CHAPTER 10

Medicare coverage policy and use of low-value care

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Chapter summary

Some researchers contend that a substantial share of Medicare dollars is not spent wisely. Many new services disseminate quickly into routine medical care in fee-for-service (FFS) Medicare with little or no basis for knowing whether or to what extent they outperform existing treatments. In addition, there is substantial use of low-value care—the provision of a service that has little or no clinical benefit or care in which the risk of harm from the service outweighs its potential benefit.

In this chapter, we review the coverage processes used in FFS Medicare and by Medicare Advantage (MA) plans and Part D sponsors. FFS Medicare covers many items and services without the need for an explicit coverage policy. When an explicit coverage policy is required, some services do not show that they are better than existing covered services. Coverage policies are often based on little evidence and usually do not include an explicit consideration of a service's cost-effectiveness or value relative to existing treatment options.

MA plans are generally required to provide the same set of benefits that are available to beneficiaries under FFS Medicare. However, MA plans are permitted to use tools that are not widely used in FFS Medicare, such as requiring providers to obtain prior authorization to have a service covered and controlling utilization through the use of cost sharing. Part D plan sponsors

In this chapter

are responsible for creating and managing formularies, which are lists of drugs their plans cover. By contrast, Medicare FFS lacks the flexibility to use formularies for drugs that Part B covers.

We also review the literature on low-value care, which reveals that such care is prevalent across FFS Medicare, Medicaid, and commercial insurance plans. Evidence suggests that the amount of low-value care in a geographic area is more a function of local practice patterns than payer type.

We analyzed selected low-value services in FFS Medicare using 31 evidence-based measures. In 2014, there were between 34 and 72 instances of low-value care per 100 beneficiaries, depending on whether we used a narrow or broad version of each measure. Between 23 percent and 37 percent of beneficiaries received at least one low-value service, and annual Medicare spending for these services ranged from $2.4 billion to $6.5 billion. The spending estimates are conservative because they do not reflect the downstream cost of low-value services (e.g., follow-up tests and procedures).

We examined three case studies of care of potentially low value in FFS Medicare: the trend in starting dialysis earlier in the course of chronic kidney disease, proton beam therapy, and H.P. Acthar Gel® (Acthar, a drug covered under Part D). The timing of starting dialysis for end-stage renal disease is a matter of clinical judgment, guided by values of residual kidney function and symptoms and comorbidities present in affected patients. Between 1996 and 2010, there was a trend toward initiating dialysis earlier in the course of chronic kidney disease. Since 2011, this trend has moderated because of the availability of comparative clinical evidence showing that the early initiation of dialysis is not associated with improved outcomes. We estimate that dialysis spending in 2016 for FFS Medicare patients who initiated treatment with higher levels of kidney function (i.e., earlier in the course of chronic kidney disease) ranged from $500 million to $1.4 billion.

Proton beam therapy—a type of external beam radiation therapy used primarily for cancer treatment—was initially used for pediatric cancers and rare adult cancers. However, its use has expanded in recent years to include more common conditions, such as prostate and lung cancer, despite a lack of evidence that it offers a clinical advantage over alternative treatments for these types of cancer. Medicare's payment rates are substantially higher for proton beam therapy than other types of radiation therapy. From 2010 to 2016, spending and volume for proton beam therapy in FFS Medicare grew rapidly, driven by a sharp increase in the number of proton beam centers and Medicare's relatively broad coverage of this treatment. During that

period, spending rose from $47 million to $115 million. Prostate cancer was by far the most common condition treated by proton beam therapy in Medicare.

Acthar is an older, Part D–covered drug that has experienced rapid growth in price and Medicare spending over the last several years, despite weak evidence that it is effective for adult indications. Between 2001 and 2017, the average price per vial increased from $748 to $38,000. Between 2011 and 2015, Medicare spending for Acthar increased from $49 million to $504 million. Fewer than 2,000 clinicians prescribed Acthar to beneficiaries in 2015, and 71 percent of them received at least one nonresearch payment from the manufacturer of Acthar related to the drug. These financial relationships raise questions about conflicts of interest among prescribers of Acthar.

Finally, we discuss six tools that Medicare could consider using to address the use of low-value care.

- Expanding prior authorization, which requires providers to obtain approval from a plan or payer before delivering a product or service, could help reduce the use of low-value care. Although CMS has tested this approach to reduce unnecessary use of power mobility devices, nonemergent ambulance transports, and hyperbaric oxygen therapy, it has not been widely adopted by Medicare.
- Implementing clinician decision support and provider education could decrease low-value care, and studies show that these tools have reduced inappropriate prescribing of antibiotics.
- Increasing cost sharing for low-value services has the potential to reduce their use. Although Medicare does not currently do so, other health plans and payers have raised cost sharing for targeted low-value services, and an evaluation of one program found that it reduced the use of these services.
- Establishing new payment models that hold providers accountable for the cost and quality of care—such as accountable care organizations (ACOs)—creates incentives for organizations to reduce low-value services. Preliminary evidence indicates that Pioneer ACOs (which shared in both savings and losses) were able to reduce low-value care.
- Revisiting coverage determinations on an ongoing basis has the potential to both decrease use of low-value services and result in the development of more rigorous clinical evidence. However, Medicare infrequently revisits its national coverage determinations. Moreover, nearly all of the reconsiderations that

Medicare opened over the past five years have been at the request of external parties (e.g., manufacturers, physicians, and medical associations) and have resulted in expanding coverage for the service under consideration.

