The Center for Translational and Policy Research on Personalized Medicine

Assessing Cost-Effectiveness ("Assessing Real-World Value") for Oncology-based Molecular Diagnostics

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Today's Discussion

- What do we know about the use of cost-effectiveness analysis (CEA) for molecular dx in oncology?
- What are the challenges?
- What are ways of moving forward?

Recommendations for Moving Forward

- Focus on value not cost-effectiveness only
  - Methods
  - Public discourse

- Focus on real-world analyses of value
  - Descriptive in addition to prescriptive
  - Consider the full context of care

We Don’t Want to Consider Costs - But We Will

Despite increasing concerns about high health care costs, new survey finds little support among Americans for decisions that limit use of high-cost prescription drugs and treatments *{HSPH, 2011}*

As the country searches for ways to curb health care spending, consideration of the cost- effectiveness of health interventions will unavoidably be part of the health care debate *{Neumann & Weinstein, NEJM, 2010}*

For the first time a majority of physicians show an increased willingness to consider the cost implications of the products they use *{Bain & Co., 2011}*

An Economist Puts her Money Where her Mouth Is – Why Putting Skin in the Game is Essential but Challenging in Current Health Care System *{Phillips, pending}*

Will Molecular Dx Enable More Cost-Effective Care?

- Being able to target interventions SHOULD save money or least provide better care at lower cost

But -

- Dr. Ezekiel Emanuel, oncologist/health policy adviser to Obama: personalized medicine *hype, a myth, and unaffordable*

- More testing can be medical equivalent of Moore's Law – testing causes more visits to the doctor resulting in exponentially MORE visits to the doctor *(thednaexchange.com, 2011)*

Are Molecular Dx for Cancer Cost-Effective?

- CUAs of diagnostics
- CUAs of cancer (14% of total) – breast cancer followed by colorectal & hematologic cancers
- Cost-effectiveness for dx & cancer similar to other conditions
  - ~1/2 have ICER <$50K per QALY gained
  - ~10% save money
  - ~10% cost more & less health
  - *But prior to growth of high cost dx & cancer drugs*

- CUAs of molecular dx for cancer (DRAFT, through Fall 2011 N=64, "molecular" only)
  - ~20% cost more & less health (so HIGHER)

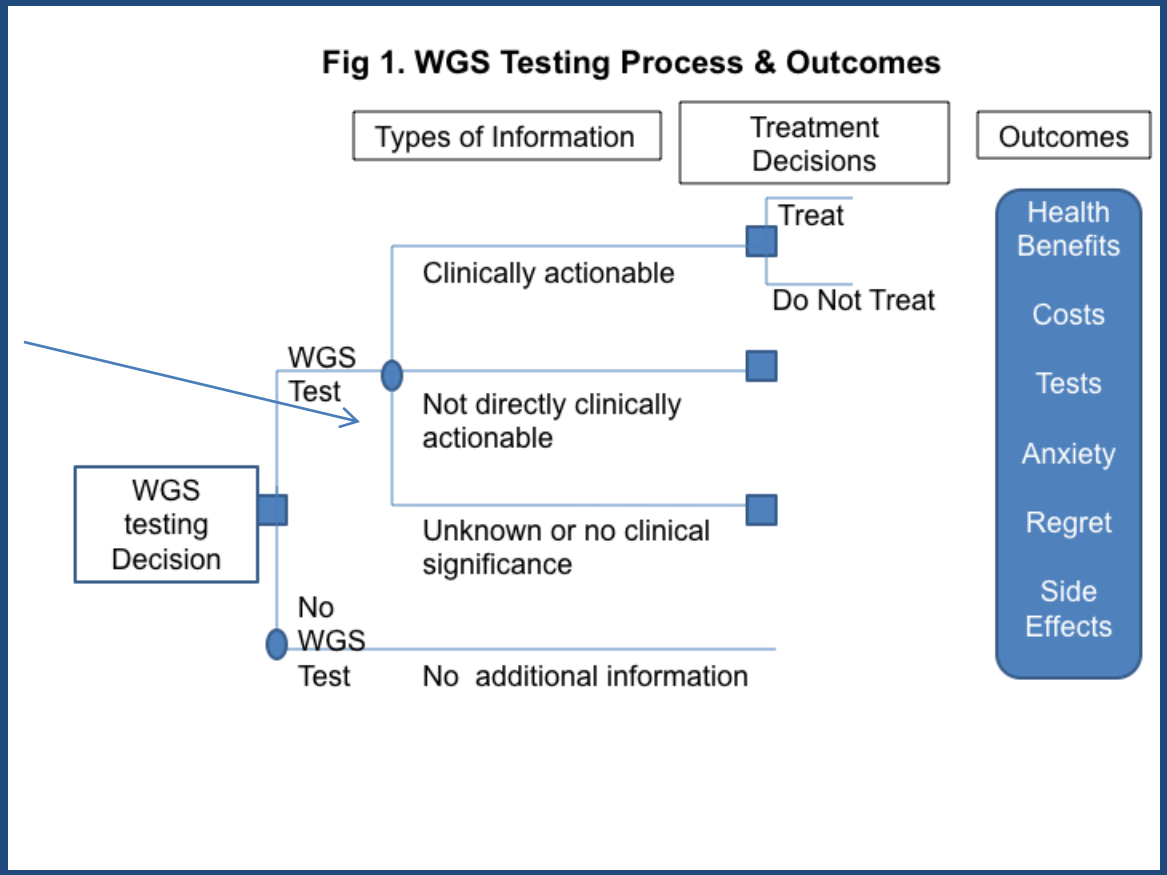
Tufts registry of cost-utility analyses (CEVR)

