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"21st Century Cures: Barriers to Ongoing Communication and Evidence Development."

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Chairman Pitts, Ranking Member Pallone, and Members of the Subcommittee, my name is Louis Jacques, and I am testifying as an individual with experience on the topic of this hearing. From October 2009 through February 2014, I was the Director of the Coverage and Analysis Group at the Centers for Medicare & Medicaid Services. I was a division director in that group from June 2004 until my appointment as the group director. During my tenure there we implemented Coverage with Evidence Development (CED) and the FDA – CMS Parallel Review pilot initiative. We also revised CMS regulations pertaining to Medicare coverage in FDA approved Investigational Device Exemption (IDE) clinical trials, and executed a memorandum of understanding between FDA and CMS. I am currently a Senior Vice President at ADVI, where I am also Chief Clinical Officer and a partner. ADVI has offices in Austin, San Francisco and Washington DC. ADVI's mission is to help healthcare companies and organizations develop and articulate evidence that is informative and persuasive for patients, practitioners and public and private healthcare payers.

Background

While CMS has consistently expressed a desire to support evidence based medical technology innovation, this goal would be better accomplished if CMS had clearer authority and greater administrative agility in Medicare coverage and payment for innovative technologies that are in the adolescent phase of their overall product life cycle. This would allow CMS to establish and implement a clearer and more predictable paradigm for coverage of certain technologies that may receive FDA approval or clearance despite the lack of sufficient evidence relevant to the Medicare beneficiary population, in particular the elderly who have multiple comorbid medical conditions.

The historic practices of many medical technology developers reflect insufficient knowledge and attentiveness to clinical questions that are relevant to patient care and health insurance. While this issue is not unique to a particular product category, it can be a particularly vexing challenge in the medical device sphere where the "garage based inventor with a good idea" ethos coexists in the same space with large comparatively more sophisticated multinational firms. This leads to interactions with insurers that can be mutually frustrating, more so if the manufacturer claims enhanced clinical or economic value for a device cleared under 510(k) as substantially equivalent

to a predicate device. The ultimate accuracy of these claims could be addressed with a clinical study but a small company may have limited funds to support additional development or research by the end of its FDA review. I believe that the opportunity for earlier engagement with representative public and private insurers would help these companies make better informed choices at earlier stages in product development, before they commit more resources to a strategy that would predictably fall short.

Medicare is not the only relevant insurer, but the difference between FDA's regulatory standard (safe and effective) and Medicare's overarching standard (reasonable and necessary) is a frequent discussion topic. While this difference is appropriate since the agencies have distinctly different mandates, both agencies share broader national goals to improve public health and protect beneficiary access to those products and services that demonstrate genuine benefit. As a practical matter, FDA approval for drugs and biologics, devices and diagnostic tests puts the product on the store shelf, but prudent purchasers should not be expected to reflexively buy every stocked item without regard to their own needs and priorities.

CMS' experience over the past decade is illustrative of the factors that may constrain the wide adoption of certain innovative technologies. Several of these factors relevant to Medicare coverage and payment are illustrated below. While there is significant alignment among payers on the need for pertinent clinical evidence, commercial or other governmental health insurers may have different flexibility on other factors.

1. There are innovative products and services that do not clearly fall within the statutory scope of the Medicare program. Early engagement with CMS could help companies anticipate this issue and develop better strategies.

The Social Security Act (the Act) establishes the scope of the Medicare benefits under parts A and B. These 50-some "benefit categories" include items and services such as inpatient hospitalization, drugs administered incident to a physician service, durable medical equipment (DME), physician care, etc.

Medicare pays for external drug pumps under the DME benefit. An innovative external drug pump may have characteristics that place it outside the statutory definition of DME.

Medicare does not cover "vaccines" except in certain circumstances such as influenza and pneumococcal immunizations. Thus, certain cancer immunotherapies may unnecessarily pose questions of their inclusion in the Medicare benefit, particularly if they are described as "vaccines" in the press.

A smartphone based technology could be excluded because smartphones are not medical devices. This limits Medicare's ability to consider coverage and payment for applications (apps) that could potentially take the place of certain physician or provider services that currently entail physician supervision.

2. The available evidence at the time of initial marketing does not clearly establish the clinical value of a new technology in the insurer's population of interest. CMS has tried to address this

issue with its Coverage with Evidence Development (CED) initiative, but there have been impediments to the more agile and efficient implementation of CED.

Medical device trials are generally much smaller than drug trials, and often exclude populations of interest from enrollment. Commonly, older patients with multiple comorbid conditions, i.e. typical Medicare beneficiaries, are not well represented in clinical trials done for FDA approval. Under the 510(k) paradigm some devices may be cleared for marketing with no relevant clinical trial evidence at all.

