Director's Update Francis S. Collins, M.D., Ph.D. Director, National Institutes of Health Council of Councils Meeting September 1, 2015



Topics

Budget

- 21st Century Cures
- Tissue on a Chip
- Precision Medicine



NIH Program Level in Nominal Dollars and Constant Dollars



Topics

Budget

- 21st Century Cures
- Antimicrobial Resistance
- Precision Medicine
- Tissue on a Chip



21st Century Cures



ENERGY & COMMERCE COMMITTEE

UNITED STATES HOUSE OF REPRESENTATIVES CHAIRMAN FRED UPTON

Energy and Commerce Cures



114th CONGRESS 1st Session

A BILL

"21st Century Cures Act"

To accelerate the discovery, development, and delivery of 21st century cures, and for other purposes.

A path to 21 st century cures

Highlights:

Reps. Fred Upton, Diana DeGette

- Establishes Innovation Fund (\$8.75B over 5 years)
- Raises cap on loan repayment program
- Seeks to reduce administrative burden for researchers
- Requires Strategic Plan …

SEC. 1021. NIH RESEARCH STRATEGIC PLAN.

Section 402 of the Public Health Service Act (42 U.S.C. 282) is amended-

"(m) Research Strategic Plan.—

"(1) FIVE-YEAR PLANS FOR BIOMEDICAL RESEARCH STRATEGY.—

Strategic Plan Framework

Overview

- Mission of NIH
- Unique moment of opportunity in biomedical research
- Current NIH-supported research landscape
- Constraints confronting the community in the face of lost purchasing power

Fundamental Science

- Foundation for progress
- Consequences often unpredictable
- Advances in clinical methods stimulate progress
- Technology leaps catalyze advances
- Data science increases impact/efficiency

Health Promotion/Disease Prevention

- Importance of studying healthy individuals
- Advances in early diagnosis/detection
- Evidence-based elimination of health disparities

Treatments/Cures

- Opportunities based on molecular knowledge
- Breakdown of traditional disease boundaries
- Breakthroughs need partnerships , often come from unexpected directions

Setting Priorities

- Incorporate disease burden as important, but not sole factor
- Foster scientific opportunity; need for nimbleness
- Advance research opportunities presented by rare diseases
- Consider value of permanently eradicating a pandemic

Enhancing Stewardship

- Recruit/retain outstanding research workforce
- Enhance workforce diversity
- Encourage innovation
- Optimize approaches to inform funding decisions
- Enhance impact through partnerships
- Ensure rigor and reproducibility
- Reduce administrative burden

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Human Tissue Chip for Drug Screening

Goal: develop biochip to screen for safe, effective drugs

- Liver, heart, lung, other cell types
- Use to predict toxicity; efficacy

NIH phase 1 awards (2012): to create individual chips

- Twelve projects to develop 3-D cellular microsystems representing human organ systems
- Seven projects to explore potential of stem cells to differentiate into multiple cell types



Support 11 institutions, to collaborate over three years



Tissue Chip Example: Blood-Brain Barrier (BBB) Chip



BBB-on-a-chip concept



Actual BBB chip



John P. Wikswo, Vanderbilt

Human Tissue Chip, Phase II: Toward an Integrated Microphysiological System

- Integrating BBB, gut, liver, and kidney
 - Neurovascular:
 Vanderbilt
 - Gut: Johns Hopkins
 - Liver: University of Pittsburgh
 - Kidney: University of Washington
- Tissue chip integration supported by DARPA and the Defense Threat Reduction Agency





Miniature devices that mimic human organs could help to replace animals used in drug testing.

