Evaluating New Medical Technologies: FDA, Medicare and Private Payers

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NAMDRC Conference
March 28, 2014
Ms. Koster has declared no conflicts of interest related to the content of her presentation.
Disclosures

• I have no commercial or academic conflicts of interest

• Employed by the Southern California Permanente Medical Group (SCPMG), which contracts exclusively with the Kaiser Foundation Health Plan to provide medical services in the U.S.

• All activities funded by SCPMG and The Permanente Federation

• External Affiliations/Positions:
  – Chair, Guidelines International Network/North America
  – Former member of the Institute of Medicine’s (IOM) Committee on Standards for Systematic Reviews of Comparative Effectiveness Research
  – Reviewer for the IOM report, Standards for Developing Trustworthy Clinical Practice Guidelines
  – Technical Advisory Committee Member, Joint Commission Osteoporosis Task Force
Learning Objectives

1. Understand how new medical technologies are evaluated and approved by the FDA, Medicare, private payers and health systems.

2. Learn about the evaluation standards for new technologies used by regulatory, government and private payers/health systems.

3. Know where to find information and resources on new technology evaluation.
Key Determinants

- Regulatory (FDA)
- Coverage Determinations (Medicare, private payers)
- Payment
- Classification & Coding

New medical devices

Will the technology be approved?
Will the technology be paid for?
How much will be paid?
How will the technology be billed?
Regulatory Requirements – FDA
Center for Devices and Radiological Health (CDRH)

- All medical devices marketed in U.S. undergo Food and Drug Administration (FDA) classification and clearance/approval
- Decisions based on data demonstrating “safety and effectiveness”
FDA Device Classification System

- Three categories of regulatory control based on intended use of device and level of risk to patient
- Higher risk = higher class, greater regulatory controls, more evidence of effectiveness/safety required

Class I  “Low Risk”
- Device considered of minimal harm to patient
- “General controls” usually sufficient (registration, labeling, good manufacturing practices, etc.)
- Generally exempt from premarket review/approval requirements
- ~50% of classified devices

Class II  “Moderate Risk”
- Moderate risk to patient
- General + special controls
- Requires premarket notification (510k) application
- Most devices submitted as “substantially equivalent” to already approved “predicate” devices ("as safe & effective")
- Clinical data required for small number 510(k) applications

Class III  “High Risk”
- High risk to patient
- Not substantially equivalent to predicate devices; novel tech, new indication, or “high-risk” iterative medical devices
- General + special controls
- Requires premarket approval (PMA) application, with “sufficient, valid scientific evidence of effectiveness/safety
• Postmarket *approval* studies can be required of Class III devices

• Postmarket *surveillance* studies can be required of Class II and III devices
FDA Investigational Device Exemption (IDE)

- Allows use of investigational devices in clinical studies to collect safety and effectiveness data

- IDEs apply to:
  - New devices *not yet cleared* for marketing
  - *Specific modifications* to approved devices
  - *New intended uses* of approved devices

- Level of regulatory control depends on level of risk:
  - Significant Risk: “Potential for serious risk to the health, safety, or welfare of a subject” (e.g., implants, shunts, sutures, etc.)
  - Nonsignificant Risk: Only IRB approval required prior to initiation of clinical study; no IDE submission required

Coverage: CMS/Medicare

- Determines coverage and reimbursement for medical services, treatments or technologies impacting Medicare beneficiaries
  - Must be “reasonable and medically necessary”
  - Demonstrate improved health outcomes over existing practices

### Goals
- Ensure health care security for program beneficiaries
- Improve the health outcomes of populations of patients
- Provide access to high-quality, affordable medical care
- Maintain quality care while controlling costs

### Activities
- Cover “reasonable and necessary” medical services
- Require transparent, evidence-based process that demonstrates improved health outcomes
- Develop/standardize coverage and reimbursement policies
- Develop incentives to maintain quality and costs
Medicare National Coverage Determination (NCD)

• “Reasonable and Necessary”
  – Sufficient evidence that the medical intervention improves patient health outcomes in the Medicare population
  – May differ from evidence required for FDA approval

• Increasingly linking coverage decisions to evidence:
  – Technology assessments conducted by CMS staff and/or
  – External technology assessments (commissioned or conducted by outside organization) and/or
  – In consultation with the Medicare Evidence Development & Coverage Advisory Committee (MEDCAC)
  – For new tech, coverage is sometimes linked to participation in clinical trials to encourage evidence development
FDA and Medicare – Important Distinctions

