Value for Money for Medical Devices: A U.S. Perspective

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The Horizon of New Devices

- Diagnostics: Virtual colonoscopy
- Devices: Computerized knee
- Procedures: Breast MRI
FDA Definition of a Medical Device - Section 201(h) of the FD&C Act

• Diagnosis, cure, mitigation, treatment or prevention of disease or condition

• Affects the structure or function of the body

• Does not achieve intended use through chemical reaction

• Is not metabolized to achieve effect

“Six Reasons Why Devices Are Different”

1. Many are diagnostic (Dx)
2. Difficulty in doing RCTs
3. Efficacy depends on how it is used
4. Implementation may have wider economic implications
5. Lack of equivalent clinical evidence
6. Prices more likely to change over time

“Although the methods of economic evaluation can be equally applied to drugs and devices in general, we have identified several specific methodological issues that require more attention if reliable and informative evaluations of devices are to be conducted.”
In HTA, health-care decision-makers and payers require a common metric from the HTA process to appraise the evidence for health-care technologies across different medical conditions. The cost per quality of life in many settings has become that gold standard metric, regardless of whether a policy maker is evaluating a drug, medical device, or any other health-care technology.

Separation of Marketing Approval (Regulatory) from Coverage and Reimbursement (Payers)

- Economic rationale:
  - Regulatory licensure is about product “quality”, i.e., sufficient and adequate benefit-risk balance to be marketed.
  - Reimbursement is about valuation, and is “market determined.”

Both regulation and reimbursement have important implications for who bears the cost of collecting data and the return on investment—and hence incentives for R&D.
Differences in Regulatory Process for Drugs vs. Devices

• Both aim to generate evidence on benefit-risk balance. But the assessment of benefit-risk balance remains qualitative and subjective.

• For drugs, small differences in molecule in the same drug class can mean a big difference in clinical outcomes
  – How we regulate new chemical entities vs. generics vs. biosimilars reflects an appreciation of this.

• For devices, we have taken a more complicated and nuanced approach to appraising benefit-risk. (FDA defines 1700 generic types of devices in 16 medical specialties.)

  S. Ramsey, “Apples and Oranges”, 2009

US-FDA Regulation of Devices

Reflects expected risks:

• **Low Risk -- Class I** -- “General Controls”. Must register, but exempt from submitting evidence.

• **Medium Risk – Class II** – “General and Special Controls”—most require submission of Premarket notification (510K) to assess whether “substantially equivalent” to an existing device.

• **High Risk -- Class III** – “Special Control” is “pre-marketing authorization” (PMA) –requires evidence
  – Device found not “substantially equivalent” to existing device
  – Needs proof of “reasonable assurance” of safety and effectiveness
Problems with Device Regulations Are More Basic

• FDA rarely asks for evidence from randomized, controlled studies
  – 510(k): 98% of new devices approved this way
    • Controlled trials RARE
    • If all that has been established is “substantial equivalence” to a predicate device, what evidence exists that warrants use?
  – PMA: 2% of new device approvals
    • Controlled trials not required, occasionally done
    • Randomization and blinding RARE
    • Endpoints often focus on analytical validity (e.g., sensitivity, specificity)
• FDA regulations do not address the issue of operator skill as a factor influencing device effectiveness

Source: S. Ramsey, 2009

Device Safety in the Spotlight

• There most likely will be a greater focus on safety of devices.
  – There is a new guidance on benefit-risk assessment

• We need to remember that evidence is costly, and increased requirements raise costs, lower the rate of return, and could slow innovation.
  – We need to assess the value of this information.
  – We need to create a level playing field among technologies.
There is a broad consensus on how to do economic evaluation.

Traditional Hierarchy of Evidence – Where do economic evaluations fit?

**Best:**
- Meta-analysis of large randomized head-to-head trials
- Large, well-designed head-to-head randomized controlled clinical trials (RCT)
- Long-term studies, real clinical endpoints
- Well accepted intermediates
- Poorly accepted intermediates
- Smaller RCTs, or separate, placebo-controlled trials
- Well-designed observational studies, e.g., cohort studies, case-control studies
- Safety data without efficacy studies
- Case series, anecdotes

**Least:**
- Expert opinion, non-evidence-based expert panel reports, and other documents with no direct clinical evidence

Sir Michael Rawlins’ Harveian Oration (Lancet, 2008)

Experiment, observation, and mathematics, individually and collectively, have a crucial role in providing the evidential basis for modern therapeutics. Arguments about the relative importance of each are an unnecessary distraction. Hierarchies of evidence should be replaced by accepting—indeed embracing—a diversity of approaches.
Some Economic Considerations: Reimbursement and Financing Device R&D

- There are important differences between drugs and devices.
  - Drugs:
    - Stronger market exclusivity and patent protection
    - 8-12 years from idea to approval
    - Costs of R&D, trials, and fees can be over $800 million
  - Devices:
    - Easier to work around patents.
    - No “data exclusivity” period.
    - Reimbursement system in the U.S. is complex and variable for different types of devices.

Conclusions

- There are some important differences between the drugs and devices that will affect the costs of developing data and evidence, and hence will affect the health technology evaluation of devices in practice.
  - HTA authorities will need to be cognizant of these differences.
  - Regulatory and economic evaluations of new technologies will need to rely upon models—benefit-risk and cost-effectiveness models. Let’s make them more explicit.
- Policies affecting the costs of gathering evidence and the rewards for innovation need to consider “dynamic” incentives for innovation and not focus only on “static” incentives for cost minimization.
- Yes, a flexible approach is required for both what constitutes evidence and assessing value over time.
Thanks!

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