- Linking information about the comparative clinical effectiveness and cost-effectiveness of health care services to FFS coverage and payment policies has the potential to improve the value of Medicare spending. Medicare's coverage process considers, but does not require, comparative clinical effectiveness evidence, and the program's rate-setting processes generally do not consider such evidence. For most items and services, Medicare lacks statutory authority to consider evidence on cost-effectiveness in either the coverage or the payment process. ■

TABLE 10-1 **Overview of Medicare's coverage processes for Part A and Part B services**

	Type of coverage policy	Who develops policy	Where policy applies
Existing billing code or bundled payment system	Explicit policy may not be necessary if service is in existing code or bundle	CMS	Nationwide (binding on all contractors)
NCD	Explicit policy	CMS	Nationwide (binding on all contractors)
Program manuals and memos	Explicit policy	CMS	Nationwide (binding on all contractors)
LCD	Explicit policy that can apply to a service that existing NCDs do not address or policy that further defines an NCD	Medicare's contractors (medical directors)	Contractor's regional jurisdiction; policy for a given service can vary across regions

Note: NCD (national coverage determination), LCD (local coverage determination).

Source: MedPAC analysis of the statute and CMS program manuals and guidance.

Primer on Medicare coverage policy

Medicare provides coverage for a broad range of health care services under its Part A, Part B, Part C, and Part D programs, as enumerated in Title XVIII of the Social Security Act. For Part A and Part B services furnished in fee-for-service (FFS) Medicare, the statute requires that the program cover items and services that are included in a Medicare benefit category, are not statutorily excluded, and are "reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member." Although the statute sets forth the broad categories of benefits covered by Medicare, neither the statute nor the regulations provide an all-inclusive list of the specific items and services that are reasonable and necessary.

Medicare coverage decisions for most Part A and Part B services are made at both the national level (by CMS) and local level (by Medicare's administrative contractors). However, many services do not require an explicit coverage determination, such as services paid through CMS's prospective payment mechanisms. Medicare is not required to consider comparative clinical effectiveness evidence in the coverage process, and the program lacks explicit statutory authority to consider a service's cost-effectiveness or value when making coverage decisions.

Under Part C, Medicare Advantage (MA) plans are required to provide the same set of benefits that are available under FFS Medicare, except that FFS Medicare covers hospice care and covers certain services associated with clinical trials under Medicare's Clinical Trials Policy for MA enrollees. However, MA plans are permitted to use medical management tools not available in FFS Medicare, such as requiring providers to seek prior authorization to have a service covered. Plans also have leeway in controlling utilization through beneficiary cost sharing.

Part D plan sponsors are responsible for creating and managing formularies, which are lists of drugs their plans cover. Part D law and regulations place some constraints on which drugs plan sponsors may cover and how those sponsors operate their formularies. By contrast, Medicare FFS lacks the flexibility to use formularies for drugs that Part B covers.

Medicare coverage for Part A and Part B items and services

As summarized in Table 10-1, there are several ways for services to be covered under FFS Medicare. Medicare coverage occurs for many Part A and Part B items and

services without the need for an explicit coverage policy. If a service falls under a Medicare benefit category and can be reimbursed on the basis of an existing billing code or a bundled payment system (e.g., inpatient prospective payment system), Medicare may cover it without an explicit coverage policy.

When an explicit coverage determination is required, CMS and Medicare administrative contractors (MACs) develop policies at the national and regional level, respectively, to determine whether a service meets one of the covered benefit categories and is reasonable and necessary, in which case, it is covered. MACs develop the majority of explicit coverage policies. These policies, referred to as local coverage determinations (LCDs), determine coverage of specific medical services that apply only in the contractor's regional jurisdiction. LCDs must be consistent with the statute, regulations, and national policies for coverage, payment, and coding.

In addition to the LCD process, CMS develops coverage determinations for specific medical services that apply nationwide through the national coverage determination (NCD) process. A small subset of NCDs links a service's national coverage to participation in an approved clinical study or to the collection of additional clinical data. This policy is referred to as coverage with evidence development (CED), and its goal is to expedite early beneficiary access to innovative technology while ensuring that patient safeguards are in place. The process of developing both LCDs (that are new or have undergone major revision) and NCDs provides opportunities for public comment, and both types of coverage determinations are available in the Medicare Coverage Database on CMS's website.

