What’s Next: People-Powered Knowledge Generation from Digital Data

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January 13, 2017
Founder, Hugo
Personal health information platform.
Social enterprise to empower people with their health-related data.

Others:
PCORI Board; United HealthCare Scientific Advisory Committee; Grant from JNJ to distribute clinical trial data; grant from Medtronic, in collaboration with FDA, to improve device surveillance; contracts with CMS to develop performance measures; contract with Blue Cross for technology assessment; AHRQ and NIH grants
Problem Statement

- The current knowledge generation enterprise is inadequate to keep up with the information needs of patients, clinicians, health systems, regulators, and others.
The answer is unlikely to be more $$

US Funding for Medical Research by Source, 1994-2012

Clinical research dominates $$

Pharmaceutical Industry Medical Research Funding by Phase of Research, 2004-2011

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<thead>
<tr>
<th></th>
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<tbody>
<tr>
<td>Uncategorized</td>
<td>4.2 (9)</td>
<td>1.7 (3)</td>
<td>-11.9</td>
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<tr>
<td>Phase 4</td>
<td>6.4 (13)</td>
<td>4.8 (10)</td>
<td>-3.9</td>
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<tr>
<td>Approval</td>
<td>4.5 (9)</td>
<td>4.1 (8)</td>
<td>-1.2</td>
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<tr>
<td>Phase 3</td>
<td>12.6 (26)</td>
<td>17.6 (36)</td>
<td>4.9</td>
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<tr>
<td>Phase 2</td>
<td>4.9 (10)</td>
<td>6.2 (13)</td>
<td>3.3</td>
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<tr>
<td>Phase 1</td>
<td>3.2 (7)</td>
<td>4.3 (9)</td>
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<td>Prehuman/preclinical</td>
<td>12.5 (26)</td>
<td>10.6 (22)</td>
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<tr>
<td>Overall</td>
<td>48.3</td>
<td>49.3</td>
<td>0.3</td>
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Inefficiencies

Screening
Enrollment
Retention
Follow-up
The Result
Another Issue: Time
Cardiovascular Safety of Celecoxib, Naproxen, or Ibuprofen for Arthritis

Steven E. Nissen, M.D., Neville D. Yeomans, M.D., Daniel H. Solomon, M.D., M.P.H., Thomas F. Lüscher, M.D., Peter Libby, M.D., M. Elaine Husni, M.D., David Y. Graham, M.D., Jeffrey S. Borer, M.D., Lisa M. Wisniewski, R.N., Katherine E. Wolski, M.P.H., Qiuqing Wang, M.S., Venu Menon, M.D., Frank Ruschitzka, M.D., Michael Gaffney, Ph.D., Bruce Beckerman, M.D., Manuela F. Berger, M.D., Weihang Bao, Ph.D., and A. Michael Lincoff, M.D., for the PRECISION Trial Investigators*
Can we break through the constraints?

\[
\begin{align*}
\text{Better} \land \text{Faster} & \rightarrow \lnot \text{Cheaper} \\
\text{Faster} \land \text{Cheaper} & \rightarrow \lnot \text{Better} \\
\text{Cheaper} \land \text{Better} & \rightarrow \lnot \text{Faster}
\end{align*}
\]
The Goal

BETTER
FASTER
CHEAPER
Kingdom of Research
Kingdom of Research

- **Subject:** A person who is under the dominion or rule of a sovereign.
- Often does not receive results.
- Often does not feel part of the team.
- Often does not receive thanks.
My own journey...
My own journey...
The Basic Idea...

• People as partners instead of subjects could unleash the means to achieve better, faster, cheaper... more impactful... knowledge generation.
Lessons Learned
Time is Right

Basic EHR adoption increased while certified EHR adoption remained high

Figure 1: Percent of non-Federal acute care hospitals with adoption of at least a Basic EHR with notes system and possession of a certified EHR: 2008-2015

- **Certified EHR**
  - 9.4%
  - 12.2%
  - 15.6%
  - 27.6%
  - 44.4%
  - 59.4%
  - 75.5%
  - 96.9%
  - 96%

- **Basic EHR**
  - 71.9%
  - 85.2%
  - 94%
  - 96.9%
  - 83.8%
Time is Right
The study is open: Participants are now recruiting investigators

