Evidence of Clinical Utility of Molecular Diagnostics in Oncology: A Workshop

May 24, 2012
PEW Conference Center

INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

CMTP
Center for Medical Technology Policy

Roundtable on Translating Genomic-Based Research for Health

Clinical Practice
Public Health

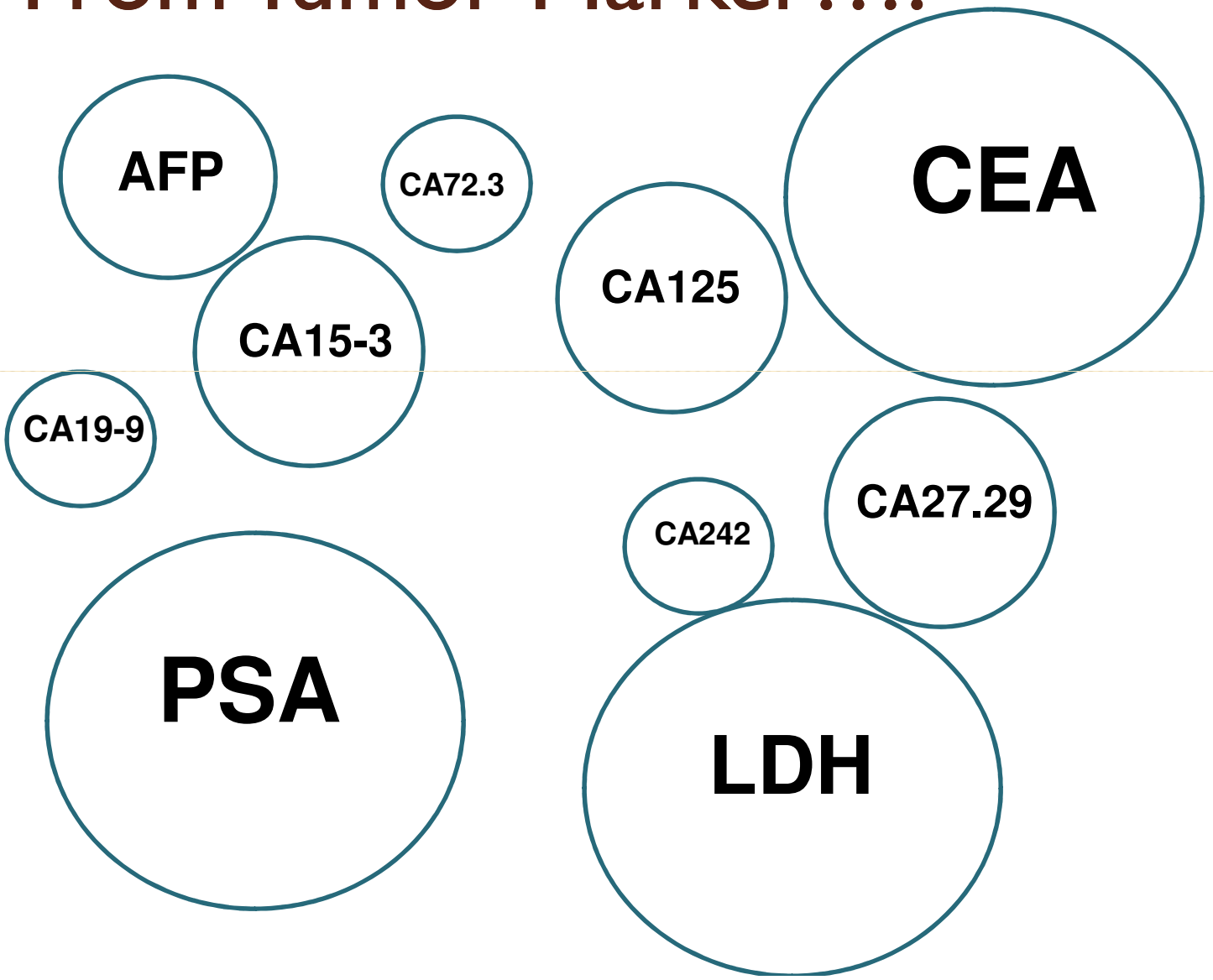
Overview

Drug
