

Bits and Bytes: Making Patient Information Available and Meaningful to Researchers

September 16, 2011

1:00-2:30 pm (Eastern)

Part of the FasterCures TRAIN Webinar Series

Today's Webinar Speakers



Carolyn Compton, M.D., Ph.D.
Director, Office of Biorepositories and Biospecimen Research at
the National Cancer Institute



David Blumenthal, M.D., M.P.P.
Samuel O. Thier Professor of Medicine and Health Policy,
Harvard Medical School, and former National Coordinator for
Health Information Technology



Sharon F. Terry, President and CEO, Genetic Alliance



MODERATOR:

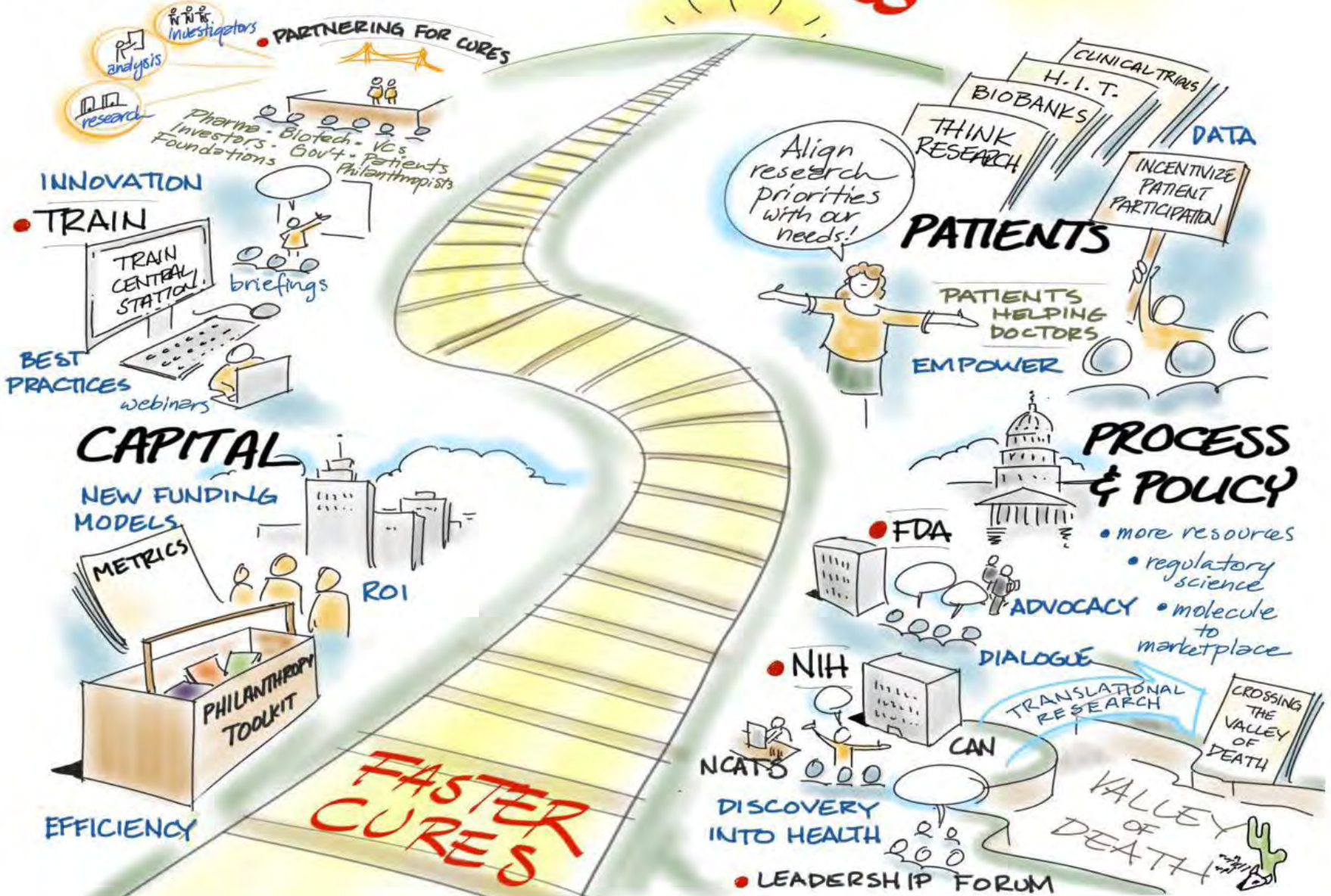
Margaret Anderson, Executive Director, *FasterCures*



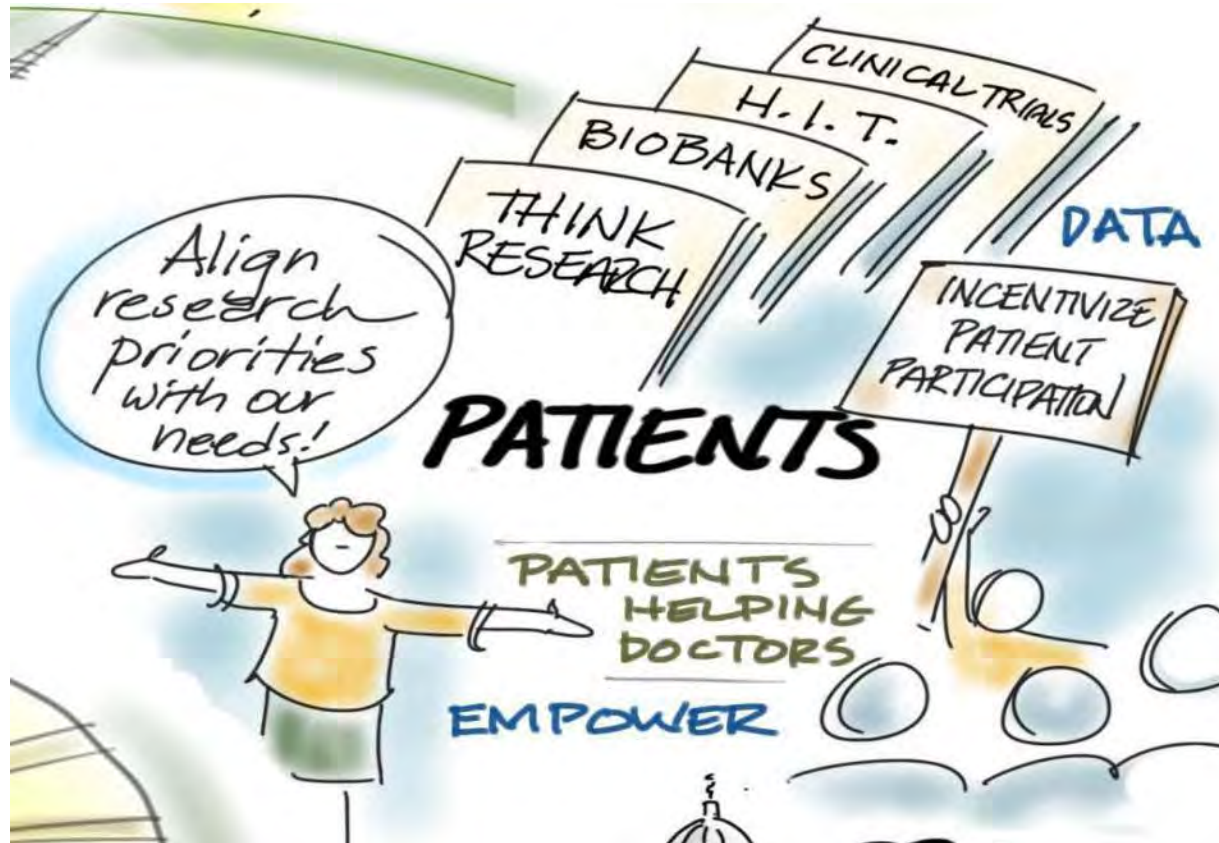
**It's not just our name.
It's our mission.**

