Precision Medicine: Harnessing Biomedical Data to Improve the Prediction, Prevention, Diagnosis and Treatment of Disease

Session #61, February 20, 2017

Jason Levine, MD, Assoc. Director of Clinical Informatics, NCI/NIH
Abdul R. Shaikh, PhD, MHSc, Director, PwC
Speaker Introduction

Jason Levine, MD
Associate Director, IT and Clinical Informatics
Center for Cancer Research, NCI/NIH

Abdul R. Shaikh, PhD, MHSc
Director, Population Health Innovation
PwC
**Conflict of Interest**

Jason Levine, MD
- No real or apparent conflicts of interest to report.

Abdul R. Shaikh, PhD, MHSc
- Past advisor to the Patient-Centered Outcomes Research Institute (PCORI)
Session Overview

This session will provide insight on how innovative national investments in biomedical research and health IT are advancing precision medicine approaches, helping to accelerate cross-sector collaborations between researchers, patients, practitioners and other healthcare system stakeholders for enhanced prediction, prevention, diagnosis and treatment of disease.

- Innovative national investments in biomedical research and health data
- Precision medicine and omics
- Case studies: Bringing patients into the research-to-practice enterprise
- Discussion
Innovative National Investments in Biomedical Research and Health Data

Biomedicine is part of a broader tectonic shift taking place in the culture of science towards a research enterprise that is better at:

I. **Rewarding transparency** in scientific process
II. **Incentivizing data sharing** and secondary use of data
III. Driving more **impactful collaborations** for research and practice
IV. Evolving new models for **sustainability and impact**
Learning Objectives

• Describe how emerging technologies, tools and platforms for precision medicine are driving scientific discovery for treatment and prevention of disease
• Recognize how to leverage and engage with leading health data infrastructure initiatives such as TOPMed, FDA Sentinel and PCORnet
• Analyze the value of NIH’s large population cohort studies for advancing health IT partnerships to enhance assessment of at-risk patients, developing improved options for treatment and prevention
• Identify the opportunities and challenges for advancing research and population health through integrating biomedical and clinical research data with other rich sources of data (e.g., claims data)
• Discuss factors supporting sustainable collaborations amongst researchers, health providers and industry for advancing the potential of precision medicine
Access to publically-funded biomedical data allows the optimization of treatment decisions and enhancement of outcomes – potential impacts including improving the quality of care (i.e., reduce hospital admissions/stays, improve disease management) and quality of life.

Patient-centered outcomes research data from EMRs can be combined with other data to help tailor clinical treatment based on individual patient attributes and the bio-psycho-social determinants of disease.

New data collection tools can enhance the collection and use of patient-reported outcome measures for advancing approaches to patient-centered precision medicine research.
Innovative National Investments in Biomedical Research and Health Data (cont.)
Precision Medicine and Omics

A **new frontier** for providing the best available treatment and preventive care for each patient.

- Accounts for individual variability in environment, lifestyle, and genes
- Leverages health IT innovation to harness very large sets of linked health-related data
- Brings innovation through new tools and approaches — high-throughput analysis, patient-reported data, analytics, molecular diagnostics

Feasible for:
- Performing omics analyses for **every single patient** in research studies
- Correlating omics findings with known drug targets and with toxicity-prone conditions in near-real-time
Omics: Actionability Challenges

Responsible disclosure of high-probability findings that:

- Don’t have **direct implications** on the condition being treated
- Have potentially-serious implications on **family members** of the patient
- May impact family health **across generations**
- May not have **clear treatment** protocols

Deyholos, MK & Harrington, MJ (2013). Open Genetics - Fall 2013
Patient-Centered Precision Medicine

As “personalized” as precision medicine can be — the patient directly reports their own perspective on their disease and the therapeutic experience (i.e., PROs/PROMs).

At times, PROs may stand in opposition to clinician-observed measures that have governed clinical research since time immemorial.

PROs provide another layer of data to inform clinical and omics discoveries, placing scientific findings into the context of the individual patient’s real-world experience.
Case Studies

Two examples on how health IT is bringing the patient perspective into the research-to-practice enterprise:

1. **Scribe**: A system enabling the collection of patient-reported outcome measures for clinical research

2. **PCORnet**: The National Patient-Centered Outcomes Research Network

---

NATIONAL CANCER INSTITUTE
Center for Cancer Research
Case Study 1: Scribe

Robust system allowing electronic collection of patient-reported outcome measures (PROM) data directly from patients to integrate directly into clinical research.

Developed at the CCR, and will be used in CCR’s clinical omics protocol coming online this year.

Fulfills the need to feed PROM data into the precision medicine pipelines that are being used to process all the other clinical and omic data collected on research studies.
Case Study 2: The Patient-Centered Outcomes Research Institute (PCORI) was authorized by the ACA to fund comparative effectiveness research (CER) to help patients, caregivers, and clinicians make evidence-based decisions on healthcare = *Research with patients, not just for patients.*

**PCORnet: The National Patient-Centered Clinical Research Network**
- Comprised of leading AMCs, hospitals, universities, patient advocacy groups, foundations, and other health-related organizations across 13 Clinical Data Research Networks (CDRNs), 20 Patient-Powered Research Networks (PPRNs), and a Coordinating Center.
- Covers 100M+ lives in a ‘network of networks’ that involves patients in research
- Represents the convergence of patient-centered research with precision medicine and population health
PCORnet is in Phase II of its development:

- Expanding **generalizability and diversity of data**
- **Common Data Model** (CDM) v3.1 open access
- **PCORnet Commons** and **Front Door**
- Conducting a **broad range of CER**: Aspirin/CVD, obesity/weight loss surgery; antibiotics in children…
- **Health Payer Demonstration Projects**: Humana and Healthcore/Anthem

**Coordination and stakeholder engagement** are essential for PCORnet to be successful in driving new collaborations spanning the healthcare spectrum.
Access to publically-funded biomedical data allows the optimization of treatment decisions and enhancement of outcomes – potential impacts including improving the quality of care (i.e., reduce hospital admissions/stays, improve disease management) and quality of life.

Patient-centered outcomes research data from EMRs can be combined with other data to help tailor clinical treatment based on individual patient attributes and the bio-psycho-social determinants of disease.

New data collection tools can enhance the collection and use of patient-reported outcome measures for advancing approaches to patient-centered precision medicine research.
Discussion

• How are emerging technologies, tools and platforms for precision medicine driving scientific discovery for treatment and prevention of disease?
• How might organizations spanning the healthcare ecosystem engage with, and leverage greater value from leading health data infrastructure initiatives?
• What are some of the challenges and solutions for the ‘practical’ integration of biomedical and clinical research data with other sources of data?
• What are some promising methods and approaches for advancing patient-centered health IT for improved outcomes and cost savings?
• How can ‘basic’ biomedical data be used to enhance assessment of at-risk patients and improve the diagnosis, treatment and prevention of disease?
• What are some promising models and approaches for developing sustainable collaborations amongst researchers, health providers and industry for precision medicine?
Continuing the Conversation

• Jason Levine, MD  
  levineja@mail.nih.gov  
  https://www.linkedin.com/in/jasonlevine

• Abdul Shaikh, PhD, MHSc  
  shaikh@us.pwc.com  
  @abdulrshaikh  
  www.linkedin.com/in/arshaikh