Update on Comparative Effectiveness and Health Information Technology Legislation and Policy Developments, and Impact on Pharmacoeconomics

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Update on Comparative Effectiveness Legislation and Considerations to Ponder

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ISPOR Health Science Policy Council Mission

To advise the Society on important science, research and policy issues in pharmacoeconomics and outcomes research.

Disclaimer

The views expressed in this presentation are my personal views and do not necessarily represent the views of sanofi-aventis

Talking Points

• Legislation
• Considerations
Options would create incentives for health care providers to focus on high quality care and to closely coordinate with a patient’s other doctors and providers.

Infrastructure Investments: Tools to Support Delivery System Reform – Comparative Effectiveness Research

- Ensuring Credible and Objective Research
  - Development of methods and standards for such research
    - Independent expert committee charged with developing methodological standards for this type of research should be established.
    - Research could be guided by expert advisory panels or subject to a peer review process
  - Transparency and Public Input
    - Public comment and input should be integral to CCERs
  - Options include:
    - Comment on research agenda, design, draft reports, priorities, and dissemination approaches
    - Peer-review of research designs and findings
    - Research findings publicly disseminated and easily understood

Infrastructure Investments: Tools to Support Delivery System Reform – Comparative Effectiveness Research

- Patient Safeguards
  - Institute considers patient subgroup responses to different strategies when designing/approving studies
  - Institute disseminates findings but prohibited from issuing medical practice or coverage and reimbursement recommendations
  - Create limits on the use of the research by HHS
    - Process must be transparent
    - Relies on all available evidence
    - Considers effects on beneficiary populations
  - Allows for public comment on draft proposals using the (CER) information
  - This would prohibit HHS agencies from creating a fast-track process for automatically linking the research findings to coverage or reimbursement decisions in public programs.
**Talking Points**

- Legislation
- Considerations

**Considerations to Ponder**

- AHRQ, NIH, and the Secretary will use ARRA funds to evaluate and develop comparative clinical effectiveness research (CCER) methodologies and to conduct CCERs for predetermined priorities
- ARRA investment in EMR/EHR/PHR/eRx will yield aggregated electronic data bases practitioners and researchers could search using clinical management software for CCE information
- Use of CCERs in coverage and reimbursement decision making by payers is unclear
- Issues exist around the definition of “cost” and when and how it should be used

**Considerations to Ponder**

- Evidence needed on the usefulness and benefits of value methodologies for health care providers, patients, and payers
- Unclear if providers and patients know how to use CCER Guides and whether they are using them to make health care decisions
- Research needed on when CCER reports expire and should be reexamined
- Periodic monitoring or auditing of procedures and processes for conducting and using CCERs may be necessary to assure quality for providers, patients, and payers
- Focus of providers, patients and payers will be on Value and Evidence, not cost and efficacy

**Summary**

- Policy Options
- Considerations

**What is Comparative Effectiveness, and Why Do We Need It?**

Joel Hay PhD
Department of Pharmaceutical Economics & Policy
University of Southern California
Los Angeles, CA, USA
What Is Comparative Effectiveness?

We spend $2.7 trillion on health care annually

Rand estimates that 1/3 of this medical spending is harmful or useless

$900 billion per year! This is larger than the Obama stimulus package

Evidence is Important

Evidence Eras in the US

1970's: Health Technology Assessment (HTA)
1980's: Effectiveness Research
1990's: Outcomes Research
2000's: Evidence-Based Medicine

Of Late: “Comparative Effectiveness Research”

Coming?: “Payment for Outcomes”

Why Don't We Know What Works?

Current institutions under-provide such data. In terms of medical interventions, estimates of the share of existing interventions that have a solid evidence base vary, though many researchers believe the share is “well below half.”

David Eddy, a leading advocate of evidence-based medicine, estimates the share to be as low as 15 percent.
Medicine Is not Evidence-Based

Scientific Evidence Underlying the ACC/AHA Clinical Practice Guidelines

Why a Hierarchy of Evidence?