COLLABORATION

MEDICAL PROGRESS



Strategic Objective: Increase patient engagement in research, optimize use of patient data



FasterCures' PHD (Patients Helping Doctors) identifies ways to better engage patients in clinical research

Improved biospecimen collection

Integrating research into health IT

More effective clinical trial designs



JUST RELEASED TODAY

FasterCures

Banking on Trust

The Future of Research with Human Biological Materials

FasterCures

Still Thinking Research

Strategies to Advance the Use of Electronic Health Records to Bridge Patient Care and Research

Banking on Trust

The Future of Research with Human Biological Materials

- We found that the key to achieving success in biobanking lies in building the foundations of trust among patients, advocacy groups, healthcare providers, and researchers.
- Those who are collecting and using samples for research must earn the trust of those who donate their samples.
- This paper describes a number of ways to earn that trust.

Some Principles for Progress in Biobanking



- The public needs to understand that medical progress is dependent on wide participation in research.
- Craft a consent (or opt out) mechanism that outlines a fairly broad scope of research.
- Remember that consent is a voluntary process.
- Biobank governance and leadership must continue to engage in public consultation and education.
- Resources are needed to support the careful collection, storage, and maintenance of specimens.



Carolyn Compton, M.D., Ph.D.
Director, Office of Biorepositories and
Biospecimen Research at the National
Cancer Institute

Perspectives on biobanking

Molecular Data



Diagnosis / Therapy

DETERMINES QUALITY HERE



PERSONALIZED CANCER CARE



Biospecimen Analysis

Biospecimen Collection

QUALITY HERE



Biospecimen Processing and Banking

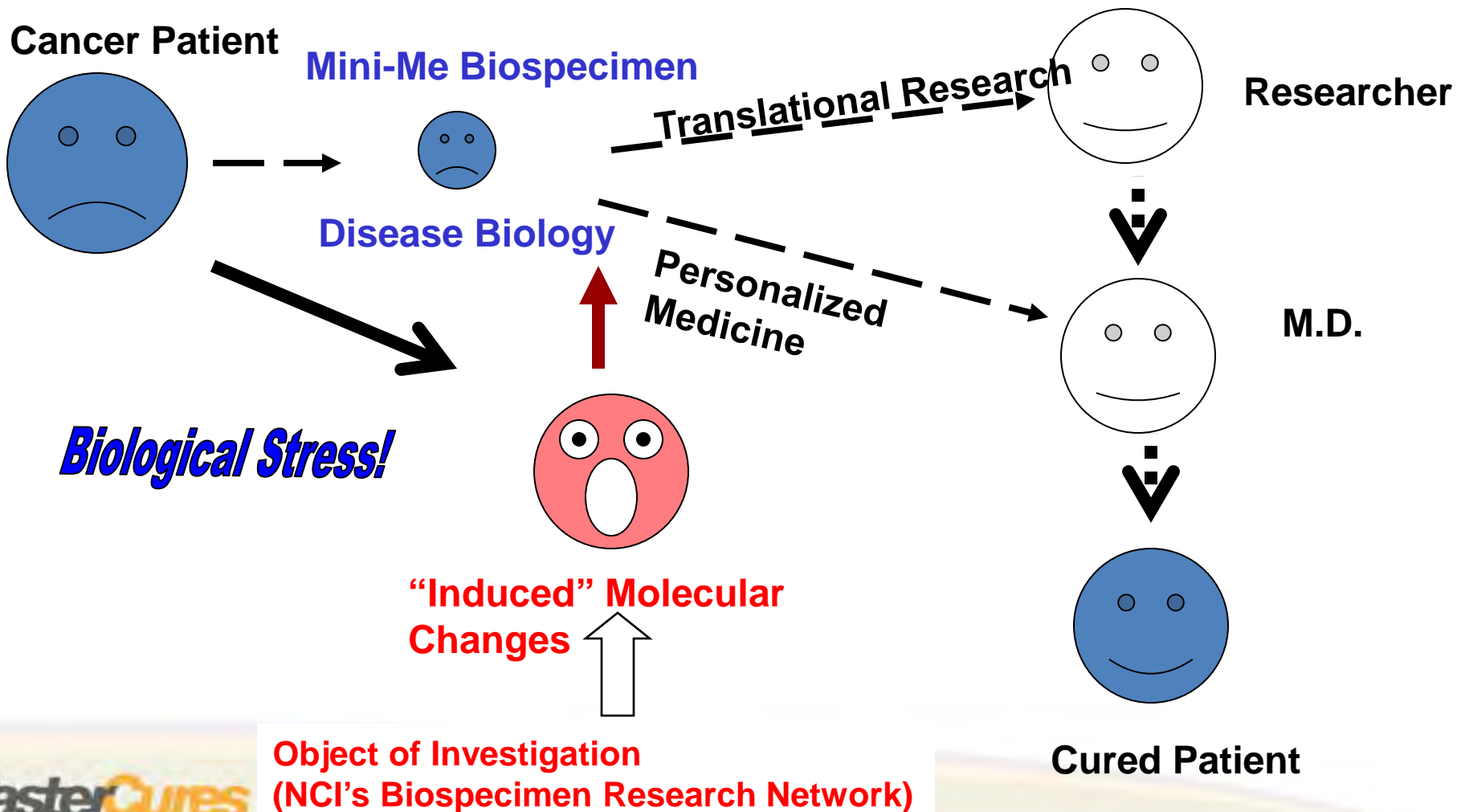
Perspectives on biobanking

- Biospecimens are a powerful and rich source of essential information.
- The technological power to reveal and understand the molecular complexity of biospecimens is greater than ever before, providing new windows of opportunity for diagnostics and therapies
- Cautionary note: garbage-in, garbage-out . Specimen quality is key. The technological capacity exists to produce low-quality data from low-quality analytes with unprecedented efficiency, and we now have the ability to get the wrong answers with unprecedented speed.
- Specimen annotation is essential. The value of biospecimens in research also is linked to the type, amount, and quality of data associated with the samples.
- Human biospecimen resources have a heavy responsibility, as honest brokers, to maintain the trust of patients who have donated their samples and data.