• **Different goals/expectations:**
  - **FDA:** “reasonable assurance of safety and effectiveness” of a device
  - **Medicare:** “reasonable and necessary for the diagnosis or treatment of illness or injury to improve the functioning of a malformed body member”

• **Different evidence requirements:**
  - **FDA:** Sufficient evidence that the device is *safe and effective* for *specific indications* within a *defined population*
  - **Medicare:** Sufficient evidence that the medical intervention *improves patient health outcomes over existing practices* in the *Medicare population*
Types Medicare Coverage Determinations

- **National Coverage Determination (NCD)**
  - External Requests: Initiated by Medicare beneficiaries, manufacturers, providers, suppliers, medical professional associations, or health plans
  - Internal Requests initiated by CMS

- **What triggers an NCD (new items/services)?**
  - Technology considered a **substantial clinical advance**, likely to result in significant health benefit if available more rapidly
  - Rapid access likely to have significant programmatic impact on Medicare policies
  - Uncertainty around health benefits, patient selection or facility/staffing requirements

- **Types of NCD decisions:**
  - National Coverage or Noncoverage
  - National Coverage with restrictions (specific populations, providers, facilities, evidence development, etc.)
CMS National Coverage Determination (NCD) Process

1. Preliminary Discussions
   - Benefit Category

2. National Coverage Request
   - (internal or external sources)
     - Internal Technology Assessment

3. Draft Decision Memorandum Posted
   - 6 months
   - 30 days
   - 60 days

4. Public Comments

5. Final Decision Memorandum and Implementation Instructions
   - Reconsideration
   - Department Appeals Board

- External Technology Assessment
- Medicare Coverage Advisory Committee
- Staff Review

9 months

• **Local Coverage Determination (LCD)**
  – If no NCD, coverage at discretion of local Medicare contractor
  – If a “final” NCD is issued, local contractors must amend/withdraw LCDs not consistent with NCD

• **Coverage with Evidence Development**
  – Medicare coverage of specific promising technologies conditional on patient participation in a registry or clinical trial

• **Experimental/Investigational Devices**
Medicare Reimbursement for New Technologies

*Investigational Medical Devices*

- Interagency FDA-CMS agreement to categorize devices in clinical trials for purposes of eligibility for Medicare coverage.

- **Category A: “Experimental/Investigational Devices”**
  - “Significant Risk” – absolute risk of device not yet established
  - Not eligible for Medicare coverage; considered "*services related to a non-covered device*"
  - Investigators conducting clinical trials must address cost issues to ensure that Medicare patients are not adversely affected by decision to participate

- **Category B: “Non-experimental Investigational Devices”**
  - Devices for which FDA does not require IDE – safety/efficacy established
  - Eligible for Medicare coverage: "*Payment for covered devices and related services will be based on, and may not exceed, the amount that would have been paid for a currently used FDA-approved device and related services serving the same medical purpose.*"
Medicare Reimbursement for New Technologies
Inpatient Hospital Prospective Payment System (IPPS)

• **New technology add-on** (42 CFR Part 412, CMS Guidance)
  
  – CMS must determine that new technology is a *substantial clinical improvement* relative to current or previous diagnosis or treatment
  
  – Data reflecting the cost of new technology must not yet be available in the data used to recalibrate the DRGs
  
  – DRG payment rate otherwise applicable to the new technology would be inadequate (i.e., average charges for cases using the new technology will be in excess of the amount determined by CMS formula)
Medicare Reimbursement for New Technologies
Hospital Outpatient Prospective Payment System (OPPS)

• Transitional pass-through payment (at least 2, no more than 3 years):
  – CMS must determine that there is a substantial clinical improvement over current treatments
  – If “pass-through” status is approved, a new device category is established, along with coding information
  – Payment: Hospital’s charge, adjusted to actual cost, minus APC payment amount for device
Medicare Reimbursement for New Technologies
Hospital Outpatient Prospective Payment System (OPPS)

• New Ambulatory Payment Category (APC)
  – Intended for complete services/procedures that cannot be appropriately billed under an existing HCPCS code or APC
  – Must be “truly new”
  – Must be distinct procedure with a beginning, middle and end
  – Not appropriate for devices that could qualify for pass-through payment under OPPS
  – Device manufacturers don’t apply for a new tech APC, they request to be placed within a category
Goals

- Provide health care for plan enrollees and dependents
- Improve the health outcomes of populations of patients
- Provide access to high-quality, cost-effective medical care
- Ensure quality of care, cost control, financial stability

Activities

- Cover “medically necessary” health care services
- Evaluate clinical benefit, economic value, resource implications
- Develop payer-specific coverage/practice policies based on evidence & value
- Manage new tech through resource stewardship, UM or other systems

Determine coverage for medical services, treatments or technologies impacting health plan enrollees and dependents

- Must be “medically necessary” – not experimental/investigational
- Consider: Clinical effectiveness + improved health outcomes + economic value/resource implications + patient preference
Factors Influencing Payer Coverage

- FDA 510(k) clearance or premarketing approval does not guarantee coverage from health systems and other payers.

