

A 21st Century Vision for Clinical Research

Ralph I Horwitz

Jill Abell

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The Time is Now

Vision for Research in a Learning Health System:

Critical elements are available

- Advanced Research Methods
- Sophisticated Digital Infrastructure
- Willing Clinicians, Administrators & Payers
- Empowered Patients & Caregivers

Advanced Methodological Toolkits

- Real-World RCTs
 - Pragmatic, LSTs, Cluster-Based, Point of Care, EMR Embedded, Web-Enhanced
- Observational Studies
 - New-User Design, Inception Cohorts, Self-Controlled Case Series, Propensity Scores, Instrumental Variables
- Statistical Tools
 - Hierarchical Models, Time Varying Confounders, Classification and Regression Trees (CART), Computational Modeling Clinical Data

Sophisticated Digital Infrastructure

- Enables collection, storage, linkage and analysis of data in real-time
- Ability to capture data longitudinally and link from various sources (including remote capture from patients)
- Capable of providing real-time feedback

Goal: To develop a secure global network for multi-institutional collaborative research

- Common language for medical terms
- Agreement on clinically meaningful outcomes
- BD2K

Leaders are Ready

Overall impatience with the time to translate research into practice

- Concerns of “waste” and inefficiency in the system
- Growing intolerance of avoidable error
- Willingness to experiment with payment systems

Physicians are Leaders

- Increased accountability for success
- Move towards personalized/stratified medicine
 - Desire to tailor treatment for greatest benefit at lowest cost

ABIM Initiative on “Choosing Wisely” to achieve
Better Care at Lower Costs

Patients & Caregivers are Waiting

- Emphasize Patient & Caregiver empowerment and engagement as critical elements of the learning process.
- Capture of the patient experience (symptoms, function) is critical for ALL clinical studies.
- Enhance patient trust in the health system.
- Elicit patient preferences and incorporate into care

Regulatory Science

The science of developing new tools, standards and approaches to assess the safety, efficacy, quality and performance of FDA-regulated products

IMEDS, OMOP and Mini Sentinel are leading examples of this commitment to evidence-based decision making

“regulatory science is the critical link—the bridge—between cutting-edge discoveries and real-world diagnostics, treatments, and cures”

-Hamburg

Considerations

- Engagement: What strategies should be employed to engage Payers? Providers? Patients?
- Trust is essential. How do we gain the trust of patients and caregivers?
- Communication: How do we disseminate what we learn to ensure rapid translation into practice?
- Ethical Framework: How do we evaluate and maintain best practices while protecting patients?
- Finance: How do we fund, allocate and sustain this work?

Financial Sustainability Considerations

The exchange of health care information is very worthwhile but a sustainable model of financial support is lacking.

- FDA spends ~\$10M/year to make data from 18 health plans' claims and EHR usable for Mini-Sentinel.
 - Several more million every year is needed to support design and analysis. Costs range from negligible (not \$0) to over \$1M.
- The REDUCE MRSA cluster randomized trial conducted in 43 hospitals required hundreds of thousands of dollars to transform, clean, and analyze the data.

Funding and sustainability must be taken into consideration.

21st Century Vision for Clinical Research

- Inspiring initiative that captures both the aspirations and realities of medicine in the real world
- Set a course and provide a map to achieve our goal of best care, lower costs and highest achievable levels of population health

Presentations & Discussion

Methodological Considerations

- *Elliott Levy, BMS*
- *Deborah Zarin, National Library of Medicine*

Organizational Culture

- *Joe Selby, PCORI*

Patient Engagement and Trust

- *Stacey Linh, Sisters by Heart*

Open Discussion

- *All*