Perspectives on biobanking: Needs assessment

- Need for evidence-based technical standards that assure fit-for purpose (technology appropriate) specimens for today's technologies.
- Need for implementation of best practices created by authoritative sources: challenged by cost and lack of reimbursements sources, training and education of personnel, regulation and accreditation, and reimbursement codes to pay for the professional time and expertise needed to support the labor-intensive front-loaded operations
- Need for investment in biospecimen science to create the evidence base for data-driven standards.
- Need to address the harmonization of non-technical (social, political, and legal challenges) as well as the technical needs related to specimen and data sharing

Understanding the Biology of Biospecimens: The Goal of Biospecimen Science



Biospecimen Lifecycle: Pre-analytical Factors Affect Molecular Composition and Integrity

Specimen is **viable** and biologically reactive

Molecular composition subject to further alteration/degradation

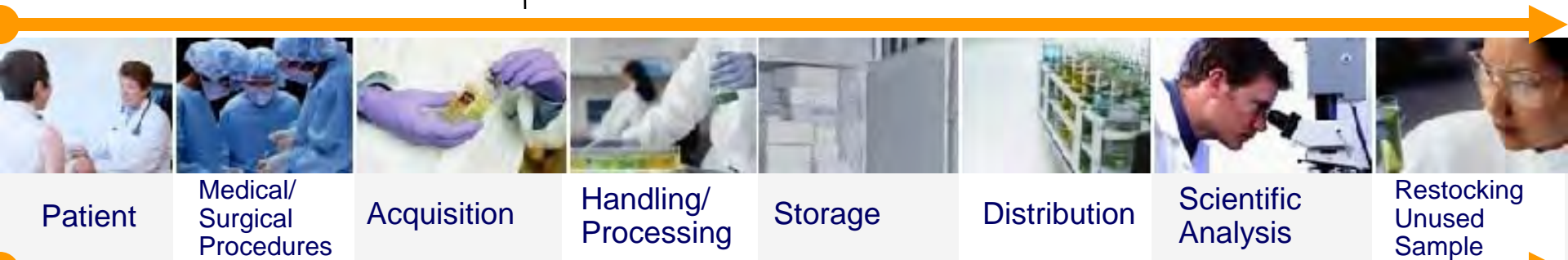
Factors (examples):

- **Antibiotics**
- **Other drugs**
- **Type of anesthesia**
- **Duration of anesthesia**
- **Arterial clamp time**

Time 0

Factors (examples):

- **Time at room temperature**
- **Temperature of room**
- **Type of fixative**
- **Time in fixative**
- **Rate of freezing**
- **Size of aliquots**



Patient

Medical/
Surgical
Procedures

Acquisition

Handling/
Processing

Storage

Distribution

Scientific
Analysis

Restocking
Unused
Sample

Pre-acquisition

Post-acquisition

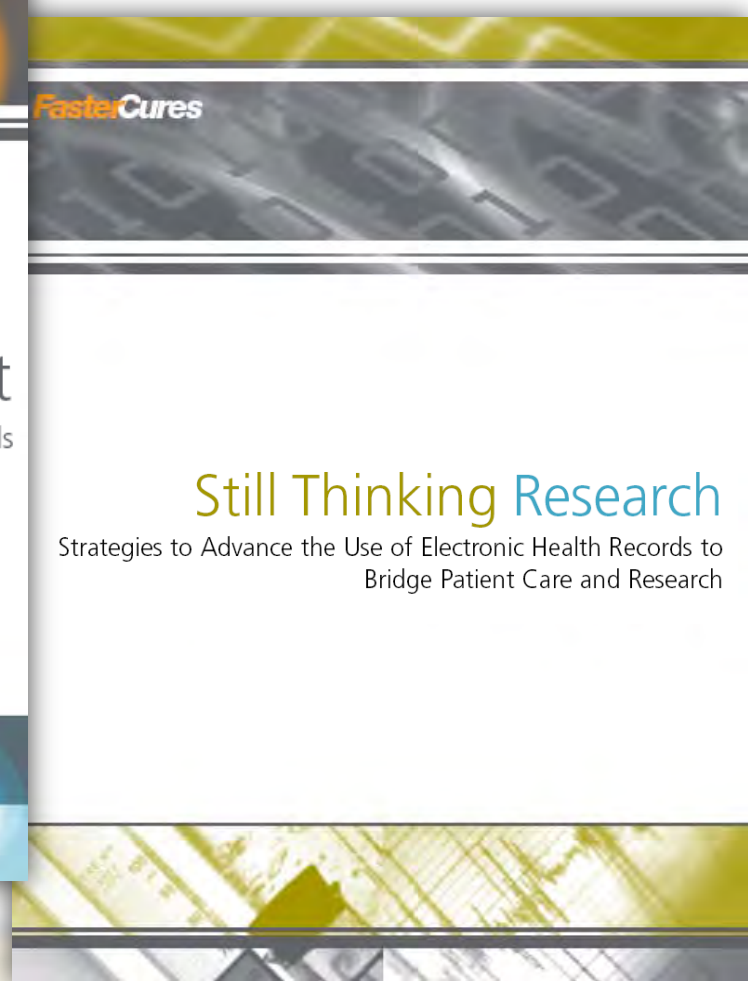
Perspectives on biobanking

- Thinking up:
 - Regulation/accreditation of biobanks
 - Biospecimen science: fund, publish, demand as basis for SOPs for research, product development and regulation, and clinical practice
 - A national biobank as an open access source of benchmark samples: Public private partnership in a pre-competitive business model
 - Unmet need nationally and internationally for standardized (benchmark) specimens of all types from all types of patient populations for benchmarking in key activities such as qualifying unknown samples for scientific use, diagnostic assay development/co-development, technology development, and calibration of assay or technology performance across labs

Biobanking opportunities

- NCI's Best Practices for Biospecimen Resources: authoritative, public process of development, state-of-the-science recently updated: human specimen resource and USA-centric
 - technical best practices
 - ethical, legal, policy best practices
 - Harmonization with ISBER (International Society for Biological and Environmental Resources): broader mandate for other types of specimens; international focus
- NCI experiences:
 - Building the standards of biobanking
 - Building the science for biobanking
 - Funding technology development aimed at creating solutions FOR biobanking
 - Building the missing infrastructure for the most challenging specimen acquisition
 - Creating strategic alliances for implementation , harmonization, synergy: FDA, NIST, CAP and international biobanking and biospecimen science groups

JUST RELEASED TODAY

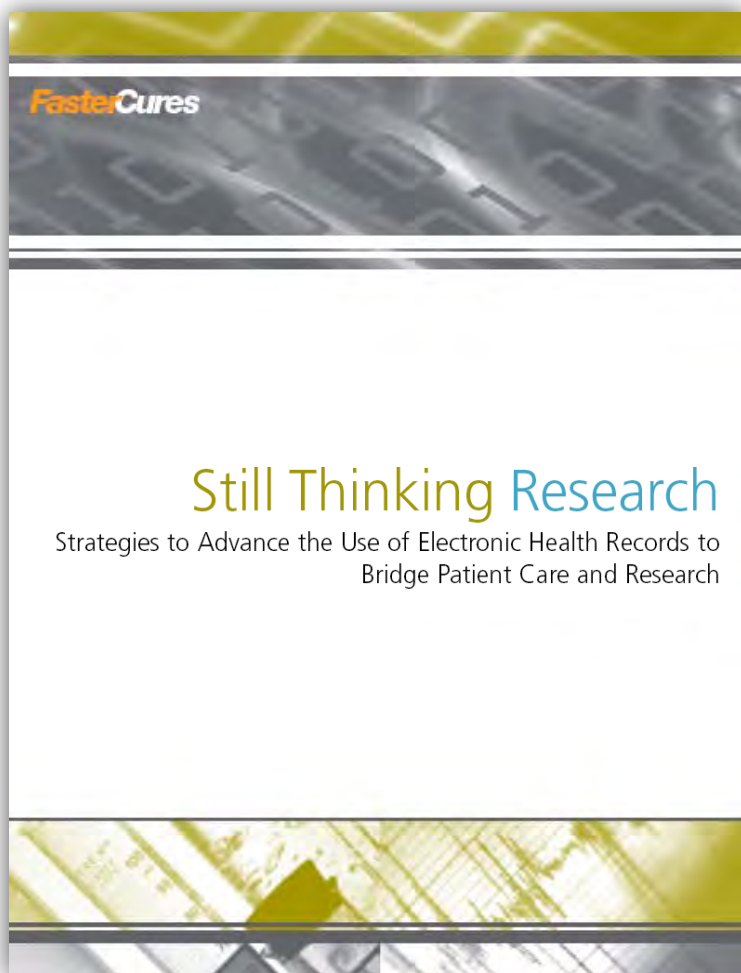


Still Thinking Research

Strategies to Advance the Use of Electronic Health Records to
Bridge Patient Care and Research

- Although the horizon has some bright spots, and there is more activity in this area than in 2005, the health IT infrastructure as it exists today is still falling short of its potential to increase understanding of disease progression and advance biomedical innovation.
- As the volume of digitized patient data grows, a lack of functionality and user interfaces that allow investigators to interact with and study those data are leading to a host of missed research opportunities.

