A Vision For A National Patient-Centered Research Network

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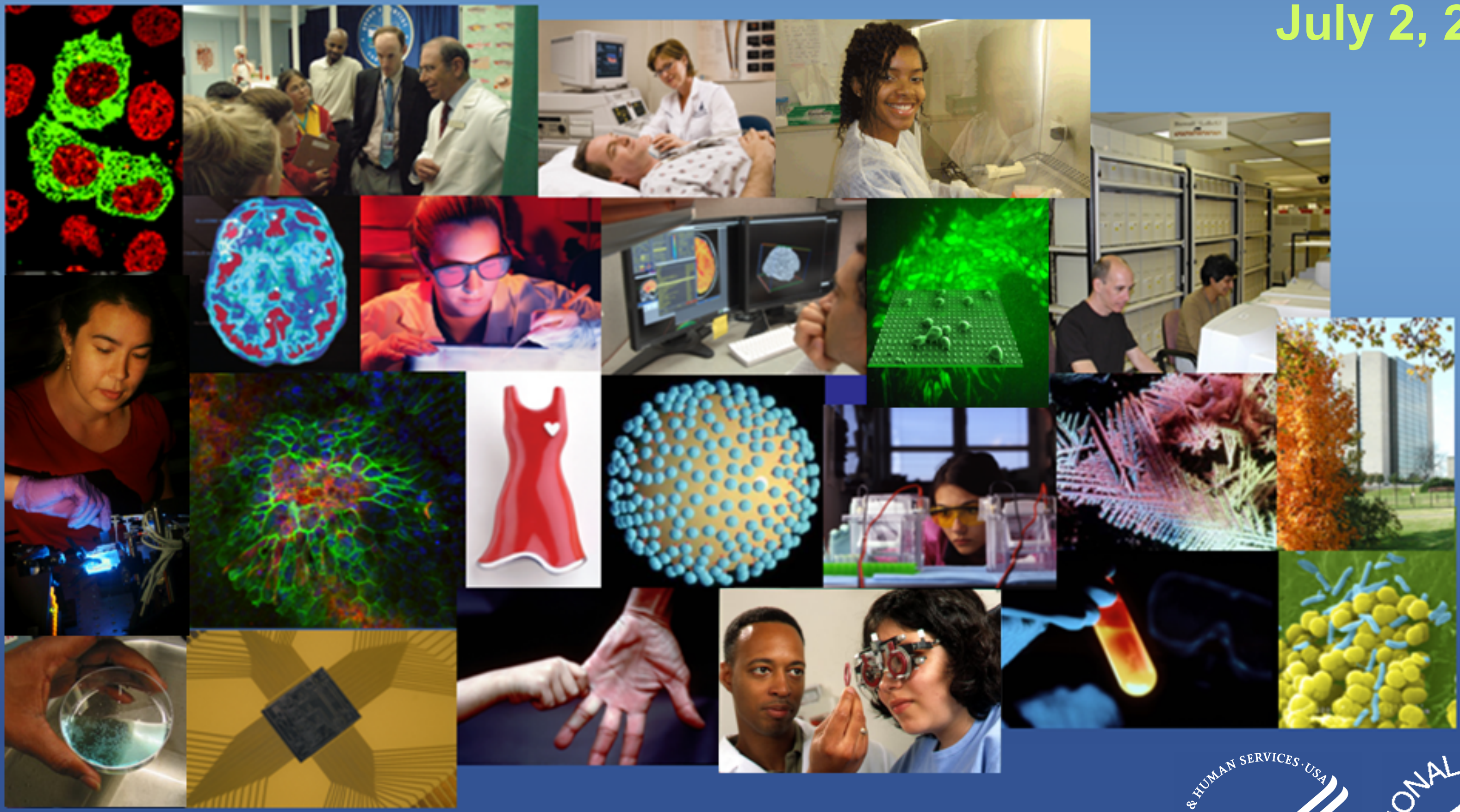


DEPARTMENT OF HEALTH & HUMAN SERVICES · USA

Why is it so hard to do effective and efficient clinical research?

- Few pre-existing cohorts of substantial size
- Even fewer with broad disease relevance
- Absence of longitudinal follow up
- Paper medical records the norm until very recently
- Lack of population diversity
- Vexing consent issues
- Multiple IRBs
- Privacy and confidentiality challenges
- Chronic difficulty achieving enrollment goals
- Limited data access
- Heavy costs of start-up and shut-down

Imagine …

A National Patient-Centered Research Network

- Bringing together 20–30 million covered lives, with
  - Good representation of gender, geographic, ethnic, age, educational level, and socioeconomic diversity
  - Broad opt-in consents from 80 - 90% of participants
  - Longitudinal follow up over many years
- Offering a stable research infrastructure
  - Including trained personnel in each of the participating health services organizations
  - Making it possible to run protocols with low marginal cost

Imagine …
A National Patient-Centered Research Network

- Drawing on electronic health records (EHR) for all patients, with
  - Interoperability across all sites
  - Meaningful use for research purposes
- An efficient Biobank
- Promoting data access policies that provide for broad research use but protect privacy and confidentiality
- Providing governance with extensive patient participation in decision making