Challenges to Use of CEA

- Lack of data on effectiveness & costs
- Need to consider impact of dx on downstream decisions & outcomes
- No or limited use of CEA by stakeholders *{Basch et al. Trosman et al}*
  - No: FDA, CMS, patients
  - Limited: providers, industry, payers, guidelines
- Unique to (1) molecular (2) dx (3) cancer?
  - Esp. challenging due to evolving nature/complexity/uncertainty + nature of disease
  - May require consideration of proband + family members if inherited , e.g., CE of Lynch syndrome screening *{Ladabaum et al, 2011}*

Next Big Dilemma
Value of Whole Genome Sequencing
What is Value of "Knowing"?

Low risk
Medium risk
High risk



{Berg 2011}
{Phillips et al ongoing}

Focus on Value Not CEA Only: Choosing Right Pie

- CEA is hard sell
  - Methodological concerns
  - Lack of support for explicit consideration of costs

Frameworks for Assessing Value

- *Is the slice of pie worth it?*
  - CEA
  - Benefits vs. Risks, e.g., comparative clinical effectiveness vs. comparative value matrix (*ICER*)
- *How big is the pie & is slice worth it given size?*
  - Cost-Benefit Analysis, COI, Magnitude Analyses, Preference Analysis/WTP, Value of Information, Multi-Criteria Decision Analysis
- *Can we afford this specific pie?*
  - Budget Impact Analysis

Considering Cost in Larger Context

Steps in Multi-Criteria Analysis

1. Establish decision context. What are aims? Who are decision makers?
2. Identify options
3. Identify objectives & criteria that reflect value associated with consequences of each option
4. Describe expected performance of each option against criteria & score options
5. Assign weights for each criteria to reflect relative importance
6. Combine weights & scores for each of the options to derive overall value
7. Examine results
8. Conduct sensitivity analysis of results