Development

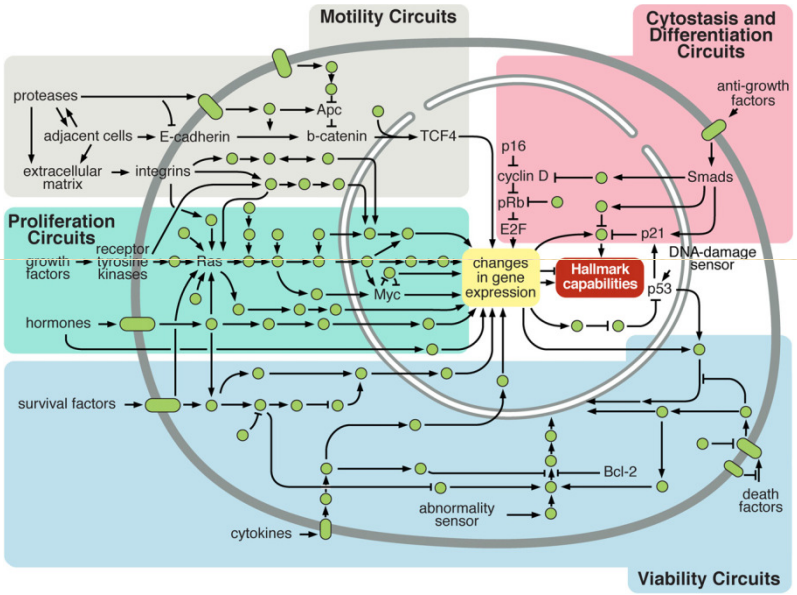
Diagnostic
Applications

"The purpose of the Roundtable is to explore and implement strategies for improving health through the translation of genomic and genetic research findings into medicine, public health, education and policy"

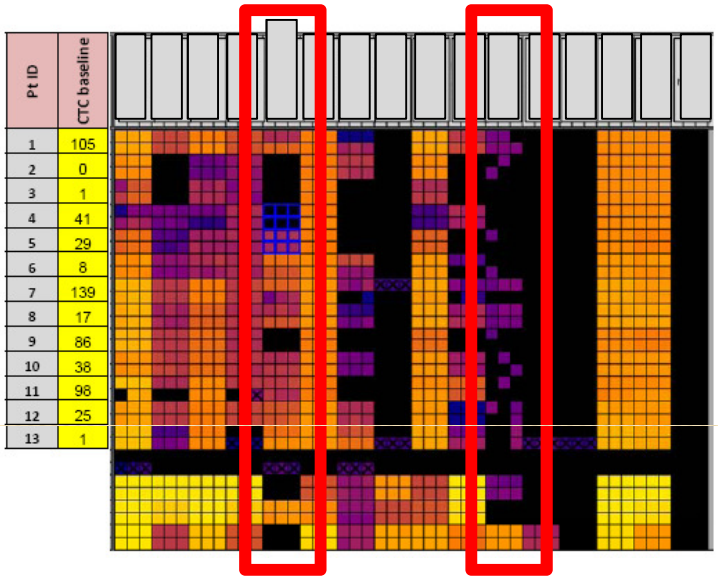
Situation:
From Tumor Marker….

