Patient Centered Outcomes Research

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- All material presented is publicly available information.
Challenges to our Healthcare System

- Unsustainable increase in health spending – about $2.3 trillion per year in the U.S.
  - Large geographic variations in spending
- Costs driven by technology related changes in medical practice (financial incentives)
- Large variations in clinical care when evidence is unclear
- Uncertainty about best practices involving treatments and technologies
- Overall poor quality of care compared to other developed countries
## Attributes of U.S. HRRs in Different Quintiles of the EOL-EI*

<table>
<thead>
<tr>
<th>Variable</th>
<th>Quintile of EOL-EI</th>
<th>Ratio (Highest to Lowest)</th>
</tr>
</thead>
<tbody>
<tr>
<td>EOL-EI, $\dagger$</td>
<td>9074 10 636 11 559</td>
<td>12 598 14 644 1.61</td>
</tr>
<tr>
<td>Per capita Medicare spending, $\ddagger$</td>
<td>3922 4439 4940</td>
<td>5444 6304 1.61</td>
</tr>
<tr>
<td>Hospital characteristics$^g$</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Overall supply (beds per 1000), n</td>
<td>2.4 2.6 2.9 2.9</td>
<td>3.2 1.32</td>
</tr>
<tr>
<td>Beds in teaching hospitals, %</td>
<td>10.2 18.1 13.8</td>
<td>20.8 28.1 2.76</td>
</tr>
<tr>
<td>Beds in hospitals with &gt; 300 beds, %</td>
<td>31.6 37.4 38.7</td>
<td>43.8 57.2 1.81</td>
</tr>
<tr>
<td>Physician supply (per 10 000), n$^s$</td>
<td>184.8 189.4 184.4</td>
<td>204.6 242.4 1.31</td>
</tr>
<tr>
<td>Medical specialists</td>
<td>26.9 28.8 28.6</td>
<td>34.8 44.4 1.65</td>
</tr>
<tr>
<td>General internists</td>
<td>21.3 23.4 22.6</td>
<td>28.5 37.3 1.75</td>
</tr>
<tr>
<td>Family practitioner/GP</td>
<td>35.9 31.3 29.6</td>
<td>25.9 26.5 0.74</td>
</tr>
<tr>
<td>Surgeons</td>
<td>43.8 45.6 46</td>
<td>50.3 56.4 1.29</td>
</tr>
<tr>
<td>All other specialties</td>
<td>56.8 60.3 57.5</td>
<td>65.1 77.7 1.37</td>
</tr>
<tr>
<td>Medicare enrollees in HMOs, %</td>
<td>12.1 6.8 7.3</td>
<td>7.7 15.3 1.26</td>
</tr>
<tr>
<td>Residents in metropolitan areas, %</td>
<td>77.5 81.9 82.3</td>
<td>79.2 97.4 1.26</td>
</tr>
</tbody>
</table>

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* EOL-EI = End-of-Life Expenditure Index; GP = general practitioner; HMO = health maintenance organization; HRR = hospital referral region.

$\dagger$ Average age-sex-race–adjusted per capita fee-for-service spending on hospital and physician services in the HRRs within each quintile for Medicare enrollees age 65–99 years who were in their last 6 months of life. For details, see Methods.

$^s$ Average age-sex-race–adjusted 1996 annual per capita fee-for-service spending in the HRRs within each quintile on all Medicare services among enrollees age 65–99 years (9).

$^g$ Key attributes and average per capita supply of the specified medical resource in the HRRs within that quintile. Per capita supply is calculated per 1000 or per 10 000 residents of the general population within the HRRs (9).
Table 5. Quality of Care according to Level of Medicare Spending in Hospital Referral Region of Residence

<table>
<thead>
<tr>
<th>Variable</th>
<th>Quintile of EOL-EI</th>
<th>Test for Trend‡</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>1 (Lowest)</td>
<td>2</td>
</tr>
<tr>
<td></td>
<td>%</td>
<td>%</td>
</tr>
<tr>
<td>Acute MI cohort‡</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Received reperfusion within 12 hours</td>
<td>55.8</td>
<td>55.3</td>
</tr>
<tr>
<td>Received aspirin in the hospital</td>
<td>87.7</td>
<td>87.0</td>
</tr>
<tr>
<td>Received aspirin at discharge</td>
<td>83.5</td>
<td>82.5</td>
</tr>
<tr>
<td>Received ACE inhibitors at discharge</td>
<td>62.7</td>
<td>60.0</td>
</tr>
<tr>
<td>Received β-blockers in the hospital</td>
<td>61.5</td>
<td>61.0</td>
</tr>
<tr>
<td>Received β-blockers at discharge</td>
<td>52.7</td>
<td>53.2</td>
</tr>
<tr>
<td>MCBS cohort‡</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Preventive services</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Received influenza vaccine</td>
<td>60.3</td>
<td>56.3</td>
</tr>
<tr>
<td>Received pneumonia vaccine</td>
<td>29.4</td>
<td>28.7</td>
</tr>
<tr>
<td>Received Papanicolaou smear (among women without hysterectomy)</td>
<td>40.8</td>
<td>36.9</td>
</tr>
<tr>
<td>Received mammography (among women age 65–69 y)</td>
<td>48.7</td>
<td>46.9</td>
</tr>
</tbody>
</table>

* ACE = angiotensin-converting enzyme; EOL-EI = End-of-Life Expenditure Index; MCBS = Medicare Current Beneficiary Survey; MI = myocardial infarction.
† Arrows show the direction of any statistically significant association (P ≤ 0.05) between the percentage of patients receiving a specified service and regional EOL-EI differences. An arrow pointing upward indicates that as spending increases across regions, the percentage of patients receiving a specified service increases. A P value greater than 0.05 was considered not significant.
‡ Values are for patients who were ideal candidates for the specific treatment, defined as having no absolute or relative contraindication.