Four Recommendations for Action

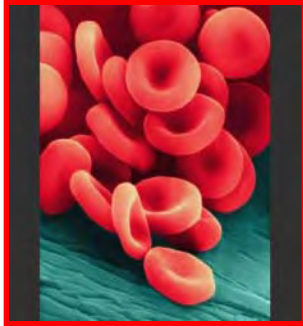


- Clinical trial screening and matching should be included as a measure for “Meaningful Use” of electronic health record systems.
- The National Institutes of Health (NIH) should articulate a strategy that will align its programs with the recommendations of the Office of the National Coordinator (ONC) Federal Health IT Strategic Plan.
- The ONC should develop an initiative with pilot projects that would create medical research IT modules.
- Nationwide Health Information Network (NwHIN) should expand its standards and policies to include clinical research and research centers in the network of information exchange.



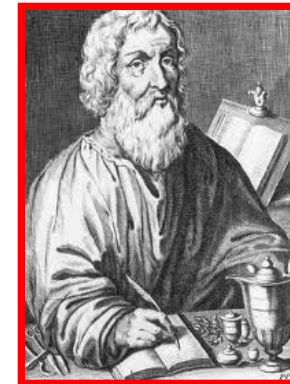
David Blumenthal, M.D., M.P.P.
Samuel O. Thier Professor of Medicine and
Health Policy, Harvard Medical School, and
former National Coordinator for Health
Information Technology

Moving Past Hippocrates



Information is the *lifeblood* of medicine

We manage information as *Hippocrates* did in 400 B.C.



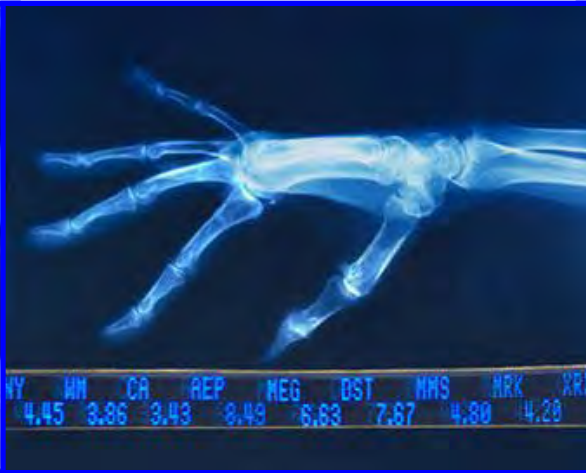
Health IT is the *circulatory* system of modern health care

More Practically



Electronic Health Record

Electronically capturing and processing information about patients



Health Information Exchange

Exchanging health information

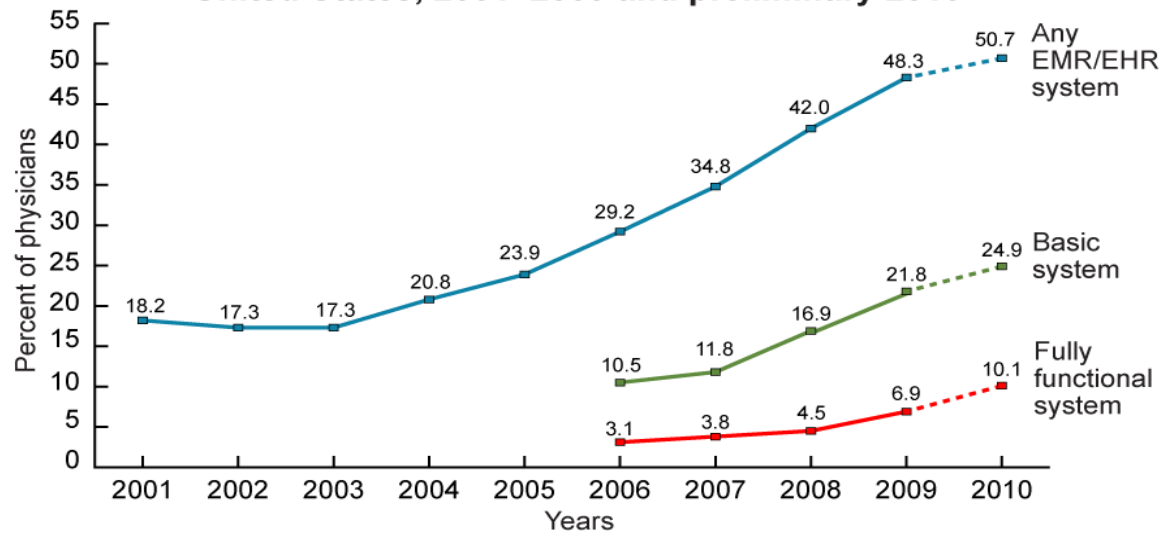


Clinical Decision Support

Improved care decisions

Current Levels of Adoption by Ambulatory Physicians

Figure 1. Percentage of office-based physicians with electronic medical records/electronic health records (EMRs/EHRs): United States, 2001–2009 and preliminary 2010



NOTES: Any EMR/EHR is a medical or health record system that is either all or partially electronic (excluding systems solely for billing). The 2010 data are preliminary estimates (as shown by dashed lines), based only on the mail survey. Estimates through 2009 include additional physicians sampled from community health centers; prior 2008 combined estimates were revised to include those physicians (4). Estimates of basic and fully functional systems prior to 2006 could not be computed because some items were not collected in the survey. Fully functional systems are a subset of basic systems. Some of the increase in fully functional systems between 2009 and 2010 may be related to a change in survey instruments and definitions of fully functional systems between 2009 and 2010 (see Table for more details). Includes nonfederal, office-based physicians. Excludes radiologists, anesthesiologists, and pathologists.
SOURCE: CDC/NCHS, National Ambulatory Medical Care Survey.

Source: National Center for Health Statistics, National Ambulatory Medical Care Survey, 2010.

