

**caHUB**  
The Cancer  
Human Biobank



## The Cancer HUMAN Biobank (caHUB)

**Carolyn C. Compton, M.D., Ph.D.**  
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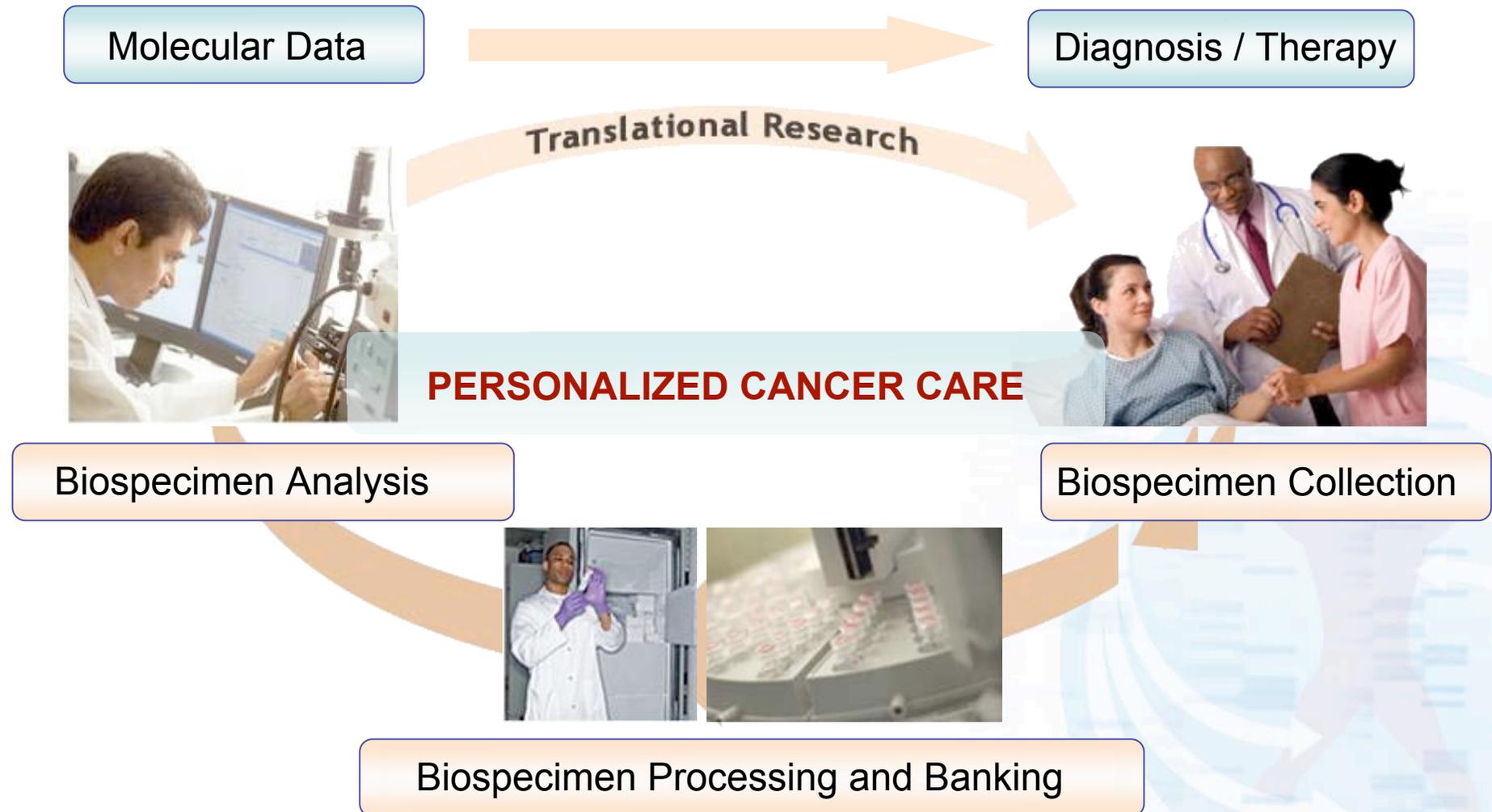
U.S. DEPARTMENT  
OF HEALTH AND  
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National Institutes  
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# Getting to Personalized Medicine