- Randomized controlled trials are the only way to estimate unbiased treatment effects
- In any observational dataset (cohort, case control, pre/post, etc.) there is always the possibility that treatment effects are biased by observable or unobservable confounders
- It is impossible to test whether the methods and variables used to correct treatment bias are correct

RCTs Better Than Other Studies

- All treatment selection methods use un-testable assumptions
  - Propensity score methods require a crucial assumption that unobservable factors are random
  - Heckman selection bias methods require distributional assumptions
  - Instrumental variable methods require IVs

The Evidence Wave

Today, the US is experiencing a new "Evidence Wave."

Bryan R. Luce, PhD, MBA
Chair, ISPOR Health Science Policy Council & Senior Vice President, Science Policy, United BioSource Corporation, Bethesda, MD, USA
Consider what (former) CBO (soon OMB) Director Peter Orszag presents:

**The Relationship Between Quality and Medicare Spending, by State, 2004**

Composite Measure of Quality of Care

Source: Data from AHRQ and CMS (as presented by Dir. Orszag, CBO)

**Stakeholders, Especially Health Plans, Call for a Centralized CER Entity**

- November 2006: Wilensky HA Article
- May 2007: BCBS proposal
- June 2007: AHIP proposal
- July 2007: IOM recommendation
- August 2007: Baucus/Conrad CER Institute
- October 2007: Kennedy HELP
- December 2007: Waxman HEC
- February 2008: President’s FY09 Budget doubles funding for AHRQ’s EHC Program
- August 2008: Sen Baucus and Conrad bill
- January 2009: ARRA includes $1.1 billion for CER efforts
- December 2009: Sen Baucus releases health care reform proposal

**American Recovery and Reimbursement Act of 2009 Includes...**

- $1.1 billion for CER efforts
- $300 M for AHRQ
- $400 M for NIH*
- $300 M OS ($1.5 M for IOM priority setting)
- Establishes the Federal Coordinating Council for CER

*Trial seed money!

**So, the Future (Relative to CER), Given...**

- IOM, Payers, Clinical Leaders, Academics, Politicians all calling for:
  - Investment in evidence (generally)
  - New CER Institute (specifically)
- Obama as President
- HHS on board
- Orszag as OMB

**So, the Future (Relative to CER), Given...**

- Baucus: Senate Finance (with Conrad on board)
- Kennedy: Senate HELP
- Waxman: House Energy & Commerce

*in light of...*
The Financial Crisis, with...

- Health Costs believed to big part of problem
- Evidence generation believed to important element of the solution
- Waning political opposition to new evidence policies
  - Traditional opponents somewhat co-opted as included stakeholders

Conditional Coverage (including P40) being tested by...

- Medicare
- NICE
- Canada
- Other EU countries

...that we will continue to see...

- Sustained federal funding for CER to include
  - Federally-funded comparative trials
  - Investment in infrastructure (especially HIT, EMR)
  - More $ for traditional systematic reviews/HTA activity
- Strengthening “conditional coverage” policies by CMS, and possibly major private payers
- Increased CER trial activity by manufacturers to preempt the above

Risky Ventures for Manufacturers, e.g...

- PROVE IT (Pravastatin or Atorvastatin Evaluation and Infection Therapy–Thrombolysis in Myocardial Infarction) trial*
- ENHANCE (Effect of Combination Ezetimibe & High-Dose Simvastatin vs. Simvastatin Alone on the Atherosclerotic Process in Patients with Heterozygous Familial Hypercholesterolemia) trial

Neither trial concluded in favor of the sponsors’ products.

...that we will continue to see...

- Systematic Reviews by DERP, IQWiG, and Others

- Finding no direct, H-H comparisons, concluding: “no evidence of difference”
- Being interpreted (incorrectly) by some payers as...
- Evidence of no difference!

All of which means (to me)...

Traditional Comparative Trials are 1) Costly; 2) Take Lots of Time, e.g...

- Women’s Health Initiative: $725 M; 5.2 yrs; follow up to 2010
- ALLHAT (Antihypertensive and Lipid-Lowering Treatment to Prevent Heart Attack) Trial: $135 M; 5 yrs
- CATIE (Clinical Antipsychotic Trials of Intervention Effectiveness): $40 M; 4 yrs

and can be...
Thus...

Although the new CER evidence wave is, in large part, an enlightened approach to...