BIOTECHNOLOGY

'Organs-on-chips' go mainstream

Topics

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Precision Medicine Initiative: Timing is Everything

nature

insight commentary

VOL 429 27 MAY 2004

The case for a US prospective cohort study of genes and environment

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National Human Genome Research Institute, National Institutes of Health, Building 31, Room 4B09, MSC 2152, 31 Center Drive, Bethesda, Maryland 20892-2152, USA (e-mail: fc23a@nih.gov)

Information from the Human Genome Project will be vital for defining the genetic and environmental factors that contribute to health and disease. Well-designed case-control studies of people with and without a particular disease are essential for this, but rigorous and unbiased conclusions about the causes of diseases and their population-wide impact will require a representative population to be monitored over time (a prospective cohort study). The time is right for the United States to consider such a project.





"And that's why we're here today. Because something called precision medicine ... gives us one of the greatest opportunities for new medical breakthroughs that we have ever seen."

> President Barack Obama January 30, 2015



The NEW ENGLAND JOURNAL of MEDICINE

January 30, 2015

Perspective

A New Initiative on Precision Medicine

Francis S. Collins, M.D., Ph.D., and Harold Varmus, M.D.

66 Tonight, I'm launching a new Precision Medicine Initiative to bring us closer to curing diseases like cancer and diabetes — and to give all of us access to the personalized information we need to keep ourselves and our families healthier."

- President Barack Obama, State of the Union Address, January 20, 2015

The proposed initiative has two main components: a nearterm focus on cancers and a longer-term aim to generate knowledge applicable to the whole range of health and disease. Both components are now within our reach because of advances in basic research, including molecular biology, genomics, and bioinformatics. Furthermore, the initiative

www.nih.gov/precisionmedicine

Precision Medicine

Concept is not new

- Consider prescription eyeglasses, blood transfusions...
- Prospects for broader application raised by recent advances in basic research, technology development, genomics, proteomics, metabolomics, EHRs, Big Data, mHealth, etc.
- Reinforced by 2011 National Research Council report

What is needed now

- Development of rigorous research program to provide scientific evidence needed to turn concept into reality
- Recruitment of the best and brightest from multiple disciplines to join the team



PMI for Oncology

Goal: Apply tenets of precision medicine to cancer To reach this goal, PMI-Oncology will enable research to:

- Use NCI clinical trials as models
 - NCI-MATCH: solid tumors, lymphomas (multi-drug, multi-arm)
 - Lung-MAP: squamous cell lung cancer (multi-drug, multi-arm, randomized)
- Identify new cancer subtypes, therapeutic targets
- Test combination therapies
- Partner with private sector to test precision medicine
- Understand and combat drug resistance



PMI: National Research Cohort

≥1 million U.S. volunteers

- Numerous existing cohorts (many funded by NIH)
- Outreach to underrepresented groups
- New volunteers
- Participants will be:
 - Centrally involved in design, implementation
 - Able to share genomic data, lifestyle information, biological samples – all linked to their electronic health records
 - Can choose how, when to participate in research studies
- Will forge new model for scientific research that emphasizes:
 - Engaged participants
 - Open, responsible data sharing with privacy protections







Patient Partnerships

EHRs





Technologies





Data Science

PMI: ACD Working Group and Its Public Workshops

ACD Working Group (formed March 30, 2015)

 Charge: develop a vision for PMI and advise on design of the national research cohort

Public Workshops

- Unique scientific opportunities for the national research cohort
 - April 28–29, Bethesda, MD
- Digital health data in a million-person precision medicine initiative cohort
 - May 28–29, Nashville, TN
- Participant engagement and health equity
 - July 1–2, Bethesda, MD
- Mobile & Personal Technologies in PM
 - July 27–28, Santa Clara, CA



PMI: ACD Working Group Select Considerations To-Date

Cohort: efficient & inclusive

- Welcomes people of all ages including participants in existing cohorts, members of underserved and underrepresented groups
- Data: secure & accessible
 - Strongly de-identified to increase research use
- Participants: engaged & energized
 - Flexible, transparent experience with return of information and privacy protections
- Technologies: innovative & interoperable
 - Platform for validating applications of mobile and personal devices; finding new uses for participant-provided data



Report will be delivered to the ACD later this month



NIH. Turning Discovery Into Health directorsblog.nih.gov @NIHDirector







