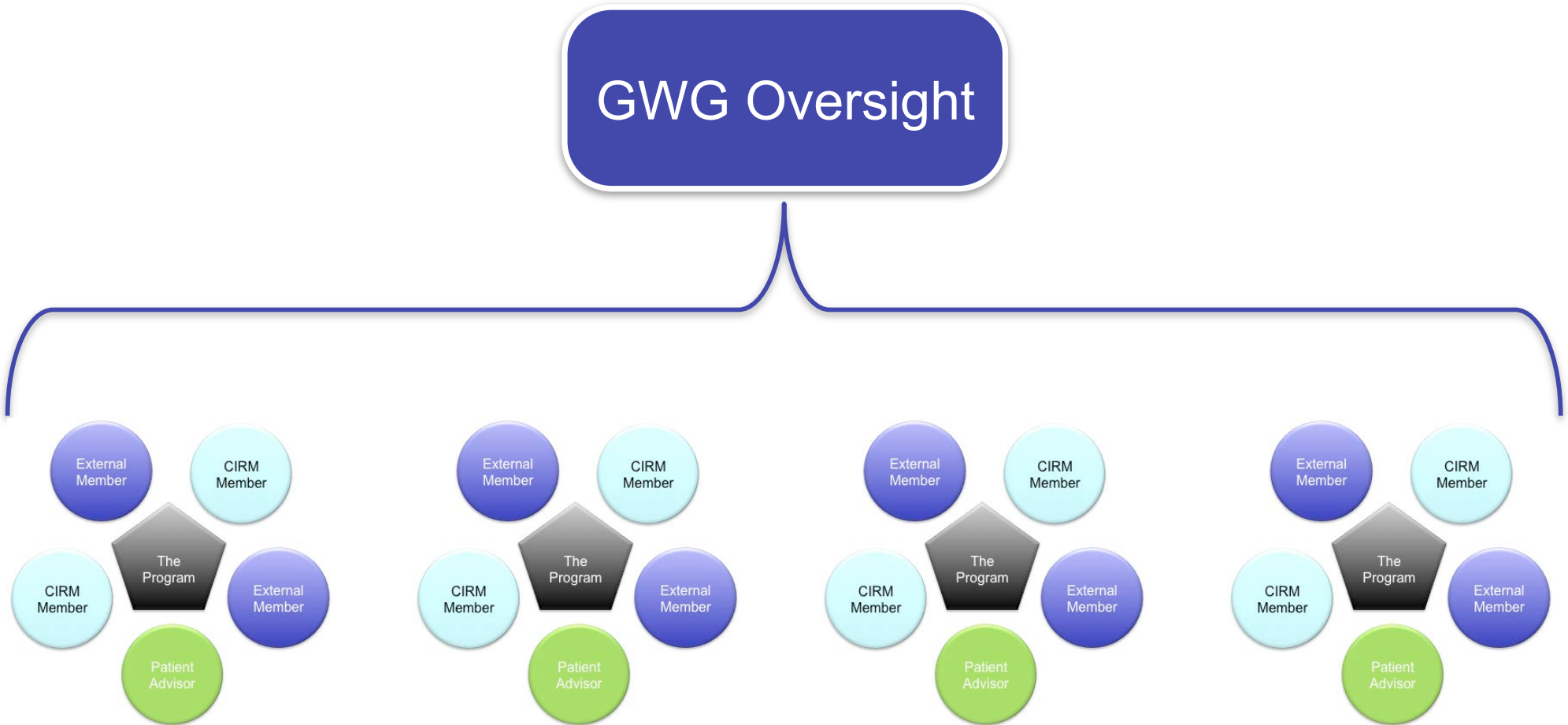
Seamless progression

Rapid future funding decisions

