Assessing the Economics of Genomic Medicine

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Workshop: Background

• Low cost genome sequencing are being considered for routine clinical use
• Tension exists between experts who feel obtaining this data before having a clear clinical indication is premature and those who feel that this information will empower HCP/patients for proactive decision making
• Available sequencing data could also be used at POC
Workshop: Structure

• Workshop aimed at addressing one aspect of this debate:
  – Economic issues that may arise in the course of integrating genomic data into health care

• Assumptions
  – WGS costs are acceptable and fixed (this does not address interpretation costs)
  – Data storage costs are acceptable and fixed (this does not imply that electronically stored data is transportable)
  – These tests are available in a health care encounter
Workshop: Structure

• Three case scenarios: Followed one individual over three life events over a 15 year span

• Three different models:
  – Targeted mutation analysis: current standard of care
  – WGS with provision of data only relevant to the clinical situation and a few actionable variants
  – WGS with provision of data relevant to the clinical situation, actionable variants and other potentially significant secondary findings including lower effect size variants

• Identify research needs that arise from Day 1
Workshop: Structure

- Overview of Genomic Medicine and Health Economics
- Case scenarios: three different stakeholder perspectives
  - Clinician
  - Futurist
  - Patient/consumer
- Economics panel discussion
Promise of Genomic Medicine

– Potential to shed light on the genetic underpinnings of every disease
– Assessing risk of common diseases
  • And doing something about it
– Pre-emptive delineation of select PGx variants
– As an adjunct to newborn screening
– Finding those relatively unusual individuals who are at high risk of preventable disease
– Enabling a variety of reproductive decisions
Genomic Scorecard

- Powerful diagnostic tool for patients with primary genetic disorders
- Broad preemptive PGx application
- Improved treatment of cancer through genomic somatic analysis
- Prevention of common diseases through genomic risk assessment
- Prevention of rare diseases through selective genomic discovery of highly penetrant mutations
- Utility in newborn screening
- Preconception screening to inform reproductive choice

Courtesy of Jim Evans
### Types of Economic Evaluation in Health Care

<table>
<thead>
<tr>
<th>Study Design</th>
<th>Costs Measured?</th>
<th>Outcomes Measured?</th>
<th>Strengths</th>
<th>Weaknesses</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cost-minimization</td>
<td>Yes</td>
<td>Not necessary</td>
<td>Easy to perform</td>
<td>Useful only if outcomes are the same for both interventions</td>
</tr>
<tr>
<td>Cost-benefit</td>
<td>Yes</td>
<td>Yes, in monetary terms</td>
<td>Good theoretical foundation; can be used within health care and across sectors of the economy</td>
<td>Less commonly accepted by health care decision makers; evaluation of benefits methodologically challenging</td>
</tr>
<tr>
<td>Cost-effectiveness</td>
<td>Yes</td>
<td>Yes, in clinical terms (events, life years)</td>
<td>Relevant for clinicians; Easily understandable</td>
<td>Cannot compare interventions across disease areas when using disease specific endpoints.</td>
</tr>
<tr>
<td>Cost-utility</td>
<td>Yes</td>
<td>Yes, in quality-adjusted life-years (QALY)</td>
<td>Incorporates quality of life; Comparable across disease areas and interventions; Standard</td>
<td>Requires evaluation of patient preferences; Can be difficult to interpret</td>
</tr>
</tbody>
</table>
Health Economics is Truly About Measuring Value

• Cost effectiveness analysis evaluates not only cost but also benefits of a health care intervention to assist in decision making
  – Is the improved clinical outcome enough to justify the intervention?

• Also assesses downstream consequences
Interpretation of CEA results

- NW Quadrant: Costs and Effects Lower
- NE Quadrant: Costs and Effects Higher
- SW Quadrant: Bad
- SE Quadrant: Good

 Majority of Interventions fall here
Simple Misconceptions

1. ‘Cost-effective’ = ‘Cost-saving’
2. Expensive interventions are not cost-effective.
3. Inexpensive interventions are cost-effective.

Veenstra
Health Economics Summary

• Helping people to understand what’s at stake – what’s the decision

• Careful CEA is about analyzing decisions
  – Clarify assumptions
  – Evaluate uncertainties
  – Not primarily about costs, but about trade-offs

Veenstra
Is NextGen Sequencing Cost Effective?

- It’s not about the cost, as much as ...
  - what is the **outcome** being measured?
    - base pairs sequenced
    - number of variants identified
    - diagnoses
    - clinical actions
    - patient outcomes – morbidity and mortality
- ... and what is the comparator?
How do we determine the effect of genomics on the health care system?

What do we have to address in order to make this assessment?
Assessing the Needs

- Requires a spectrum of expertise/perspectives
- Some are strictly economic research, most are not
  - Technology development – ongoing
  - Epi research
  - Behavioral research
  - ELSI/education
  - Health Services
I. Evidence – Comparative Effectiveness Research (CER)

- Need for evidence base development – collaboration, infrastructure with clinical trials groups
- Need for innovative approaches to CER prioritization.
- Determining if and how genomic sequence information modifies healthcare provision and patient outcomes.
- Impact of increasing accuracy of sequencing on patient outcomes and costs.
- Evaluation of proper use of family history to guide medical decision making, integrated into HIT infrastructure.
II. Health Economics Methods

- Need better (quicker) approaches and frameworks to performing health economic evaluations of genomic testing.
- Evaluation of evidence thresholds for data in hand versus data that must be obtained, and cost of further research.
- Divergence of economic assessment models in public health, clinical care, and academics.
- In the setting of a disruptive technology and a zero sum game/ shrinking pool of resources what/who will be replaced and how to fund genomic interventions?
III. Health Economics Applications

- When is genomic sequencing cost-effective? E.g., NBS scenario with data being used over the lifespan.
- Better education of genomic scientists regarding economic analysis/integration of economic analysis and on-going studies.
- Methods/infrastructure (including informatics) in health systems to follow downstream consequences of providing sequence data.
- Is cost reduction demonstrable? Do ACO’s provide a possible mechanism for more efficient health care delivery of genomic technologies?
- Study of provider preferences for provision of genomic medicine – evaluation of barriers to implementation.
- Economic incentives for test and evidence development with value-based and specific pricing versus old system (CPT stacking)
- Determination of relative contribution of environment-setting on cost-effectiveness.
IV. Patient-Centered Outcomes

- Developing outcomes data on informed consent/study of efficient methods for patient education regarding informed consent.
- Stakeholder engagement; methodology to increase participation in clinical trials.
- Development of improved methods for assessing value/personal utility/patient preference in economic analysis.
- Potential for genomic medicine to exacerbate disparities, including applicability of information to minority populations and SES disadvantages. Focus on interventions.
Discussion Points

• Zero sum gain: shrinking pool of resources, how to fund genomic interventions?

• Need new ways to obtain evidence
  – Improved methods for assessing value/personal utility/patient preference

• Real world versus academic exercise

• Access issues

• Burdon of additional information that is poorly understood

CENTER FOR GENETIC MEDICINE
Assessing the Economics of Genomic Medicine

• Workshop Chair: Greg Feero, MD, PhD
  – Co-chair: Cathy Wicklund, MS, CGC
  – Clinical Practice and Public Health small group

• IOM Project Staff
  – Adam Berger, PhD, Roundtable Director
  – Claire Giammaria, MPH, Research Associate
  – Tonia Dickerson, Senior Program Assistant