FDA Medical Products through the Lens of Precision Medicine

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Medical Products and Tobacco

Video Presentation for MHLW and PMDA's New International Strategic Plan Celebration

September 3, 2015

Overview

- **The New Ecosystem**
- **Key Themes**
  - Outcomes
  - Evidence Generation
  - Patient Centeredness
  - Science of Decision Making
  - Ethics
  - Globalization
  - Partnerships across the ecosystem
- **The Precision Medicine Initiative**

The New Ecosystem

- Patients and their advocates
- Providers and Health Systems
- Medical Products Industry
- Payors
- Federal Agencies
- Boundaries are blurred and intentionally brought together
- Democratization of information

Dimensions of Regulation: One size does not fit all!

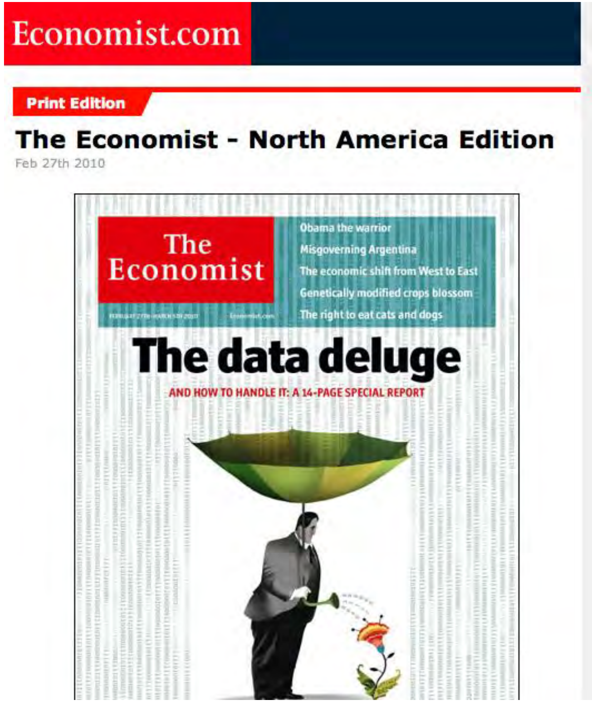
- "FDA is charged with managing the regulatory divide between data-driven pre-market review on one side and the larger healthcare ecosystem in which medicine is practiced on the other side"
- How should we regulate the full range of software, decision support, devices, drugs and biologics?

What is on the Horizon?

consortia-pedia

a FasterCures project
A CENTER OF THE MILKEN INSTITUTE

FasterCures initiated the Consortia-pedia project to better understand the breadth and scope of approaches that a wide range of consortia have adopted to bring together non-traditional partners with a shared R&D goal.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Biomarkers

- Biomarkers are not new
- Used to develop almost all successful medical products
- Now have an almost magical quality

Evaluation of Biomarkers and Surrogate Endpoints in Chronic Disease

Authored by the Committee on Qualification of Biomarkers and Surrogate Endpoints in Chronic Disease

Edited by Christine M. Micheel and John R. Ball

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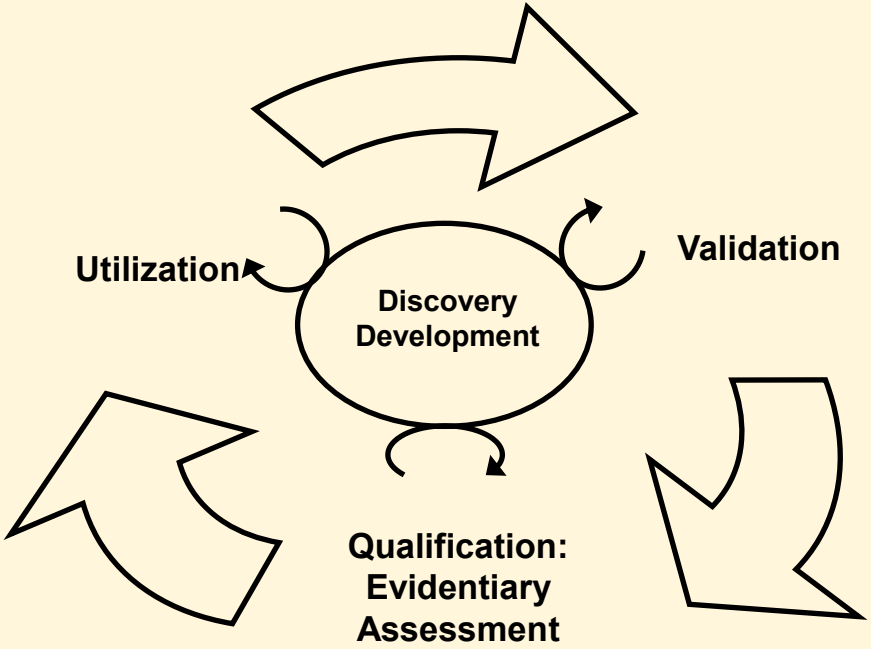
Biomarker Evaluation Framework