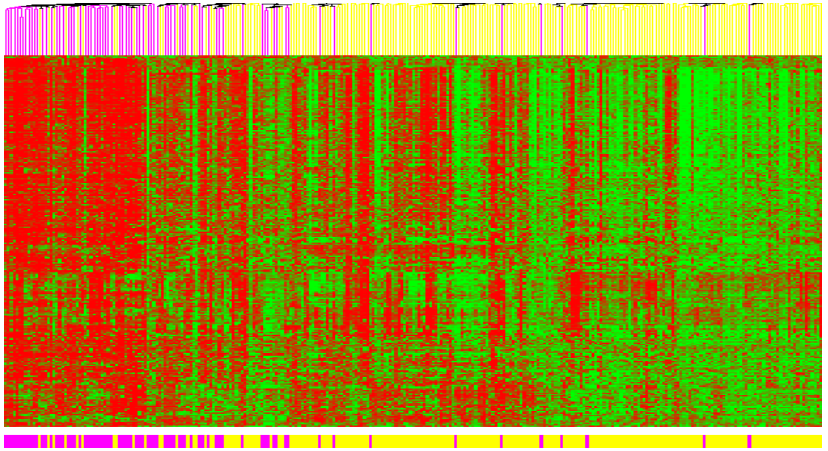


…to Biomarker

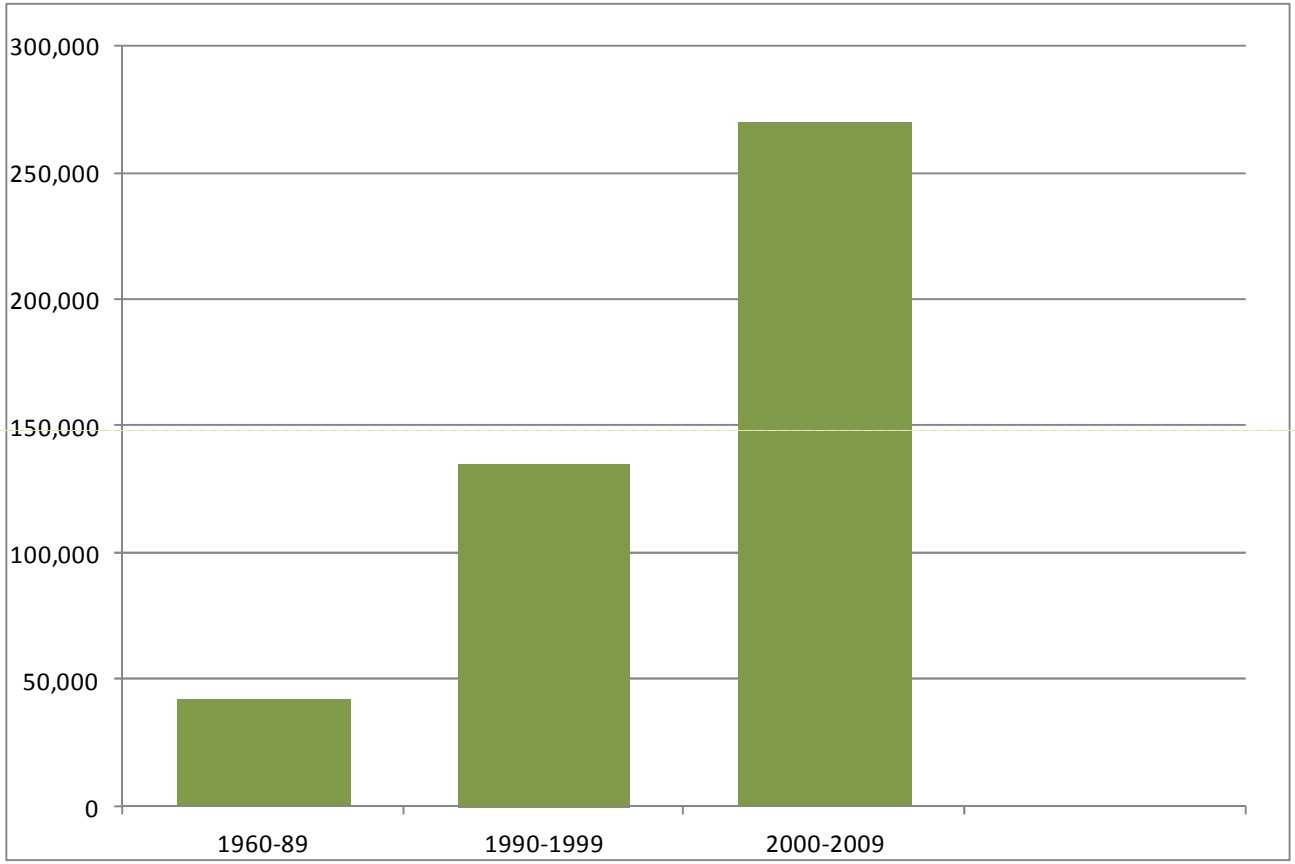


Hanahan, et al., Hallmarks of Cancer, 2011; Cell;144:646





Biomarker Publications: 1960-2010



National Library of Medicine, Pub Med.