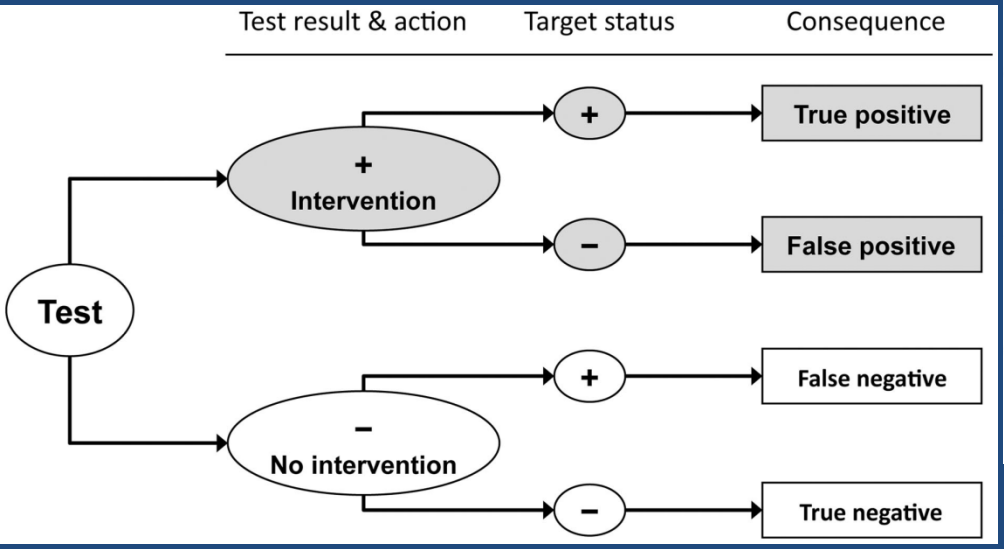
Costs
Benefits

Focus on Real-World Analyses of Value

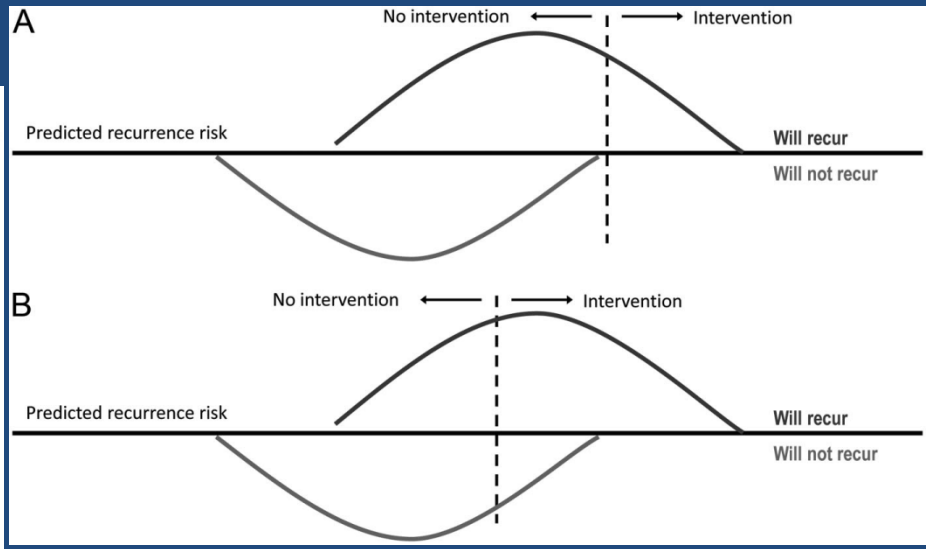
- CEAs based on “ideal world” may not adequately reflect actual implementation

CEA of Targeting Requires Analysis of Method of Targeting *(Elkin et al, 2011)*

- Assessing impact of targeted intervention requires explicit consideration of method of targeting
  - However, few CEAs of breast cancer explicitly evaluated relationships among method of targeting, accuracy of test, & outcomes of intervention



CEAs may assume perfect tests



CEAs may not consider the impact of test thresholds

Example: Gene Expression Profiling for Breast Cancer Recurrence Risk

- One approach is to conduct CEA examining 21 gene signature test (Oncotype*Dx*) to usual care
  - Could also examine comparison to other tests (e.g., Mammaprint) & to actual care
  - Could use data on test performance & impact of test on chemo use from both controlled & observational studies
  - Could examine preferences of patients for foregoing chemo if identified as low risk & how cost impacts that decision
  - Could examine how use of test will impact specific plan's population & budget

Why Real World May Impact CE

(Phillips, JAMA, 2008)

No data on uninsured, Medicaid recipients, or minorities

~20% of IHC tests at community labs may be inaccurate

Up to 20% of negative women still get Herceptin

Cost-effectiveness analyses assume perfect testing

Some women get IHC, some FISH, some both

Claims & medical records for testing do not match 25% of time

60% of positive women – esp. lower income – do not get Herceptin

Conclusion: What Type of Pie do you Want?

- CEA is being used to assess molecular dx for cancer but methodological & political challenges
- Focus on value not cost-effectiveness
  - Methods
  - Public discourse
- Focus on real-world analyses of value
  - Descriptive in addition to prescriptive
  - Consider the full context of care