Recent events inspire optimism that a new age is dawning, one in which lay people have an active role in advancing biomedical research and health care delivery. Two ongoing experiments will deeply involve the public in these endeavors: the U.S. Precision Medicine Initiative (PMI) and the National Patient-Centered Clinical Research Network (PCORnet). PCORnet has already launched 20 patient-powered research networks designed to be led and animated by people who have an affinity with one another because of either shared disease, geography, experience, or identity (1). When U.S. President Barack Obama announced the PMI, he stated emphatically that people would be, not patients or even participants, but rather, partners in clinical research (2, 3). In the hours and days that followed, Francis Collins, director of the U.S. National Institutes of Health (NIH), reiterated this view, using words such as “participant” and “partner” when referring to people involved in clinical research (1). As a veteran citizen scientist and patient advocate (4), I was moved to tears to hear such proclamations from people other than my passionate fellow advocates. However, PCORnet’s efforts and PMI’s endeavor to enroll a million people—called All of Us—will spur the advancements we seek only if we, the people, take advantage of these unprecedented opportunities and act with boldness to overcome myriad misaligned incentives, business interests, and general inertia against change.

Building the Wei: True participation

Thousands of individuals affected by common and rare conditions indicate that they do not wish to be referred to as patients. “Patient” describes a person sitting on the exam table in a flimsy johnie—the epitome of information and power asymmetry. This is not how the millions of people living with chronic or genetic diseases view themselves. We are also not subjects—that is, “a person or thing being studied.” Words matter, and using the word “participant” recognizes the actual engagement necessary to revolutionize clinical research and the resulting health interventions. Some investigators think that the term “participant” is a misnomer and should not be used. If that is so, and people with illnesses are not participants or partners in clinical research, then it is time to change that.

Sometimes investigators convey with enthusiasm that advocates are “invited to the table.” However, an invitation to the table implies that it is owned or managed by the investigators and not by all stakeholders. Participants want not only to be invited to the table but also to design and host the meal with other stakeholders. There is a great deal of “us and them” language in biomedical research. Investigators point to “those patients,” and activists complain about “those investigators.” Clinicians are often left out of the process completely. When these roles are considered dichotomous and separate instead of part of a continuum, it is difficult to create authentic partnerships.

Participants have a place throughout the research continuum, including the proposal and prioritization of research questions, study design, engagement of study participants and their recruitment and retention, conduct of research and data analysis, and implementation and dissemination of results and, often, individuals’ own data. However, if we intend to engage a large and diverse array of people in clinical research, participation has to be made as frictionless as possible by creating mechanisms in communities where people live, work, and play, with community representatives leading the way. In addition, the research conducted must have relevance to the engaged parties by addressing questions that arise from communities of participants. If a study is built on the needs of individuals, families, and communities, then the results of research must be transparent and tangible—traits that run counter to the current culture. Researchers often do not return even the published results to the participants, let alone a lay summary or other accessible communication. If an effective intervention results from a clinical study, the process can take more than 10 years for the new intervention to be integrated into clinical practice (5). The new U.S. national efforts, particularly PCORnet, with its intention to establish a system that learns from the real-world experience of clinicians and patients to improve care through comparative effectiveness research, present an opportunity to change this.

Consumers hope that the improvements accelerated by PMI and PCORnet will go beyond diagnostics, therapies, and treatments to include more efficient systems. For example, if an auxiliary benefit of improvement of health information exchange is that it is easier to transfer one’s child’s immunization record to their school or camp or to connect one’s electronic health record to a pharmacy to get a flu vaccine, then one might be more inclined to participate in these national efforts. Proximal benefits of big data in health and biomedical research must accrue and be felt by individuals, as they are in other consumer scenarios (such as the traffic app Waze and the music app Spotify).
Time is Right

PERSPECTIVE

PATIENT ENGAGEMENT
On the path to a science of patient input

Margaret Anderson* and K. Kimberly McCleary*

It is early days in the creation of a science of patient input. Participants are establishing rigorous methods to better integrate patient perspectives, needs, and priorities throughout biomedical and bioengineering R&D and care delivery to patients. To assess progress and unmet needs, FosterCore tracked more than 70 collaborative initiatives clustered in six categories that are defining and shaping this developing field. No longer is patient engagement a fanciful notion as it was at the start of its journey in 2005, and the rush of activity is welcome and vital.