Moving Forward…

3 Big Challenges in Biomedicine

1) Lack of information over the time dimension:

Periodic measurements to assess biology and human health are made periodically in visits to healthcare or research

2) Missing systems biology:

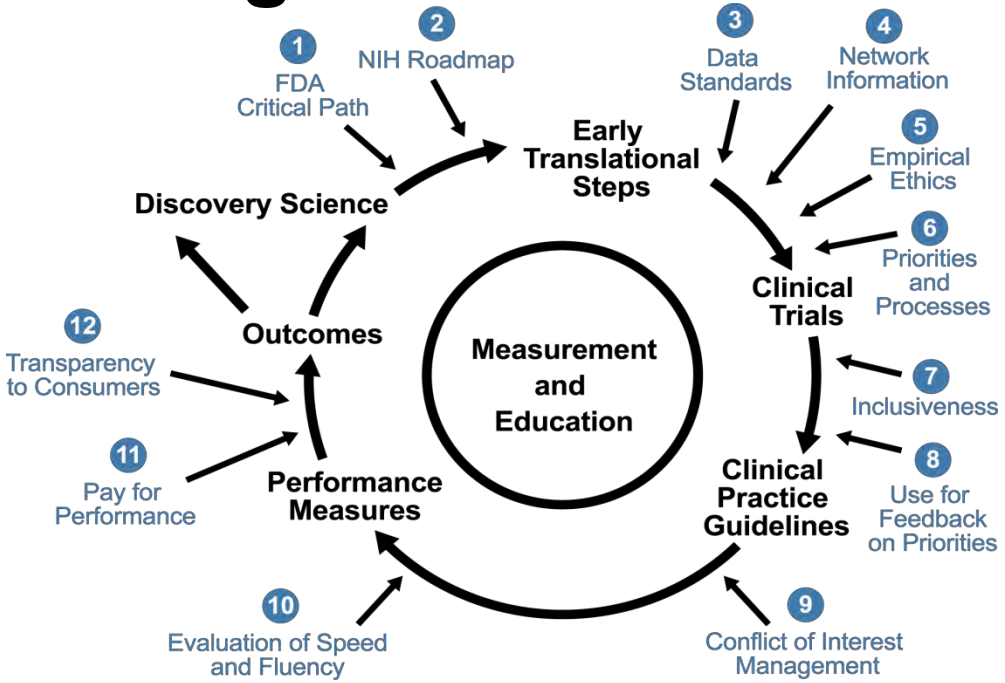
When developing concepts of human biology or drug development we make limited measurements focused on specific mechanisms

3 Big Challenges in Biomedicine

3) Missing the ability to measure the interactions of biology, sociology, environment and decision-making that could enable optimization of individualized and population health:

- Although we know that health and disease are the product of the interactions of genes, multiple derivative biological systems, environment, social context and personal decisions, we tend to look at one part at a time

Generating Evidence to Inform Policy



Our global clinical research system is well-intentioned but flawed

- High percentage of decisions not supported by evidence (Tricoci P et al. JAMA 2009;301:831-41)
- Health outcomes and disparities not improving
- Current system is:
  - Too slow, too expensive, and not reliable
  - Doesn't answer questions that matter most to patients
  - Unattractive to clinicians and administrators

We are not generating the evidence we need to support the healthcare decisions that patients and their doctors have to make every day.

