



**OBBR**

Office of Biorepositories  
and Biospecimen Research

## The Cancer HUMAN Biobank (caHUB)

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Director, Office of Biorepositories and Biospecimen Research

BRN Symposium  
March 25, 2010



**caHUB** The Cancer  
Human Biobank

NATIONAL  
CANCER  
INSTITUTE



## The NCI Addresses the Challenge

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### Consensus of the Broad Scientific Community:

The lack of high-quality, clinically annotated human specimens has become the limiting factor for translational cancer research.

### The NCI Moves Stepwise Towards Solutions:

- Standards
  - The *NCI's Best Practices for Biospecimen Resources*
- Science
  - The Biospecimen Research Network
  - The Biospecimen Research Database
  - The Innovative Molecular Analysis Technologies
- **Specimens and Service**
  - **The Cancer Human Biobank**





## Understanding the Problem: The Siloed National Biobanking Landscape

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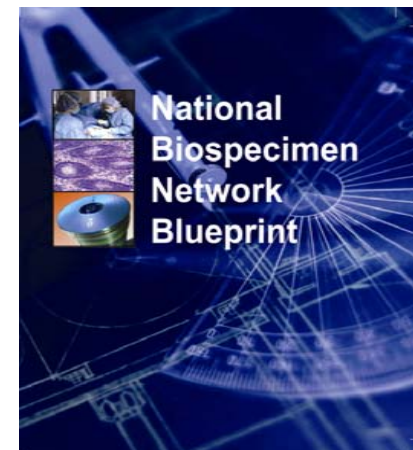
- Collection, procession, storage procedures differ
  - Degree and type of data annotation varies
  - Scope and type of patient consent differs
  - Access policies are lacking or unknown to potential users
  - Materials transfer agreement conditions differ
  - Supporting IT structures differ in capacity and functionality
- **WIDE VARIATION IN QUALITY OF SPECIMENS AND DATA**

## Consensus for a Solution: The National Biospecimen Network Blueprint (2003)

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### Key principles for a national biobank:

- Standardized procedures for biospecimen collection and distribution
- Standardized data sets and data vocabulary
- Integrated information technology system to support all functions
- Harmonized approaches to ethical and legal issues
  - Standardized consent, MTAs
- Transparent governance and business models
  - Transparent access policies
- Large well-designed, standardized specimen sets





## caHUB Is Founded on NBN Principles

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### The Cancer Human Biobank vision:

- **unique, centralized, non-profit public resource**
- **source of adequate and continuous supplies of human biospecimens and associated data of *measurable, high quality* acquired within an ethical framework**
- **source of high-quality biobanking services for the community**



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## Issues for caHUB During the Past Year

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- **Verification of the need for caHUB**
- **Development planning**
- **Fundamental details**
  - **Who will provide the specimens**
  - **Who will use the specimens**
  - **How data will be collected and handled**
  - **How the specimens will be used (scientific purposes)**
- **Business plans and timelines**
- **Funding: \$60M ARRA funds allotted to caHUB in 2009**



