

“Rapid Learning” Research Capabilities

Biomedical Science, Health Care & Public Health

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Introduction

- Launch of *Precision Medicine Initiative*, a flagship for revolutionary changes in research, health care & public health.
- A rapid-learning health system. To learn as much as possible, as soon as possible, about the best care for each patient – and to deliver it.
- Digital technologies enable new, exciting answers to “How fast can we learn?”

New Research Capabilities – BD2K

- Desktop access to the world's evidence base
- Clinical trials
- Observational studies
- Global collaborations

New Research Capabilities

- Desktop access to the world's evidence base for biomedical science
 - NIH national reference databases, including 1 M person *Precision Medicine Initiative*, w/ research tools
 - NIH Commons of open science research data clouds for all NiH-supported research; BD2K centers of excellence; data standards, reporting requirements, archive and use financing
 - FDA on-line clinical research databases (de-identified)
 - NCI 3 cancer cloud pilots (early 2016), exabytes of data & research tools

New Research Capabilities

- Faster, less expensive clinical trials & standing trials networks
 - TASTE study: \$300,000 vs \$10 M, in research system with established computerized registries, i.e. 95% + savings (Lauer-NEJM)
 - NCI MATCH: 2,400 sites (July 1), simultaneous testing of 15-20 protocols, standardized comprehensive data; Bayesian predictive model for informed choice of best option; potential enrollment of most patients vs 3-4%.
 - Standing trials networks: CER comparable results at time of market entry
 - FDA: Learning Medical Device Ecosystem: A Neural Network; *Precision FDA* (beta)

New Research Capabilities

- Faster, less expensive observational studies & learning networks
- Far more individual level, clinically rich, longitudinal data, hundreds of million patient years of data.
 - NIH *Precision Medicine Initiative* and reference databases. National data & research support center for NIH, FDA, PCORI with common data model and research tools (Harvard-Rich Platt), organized research registries, databases and learning networks (e.g. FDA mini-sentinel 150M+ patients, 300 M+ patient years), 29 PCORI networks, NIH HCS Collaboratory (academic centers), HCS Research Network (Kaiser, Geisinger, et. al.), VA. Optum (150 M patients).
 - 2-3 weeks per study vs 2+ years, 95% faster (Platt, et. al. NIH Grand Rounds), 20x number of studies annually

New Research Capabilities

- Global collaboration
 - UK: 65 M EHRs, 500,000 biobank, cancer registry, social welfare data, etc, 24 universities, 4 bio-informatics centers of excellence; Farr Institute
 - EU TRANSFoRM initiative for rapid learning health system (10 countries); EHR4CR
 - OHDSI.org (started by Foundation for NIH) (11 countries, 600 M patients; common data model, 10 open-source software research tools)
 - GA4GH genetics data-sharing agreement, 41 countries

New Research Capabilities – K2P, P2BD

- Effectiveness and safety
- Financing and delivery
- Professional education & delivery support
- Patient engagement, data and support

New Research Capabilities – K2P, P2BD

- Effectiveness & safety: FDA mini-Sentinel, 29 PCORI networks & \$600 M CER studies, NIH-sponsored networks (cardiovascular, mental health, etc.)
- Financing & delivery
 - \$10 B CMS Innovation Center, dozens of delivery system models, Partnership for Patients, Million Hearts, Strong Start
 - Sec. Burwell: 30% of payments in alternative payment models by 2016, 85% of FFS in pay-for-performance in 2016, as part of all-payer strategy

New Research Capabilities – K2P, P2BD

- Professional education & decision support
 - Physician desktop access to world’s clinical evidence base (research studies and “patients like me” databases), predictive models for individual diagnosis and comparison of treatment options, on-line and peer network consultations, e.g. WATSON, Archimedes/ARChES, Adjuvant
 - ASCO’s rapid learning cancer system (CancerLinQ)
 - Project ECHO rapid diffusion of new knowledge and specialist-level care in rural and underserved communities using case-based learning, peer networks and video-conferencing
 - Pediatric specialist CME based on sharing data with specialty registry

New Research Capabilities – K2P, P2BD

- Patient engagement, data & support
 - Patient networks (cystic fibrosis, Patients Like Me)
 - Apps
 - Mobile devices, internet of things, nano-technologies
 - Genome analyses & much more, e.g.23 and Me, Eric Topol's *The Creative Destruction of Medicine* – patient-centered, on-line diagnoses, prevention, treatment advice
 - UK international diabetes Knowledge Portal data cloud for patients and open science, 100,000 patient records

RL Research Science Software

- NIH
 - National Center for Biotechnology Information (48 tools)
 - BD2K & BD2K Centers of Excellence (Stanford, USC, UCLA, UC Santa Cruz, Wisconsin, Illinois, Pittsburgh, Memphis, University of Illinois, Mt. Sinai, Broad, Harvard)
 - NCI: National Cancer Informatics Program ; 3 cancer genomics cloud pilots (Broad, Institute for Systems Biology, Seven Bridges Genomics); NCI Biomedical Research Branch (Array)
- FDA
 - Precision FDA

RL Research Science Software

- POPMedNet & coordinating centers
- CMS : Chronic Conditions Data Warehouse; Virtual Research Data Center
- NSF: iplantcollaborative.org
- Farr Institute (UK)
- Observational Health Data Sciences and Informatics (OHDSI) (international)
- Global Alliance for Genomics and Health, (37 countries, 380 members),
Accelerating Medicines Partnership

What's Next?

- From research capabilities to research funding and rapid learning
- Filling gaps: patients with multiple conditions, high cost-patients, minorities, pregnant women and children, socio-economic data, environmental data, culture
- NSF: toward a science of rapid-learning systems and a rapid-learning society (15 program areas, agriculture, education, economics)
- A rapid learning public health system? NSF, “Precision Public Health for the Era of Precision Medicine” (Khoury et al Nov 2015)

A RL Public Health System

- NSF “use case” requirements: The LHS develops the capability to detect disease clusters or outbreaks; maintain a geographical information system capturing the spread of the disease; and convey important information to public health officials, care providers, and the general public.
- A nationwide geographic-based disease information system is automatically populated by EHR data as new cases are diagnosed. The system can provide views of varying geographic granularity to local, municipal, state, and national public health agencies

RL Public Health System (cont)

- The system will detect unusual rates of naturally occurring diseases, adverse drug reactions, responses to environmental exposures, and other health outcomes that affect all people equally or a vulnerable segment of the population; have temporal clustering that is acute, sub-acute, or chronic; are “expected” or unexpected; and are common or rare. .
- The system can integrate data from multiple sources including electronic data sources, Internet search engines, retail sales databases, and others.
- The system can alert clinicians of the likelihood that the condition will affect their practice localities.
- The system can learn from data variations over space and time.