

NIH Center for Regenerative Medicine

Catalyzing stem cell-based translation and therapy development



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**Director, National Institute of Neurological Disorders and Stroke
Council of Councils Meeting
August 15, 2011**



Center for Regenerative Medicine Planning

Aug 09

Nov 09

Jan 2010

Feb 2010

Jun 2010

Aug 2011

Dr. Collins appointed as NIH Director

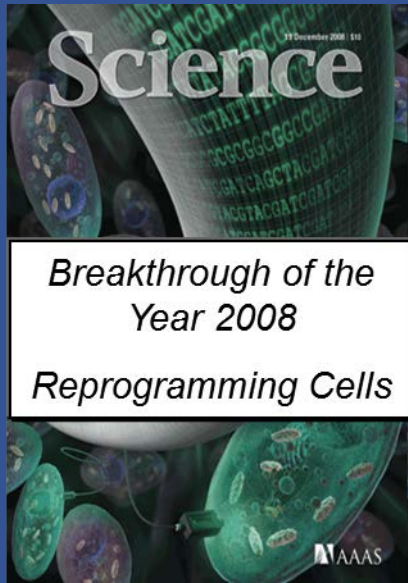
Presentation of priorities to CoC includes facilitation of stem cell translation

Workshop held to identify opportunities; focused on how a gov't resource in the NIH intramural program could have broad impact

Vision for CRM discussed with IC Directors

Pilot projects established in intramural labs to build critical mass of collaborators

Mahendra Rao named CRM Director



Translational Applications of Stem Cells

- Major science breakthroughs in iPS and trans-differentiation technologies
- Opportunities for impacts on medicine immense
- Enormous challenges to bringing stem cells safely to the clinic



NIH Intramural Program is well-positioned to take advantage of this opportunity:



NIH Clinical Center: experience with clinical studies of well-defined patient cohorts, including life-threatening, rare and neglected diseases; a GMP facility for cellular therapies; expertise in gene therapy/stem cell transplantation



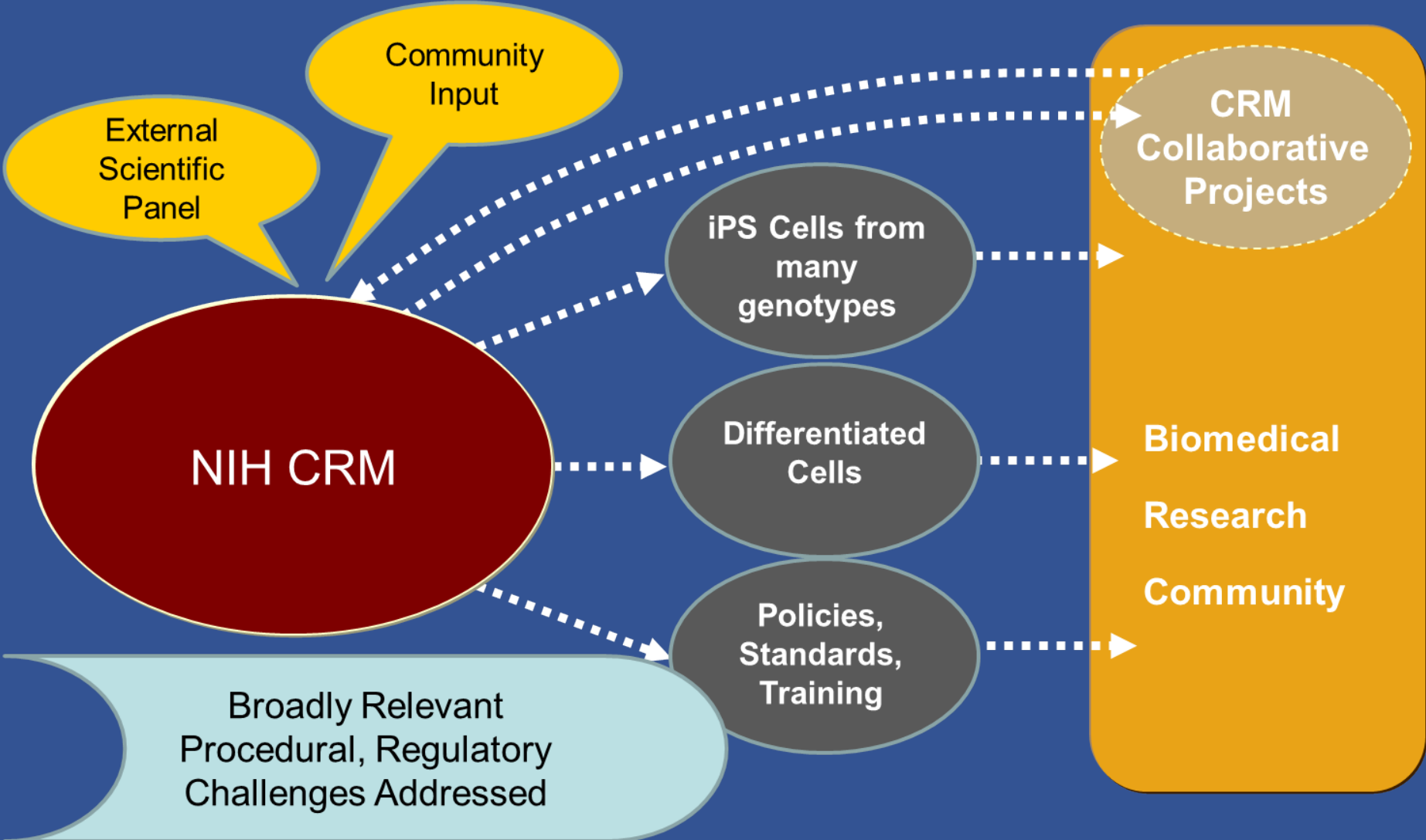
NCGC HTP Screening Facility: small molecules/drugs for phenotype correction, promotion of pluripotency or trans-differentiation, and siRNA screens; SC derivatives also useful for screening for drugs to correct defects, tox screens, etc