# Availability of Adequate Biospecimens

- A Roadblock to Co-Development

“A major hurdle for the co-development of a diagnostic test with a drug is the importance of obtaining and securing adequate specimens from patients in the clinical trials that can be used as evidence of drug efficacy and/or safety. ....Clinical trial specimens should be banked in optimal storage conditions to enable subsequent test development and/or retrospective hypothesis generation or confirmation of test performance. “

*U.S. Department of Health and Human Services, Food and Drug Administration.  
Drug-Diagnostic Co-Development Concept Paper 2005  
[www.fda.gov/cder/genomics/pharmacoconceptfn.pdf](http://www.fda.gov/cder/genomics/pharmacoconceptfn.pdf)*

“It is important to realize that biospecimen collection varies across populations; how they are handled and differences in sample processing variables can dramatically affect the results of a trial. Thus, appropriate condition for collection, handling, and storing study samples need to be standardized .... to preserve the stability and integrity of the analyte.”

*Chau, C.H., Rixe, O., McLeod, H., and Figg, W.D. Clinical Cancer Research 2008; 14(19) October . Validation of Analytic Methods for Biomarkers Used in Drug Development.*

## Why Is It Difficult to Acquire High-Quality Specimens and Data?

- 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**

# The NCI Addresses the Challenge

## Consensus of the Broad Scientific Community:

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

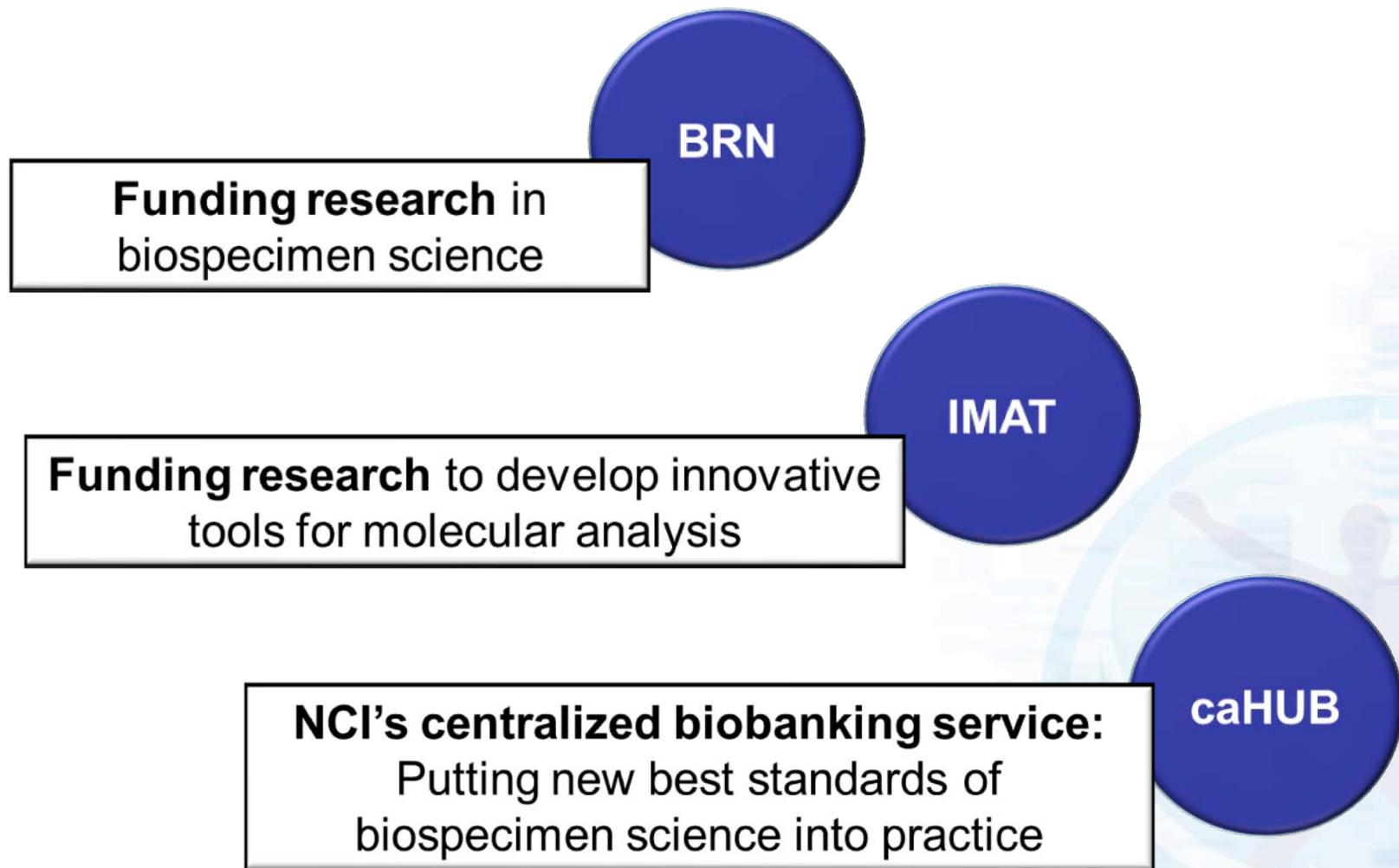
## The NCI Moves Stepwise Towards Solutions:

### Office of Biorepositories and Biospecimen Research

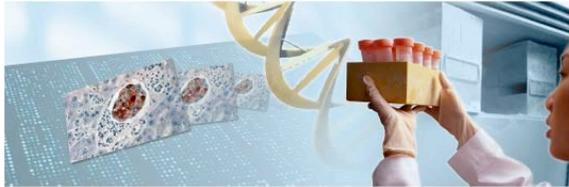
- Standards
  - *NCI's Best Practices for Biospecimen Resources*
- Science
  - The Biospecimen Research Network program
  - The Innovative Molecular Assessment Technologies program
- **Specimens and Service**
  - **The Cancer Human Biobank**



# OFFICE of BIOREPOSITORIES and BIOSPECIMEN RESEARCH



# Standards: NCI Best Practices for Biospecimen Resources



## National Cancer Institute Best Practices for Biospecimen Resources

June 2007

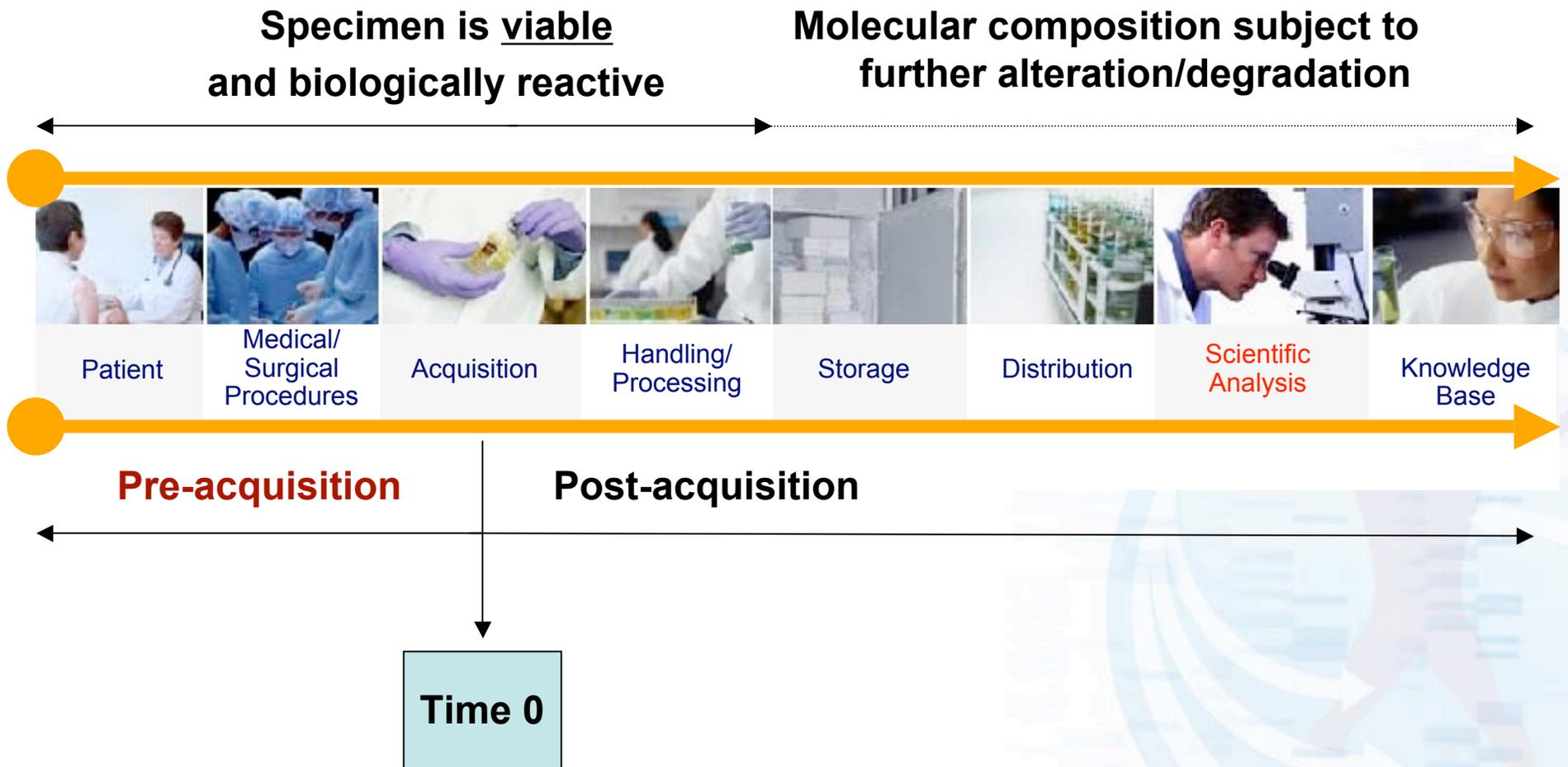
Prepared by:  
National Cancer Institute  
National Institutes of Health  
U.S. Department of Health and Human Services

### Objectives:

- Unify policies and procedures for NCI-supported biospecimen resources for cancer research
- Provide a baseline for operating standards on which to build as the state of the science evolves
- **Updated in September 2010**

<http://biospecimens.cancer.gov>

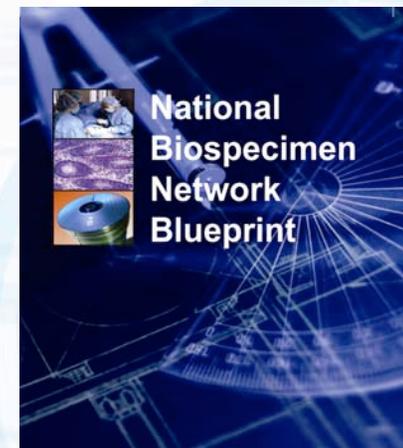
# Biospecimen Science: Moving Towards Evidence-Based SOPs



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

## 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 approached to ethical and legal issues
  - **Standardized consent, MTAs**
- Transparent governance and business models
  - **Transparent access policies**
- Large well-designed, standardized specimen sets



# National Biospecimen Resource: caHUB

A unique, centralized, non-profit resource that will provide high-quality biobanking services to ensure the collection of human biospecimens and associated data of measurable, high quality acquired within an ethical framework.