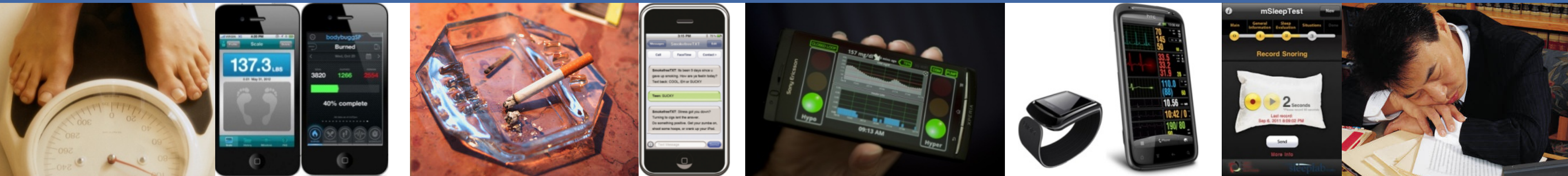
What Could We Do With a National Patient-Centered Research Network?

- Rapidly design and implement observational trials
  - At very low cost
- Quickly and affordably conduct randomized studies
  - Using individual or cluster design
  - In diverse populations and real-world practice settings
- Significantly reduce usual expenses associated with start-up and shut-down of clinical research studies

Examples of Studies That Could Be Facilitated By A National Patient-Centered Research Network

mHealth Applications

- Prevention
  - Monitor obesity management programs
  - Assess sleep apnea at home
  - Support tobacco cessation
- Chronic disease management
  - Continuous glucose monitoring for diabetes
  - Monitor ambulatory blood pressure in real time
  - Continuous EKG monitoring for arrhythmias
- National patient-centered research network would ...
  - Provide a real world laboratory for assessing whether mHealth-based interventions actually improve outcomes



Examples of Studies That Could Be Facilitated By A National Patient-Centered Research Network

Low Back Pain (LBP)

- Most acute LBP resolves with conservative management
- But about 20% of LBP becomes chronic
  - Common treatments: medications–physical therapy–chiropractic/manipulative therapy–acupuncture–surgery
  - Complex fusions for spinal stenosis up 15x in recent decades
- National patient-centered research network would ...
  provide large # of participants; longitudinal follow-up to
  - Determine how to prevent acute LBP from progressing to chronic
  - Compare risks and benefits of common treatments
  - Discern appropriate use of lumbar imaging for evaluation

Examples of Studies That Could Be Facilitated By A National Patient-Centered Research Network

Large-Scale Pharmacogenomics

- Example -- Clopidogrel (Plavix): powerful antiplatelet drug used in patients at risk for heart attack, stroke
  - CYP2C19 genotype may identify decreased responsiveness
  - FDA added black box warning – but other research has raised doubts about clinical importance of CYP2CI9 genotype
- National patient-centered research network would … facilitate trials to examine conflicting data
  - Large-scale, rapid-fire clinical trial of patients with acute coronary syndrome, recent stroke, recent placement of drug-eluting stent
    - Randomized trial (individual or cluster)
    - Only short-term (e.g. 6 to 12-month) follow-up needed
  - Model could be applied to other pharmacogenomic questions

By synchronizing with EHR data, one could do large definitive trials quickly at low cost

What Could Go Wrong?

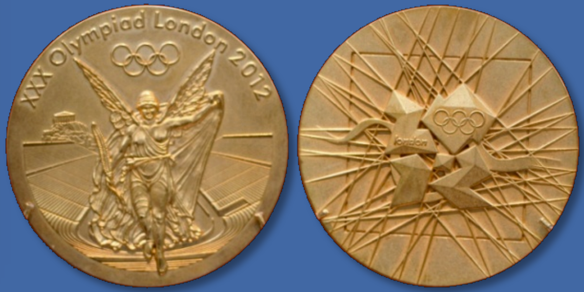
- EHRs won’t turn out to be that useful for research (hey, we’d better solve that one at this meeting!)
- Business managers of health services organizations will perceive a conflict between health care delivery and research
- Patients (especially underrepresented groups) will be unwilling to participate
- The network will be too large to evolve when it needs to, and will become quickly ossified
- An entitlement will be created – once a node in the network is supported, it can never be terminated

Why Now?

- For the first time in the U.S., health services organizations with EHRs have reached the point of making this network feasible on a large scale
- Scientific opportunities and the urgency of getting answers to clinical questions have never been greater
- If we are ever to engage a larger proportion of the American public in medical research, we need to come to them – in partnership
- General feasibility has been demonstrated through modest prior efforts (e.g. HMORN, eMERGE, etc.)
- **PCORI has arrived on the scene** – and successful establishment of this Network, potentially with NIH and AHRQ as partners, could be PCORI's most significant contribution and enduring legacy

2012: An Olympic Year

Patient-Centered Outcomes Research Works Best as a Team Sport



So let’s go for the gold!