Opportunity for Intramural/Extramural Partnerships and Use of NIH Clinical Center: CRM and Clinical Center expertise and resources to be shared with extramural collaborators, with overarching goal of resolving broadly relevant challenges to cell therapy development

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A resource for the entire community





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Create a world-class Center of Excellence in stem cell technology

Timeline:

FY11

Hire a **Center Director** and **renovate space**, provide **seed money** for pilot studies

FY12

Optimize generation of iPS cells from healthy and patient cohorts, investigate differentiation strategies, solicit nominations from the community for genotypes from which to **make iPS and differentiated cells**; **initiate extramural/intramural collaborative projects**

FY13

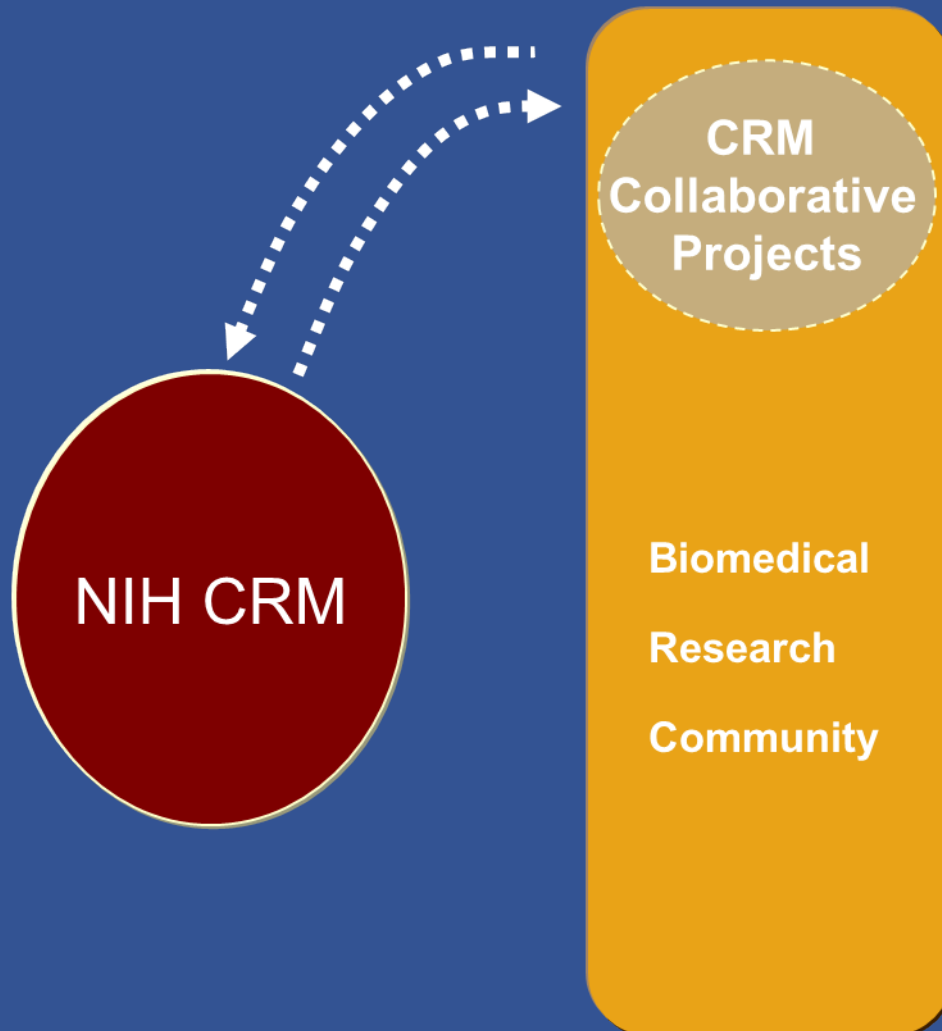
Develop **community-wide standards** for stem cell generation and differentiation; bank and **distribute** iPS cells, their derivatives, and other stem cell types; initiate NCGC screens