- **High-quality** samples and associated data
- **Prospective** scientific design of collection strategies
- **Standardized** processing and annotation of all specimens
- **Centralized** operations for QC, pathology analysis, storage
- **Transparent** access policies
- **Cutting-edge**: leadership for biospecimen resources (biobanking tools, biospecimen science, training and education)

# caHUB Collection Strategies and Standard Operating Procedures (SOPs)

## SOPs

- **Tissue collection SOPs** for each organ and cancer to be collected
- **Specimen qualification SOPs** will incorporate morphologic, morphometric, and molecular qualification metrics
- **Blood collection SOPs** will define collection and processing steps

## Quality management

- **Quality monitoring** will evaluate each step of process flow using well-defined quality criteria
- caHUB will identify critical process steps that require continuous, detailed monitoring
- All SOPs and QC procedures will be integrated into the caHUB **Quality Management Plan**

# caHUB, A Transformative Initiative

## 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 reconsent 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
- Ensures compliance reducing implementation and monitoring costs

## Stronger Clinical Correlation

- Quality and uniformity of data promotes more accurate modeling
- Avoided re-collection of data, saving time and cost

# caHUB, A Transformative Initiative

## More Efficient Product Development and Regulatory Approval

- Higher quality samples helps advance biomarker research
- Higher quality specimens helps reduce clinical trials timeframes and costs
- FDA recognition of “platinum” status specimens may lead to more rapid approvals for new drugs and diagnostics

## More Efficient Technology Development and Clinical Implementation

- Standardized biospecimens allow direct performance comparisons
- Standardized biospecimens allow calibration, performance monitoring and operator proficiency testing

## Added Clinical Value: Improved Standards of Care

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

## 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)**

# caHUB Timeline

## Planning

- Vision
- Business plan
- Policies
- Working groups
- Cost recovery
- Market research

## Phase 1

- caHUB pilot
- quality management
- procurement
- operations
- data coordination
- R&D
- ARRA support
- Proof of principle
- Public products

## Phase 2

- Re-assessment
- Expansion
- Economic analysis
- Special collections
- Partnerships
- Training services