The Federal Government's Response: HITECH ACT

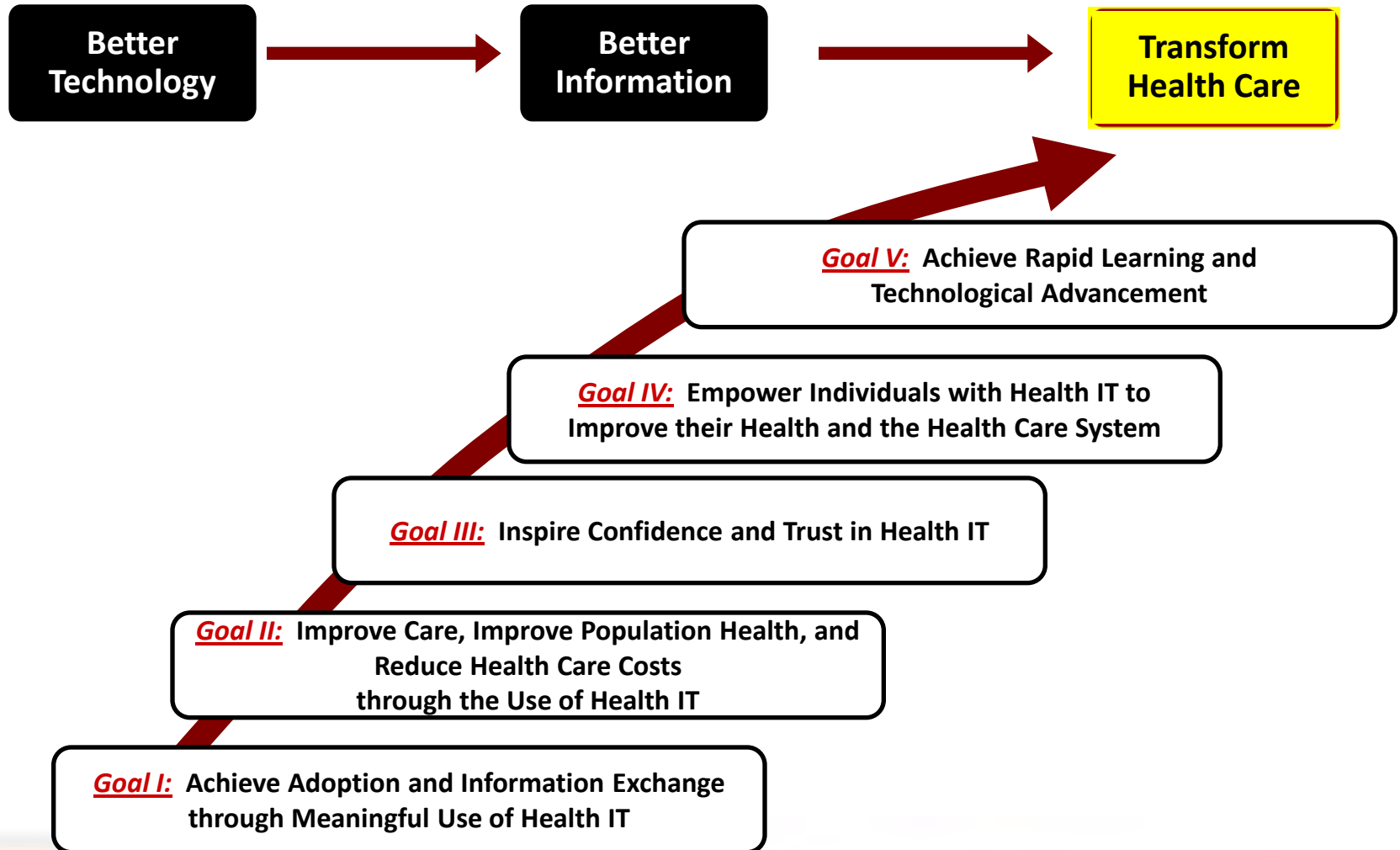


- Part of American Recovery and Reinvestment Act of 2009 (ARRA)
- Addresses major barriers to adoption, and much more:
 - Money, market reform
 - Technical assistance, support/workforce shortages
 - Health information exchange
 - Privacy and security

Money / Market Reforms

- Meaningful use framework:
 - Rewards the effective (meaningful) use of *certified* EHRs
 - Has created a vibrant competitive market in the health IT sector
- Key provisions:
 - Clinicians: \$44,000 / \$63,750 over 5-10 years
 - Hospitals: \$2 million bonus plus per DRG payments
 - Penalties after 2015
- Total:
 - \$9-27 billion over 10 years

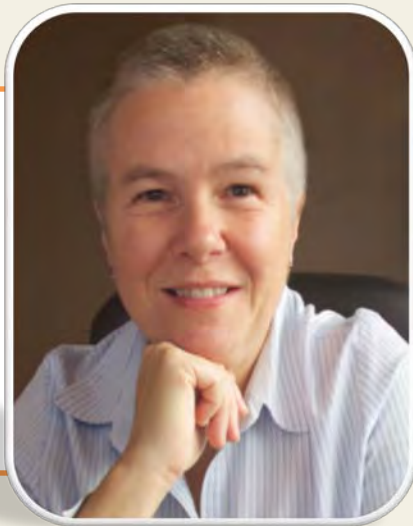
Going Forward: Federal HIT Strategic Plan 2011-2015



Key Challenges for Research: HIE

Health Information Exchange (HIE)

- Interoperability
- Governance
- Privacy and Security
- Economic case for exchange



