A Genomics-Enabled Rapid Learning Health System

Goals for 2015 & 2016

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A Rapid-Learning Health System

- Core Concept: *A computerized health system designed to use large, new distributed databases and learning networks with tens of millions of (privacy-protected) patients records*

- Goal: *To learn as quickly as possible about the best medical care for each person – and to deliver it !*

- Where we are today – pre-genomics RL system
- Upgrades for genomics RL System: data, research, Rx testing, CER, coverage & payment, public health, clinical decision support, high quality care

A RL National Research System

- NIH (CTSA, Collaboratory), FDA (mini-Sentinel), PCORI (29 networks) (Richard Platt et. al., NIH Grand Rounds, 11/14/2014)

- A Common Data Model (pre-genomic, selected from EHRs & claims), national system for computerized studies & clinical trials, with distributed database networks, national coordinating center(s), automated study design, data quality checks, analysis and reporting tools – potential 150 M + patients (privacy protected)

- Performance gains:
  - Database studies: 2 years→2/3 weeks (3.9m patients, 18 locations)
  - Randomized registry trials: $10 M+ → $300,000 (TASTE trial)
  - Drug safety: months → 24 hours

RL Benefits (pre-genomics)

- Data-scarcity → data abundance
- Many more studies, faster learning (10-20 x +/year; 1-2 decades of studies/year)
- Many more patients & sub-groups & diseases (seniors, children, patients with multiple illnesses, minorities, rare diseases)
- Many more questions, many more patient & physician- useful studies –> a CER science for informed choices, better care
- Many more researchers & learning networks, collaborations, data analysis firms (e.g. Optum)

RL Benefits (pre-genomic)

- Comparable research results, growing evidence base (150 M patient years/annually) & re-useable data
- More research funders (specialty societies, patient groups, health plans, hospital groups, ACOs, foundations)
- Better predictive models for patients (RCTs + databases)
- A national research system designed for discovery science (quickly/inexpensively generating & testing theories)

Economics of Data-Sharing

- If 10 institutions each share 100 cases
  - Database = 1,000 cases
  - *Every* institution gets 900 added cases for a contribution of 100 = 9:1
- If 100 institutions each share 1,000 cases
  - Database = 100,000 cases
  - *Every* institution gets 99,000 added cases for a contribution of 1,000 = 99:1
- Data-sharing is a high pay-off strategy. More data-sharing multiplies benefits.

Upgrading to Genomics-Enabled RL Health System -- Data

- Key strategy – Add genomics data to the Common Data Model's patient records, RL research system (include VA), and RL studies
  - What data?
  - How many patients?
  - Lessons from the pioneers?
  - What population cohorts are needed for "precision medicine", genetics-enabled research, drug development, CER, personalized (N=1) medicine?
- Funding: NIH institutes
- Time frame: 2015 & 2016

NIH Upgrades, 2015-2016

- Expand use of the new Master Protocol trials system
  - Lung cancer pilot, 200 collaborating cancer centers, same protocols, data & methods
  - Uses genomic profiling/predictive model to match patients to one of five experimental drugs

- Potential benefits: faster, smaller trials; high patient enrollment (3%→ 100%); enormous increase in cancer genomics data; automatic CER; refined profiling & predictive models; precision medicine

NIH Upgrades, 2015-2016

- The Commons: databases from publicly-supported studies go into a system of "conformant" data clouds for open science
  - Research funding to include funds for curating and archiving databases; "vouchers" allow researchers to access, analyze & use the data

- BD2K centers of excellence, national reference databases for genomics-enabled science, GA4GH, NLM-Medline

FDA Upgrades, 2015-2016

- A new technologies RL system for genetically-informed therapies
  - National registries, standardized data, "coverage with evidence development" for study period, e.g. first 3 years post-approval
- A continuous trials system for major diseases, experimental protocols become a new study arm, automatically producing CER data
- FDA scientific and clinical databases publicly available in a Commons cloud
- Oversight of predictive models for informing public about benefits and risks (beyond patient package inserts)

PCORI Upgrades, 2015-2016

- Expand PCORnet capabilities in collaboration with NIH, FDA, others
- Identify "patient-centered" research needs for genomics-enabled health care. Propose national work plans for who is accountable for answering the priority questions, by when
- Fund CER priority studies for genomics-enabled personalized health care & precision medicine
- Engage patient groups, professional societies, health plans, hospital groups, ACOs, others for collaborative funding of CER research, using fast, affordable RL system vs NIH 7 yrs
- Develop predictive models for patients and physicians to compare benefits and risks of options

CDC Upgrades, 2015-2016

- Expand and enrich a genomics-enabled research system for epidemiology & public health science, e.g. HuGENet

- Enhance a real-time national tracking & RL network for public health emergencies, e.g. ESPnet (Mass)

CMS Upgrades, 2015-2016

- Support a genetics-enabled RL cancer system for Medicare and Medicaid
  - All Medicare/Medicaid cancer data (Genetics + EHR, claims, collected and reported to national "cloud" system (privacy protected). Coverage for genetics sequencing & analysis, predictive services
  - Use $10 B Innovation Center to test & advance "best practices" in genomics-enabled cancer care. Use "pay for performance" to accelerate quality
  - Collaborate with ASCO's RL cancer system

CMS Upgrades, 2015-2016

- With FDA, use "coverage with evidence development" to support a new technologies RL system for genomics-enabled medicine, e.g. new cancer treatments
- Bring Medicare and Medicaid populations into a genetics-enabled RL system, e.g. special needs children, multiple chronic illness patients, Alzheimer's
- Use the RL cancer system as a model for advancing genetics-enabled healthcare
- Support development and use of predictive models, clinical decision support

Concluding Observations

- The investments in a RL health system (pre-genomics) have created foundations and opportunities for a genomics-enabled RL health system

- Failure to act may lead to chaos, massive amounts of genetics-health data paid for by health system but not available for learning, lost opportunities

- A genetics-enabled RL health system will need collaboration among government agencies and the health sector