Harnessing the Potential of Comparative Effectiveness Research and Delivery System Innovation through Practice-Based Research

Glen Mays

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What the US gets for its investment

Figure 1. There are large differences in life expectancy and health care spending across OECD countries 2008.1

1. Or latest year available.
Source: OECD Health Data 2010.
Getting what we pay for?

Projected Spending on Health Care Under an Assumption That Excess Cost Growth Continues at Historical Averages

(Percentage of gross domestic product)

Source: Congressional Budget Office.
Factors driving growth in medical spending

Roehrig et al. Health Affairs 2011
Preventable mortality in the U.S.

Preventable Deaths per 100,000 population

Source: Commonwealth Fund 2008

Countries’ age-standardized death rates before age 75; including ischemic heart disease, diabetes, stroke, and bacterial infections. See report Appendix B for list of all conditions considered amenable to health care in the analysis.
Geographic variation in preventable mortality

Life Expectancy Differences
7.2 year gap among males
4.8 year gap among females
Research investments & expectations

National Institutes of Health Budget by Institute, 1998-2009 *
(budget authority in billions of constant FY 2008 dollars)

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Discovery to delivery diffusion

Events in the diffusion of ECMO technology

National Institute of Child Health and Human Development, 1990
Failures in T2 Translation: Variation in Use

Medicare Spending per Beneficiary, by Hospital Referral Region, 2005

Source: Congressional Budget Office based on data from the Centers for Medicare and Medicaid Services.
## Failures in T2 Translation: Under-use of effective care

The NEW ENGLAND JOURNAL of MEDICINE

### Table 3. Adherence to Quality Indicators, Overall and According to Type of Care and Function.

<table>
<thead>
<tr>
<th>Variable</th>
<th>No. of Indicators</th>
<th>No. of Participants Eligible</th>
<th>Total No. of Times Indicator Eligibility Was Met</th>
<th>Percentage of Recommended Care Received (95% CI)*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Overall care</td>
<td>439</td>
<td>6712</td>
<td>98,649</td>
<td>54.9 (54.3–55.5)</td>
</tr>
<tr>
<td>Type of care</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Preventive</td>
<td>38</td>
<td>6711</td>
<td>55,268</td>
<td>54.9 (54.2–55.6)</td>
</tr>
<tr>
<td>Acute</td>
<td>153</td>
<td>2318</td>
<td>19,815</td>
<td>53.5 (52.0–55.0)</td>
</tr>
<tr>
<td>Chronic</td>
<td>248</td>
<td>3387</td>
<td>23,566</td>
<td>56.1 (55.0–57.3)</td>
</tr>
<tr>
<td>Function</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Screening</td>
<td>41</td>
<td>6711</td>
<td>39,486</td>
<td>52.2 (51.3–53.2)</td>
</tr>
<tr>
<td>Diagnosis</td>
<td>178</td>
<td>6217</td>
<td>29,679</td>
<td>55.7 (54.5–56.8)</td>
</tr>
<tr>
<td>Treatment</td>
<td>173</td>
<td>6707</td>
<td>23,019</td>
<td>57.5 (56.5–58.4)</td>
</tr>
<tr>
<td>Follow-up</td>
<td>47</td>
<td>2413</td>
<td>6,465</td>
<td>58.5 (56.6–60.4)</td>
</tr>
</tbody>
</table>

McGlynn et al. 2003
Failures in T2 Translation: Under-use of effective care

McGlynn et al. 2007
Failures in T2 Translation: Over-use of costly, equivocal care
Failures in T2 Translation:
Disparities in care

- Pediatric hospital admissions for asthma
- Prenatal care in 1st trimester
- Pneumococcal vaccination
- Children counseled on physical activity
- Colorectal CA screening
- Depression treatment
Less than 50% of the population at risk is reached by:

- Smoking cessation
- Aspirin use
- Colorectal cancer screening
- Influenza vaccines
- Nutrition and physical activity programming
A changing paradigm for health research

- Research often fails to inform the choices faced by clinicians, patients, payors, policy-makers
- Need head-to-head comparisons of all relevant choice options and combinations
- Need evidence on effectiveness in real-world clinical & community settings
- Need to know whether/why interventions work for some and not for others (treatment heterogeneity)
- Need to determine value from the consumer’s perspective (patient-centered outcomes and costs)
CER Defined

- "Comparative effectiveness research is the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor disease and improve the delivery of care.

- The purpose of CER is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population levels."

-National Academy of Sciences Institute of Medicine, 2009
Fundamental empirical questions

- Which programs, interventions, policies (*mechanisms*)…
- Work best (*outcomes*)…
- In which institutional & community settings (*contexts*)…
- And why (*causal pathways, interactions*)?