Lumbar artificial disc technology is a good example. The pivotal clinical trials excluded subjects over age 60, and persons with osteoporosis. Given the advanced aged and predominance of women among Medicare beneficiaries, the evidence base could not be reasonably applied to the core Medicare population.

Clinical trials often employ outcomes that poorly identify the ultimate impact on the patient. These may be only short term outcomes for devices that are intended to last for years, nonclinical performance targets or potentially misleading surrogate laboratory outcomes that poorly reflect the patient experience of illness. Other significant limitations include small sample sizes, absence of randomization or adequate controls, and additional sources of bias that limit the persuasiveness of the reported results.

3. Historic coding paradigms can be uninformative to the extent that the insurer cannot identify the specific item or service for which it is paying. This "blind buying" creates reluctance among insurers, and prevents the establishment of brand value for higher performing technologies.

Molecular diagnostic tests are the clearest example of this practice, in which claims for payment historically comprised "stacks" of nonspecific technical procedures performed in the processing of the test sample. The recent Protecting Access to Medicare Act of 2014 (PAMA) legislation addresses this issue with a requirement for granular, product specific coding and a new payment calculation for "advanced diagnostic tests" that meet certain criteria. PAMA creates an incentive to invest in higher performing technologies that can be favorably covered and paid based on evidence of enhanced value.

Despite the newness of these provisions, I am aware of some interest in the venture capital community that a similar paradigm could be applied to other innovative technologies that meet consistent and transparent prespecified requirements. This could address a common complaint that new technologies are billed with nonspecific or temporary codes that some stakeholders believe dissuade adoption by physicians and hospitals.

Opportunities

A. There is significant stakeholder interest in expanding the CMS initiatives that support medical technology innovation but CMS has limited capacity to respond.

External stakeholders have told me they want more opportunities for Coverage with Evidence Development (CED) and FDA-CMS Parallel Review, as well as interaction with private payers under a neutral umbrella.

A recent example of CED is the 2012 decision to cover transcatheter aortic valve replacement (TAVR) in the context of national registries and clinical trials. CMS, with a joint formal request from the American College of Cardiology and the Society of Thoracic Surgeons, established predictable Medicare coverage for current and future FDA approved indications of TAVR; as well as coverage in future clinical trials for unlabeled indications. The resulting data after one year prompted FDA to expand the label for TAVR without the need for an additional clinical trial.

Unfortunately, CMS' ability to engage is limited by historic interpretations of its authorities, and by severely reduced resources in the Coverage & Analysis Group (CAG) that oversees these initiatives. Under current statute, CMS only initiates CED under AHRQ's authority: 1862(a)(1)(E) of the Act, which references AHRQ's authority to conduct Medicare research under section 1142. CMS only implements CED through the National Coverage Determination (NCD) process. Due largely to staffing cuts, the annual number of NCDs published has dropped from approximately 12-13 (FY 2007 and 2008) to 5 (FY 2012) and 6 (FY2013). Competing agency priorities, e.g. expanded coverage of prevention, further limit the application of NCD assets to innovative technologies and CED.

Similarly, with limited resources CMS cannot match FDA's bandwidth on potential parallel review candidates. Despite expressed interest from device manufacturers, the parallel review pilot has been limited to only two participants. CMS also does not have a counterpart to FDA's Entrepreneurs in Residence (EIR) program to bring in-house experience from private payers, outside innovative thinkers, etc.

B. *Responding to stakeholder input, CMS recently revised its regulations regarding coverage of items and services in FDA approved Category B IDE (investigational device exemption) clinical trials.*

While CMS approval is not required to conduct IDE trials, those manufacturers who choose to bill Medicare for trial costs must request coverage. Manufacturers had noted inconsistencies and inefficiencies in the historic paradigm that required separate coverage requests and approvals from each local Medicare contractor. CMS in the CY2014 Physician Fee Schedule regulation established basic criteria and a centralized application and review process.

This new process serves three complementary goals. First, it provides important financial support for approved research studies. Second, the sponsor can obtain CMS feedback on the design of the trial, especially on the inclusion of subjects who are representative of the targeted Medicare beneficiary population and the relevance of the proposed outcomes to meaningful changes in patients' experience of illness. Third, CMS can clarify any assumptions that the manufacturer may have about benefit category, coding, payment bundles etc. that may impact the financial projections that inform investors.

The successful implementation of this initiative (effective date January 1, 2015) depends on CAG having adequate resources (staff and budget) to quickly review IDE protocols and publish a real time list of approved trials.

