

How Much Benefit is Good Enough?

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Beyond the Randomized Trial

- Randomized trial may establish efficacy of treatment
- Trial data must be translated into policy
- Policy decisions are value-laden

Is the Intervention Good Enough?

- To make treatment widely available
 - Develop new centers, train physicians
- To substitute new treatment for existing approach
 - Change formularies; modify guidelines
- To pay for new intervention
 - Third party payers (Medicare, health plans)

Current Decision-Making Process

- Practice guidelines
- Food and Drug Administration approval
- Centers for Medicare and Medicaid process for reimbursing new technology

Basis for Policy Decisions Today

■ Utilitarian approach

- If on average patients who get new rx are better off, make rx available
- Acceptable to make some people worse off if there is net improvement

Values Implicit in Current Approach

- Some people may be harmed by intervention, but that's all right as long as treated group benefits on average
- If there is a risk of an adverse effect, intervention may be withheld from all
- Cost is not a relevant consideration
 - Resource allocation, justice unimportant

Example: CMS Coverage Decisions

- CMS mandate: provide services that are reasonable and necessary
 - For its 42 million beneficiaries
- Repeated efforts to define criteria have failed

CMS Approach

- Many decisions made by local carriers
- Controversial decisions referred to CMS for national decision
- Manufacturers, professional organizations can request policy decision
- Requesters submit outcomes data

CMS Decision-Making

- Exhaustive review of scientific evidence
 - by Coverage and Analysis Group
- Technology assessment
 - May be commissioned by AHRQ
- Comprehensive review
 - by Medicare Coverage Advisory Committee

CMS Issues Decision Memorandum

- To inform public of decision
- Specify reasons for decision
- Outline process followed
- Summarize evidence considered
 - May specify conditions for use
 - May demand further clinical trials

Medicare Coverage Advisory Committee

- Chartered 1998
- Advisory role to CMS
- Composed of 100 representatives from science, medicine, economics, law
- Subcommittee chosen for each decision
 - Includes non-voting representatives of public, industry
 - Indicates whether reported benefits translate into improved net health outcomes

How CMS Approach Plays Out: Left Ventricular Assist Device

- Involves surgical implantation of prosthetic pump
- LVAD accepted rx for patients awaiting heart transplant
- New indication CMS asked to pay for: definitive rx for older, sicker patients

LVAD as “Destination Therapy”

- 68 patients assigned to device; 61 controls
- 48% decrease in all-cause mortality with rx
- Survival rate with device 52% at 1 year
- Survival rate with device 23% at 2 years
- Quality of life improved
- High morbidity: infections (28%), bleeding (42%)

■ Rose et al; NEJM 2001

CMS Evaluation Process

- CMS receives request to reimburse for new indication
- Medicare Coverage Advisory Committee input (votes 6:1 to approve subject to constraints on eligibility)
- CMS repeatedly postpones decision
- CMS agrees to provide coverage
- CMS sets reimbursement at \$70,000

Actual Cost of LVAD

- Device alone: \$65,000
- Hospitalization total: \$200,000
- Total cost/year to Medicare program:
 - \$350 million (5000 cases)
 - \$7 billion (100,000 cases)

Beyond Outcomes Data

- What if we consider cost?
- What if we factor in values?

Suppose Cost is Included in Decision-Making

- Cost-effectiveness is technique for selecting among competing options when resources are limited
- Cost-effectiveness ratio=
$$\frac{\text{Cost (new)} - \text{Cost (current)}}{\text{Effectiveness (new)} - \text{Effectiveness (current)}}$$

Quality-of-Life Considerations

- $QALY = \text{life expectancy} \times \text{quality of remaining years}$
- Single score to evaluate health state based on mobility, pain, anxiety...
- Assigns utilities to health states
 - Year of perfect health=1
 - Death=0
 - States may be worse than death (negative)

Standards for C-E Analysis

- Use societal perspective
- Include all health effects, costs that flow from intervention
- Include benefits and harms
- Use $CE = \frac{\text{Cost}(\text{new}) - \text{Cost}(\text{old})}{\text{QALYs}(\text{new}) - \text{QALYs}(\text{old})}$

Incorporating Values into Policy

- Give preference to patients in poor health
- Give preference to vulnerable populations
- Give preference to interventions that are of large magnitude (even if few benefit)

What Might LVAD Decision-Making Look Like with Values, Cost?

- Start with scientific data
- Use cost-effectiveness threshold
- Vary C-E threshold depending on special circumstances

Cost-Effectiveness of LVAD

- CE ratio/QALY: best est. \$802,700/QALY
 - Low: \$500,000/QALY
 - High: \$1.4 million/QALY
- Benchmark \$50,000-\$100,000/QALY
 - Blue Cross/Blue Shield with Southern Permanente Health Plan and Kaiser Foundation

Policy Conclusions Using New Approach: Case of LVAD

- Start with scientific data; conclude small net benefit with many side-effects; consider rx
- Does intervention meet cost effectiveness threshold of \$100,000/QALY?--NO
- Should threshold be modified to give preference to patients who are extremely ill?—YES
- Does intervention meet higher threshold, eg \$200,000/QALY?--NO

From Observational Studies to Clinical Trials to Public Policy

- Start with science
- Add in cost-effectiveness
- Temper with explicit, previously agreed upon values