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Agenda Item #19
CIRM Clinical Advisory Panel (CAP)
ICOC update December 17, 2015

Ramona Doyle
Vice President, Therapeutics

Review of rationale for CAP Program

- **Purpose**: help accelerate successful development of therapies for patients by bringing added expertise to funded clinical projects
- **Benefits:**
  - Improves the likelihood of clinical trial success
  - Keeps CIRM aware and informed on progress, challenges and opportunities of funded clinical projects
  - Provides additional avenue for CIRM engagement with patients and disease are experts

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CAP – a strategic partnership

"We're in, we're invested"

- Conduct open, honest discussions
- Identify opportunities to improve projects
- Agree on challenges and obstacles and work together to address them
- Agree on what "success" looks like for each project

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CAP-How we Measure Success

Success is:

- Good science
- Well-conducted trial
- Good decision making
  - driven by science and data
- A clear answer
  - positive and negative results can both help drive the field forward

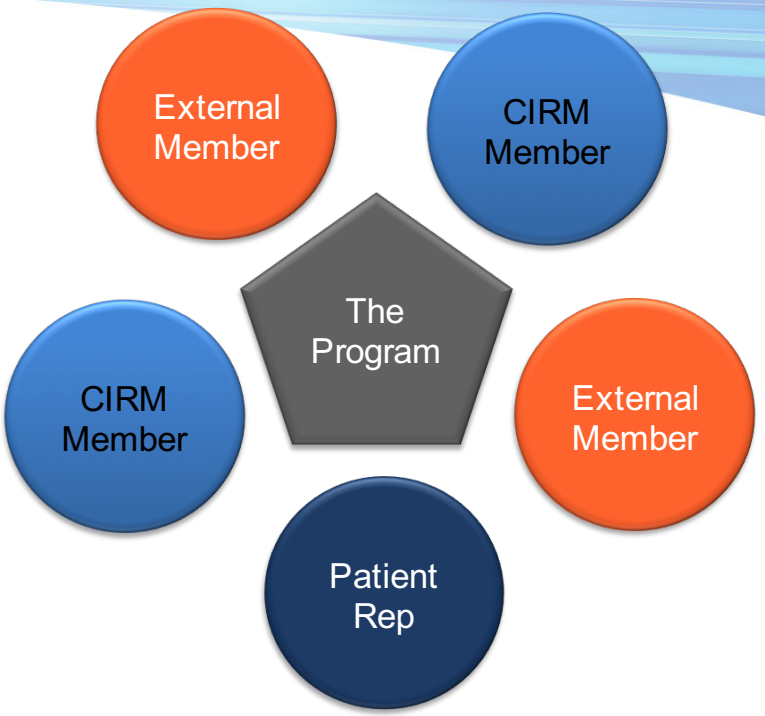
CAP Composition

Standing "CAP" for each project

Tailored to the needs of the project

At Minimum:

- 1-2 CIRM Team Members
- 1-2 External Scientific Advisors
- Patient Representative
- Ad Hoc Specialists



CAP Composition

External Member

- Disease area expert
- Scientist with expertise in CMC/manufacturing
- Clinical Trial Specialist

Ad Hoc Member

- Regulatory Specialist
- Statistician
- Toxicologist

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CAP Logistics

- CAPs meet with the teams **quarterly**
- Meetings occur ~3-4 weeks after a Progress Report is due
  - Telephonic, scheduled in advance
  - Additional ad hoc meetings if needed
  - In person meetings if needed
- The Progress Report serves as the Briefing document
  - Used to inform the CAP for a meaningful discussion

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CAP Progress Report

- **6 face-to-face CAP kickoff meetings since program start in July 2015**
  - CLIN2-08239, Dillman, Caladrius Biosciences, Inc
  - CLIN1-08235, Wang, Cedars-Sinai
  - DR3-06945, Kohn, UCLA
  - CLIN2-08231, Kohn, UCLA
  - SP3A-07536, Zaia, City of Hope
  - AP1-08039, Foyt, ViaCyte Inc. & SP1-06513, Foyt, ViaCyte Inc.

- **4 additional CAP kickoff meetings scheduled for Q12016**
  - CLIN2- 08280 Gringeri, ImmunoCellular Therapeutics
  - SP3A-07552 Lebkowski, Asterias Biotherapeutics
  - CLIN2-08289, Abedi, UC Davis
  - DR3-07438, Humayun, USC

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CAP Progress Report – Grantee feedback

- “This was a very productive meeting and I sense the desire of the CAP to be of considerable assistance. We look forward to continued interaction.” – Grantee

- “ We greatly appreciated the experience and engagement of, and interaction with, the entire team. I know that the discussion has already stimulated some specific activities to further the goals of the project. We are looking forward to your recommendations and our next interaction.” – Grantee

CAP Progress Report – Patient Representative feedback

- Don't create false hope during the consenting process
- Consider the patients so to not exhaust them with study procedures
- Do collect data from the people that decide to not enroll in the study

  —Ask them w*hy? Ask what are their fears…Ask how you could make it easier for them to enroll.*

Future opportunities for CAP

- Expand pool of expert advisors
- Enhance engagement with patient communities
- Improve value of interaction for grantees
- Educate stakeholders in best practices for clinical trials in stem cell therapies

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