



**OBBR**

Office of Biorepositories  
and Biospecimen Research

## **NCI Best Practices Forum**

***The Importance of Biospecimens in Cancer Research:  
It Begins with Patients and Ends with Patients!***

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**NATIONAL  
CANCER  
INSTITUTE**



# Today's Agenda

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**Introduction**

**The Current System**

**Challenges of the Current System**

**Solutions/Moving Forward**



# Key Definitions

**Biospecimen:** Tissue, blood, urine, or other human-derived material. A single specimen may generate several samples. (Also called aliquots)

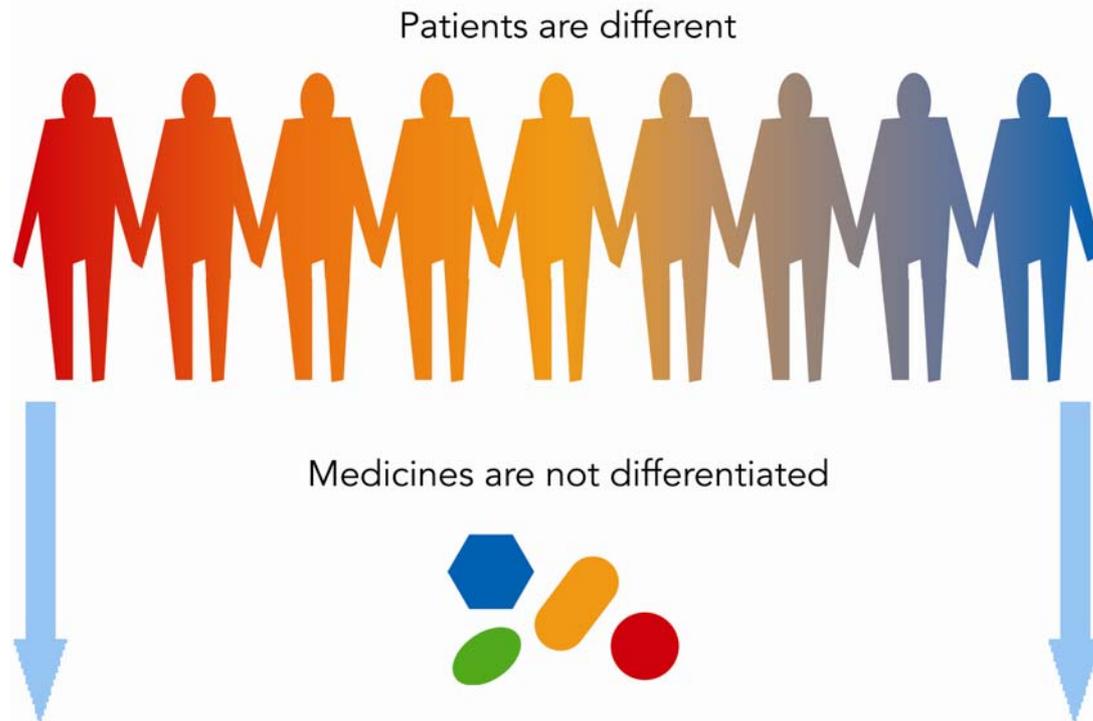
**Biospecimen Resource:** A collection of specimens and related data, the storage facility, and all relevant policies. (Also called: biorepository, biobank, tissue bank)

**Best Practices:** Standard operating procedures that are considered up-to-date and scientifically based and address all relevant ethical, legal, and policy regulations.

**Biomarker:** A substance (eg, a protein) sometimes found in the blood, other body fluids, or tissues that may indicate the presence of, susceptibility to, or extent of a disease.

