NCI and the Common Fund

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Director, Division of Program Coordination, Planning, and Strategic Initiatives
June 23, 2014
Changes Brought by the Reform Act

2004: NIH Roadmap is launched

December 9, 2006: Congress unanimously passes a reauthorization bill affirming importance of NIH and its vital role in advancing biomedical research to improve the health of the Nation

Establishes the Division of Program Coordination, Planning, and Strategic Initiatives (DPCPSI) within the Office of the Director and the NIH Common Fund to provide a dedicated source of funding to enable *trans*-NIH research
Criteria for Common Fund Programs

- **Transformative**: Must have high potential to dramatically affect biomedical and/or behavioral research over the next decade
- **Catalytic**: Must achieve a defined set of high impact goals within 5-10 years
- **Synergistic**: Outcomes must synergistically promote and advance individual missions of NIH Institutes and Centers to benefit health
- **Cross-cutting**: Program areas must cut across missions of multiple NIH Institutes and Centers, be relevant to multiple diseases or conditions, and be sufficiently complex to require a coordinated, trans-NIH approach
- **Unique**: Must be something no other entity is likely or able to do
Current Common Fund Programs (FY14)

Common Fund ~$533M

- Undiagnosed Diseases Program
- Extracellular RNA Communication
- NIH Center for Regenerative Medicine
- Big Data to Knowledge (BD2K)
- Protein Capture
- Single Cell Analysis
- Nanomedicine
- Knockout Mouse Phenotyping
- Global Health
- Metabolomics
- Glycoscience

- Illuminating the Druggable Genome
- Regulatory Science
- NIH Medical Research Scholars
- Human Microbiome

- Health Economics
- Structural Biology
- Science of Behavior Change

- Bridging Interventional Development Gaps (BrIDGs)
- Gulf Oil Spill Long Term Follow Up
- HCS Research Collaboratory
- Nanomedicine

Pioneer Awards
New Innovator Awards
Transformative Research Awards
Early Independence Awards

Library of Integrated Network-Based Cellular Signatures (LINCS)
Genotype-Tissue Expression
Epigenomics
4D Nucleome

Big Data to Knowledge (BD2K)

http://commonfund.nih.gov/
The CF represents a significant investment and a new way of managing science:

- Over $4 Billion expended since inception
  - FY 2014 budget of $533,039,000
  - Similar to mid-sized IC budgets

- All CF programs are managed by multi-IC teams.
- OSC staff are part of each team and provide a bidirectional link between each team and OD Leadership.
- NCI led programs include exRNA Communication, Metabolomics, and 4D Nucleome
Epigenomics is the study of chemical modifications that occur “on top of” the genome. These modifications do not change the underlying DNA sequence, but can regulate when and where genes are expressed.

Alterations in the epigenome are linked to many different kinds of diseases, including cancer.
Epigenomics Program

- Tools, technologies, and data sets generated by this program will enable cancer researchers exploring how the epigenome influences cancer risk and disease progression.
- Several Epigenomics grants have focused on cancer (metastatic breast cancer, Barrett’s neoplastic progression, tumor stem cells).
Epigenomics Research Leads to Link Between Early Environmental Exposures and Risk of Adult-Onset Diseases, Including Cancer

1. Environmental Influence (in utero or childhood)
2. Regulatory DNA Active During Early Development
3. Effects on Adult Health

- Autoimmune Diseases
- Diabetes
- Cancer
- And More
The Patient-Reported Outcomes Measurement Information System (PROMIS) aims to provide clinicians and researchers access to efficient, precise, valid, and responsive adult- and child-reported measures of health.

PROMIS creates a state-of-the-art assessment system for self-reported health.

Many PROMIS outcomes are directly related to the mission of NCI.

PROMIS delivers a quantifiable and reproducible method to assess how patients are feeling, including people with chronic or long-term diseases, such as patients undergoing lengthy cancer treatment. PROMIS has also been applied to pediatric cancer patients.
Central issue: lack standardized patient-centered outcome measures

• Many ways to measure BUT little comparability across tools
• Hinders ability to share, interpret, integrate results

Measurement systems already developed with NIH Funds

• PROMIS®: Patient Reported Outcomes Measurement Information System®
• NIH Toolbox: NIH Toolbox for Assessment of Neurological & Behavioral Function
• Neuro-QOL: Quality of Life Outcomes in Neurological Disorders
• ASCQ-Me: Adult Sickle Cell Quality of Life Measurement Information