Sharon F. Terry
President and CEO
Genetic Alliance

Power to the People: Participant Ownership of Clinical Trial Data

Sharon F. Terry^{1*} and Patrick F. Terry²

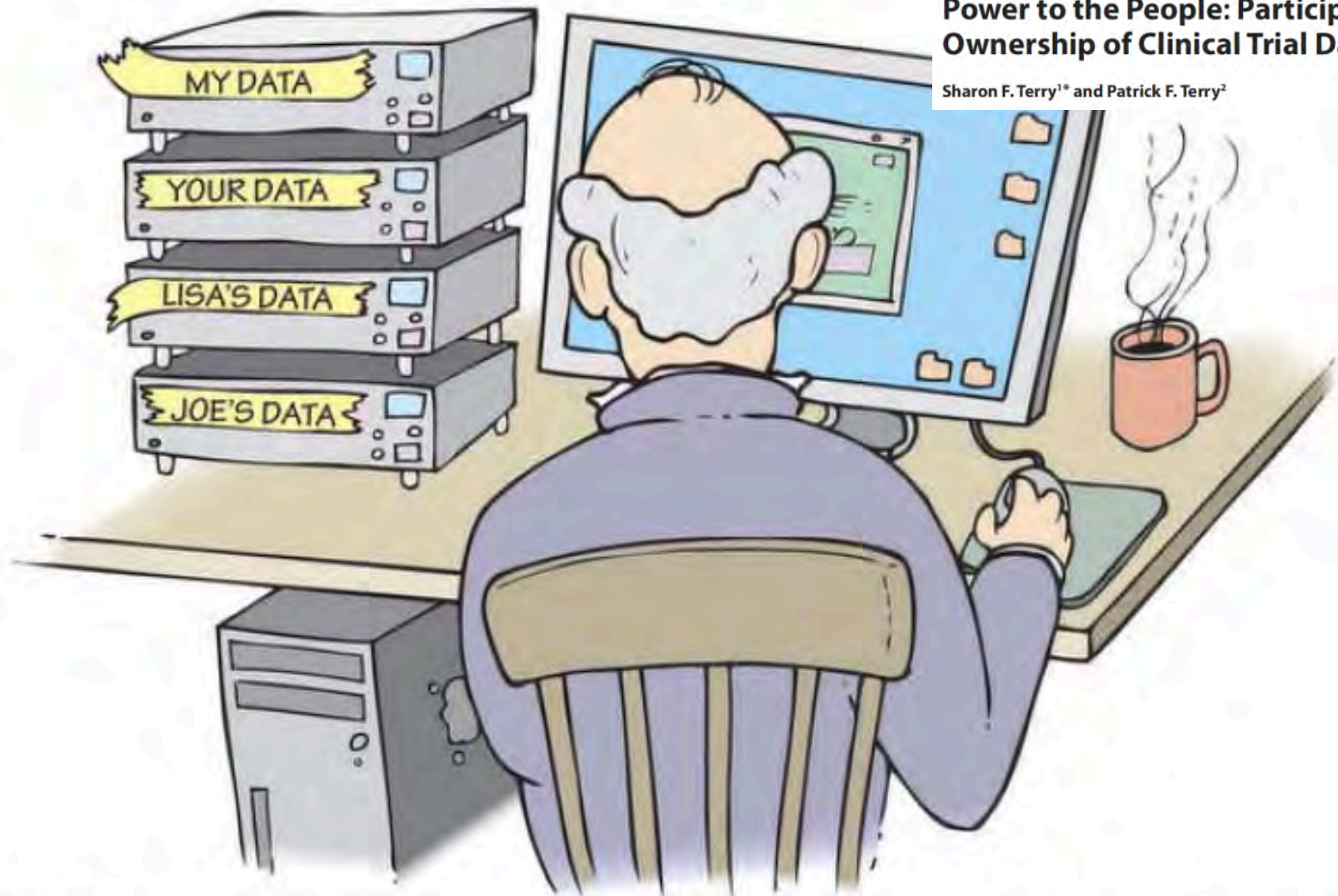


Fig. 1. “What’s my data is mine and what’s your data is also mine.”—Sydney Brenner, on data-mining (18).

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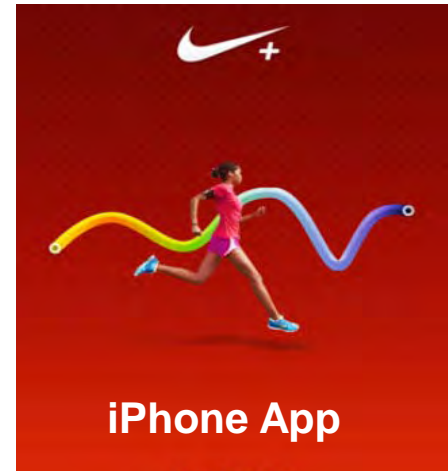
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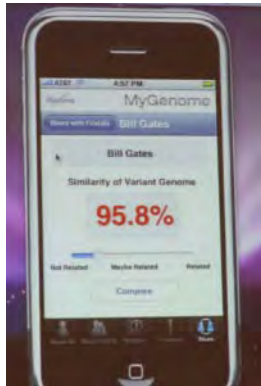


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LOVE/AVON Going Beyond a Cure



Digital Weight Scale



23andMe

facebook

DNA Warehousing – Newborn Screening

It is the moral imperative of every person on the planet to freely share their health information.

Paraphrase of Jamie Heywood, Co-founder, Patients Like Me



"We were appalled when we found out. Why do they need to store my baby's DNA indefinitely? Something on there could affect her ability to get a job later on, or get health insurance."



COURTESY GREGG ANDERSON

The Path to Solutions

- TRUST (it is hard to measure, to regulate, to codify)
- Community participation: disease, geographic, affinity, issues, faith
- Context is critical
- All information is not created equal, and where it lives, and who it might see it later, is important
- Technology enables much more today than even 5 years ago



Genetic Alliance Registry & BioBank

30,000 samples + 20,000 clinical records

Cross-disease, Trust Community

Cooperative – extensible,
interoperative, cost-sharing platform

Based on a Local (Global) Community

Trusted Agent

Personal Preferences in Data Sharing to Accelerate Translational Research

Technology that allows each person to grant “private access” to all or selected parts of their confidential personal information based on their particular needs and interests



Your solution for controlling who sees your personal health information. [Sign In or Register](#)

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“ You can trust **PrivacyLayer** to let you manage who can and cannot gain access to your health information. ”

LeRoy E. Jones, CISSP
Chief Executive of OSI Health, Inc.
Program Manager, Healthcare IT Standards Panel
*Chief Technology Officer for PrivateAccess LLC

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Toolbox

- My Account
- My Family
- Privacy Settings
- Privacy Alerts
- Audit Log
- Toolbox Help

Your Privacy is Our Priority PrivateAccess is pleased to offer users free use of PrivacyLayer™. When you see our “Privacy Assured”

Manage Your Records With PrivacyLayer, you decide who can see your information, when, and for exactly what purpose.



PrivacyLayer

Sign Up Select a Guide Set Privacy Preferences Share Health Info

Privacy Preferences: PrivacyLayer™ makes it easy to select your privacy preferences. Click on view your Guide's three suggestions. When you decide which description preferences, click the “Next” button found below.

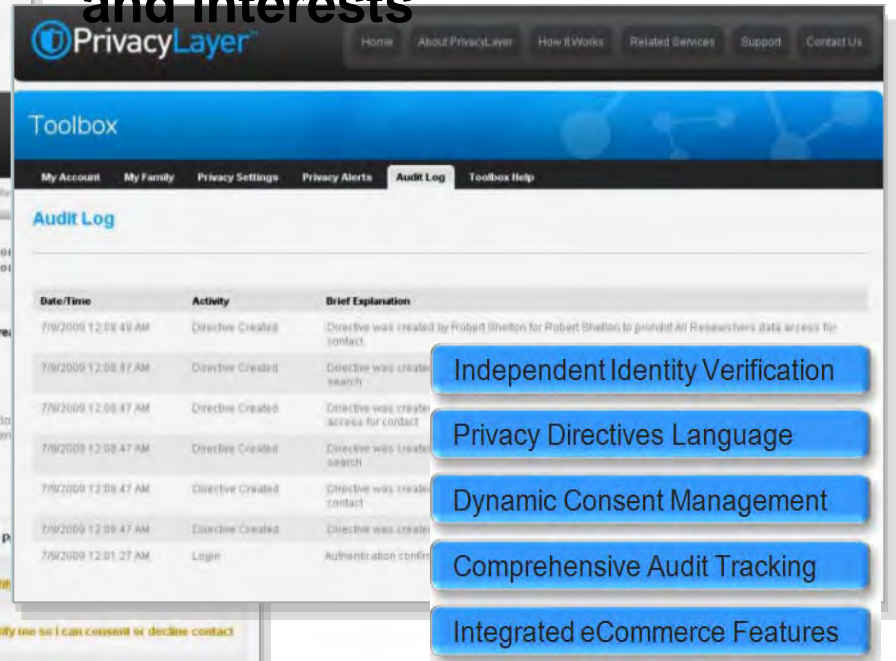
Lower privacy concerns Moderate privacy concerns Greater privacy concerns

Here's what your guide suggests if you have greater privacy concerns:

“ I realize that some value privacy to the point where they're uncomfortable disclosing information to someone they don't know. In that case, I'd suggest these settings, when learn more before you provide any contact details. ”

Researcher & Research Groups	Search Preferences <i>what's this?</i>	Contact P
Dr. Giard	Allow to see my anonymous information	Notify
All K&A Researchers	Allow to see my anonymous information	Notify me so I can consent or decline contact
All Researchers	Prohibit from searching	Prohibit all contact