# Today's Medicine Challenge: One Size Doesn't Fit All

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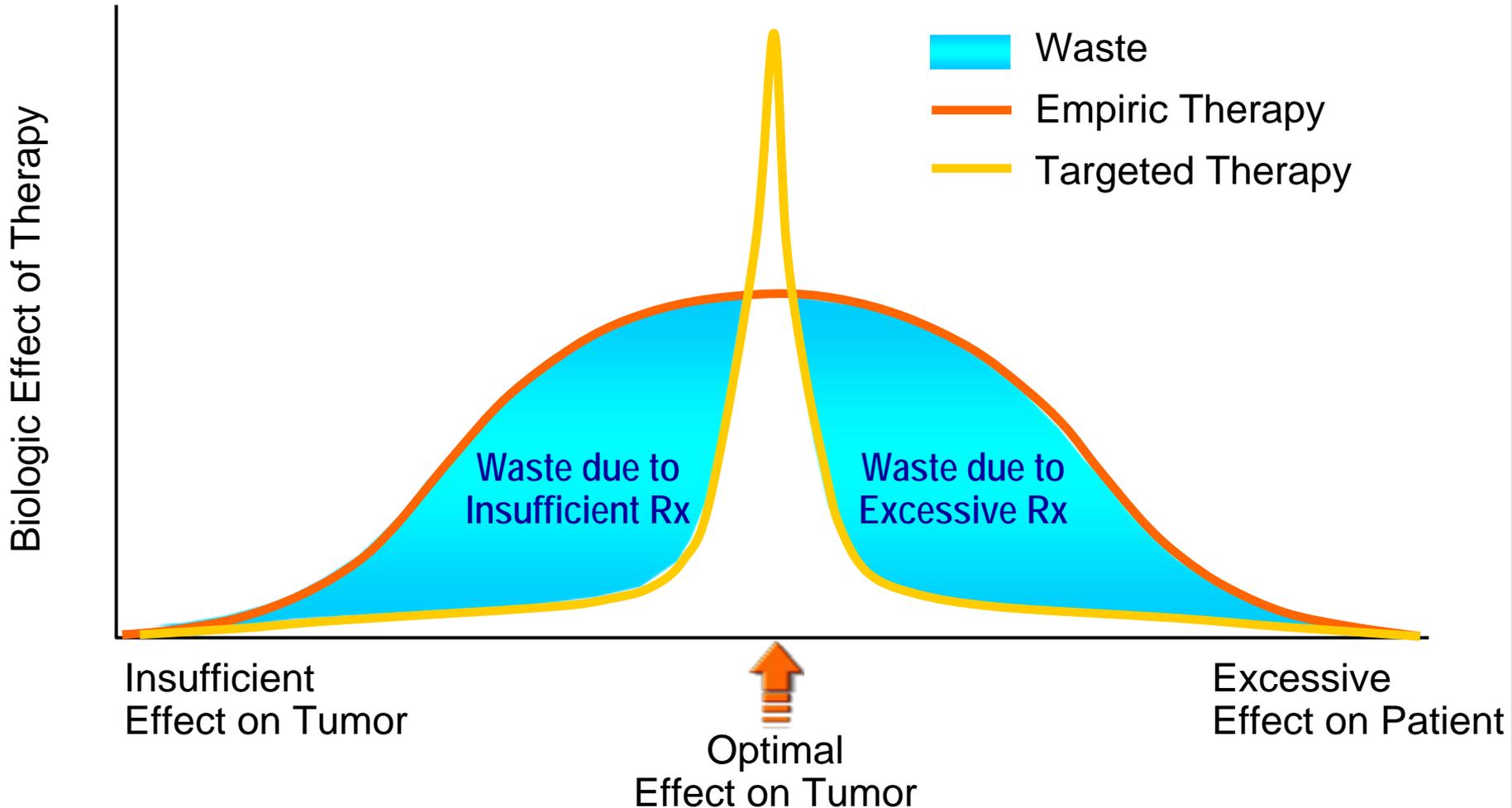


~ 30% of patients do not benefit from medicines<sup>1</sup>  
(100,000 deaths and 2.2 million nonfatal events from ADR in the US in 1994)

<sup>1</sup>JAMA 1998, 279: 1200

Source: Bayer HealthCare Diagnostics and Burrill & Company

# Improved Effectiveness with Individualized Oncology



# Key Definitions

**Molecular Medicine:** A branch of medicine that develops ways to diagnose, treat and prevent disease by understanding the way genes, proteins, and other cellular molecules work.

**Personalized Medicine:** Medical practices that are tailored to an individual patient and individual patient's specific disease based on the molecular characteristics of each.



Image courtesy of Science, May 26, 2006

# 21<sup>st</sup> Century Cancer Research Depends on Biospecimens

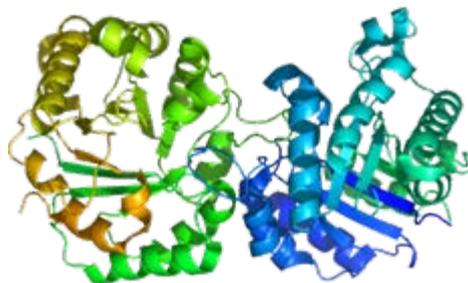
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## Finding the targets for detection, therapy, prevention

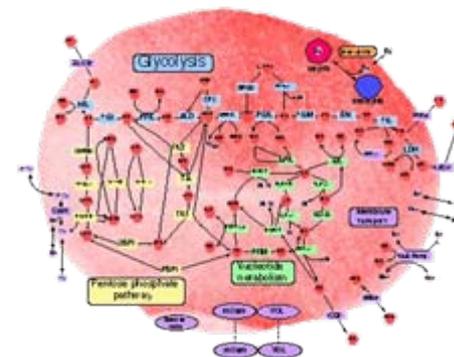
Genomics



Proteomics



Metabolomics



All Depend  
On High-Quality  
Human Biospecimens



# How Biospecimens Are Used in Today's Cancer Research

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**Specimens are needed to:**