Patient Centered Outcomes Research

- Also known as Comparative Effectiveness Research (CER)
- AHRQ definition: “…a type of health care research that compares the results of one approach for managing a health problem to the results of other approaches. CER usually compares 2 or more types of treatment, such as different drugs, for the same disease. CER also can compare types of surgery or other kinds of medical procedures and tests.”
- Definition expanded to include comparison of behavioral change strategies and delivery systems
Essential Characteristics of PCOR

- Compares 2 or more different health care service approaches to managing a specific problem
- Options must be applicable in the “real world
- Outcomes measured must be meaningful to patients
- Provide information on which specific types of patients would benefit most from the interventions being studied
- Studies should have significant statistical power to meaningfully inform patients and providers
CER = Comparative Effectiveness Research

EBM = Evidence Based Medicine

HTA = Health Technology Assessment
Translation 3: Transformation to Deliver High Quality Care

What

T1 Translation from basic science to human studies (Bench to Clinical)

Clinical Science and Knowledge

Who

T2 Advance of tested ideas from basic science (Which Bedsides Need Care)

How

T3 Translation and transformation (Quality at All Bedsides)

Increased Quality & Value

Basic Biomedical Science ➔

Efficacy Research (Phase 1, 2, and 3 RCTs, e.g.)

Effectiveness Research
Health Services Research
Outcomes Research
Comparative Effectiveness Research

What Works ➔ Identifying Gaps ➔ Delivering Care Against the Gaps

Scaling
Spread
System Redesign
Learning Networks
Implementation
Quality Improvement
PCOR: Types of Studies

- **Pragmatic Randomized Controlled Trials**
  - Test interventions in real world conditions
  - Enroll patients similar to those seen by practicing providers
  - Allow adjustments based upon new information

- **Systematic Reviews (evidence synthesis)**
  - AHRQ Evidence based Practice Centers (EPCs)
  - Cochrane collaboration

- **Observational Studies**
  - Cohort studies
  - Use of large datasets – claims and clinical information
Examples of CER Studies

- **Diabetes Prevention Program (NIH)**
  - Compared Lifestyle changes vs. Metformin vs. Placebo in patients with pre-diabetes. Outcome: Development of diabetes
  - Lifestyle changes alone most effective

- **COURAGE Trial**
  - Compared angioplasty and stent (PCI) vs. medical therapy in older patients with stable angina
  - Medical therapy alone just as effective as PCI in preventing death or MI
Why should the Federal Government invest in PCOR?

- Rising healthcare costs - $2.3T per year in U.S.
  - Driven by technology and financial incentives
- Large geographic variations in care, especially when evidence is unclear
- Overall poor quality of care compared to other developed countries
  - Some US communities achieve lower costs and higher quality
- Production and use of better evidence may lower unnecessary costs
- 2007 Commonwealth Fund Report
  - Potential health system savings of $368 billion over 10 years, shared by all payers.
Federal Investments in PCOR - MMA

- Medicare Modernization Act of 2005 – Agency for Healthcare Research and Quality (AHRQ) Effective Health Care Program
  - Goal is to develop and disseminate better evidence about benefits and risks of alternative choices
  - Review and synthesize published and unpublished scientific evidence on a topic
  - Generate new evidence to address knowledge gaps
  - Translate research into concise reports for practical use by providers, patients and policy makers
Federal Investments in PCOR - ARRA

- American Recovery and Reinvestment Act 2009
  - $1.1 Billion allocated for CER
  - Created the Federal Coordinating Council for CER
  - Investments
    - Research
    - Data Infrastructure for CER
    - Dissemination and Translation of Results
    - Human and Scientific Capital (Researcher training and methods development)
  - All: primary focus on priority populations, conditions, and interventions
Federal Investments in PCOR - ARRA

- Institute of Medicine Report on CER – identified 100 top priority CER topics for funding
  - Compare treatment strategies for atrial fibrillation (surgery, ablation, drugs)
  - Compare effectiveness of robotic surgeries vs. conventional surgeries for common operations
  - Compare effectiveness of different strategies to enhance patient medication adherence
  - Compare effectiveness of obstetrical care with and without the use of ultrasound in normal pregnancies
Federal Investments in PCOR – Affordable Care Act

- Affordable Care Act of 2010 created the Patient Centered Outcomes Research Institute
  - Private, non-profit corporation with Federal representation from NIH and AHRQ
  - Identify priorities for research, especially for chronic conditions
  - Establish and continuously update a national research agenda
  - Establish a Methodology committee to improve the science and methods of PCOR
Federal Investments in PCOR – Affordable Care Act

- Requires AHRQ to disseminate research findings published by PCORI, train researchers in CER methods, and develop a publicly available database of CER studies and information.
- Requires the Secretary to invest in the building of data capacity in coordination with other Federal health programs.
- Limits the use of evidence from the research conducted.
PCORI Goals

- Stakeholder Engagement
- Review of existing and ongoing CER
- Fund projects to inform PCORI priorities and identify knowledge gaps
- Disseminate research findings to the public
- Expand research capacity (training and infrastructure)
- Reduce health disparities
Questions?