- CMS/Medicare NCDs/LCDs define what can be reimbursed, but other factors may determine service provision:
  - Clinician judgment re “medical necessity” of intervention
  - Assessment of patient-specific benefit/risk

- Higher level of evidence required demonstrating:
  - Effectiveness and safety compared to existing treatments
  - Long-term, sustained improvement in health outcomes
  - Patient preferences and burden of suffering
  - Resource impact/value
Factors Influencing Payer Coverage (cont.)

• Manage new technology through clinical and coverage policies and statements, resource utilization management, contracting, etc.

• Increasing demand for patient-centered, evidence-based approach to determine coverage and payment
  – “Value” assessed through evaluation of evidence on clinical and economic outcomes, resource implications and patient preferences

• Rely on existing tech assessments from external sources when making coverage determinations
  – Although tech evaluation standards exist, no uniform process
  – Potential for variation in new tech coverage processes and criteria across payers
Process Flow Map

Source: http://www.ispor.org/htaroadmaps/usmd.asp
One Payer, Multiple Outcomes

Source: http://www.ispor.org/htaroadmaps/usmd.asp
New Tech Evaluation: Evidence Review Sources

- Collect, summarize, and evaluate scientific studies, evidence reviews and information from key sources:
  - Medical literature databases (e.g., PubMed, EMBASE, etc.)
  - Subscription evidence review databases (examples)
    - Cochrane Collaboration
    - ECRI Institute
    - Hayes Inc.
  - Technology Assessment Organizations
    - Agency for Healthcare Research & Quality (AHRQ)
    - Blue Cross Blue Shield, Technology Evaluation Center
    - California Technology Assessment Foundation (CTAF)
  - FDA, CMS/Medicare, clinical trials registries
  - Professional society guidelines and statements
  - Medical policies of other health plans
New Tech Evaluation: Evidence Grading

- No common evidence-grading system, but more than 70 international groups (including ERS/ATS) have now adopted or use “GRADE”

Four Categories of Quality of Evidence:

"HIGH" ● “MODERATE” ● “LOW” ● “VERY LOW”

Based on:

- Methodological quality of evidence
- Likelihood of bias related to recommendation
- By individual outcome & across all outcomes

Separates judgments about:
- Quality of evidence
- Strength of recommendation

Two Grades of Recommendations:

WEAK ("conditional")       STRONG (for or against an action)

Based on:

- Quality of evidence
- Balance of benefits and downsides
- Values and preferences
- Resource use

Grading of Recommendations Assessment, Development and Evaluation (GRADE) Working Group:
http://www.gradeworkinggroup.org/
Example – Managed Health System New Technology Process
Kaiser Permanente Southern California Region
Medical Technology Management Process (MTMP) Process

START
Request for regional new technology evidence review

MTAT Review
Evaluate Evidence

MTDST Review
Consider Deployment

Recommend MTDST review?
Yes
No

Recommend deployment?
Yes
No

MTAT: Medical Technology Assessment Team
MTDST: Medical Technology Deployment Strategy Team

• *MTMP decisions do not result in benefit “coverage policies”*
• *Permanente Medical Group physicians determine “medical appropriateness” of care*
What’s up with Bronchial Thermoplasty???
— At a Glance —

2006/2007
RISA+AIR
study data published

2010
AIR2
study data published

4/27/10
510K clearance¹
(Class III high risk)

2011
AIR
(5 years)²
study data published

1/1/12
Transitional Pass-
Through Payment Approval

2013
RISA+AIR2
(5 years)²
study data published

2006-07 ← 2010 → ← 2011 → ← 2012 → 2013 2014

1 5-year post-treatment study required
2 BT group only followed through 5 years
Bronchial Thermoplasty – Health Plan Coverage

