

# Strategic Transformation of Population Studies

## NHLBI Council/BEE Working Group's Recommendations to the NHLBI Advisory Council October 22, 2014

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1

## Objectives

Rationale for Change

Working Group Charge and Process

Working Report Recommendations

2

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3

## Why a strategic transformation now?

### Unprecedented expansion of health information

- New opportunities: exploring the boundaries of phenotype
  - Digital data sources
  - Mobile health (wearable devices)
  - EMR, claims
- New analytical challenges and complexity
  - How do we make use of large scale phenotypic information?

### Fiscal challenges

4

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5

## Working Group Charge

To make actionable recommendations to the  
NHLBI Advisory Council on:

How the NHLBI could take advantage of new  
scientific opportunities and delineate future  
directions for epidemiology and population  
sciences research in heart, lung, blood and  
sleep (HLBS) diseases

6

## **Development of Report: Deliberative Process and Timeline**

- Charge given to a Working Group of NHLBI Council and Board of External Experts (BEE) (Fall 2013)
  - Membership
    - 9 members
      - 5 from NHLBI Advisory Council
      - 4 from NHLBI BEE

7

## **Development of Report: Deliberative Process and Timeline**

- Working Group Deliberative Process
  - 18 meetings from September 2013 to August 2014:
    - 17 conference calls
      - including 19 webinars
        - » to review key aspects of NHLBI-funded cohorts
        - » to garner information on new methods for data acquisition
    - In-person meeting in Bethesda, MD on May 13-14, 2014

8

## Development of Report: Deliberative Process and Timeline

- Presentation of Working Group draft report and recommendations to NHLBI Advisory Council for input and consideration (October 22, 2014)
- Report finalized and submitted (October – November 2014):
  1. To the Director, NHLBI for consideration
  2. For journal publication to facilitate broad dissemination
  3. To NHLBI website for posting

9

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10

## 7 Recommendations\*

1. Scientific forum on population sciences
2. Launch e-epidemiology, particularly in collaboration with other organizations & agencies
3. Building the data-science workforce
4. Dynamic compendium of epidemiologic resources
5. Integration of epidemiology & clinical trials
6. Cohort consortium
7. Competitive external evaluation of cohorts

\*The order does not indicate prioritization

11

## 1. Scientific Forum

NHLBI should convene a scientific forum to determine the major scientific questions and methodological needs in epidemiology and population science over the next 10-20 years.

12

## 1. Scientific Forum

- NHLBI should convene a scientific forum through avenues such as blogs, workshops, and strategic planning groups with the specific goal of identifying the high priority questions for population science.
- Key public health questions and unmet needs should be identified and prioritized by engaging relevant scientific communities.

13

## 1. Scientific Forum

- Include broad scientific communities – not only epidemiologists , clinical researchers, and basic scientists but also, for example:
  - industry, pharmaceutical companies,
  - members of the disease-affected communities, other NIH institutes and Centers
  - professional societies, public health experts
- Process should be integrated with NHLBI's ongoing Scientific Visioning work.

14

## 2. e-Epidemiology

NHLBI should actively engage in studies to establish the validity, reliability, and scalability of electronic tools for primary data collection. In doing so, NHLBI should partner with other organizations and agencies.

15

## 2. e-Epidemiology

- Dramatic expansion of health information
- Diverse data sources, large datasets, rapid change
- Validation of data required before full field deployment
- Reliability and validity of new data sources require on-going study and evaluation

16

## 2. e-Epidemiology

### **NHLBI should:**

- Actively promote investigator-initiated testing of innovative approaches for data collection & analysis
- Support partnerships & programs to develop, validate and share methods to use digital tools for research
- Partner with NIH Office of Associate Director for Data Sciences and others as appropriate (e.g., health systems, other NIH Institutes & Centers, insurers, international collaborations)

17

## 3. Data-science Workforce

**NHLBI should help establish an adequate workforce to conduct population sciences “of the future,” and one approach is to create multifaceted and complementary career development grants.**

18

### 3. Data-science Workforce

**NHLBI should:**

- Prepare a new generation of investigators to work in the new digital enterprise – extensive training in data sciences & new analytic methods
- Partner with others working in this area, including the NIH Office of Associate Director for Data Sciences
- Promote and recognize team science, including a focus on dissemination research

19

### 4. Compendium of Epi Resources

Resources should be dedicated to creating a dynamic compendium of large epidemiologic resources including cohort studies, clinical trials data sets, registries, biorepositories, and other relevant epidemiologic resources to assist the research community in identifying and accessing key existing resources and to improve the return on the investment from these studies.

