A Rapid-Learning Health System

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_A National Data System for Clinical Research: Development & Uses_

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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