

Coverage with Evidence Development: Where Do We Go From Here?

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Mark B. McClellan
Director, Engelberg Center for Health Care Reform
Senior Fellow, Economic Studies
and Leonard D. Schaeffer Chair in Health Policy Studies
The Brookings Institution

Overview

- CED Re-Set: Much At Stake
- CED Experience: Emerging Evidence, and Emerging Challenges
- Opportunities and Risks for the Future
 - Better Infrastructure
 - Better Methods
 - Better Sustainability
- Biggest Challenge: How to assure that benefits substantially outweigh costs?

Establishment and initial use of coverage with evidence development (CED)

- Goal of CED: Providing beneficiaries with better or earlier access to a treatment that is reasonable and necessary, while developing better evidence to inform care decisions.
- Potential benefits of CED
 - More rapid access for Medicare beneficiaries to new interventions
 - Improved post-market evidence development, providing important new knowledge for care decisions
 - Clearer understanding for patients, providers, and payers regarding the risks and benefits of a new intervention
- Since 2006, 15 percent of national coverage determinations (NCDs) have incorporated CED.*

Some earlier experiences with CED

Intervention	Year CMS Initiated CED	Type of Prospective Study	Outcome After CED
Lung-Volume Reduction Surgery (LVRS)	1996	RCT (National Emphysema Treatment Trial)	Limited coverage
Implantable Cardioverter-Defibrillators (ICDs)	2005	Registry (ICD Registry)	Continued coverage under CED
Off-Label Use of Biologics Approved for Colorectal Cancer	2005	9 NCI-sponsored clinical trials	No official change, but many of the off-label indications in question are now listed in the compendia and covered
Positron Emission Tomography (PET) in Cancer	2005	Registry (National Oncologic PET Registry)	Continued CED for monitoring and restaging; granted coverage for diagnosis and staging
Stenting and Aggressive Medical Management for Preventing Recurring Stroke in Intracranial Stenosis (SAMMPRIS)	2006	RCT (SAMMPRIS)	Non-covered

Current challenges with CED

- Explicit, systematic approach for prioritizing the application of CED is lacking
 - Limits its ability to be used in a systematic and predictable way that could maximize benefits and minimize costs.
- CED generally has been applied on a case-by-case basis within the time frame of an NCD.
 - Parameters of the CED arrangement must often be made based on existing/planned studies.
 - May not provide evidence most needed by Medicare beneficiaries.
 - Example: Off-label use of biologics approved for colorectal cancer.
- CED has significant associated costs, beyond costs of coverage.
 - Infrastructure and resources for data collection and analysis.
 - Relative benefits vs. costs of CED as applied to date are unclear.
 - Impact on access to technology (can be positive or negative).
- No standing infrastructure for CED studies.
 - Translates into lengthier ramp-up time and higher cost per application.