In the 21st century, market research is a business imperative for most industries.1, 2013—decades after Steve Jobs famously said, “A lot of times, people don’t know what they want until you show it to them”—Apple started a market research group that sends anonymous surveys to invited users to find out exactly what they want from their devices. In January 2016, IBM formally launched its consumer innovation process to shift its culture to focus on users’ needs.1 Health care and the research and development (R&D) of biomedical products have lagged behind other technology sectors in moving toward consumer-centered practice. Now, as a result of multiple cultural influences and pragmatic factors, the mindset of these stakeholders is changing, and the patient’s voice is expanding.2, 3 Momentum is building to incorporate patient preferences into the biomedical R&D system so that products and services better align with patient needs, improve individual and public health, and reduce time and spending on unproductive care.

With a broad network of stakeholders—patient organizations, industry, academic, government, and funding agencies—FosterCore has a distinct vantage point into the landscape of new patient-centered activities; such information is crucial to the creation of a new field. The science of patient input. The goals of this new field are to develop rigorous methods so as to better integrate patient perspectives, needs, and priorities across the translational research continuum. In this Perspective, we summarize and encourage broad use of resources that are already available, and we capture a baseline assessment to benchmark growth and identify areas of unmet need. We don’t want a minute wasted on duplicating efforts.

WHO’S ON FIRST?
Through an environmental scan, we tracked more than 70 collaborative initiatives, clustered in six categories, that are defining and shaping patient-centered practice and policy (Tables 1 and 2). Within these 70 initiatives, nearly 40 discrete supporting entities are assembling resources, providing direction, and tracking milestones. Each entity approaches this field from a different vantage point, which is what makes the effort so promising. It is natural—and essential—that the work required to create the field of patient input be performed through strong collaborations composed of highly interactive, diverse organizations.

FORMING SOLID PLATFORMS: FRAMEWORKS AND MODELS
Some of the first formal efforts to outline the science of patient input borrow from software development, the use of frameworks to provide a logical structure for organizing information, identifying sources of the information, and suggesting ways it might be used and viewed by distinct parties.2, 3 Frameworks serve different purposes, with varied approaches and audiences. It is important to be familiar with those frameworks because they lay the groundwork for much of the ongoing and future work in this space.

The Clinical Trials Transformation Initiative (CTTI) created perhaps the most recognizable tool, and its work has become a gold standard. CTTI is a public-private partnership supported by the U.S. Food and Drug Administration (FDA) and members of pharmaceutical companies and patient organizations and has popularized a visual chart or framework that identifies points at which clinical trial sponsors and regulators might engage patients along the R&D continuum for pharmaceuticals.4 A companion framework for medical devices was developed by another public-private partnership, the Medical Device Innovation Consortium (MDIC), which built detailed considerations into an FDA Center for Devices and Radiological Health (CDRH) diagram of places in the total product life cycle of medical devices at which patient preferences information might enhance product development.5

The Patient-Centered Outcomes Research Institute (PCORI) requires that all of its funded investigators partner with patients from the beginning of the application process through completion of the study and dissemination of its results. To guide formation of meaningful partnerships with patients, PCORI developed a Patient Engagement Rubric (6) and a compensation framework (7) that now guide applicants, reviewers, and award recipients at every step. The engagement principles outlined in the rubric—reciprocal relationships, colouring partnership, trust, transparency, and honesty—have become the essential characteristics of patient partnerships in R&D and health care delivery. These initiatives, like most of the others identified here, use the U.S. regulatory system as a foundation. Composed of industry and patient groups, the Patient-Focused Medicines Development partnership is leading an effort to develop a comprehensive global framework for patient engagement.

Recently, we have seen a surge in frameworks being used by a number of organizations to help define the value of certain drugs and medical products for insurance coverage decisions. Frameworks assessing the value of medicines have been put forward by the American Society for Clinical Oncology, Institute for Clinical and Economic Review, National Comprehensive Cancer Network, and others; however, most efforts to date have

The power of the science of patient input lies in the data, but two key challenges are locating sources of relevant and robust patient data and determining how best to apply them.
Patient engagement offers the promise of advancing more personal and efficacious medical products faster than the typical ~15-year discovery-to-market timeline.
The time is right for individuals to become active participants in clinical trials, to create a movement to build the commons with such data, and to actively share them.
"Second opinions matter. Information prevents redoing." —Kathryn B., California

Get the information you need to better manage your health, or care for loved ones. With online access and communication tools, patients can ask questions, share concerns, and provide pertinent information to their providers at their convenience – at night, over the weekend, even on a holiday.

Learn WHY You Should Request Your Health Data
“Tonight I’m launching a new Precision Medicine Initiative to bring us closer to curing diseases like cancer and diabetes.

And to give us all access to the personalized information we need to keep ourselves and our families healthier.”

