

A Rapid-Learning Health System

*A National Data System for Clinical
Research: Development & Uses*

Lynn Etheredge
National Committee on Vital and Health Statistics
July 17, 2007

Overview

- **A Rapid-Learning Health System**
 - Concept, needs, capabilities, developments
- **Uses of computerized research databases for rapid learning**
 - Examples: comparative safety & effectiveness, genetic and clinical data associations, heterogeneity of results, hypothesis generation & testing, filling inferential gaps in clinical evidence, practical clinical trials & innovative research designs, predictive models
- **Designing a National Data System for Clinical Research**

A Rapid-Learning Health System

- A new national process that uses computerized EHR databases to enable real-time learning from tens of millions of patients annually
 - A high-potential research environment
 - Data-poor --> data-rich
 - Research can be done quickly and inexpensively: “research at the speed of thought”
 - Exponential growth in research studies and the evidence base for clinical care
- *National Goal:* Learn about the best uses of new technologies at the same rate that the health system produces new technologies

The Need for Rapid Learning

- **New medical knowledge and technology advance much faster than clinical evidence about their best use**
 - Even well-intentioned physicians and patients confront many uncertainties in making decisions
 - Advancing evidence-based medicine is a slow process
 - Expanding technology use is a major cost driver
- **Randomized clinical trials (RCTS)**
 - Have been the “gold standard”, will be important
 - But “take too much time, are too expensive, and are fraught with questions of generalizability”

The Need for Rapid Learning

- There are major “inference gaps” in the evidence base for clinical care
 - RCTs mostly use younger populations, with single diagnoses, and brief study periods - and leave out typical patients
 - Medicare & Medicaid populations are largely excluded from the clinical trials database
 - 85 million enrollees
 - Federal expenditures: \$ 600 billion annually, \$3.5 trillion in next five years, \$8 trillion in next decade !!
- The evidence base is weakest where physicians, patients, and public decision-makers need it to be strongest

The Need for Rapid Learning

- There are major areas of medical care that lack quality and outcomes measures, evidence-based guidelines, and performance reporting
 - IOM, AHRQ (MMA 1013), NQF, AQA, HQA, APQ, NCQA...
- Clinical research databases and registries are typically small, unique, specialized, difficult to find, access and use, non-comparable, and proprietary
- Most of what could be learned from the individual experience of tens of millions of patients each year (and \$2 trillion/year of health expenditures) is now lost
 - Pediatric oncology vs Medicare cancer care

National Learning Leadership

- **Integrated delivery systems**
 - Kaiser-Permanente: 8 M EHRs
 - Geisinger: 3M EHRs
 - VA: 8 M EHRs
- **Research networks (“virtual research organizations”)**
 - HMO Research Network (15 HMOs, 20 M patients)
 - Cancer Research Network (NCI)
 - Vaccine Safety Datalink (CDC)
- **A National RL system**
 - RL networks for enrolled populations, health conditions, technologies, geographic areas, age cohorts, special populations

Recent and Future Developments

- **FDA's Sentinel Network**
 - S. 1082, passed Senate 93-1 on May 9, 2007; 100 million patient records by 2012; core of a US national system for comparative effectiveness and safety research
- **AHRQ: \$15 M initiative for developing RL networks**
- **Archimedes/ARCHEs - predictive computer model**
 - Computers + mathematical models + systems biology + RCT studies + EHR databases; rapid learning “on turbo”; RWJF national support
- **Proposals for a national comparative effectiveness system**
 - Ways & Means hearings (June 12), MedPac, CBO testifying favorably

Recent and Future Developments

- **NIH**
 - RL Heart Research Network (NHLBI)
 - EHR-Genomics Research RFP (NHGRI)
 - CTSA (Clinical & Translational Science Awards), \$ 500M (NCRR)
 - NCI: CaBIG
 - Other institutes?
- **CMS**
 - Part A, B, D integrated research files (45 million persons)
- **EPIC & EPIC EHR users**
 - 50 M + EHRs
 - National RL network for children's health?

Rapid Learning and the Future of Research

- **Methods**
 - RCTs --> RL database research, predictive models, practical clinical trials
- **Organization**
 - Large research databases & programs
- **Financing**
 - Much more financing, many more studies
- **Collaboration**
 - Multiple research teams, common computer-searchable databases (e.g. genome project), networks
- **Users**
 - Physicians, patients, payers, delivery systems, public decision-makers, bio-tech industry, biomedical researchers, etc.

Rapid Learning and the Future of Research

- Uses (research databases <--> individual records)
 - Comparative safety and effectiveness, S. 1082 (FDA)
 - Genetic & clinical data integrated studies (deCODE, UK)
 - Heterogeneity of results; targeting effective therapies, risk-mitigation
 - Hypothesis generation & testing
 - Filling “inferential gaps” in clinical evidence
 - Practical clinical trials & innovative research designs
 - Predictive models

A National Data System for Clinical Research

- Adopt a national goal to advance clinical care as rapidly as possible
 - HHS and/or new public-private entity
- Develop a national system of rapid-learning networks
 - Covering all diagnoses and patient sub-populations
 - With standards for certified research databases and registries
 - For clinical research
 - For development of evidence-based quality and outcomes measures, treatment guidelines, and performance reporting
 - Supporting mission of HHS health agencies (FDA, AHRQ, CDC, NIH, CMS, SAMHSA)

A National Data System for Clinical Research

- **Develop a national system for comparative effectiveness studies of new technologies**
 - Require reporting for new technologies, using computerized EHR databases and RL networks, at market entry
 - A national “coverage with evidence development” policy
 - Periodically re-assess technologies and future research needs
 - Learn as much as possible, as soon as possible, about the best use of new technologies

A National Data System for Clinical Research

- **Develop National Database(s) for Clinical Research**
 - Drawn from RL networks databases by inclusion and/or statistical sampling, from RCTs & publicly-funded research.
 - Fully de-identified data
 - Including genomic information, EHR/CDISC-level clinical data, quality, outcomes, and performance measures
 - Similar to human genome project database(s) for collaborative research; NLM (Medline); US economic research databases (Bureau of Labor Statistics, Commerce Dept., SEC reports)
 - Open access for all persons

Summary

- **New EHR research databases have great potential to advance the evidence base for clinical care**
- **A National Data System for Clinical Research will require public and private collaboration**
 - A national goal to advance clinical care as rapidly as possible
 - Rapid learning networks for all diagnoses
 - A national system for comparative effectiveness studies
 - National database(s) for clinical research