4-year Trans-NIH RFA (U2C) led by NCI with co-fund from 12 NIH ICs

• GOALS: Integration, Dissemination, Sustainability
• Step-down funding (25-50% reductions in Years 3 and 4) - less reliance on NIH
• NCI Contact: Ashley Wilder Smith, PhD, MPH: Ashley.Smith@nih.gov
<table>
<thead>
<tr>
<th>IC</th>
<th>Amount (Thousands, Years 1 &amp; 2)</th>
<th>Amount (Thousands, Year 3)</th>
<th>Amount (Thousands, Year 4)</th>
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<td>$75</td>
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<td>$75</td>
<td>$50</td>
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<tr>
<td>NIMH</td>
<td>$100</td>
<td>$75</td>
<td>$50</td>
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<tr>
<td>TOTAL</td>
<td>$3.2M</td>
<td>$2.4M</td>
<td>$1.85M</td>
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Molecular Libraries (2004-2014)

Small Molecule Repository

<table>
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<tr>
<th>Compound Class</th>
<th>Number</th>
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<tr>
<td>Diversity Compounds (DC)</td>
<td>335,528</td>
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<tr>
<td>Controlled Substances (DEA)</td>
<td>141</td>
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<tr>
<td>Non-Commercial (NC)</td>
<td>38,626</td>
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<tr>
<td>Natural Products (NP)</td>
<td>1,956</td>
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<tr>
<td>Specialty/Known Bioactive (SS)</td>
<td>2,770</td>
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<tr>
<td>Targeted Libraries (TL)</td>
<td>10,791</td>
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<tr>
<td><strong>Total</strong></td>
<td><strong>389,812</strong></td>
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Program Outputs:
- 365 probes
- 26 in pre-clinical development
- 7 in clinical development
- 132 patented discoveries
Library of Integrated Network-based Cellular Signatures (LINCS)

- LINCS Data Harnessed to Help Reveal How Cancer Cells Continuously Reproduce (Science, July 19, 2013)
- Drs. Sorger, Golub, and Califano are all part of the NCI ICBP program. There are significant synergies between the two programs
- The CF funds Dr. Golub to generate large sets of data
- ICBP uses the generated LINCS data
- LINCs integrates with the TCGA
## High-Risk High-Reward (HRHR) Research Awards

<table>
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<tr>
<th>Who?</th>
<th>All career stages</th>
<th>All career stages</th>
<th>Early stage Investigators</th>
<th>Junior investigators (within 1 year of Ph.D. or medical residency)</th>
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<tbody>
<tr>
<td>What?</td>
<td>Transformative ideas that may involve large budgets</td>
<td>Creative scientists proposing paradigm shifting research</td>
<td>Early stage investigators proposing high potential impact research</td>
<td>Junior scientists ready for research independence</td>
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<tr>
<td>All areas of basic, clinical and translational science within the NIH mission</td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>Budget?</td>
<td>Up to $25 million per year for 5 years</td>
<td>Up to $500,000 per year for 5 years</td>
<td>Up to $300,000 per year for 5 years</td>
<td>Up to $250,000 per year for 5 years</td>
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<tr>
<td>Prelim data?</td>
<td>Preliminary data not required</td>
<td>Preliminary data not required</td>
<td>Preliminary data not required</td>
<td>Preliminary data requirements less stringent than R01 award</td>
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</table>
Dr. Reya is using her Pioneer Award to develop high-resolution strategies to visualize the behavior of living stem cells during growth, regeneration, and cancer formation.
Discovery of Rational Companion Therapeutic Targets to Optimize Cancer Treatment

Trever Bivona, MD, PhD

**Project goal:** To create an intellectual foundation and experimental platform that will optimize the personalized treatment of cancer patients and improve their survival.

This new approach will use complementary tools, including

- cancer genomics
- genetic screens
- systems network analyses
- clinical therapeutics
- prospectively acquired human clinical data
Engineering of computational receptors and gene circuits for T-cell immunotherapy

*Yvonne Chen, PhD*

Dr. Chen’s Early Independence Award will allow her to improve the safety and efficacy of adoptive T-cell therapy.

- Will address a critical barrier to progress by developing multi-functional genetic constructs previously unavailable in the T-cell therapy toolbox
- Will generate T cells with more robust and precisely targeted anti-tumor activities for immunotherapy against cancer

*Cancer immunotherapy was named 2013 Breakthrough of the Year by Science.*
The Need for Evaluation: What do we want to know?
Assess and advise on the processes used to manage the CF, including those used to plan and implement/oversee programs.

1. Are planning processes optimal for identifying program areas that meet the CF criteria?
2. Are management/oversight processes optimal for achieving program goals?

Report presented on June 20, 2014
Council of Councils Common Fund Evaluation
Overview of Recommendations

Strategic Planning
- Continue efforts to engage a broad range of stakeholders, while exploring new options for gathering ideas
- Enhance communication about the strategic planning process/activities, clearly articulate goals/criteria of Common Fund programs
- Allow greater flexibility in strategic planning and increase opportunities for feedback
- Enhance transparency of decision-making process

Program management
- Strengthen communication between Common Fund staff and IC Working Group members; strengthen communication between all NIH Working Group members and PIs
- Enhance evaluation of Common Fund programs
- Facilitate dissemination of information about Common Fund programs and their deliverables to the extramural research community
Thank you