# Pilot Phase activities of caHUB

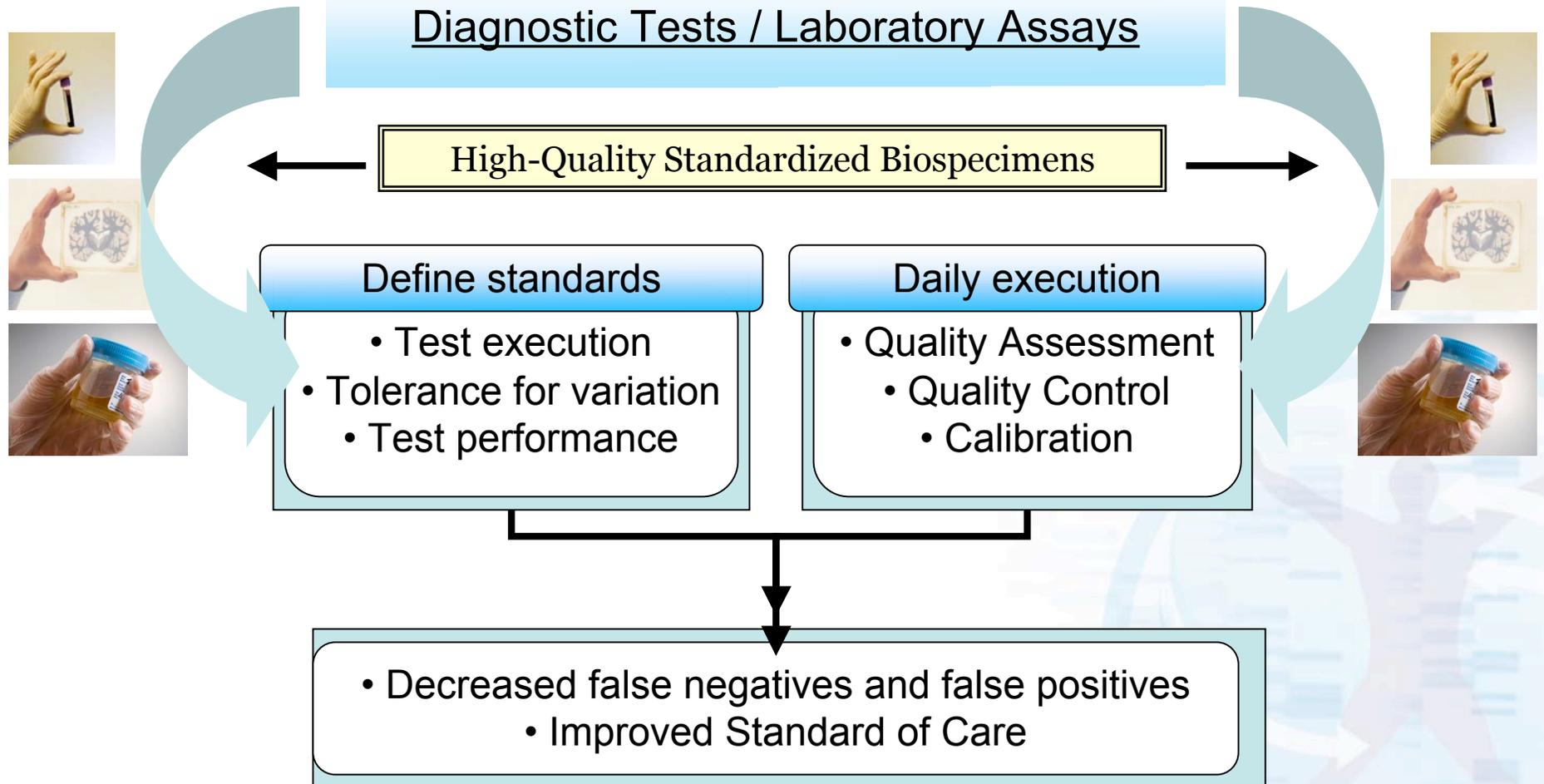
## ***\*Biospecimen sets for diagnostic assay development and validation***

Special collections of biospecimens to support specified research questions

Biospecimens for standards development, specifically in support of advanced genomics platforms

Biospecimen infrastructure support for clinical trials.

# Biospecimens Throughout a Product's Lifespan



## caHUB is Uniquely Posed to Provide Diagnostic Development Reference Sets

Collections of high quality biospecimens and associated data developed in accordance with regulatory guidelines, accompanied by histo-type matched, experimentally varied sets of biospecimens in the form of tissue microarrays (TMAs) will serve as the foundation for standardized, efficient and cost effective assay co-development.

- A unique network of collection sites procuring biospecimens under highly standardized protocols
- Biospecimen research infrastructure to create highly annotated experimentally varied reference sets
- Close relationships with regulatory and standardization federal agencies
- Government program serving the needs of the public

# caHUB Reference Sets Lower the Boundaries to Co-Development

- **Increased Efficiency** – Setting the standards
  - ✓ Standardized biospecimen and data reference sets create a common ground and facilitate translation of work performed in one step to the next
- **Reduction in Time to Market** - Standardized biospecimen and data sets
  - ✓ Lead to Reduction in repetition of research
  - ✓ Facilitate the production of performance metrics (sensitivity, specificity, dynamic range),
  - ✓ Reduce time and effort in regulatory approval process
  - ✓ Reduce time invested in products unable to achieve regulatory approval
- **Beneficial Economics**- Investment in common infrastructure
  - ✓ Leads to results that are translatable across research groups, products and platforms capitalizing on all previous investment
  - ✓ Facilitates independent assay validation to assure healthcare providers, regulators and payors of performance and accuracy
  - ✓ Enables development of accurate, reliable tests facilitating appropriate administration of costly therapies saving in drug costs, treatment time and improvement in health outcomes