- Improve health care decision-making
- Better target medical products and HC services
- Contain costs

...to my mind, CER trials are unsustainable without transformational change in generating evidence

Three Potential Transformational CER Initiatives: CMTP, CTTI, PACE

- Center for Medical Technology & Policy (CMTP) (Collaborative stakeholder and proof of concept movement)
- Clinical Trial Transformation Initiative (CTTI) (Movement to streamline operations, logistics, and regulatory inefficiencies)
- Pragmatic Approaches to Comparative Effectiveness (PACE) Initiative (Streamlining analytical...including Bayesian adaptive... methods)

Some CER Trial Questions to Address

- How can we make maximum use of the evidence we have when designing a CER trial?
- When do we know (what is the minimal threshold) when we have “just enough” evidence for a real world (read, coverage decision maker; clinical guideline committee) decision? Is there any such thing as “p = .05” in the real world?
- To what extent can dynamic predictive simulation reduce risk of failure? Or improve opportunity for success in designing the pragmatic trial?

Some CER Trial Questions to Address (continued)

- Can comparative effectiveness trials be designed to evaluate heterogeneity of treatment effect; of patient preference, adherence, etc.? (Role for adaption process?)
- Can we design continuous learning trials to mirror IOM’s “learning health care system” concept? For instance, can we adapt the trial design as we gain experience in a drug’s place in therapy?

Some CER Trial Questions to Address (continued)

- How much does cost, time and/or risk need to be decreased for a manufacturer to be willing to fund a CE trial of its product? (e.g., what is the ROI elasticity?)
- To what extent can/should Phase 3/3B be coordinated with CER needs of payers?
Comparative Effectiveness Studies: The Value of Research and Cost Analysis to Managed Care

Diana Brixner RPh, PhD
Executive Director, Pharmacotherapy Outcomes Research Center, University of Utah, Salt Lake City, UT, USA

Why Comparative Effectiveness: Payers need information beyond RCTs ... Efficacy and safety in a small population with a restricted study protocol

RCTs Randomized Clinical Trials
GAP
Decision makers need real world information to make health care decisions for large populations within defined budgets

Real World Data

What is the Role of Comparative Effectiveness?

• To provide information between alternative therapies as to which works better in actual practice
• Sources for this information
  • Patient registries
  • Head to head observational studies
  • Systematic reviews
  • Retrospective comparisons
    • Insurance claims databases
    • Electronic medical Records

Will Comparative Effectiveness be used for Cost-Effectiveness

• This is a concern by those who believe cost-effectiveness would limit access to medications
• However, we can not conduct comparative effectiveness in a vacuum that does not consider costs
• Payers will consider effectiveness and costs

The Controversy of Comparative Effectiveness

• Supporters believe CE to be a key part of reform to improve efficiency and direct money towards the most effective treatments
• Opponents believe CE will limit patient access to medical treatment, and be used to deny needed care
• These results may indeed help save money, but the real test is how they affect patient outcomes

“If we appropriately execute comparative effectiveness studies, then the cost-effectiveness analyses that are conducted also will improve based on a more accurate denominator.”

- ISPOR Past, Current and Elected Presidents

http://www.ispor.org/workpaper/paper_comments/index.asp
The Comparative Effectiveness Scenario

- If 95% of patients improve on treatment regimen A, will insurers cover treatment regimen B if it only works for the remaining 5%?
- If treatment B costs 100 times as much as treatment A, how difficult would it be to get an exception?
- Development of new approaches
  - Coverage with evidence
  - Value Based Insurance Design

Wall Street Journal: April 14, 2009

Push to Compare Treatments Worries Drug, Device Makers

By JANC ZIANO

WASHINGTON -- Federal health-care agencies are getting $1.1 billion in economic-stimulus funds for research comparing the effectiveness of various treatments. But drug and medical-device makers, along with some members of Congress, say they are worried the findings will be used to limit patients' options.

Answering Basic Questions
(Source: Drummond et al., IJTAHC, June, 2008)

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Summary

- What are MCO expectations for comparative effectiveness research?
  - Who will provide it
  - Who will evaluate it
  - How will it be delivered
- Will comparative effectiveness improve decision making?

Summary

- Will cost be considered alongside comparative effectiveness?
- How will formulary processes accommodate comparative effectiveness?

Reaction & Discussion
Thank you for attending

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