

Institute of Medicine
National Cancer Policy Forum

Policy Issues in the Development of
Personalized Medicine in Oncology

**TECHNOLOGICAL HURDLES:
A PATIENT ADVOCATE PERSPECTIVE**

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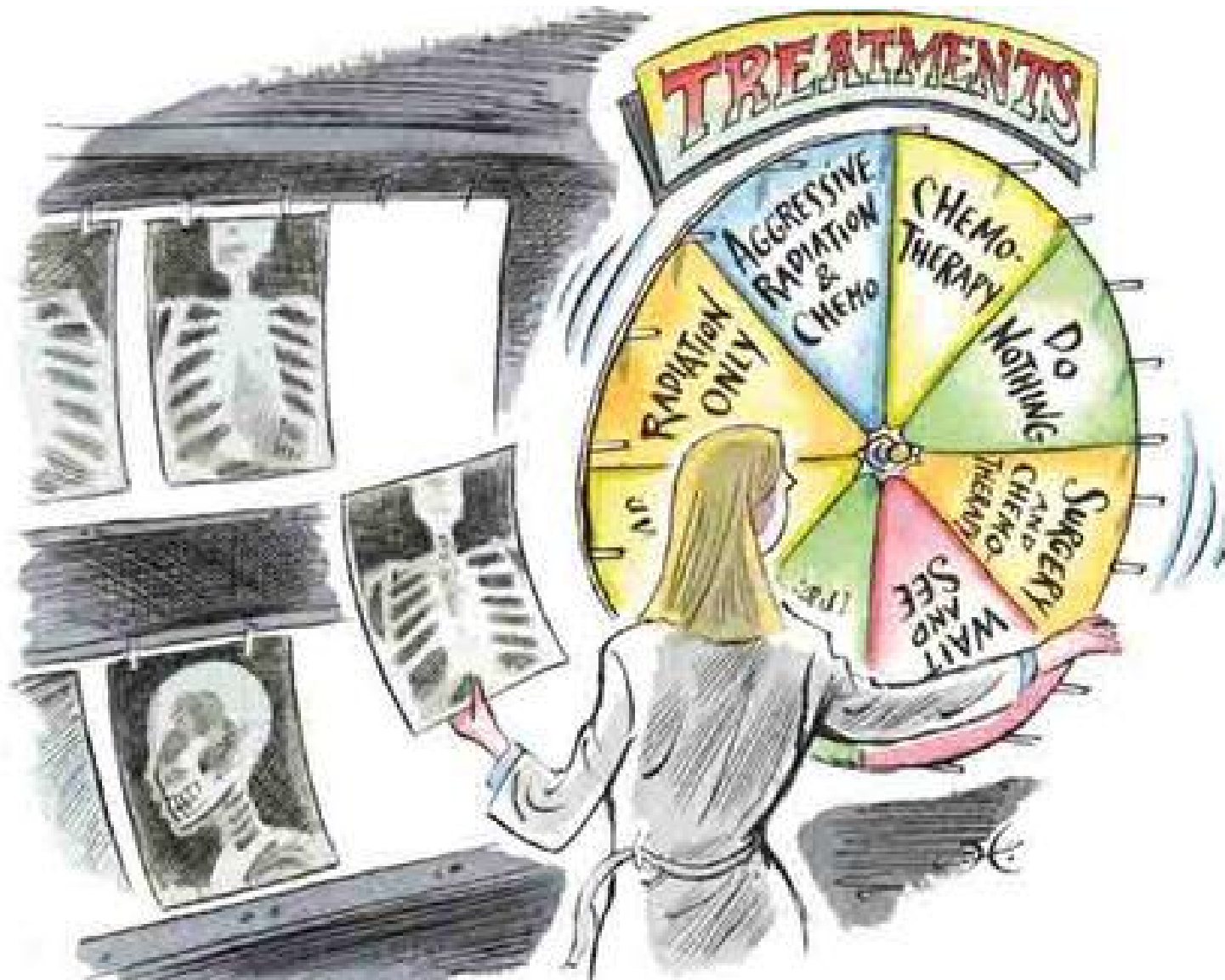


National Breast Cancer Coalition

- Mission: To end breast cancer
- Three goals
 - § Research: innovation and collaboration
 - § Access: guaranteed access for all
 - § Influence: trained advocates at every table
 - § Focus on evidence based decision making and system change



