

CIRM Grant Processes

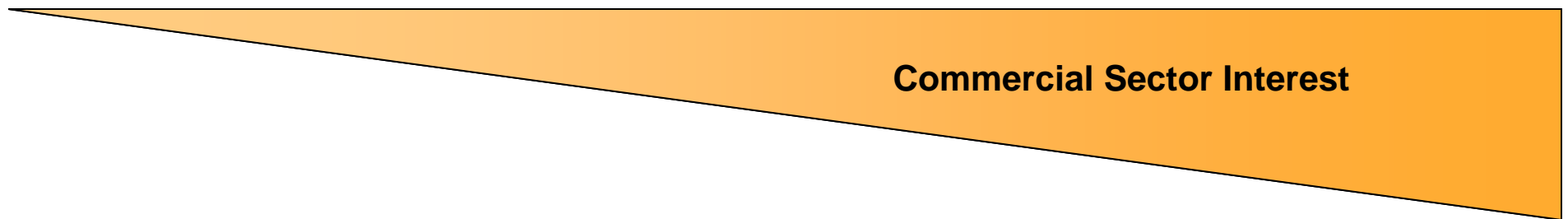
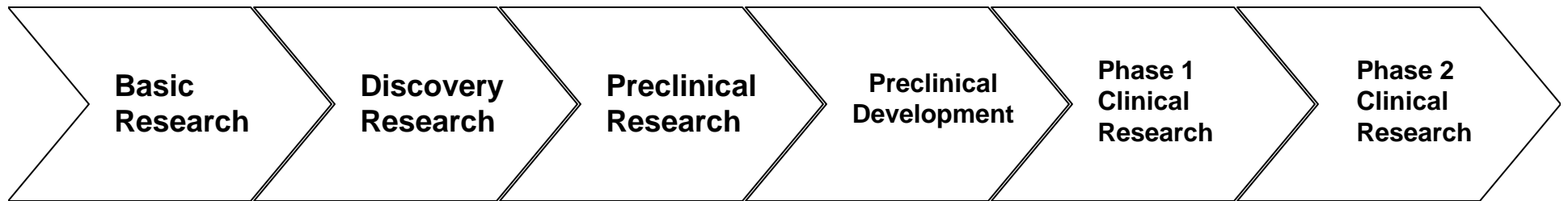
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Moving Discoveries into the Clinic: Engagement of the Commercial Sector

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

“Valley of Death”



Grants (and Loans)



Facilitating Commercial Sector Participation in Awards: What CIRM Has Done/Is Doing

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- Science Office Industry Experience
 - Over 40 years in aggregate
 - Amgen, Chiron, Geron, Cerus
- Recruiting reviewers in industry and with drug development experience
- Modifying application forms to emphasize relevant experience and success whether industry or academia
- Review criteria and instructions to reviewers (when applicable)
- Co-PIs
- Co-funders



Collaborative Funding Models: Genome Canada and Victoria

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- MOU
 - To “explore opportunities for collaborative evaluation, funding and monitoring of applications for stem cell research”
 - Subject to legal and policy framework (tricky)
 - CIRM funds stay in California

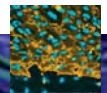
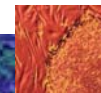
LEADING TO:

- Involvement in CIRM Disease Team Awards
- California-Canada teams: Cancer Stem Cells
- Joint RFA mechanism?
- State of Victoria collaboration with Early Translation



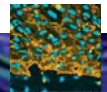
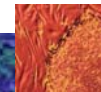
What goes into an RFA?

- OUR MISSION IS THE FRAMEWORK: Short and long-term goals
- Science officer expertise
- Meetings/literature
- Patient advocacy
- Conferences (investigator initiated)
- Workshops (CIRM-initiated)
- Expert panels/consensus groups
- **PROGRESS REPORTS from CIRM GRANTEES**



Science Officer Expertise

- Pat Olson PhD, DSA (biologic, drug development, inflammation, cancer biology)
- Uta Grieshammer PhD (developmental biology, genetics)
- Rosa Canet-Aviles PhD (neurodegeneration)
- Asha Nigh PhD (neurobiology)
- Sohel Talib PhD (immunology, cell therapy)
- Gil Sambrano PhD (training, signal transduction)
- Bettina Steffen MD (surgery, immunosuppression)
- Michael Yaffe PhD (mitochondria, cell biology)
- Marie Csete MD, PhD (critical care, stem cell microenvironment)



