The Importance of Best Practices to Patients, Survivors, Advocates, and the General Public

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December 3, 2007
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It’s All About the Patient: Putting Biospecimen Research In Perspective

Paula Kim, TRAC- Translating Research Across Communities
NCI Biospecimen Best Practices Forum
November 5 2007, Conference Center at Harvard Medical School
OBBR NCI – NIH, U.S. Department of Health and Human Services
HOPE

Genomics
Proteomics
Pharmaco-genomics
From the standpoint of medicine as an art for the preparation and cure of disease, the man who translates the hieroglyphics of science into the plain language of healing is certainly more useful.

-William Osler, Physician
100 Years and Counting...

• Taken over a century, and now biospecimens, technology, and science for patient benefit is coming of age

• Biospecimens are precious human resources filled with unique genetic information

• The privilege of using biospecimens to advance research into clinical application brings great responsibility not to be taken lightly

• Biospecimens are part of resource toolkit
U.S. Cancer Mortality Rates

Getting a New Product Through This Process Is Not Easy... Many Years and Lots of $$$

Courtesy of The Coalition of Nat. Cancer Cooperative Groups
Patient Dilemma with Words and Decisions…

Invasive? Non-invasive?
Academic Center? Local?
Informed Consent?
How to pick treatment?
Do my kids need genetic testing?
Where is my tissue?
Eligible? Insurance?
Am I Going to Live?

Personalized medicine?
Biospecimen?
Biopsy? 2\textsuperscript{nd} pass?
Targeted Therapies?
EGFR Inhibitor?
Proteomics?
The Promise

• Personal Targeted Therapies
• Seek and Control instead of Search and Destroy
• Genetic Testing
• Reduced Toxicities
• Predictive/Preventive Capability
• Reduced drug failure rates
• A CURE

Hype or Not?
All Drugs Are Targeted Therapies

- One problem is the numbers of **compounds** AND **targets** known to work together well are few and far between…here are a few examples of progress…
  - Trastuzumab-Herceptin® → HER 2
  - Cetuximab-Erbitux® → EGFR
  - Imatinib mesylate-Gleevec® → KIT
  - Erlotinib-Tarceva® → EGFR
Connect The Dots

Patients, Volunteers, Groups & Public

Academic & Community

Public Agencies & Policy Makers

Industry & Private Sector

B = Biospecimen
Why We Need To Keep Moving In the Right Direction

Information is not knowledge
• Competing Priorities
• More Survivors
• Shrinking Budgets- Expanding Costs
• Increased Regulations
• Fast Moving Science
• Requirements and Expectations
| Federal Law | State Law |
| HIPAA; IRB; | Common Rule |
| Black Letter Law | Common Sense |
| Benefit | Risk |
| Simplification | Complication |
| Misuse | Appropriate |
| No patient uptake | No participation |
| No biospecimens | No research |
| No progress | No answers |
| No cures | No hope |
Consequences of Poor Practices -

- False + and False -
- Inaccuracy; non-reproducibility
- Erodes public confidence
- Wastes time & money
- Impedes clinical benefit
- Takes another 100+++ Years
Earn Public Trust & Confidence

- Confidence in you and your team
- Confidence in the reason
- Confidence in the test
- Confidence in the system
- Confidence in the procedure
- Confidence in the test results
- Confidence in the diagnosis
- Confidence in the recommendation
- Confidence in the outcome
- Confidence…period
Biospecimen Best Practices Are More Than Just Freezer Temperature and Hold Time

- Partnering with the public and community
- Privacy and patient protection, cultural sensitivity
- Data Sharing and bioinformatics
- Handling of biospecimens
- SHARE, promote and provide research access
- Balancing Intellectual property
Imagine making choices and decisions that affect our lives... without access to guidance, information, and resources?

Imagine trying to build your dream house without well designed plans and standards or a qualified architect or builder.

**BEST PRACTICES, STANDARDS AND BLUEPRINTS ARE CRUCIAL WHETHER IT’S A HOUSE OR A BIOSPECIMEN!**
Commitment for:
Infrastructure + resources + best practices can yield high-quality annotated biospecimens

The Potential Upside

Data + specimen with integrity becomes Information into knowledge that puts real discovery into real life in real time for patient benefit
Examples of Novel Biospecimen Partners
In Progress

- Friends for Life study
- Arizona Myeloma Network
- Research Advocacy Network patient and IRB education materials
- Genetic Alliance Biobank (e.g., IBCRF) – Broad Based, Rare diseases
Research Advocates: Partners In Progress

- Scientific committees
- Clinical trial design
- IRB; Data Safety Monitoring Board
- Grant Review
- Biospecimens
- Bring The Patient Perspective
“We must harness the scientific, industrial, and social will to accelerate discoveries into accessible and meaningful clinical applications. Patients, families, friends, and loved ones are counting on us...Because in the end, it’s not about you, or me, or the science, it’s about the patient.”

Paula Kim
Research & Patient Advocate
Patient and Advocate Perspectives
Don’t Leave Home Without Them

Research Advocacy Network
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