#### **Innovation and Evidence**

Sean Tunis MD, MSc IOM Workshop on Translating Genomic-Based Research for Health (12/4/07)



### **Reasonable and Necessary**

- <u>Adequate evidence</u> to conclude that the item or service:
  - improves net health outcomes
  - generalizable to the Medicare population
  - as good or better than currently covered alternatives



Medicare guidelines for evaluation of dx tests

- Question 1: Is the evidence adequate to determine whether the test provides more accurate diagnostic information?
- Question 2: If the test changes accuracy, is the evidence adequate to determine how the changed accuracy affects health outcomes?



## **FDG-PET for Alzheimer's**

- Adequate evidence that PET had better sens/spec than expert clinical evaluation
- Available treatments have limited efficacy, relatively safe
- Decision model concluded dominant strategy was to treat based on clinical evaluation
- Medicare does not cover\*



## Private Payer on Dx Clinical Utility

 Ambulatory ECG recording is experimental and investigational because of a lack of peerreviewed published reports of prospective clinical trials of the effectiveness of the distinct features of this service in improving clinical outcomes over standard cardiac event monitoring services.



#### **CTAF on Gene Expression Profiling**

• Predictive accuracy of Oncotype Dx high for recurrence;

never compared to standard risk assessment tools

- NSABP B-14 showed that low risk pts randomized to chemo followed 10 years did no better than those without chemo
- TAILORx and MINDACT trials (10,000 and 6,000 pts) now underway



# NHIC / CMS Policy on Oncotype Dx



## The Growing Tension

- Payers, docs, pts demanding more evidence on comparative effectiveness and value

   Regulatory approval no longer sufficient
- Evidence requirements for coverage often poorly defined, inconsistent, not feasible
- Reimbursement and regulatory evidence requirements not well aligned
- Many important questions may require collaborative efforts to define / answer



## **CMTP Basics**

- <u>Structure</u>: private, non-profit
  - 2006-07: foundations, government grants
  - 2008+: diverse membership and grants
- Primary Mission:
  - to support collaborative activities that will improve prospective studies of new medical technologies
- <u>Guiding vision</u>:
  - Creative strategies needed to have robust innovation, rapid translation, and good evidence



## **Programs and Functions**





## **Coverage Guidance Documents**

- Define evidence requirements of payers, patients, and clinicians
  - Primary audience is product developers
  - Analogous to FDA regulatory guidance
  - Purpose is reduce uncertainty, increase consistency, reflect feasibility
- Multi-stakeholder workgroup develop draft
- Iterative public comment process
- Pilot project gene expression profiling for breast cancer



#### **Collaborative Protocol Development**

- Radiation therapy for prostate cancer
- Cardiac CT Angiography (64-slice CT)
- Bariatric surgery in patients with diabetes/obesity
- Molecular dx topic under development
- Conditional reimbursement model



### **Contact Info**

- <u>sean.tunis@cmtpnet.org</u>
- <u>www.cmptnet.org</u>
- 410-963-8876



## **Evidentiary Dilemma**

- Quality of evidence is a continuous function; better evidence takes time
- Many critical health care decisions are dichotomous; pressure to decide early
- Many promising technologies for which definitive evidence will take years / \$\$\$
- More options might be useful
  - Coverage with evidence development
  - Risk-sharing on pricing
  - Money-back guarantee



# Use of CED by CMS

- Lung volume reduction surgery
- FDG-PET for suspected dementia
- Off-label use of biologics approved for colorectal cancer
- Implantable defibrillator for prevention of sudden cardiac death
- FDG-PET for use in oncology
- Home oxygen therapy



## **CED Challenges**

- Adequacy of study design methods
- RCTs viewed as equivalent of non-coverage
- What evidence might change policy
- Uncertain funding source for studies
- Complex and resource intensive
- Lack of neutral forum to balance interests



#### **Prostate Cancer Workgroup**

- Radiation Oncologists
- Community-based Cancer centers
- Academic cancer centers
- Patient Representative
- Clinical Researchers
- Siemens Medical
- Varian Medical

- AHRQ
- NIH (NCI)
- Aetna
- Wellpoint
- Blue Shield California
- United Healthcare



#### **Priorities for Evidence Development**

- Extract prioritized research agenda from EPC reports and other systematic review
- Multi-stakeholder workgroup
- Iterative draft-comment process
- Pilot project PCI vs CABG for stable CAD
  - Working with Stanford/UCSF EPC
  - Funded by AHRQ



## Applied Policy/Methods Projects

- Model benefit language for CED
  - CHCF-funded project
  - Collaboration with Aetna, GE, NBGH
- International workshop on CED
  - Collaboration with NICE, AHRQ
- Methods for pragmatic clinical trials
  - Collaboration with McMaster, University of Toronto, MRC (UK), others

