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

Align research priorities with our needs!

Patients

Incentivize patient participation

Empower

Patients helping doctors

Data

Clinical trials

H.I.T.

Biobanks

Think research
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

Banking on Trust
The Future of Research with Human Biological Materials

Still Thinking Research
Strategies to Advance the Use of Electronic Health Records to Bridge Patient Care and Research
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 Collection ➔ Biospecimen Processing and Banking

QUALITY HERE
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

Cancer Patient

Mini-Me Biospecimen

Disease Biology

Biological Stress!

“Induced” Molecular Changes

Object of Investigation
(NCI’s Biospecimen Research Network)

Translational Research

Personalized Medicine

M.D.

Researcher

Cured Patient
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):
- Time at room temperature
- Temperature of room
- Type of fixative
- Time in fixative
- Rate of freezing
- Size of aliquots

Factors (examples):
- Antibiotics
- Other drugs
- Type of anesthesia
- Duration of anesthesia
- Arterial clamp time

**Time 0**

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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

Banking on Trust
The Future of Research with Human Biological Materials

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

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

Goal I: Achieve Adoption and Information Exchange through Meaningful Use of Health IT

Goal II: Improve Care, Improve Population Health, and Reduce Health Care Costs through the Use of Health IT

Goal III: Inspire Confidence and Trust in Health IT

Goal IV: Empower Individuals with Health IT to Improve their Health and the Health Care System

Goal V: Achieve Rapid Learning and Technological Advancement

Better Technology → Better Information → Transform Health Care
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
Fig. 1. “What’s my data is mine and what’s your data is also mine.”—Sydney Brenner, on data-mining (18).
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."

Karen Brown, Nurse, new mother, Florida
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

BioBank.org
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.

PrivateAccess.com
Results of Early Projects

- 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
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.

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

The Crowd + The Cloud = Our Future

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 qced 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

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MODERATOR:

- Margaret Anderson, Executive Director, FasterCures
Archive of this Webinar: www.fastercures.org/train
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An opportunity to find allies who can help you advance your medical research goals.

A platform for solutions needed to accelerate progress.