

# A Rapid-Learning Health System

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

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# 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