Report of the External Review

December 2010

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President

Review Report of the External Advisory Panel Dec 2010

1. **Maintain focus on meaningful scientific excellence:** *Ensure the scientific integrity of the grants review is maintained/improved.*
2. **Sustain fundamental discovery:** *Maintain a strong basic science discovery and training programs.*
3. **Paving a path from fundamental to translational research, Translational Medicine, Product Development and Healthcare Delivery**: *Develop a more aggressively proactive approach to identify innovation across the whole therapeutic landscape. Fully engage industry in delivery of therapeutics.*
   - *Gather intelligence on rapidly moving research and translation though industry advisory connections, key conferences, scientists reviewing "hot areas", science and industry advisory groups, collaborative funding partners and societies.*
   - *Identify Californian partners and arrange appropriate linkage*
   - *Enable appropriate entry into Review process*

   Note: have been doing this with CFPs – needs more focus

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4. **Portfolio prioritization process:** *Create an Advisory Group including outside interdisciplinary experts to conduct a critical assessment of current portfolio with objective benchmarks of current portfolio to identify programs that deserve CIRM support to move forward and those that should not.*

   - *Include the consideration of "relevance"- a key consideration for industry support of the merit of early stage projects.*

   Focus the number of projects to those that it believes have the greatest chance of development progress and clinical success, given reasonable timelines and budget.

   - *Consider also support of critical needs in translation that encompass a wider spectrum than single diseases conditions.*

     Note: CIRM portfolio analyses

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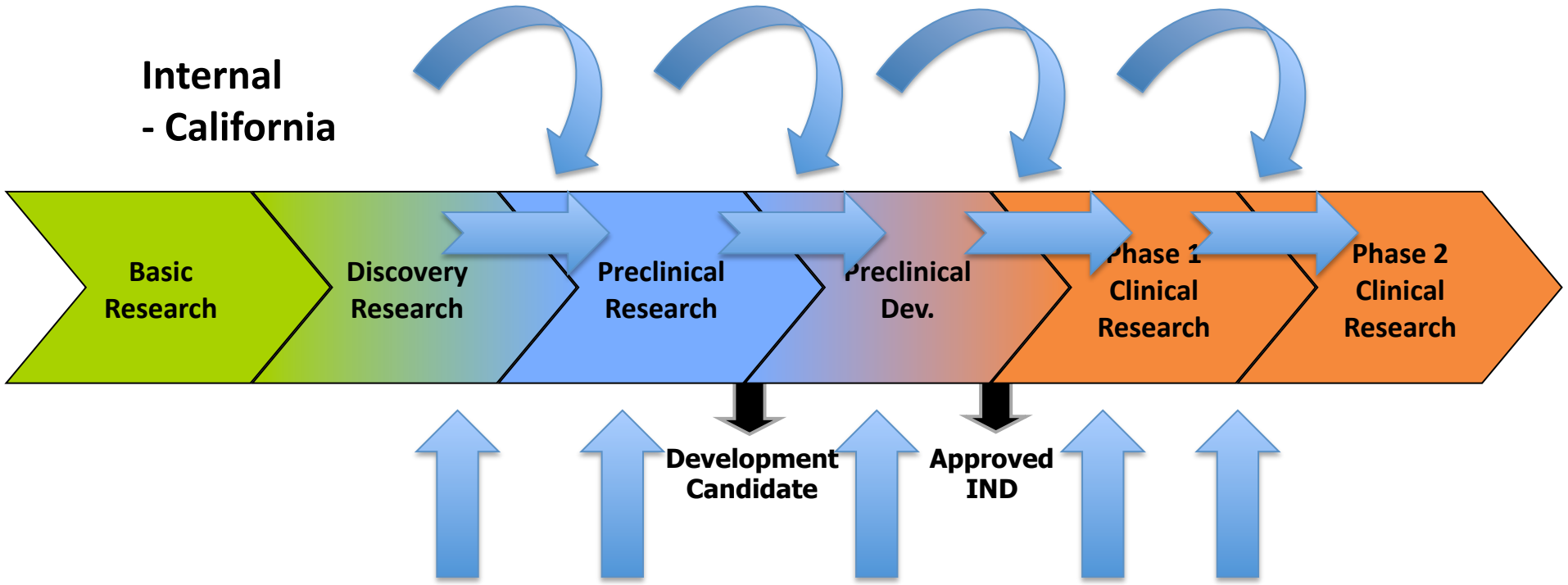
5. Develop an open innovation, porous pipeline policy:

Clinical projects may come from either inside or outside of CIRM funded research, including out of industry and even from outside of California.

- *More flexible project funding process with less administrative documentation and rolling funding cycles to allow for innovative projects to be captured in the CIRM portfolio, particularly from industry, that may not fall within the established RFA cycles*
- *Projects could enter the CIRM pipeline at any stage of preclinical or clinical development*

Accelerating Research Phase II



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6. **Social, ethical, health care delivery and regulatory issues:** *Identify and stimulate research in critical ethical, economic, manufacturing, health delivery and social issues needed for translation into policy and practice:*
   - *Establish an advisory group to identify the critical issues and the primary activities needed to resolve these.*
   - *Standards for manufacturing and cell integrity for research and clinical use should be developed and is a role for CIRM.*
   - *The possibility of providing delivery through CIRM sponsored stem cell clinical units (e.g., α clinic model should be examined and pursued)*
   - *Diversity in CIRM activities need to be continuously examined. Strategies addressing economic barriers, that are in early development, should be actively pursued*

   Note: progress is being made on regulatory issues and their resolution and further work is needed to include international harmonization.

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7. **Industry engagement**: *Enable a more significant engagement with the biopharmaceutical industry. Be more accommodating to industry time/financial restraints.*
   - *Establish the scope and interest of all sectors of the bioipharma industry in providing services, partnering with CIRM and academia, developing major critical capacity e.g., cell manufacturing, standards, international connections, clinical cellular therapeutics, etc.*
   - *Develop rapid entry mechanisms for accessing grant review for industry.*
   - *Streamline grants award processes. Increase industry reviewers for more advanced RFAs.*
   - *Develop strategic partnerships to develop regenerative medicine with biopharma.*
   - *Enhance IP utilization and uptake from CIRM projects.*

     - Appoint an industry advisory committee. *Make engagement effective and complementary.*
     - Make the loans program acceptable to industry. *Allow for negotiated entry to loans.*

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8. **International partnerships:** Broaden and diversify CFPs.
    - *Expand CFPs by identifying excellence, missing capacity, rapid developments and interest to collaborate - that have logical partnership potential in California.*
    - *Encourage multiple international/interstate partnerships with CIRM.*
    - *Explore international industry partnerships that can accelerate clinical therapies – IP and agreements protecting Californian interests.*
    - *Connect/partner with NIH Clinical Institute for clinical trials.*

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9. **Outreach and Education:** Significantly increase the quality and breadth of community outreach and education.
    - *Increase the capacity and raise the interaction with all forms of media to enhance the recognition of CIRM progress and potential contributions to community health.*
    - *Continue to expand patient advocacy and education, including cooperation with FDA and other government agencies.*
    - *Develop media interest articles, videos and other communication tools.*
    - *Utilize scientists with media skills to provide regular updates on CIRM project progress.*

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10. Governance issues: Re-examine the role of ICOC and Management in delivery of the Stage II CIRM program of enhanced effectiveness.

- *Enable the enhanced CIRM program*
- *Be less concerned by process documentation and more flexible for enhanced outcomes*
- *Management is too lean for delivery of the opportunities*