Comparative Effectiveness Research: Importance and Utility for CMS
Reflections from NIA

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“Begin with the End in Mind”

**CMS Mission**
- To ensure effective, up-to-date health care coverage and to promote quality care for beneficiaries

**CMS Vision**
- To achieve a transformed and modernized health care system.
- CMS will accomplish our mission by continuing to transform and modernize America's health care system.

Steven Covey; CMS website
Number of Medicare Beneficiaries
1970-2030

* Numbers may not sum due to rounding.

Source: CMS, Office of the Actuary.
ACA Expands Coverage: 2014--

• Expand Medicaid to all individuals under age 65 with incomes up to 133% of the poverty level ($14,400/individual or $29,300/family of 4).
• Create new Health Insurance Exchanges where individuals and small employers can purchase coverage (subsidized for eligible individuals and families with incomes up to 400% of the poverty level)
  – Impact 15.9 additional covered lives by 2019.
• Medicare covered lives unchanged, growing
Donald Berwick, MD

- Improve the health of the population;
- Enhance the patient experience of care (including quality, access, and reliability); and
- Reduce, or at least control, the per capita cost of care.
Clinical decision-making

- **Evidence**
  1. Patient data
  2. Basic, clinical, and epidemiologic research
  3. Randomized trials
  4. Systematic reviews

- **Patient/Physician Factors**
  1. Cultural beliefs
  2. Personal values
  3. Experiences
  4. Education

- **Clinical Decision**

- **Guidelines**

- **Ethics**

- **Constraints**
  1. Formal policies, laws
  2. Community standards
  3. Time
  4. Reimbursement

CMS Needs Information

- Should we pay for this service? = Coverage
- Did we pay for the correct service correctly? = Payment
- Was the service we paid for performed optimally? = Quality
- How should we transform health care? = Innovation
• CER: Comparative Effectiveness Research
• HTA: Health Technology Assessment
• EBM: Evidence-Based Medicine
CER, HTA and EBM

- **Comparative effectiveness research (CER)**
  - Research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat and monitor health conditions in “real world” settings
  - Evidence generation and synthesis

- **Evidence based medicine (EBM)**
  - Evidence synthesis to assist patients’ and/or physicians’ decisions. Individual clinical decision making (also clinical guidelines and quality measures)

- **Health technology assessment (HTA)**
  - Evidence synthesis used to inform reimbursement coverage decisions. Considers clinical effectiveness, safety, cost-effectiveness (benefits vs. harms and economic evaluation)

Coverage

Should we pay for this service?
Carotid Stenting Coverage Decision

• Patients who are at high risk for carotid endarterectomy (CEA) and symptomatic carotid artery stenosis ≥ 70%, covered for FDA-licensed CAS systems

• Otherwise (symptomatic carotid artery stenosis between 50% and 70%, and asymptomatic carotid artery stenosis ≥ 80%), in accordance with
  – NCD: post approval studies
  – Category B IDE clinical trials regulation
  – clinical trials policy

CT Colonography Coverage Decision

• “The evidence is inadequate to conclude that CT colonography is an appropriate colorectal cancer screening test .... CT colonography for colorectal cancer screening remains noncovered.”

• Findings not necessarily generalizable
  – (age 58 vs ~75 years)
  – Potential risks incompletely evaluated

• USPSTF: Insufficient evidence

CMS National Coverage Decisions

- National Coverage
- National Noncoverage
- National Coverage with restrictions
  - Specific subpopulations
  - Specific providers/facilities
  - Evidence development (CED)
Steps to Medicare Coverage Determination and Payment

Outside of CMS:

• Congress determines benefit categories
  – This section of talk focuses on Medicare Part A/B
• FDA approves drugs/devices for market

Within CMS:

• Coverage
• Coding
• Payment
Social Security Act 1862(a)(1)(A-B)

Reasonable & Necessary

“...no payment may be made...for items or services - which, except for items and services described in a succeeding subparagraph, are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member ,... which are not reasonable and necessary for the prevention of illness”
What is a Covered Service?

An Item or Service:

• for which there is a Medicare Benefit Category (& service meets benefit category requirements)

• which is not Statutorily Excluded based on 1862 (a)(2)-(15)

• which is Reasonable and Necessary based on 1862 (a)(1)(A or B)
How Does CMS Apply R&N Today?

• Sufficient level of confidence that evidence is adequate to conclude that the item or service:
  — improves health outcomes
  — generalizable to the Medicare population
• Similar to health technology assessment conducted by other payors/systems.
CMS Assessment of Evidence

- Synthesis
  - Staff assessment
  - Commissioned assessment
- Evidence-based guidelines
- Clinical trials
- Observational studies
- Comparative effectiveness?
Quality of Evidence

• Awards or deducts points for a study’s description of
  – Randomization,
  – Double blinding,
  – Withdrawals and
  – Dropouts.

• Used in CMS decision memo for acupuncture

• Typically no explicit standard described

Health Outcomes for Coverage

**More Impressive**

- Longer life and improved function/participation
- Longer life with arrested decline
- Significant symptom improvement allowing better function/participation
- Reduced need for burdensome tests and treatments

**Less Impressive**

- Longer life with declining function/participation
- Improved disease-specific survival without improved overall survival
- Surrogate test result better
- Image looks better
- Doctor feels confident
CMS Assessment of Evidence

- Synthesis (or commissioned assessment)
- Evidence-based guidelines
- Clinical trials
- Observational studies
- May include CER results (ACA 1182)
  - Iterative and transparent (public) process
  - Considers subpopulations
  - Not sole basis for non-coverage
Responsiveness to new evidence
RCT Data: Comparative effectiveness by age

Funding: Stroke Association
Coronary revascularization age $\geq 85$

3-year outcomes (observational)


Funding: NIA, NIGMS
Longitudinal studies extend and generalize RCT findings

Guralnik J, et al. JAGS 58;2010:S337-342
Economic data for coverage?