President Barack Obama
2015 State of the Union Address  |  January 20, 2015
Precision Medicine Initiative

Recommendation 5.10: PMI should support development and evaluation of tools that enable individuals to acquire, transmit, and continuously update their EHR data to the PMI cohort from multiple provider organizations.
NIH and ONC announce Sync for Science to enable patients to donate data to Precision Medicine Initiative

EHR makers including Allscripts, athenahealth, Cerner, drchrono, Epic and McKesson said they will embrace open specs including S4S APIs and FHIR to connect research apps to electronic health records software.
CONSUMER-MEDIATED EXCHANGE
Consumer-mediated exchange provides patients with access to their health information, allowing them to manage their health care online in a similar fashion to how they might manage their finances through online banking. When in control of their own health information, patients can actively participate in their care coordination by:

- Providing other providers with their health information
- Identifying and correcting wrong or missing health information
- Identifying and correcting incorrect billing information
- Tracking and monitoring their own health
- Learning more about the benefits of consumer-mediated exchange.
Sync for Science

• Develop methods to facilitate individually-controlled clinical data donations to the PMI Cohort.

• Accelerate and guide the national ecosystem for patient-mediated data access through APIs.

I believe that the most important requirement for the new knowledge network envisaged by the “Precision Medicine” report is that it be driven by patients.
For the benefits of digital medicine to be fully realized, we need not only to find a shared home for personal health data but also to give individuals the right to own them.
Time is Right

Unpatients—why patients should own their medical data

Leonard J Kish & Eric J Topol

For the benefits of digital medicine to be fully realized, we need not only to find a shared home for personal health data but also to give individuals the right to own them.

10 often said that data are the new oil, or the new gold, but they are much more like a New World distinguishable, at least in part, by new maps. Indeed, the planet is becoming a new world of relationships, descriptive data and information flows. There are now over 3.5 billion registered on Facebook (Menlo Park, CA, USA) and a Swedish start-up called Traude (Stockholm) has assembled a phone directory of +1 billion human beings, with the intent of having every person on the planet in its directory. Social graphs that depict relationships between people and organizations are the new maps of a connected humanity—maps of people, organizations and many other dimensions of data that reveal how things are related. As recent examples, we’ve seen months of activity data from 22 million Americans and over 230 million nights of sleep data2. Such global data efforts have not yet reached medicine, but their arrival is both inevitable and imminent.

In parallel to these social graphs and global data sets, there is an unprecedented and rapidly developing capability to digitize a human being. Creating the equivalent of a Google medical map or the medical essence of an individual would integrate multiple layers of pharmacokinetic, physiological, anatomic, biologic and environmental information. Just about everything that makes a human tick can now be quantified like never before by means of sensors, sequencing, laboratory tests and scans. Recently, it has been shown that a single drop of blood could be used to reveal the volume of an individual’s exposure, uncovering not only which viruses the person was exposed to but when5, for just $25. This exemplifies our newfound and accelerated ability to capture and analyze human data, which most of us could not even fathom a few years ago.

Such medically relevant data from an individual is not a one-off gathering. Rather than simply falling under the definition of big data, the data can be, and often are, obtained longitudinally, over the course of a lifetime, fulfilling the idea of long data. Furthermore, such data are contextualized, often in real time in a personal world. Enabled by mobile technology, an external ‘wisdom of the body’ (in contrast to Walter Cannon’s classically described autoregulation, homeostasis, in his book Wisdom of the Body6) can be developed with feedback of integrated data to the individual (Fig. 1). Soon, enough, virtual medical assistants will emerge that incorporate machine learning about a person and could include everything medical, as well as the person’s lifestyle, behaviors, social network, finances and how they are interrelated. Quickly, one can imagine that, just from a watch that collects blood pressure with every heartbeat, analytics of data can be sent and be generated on an individual basis. Much of the data will fall into the category of patient-generated data and will ultimately eclipse the amount of data captured today in clinical electronic medical records.

Yet currently there is no ‘home’ for such data over time, at either the individual or the population level. Although there are early proposals for how some of it could be bundled with other medical electronic record, it seems unlikely this will occur. In the United States at least, given the landscape of balkanized health records and multiple providers of care for each person. Imaginarily, we’re looking at the prospect of a new, high-definition picture of individual human beings, and at the same time for that personal data to be homeless, dispersed and inaccessible. Where the data live will determine the maps we can create and the directions we can go in with health, both as individuals and as a society. We propose here that the key step to liberating personal health data and realizing their true potential in human research and clinical practice the provision of data management systems that give individuals the right to own their own data. The technological advances developed for evolving digital currency systems, which allow individuals to hold and secure digital assets without a central authority, are being used to create new digital property systems, including personal medical data property. Whatever the means, it is critical for individuals to own or at least control ownership of their data in order for the real benefits of a new, data-driven high-definition era of medicine to be realized.