- Still considered “experimental, investigational and/or unproven”
- Concerns about rate of adverse events, ED visits and hospitalizations reported during initial treatment period in studies (benefits/harms)
- Low number (n=225) of subjects and only BT subjects followed in 5-year extension studies (risk of study bias)

<table>
<thead>
<tr>
<th>Health Plan</th>
<th>Date(s)</th>
<th>Policy</th>
</tr>
</thead>
<tbody>
<tr>
<td>Aetna</td>
<td>1/2014</td>
<td>“experimental and investigational”</td>
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<tr>
<td></td>
<td></td>
<td>“effectiveness has not been established”</td>
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<tr>
<td>Anthem-Blue Cross</td>
<td>10/2013</td>
<td>“Investigational and Not Medically Necessary”</td>
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<tr>
<td>Cigna</td>
<td>6/2013</td>
<td>“experimental, investigational or unproven”</td>
</tr>
<tr>
<td>Health Net</td>
<td>6/2013</td>
<td>“investigational”</td>
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<tr>
<td></td>
<td></td>
<td>“limited peer-reviewed studies available data on long-term safety and efficacy”</td>
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<tr>
<td>United Healthcare</td>
<td>10/2013</td>
<td>“unproven for treating asthma”</td>
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<td>“...large-scale sham treatment trials are needed to...draw definitive conclusions that the long-term improvements...outweigh the increased short-term risk of adverse events and hospitalizations.”</td>
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### Professional Society Positions

- **European Respiratory Society/American Thoracic Society**

<table>
<thead>
<tr>
<th>Context</th>
<th>Recommendation</th>
<th>Strength</th>
<th>Quality of evidence</th>
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<tbody>
<tr>
<td>Bronchial thermoplasty</td>
<td>We recommend that bronchial thermoplasty is performed in adults with severe asthma only in the context of an Institutional Review Board approved independent systematic registry or a clinical study (recommendation, quality evidence)</td>
<td>Strong</td>
<td>Very low</td>
</tr>
</tbody>
</table>

**Source:** Chung KF et al. ERS/ATS Guidelines on Severe Asthma. Eur Respir J 2014;43: 343–373
**Professional Society Positions (cont.)**

<table>
<thead>
<tr>
<th>Values and preferences</th>
<th>Remarks</th>
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<tbody>
<tr>
<td>This recommendation places a higher value on avoiding adverse effects, on an increased use of resources, and on a lack of understanding of which patients may benefit, and a lower value on the uncertain improvement in symptoms and quality of life</td>
<td>This is a strong recommendation, because of the very low confidence in the currently available estimates of effects of bronchial thermoplasty in patients with severe asthma. Both potential benefits and harms may be large and the long-term consequences of this new approach to asthma therapy utilising an invasive physical intervention are unknown. Specifically designed studies are needed to define its effects on relevant objective primary outcomes, such as exacerbation rates, and on long-term effects on lung function. Studies are also needed to better understand the phenotypes of responding patients, its effects in patients with severe obstructive asthma (FEV₁ &lt; 60% of predicted value) or in whom systemic corticosteroids are used, and its long-term benefits and safety. Further research is likely to have an important impact on this recommendation.</td>
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American College of Chest Physicians (ACCP):

March 27, 2013 – Statement on website: (cites CTAF evidence assessment)

“Bronchial thermoplasty offers treatment for patients with severe asthma who continue to be symptomatic despite maximal medical treatment. The American College of Chest Physicians (ACCP) believes the literature supports bronchial thermoplasty as a therapeutic option for patients with severe asthma and should no longer be considered experimental.

Despite the creation of CPT codes and available reimbursement from Medicare, many of our ACCP members are having difficulty obtaining reimbursement from third party payers.

The ACCP is providing our members with a template appeal letter that can be used to appeal for reimbursement from third party payers for bronchial thermoplasty.”
• **British Thoracic Society (BTS, 2011):**
  
  – Bronchial thermoplasty is a possible treatment option in selected patients with severe persistent asthma already on maximal therapy, although its place in the treatment of asthma remains to be established. (Evidence Grade “A”)
  
  – Long-term safety and efficacy remain unclear. Hence, treatment should be limited to a few specialist centres in carefully selected patients. Longer-term follow-up of treated patients is recommended. (“Good Practice Point”)

• **Global Initiative for Asthma (GINA, 2012):**
  
  – “possible option” in countries where it is offered
  
  – “...longer-term follow-up of larger number of control and active patients is needed to assess effectiveness”
  
  – “...caution should be used in selecting patients for this procedure.”
Questions/Discussion