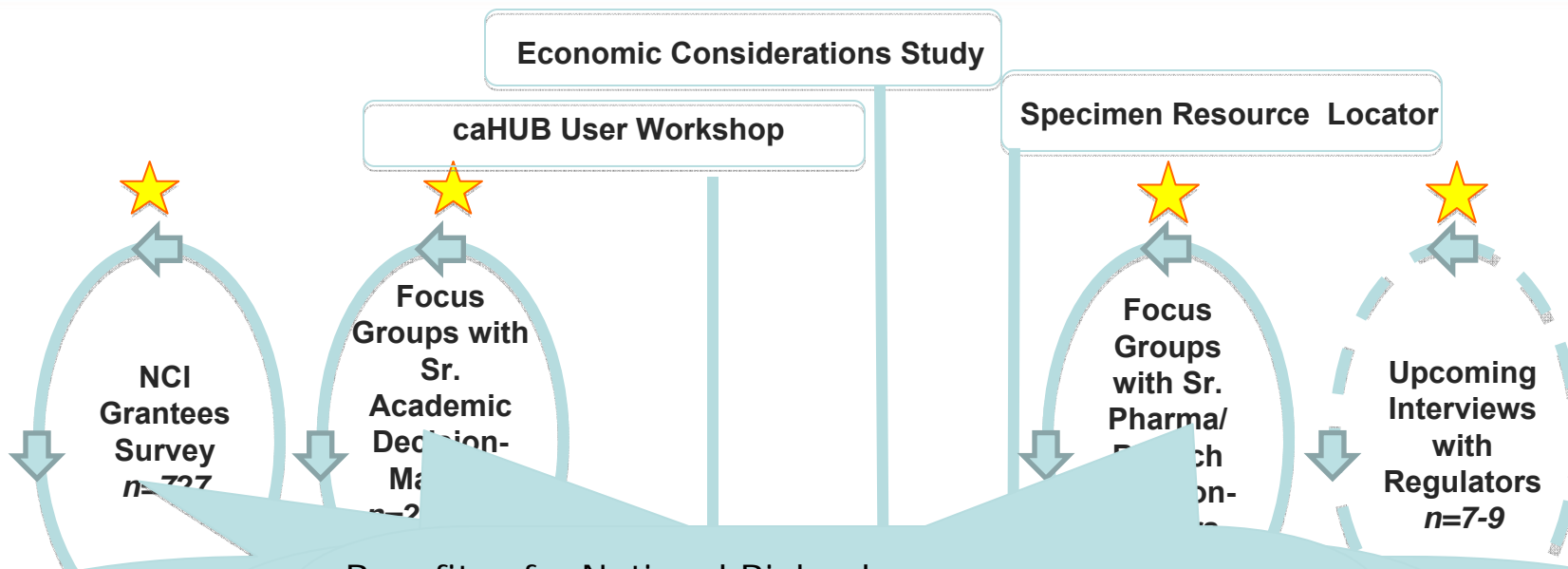
## The Need for caHUB

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- **The need for caHUB has been clearly enunciated from all sources:**
  - **Survey of 5,000 NCI investigators**
  - **Market research using focus group sessions with academia, industry, regulator decision-makers**
    - **Office of Management and Budget-approved**
    - **Performed by NCI Office of Communication and Education with Strat@com**
  - **Interviews with commercial tissue providers and industry users (economics considerations study by Booz Allen Hamilton)**
  - **caHUB Users Workshop**
  - **Mining of request data from the NCI Tissue Locator: last 7 years**
  - **Direct input to OBBR from potential users: CTEP, NCI Patient Characterization Center (PCC), numerous biomarkers programs**



# Stakeholder Feedback



**Key Findings:**

- Biospecimen collection and storage
- Both the collection and storage
- Researcher and clinician
- Researcher and clinician

**Benefits of a National Biobank:**

- Inspire confidence in quality of specimens
- Ensure ethical collection standards

**Development Challenges:**

- IP constraints

**Strong support for a national biobank concept from all stakeholders**

**Barriers to Contribute:**

- IP constraints





## Summary: Market Research Results

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- **There is clear and universal need for a National, Standardized, Human Biospecimen Resource (NSHBR)**
- **For all audiences, the level of consistency and standardization that could be offered is the most important benefit**
- **An NSHBR has the opportunity to define standard operating procedures (SOPs) for the field/industry**
  - **In fact, stakeholders are counting on it**



## caHUB Collection Design: Informed by User Need

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### In high demand and short supply:

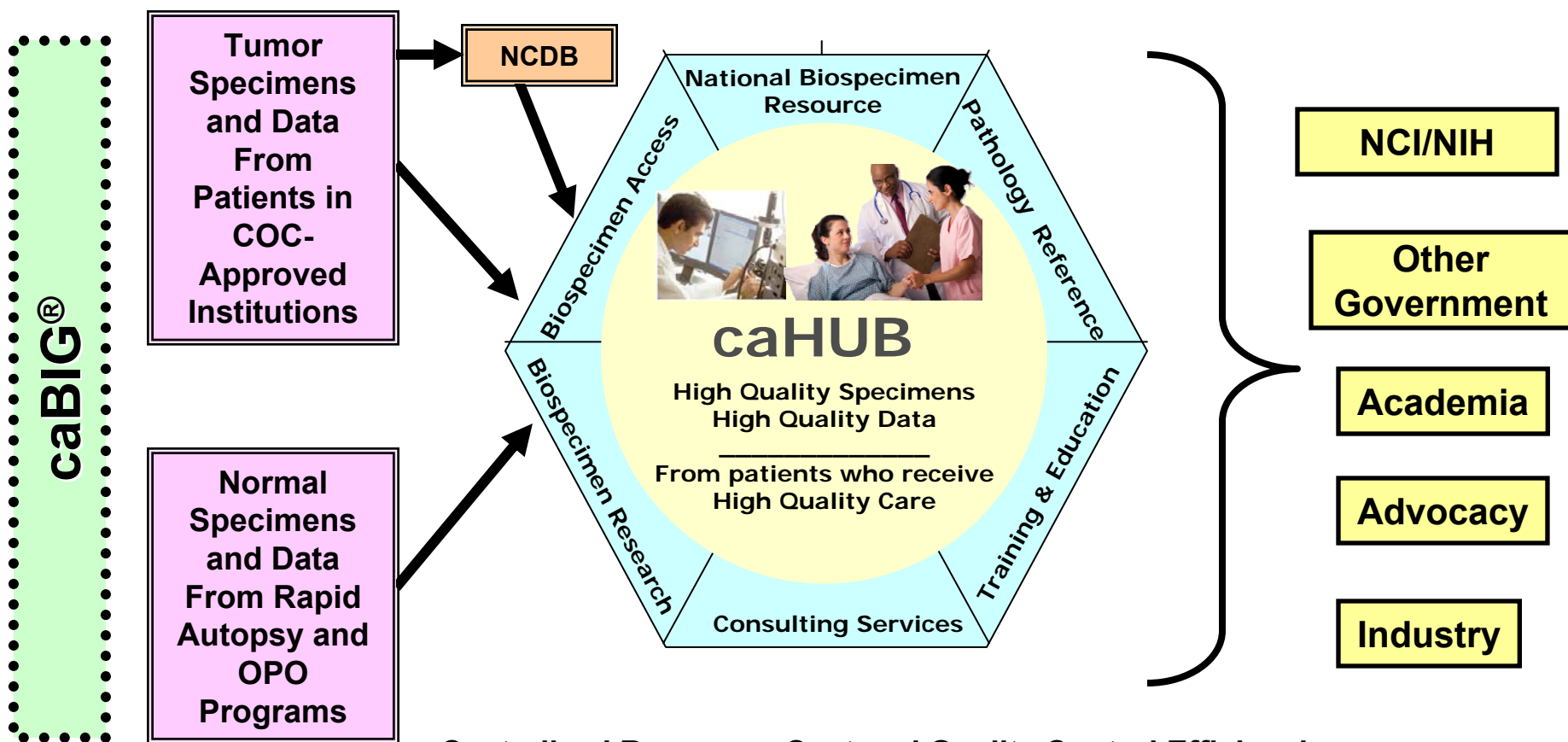
- **Benchmark samples:** biospecimens collected through standardized collection, handling, storage, processing and distribution procedures, with strict quality control and associated metrics
  - Comparable standard-of-care samples to test “generalizability” of new products
- **Cases with multiple aliquots:** Confirmation of prior studies or the opportunity to contribute information to prior studies based on new technologies
- **Statistically valid numbers of biospecimen sets**
- **Fully defined “patient case sets”**
  - Tumor
  - Adjacent normal tissue
  - Tumor periphery (invasive border)
  - Pre- and post operative blood samples
  - Urine
  - **Rich clinical data and outcome information for patient**
- **Non-surgical samples: normal tissues; metastases; premalignant disease**



# caHUB: Centralized Model

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- HIGH QUALITY SPECIMENS • HIGH QUALITY DATA • FROM PTS RECEIVING HIGH QUALITY CARE



Centralized Resource: Cost and Quality Control Efficiencies



## **The caHUB Comprehensive Data Resource: The Heart of the HUB**

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- **Provide user access to caHUB**
- **Ensure patient privacy and confidentiality**
- **Allow submission of data on patients and specimens**
- **Integrate patient, specimen and analysis data from multiple sources**
- **Perform QA/QC on all data**
- **Allow researchers to query specimen inventory based on data types**
- **Allow requesting of specimens**
- **Allow requesting, analysis, and download of datasets**



## The caHUB Business Model: A Commodities and Services Model

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### **COMMODITIES: Cost Recovery**

