Comparative Effectiveness – Implications for MedTech Companies

March 30, 2011

Randel Richner, BSN, MPH,
Founder and President, Neocure Group, LLC
Presentation Overview

• Health Reform: Comparative Effectiveness and the New Evidence Standards
• What Does Comparative Effectiveness Mean?
• Methods of Market-focused Evidence Development
• What This All Means for Manufactures: A New Evidence Imperative
NEW STANDARDS FOR EVIDENCE RESULTING FROM HEALTH REFORM
US Health Reform Represents A Chance To Showcase Promising Technology

“Three broad aims to guide and assess local, state, and national efforts to improve health and the quality of health care.”

<table>
<thead>
<tr>
<th>Government Expects:</th>
<th>Impact on New Technology Reimbursement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Better Care</td>
<td>• Universal coverage</td>
</tr>
<tr>
<td></td>
<td>• Medical home</td>
</tr>
<tr>
<td></td>
<td>• Quality benchmarks in hospitals, and for physicians</td>
</tr>
<tr>
<td>Healthy People/Healthy Communities</td>
<td>• Coordination of care</td>
</tr>
<tr>
<td></td>
<td>• Coverage limitations</td>
</tr>
<tr>
<td></td>
<td>• Coverage with evidence development</td>
</tr>
<tr>
<td></td>
<td>• Comparative effectiveness; cost-effectiveness</td>
</tr>
<tr>
<td>Affordable Care</td>
<td>New payment models:</td>
</tr>
<tr>
<td></td>
<td>• Hospital-driven</td>
</tr>
<tr>
<td></td>
<td>• Accountable Care Organizations (ACOs)</td>
</tr>
<tr>
<td></td>
<td>• Bundled/episode based payments;</td>
</tr>
<tr>
<td></td>
<td>• Medical-home</td>
</tr>
</tbody>
</table>

Source: US HHS; Interagency Working Group is authorized by the Affordable Care Act, Section 3012.
### Initiatives legislated by Congress

<table>
<thead>
<tr>
<th>Initiative</th>
<th>Goal</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Independent Payment Advisory Board (IPAB)</strong></td>
<td>• Goal: reduce the per capita rate of growth in Medicare spending</td>
</tr>
<tr>
<td></td>
<td>• Will be required to produce annual reports addressing cost control functions, health care costs, access to care, quality of care, utilization of services, and comparisons by types of providers and by region</td>
</tr>
<tr>
<td><strong>CMS Center for Medicare &amp; Medicaid Innovation (CMI)</strong></td>
<td>• Goal: To test innovative payment and service delivery models to reduce program expenditures while preserving or enhancing quality of care</td>
</tr>
<tr>
<td></td>
<td>• Entity within the CMS that will initiate efforts by January 1, 2011</td>
</tr>
<tr>
<td></td>
<td>• $10 billion in funding for the CMI</td>
</tr>
<tr>
<td><strong>Patient Centered Outcomes Research Institute (PCORI)</strong></td>
<td>• Goal: nonprofit corporation designed to set a national agenda for comparative effectiveness research (&quot;CER&quot;) efforts</td>
</tr>
<tr>
<td></td>
<td>• Data should not be construed as &quot;mandates for practice guidelines, coverage recommendations, payment, or policy recommendations.&quot;</td>
</tr>
<tr>
<td></td>
<td>• CMS is permitted to take the Institute's findings into consideration when making coverage determinations, but <strong>CMS cannot rely solely on the Institute's findings, and it must provide an opportunity for public input</strong></td>
</tr>
</tbody>
</table>
The “Official” CER Initiative: PCORI

• PCORI established as new nonprofit with multiple governance and advisory boards
  – Conduct range of Comparative Effectiveness Research (meta-analysis, RCTs, novel approaches) within guidance from methods advisory panels
  – Priorities must account for “…relative value determined based on the cost of conducting research compared to the potential usefulness of the information produced by research....”

• Recovery Act provided $1.1 billion for patient-centered, comparative effectiveness research (CER)
  – $400 million to NIH
  – $400 million to the Office of the Secretary (HHS)
  – $300 million to the Agency for Healthcare Research and Quality (AHRQ).
2011: Establishing PCOR National Priorities

In order to determine the appropriate priorities for further research, the PCORI has outlined assessment criteria:

- Innovative Research
- High value disease/disorders
- Impact of employer principles
- Potential for multiplicative effect
- Health disparities research on priority populations
- Disease areas & delivery models that are otherwise not incentivized for study

FIGURE 5-1 Distribution of the recommended research priorities by primary and secondary research areas.