- **Identify biomarkers (unique targets) in cancers**
- **Develop biomarker-targeted diagnostics and therapeutics**
- **Accelerate molecular medicine**
  - Herceptin targets Her2-neu; Iressa targets EGFR
- **Identify new uses for existing targeted drugs**
  - Gleevec → CML / Gleevec → GIST

# The Current System

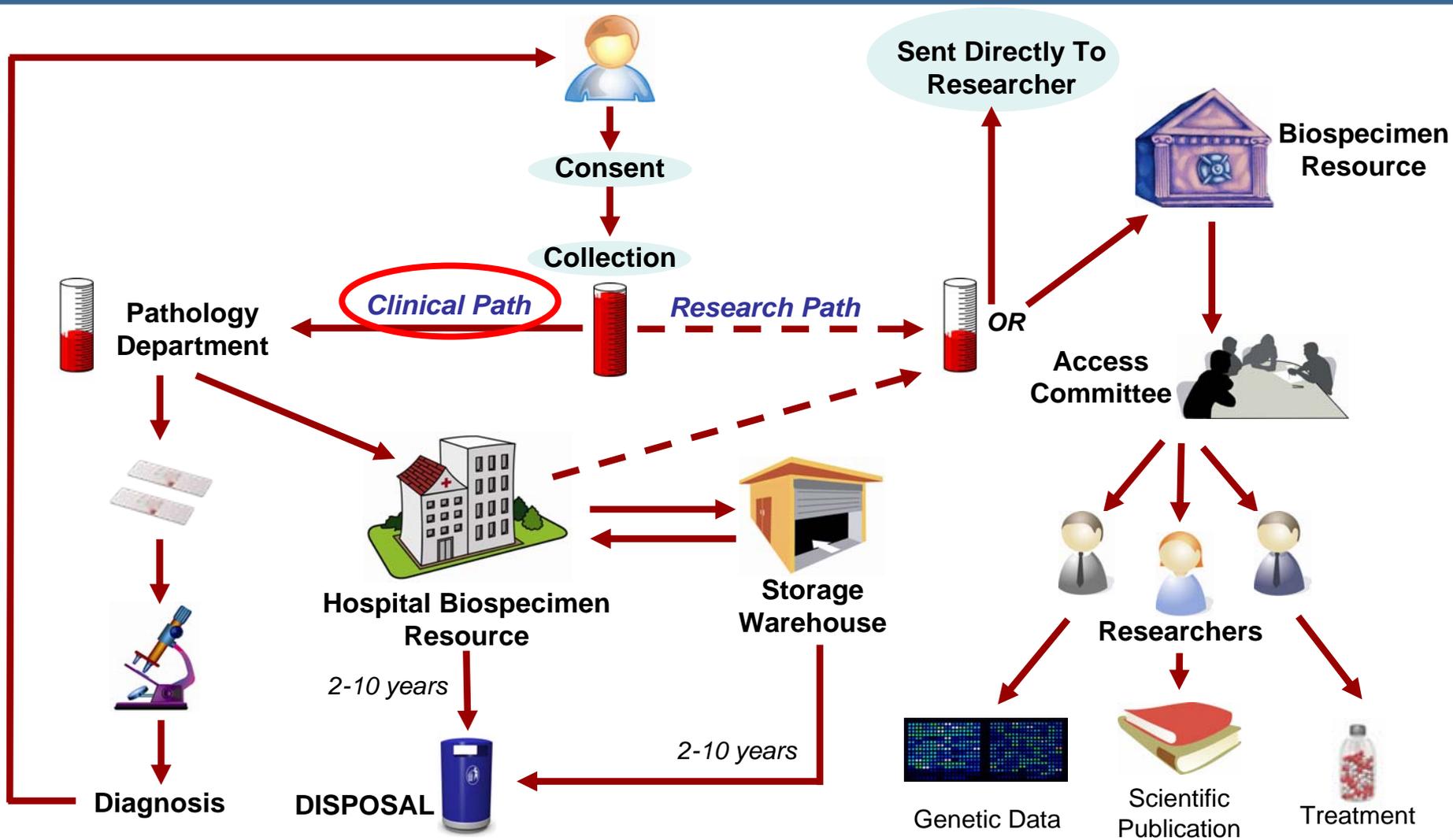
# The Basics

- Biospecimen Resource = Collections or “libraries” of diseased and/or normal human biospecimens
- Have existed for 100+ years
- Originated in pathology departments to confirm diagnosis and guide treatment pre/post surgery
- No national standards exist for biospecimen resources that collect and store specimens for use in research
- No regulatory body oversees biospecimen resources