#### **Distribution of specimens and data**

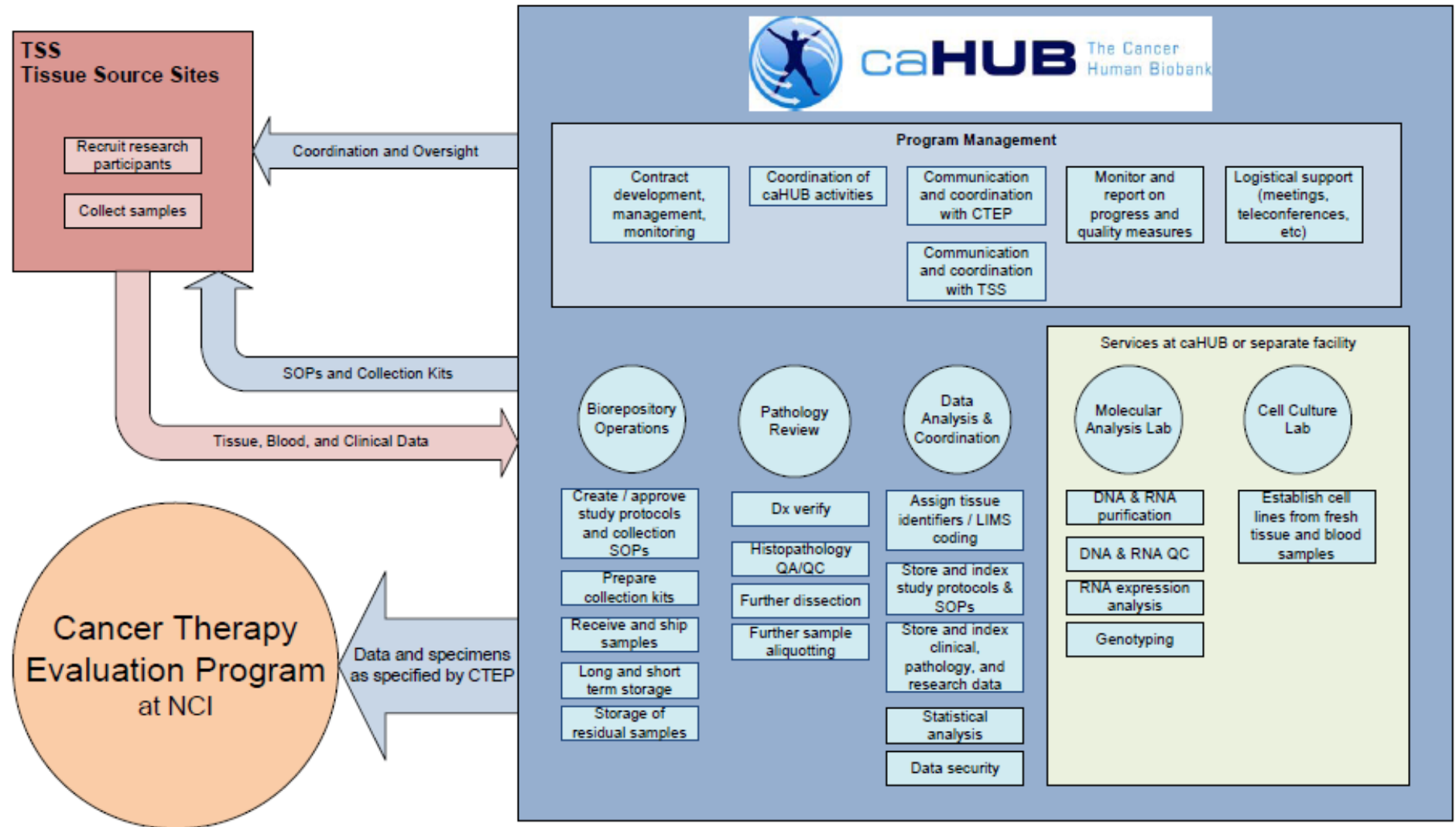
Increasing value of aliquots over time with increasing data richness: Time-dependent maturity

### **SERVICES: Revenue Generation**

Build on existing infrastructure and improve return on investment: Not time-dependent

- **Biobanking services to other initiatives**
  - Other NCI/NIH
  - Rare diseases
  - Advocacy
- **Education and training**
  - Pathology and laboratory functions
  - Operating room functions
  - IT and data management
  - Biostatistical and analytic methods
- **Consulting services**
  - Biobanking methods and best practices
- **Biobanking support service to industry**
  - Assay development
  - Clinical trials
- **Laboratory space and services**
  - Research incubator functions
  - Longer term in-house research contracts

# caHUB as Service Provider: CTEP Example

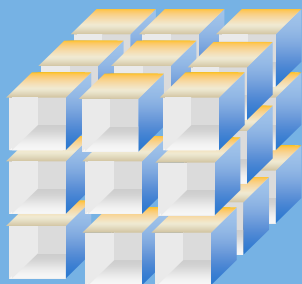


# caHUB Business Model

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## Commodities and Service-Based Model

caHUB Platinum Specimen Catalog



Genotyping Protein Microarrays  
Molecular Derivative Isolation DNA Expression Profiling  
Proteomics Analysis Sequencing



GTEX



PCC



CTEP  
(clinical trials)



TCGA



Advocacy

Pathology Review

Quality Assurance

Laboratory Best Practices

SOP Training

Research Services

Sample Orders

Data Orders

Customized Processing Services

Managed Collections  
"Front Door" Concept

Center of Excellence  
Training



## Continuous Process Improvement

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- **caHUB will establish policies and procedures that will ensure its continued responsiveness to the changing needs of the research and product development communities**
  - Goals for performance metrics and success factors
  - Routine assessment of performance against metrics
  - Customer Satisfaction Program
  - Ongoing market analysis to be responsive to trends in translational research and advances in biomedical technology
  - Transparent mechanism for receiving and responding to pub