WHAT DOES COMPARATIVE EFFECTIVENESS MEAN?
An Array of Definitions: “Comparative Effectiveness”

The term for comparative effectiveness has gone through many iterations over the years, but each definition has the same goal: *To understand the impact of clinical interventions on patient outcomes and to justify and quantify the value of such treatment compared to current practice.*
Defining “Patient-Centered Outcomes Research”

The definition of PCOR & “comparative effectiveness” continues to evolve

• All definitions describe need to improve patient outcomes using evidence-based, real-world information

Draft Definition 1:
Patient-centered outcomes research seeks to understand and improve the effects of healthcare and prevention services on outcomes important to all persons with disease or at risk for disease considering individual perspectives, needs, preferences, biological, environmental, behavioral, and cultural determinants of health.

Draft Definition 2:
Patient-centered outcomes research is targeted specifically to help patients live longer, live better, or preferably, both. It highlights and enhances the fundamental right of patients to choose specific interventions and strategies to prevent, treat, or manage conditions using evidence-based information on the relative comparative benefits and harms of different options. It also incorporates research on interventions that are targeted to individual-level biological, environmental, behavioral, and cultural characteristics and preferences.

Draft Definition 3:
Patient-centered outcomes research is research that determines the real world effectiveness (results) of interventions on outcomes that persons’ care about, including survival, function, symptoms, and health-related quality of life. PCOR includes variability in individuals’ risk of experiencing the outcomes and likelihood of benefit or harm of the interventions.

Draft Definition 4:
Patient-centered outcomes research seeks to understand and improve the effects of health services. It incorporates patient’s perspectives. By including biological, environmental, behavioral, and cultural components and in setting research priorities. It must optimize outcomes while taking account of multiple competing demands of: 1) Population health considerations 2) Other stakeholder perspectives (payers, policymakers and industry) 3) Barriers to implementation; Generates guidance to enable patient participation and overcome barriers to implementation.

Comparative effectiveness research justifies pricing levels and seeks to demonstrate clear patient outcomes.
What is the Value of Comparative Effectiveness Research?

“Comparative Effectiveness Research typically will focus on realistic decisions confronting patients and their clinicians in actual practice.... Because of this focus on effectiveness as opposed to efficacy, these investigations will likely rely on both prospective trials and observational data to determine relative value in real-world settings.”

-- IOM Roundtable on Evidence-Based Medicine

Outcomes from CER studies will drive utilization patterns and may potentially limit coverage & reimbursement rates if new technologies cannot demonstrate incremental benefit against standard of care.
METHODS OF MARKET-FOCUSED EVIDENCE DEVELOPMENT
Direct CER

- Head-to-head randomized clinical trials (RCTs) (Example)
  - Very large, costly, and time consuming
  - Compares technologies across same controlled population, time, and methods
  - Approach is not real-world based, and the impact on actual practice has been debated, particularly given the time and cost invested

- Prospective Registries (Example)
  - Typically completed to comply with safety demands of regulatory agencies (e.g., FDA, EMA, etc.)
  - Can be designed to collect comparative information for competing technologies
  - Findings demonstrate respective outcomes within the studied population (correlations), but rarely (if ever) demonstrate causation
Indirect CER

- Use Secondary Database Analyses (Example)
  - Includes claims database, chart review, registries, inpatient/outpatient datasets, etc.
  - Data collected results in potential to identify correlations more readily than causations
  - Assumptions for CER based on real-world outcomes, but not always reflective of true comparison (e.g., is the observed outcome a result of the technology or the practice?)

\[ X \leftarrow Y \rightarrow Z \]
WHAT THIS ALL MEANS FOR MANUFACTURES: A NEW EVIDENCE IMPERATIVE
Health Reform: Payment Tied to Episodes of Care

• Reform-based efforts focusing on paying for episodes of care, as opposed to individual procedures
  – Makes providers financially responsible for added costs resulting from low quality care
  – If reimbursement rates are set correctly, high-quality performance should be rewarded through cost savings associated with high-quality patient care
• Link payment to quality care; drives CER with heightened standards while minimizing costs
• Pressure on providers to prove patient outcomes will translate to increased demands of evidence of comparative effectiveness from manufactures to support their value propositions
Strong Shift Towards Higher Level of Evidence Requirements