20

## 4. Compendium of Epi Resources

- The architecture is envisioned to:
  - Enable nimble growth and facile navigation to optimize funding agencies' return on investment
  - Facilitate mentored access to data and specimens in the cohort studies, encourage efficiency in future studies, and minimize redundancy in data collection
  - Incorporate timely information about rapidly enlarging data sets and new patient populations
- NHLBI should also seek partnership with other national & perhaps international organizations

21

## 4. Compendium of Epi Resources

- Recommend a multi-step process:
  - Step 1:** Inventory of all resources including cohort studies, clinical trials data sets, registries, biorepositories
  - Step 2:** Design and development of compendium and user interface
  - Step 3:** Mechanisms to foster access to these resources and collaboration

22

## 5. Integration of Epi and Clinical Trials

Where genuine efficiencies can be created, NHLBI should encourage integration of clinical trials and epidemiology studies.

23

## 5. Integration of Epi and Clinical Trials

### **Opportunities for Integration**

- Clinical trial databases (especially those from pragmatic trials that enroll broadly representative populations) can provide datasets to conduct observational studies.
- Cohort studies can recruit trial participants (if intervention not inconsistent with goal to describe natural history).
- Within healthcare systems, clinical trials and observational cohorts allow for parallel evaluation of the implementation of preventive and therapeutic strategies.
- Closed systems of care can be efficient settings for recruiting and following patients in clinical trials and cohort studies.

24

## 5. Integration of Epi and Clinical Trials

### Practical Considerations

- Scientifically justified and operationally practicable
- Create genuine efficiencies
- Aims of trials & cohort studies should not interfere
- Integration of clinical trials and cohort studies enhanced by dynamic compendium
- Experiences from others, e.g., NIH Collaboratory and FDA Mini-Sentinel, might help provide guidance on opportunities and challenges of integration

25

## 6. Create a Cohort Consortium

NHBLI should create a Cohort Consortium to support large-scale collaborations and provide a coordinated, interdisciplinary approach to address scientific questions, achieve economies of scale, create opportunities for collaboration, and accelerate the pace of research and the implementation of new methods.

26

## 6. Create a Cohort Consortium

- Population-based cohorts provide unique opportunities with proven value and should retain an important place in the NHLBI portfolio.
- Power of current cohorts should be preserved by infrastructure funding sufficient to preserve value.
- Current silo-like approach to funding/managing large-scale epidemiologic studies leads to inefficiencies and missed scientific opportunities.
- NHLBI should create the Cohort Consortium to synthesize, virtually, the existing studies into a coordinated management and scientific structure.

27

## 6. Create a Cohort Consortium

### **The NHLBI Cohort Consortium Should:**

- Foster harmonization of existing data
- Implement de novo data collection methods across cohorts *while* preserving unique features of each cohort and the creativity of its investigators
- Provide opportunities for creating large synthetic cohorts that expand the representation beyond any single study
- Evaluate methods to incorporate data from mobile, home monitoring, and electronic medical records to supplement and widen the duration between examinations

28

## 6. Create a Cohort Consortium

### **The NHLBI Cohort Consortium Should Also:**

- Link with cohorts funded by other NIH Institutes and Centers to build a broad national research resource
- Form a Steering Committee (composed of investigators and other senior scientists) for policy, management, and scientific direction of the Cohort Consortium
  - Committee could advise NHLBI director on infrastructure support, enhancing efficient resource utilization
  - This process could reduce the marginal/incremental cost for new studies and encourage work across sites

29

## 7. Competitive External Evaluation of Cohorts

**NHLBI should implement a competitive peer-review-based model for its portfolio of large epidemiologic and population studies.**

30

## 7. Competitive External Evaluation of Cohorts

- Major new work and large-scale scientific investigations should be determined through peer review
- Investigator-initiated grants should be the predominant driver of the research agenda
- Basic infrastructure such as participant contact and events ascertainment serve as the foundation for hypothesis-driven applications for examination components or novel assays

31

## 7. Competitive External Evaluation of Cohorts

### **Recommend that the NHLBI:**

- Create a new competitive model for the review of infrastructure applications (e.g., an NCI-like U01 mechanism, PAR-14-160)
  - Review occurs in an NHLBI-convened study section
  - Review criteria should emphasize innovation, validation of emerging study designs, and measurement technologies to achieve efficiencies

This approach provides opportunities to preserve valuable research resources in the existing cohorts and, at the same time, to develop new forms of population studies.

32

## 7. Competitive External Evaluation of Cohorts

### **NHLBI Should Also:**

- Develop a process and timetable to transition toward the competitive peer-review-based model with a clear staging of the migration for all the cohorts, their examination components and their infrastructure support\*

\*Note: The Steering Committee of the Cohort Consortium (recommendation #6) should manage these transitions and coordinate changes.

33

## **Discussion and Questions**

34