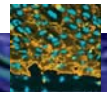
CIRM Workshops

- Predictive toxicology
 - Highlighted need for terminally differentiated cells
- GMP
 - Residual bricks & mortar funds
 - Research and clinical support structure
 - What is the state's capacity? Need?
 - Engineering principles
- Cancer stem cells (with Genome Canada)
- MRC/CIRM (synergy in collaboration?)
- Immunology: Major roadblock to translation



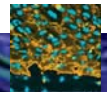
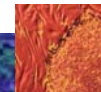
Grant Review: Legislated

- RFAs generated by the Science Office
 - Assigned Science Officers are a resource during proposal preparation
 - Acute scientific needs listed as priorities
 - Reviewers instructed about priorities
- RFA posted after concept approval by ICOC
 - Core grants may allow more flexible schedules
- Turn-around time is short between posting and application due date



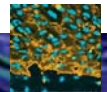
RFAs are broad but details important

- A particular scientific/medical priority
- Work at a particular stage of the translation pipeline
- Work of a certain scale (personnel, \$)
- Read both applicant instructions and reviewer instructions—Reviewers are given priorities



Grants Working Group

- By law: Non-Californians
 - 15 experts score each grant
 - 7 patient advocates
 - Programmatic review at GWG session and ICOC
- Great credentials, well-known experts
 - Good reviewers are those interested in the same field as the applicant; scientific competition is not COI
 - Reviewers who are experts in the field are essential for the process
 - Majority have some industry experience or have started companies



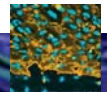
Reviewer Mindset: Key Factors

- Strength and Significance of Research Proposal
 - Not a roadshow for fund-raising: don't oversell
- Experience and track record of PI, other key personnel
- Resources bring to project (important as get into more complex projects)



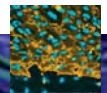
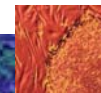
Reviewer Mindset

- Novelty
 - Context of previous work in the area must be made clear
 - Reviewers may well be in a related/similar area
 - Neglecting the pertinent literature is a common criticism
- Medical need or Impact
 - Not a market analysis
 - How the work changes current therapeutic options or impacts the field or a bottleneck



Research Design

- Descriptive studies generally viewed negatively if there is a hypothesis
- Quantitative end-points with power analyses
- Sufficient information on proprietary products
 - Researchers sign confidentiality agreements
- Hypothesis-driven research not necessary for
 - Necessary tools, assays, products that will forward research
 - Necessary research activities for development candidates

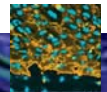
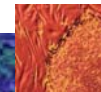


Common sources of failure



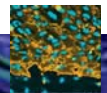
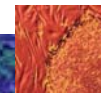
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- Poor writing
 - 10-15 applications: Well-written ones will stick out
 - Goals or hypotheses clear and reinforced
- Lack of focus
- Builds on previous work of applicant without acknowledging significant progress in field
- Evidence of previous productivity in the area is not made clear
 - Highlight previous product development, team leadership successes
 - Use preliminary data to highlight work relevant to proposal



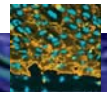
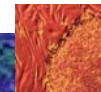
Other issues

- Are personnel, resources, time and space necessary for the work all present and accounted for?
- Are the desired results better achieved with collaborators or on your own? (Sell the synergy)
- Is the amount of money requested for the project appropriate? (Is your budget justified?)
- SCRO, IRB, IACUC take time



Progress reports: We're different

- All grants have yearly progress reports
- Science officers have assigned portfolio
- Site visits will happen
 - Science and compliance (ethics, finance)
- Stewardship of the \$ is important: Unsuccessful projects should not continue to be funded



Balancing the portfolio



- Initial funds devoted to basic research, training (facilities)
- Basic research and clinical research: both essential
- Mandate to identify clinical therapies and cures
- Disease balance? Patient advocates have an important voice