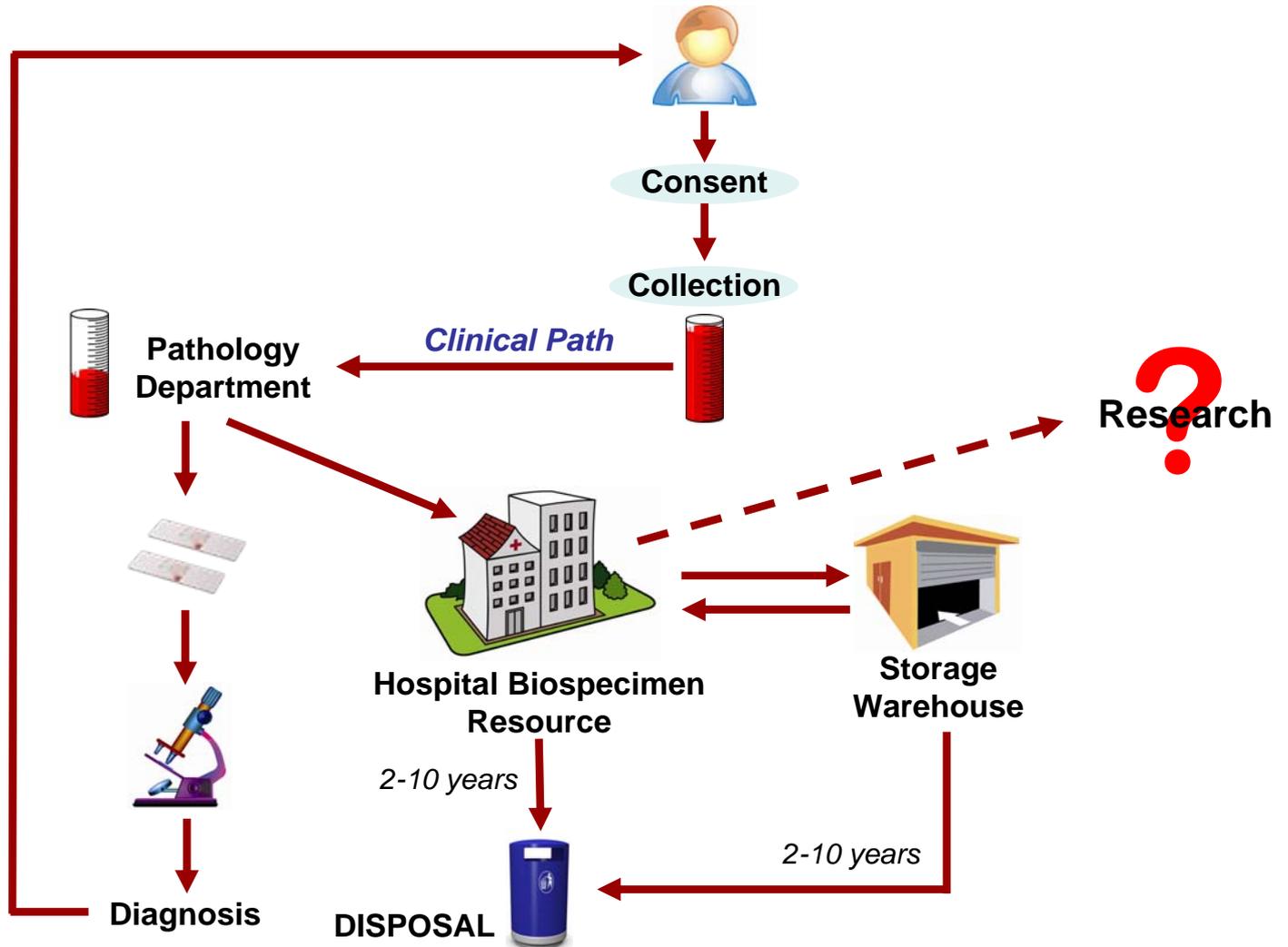
# Biospecimen Pathways

- After collection, there are two paths a biospecimen may follow:
  - ➔ **Clinical Pathway:** This path includes diagnosis and treatment. The clinical pathway benefits the individual patient.
  - ➔ **Research Pathway:** This path involves scientists doing research to enhance knowledge and advance cancer treatments. The research pathway benefits the broader population.