Breast Cancer Treatment: Choices Choices Choices



Personalized Medicine

- The promise of personalized medicine
 - § Tailored to unique characteristics of patient
 - § Certain of benefit
 - § Minimal risk
 - § Avoid that which will not help
 - § Affordable

Patients' Perspective

- Goal of any intervention, personalized or not:
 - § Effective: reduce mortality, preserve quality of life
 - § Safe: recognition of price cancer patients pay to achieve any benefit
- Expectation:
 - § Clinical intervention based on highest level of evidence and reflects changes in knowledge

Patients' Perspective

- Level of evidence to support?
 - § How to design trials?
 - § What is the clinical utility?
 - § What are the outcomes?
- Are there policies to protect individuals from discrimination?
 - How do we educate the medical community?
 - Will the cost be too high?

Level of Evidence

- When is something ready for the clinic?
 - § What level of evidence and clinical impact should the public expect before an intervention is introduced into the clinic?
- When do we know enough to take something away?
 - § What level of evidence should be acceptable to remove an intervention?

Personalized Medicine

- Technology is sometimes ahead of utility
 - § Tests that tell us information we cannot act on, or incomplete information
 - § Therapies with minimal benefit for entire population, and no test to define subpopulation that could benefit
 - § Great cost to individuals and to system - with little societal value

Breast Cancer Targeted Therapies

Examples of Personalized Medicine

- Trastuzumab (Herceptin[®])

Reality for Breast Cancer Patients

- Approximately 20% of breast cancer is Her2/neu positive
- There is a 50% relative risk reduction in recurrence for patients adding Herceptin[®] to chemotherapy
- Mortality reduction of one-third
- Questions of resistance remain -- what is the biology of breast cancers that do respond? Unknown at this time.
- Cost is high

Reality for Breast Cancer Patients

- Questions remain about testing:
 - § Controversy over which test: FISH vs. IHC
 - § Discrepancies among laboratories in quality
 - § How to determine cut offs?

Reality for Breast Cancer Patients

Educate Medical Community

§ United Healthcare Data Found:

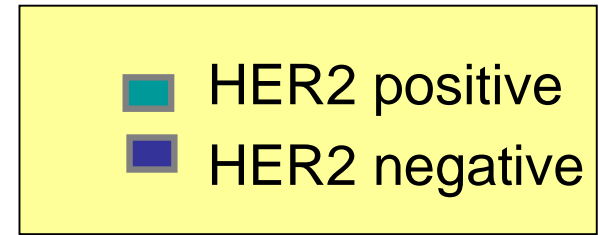
- 12% of women receiving Herceptin® never had a test at all
- There are hundreds of ways of reporting Her2 testing
- Example: Oncologists prescribed Herceptin to women based on pathology reports they assumed were Her2 positive – but they were 2+ and not 3+
- **Study retesting local Her2 lab results in central reference labs:**
 - 15% of women called 3+ were really negative
 - 10% of women called negative were really positive

Adding a 10% error rate - at least a third of women were being prescribed Herceptin based on invalid evidence.