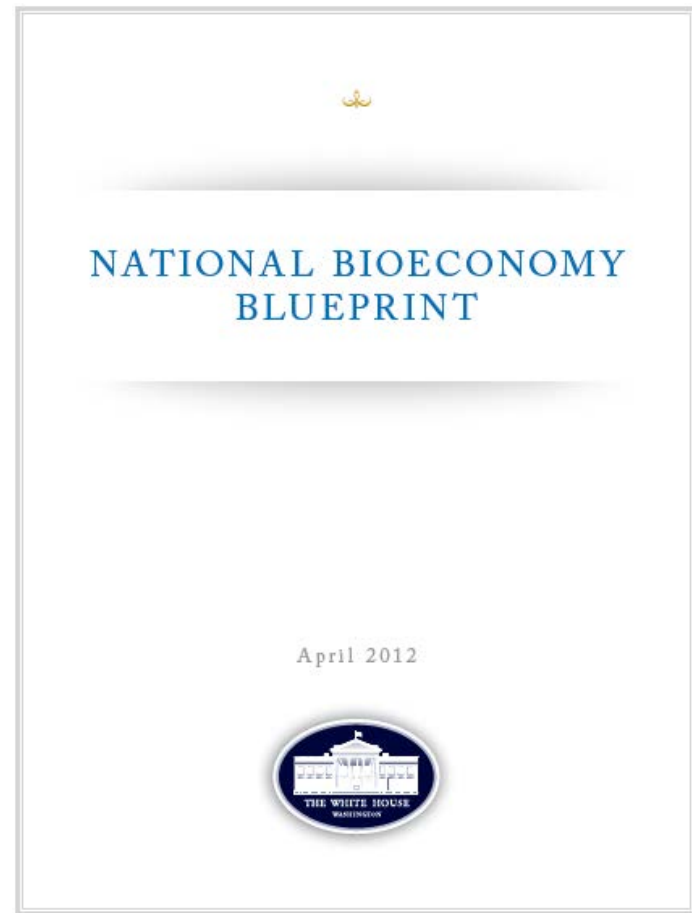
Time to Re-Evaluate CED

- What's worked well, what hasn't worked as well, and what are the opportunities for the future?
- In November 2011, CMS requested comments regarding its use of CED in Medicare.
 - Brookings held a roundtable discussion in December 2011 to consider the questions posed by CMS, bringing together a range of health care experts and perspectives.
 - Brookings and almost 50 other organizations and individuals, submitted public comments to CMS in January 2012.
- Today's meeting: Another step toward revised policies for CED.

Recent National Bioeconomy Blueprint Notes

Potential Importance of CED

- CMS is preparing to implement the next phase of CED with better defined parameters to support broader use.
- CED could be used to create “predictable incentives for innovation” while collecting evidence to ensure that new interventions are beneficial.
- Conversely, failing to address these challenges could inhibit innovation and better health.



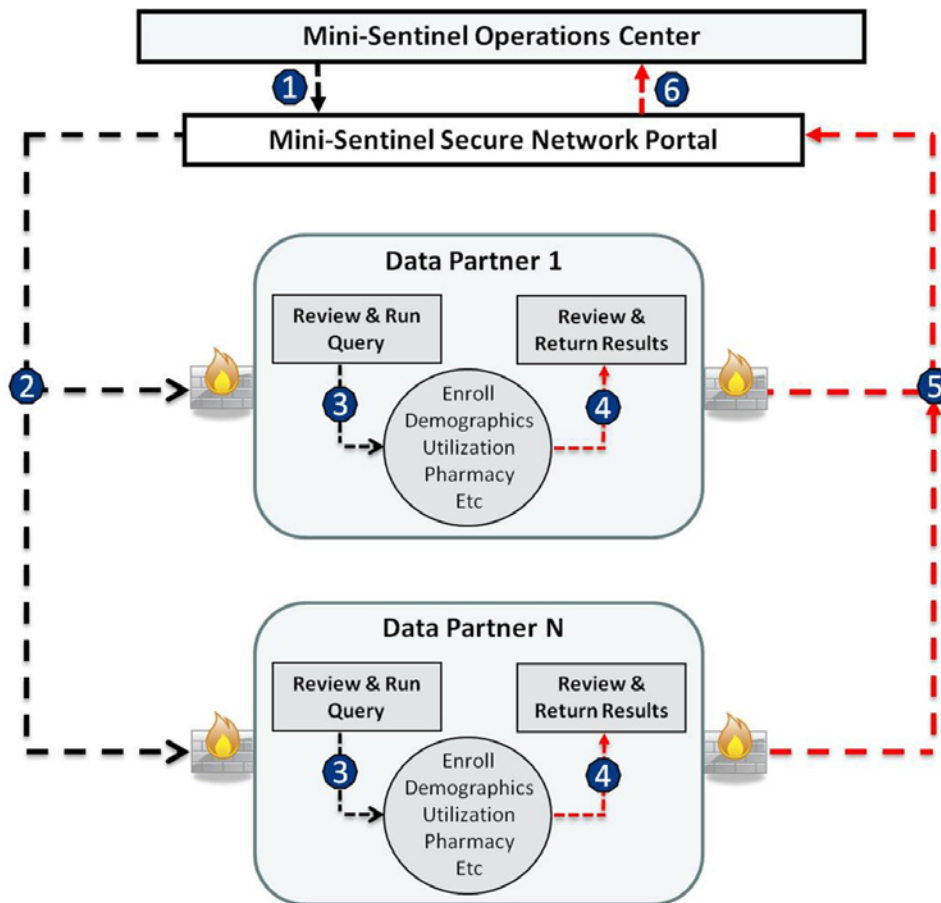
CED and a learning health care system

- Evidence needed on medical products at approval is not complete—with many opportunities to develop better evidence after approval.
 - Uncertainty regarding long-term outcomes
 - Possible differences in risks and benefits in older individuals or patients with multiple comorbidities, who are often underrepresented in clinical trials
 - Differences in effectiveness across practice settings or providers
- Growing opportunities to close post-market evidence gaps
 - Better development science (e.g., better predictors of patient benefit and risk, and validated markers of clinical outcome benefits, which can be tracked in post-market settings)
 - Registries and research networks using increasingly sophisticated electronic data and analytic methods to develop better evidence
- More support to develop better evidence
 - Public and private support for research and analysis
 - Outcomes-based payment policies (e.g., bundled payments, accountable care organizations)
 - CED