• Impacts priorities
• Use permitted for certain preventive services (MIPPA 2008 and other authority)
  – Evidence from literature
  – AHRQ-commissioned assessments
• By practice, CMS does not use for evaluation of diagnostic & therapeutic services
• QALY threshold prohibited for coverage decisions (ACA 1182)
Economic analysis

• “All FOBTs were cost-effective. Hemoccult II® at $4.50 had a cost-effectiveness ratio of $1,071 per life year gained and iFOBT at $28.00 had a cost effective ratio of $4,500 per life year saved assuming 100% compliance (lower levels of compliance would increase the cost per life year gained).”
CMS Promotes CER Data

- Coverage with evidence development
  - Registries
  - Practical clinical trials
- Linkage of claims data
User’s Guide to Patient Registries

Registries for Evaluating Patient Outcomes: A User’s Guide*

- The first government-supported handbook for establishing, managing and analyzing patient registries (now 2nd edition)
  - Designed so patient registry data can be used to evaluate the real-life impact of health care treatments
  - A milestone in growing CER efforts

* Co-funded by AHRQ & CMS
SEER-Medicare Linkage

• Created by linking 2 population-based sources
  – >1.5 million persons with cancer
  – Can be used to examine health care before, during and after cancer diagnosis

• SEER: detailed clinical, demographic and cause of death information for persons with cancer

• Medicare: longitudinal claims for all covered health services from time of eligibility to death
Quality Measurement

Was the service we paid for performed optimally?
Motivation to Measure Quality

• Need for accountability to oversight bodies and beneficiaries
• Desire to make evaluation of health care more objective
• Desire to improve value in government purchasing
Medicare program assessment

Outcome **Measure:** Improve the care of diabetic beneficiaries by increasing the rate of hemoglobin A1c and cholesterol (LDL) testing

<table>
<thead>
<tr>
<th>Year</th>
<th>Target</th>
<th>Actual</th>
</tr>
</thead>
<tbody>
<tr>
<td>2005</td>
<td>Baselines - A1c; LDL</td>
<td>84.3%; 78.1%</td>
</tr>
<tr>
<td>2006</td>
<td>Dev. baselines/targets</td>
<td>Goal met</td>
</tr>
<tr>
<td>2007</td>
<td>85.0%; 80.0%</td>
<td>86.0%; 80.3%</td>
</tr>
<tr>
<td>2008</td>
<td>85.5%; 80.0%</td>
<td>Sep-09</td>
</tr>
<tr>
<td>2009</td>
<td>86.0%; 81.0%</td>
<td>Sep-10</td>
</tr>
<tr>
<td>2010</td>
<td>86.5%; 81.5%</td>
<td>Sep-11</td>
</tr>
</tbody>
</table>

Diabetes Care & Outcomes Audit

Outcome: Glycemic control
1994-2002

HbA1C, %

*p<0.0001 comparing mean HbA1C levels in FY94 and FY02

http://www.hhs.gov/of/reports/account/acct03/sect2/PARsection2.html
PQRI 01 NQF 0059

• Hemoglobin A1c Poor Control in Type 1 or 2 Diabetes Mellitus (2008)
• Percentage of patients aged 18 through 75 years with diabetes mellitus who had most recent hemoglobin A1c greater than 9.0%
• Developed by National Committee for Quality Assurance (NCQA)

Current Quality and Performance Measure Sets

- HEDIS Healthcare Effectiveness Data and Information Set
- HOS Health Outcomes Survey
- CAHPS Consumer Assessment of Healthcare Providers and Systems
- Independent Review Entity data
- Part D Performance Measures
CMS Quality Measure Development

- Measures Priorities
- Planning
- Managing Ongoing Feedback
- Measure Implementation
- Measure Development
- Measure Reevaluation

Arrows indicate the flow of processes and feedback.
Information Gathering

Solid Foundation for Measures

- Solicited input
- Call for Measures
- Clinical guidelines
- Related measures
- Related Studies
- Empirical data analyses
- Ongoing feedback
Measure Evaluation

Importance?

Scientific acceptability of measure properties

Feasibility

Usability

STOP
Quality measure development

• Technical Expert input
• Public comment periods
• Measure specification
  – Numerator, denominator
• Consider Risk adjustment
• Measure testing
  – Reliability, validity, feasibility
• Implementation
Many participants in process
Innovation

How should we transform health care?

This is fundamentally comparative effectiveness research.
Center for Medicare and Medicaid Innovation

• Social Security Act 1115A, (ACA 3021) Creates CMI within CMS

• Purpose: test innovative payment and service delivery models to reduce program expenditures ...while preserving or enhancing the quality of care

• give preference to models that also improve the coordination, quality, and efficiency of healthcare services
Model selection: there is evidence that the model addresses a defined population for which there are deficits in care leading to poor clinical outcomes or potentially avoidable expenditures.

Model evaluation:

- quality of care, patient-level outcomes
- changes in spending (program level)
Further Information

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