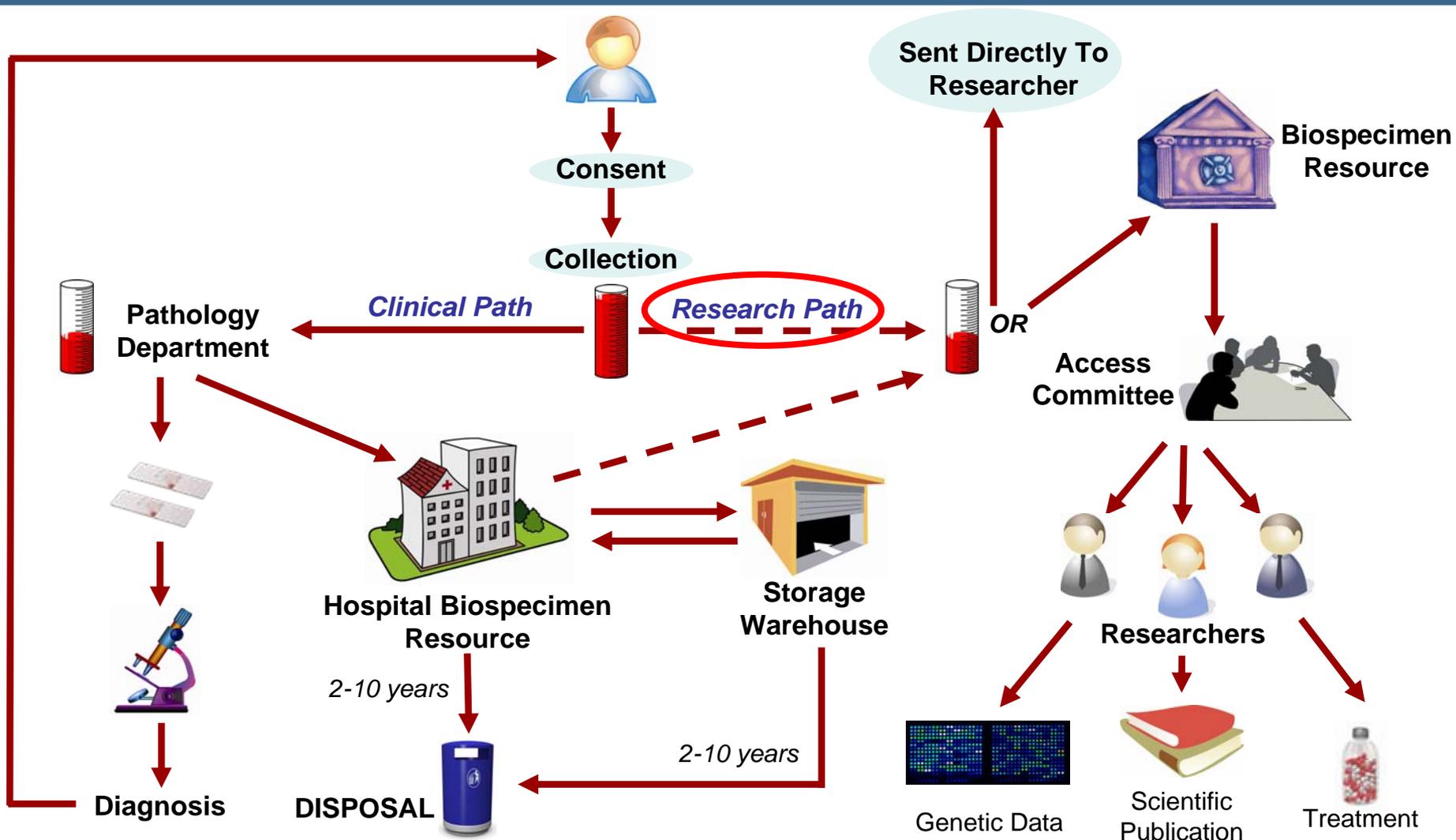
# Biospecimen Pathways



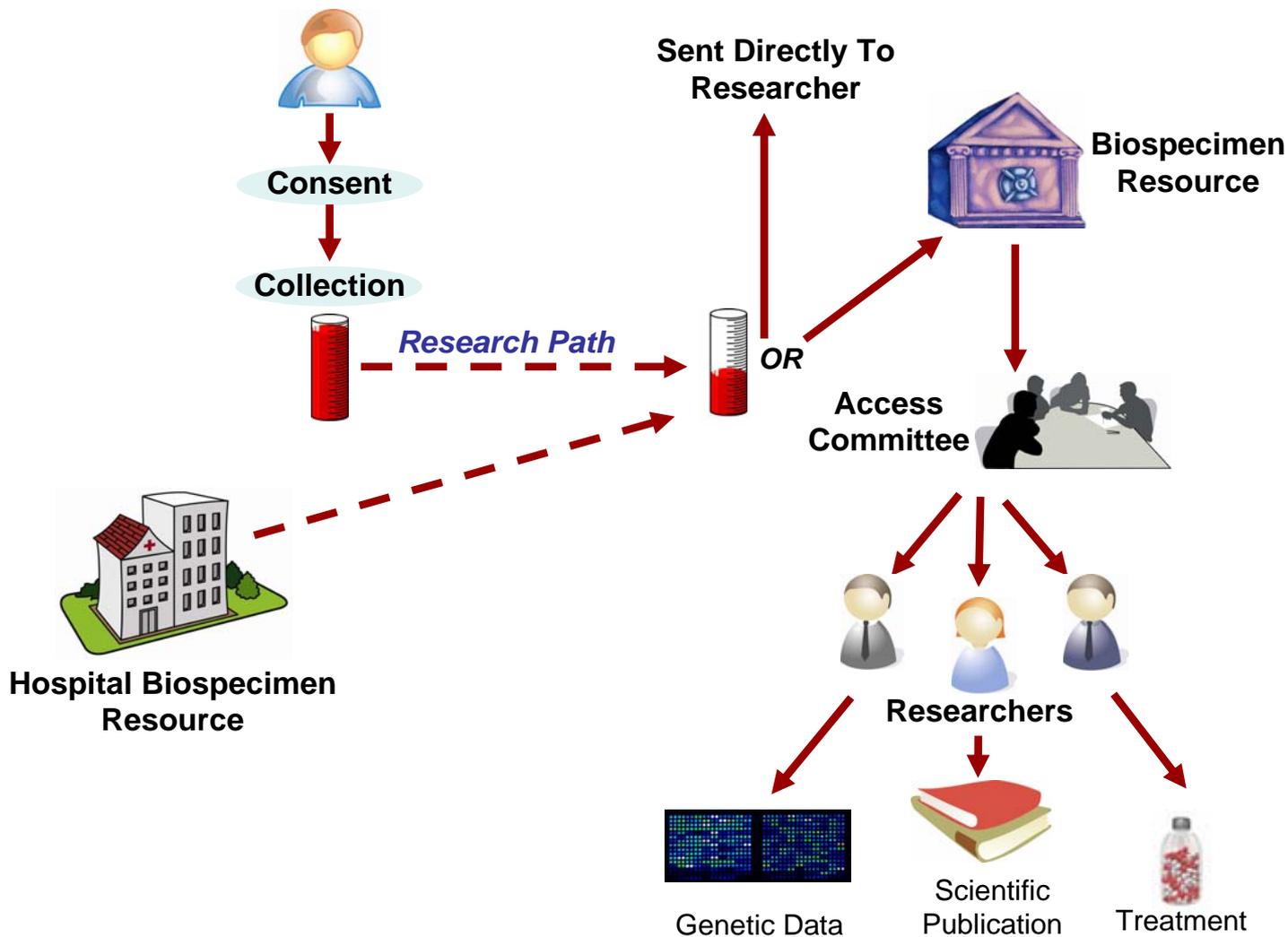
# Clinical Pathway



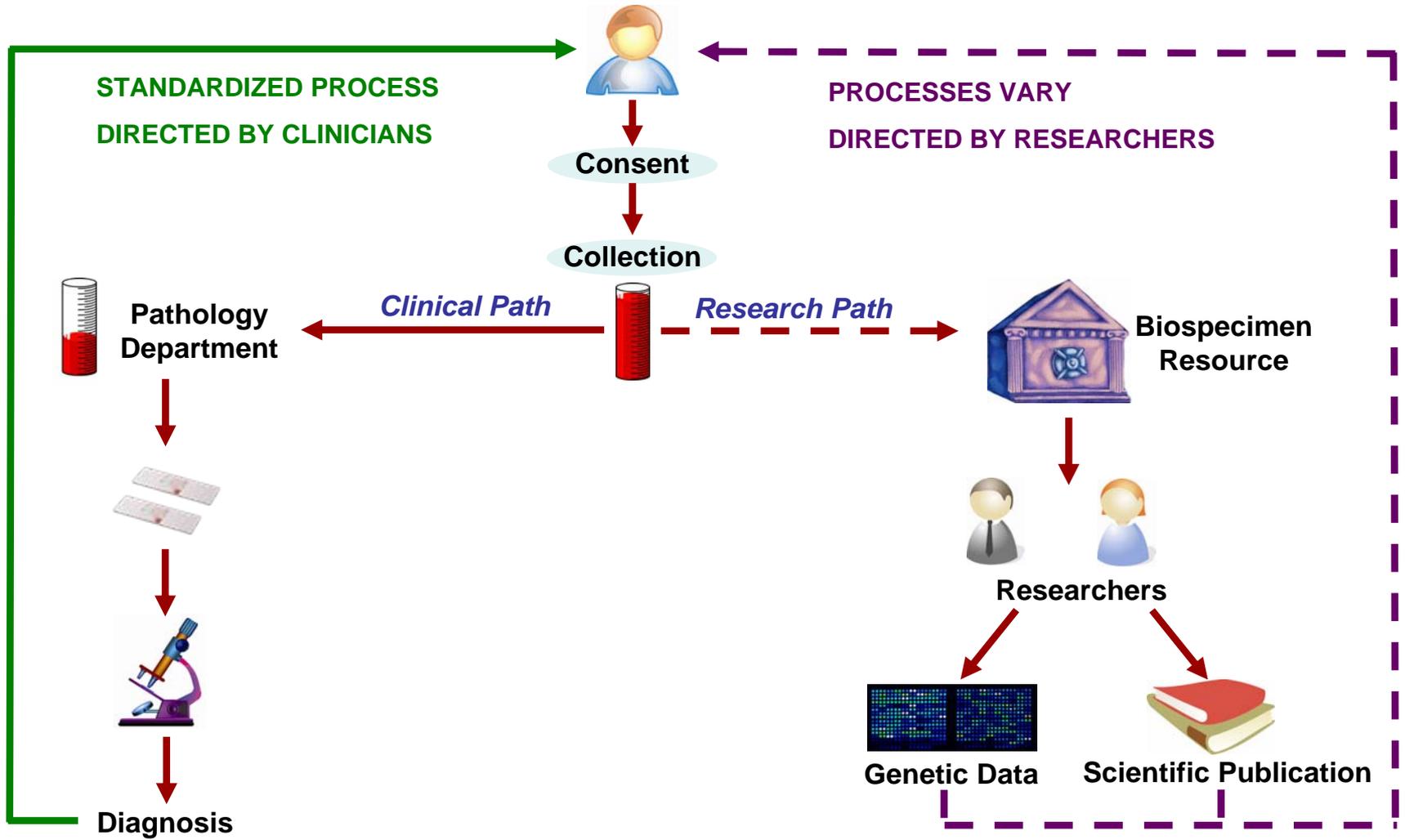
# Biospecimen Pathways



# Research Pathway



# What Happens to the Information from Your Biospecimen?



# Challenges of the Current System

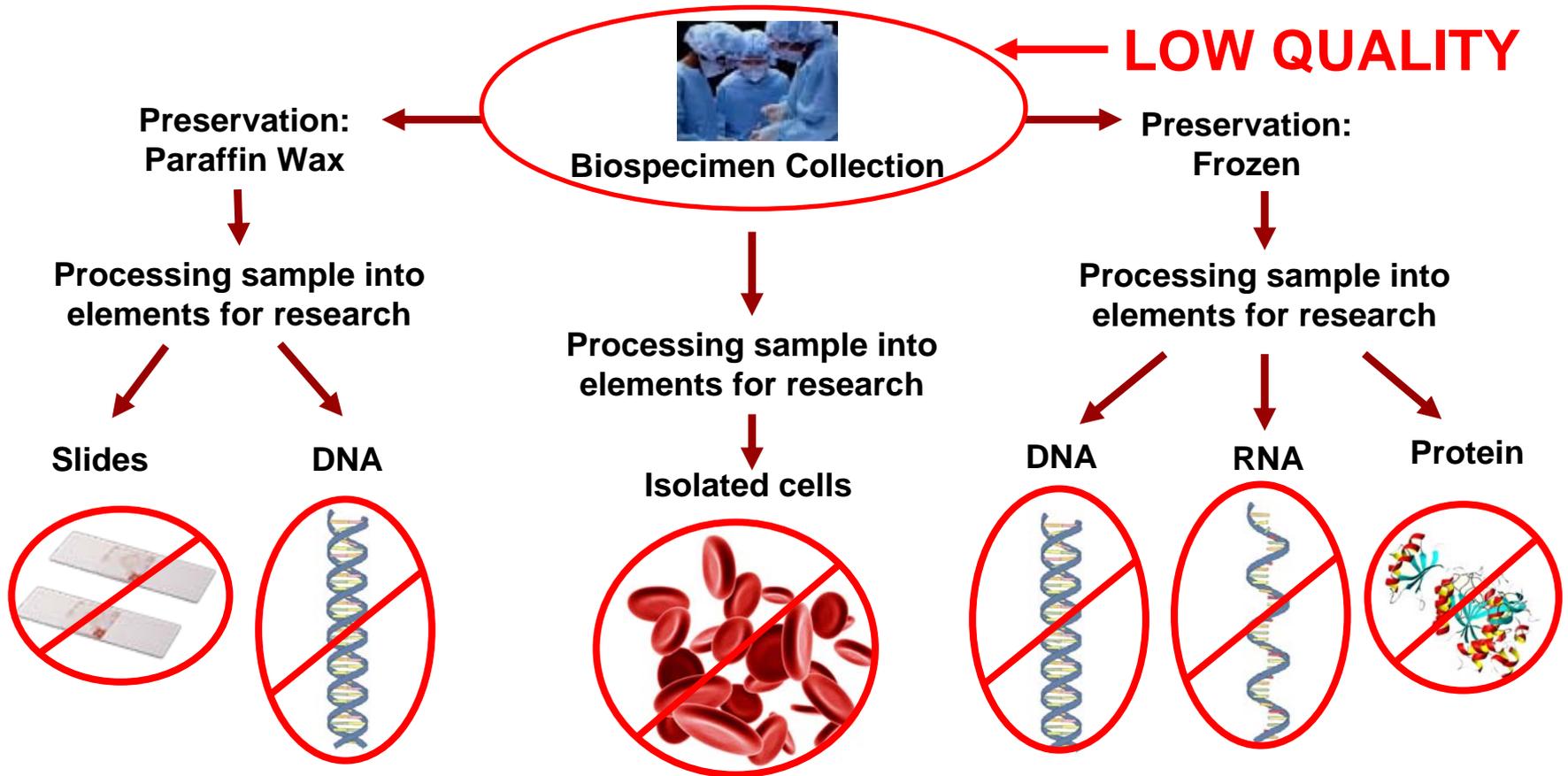


# Status of U.S. Biospecimen Resources

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- **300+ million specimens, but tissue is of unknown quality**
- **Many biospecimen resources exist, but no “network”**
- **Collection methods vary, no commonly agreed standards**
- **Approaches to patient consent & privacy protections vary**
  - ➔ Not all specimens are consented appropriately for today’s cancer research
- **Documentation of clinical data is limited and variable**
- **No common IT structure links resources together**
  - ➔ Difficult to exchange information
- **Limited access to specimens exists between institutions**

# Effects of Specimen Quality on Research



Low quality biospecimens impact multiple research efforts

## **THE GOLDEN RULE** Quality Biospecimens → Quality Research

The lack of standardization of human biospecimens compromises the quality and utility of research and the advances in cancer research that depend on them.





## Case Study: The Importance of Quality Biospecimens for Cancer Treatment

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- Herceptin is a drug used for the treatment of some breast cancers.