## caHUB, A Transformative Initiative

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### More Efficient Research



- Reduction in re-experimentation due to higher quality samples
- Avoided cost of incremental labor from PIs and lab technicians, researchers
- Avoided cost of replacing failed samples because of higher sample quality
- Avoided time delays and labor costs for recontact and recontact of patients for new studies

### More Efficient Use of Resources



- User leverage of caHUB's systems infrastructure, reducing the need to purchase and maintain requisite infrastructure
- User leverage of caHUB goods and services, decreasing labor costs to process samples in order to meet research requirements

### Ensured Implementation of Best Practices



- Increased comparability (quality and uniformity) of specimen and data sets
- Ensured compliance reducing implementation and monitoring costs

### Stronger Clinical Correlation



- Increased quality and uniformity of data for more accurate modeling
- Avoided re-collection of data, saving time and cost



## caHUB, A Transformative Initiative

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More Efficient  
Product  
Development and  
Regulatory Approval



- Higher quality analytes, advancing biomarker research
- Higher quality specimens, helping reduce clinical trials timeframes and costs
- FDA recognition of “platinum” status specimens, leading to more rapid approvals for new drugs and diagnostics

More Efficient  
Technology  
Development and  
Clinical  
Implementation



- Standardized samples allowing direct performance comparisons
- Benchmark biospecimens allowing calibration, performance monitoring and operator proficiency testing

Added Clinical  
Value: Improved  
Standards of Care



- Increased speed of transition from research standards to standards of care
- More rapid implementation and standardization of diagnostic assays in clinical laboratories



## caHUB, A Transformative Initiative

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**Improved  
Outcomes for  
Cancer Patients**



- **Increase in lives saved**
- **Improvements in quality of life**
- **Positive impact on personal economics**
- **Savings to healthcare systems**
- **Positive impact on national economics (GDP, tax revenues)**



## Team OBBR and FOBBRs

OBBR Office of Biorepositories  
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- Jim Vaught
- Helen Moore
- Nicole Lockhart
- Sherry Sawyer
- Kim Myers
- Mark Lim
- Joyce Rogers
- Jim Robb
- Joanne Demchok
- Richard Aragon
- Priyanga Tuovinen
- Benjamin Fombonne
- Ian Fore /caBIG
- Kenyon Erickson /caBIG
- Andrew Breychak /caBIG

### SAIC Frederick Colleagues

- Mark Cosentino
- Mariana Gonzales del Riego
- Steve Buia

### caHUB Planning Participants

- Elaine Gunter
- Deborah Collyar
- Neil Mucci
- Peter Fielding
- Ann Ashby
- Todd Carolin
- Dean Keser
- Don Jin
- More than 200 contributors



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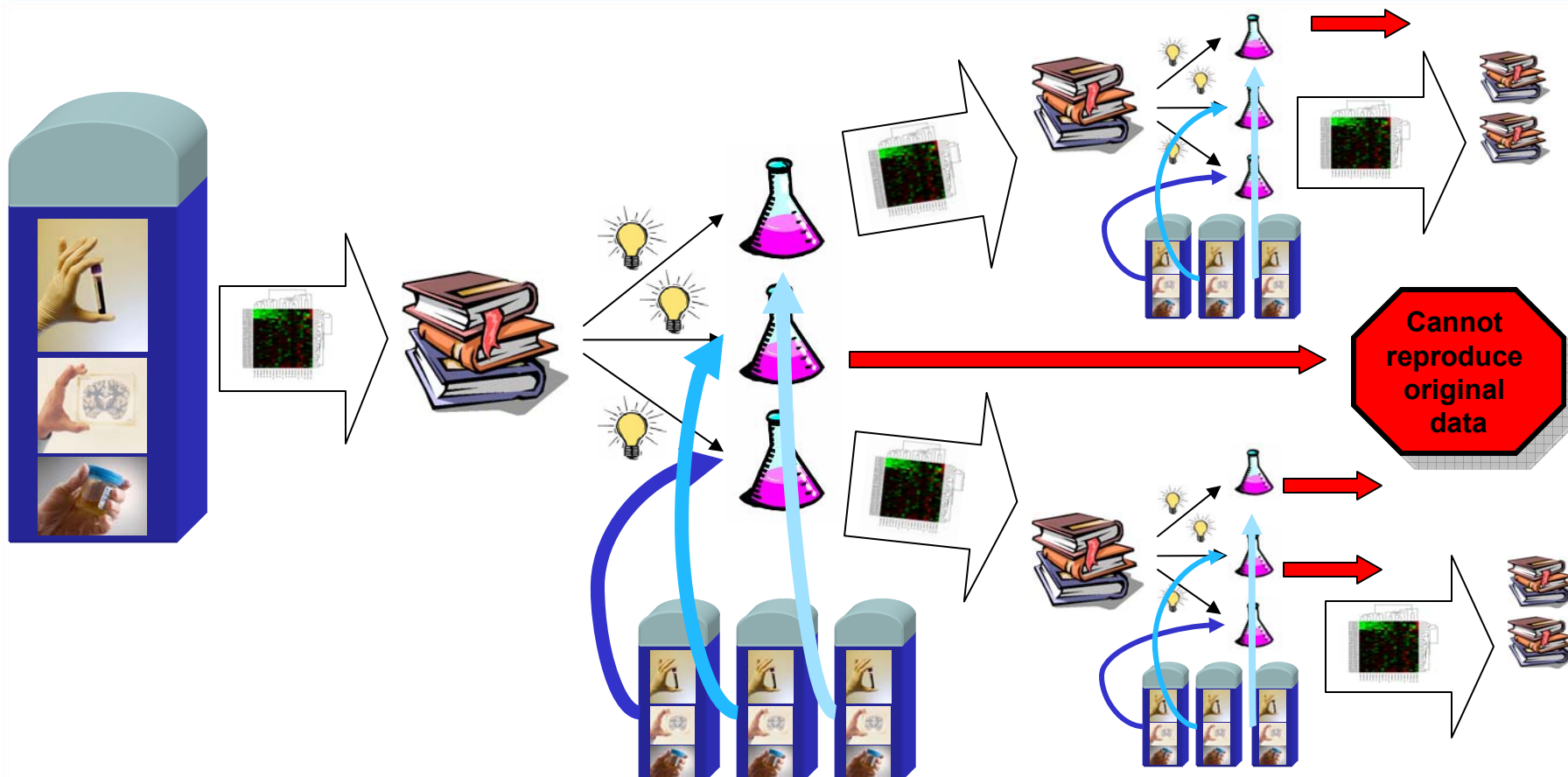
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# Silos: Biospecimen and Biospecimen Data Variation Thwarts Innovation in Medical Science