Pawson and Tilley 1997; Berwick 2008
Recent developmental history

- **2003 Medicare Modernization Act**: $30M annually for research to improve quality, effectiveness, efficiency
- **2007** federal legislation to expand CER passed House but failed Senate
- **2009 ARRA**: $1.1B to NIH and AHRQ for CER
  - Federal Coordinating Council for CER established
  - IOM Top 100 Priority Topics for CER identified
    - 50% involve health care delivery system
    - 33% address health care disparities
    - 20% address patient functional limitations or disabilities
- **2010 ACA**: $600M annually through Patient Centered Outcomes Research Institute
Methods for CER

- Require both prospective trials and observational studies
- Advanced analytic methods used to strengthen internal validity and limit bias due to selection, confounding
  - Propensity score and instrumental variables models
  - Explicit testing for treatment heterogeneity
  - Latent variable models for multiple interventions, multiple outcomes
  - Non-inferiority analyses
- Development and integration of large clinical and administrative data sources and registries
- Use of Bayesian models for synthesizing data from multiple studies, e.g. indirect treatment comparisons
Delivery System Innovations & CER

Triple Aim Goals:

- Improving individual experience with care
- Improving population health
- Reducing per-capita costs of care for populations

Key Components:

- Partnerships with individuals, families, communities
- Redesign of primary care
- Population health management
- Financial management
- Integration across continuum of care
Delivery System Innovations & CER

- Patient centered medical home models
- Community health teams
- Community health worker models
- Care transition models
- Accountable care organizations
- Bundled payment methods
- E-health and mobile health applications
Community-Level Research in CER

- Identify community-level factors that affect implementation of clinical interventions → effect modifiers
- Estimate effects of complex community-level interventions
- Estimate cumulative effects of multiple interventions at the population level
- Study the effects of environmental exposures (alone or in combination with clinical interventions)
- Evaluate spill-over effects of clinical interventions on larger populations (beyond the treated)
- Study downstream effects of prevention strategies on disease prevalence, care-seeking behavior, utilization
Primary Care & Public Health Integration

- Community Transformation Grants
- CMMI Community Health Innovation Awards
- Medicaid-funded Community Health Teams
- AHRQ Primary Care Extension Program
- HRSA Public Health Training Centers

Some Examples of Delivery System Research and CER
Example: Testing a delivery system innovation to promote VLBW deliveries in appropriate settings

- Tele-health support to promote VLBW infants delivered at NICU v. non-NICU facilities

- **Selection Problem**: complex and high-risk cases are more likely to be referred to higher-level facilities for delivery

- **Censoring Problem**: facilities may differ in rates of in-hospital mortality due to case mix and quality differences, thereby distorting measures of resource use
Example: Testing a program to promote VLBW deliveries in appropriate settings

<table>
<thead>
<tr>
<th>Model</th>
<th>IRR-LOS</th>
<th>95% CI</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Standard multivariate risk adjustment</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Tertiary NICU facilities†</td>
<td>1.25</td>
<td>1.23, 1.27</td>
</tr>
<tr>
<td>Community NICU facilities†</td>
<td>1.32</td>
<td>1.30, 1.34</td>
</tr>
<tr>
<td>2. Control for mortality censoring</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Tertiary NICU facilities†</td>
<td>1.13</td>
<td>1.12, 1.15</td>
</tr>
<tr>
<td>Community NICU facilities†</td>
<td>1.24</td>
<td>1.22, 1.26</td>
</tr>
<tr>
<td>(In-hospital mortality effect</td>
<td>0.74</td>
<td>0.53, 0.95</td>
</tr>
<tr>
<td>3. IV control for hospital selection &amp; mortality</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Tertiary NICU facilities†</td>
<td>0.93</td>
<td>0.88, 0.98</td>
</tr>
<tr>
<td>Community NICU facilities†</td>
<td>1.17</td>
<td>1.14, 1.20</td>
</tr>
<tr>
<td>(In-hospital mortality effect</td>
<td>0.23</td>
<td>0.11, 0.36</td>
</tr>
</tbody>
</table>

Significantly lower resource use when deliveries occur at tertiary NICU facilities

IRR = Adjusted incidence rate ratio, from negative binomial model
†Reference = Community hospitals without NICU
Example: Comparative efficiency of community-based vs. institutional long-term care for elderly/disabled

- CCP program links elderly and disabled Medicaid recipients with community-based long-term care services to avoid/delay need for institutional care
- Determine the impact of the CCP program on Medicaid expenditures for elderly and disabled recipients eligible for long-term care services
- Determine whether the CCP program is cost-neutral to Medicaid after accounting for both Medicaid expenditures and program operating costs
Methods: Comparison Group

Comparison Group: statistically matched on age, gender, race, eligibility category, enrollment duration, waiver enrollment, comorbidities, prior-year spending
Approximate a “statistically equivalent” control group that would be generated by random assignment. Each subject has an equivalent probability of being a CCP participant.
Estimates of Program Impact

Regression-Adjusted Spending Estimates
Example: Reductions in preventable mortality attributable to public health spending

"Communities with larger growth in public health spending experienced larger reductions in preventable mortality rates."

Mays et al. Health Affairs 2011
Example: Reductions in medical care spending attributable to public health investments

Spending estimates adjusted for age, comorbidities, and service mix

Mays et al. Health Services Research 2009
Advancing Delivery System Research with PBRNs

- Use networks to identify, design and test multi-component interventions:
  - Primary care
  - Public health
  - Dental, etc

- Take advantage of emerging clinical data resources: EMRs, registries, linkage to administrative data

- Capitalize on new funding opportunities: CMMI, PCORI, AHRQ

- Include a focus on population-level health impact