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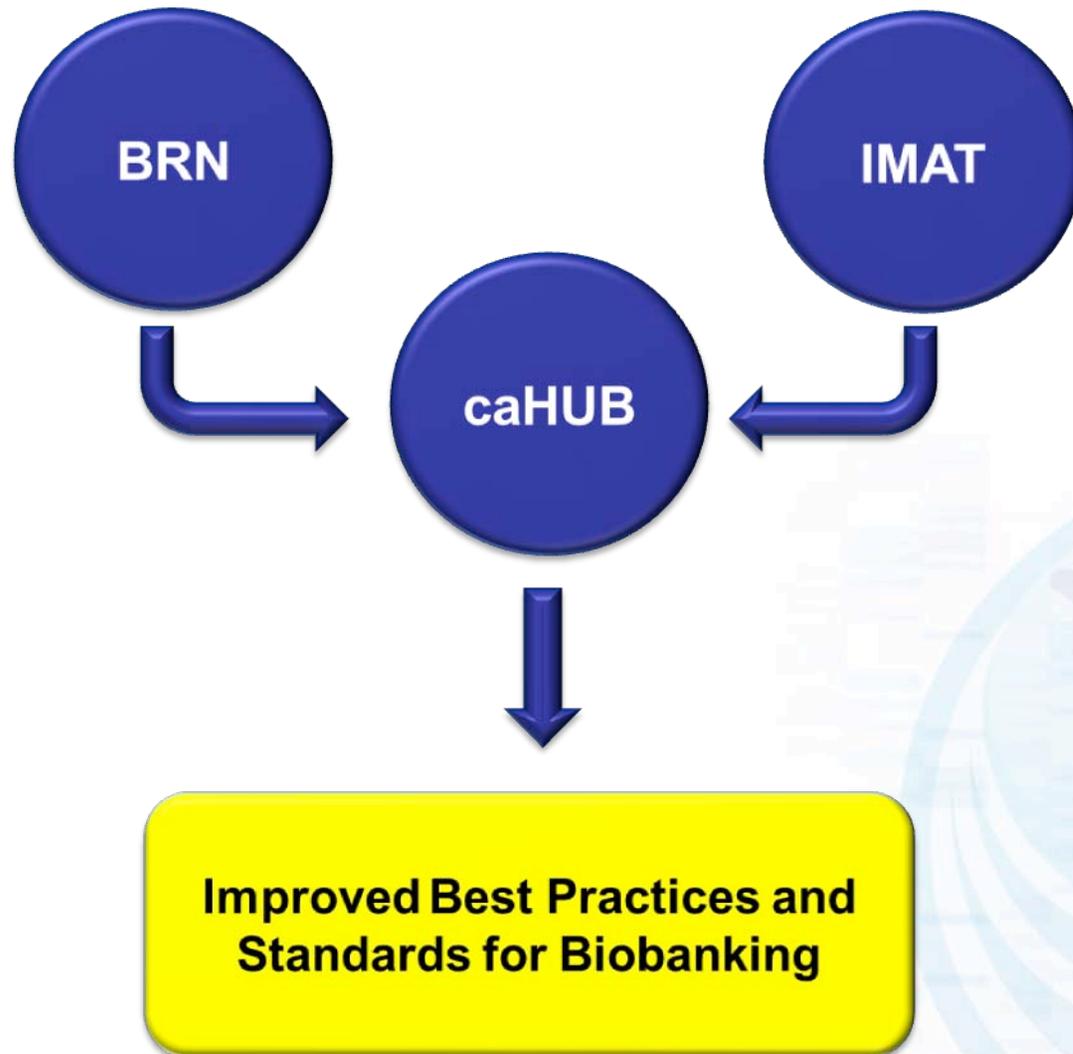
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# caHUB: Integration of New Principles of Biospecimen Science and Proof-of Principle



# caHUB Collection Design: Informed by User Need

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
  - Data associated with process variables
- 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 patients**

# caHUB: Collection and Distribution of Tissue and Associated Data