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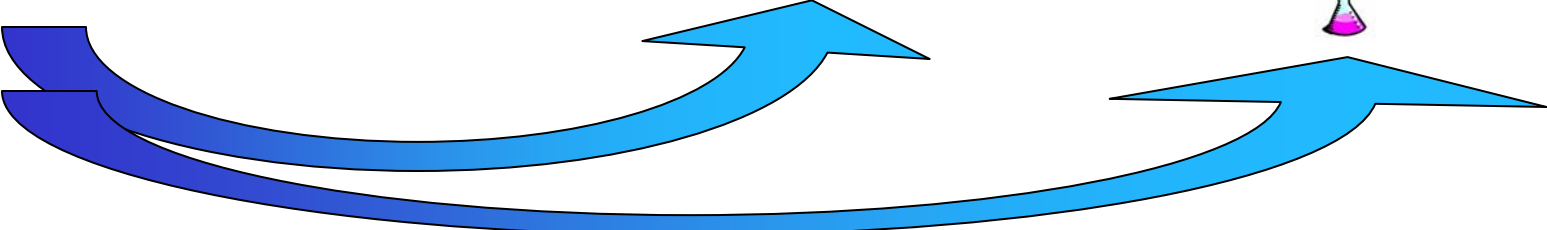
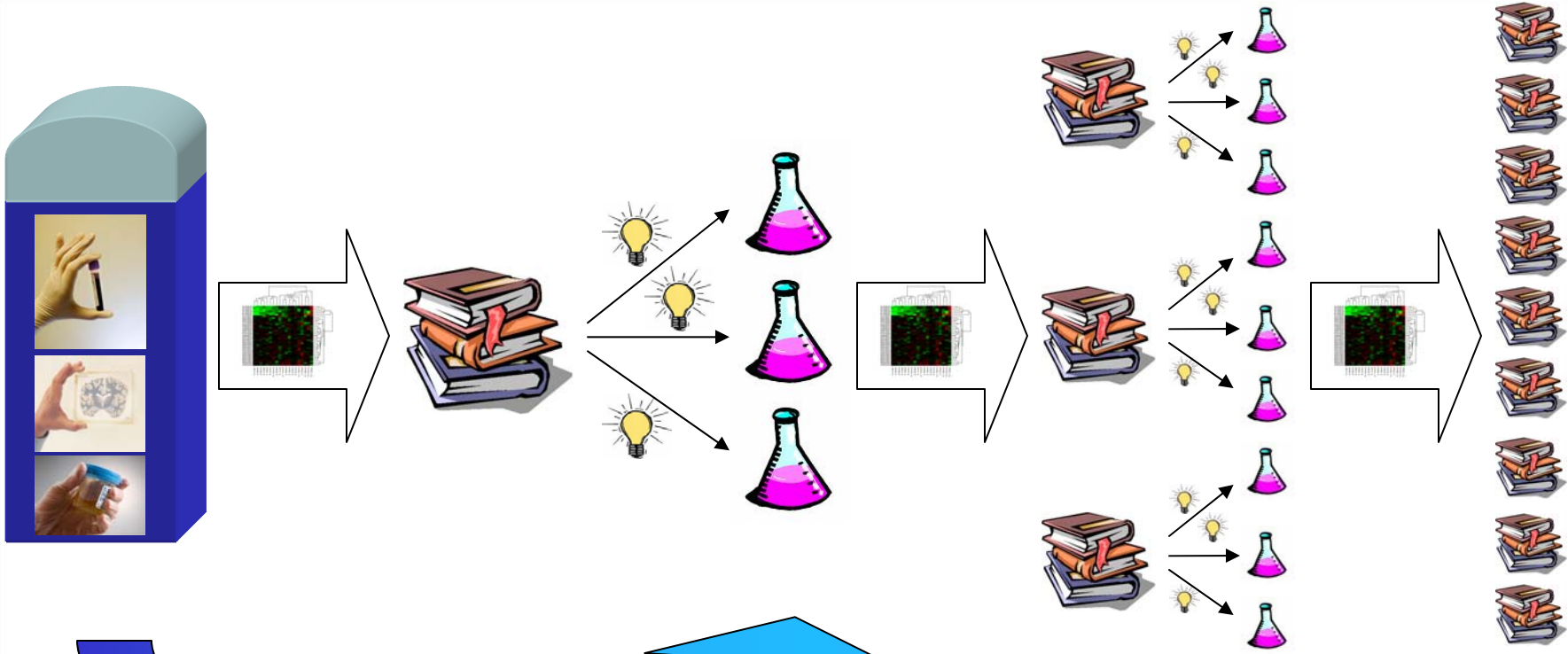