Re-engineering the Clinical Research Enterprise

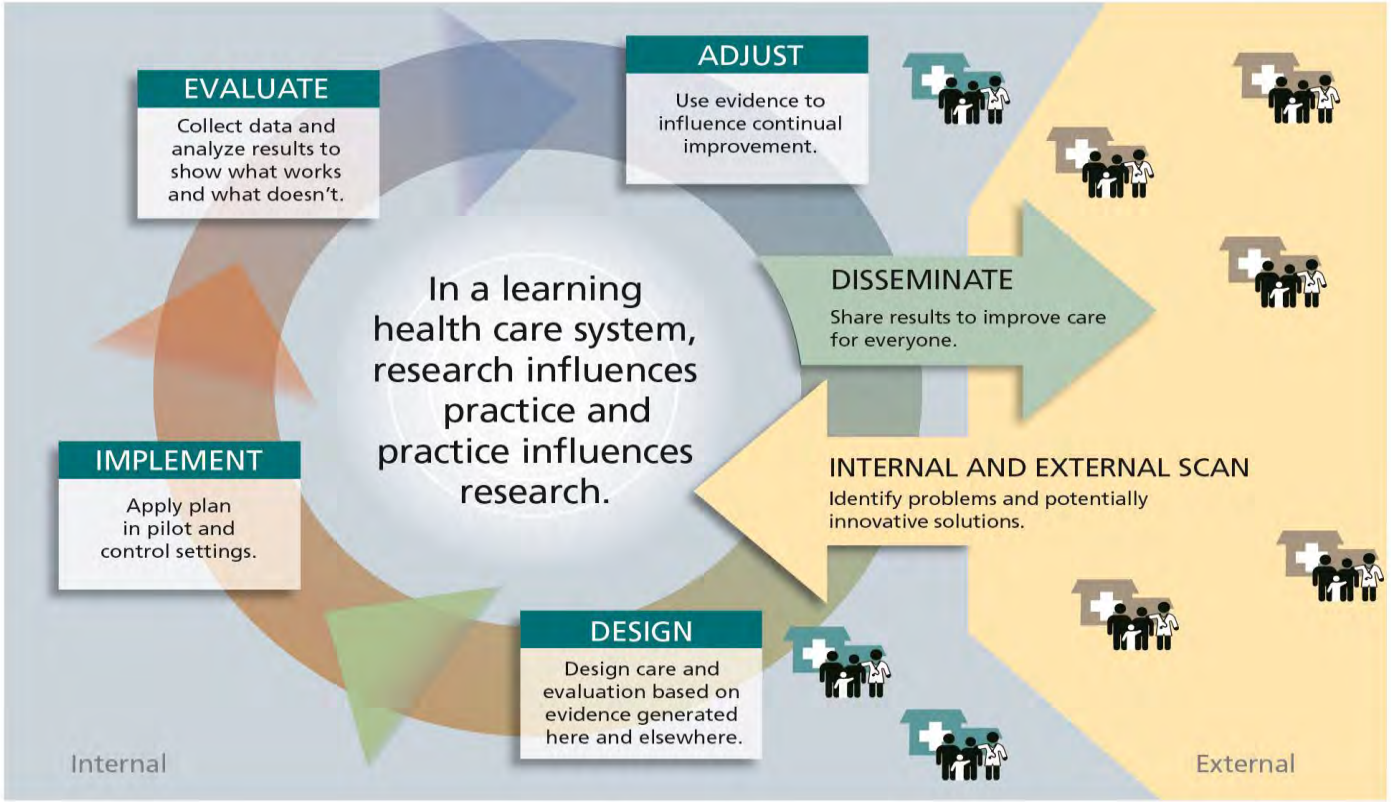
Increasing Level of Difficulty

1-3 years	4-7 years	8-10 years
Plan and start a few demonstration networks Simplify complex regulatory systems – demonstration projects Plan for networks in place for all institutes	Funding mechanism to sustain national system through consensus of all constituents ("1% solution") Simplified regulatory system in place for networks	**National Clinical Research System** creates effectiveness data that moves rapidly into the community AND data on outcomes and quality of care; sustained efficient infrastructure to rapidly initiate large clinical trials; scientific information for patients, families, advocacy groups
Establish repositories of biological specimens and standards for collection Standardize nomenclature, data standards, core data, forms for most major diseases Start a library of these elements shared between institutes and NLM Develop efficient network administration infrastructure at NIH Develop standards for capturing images for research	Data standards shared across NIH institutes Funding mechanisms evaluated to determine which are most efficient	ONE medical nomenclature with national data standards (agreed to by NIH, CMS, FDA, DOD, CDC) Data standards updated 'in real time" through networks National repository of images and samples Critical national "problem list" Most efficient network funding mechanisms in place across NIH
Create NIH standards to provide "safe haven" for clinical research Inventory and evaluate existing public-private partnerships, networks, CR institutions, and regulatory systems Establish FORUM(S) of all stakeholders Establish standards for and pilot creation of a National Clinical Research Corps Demonstration/planning grants to enhance/evaluate/develop model networks	NIH standards for safe haven in place Regulations and ethics harmonized with FDA, CMS Public private partnership mechanisms in place 100,000 members of certified "Clinical Research Corps" Standards shared across NIH	Participation in research is a professional standard (taught in all health professions schools) Study, evaluation and training regarding clinical research a part of every medical school, nursing school, pharmacy school Clinical research practices documented and updated regularly to maintain safe haven Networks provide detailed training about network specific issues

Time

Learning health care systems

GroupHealth



New York Times - December 25, 2014

- **M.B.A. Programs Start to Follow Silicon Valley Into the Data Age**

"Meanwhile, across the country, colleges are adding new courses in statistics, data science and A/B testing, which often involves testing different web page designs to see which attracts more traffic."