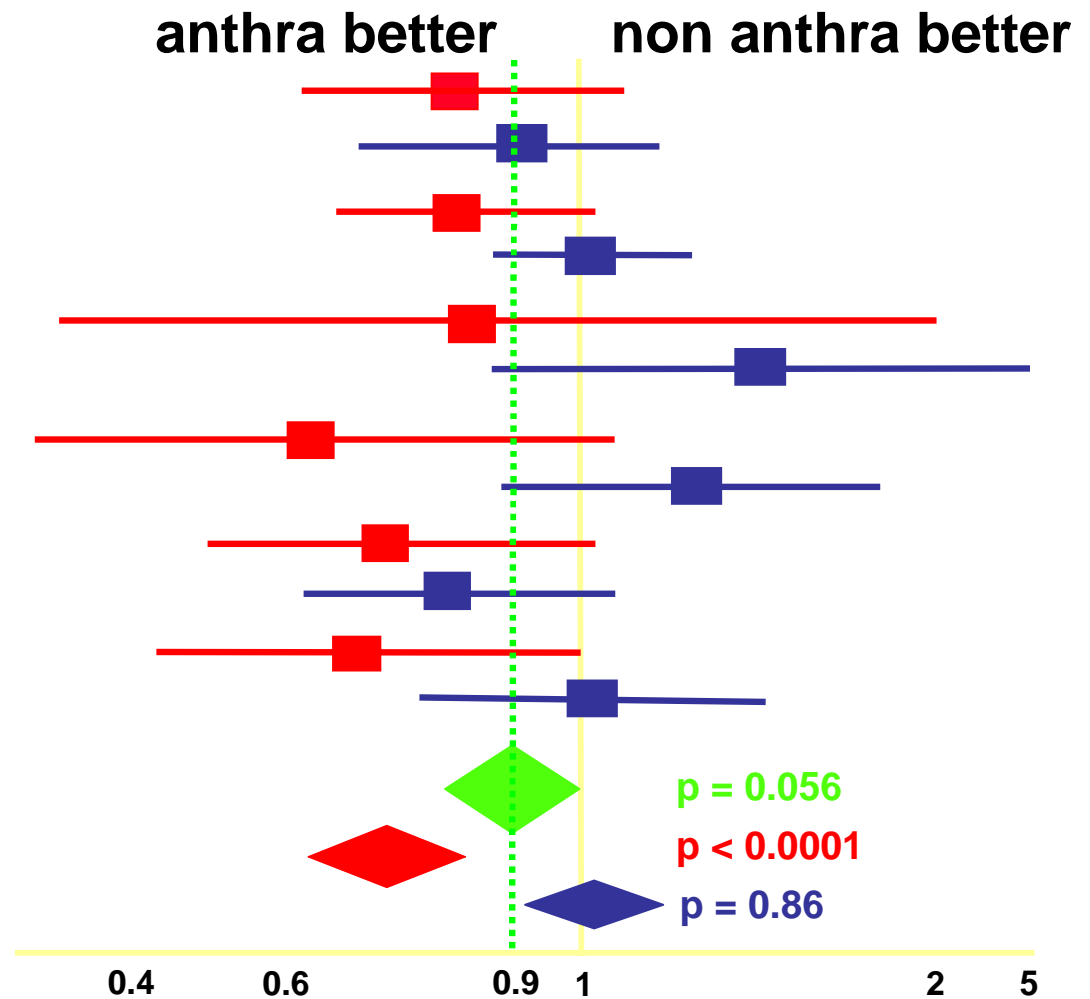
Reddy et al, Clin Breast Ca, 153-157



Overall Survival



Study	HR	95% CI
NSABP B11	0.66	0.47 - 0.92
	0.90	0.69 - 1.18
NSABP B15	0.82	0.63 - 1.06
	1.07	0.88 - 1.30
GUN 3	0.85	0.27 - 2.69
	1.64	0.85 - 3.15
Milan	0.61	0.32 - 1.16
	1.26	0.89 - 1.79
DBCG-89-D	0.73	0.50 - 1.05
	0.82	0.59 - 1.13
NCIC MA-5	0.65	0.42 - 1.01
	1.06	0.80 - 1.40
Total	0.91	0.83 - 1.00
Overall	0.73	0.62 - 0.85
	1.03	0.92 - 1.16



heterogeneity $c_{25} = 5.2$, $p = 0.39$

heterogeneity $c_{25} = 5.5$, $p = 0.36$

Test for interaction $\chi^2 = 12.0$, $p < 0.001$



Personalized Medicine: Reality for Breast Cancer Patients

- All but a few guidelines include anthracyclines
- Institutions have not moved away from this treatment
- Clinical trials include it in design

NBCC Recommendations for Moving Biomarker Research and Implementation Forward

Demonstrate clinical utility.

Ensure the tests themselves are dependable.

Continue the quest to understand the biology of disease.

Use sound methodologies to test in the clinic.



NBCC Recommendations for Moving Biomarker Research and implementation Forward

Develop agreed-upon standards and guidelines for conducting research, reporting results, and using biomarker data in clinical practice.

Co-develop tests and interventions.

Provide health care to all.

Don't neglect prevention.



Don't oversell it.



THE SCIENCE NEWS CYCLE

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Start Here

Your Research
Conclusion: **A is correlated with B** ($p=0.56$), given C, assuming D and under E conditions.



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POTENTIAL LINK
BETWEEN A AND B
(UNDER CERTAIN CONDITIONS).



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SCIENTISTS.



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Always remember why we do this.