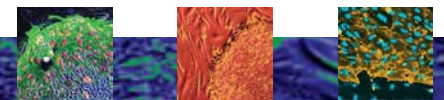
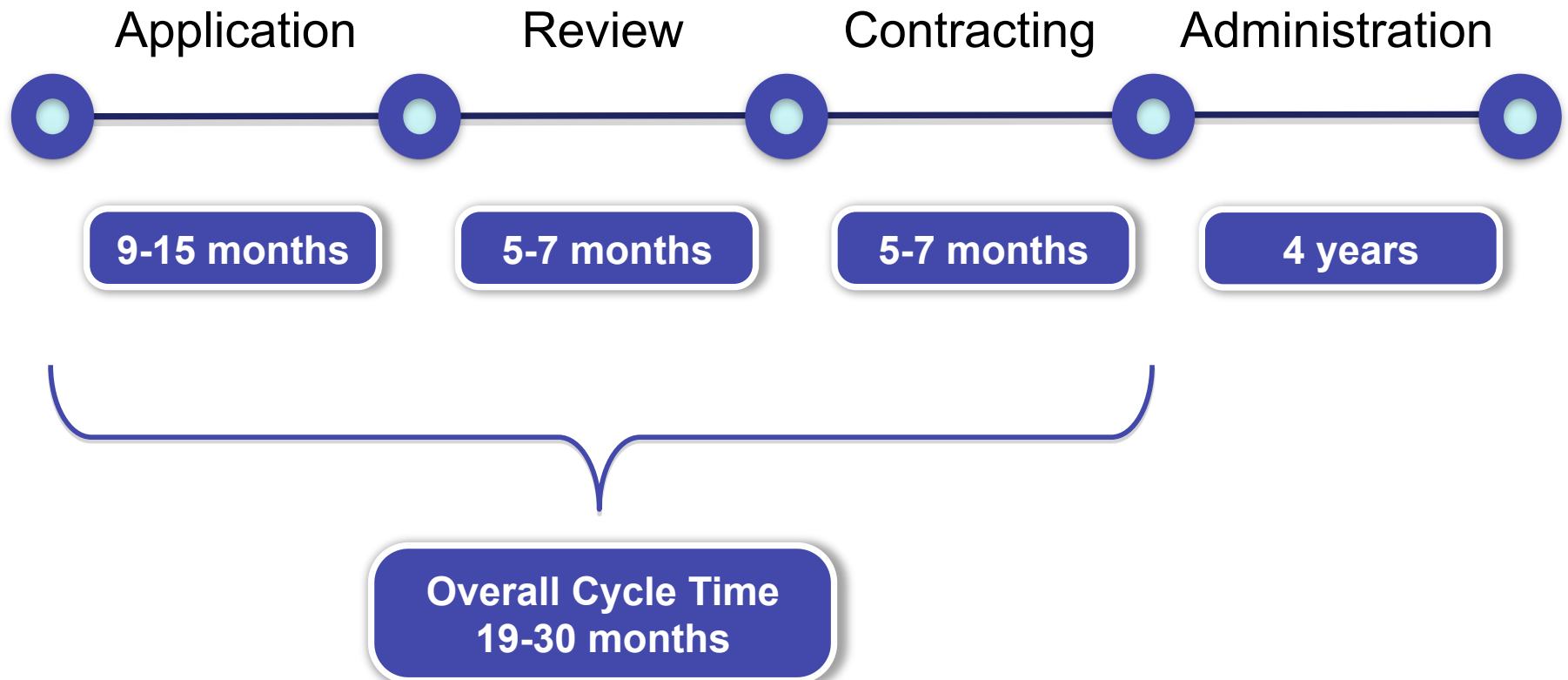


