The Pharma, Biotech and Device Colloquium

Linking Medicare Coverage to Research Participation

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Big Picture for Medicare

- Population, economic, technological, other trends accelerating Medicare spending
  - Rx coverage puts great new pressure on Medicare spending
- All major payers continue to have quality concerns
  - Large geographic variations in practice
  - Unacceptable rates of adverse events, medical error
  - Little relationship between spending and quality
  - Difficulty in limiting coverage once granted
- Some new technologies pose considerable financial risk
- Policy-makers, public, increasingly aware of need to monitor, act on post-marketing experience
- Multiple reasons for CMS to centralize policy-making
  - Business pushing Medicare to exert buying leverage
- Health IT capacity increasing, including for data collection of technology performance in practice

McClellan Vision (1)

- CMS to assume a leadership position in public health
- Fostering continued innovation for greater patient benefit depends on payment, access, and learning from experience
- Premarket RCTs, other methods, may be too limited to represent outcomes in real practice
- Promising technologies may have insufficient, premature evidence for informing practice
- Health system (including Medicare) seeks better outcomes at lowest possible costs
- Health IT is poised to enable great advances in data management, utility for research

Therefore …

McClellan Vision (2)

Upon FDA approval …

- **Provide prompt coverage**
  - **Conditional on further data collection for selected technologies**
  - **Enable use in real-world settings**
- **Conduct post-market data collection using:**
  - **Registries, practical clinical trials, other methods as appropriate**
- **Capitalize on health IT to extent possible**
  - **Widespread, rapid data collection and analysis**
- **Feed back findings to clinicians, patients, payers, technology companies**

Draft Guidance for the Public, Industry, and CMS Staff

Factors CMS Considers in Making a Determination of Coverage with Evidence Development

Document Issued on: April 7, 2005

I. Purpose of this Guidance Document

The purpose of this guidance document is to describe factors CMS may consider in a decision to extend national coverage for certain items and services with coverage linked to a requirement for prospective data collection. This approach is referred to as coverage with evidence development (CED). The primary purpose of obtaining additional evidence through CED is for the agency's use in making payment determinations, i.e., that a treatment is reasonable and necessary …. (15 pages)

Draft Guidance Acknowledges Important Aspects About Evidence Collection

For example (to paraphrase) …

- **Objectives of this process include enhancing access to technologies that improve health of beneficiaries**
- **Coverage with evidence development (CED) should only be used to address specific evidence questions**
- **CED should not duplicate existing data collection efforts of FDA or other public or private sector entities**
- **Any CED must be worth its cost**
- **CED should minimize financial and other resource burdens**
- **CMS should maintain the local coverage process**

What Kinds of Questions Might CED Be Intended to Answer? Those Pertaining to:

- Fill-in gaps on safety, side effects
- Risks and benefits not described in literature
- Risks and benefits in specific patient subgroups
- Long-term risks and benefits, QoL, utilization, costs, other real-world outcomes
- Risks and benefits of procedures not subject to FDA approval
- Effectiveness of interventions for rare diseases
- Available evidence not generalizable to providers/facilities or Medicare population
- Comparative effectiveness of new vs. standard interventions
- Clinical significance, given statistical significance (p9-10)

Fundamental Concerns with Draft Guidance (1)

Includes fundamental assumptions and other aspects that should be addressed, e.g.:

- **Definition of reasonable and necessary**
- **Two general circumstances for applying CED**
- **CMS (vs. FDA) responsibility for monitoring safety**
- **Role of utilization and costs in coverage**
- **Matching study design to the evidence question**

Fundamental Concerns with Draft Guidance (2)

Includes fundamental assumptions and other aspects that should be addressed, e.g.:

- **Burden on patients: e.g. cost, access, autonomy**
- **Burden on providers**
- **Legal authority to impose conditions**
- **Privacy concerns**
- **Trade secret and competitive information concerns**
- **Coordination with claims data**

Let's look at some of these …

CMS Authority to Make Coverage Decisions

Social Security Act, Section 1862(a)(1)(A):

" … no payment may be made .... For expenses incurred for items or services … [which] are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member."

The provision gives the HHS Secretary, acting through CMS, the authority to determine the coverage of services under Medicare.

Defining Reasonable and Necessary in CED

"The primary purpose of obtaining additional evidence through CED is for the agency's use in making payment determinations, i.e., that a treatment is reasonable and necessary." (p2)

"In general, the core consideration in determining when an item or service is 'reasonable and necessary' is the *quality of the evidence available to assess whether it improves net health outcomes."* (p3)

"In some cases, CMS will determine that an item or service is only reasonable and necessary when specific data collections accompany the provision of a service." (p6)

Is lack of evidence of effectiveness equal to evidence of a lack of effectiveness?