Biomarkers in Cancer (www.researchadvocacy.org)

Diagnostic Applications Working Group

November 2010
Generating Evidence for Genomic Test Development

November 2011
Facilitating Development and Utilization of Genome-Based Diagnostic Technologies

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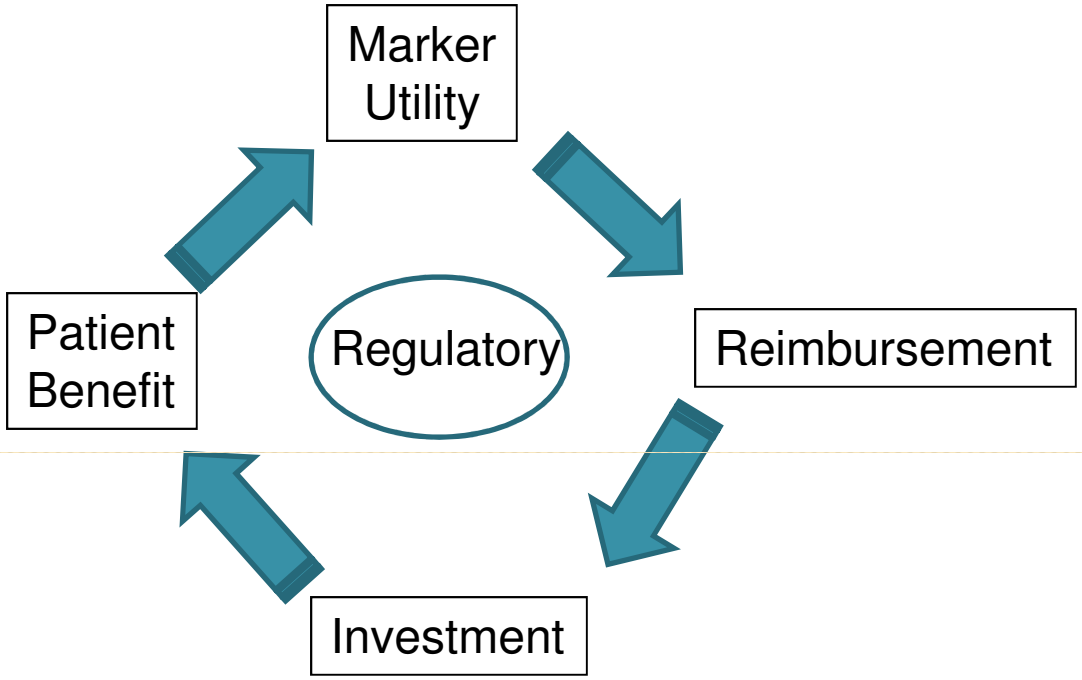
November 2010 Key Learnings

- Multiple Stakeholders and needs
- More dialog and coordination
- Clinical utility rules (?) and creates second hurdle, higher than any other
- Need to develop concept of clinical utility: what, how, when, and why?
- Analysis of evidence adapted to the clinical setting and to evidence needed for that application
- It's a bigger picture than imagined

Vicious Cycle



D. Hayes
Healthcare Provider Perspective
November 2010

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November 2011 Key Learnings

- Major changes in "system" to develop, regulate and reimburse for genomic tests
- Providers are key to enabling long-term (value) and short-term (CED) change to system
- Greater oversight by FDA in genomic test development (LDT)
- VC investment reflects state of regulatory and reimbursement in US for molecular tests
  - "Seamless Path from FDA to CMS to Private Payer"