Choose a different guide Customize Next



PrivacyLayer Home About PrivacyLayer How It Works Related Services Support Contact Us

Toolbox

My Account My Family Privacy Settings Privacy Alerts Audit Log Toolbox Help

Audit Log

Date/Time	Activity	Brief Explanation
7/9/2009 12:08:48 AM	Directive Created	Directive was created by Robert Shelton for Robert Shelton to prohibit All Researchers data access for contact
7/9/2009 12:08:47 AM	Directive Created	Directive was create search
7/9/2009 12:08:47 AM	Directive Created	Directive was create access for contact
7/9/2009 12:08:47 AM	Directive Created	Directive was create search
7/9/2009 12:08:47 AM	Directive Created	Directive was create contact
7/9/2009 12:08:47 AM	Directive Created	Directive was create
7/9/2009 12:01:27 AM	Login	Authentication confir

Independent Identity Verification

Privacy Directives Language

Dynamic Consent Management

Comprehensive Audit Tracking

Integrated eCommerce Features

Results of Early Projects

KS&A
Knowledge
Support &
ACTION

Home About KS&A Locate Information Find Resources Get Support Giving to KS&A Network with Others Search

Conferences
Newsletters
Online Discussion Groups
Research Studies and Clinical Trials
Share Your Story
Awards and Recognition

Current Research Studies and Clinical Trials

Neurocognitive Related Studies

- **NIH Klinefelter Syndrome / Sex Chromosome Variations Study**

Jay N. Giedd, M.D., Principal Investigator and Rhoshel Lenroot, M.D., Associate Investigator of The Child Psychiatry Branch of the National Institute of Mental Health are conducting a study of the effects of sex chromosomes on brain development. The goal of this study is to determine whether brain-imaging studies of children with sex chromosome variations will help uncover core biological features of these chromosomal conditions.

Male participants (5-25 years old) with XXY, XYY and XXYY sex chromosome variations are needed to participate in this study. Participation involves coming to the National Institutes of Health (NIH) in Bethesda, Maryland (just outside of Washington D.C.) for a one-day visit (Tuesdays).

For more information, please download the document linked [here](#), or contact:

Jonathan Blumenthal, M.A.
Project Coordinator
Phone: 301-435-4516
Email: jb364e@nih.gov

Child Psychiatry Branch Website: <http://intra.nimh.nih.gov/cbp/xxxy>

I'M INTERESTED

- 1,200 patients and 5 researchers initially targeted
- 90% of patient users completing survey indicate positive experience – easy to use
- 75% would recommend to friends and family
- Partnering with a “trusted source” overwhelmingly drives patient participation



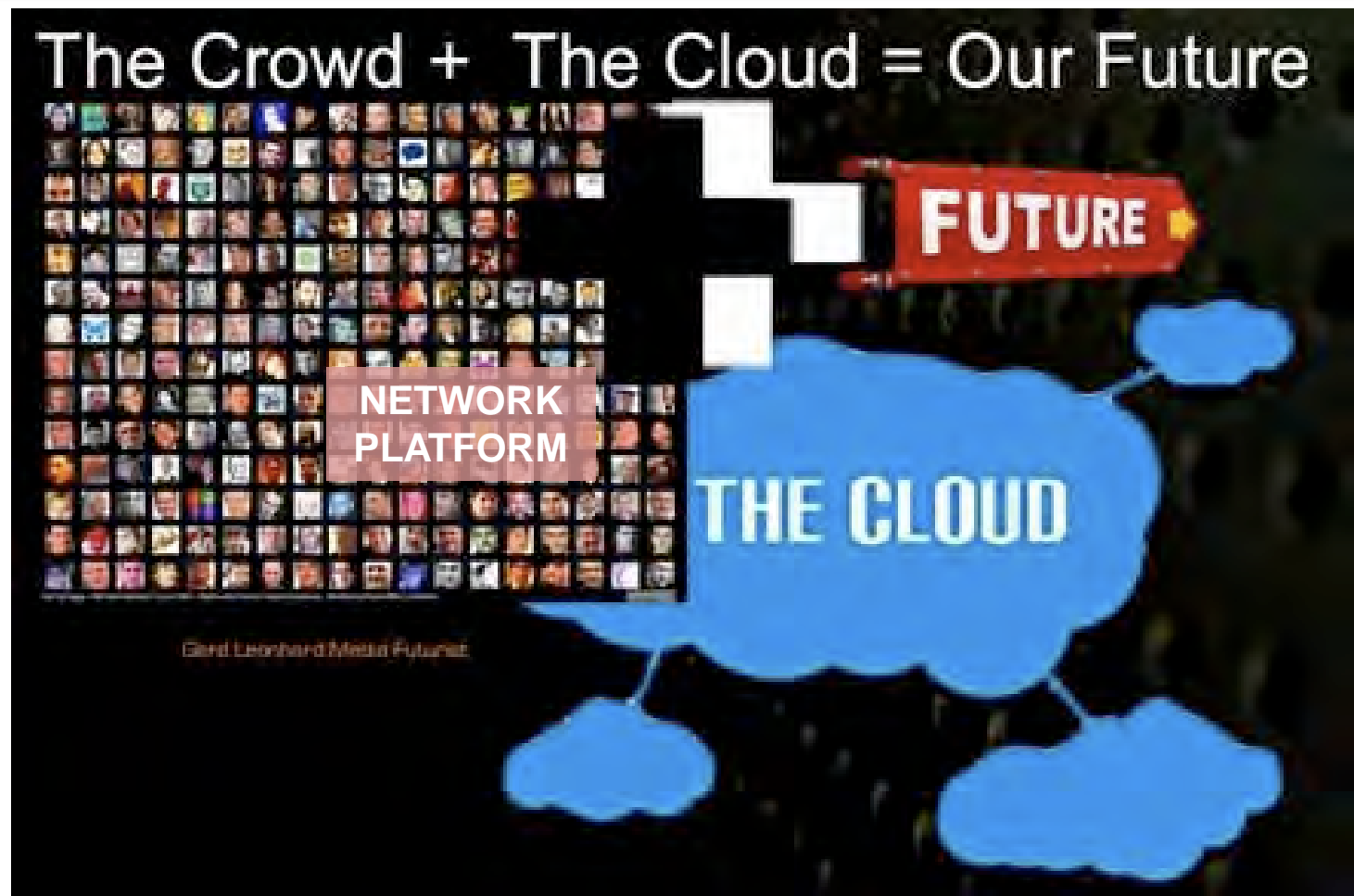
GARB Toolbox

The GARB Toolbox is designed to serve as a map of the tools and resources created by GARB. Each bright blue compartment on the left represents overarching themes and questions that often arise when establishing or improving a registry or biobank. Within each of these compartments, we have identified resources that GARB provides. By clicking on the color-coded boxes below, you will be directed to specific resources, including publications, training and mentoring tools, videos, webinars, web pages, and worksheets. Although each resource is independent and can be used in any order, the boxes are arranged with introductory material on the left and more detailed material to the right. With the help of the Toolbox, we hope to guide you to all of our different resources for registries or biobanks.

Please [click](#) here to view a PDF description of the GARB Toolbox.