Evidence Generation-Human Phenotyping

- **Systems biology now on "launching pad"**
- **The "healthy human state", risk of disease and disease will be characterized in multiple dimensions:**
  - Genetics and genomics
  - Integrated biomarkers and biological pathways
  - Integrated measures of functionality from wearable devices
  - Measures of environment and social interaction
  - Measures of patient preferences and beliefs using personal, interactive devices
  - The "time dimension" will be transformed

Evidence Generation-Human Phenotyping

- **This has major implications for technology development:**
  - "off target effects"
  - Estimates or risk and benefit from multiple simultaneous measures
  - Integration of real time information into device and pharmacological therapy

Evolution of patient engagement in research: PCORnet's history in the making

pcornet

7 Proposed Moral Obligations in a Learning Health Care System*

1) Respect the rights and dignity of patients
2) Respect clinician judgments
3) Provide optimal clinical care to each patient
4) Avoid imposing nonclinical risks and burdens on patients

*Faden RR, Kass NE, et al: Hastings Center Report; Jan 2013: Introduction and 7 commentaries

7 Proposed Moral Obligations in a Learning Health Care System*

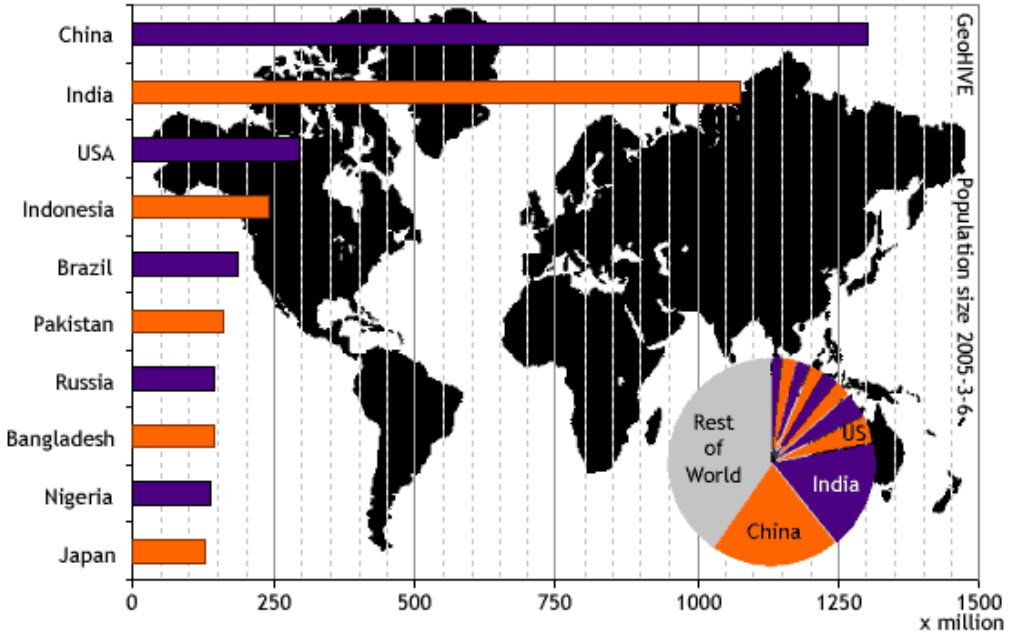
5) Address health inequalities

6) Conduct continuous learning activities that improve the quality of clinical care and health care systems

7) Contribute to the common purpose of improving the quality and value of clinical care and health care systems

*Faden RR, Kass NE, et al: Hastings Center Report; Jan 2013: Introduction and 7 commentaries

World Population Dominated by Asia



•Source: GeoHive

The U.S. Precision Medicine Initiative

NIH National Institutes of Health

DEPARTMENT OF HEALTH & HUMAN SERVICES · USA

Precision Medicine:

Concept is not new:

- **Consider prescription eyeglasses, blood transfusions…**
- **Prospects for broader application raised by recent advances in basic research, technology development, 'omics, EHRs, Big Data, etc.**
- **Reinforced by 2011 National Research Council report**

Precision Medicine:

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

Patient Partnerships

EHRs

Technologies

Genomics

Data Science

THANK YOU!

ありがとうございます