**Scientific Progress?**



# Biospecimen/Data Standardization Advances Innovation in Medical Science

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**Scientific Progress**

# Developing Biomarkers with Biospecimens of Unknown Quality

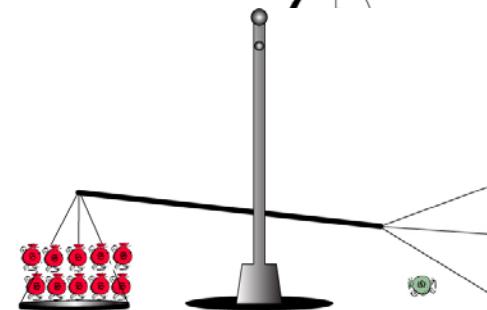
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- Analysis of Molecular Features
- Identification: Marker of Disease/Disease Feature
- Biomarker Validation
  - Milestone: Confirmation of Disease*
- Product Development
  - Diagnostic (chemical, pathologic)
  - Therapeutic drug
  - Molecular imaging tool
- Product Validation

**CANNOT REPRODUCE ORIGINAL RESULTS**

## Investment of time and money







## Accrual and Inventory Maturation

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Fiscal Year	Number of Tumor Cases	Number of Normal Cases	Total Number of Cases per Fiscal Year	Total Case Accrual	Total Aliquots per year	Total Aliquot Accrual
2011	1,000	600	1,600	1,600	41,440	41,440
2012	2,000	1,200	3,200	4,800	78,880	120,320
2013	3,000	1,600	4,600	9,400	109,240	229,560
2014	5,000	2,400	7,400	16,800	177,960	407,520
2015	6,000	2,400	8,400	25,200	206,160	613,680
2016	6,000	2,400	8,400	33,600	206,160	819,840
2017	3,000	900	3,900	37,500	98,460	918,300
<b>Total</b>	<b>26,000</b>	<b>11,500</b>	<b>37,500</b>		<b>918,300</b>	
30% Optimal Case Collection	= 3 Optimal Tumor Modules (6 FF/6 SC/12 SF/6 UF/3 CC) + 3 Optimal Normal Modules (6 FF/12 SF/6 UF)					
40% Average Case Collection	= 1 Optimal Tumor Module (2 FF/ 2 SC/ 8 SF/ 2 UF/ 1 CC) + 2 Optimal Normal Modules (4 FF/ 8 SF/ 4 UF)					
30% "Worst" Case Collection	= 1 Minimal Tumor Module (1 FF/ 1 SC/ 1 SF) + 2 Minimal Normal Modules (2 FF/ 2 SF/ 2 UF)					

# Life After Regulatory Approval: Biospecimens Throughout a Product's Lifespan

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## Diagnostic Tests / Laboratory Assays