- Payers have moved rapidly toward a stronger emphasis on evidence from high-quality scientific studies—this has led to more narrowly focused coverage policies

<table>
<thead>
<tr>
<th>Effects of Narrowly Focused Coverage Policies</th>
</tr>
</thead>
<tbody>
<tr>
<td>Speed of Adoption of New Technologies</td>
</tr>
<tr>
<td>Overall Utilization of New Technologies</td>
</tr>
<tr>
<td>Quality of Care of <strong>Covered</strong> Patients</td>
</tr>
<tr>
<td>Quality of Care of <strong>Non-Covered</strong> Patients</td>
</tr>
</tbody>
</table>
Case Study: New Procedures have Higher Evidence Thresholds (CER)

**Old Business Model Technology**
- Widespread coverage by payers; minimal pre-certification hurdles

**Open Back Surgery**
- Performed for 50+ years by surgeons
- Few studies conducted; poor outcomes
- $50,000+ per procedure

**Minimally Invasive Back Surgery**
- Performed by variety of specialists
- Launched with minimal data; robust studies now underway
- $5,000-$30,000 per procedure

**New Business Model Technology**
- Limited payer coverage; payers requiring more evidence to approve

To be category leader, clear value proposition and supporting clinical evidence is needed

---

Neocure Group LLC · info@neocuregroup.com
What This Ultimately Means for You

• All payers, and especially CMS, will pay for value as opposed to treatment

• Manufacturers need to get involved in policy development early and active roles in defining what value looks like
  – Associations and policy groups to add to the voices in Washington (e.g., MDMA, CMTP, NQF, etc.)
  – Partner with employer groups, ACOs, and care managers to define optimal courses of care and outcomes

• Data is King: Successful manufactures will be those that have market-focused studies proving positive clinical value and justifiable cost (if not cost savings)
## Developing the Value Proposition

<table>
<thead>
<tr>
<th>Early Development</th>
<th>Launch Phase</th>
<th>Post-Launch</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Assess key stakeholders</td>
<td>• Lead with compelling VP supported by Clinical &amp; Economic Evidence</td>
<td>• Enhance VP with additional evidence</td>
</tr>
<tr>
<td>• Understand unmet needs</td>
<td>• Support value pricing of new products</td>
<td>• Maintain premium pricing position</td>
</tr>
<tr>
<td>• Likely perspective on value</td>
<td>• Minimize time in hospital’s technology assessment</td>
<td>• Protect comparative effectiveness/value</td>
</tr>
<tr>
<td>• Influence on product adoption</td>
<td>• Pursue rapid product adoption; create reference clients</td>
<td>• Provide economic tools to support VP and minimize hospital contracting cycle</td>
</tr>
<tr>
<td>• Determine evidence needs to support reimbursement</td>
<td></td>
<td></td>
</tr>
<tr>
<td>• Initiate evidence-based studies for Value Proposition (VP)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>• Influence clinical study design &amp; integrate economic endpoints</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Define Optimal Value**

**Collect /Publish Data on Real-World Value**

**Leverage VP for Hospital Contracting**

Adapted from: Harding, USC, 08
Aligning Internal Resources

• Enhancing traditional HEOR and market access capabilities to satisfy HTA (e.g., database capabilities)

• Enhancing integration across company departments
  – Planning for market must start earlier, even during ongoing product development, with R&D, Clinical
  – Evidence plans created earlier may be able to support both regulatory and reimbursement needs

• Engaging in conditional coverage/risk-sharing schemes where appropriate
  – Possible partnering with payer/health plans/ACOs (Hospitals)

• Premium is on innovative efforts that enter market with stronger evidence of (comparative) effectiveness
Final Thoughts

• PCORI, IOM, AHRQ : guidance, methods recommendations, study priorities, research agendas and initiatives to conduct more clinically meaningful evidence to improve care for patients.

• Manufacturers: must offer hospitals a relevant value proposition aligned with new economic reality – evidence on clinical utility and related economic benefit (a.k.a. CER).

Technology innovators have an opportunity to contribute and insist on use of products that change the quality of care, as long as we have the evidence to back-up our claims.