Better data collection infrastructure

- For CED to be time- and resource-efficient, need data collection infrastructure that is reusable.
 - Lower startup costs for future studies
 - To date, most CED data collection has been ad hoc and for one specific purpose
- To achieve this, CED will need to rely upon the following:
 - Existing and emerging data sources used in routine care (e.g., electronic health records, claims data)
 - Data/analysis infrastructure (e.g., distributed data networks, patient registries, practical trials)
- Public-private partnerships and networks will be needed.
 - Distributed networks: Individual data mainly stays at home in health care organizations that use it for patient care.
 - Contribute relevant summary information on particular research questions through voluntary participation and shared governance.
 - Example: FDA's Sentinel Initiative

Mini-Sentinel's “distributed data” methods



1. User creates and submits query (a computer program)
2. Data partners retrieve query
3. Data partners review and run query against their local data
4. Data partners review results
5. Data partners return results via secure network
6. Results are aggregated

CED could help support, and be reinforced by, other efforts to improve the post-market data infrastructure

- Existing post-market evidence development initiatives are working toward establishing a robust data infrastructure
- Examples include the following:
 - FDA's Sentinel Initiative
 - FDA's MDEpiNet and forthcoming implementation of unique device identifiers (UDIs) to enable more detailed tracking of outcomes among patients with medical devices
 - ASPE's Multi-Payer Claims Database
 - State Multi-Payer Claims Databases and related efforts
- Investments made by PCORI, both in terms of individual studies and strengthening the infrastructure for patient-centered outcomes research, are also likely to be beneficial for CED.

Improved study designs and methods for secondary use of data

- Some CED studies have involved randomized clinical trials, but many involve observational analyses in medical practice.
- Appropriate interpretation of CED data will require further investment in research and development of appropriate study designs and methods.
 - Examples of publicly-supported methods development include AHRQ and NIH activities and potentially PCORI.
 - Example of privately-supported methods research and development to support active medical product safety surveillance: The Observational Medical Outcomes Partnership (OMOP).

CED costs and financing

- CMS has no mechanism to support costs of conducting CED.
 - Analytic infrastructure, data collection, analysis
 - Usually funded by research agencies and/or industry
- Moving away from “one-off” CED mechanisms could reduce/spread infrastructure costs.
 - Opportunities to use or build on existing/emerging registries and data collection networks .
- A more systematic and routine approach to funding CED (primarily focused on the administrative costs of conducting a CED study) would be helpful.
- Partnerships with other agencies, industry, private payers, and others could foster funding opportunities that meet the needs of patients and providers.

Many other organizations have a vested interest in better post-market evidence

- U.S. Food and Drug Administration
 - Evidence supports accelerated approvals (which may soon be expanded) and to promote post-market safety surveillance.
- Agency for Health Care Research and Quality
 - Mission includes supporting research on medical technologies and quality of care.
- Patient-Centered Outcomes Research Institute
 - Mission to support better evidence on for individualized patient decision-making.
- Many others: VA, DoD, private payers, health systems, etc.

Some Considerations for Prioritizing and Applying CED

- Proactive and predictable priorities and implementation—CED implementation is not a surprise and is efficient.
 - Best practices (standard approaches?) for data collection infrastructure and methods
 - Sustainable, appropriate mechanisms for infrastructure funding
 - Consistent methods to assure that the benefits of CED substantially outweigh its costs
 - Transparent methods to focus CED on the most important evidence questions with the best opportunity to close knowledge gaps
- Criteria for applying CED may include the following:
 - Body of evidence supporting an intervention, beyond FDA-approved indications
 - Key questions that may remain about the treatment in Medicare beneficiaries
 - Feasibility and value of developing additional evidence
 - Key details of disease area, treatment types, and other factors that may require adapting or reconsidering the criteria
- Ongoing review of priorities and criteria applied to existing CED activities will also help to inform decisions regarding whether to continue, cease, or modify it.

Next steps toward improving CED

- CED can help provide access to innovative treatments in Medicare population while generating further evidence to promote more effective use—but more evidence comes at a cost.
- Complemented by payment and benefit reforms focusing on better results, and by more sophisticated research networks, CED could provide important momentum for better, more innovative health care.
- But CED also has costs—so increasingly important to get CED right, especially if relatively routine part of coverage.
- In order to capitalize on CED's potential, CMS should work toward increasing the benefits and reducing the cost of CED application a more effective, efficient, and predictable CED system:
 - Establishing criteria for invoking CED that ensures benefits outweigh costs
 - Improving the data collection infrastructure
 - Promoting innovative study designs and methods
 - Assessing the costs and benefits associated with CED studies