FY14-16

Scale-up production of **GMP-grade cells**; work through **policy and procedural hurdles** to cell therapy development; and **advance therapy development** for specific conditions through **collaborative projects**

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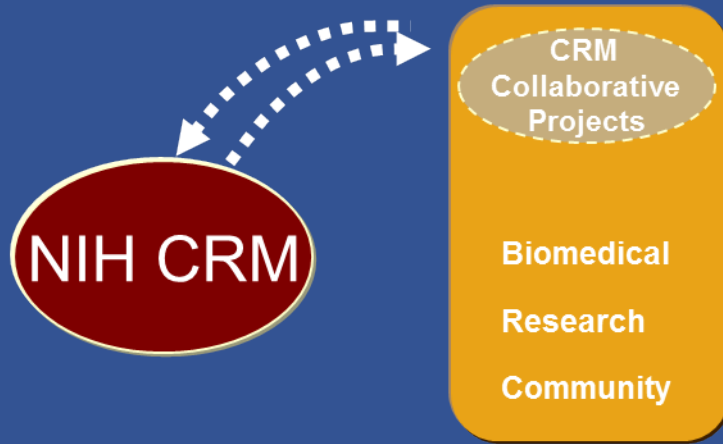
CRM Collaborative Projects: *The Concept to be Cleared*



- Funding Opportunity Announcement for funding in early FY 2013
- CRM to provide resources, expertise in stem cell use and translation; coordination with the community and other agencies to resolve procedural and regulatory hurdles
- Collaborators to provide disease-specific input
- Clinical expertise and resources from the NIH Clinical Center
- Awards to Collaborating Institutions for their part of the collaborations

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CRM Collaborative Projects: The Concept: *Does it meet CF criteria?*



• Transformative?

- Each collaborative project is expected to have extremely high impact for the relevant disease
- The research conducted will allow broadly relevant procedural and policy hurdles to be resolved through CRM's role in coordinating across the community and with other gov't agencies.

• Goals consistent with 5-10 yr timeframe?

- Each project will be a 5 yr award. The goals may fall anywhere along the translational pipeline. Funding to continue beyond the period of CF support will depend on progress and interest by ICs. The regulatory hurdles and other cross-cutting issues are expected to be resolved within the period of CF support.

• Synergy with ICs?

- ICs currently support many translational or clinical projects using cell therapy approaches. The procedural and policy hurdles are solved project by project. The overarching goal for the CRM Collaborative Projects is to facilitate the translational pipeline by harmonizing the process. This must be done within the context of specific projects but is expected to be broadly synergistic with IC funded projects.

• Requires cooperation across NIH and not redundant with other efforts?

- Harmonization of stem cell therapy development is a complex problem that will require coordination across the NIH and with other agencies. The CRM is uniquely positioned to do this, with the Collaborative Projects serving as test cases that will have broad relevance to other projects. No other entity is likely to do this.