Evolution of Clinical Utility: A Personal Perspective

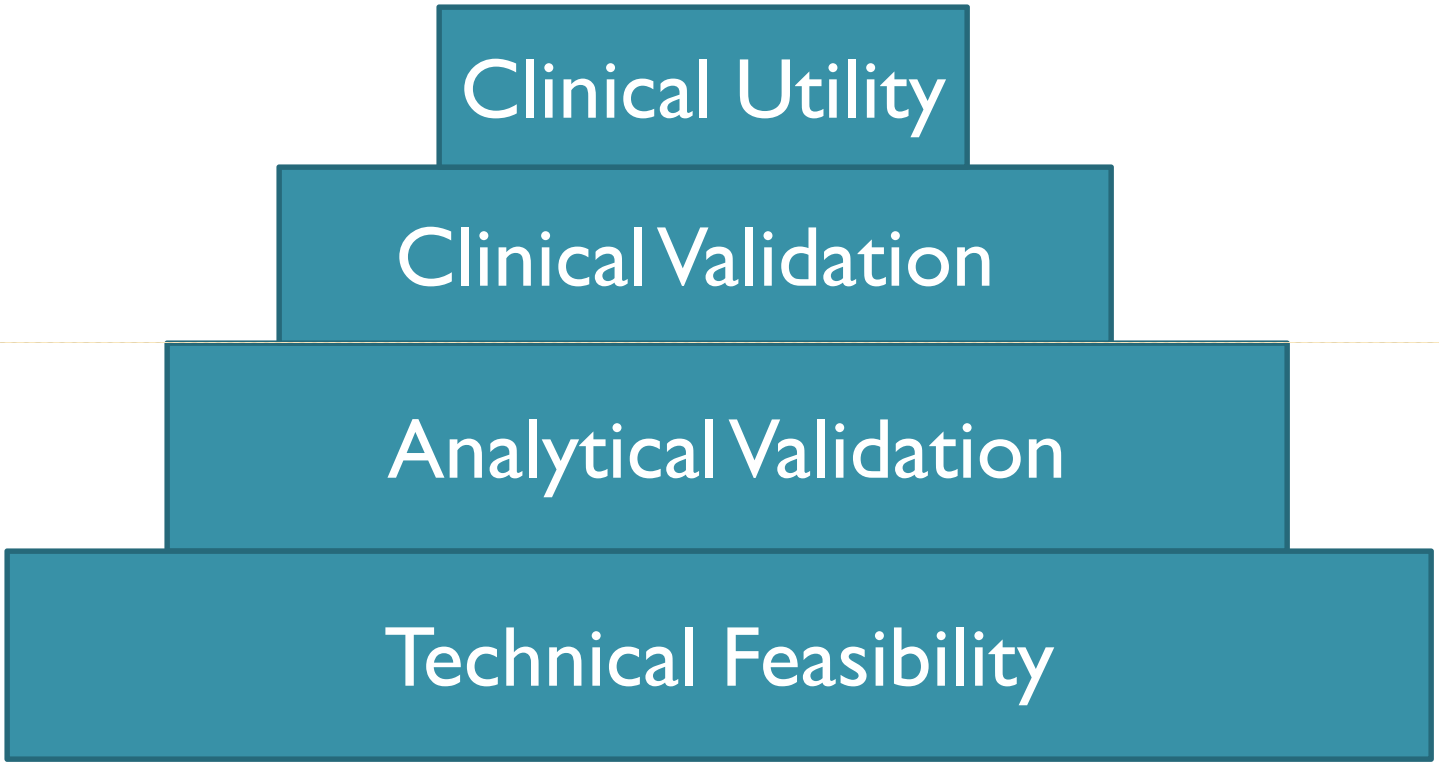
Company /Year	Dx Test	Intended Use	Regulatory Status	Nos. Pts*	Clinical Validity	Clinical Utility
HYB 1993	PSA	Early Detection	PMA	6200	Yes	No
OCD1997	Hepatitis	Dx Screening	PMA	3000	Yes	No
VRX 2004	Tumor Cells	Monitoring	dn510k	200	Yes	No/Yes
VRX 2007	2-gene BrCa	Ax Node Status	PMA	500	Yes	Yes