- Herceptin targets tumor cells that overexpress, or make too much of, a protein named HER2.
- Herceptin should only be given to patients whose breast tumors overexpress HER2.
  - About 20% of breast cancers overexpress HER2.
- The amount of HER2 protein in a tumor can be visualized by special techniques and scored from 0-3+. A higher score means that the patient is more likely to benefit from Herceptin therapy.



## Case Study: The Importance of Quality Biospecimens for Cancer Treatment

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- A 2006 study estimated ~20% inaccuracy rate in HER2 testing.
  - ➔ Some patients not receiving potentially beneficial treatment.
  - ➔ Some patients risking dangerous side effects when Herceptin is unlikely to help them.
- Lack of standard practices in specimen preparation and testing have contributed to inaccurate HER2 results.
- American Society of Clinical Oncology and the College of American Pathologists have addressed this with new standards.

# Solutions/ Moving Forward



# Goals of NCI's Biospecimen Efforts

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- **Prepare for changes in biospecimen requirements that are needed to:**
  - ➔ Conduct and advance molecular medicine
  - ➔ Drive personalized cancer medicine
- **Prepare for an increased need for biospecimens**
- **Remove a key barrier to cancer research:  
The limited availability of high-quality human specimens**



## **Background Research: Key Barriers Identified**

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- **Lack of common biospecimen resource SOPs, standards, and management principles across NCI-supported programs**