C. *The innovative CMS MolDX pilot established granular coding, coverage, and payment determination for molecular diagnostic (genomic or proteomic) tests. Recent PAMA legislation has codified in statute the core principles of the MolDX pilot, while also requiring the use of the Local Coverage Determination (LCD).*

There are well over 1000 MolDX tests purported for clinical use. For many tests there are multiple versions developed by different laboratories and based on different underlying technologies (platforms.) The published medical literature and public testimony inform us that the performance of these tests, even those marketed for the same purpose, varies in meaningful ways. It is reasonable to expect that insurers would and should recognize the higher value tests with more favorable coverage and payment. We are aware of estimates that over 500 new MolDX tests are developed every year.

We also recognize that many of these tests (home brews – laboratory developed tests) have been marketed without review by FDA. In general, the available evidence of clinical utility (actual impact on the patient if treatment decisions are based on the test result) is uneven, especially for tests that claim to predict distant outcomes. The ultimate clinical value of these tests will be determined with prospective evidence from real world use. Some of these important questions could be answered with Coverage with Evidence Development (CED), but there is no clear pathway for local CED via the LCD process. Thus a Medicare contractor acting appropriately on currently available evidence might noncover a MolDX test that could, with a more mature evidence base developed over time, have proven to be ultimately beneficial.

The statutory definition of the LCD in 1869(f)(2)(B) of the Act describes it as a coverage determination under 1862(a)(1)(A) of the Act. Thus the LCD vehicle is not currently available for CED, which is currently articulated under 1862(a)(1)(E) of the Act. Interestingly the definition of a National Coverage Determination in 1862(l)(6)(A) is broader; "a determination by the Secretary with respect to whether or not a particular item or service is covered nationally under this title."

In light of the PAMA provisions requiring the use of the LCD for MolDX test coverage, a clear path to local CED could streamline the process for diagnostic test coverage with significant benefits to innovators and CMS alike.

Conclusions and Recommendations

I. CMS needs unambiguous authority to review clinical trials when claims related to these trials will be submitted for Medicare payment. The current vehicles for coverage in clinical trials are unnecessarily siloed, preventing the publication of an integrated comprehensive policy to deal with 1) costs for routine clinical care in trials (currently under a White House Executive Order from the end of the Clinton administration); and 2) costs of the investigational care itself, including related clinical care (currently under CED or the IDE regulation.) The status quo does not clearly establish a prospective route for coverage and payment of investigational care in other settings, i.e. clinical trials beyond CED and FDA Category B IDEs.

There are many potential approaches to this issue. Some are noted below, but this is not an exhaustive list.

- The research authority in 1862(a)(1)(E) could be extended to CMS with or without preserving AHRQ authorities in parallel.
- 1862(a)(1)(A) could be amended to explicitly include items and services furnished in CMS approved clinical trials.
- A distinct new section could establish a singular broad CMS authority related to Medicare coverage and payment in approved clinical trials.

II. The definition of a Local Coverage Determination could be revised to permit LCDs to be used as determined by the Secretary within the scope of Title XVIII. This would align LCD authority with the actual scope of local contractor claims processing responsibility. With more flexible LCD authority, contractors could write LCDs to establish CED as an alternative to noncoverage for various technologies.

III. Some stakeholders have expressed interest in new payment paradigms for early stage devices with immature evidence bases. As an alternative to noncoverage, such devices could be covered but paid at a lower rate initially, for a predetermined period of time, while evidence is being collected. Payment rate increases and possibly "premium" payment levels could be attainable if the additional evidence demonstrates prespecified enhanced patient-centered value. This would align the interests of the developer, the insurers, patients and healthcare professionals to provide earlier access to new technologies while also answering important clinical questions quickly and efficiently. Such a "rapid learning" paradigm would identify both truly beneficial technologies as well as those that ultimately prove to be disappointing in subsequent use.

After a predetermined period of time the payment amount could gradually fall to a prespecified percentage of the premium price. This recognizes that a technology does not remain innovative forever, and returns resources to the payment system to support subsequent innovative technologies.

IV. The implementation of these initiatives requires stable funding and reasonable alignment of resources with the workload. The Coverage and Analysis Group has been decimated by successive cuts in staff and budget. Current staffing is approximately half of 2007 levels. Approximately one-quarter of the staff was lost to retirement, reassignment and resignations during the sequester and could not be replaced. The frequent inability to recruit external candidates has stymied a more strategic needs-based approach to staffing. Alternative funding could be considered, possibly from the Medicare Trust Fund or other sources.

Thank you for the opportunity to share my thoughts and I would be happy to answer any questions.