This is a unique moment where we may be able to provide for personal control and, at the same time, create a global knowledge medical resource.
Recommendation D:

A National Cancer Data Ecosystem for Sharing and Analysis

The enormous volume of data being generated by cancer researchers, clinicians and patients today requires a national infrastructure to share, combine and analyze those data in an easy-to-access format.
Central tenet of National Cancer Data Ecosystem is enabling the public, including all cancer patients and others, to directly contribute their data, or to request a healthcare provider do so on their behalf, for scientific research.
Patients were invited to use portals at all their providers’ practices (a solution that caused a condition sometimes called “hyperportalosis”).
Dear John: *I* want to download my records.

Dear Dr. Halamka,

I want to download all my data from my 14 years as a patient at Beth Israel Deaconess Medical Center. What button should I push?

In June you said on your blog (left, top) and on MedCity News that no patient has ever asked for that, but your tech support says you don’t have a way to do it (see red outline).

Tech Support said I should call Medical Records. I did, and they said they can’t deliver things electronically. So where is the link you say nobody has ever used?

My affective experience of trying to download all my health data from Beth Israel Deaconess (let alone everywhere else). Rendering courtesy of Mac's Photobooth app, thank you - "more accurate than a mere photograph could be"
Business models that depend on data as proprietary assets.
My Commitment

• I am committed to tilting the balance of power in health care
My Commitment

• I am committed to tilting the balance of power in health care – and it starts with giving people the ability to obtain and share their health-related data – to do it easily, automatically and securely.
• Solution must protect privacy and security while enabling each person’s access to his or her health-related data and proving opportunities to leverage it.
Approach

• People first
• Mobile first
• Excellence in UI/UX
• Stringent privacy policies and security
• Minimize dependence on data holders
How Might It Work?

Study consent and requirements developed
Study listed on platform
People enroll and donate their data
Synchronized with providers (S4S/S4H)
Study results returned to people
Research Complete

Patient-Centric Research
In the preamble to the original Privacy Rule in 2000, HHS cited a “well-established principle” that an individual (or designated personal representative) should have “access rights to the data and information in his or her health record”


From Barbara Evans... Barbarians at the Gate: Consumer-Driven Health Data Commons and the Transformation of Citizen Science
Accessing and obtaining copies of one’s health information for one’s own purpose is a right, not a privilege which is fundamental to your ability to participate in our health care system.
The right extends to a broad array of information (i.e. lab results, images, prescriptions, notes), as well as to data holders (i.e. doctors, hospitals, health plans and providers)
The Lever: Patient Rights

...get either a paper or, if records are kept electronically, an electronic copy of your records; Per page charges do not apply when the individual is requesting a copy of information maintained electronically.

Technical Advances

- **FHIR (Fast Healthcare Interoperability Resources)**
  - Data Standard (from HL7 group)

- **Oath 2.0**
  - Security authorization standard
  - Helps delegate permissions around tasks
After all, good notes can accurately pull together the patients’ stories, assessing their bodies, their minds, and a variety of intermingling needs. The Institute of Medicine agreed, urging society to see the note as a living, interactive document shared between patients and providers.

https://www.statnews.com/2016/08/19/hipaa-medical-records-patients-doctors/
Testing Assumptions

Collect.

Organize.

Share.
Ideally, PRO collection would be enabled for patients on their own smart devices in flexible user-configurable formats, perhaps through text messages, automated telephone systems, or downloadable apps.
Engage.

A new way to communicate.

Hugo provides a method for delivering customized surveys directly to users’ mobile devices. This new channel of communication can collect valuable patient reported outcome data.
Is Digital Health Finally About to Turn All That Hype Into Results?

by Sy Mukherjee  
@the_sy_guy  
MAY 4, 2016, 5:55 PM EDT
Re-Architect Clinical Research

Replace expensive, slow, brute force approach...
People-Powered Data Partnership

People become stewards of their own data by using tools that enable easy and secure acquisition and organization of their health information — including medical records, and data they may generate from surveys or connected devices — so that they may become active partners in pursuit of better health for themselves – and for those that follow them.
Thank you.

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January 13, 2017