Two General Circumstances for Applying CED

Draft guidance describes "two general circumstances under which clinical care provided may only be considered reasonable and necessary in the context of protocol-driven data collection" **as follows ...**

First General Circumstance for Applying CED

"**a particular medical intervention may have been demonstrated to improve health outcomes in a broad population of patients** … but the evidence would only be adequate, and the service therefore reasonable and necessary for the individual patient, when specific data is collected and reviewed by the provider at the time that the service is delivered. The additional evidence, in conjunction with published scientific evidence and other information available to the physician and patient, would be used to support appropriate treatment decisions for such patients." (p6)

Second General Circumstances for Applying CED

"**a particular medical intervention has yet to conclusively demonstrate an improvement in health outcomes,** but existing information clearly suggests the intervention may provide an important benefit. In this case, CMS may decide that the adequacy of the evidence demonstrating improved health outcomes can only be assured if additional information is collected, reviewed and submitted at the time of service." (p7)

CMS (vs. FDA) Responsibility for Safety (1)

Draft guidance refers to data collection on safety under CED in ways that could be FDA's responsibility e.g.:

- "Conversely, support for post-coverage evidence development to achieve a reasonable and necessary determination may help address important questions of safety and effectiveness that otherwise would be very difficult to address in the premarket setting or in the postmarket setting in the absence of CMS support." (p5)

CMS (vs. FDA) Responsibility for Safety (2)

Under factors (list of circumstances) considered in applying CED, the draft guidance includes:

- "The item or service is likely to provide benefit, but there are substantial safety concerns or potential side effects that are inadequately described in the available clinical literature." (p9)
- "When the current evidence is not generalizable to providers/facilities or the Medicare population has not been included in the available clinical studies, new evidence development may help evaluate the safety and benefit of requested items and services for our beneficiaries." (p10)

Role of Utilization and Costs in Coverage (1)

Absence of data on utilization and costs could prompt CED, and that utilization and costs are among the outcomes that would be studied under CED:

"Factors Considered in Applying CED … include:

- "Assessment of important outcomes has not been evaluated in the available clinical studies. These outcomes may include, but are not restricted to, long-term risks and benefits, quality of life, utilization, costs, and other real-world outcomes." (p9)
- "This evidence will also assist doctors and patients in better understanding the risks, benefits and costs of alternative diagnostic and treatment options." (p4)

Role of Utilization and Costs in Coverage (2)

Consider:

- **Until now, CMS has cited utilization and costs (e.g., in the form of anticipated aggregate cost impact to Medicare) among factors that might increase the priority for undertaking an NCD. *But CMS has not considered these explicitly in an NCD itself.***
- **Is interest in utilization and/or cost data alone sufficient reason to apply CED?**
- **It's one thing for utilization and/or cost data to be among the criteria for setting priorities for NCDs.**
- **It's another to weigh utilization and/or cost in the context of conducting an NCD for any particular technology.**

CMS Draft CED Guidance – Study Designs

"The following is a list of study designs that may be used to develop an evidence base:"

- **Databases –** "require entry of baseline data … used to monitor patient safety and benefit"
- **Longitudinal or cohort studies –** "patients are followed over time after baseline clinical information is collected."
- **Prospective comparative studies (also called 'practical clinical trials') –** "require a formal comparison group, can include randomization"
- **Randomized clinical trials**

How will these be selected?

Choosing Methods *in the Context of Coverage*

What's different about that?

- **Most interventions (drugs, devices, biotech) will have been approved by FDA – they're on the market**
- **FDA already requires post market studies if needed to address safety issues**
- **Coverage policy ≠ clinical decision**
- **Source, ability to pay for data collection**
- **Data collection burden imposed in practice, not investigational settings**

Burden on Patients and Providers

- Cost of Additional follow up visits and testing will impose copay burdens on patients
- Need to participate in research aspects may impose additional burdens on patients: e.g. time, travel, etc.
- Physicians and institutions will have new burdens of data collection, reporting and review
- CMS has indicated that there is no intention to make additional payment for some of these burdens (inpatient payment rule re: ICDs)
- Who should pay for database maintenance?
- Fraud and abuse concerns need to be addressed

Privacy and other Patient Concerns

Draft guidance states:

"Patient confidentiality and protection − All necessary measures should be taken to ensure patient privacy. When appropriate, there should be institutional review and informed consent." (p14)

Consider:

- **Do the data collection efforts described by CMS amount to "medical research?"**
- **Informed consent is required for most types of medical research, including that organized by HHS.**
- **Does CED condition access to care on a beneficiary's willingness to be part of medical research?**
- **Will privacy protections limit utility of the data?**

Competitive Issues

- Trade secret information may be involved
- Coverage policies are not limited to single products
  - will mix data on regulated products and non-regulated products
  - FDA approved drugs vs. compounded products
  - reprocessed devices vs. OEM products
  - FDA cleared IVDs vs. “home brew” tests
- Clinical registries mixing competitive products can involve:
  - sensitive marketing information
  - data subject to abuse in marketing
  - public disclosure of proprietary data

Integration with Claims Data

- CMS has not had great success in extracting reliable information to guide coverage from claims data
- HIPPA restraint on use of claims data
- Delay in making availability of claims data
- Claims information has very poor information content due to:
  - erratic and confusing HCPCS coding policies
  - poor quality of coding accuracy
  - lack of consistent diagnostic information on claims

How should we view coverage with evidence development by CMS?

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