  - May limit impact of research programs
- **Lack of access to information on specimens available from the portfolio of biospecimen resources supported by the NCI**
- **No common database nor a defined mechanism to access biospecimens in NCI-supported programs**

# NCI Best Practices for Biospecimen Resources

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## National Cancer Institute Best Practices for Biospecimen Resources

NCI 2011

EDWARD C. KIM  
National Cancer Institute  
National Institutes of Health  
U.S. Department of Health and Human Services

## Objectives:

- Unify policies and procedures for biospecimen resources supported by the NCI or used by NCI-supported investigators
- Based on State of the Science as defined by 3 years of due diligence



# NCI Best Practices for Biospecimen Resources

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## Includes recommendations and guidelines for:

- Operational best practices for research biorepositories
- Quality assurance and quality control programs
- Establishing reporting mechanisms
- Providing administration and management structure
- Ethical, legal, and policy issues
- Informed consent
- Access to specimens and data
- Privacy protection – HIPAA
- Ownership of specimens
- Intellectual property



# What Do the Best Practices Mean for Patients?

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**Improved Biospecimen Process:** Best practices will help standardize processes for collecting and managing specimens.

**Increased Access:** Best practices propose a set of mutual principles for how biospecimen resources are accessed and managed, hopefully allowing broad access among researchers.

**Privacy Protection:** Best practices recommend measures to protect patient privacy and recommend that patients be told how their information will be protected during the informed consent process.



# How Will the Best Practices Benefit Cancer Research?

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Improving the quality of biospecimens →

**More reliable research results**

Standardized practices →

**Results will be more comparable across studies  
and researchers will be able to use multiple  
biospecimen resources within a single study**

Standardizing access policies and encouraging  
sharing of resources →

**Greater research access to specimens**



## Why This Is Important and Urgent

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- **Large research initiatives (i.e., The Cancer Genome Atlas, TCGA) are underway**
  - ➔ Each will require large numbers of high-quality cancer and healthy biospecimens with clinical documentation
- **Cancer research is becoming tied to biomarkers found in biospecimens, and the accuracy/reliability will drive the next generation of diagnostics and therapeutics**
- **The progress being made towards reducing the burden of cancer depends on the efficiency and accuracy of these and other translational research initiatives**