Where do I begin?	What is a biobank?	Guidelines for considering a registry/biobank	Advocates are leaders in biobanking	
How do I make this a reality?	Registry/repository start-up guide	Making your organization's biobank a reality	Genetic Alliance registry/repository boot camps	
Is my organization ready?	Organizational readiness checklist	Biobank question & answer session		
How do I select a vendor?	Vendor assessment summary	Vendor assessment worksheet	Landscape analysis manuscript	
Considering Genetic Alliance?	Genetic Alliance BioBank	GARB FAQs	Virtual tour of Genetic Alliance registry solutions	GARB history manuscript
What else do I need to know?	Biobank governance	Biobank governance checklist	Resource list	
How do I stay current?	Registry and Biorepository Bulletin	BioBank discussion listserv	BioBank Ambassadors mentoring	Weekly tips

Who will build the datasets/ models capable of providing powerful safety and efficacy insights?



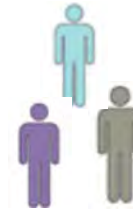
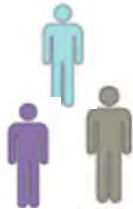
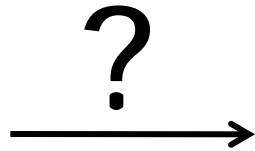
Patients Physicians Citizens Knowledge Experts

Sage Mission

Sage Bionetworks is a non-profit organization with a vision to create a “commons” where integrative bionetworks are evolved by contributor scientists with a shared vision to accelerate the elimination of human disease



Community-based data sharing and analysis is essential to build accurate models of disease



Within the analytical community:

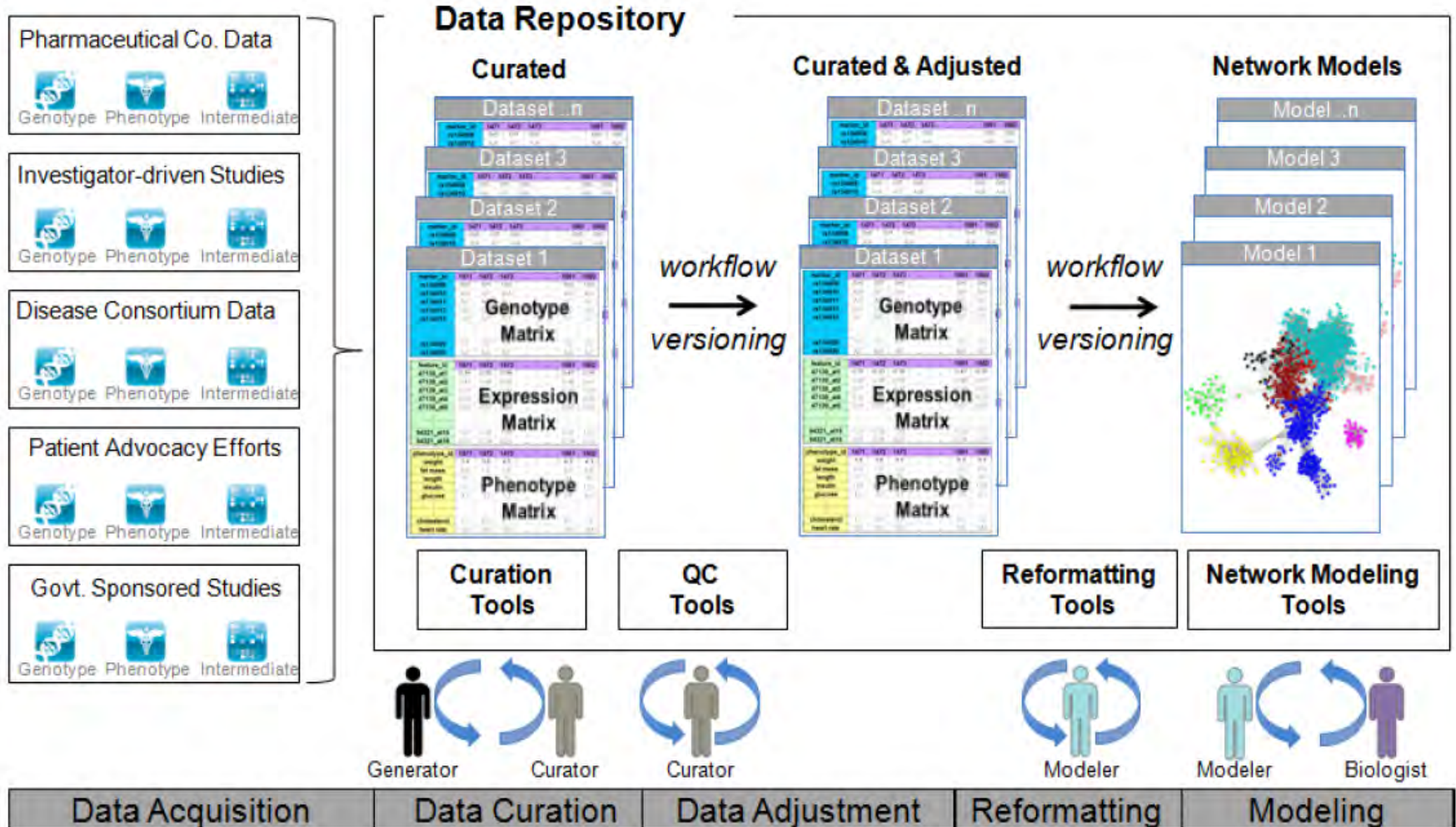
- Quality assessment of data, tools and models
- Reproducibility of models
- Reusability of data
- Validation of models across multiple disease models and patient subtypes

Across the field of biology:

- Validation of model predictions in experimental/clinical setting
- Accelerated pace towards improved therapeutic development

Open source, community-based genomic data sharing and analysis will maximize clinical impact

- Graphic of curated to qc'd to models



THAT'S MY DATA!

- Looking for a volunteer community
- Tell the story of WHY share data?
- Develop tools for you to liberate your data from academia and industry – Grant Back with Creative Commons
- Guide/educate interested participants through the legal process
- For those that decide to share data, future data collected from these select participants will be placed in a 'commons' - free and open data access for research use

“You never change things by fighting existing reality. To change something, build a new model that makes the existing model obsolete.”

Buckminster Fuller

Moderated Q&A



- Carolyn Compton, M.D., Ph.D.
Director, Office of Biorepositories and Biospecimen Research at the National Cancer Institute



- David Blumenthal, M.D., M.P.P.
Samuel O. Thier Professor of Medicine and Health Policy, Harvard Medical School, and former National Coordinator for Health Information Technology



- Sharon F. Terry, President and CEO, Genetic Alliance



MODERATOR:

- Margaret Anderson, Executive Director, *FasterCures*

Archive of this Webinar: www.fastercures.org/train



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No time to waste:

FasterCures releases new white paper on importance of translational research

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Crossing Over the Valley of Death

TRAIN CENTRAL STATION FEATURES

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Study asserts that cancer drugs approved

[Blog](#)

06/20/11: What Have We Learned from

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Send us your questions for NIH



WHAT'S NEW AT TRAIN?

Events

- **Sept. 16 Webinar** - Bits and Bytes: Making Patient Information Available and Meaningful to Researchers

Case Studies

- Michael J. Fox Foundation for Parkinson's Research
- Cystic Fibrosis Foundation

Tools and Resources

Featured Publication: Crossing Over the Valley of Death

Resource: Partnering for Cures Innovator Presentations

Tool: IP & Data Sharing Principles

Current News

TIME calls TRAIN members 'stars of venture-charity model'

Innovator Spotlight

Q&A with Susan Axelrod, CURE chair

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