### Standardized Benchmark Biospecimens

#### Define standards

- Test execution
- Tolerance for variation
- Test performance

#### Daily execution

- Quality Assessment
- Quality Control
- Calibration

- Decreased false negatives and false positives
- Improved Standard of Care





## The Standards for Personalized Medicine

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**“There is an opportunity for the NIH to be the ‘Statue of Liberty’ in creating a vision for how to collect, annotate, store and distribute samples in a standardized way.”**

**- Steve Gutman, FDA**



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# 3 Fundamental Factors That Drive Pricing

## Fit for Purpose – Relationship to Pricing

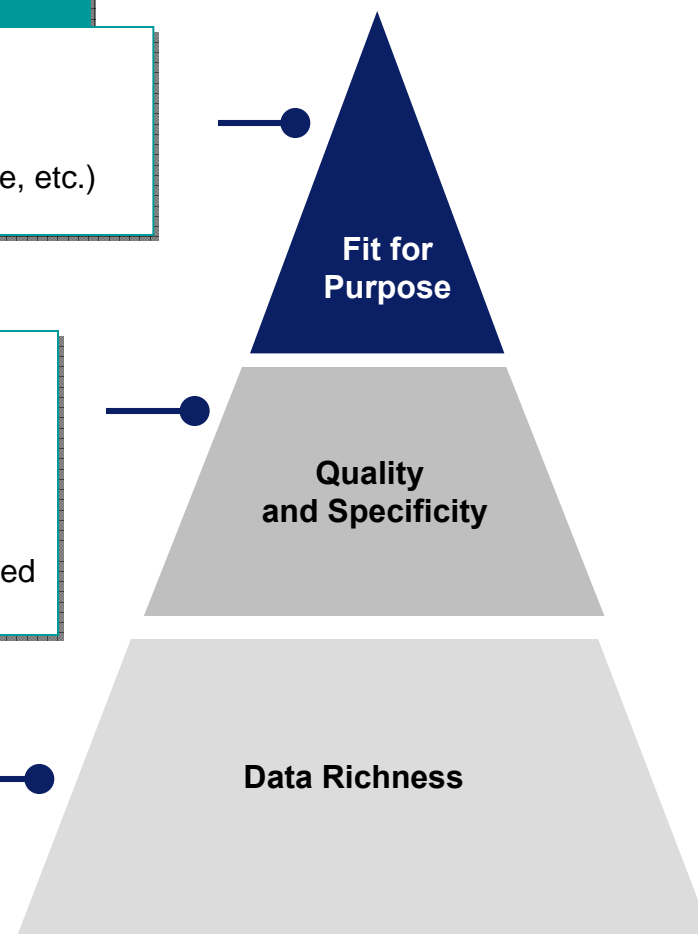
- ▶ The research application and scientific question being addressed
- ▶ Specifics of the collection protocol
- ▶ Specific project needs (e.g. normal, diseased, tissue origin, specimen type, etc.)

## Sample Quality and Specificity– Relationship to Pricing

- ▶ Quality and specificity price drivers:
  - ▶ Specimen rarity and size requirements
  - ▶ Extent of customized processing requested
  - ▶ Clinical parameters (e.g. treatments, etc.), and pathology parameters (e.g. tumor subtype, positive tissue markers) requested

## Data Richness – Relationship to Pricing

- ▶ Outcomes data are in high demand
- ▶ Comprehensive data sets may double sample price
- ▶ Customized data increases the sample price





## Importance of a National Biospecimen Resource Cited on Many Levels

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- **Genomics and Personalized Medicine Act of 2007**
- **Institute Of Medicine Report: *Cancer Biomarkers*, 2007**
- **Dept. of Health and Human Services, *Personalized Health Care Report*, Sept. 2007**
- **President's Council of Advisors on Science and Technology: *Priorities for Personalized Medicine*, Sept. 2008**
- **President's Cancer Panel Report, *Maximizing Our Nation's Investment in Cancer*, Sept. 2008**
- **Kennedy-Hutchison Cancer Bill (ALERT Bill: "War on Cancer, Part II"), 2009**
- **The NCI Bypass Budget for FY2010**

# caHUB and the Popular Press



## 8. Biobanks

By ALICE PARK

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Inside Huntsman Cancer Institute's vaults: Pancreatic tumors on ice. Lance W. Clayton for TIME

Folks at the National Cancer Institute (NCI) are heading up an effort to establish the U.S.'s first national biobank — a safe house for tissue samples, tumor cells, DNA and, yes, even blood — that would be used for research into new treatments for diseases.... By fall, the group hopes to have mapped out a plan for a national biobank; the recent stimulus showered on the government by the Obama Administration might even accelerate that timetable.



Time Magazine March 23, 2009

Time Magazine November 25, 2009