GWG Oversight of the Clinical Programs

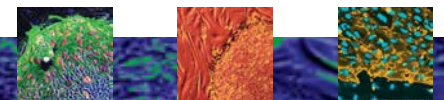
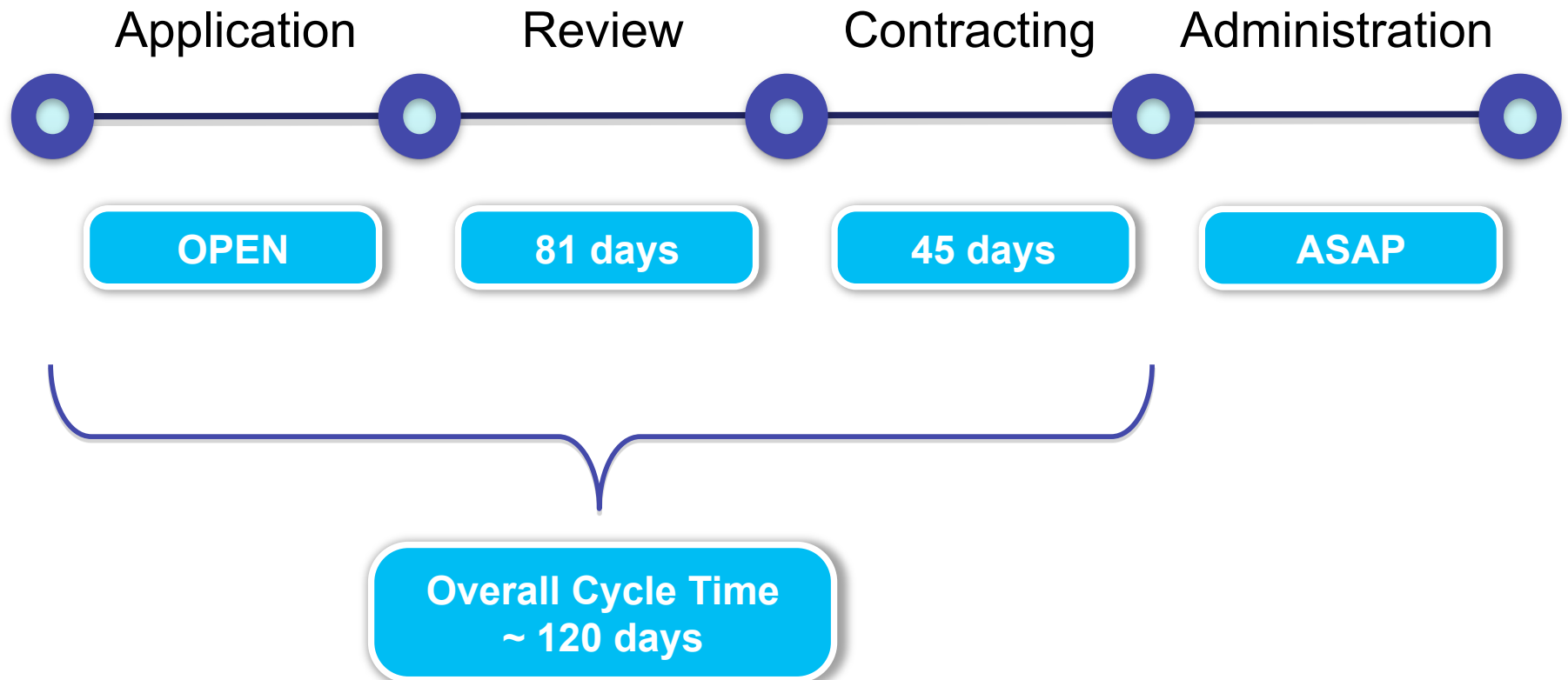
GWG Oversight