*All pts prospectively enrolled

Shifting Sands: 2004-2005

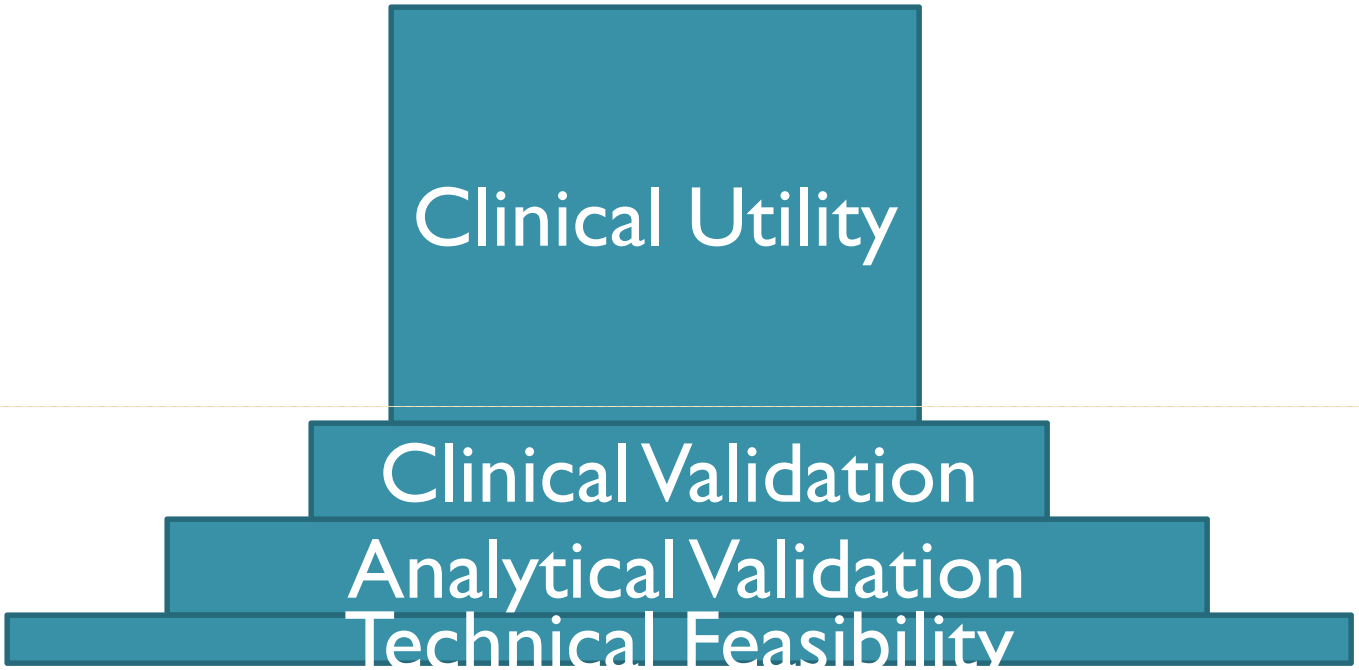
- Has anyone validated this test?
- Is this test covered?
- How will this test change the way I practice medicine?

Technology Maturation to Patient Use



Technology Maturation to Patient Use

(In Reality)



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Clinical Utility of Molecular Diagnostics in Oncology: Workshop Objectives

- Assess the evidentiary requirements for clinical validity and clinical utility that are used to guide treatment decisions in cancer
- Discuss methodologies to demonstrate these requirements and their fit with stakeholder needs
- Consider innovative, sustainable research collaborations for generating evidence of clinical utility which involve multiple stakeholders

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Sean Tunis, MD, MSc
Founder, President and CEO
Center for Medical Technology Policy

"Stakeholder-Informed Methods for Evaluating Clinical Utility"

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Priorities for Translational Breast Research

Rank	Research Question/ Topic	Total Points
1	Molecular signature to spare chemo	643
2	Mol signature optimal chemo regimen	450
3	Predict DCIS into invasive carcinoma	406
4	Determine role of stem cells	404
5.	Therapy targets for triple negative	369

International Web-Based Consultation on Priorities
For Translational Breast Cancer Research
Dowsett, M, et al., Br Ca Res. 2007;9:R81