The Current Process



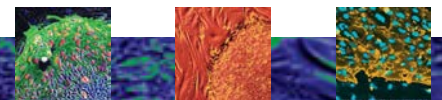
CIRM 2.0



CIRM 2.0 Rollout Plan



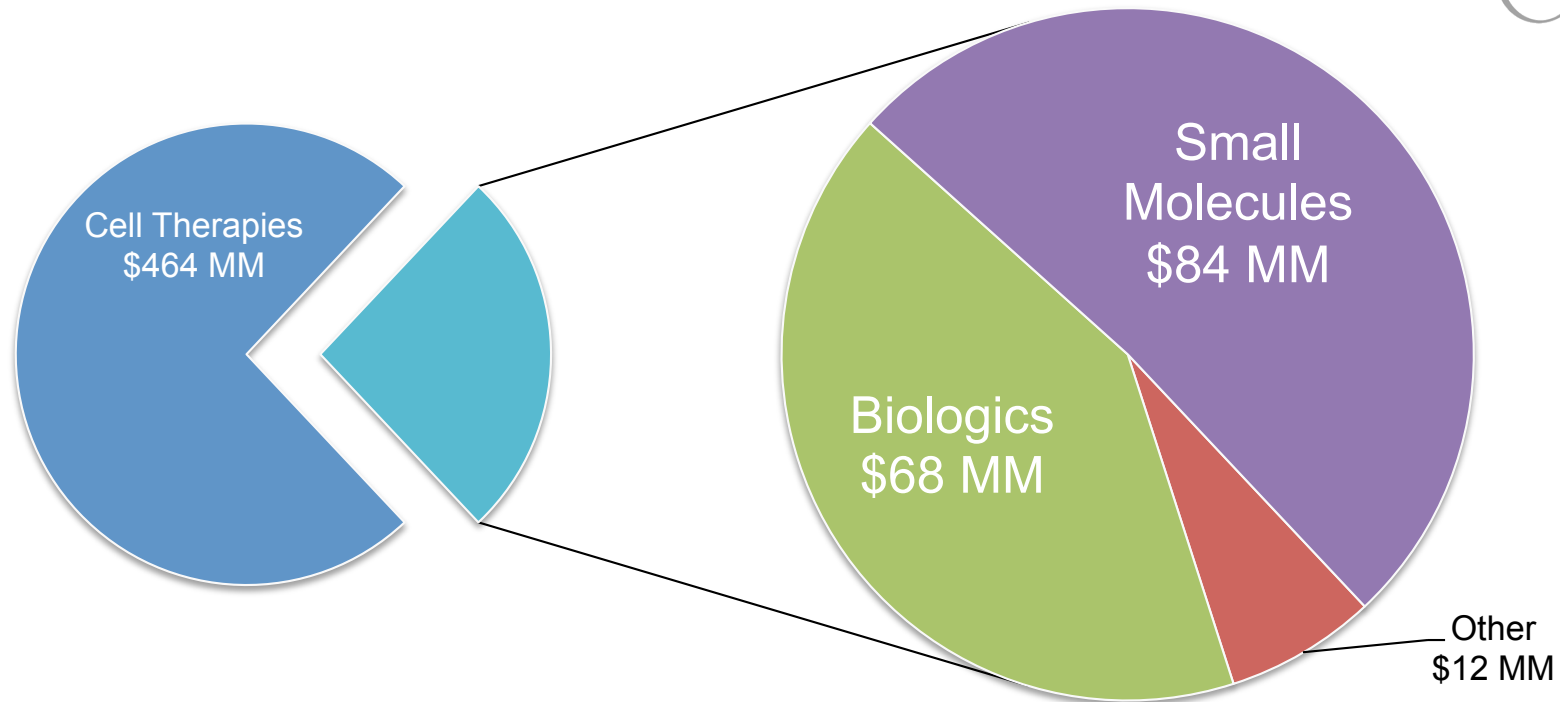
- Now – December
 - Prepare the first concept plans (Clinical)
 - Meet with the Science Subcommittee
- December Board Meeting
 - Present concept plans to the Board
- January 1st
 - Launch Clinical phase
- January Board Meeting
 - Adopt necessary policy and procedure modifications
- First Half of 2015
 - Launch Discovery and Translational modules



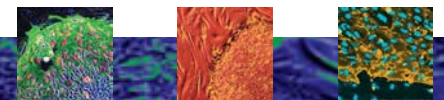
QUESTION FOR THE BOARD



Non-Cell Therapies Comprise 26% of the CIRM Portfolio

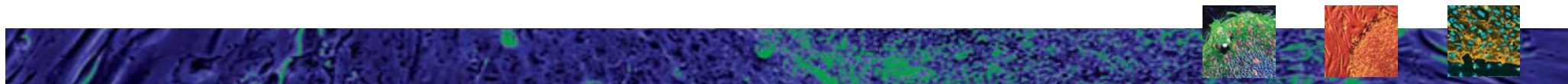


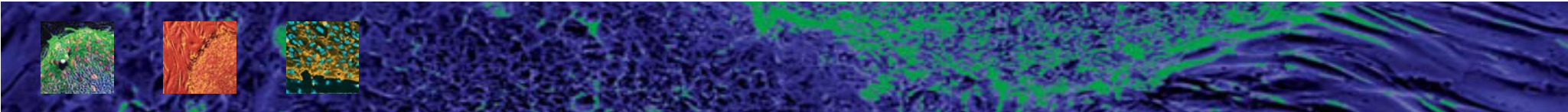
	ICOC Approved Funds (\$ MM)	% Funding	# Awards
Biologics	\$67.7	11%	6
Small Molecules	\$83.6	13%	16
Other	\$11.5	2%	2
Cell Therapies	\$463.9	74%	56
TOTALS	\$626.7		80



Question for the Board

Is there a consensus of the Board to either include or exclude non-cell therapies from participating in future funding opportunities?





CALIFORNIA'S STEM CELL AGENCY

The logo for the California Institute for Regenerative Medicine (CIIRM). It features the text "CIIRM" in a large, light grey, serif font. The letter "I" is replaced by the Great Seal of the State of California, which is a circular emblem containing a figure holding a scale and a sword, with the words "GREAT SEAL OF THE STATE OF CALIFORNIA" and "EUREKA" around the perimeter.

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Thank you!

C. Randal Mills, Ph.D.

President and Chief Executive Officer
California Institute for Regenerative Medicine