LCDs and NCDs have similarities (both specify the clinical conditions for which a service is considered to be reasonable and necessary, and both are developed either in response to requests from external parties or internally) and differences, particularly in their scope and flexibility. LCDs are applicable only to services furnished in the MAC's geographic area, while NCDs are applicable nationwide to all services. LCDs permit regional flexibility, are more responsive (compared with NCDs) to community care standards, and allow initial diffusion of new technologies (Jensen 2014). However, there is concern that LCDs result in inequitable variations in coverage across regions (Government Accountability Office 2003).

The national and local processes are not the only means by which Medicare develops and publishes coverage policies. Policies affecting the coverage of services are also published in Medicare's provider manuals and program memorandums, which are often based on the statute or regulations. CMS develops these policies, which apply nationwide to all contractors. Medicare's coding requirements may also implicitly affect the coverage of services.

Over time, Medicare's benefit categories have been expanded to allow reasonable and necessary determinations. For example:

- Beginning in 1994, the Omnibus Budget Reconciliation Act of 1993 expanded Section 1861 of the Social Security Act by covering Part B cancer drugs for indications not approved by the Food and Drug Administration (FDA) if the drug's off-label use is supported by selected third-party drug compendia.
- Beginning in 2000, an executive memorandum directed Medicare to cover the routine costs of qualifying clinical trials and cover services and items that are reasonable and necessary items to diagnose and treat complications due to participation in clinical trials.
- Beginning in 2005, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) directed Medicare to cover the routine costs of care furnished to Medicare beneficiaries in certain categories of investigational device exemption (IDE) studies.
- Beginning in 2008, the Medicare Improvements for Patients and Providers Act of 2008 gave Medicare the authority to cover selected new preventive services through the NCD process.

Although Section 1862(a)(1)(A) of the Social Security Act requires that a service covered by Medicare be "reasonable and necessary," the statute does not define this criterion. CMS and its contractors generally interpret this section to include services that are judged to be safe and effective, not experimental, and appropriate for the beneficiary's medical needs. CMS has operationalized the following definition of the reasonable and necessary standard: "Adequate evidence to conclude that the item or service improves clinically meaningful health outcomes for the Medicare population" (Jensen 2014).

In 1989 and 2000, CMS sought public comments on revising the coverage process that would have considered

Medicare's proposals to consider cost-effectiveness in the coverage process

On two occasions, Medicare tried to interpret the statute's (Section 1862 of the Social Security Act) requirement that Medicare pay only for services that are reasonable and necessary. In 1989, the agency issued a proposed regulation that explicitly considered the cost-effectiveness of services in the coverage process. The proposed rule was never finalized, with stakeholders arguing that the agency could not use criteria for coverage that extended beyond clinical evidence and that the statute did not permit the agency to deny coverage based on cost-effectiveness. In 2000, CMS released a notice of intent (NOI) on new criteria that would have considered cost in the coverage process only for services that provided equivalent clinical benefits compared with an existing covered service but that were more costly. As with the 1989 proposed rule, the new criteria included in the NOI were not finalized.

The 1989 proposed regulation to consider cost-effectiveness in the coverage process

In January 1989, CMS—then the Health Care Financing Administration—released a proposed rule that would have established in regulation criteria to determine whether a health care service was "reasonable and necessary" and therefore covered. The proposed rule sought to add cost-effectiveness to the criteria used in the coverage process to address the increasing availability of new, costly technology, stating, "We believe considerations of cost are relevant in deciding whether to expand or continue coverage of technologies, particularly in the context of the current explosion of high cost technologies" (Health Care Financing Administration 1989).

According to the proposed methodology, a service would have been considered cost-effective if:

- it was less costly and at least as effective as an alternative covered technology;
- it was more effective and costlier than a covered alternative, but improved health outcomes to justify additional expenditures; or
- it was less effective and less costly than an existing alternative for some beneficiaries but was a viable alternative for others.

CMS proposed implementing the following methodology to determine whether a service or technology was cost-effective:

- Identify the relevant alternative technologies to which the current intervention is to be compared.
- Identify all relevant outcomes from the alternative technologies and, when possible, quantify them (e.g., clinical outcomes, reduced morbidity and mortality, or qualitative outcomes).
- Identify all relevant costs expected (both Medicare and non-Medicare) from the interventions,

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a service's medical benefit and value. In 1989, Medicare formally proposed the use of cost-effectiveness as one of several criteria in its coverage process. In 2000, CMS issued a notice of intent to publish a proposed rule, which outlined an approach to develop coverage decisions that would have assessed a service's medical benefit (i.e., comparative health benefit) and added value (as assessed by total costs, not cost-effectiveness). Taking note of comments from stakeholders, including medical providers and manufacturers, the agency did not finalize either approach. Consequently, neither the NCD process nor the LCD process considers a service's cost or cost-effectiveness. The text box provides additional detail about Medicare's proposal to consider cost-effectiveness and value in the coverage process.

National coverage determination process

An NCD is a determination by the Secretary (i.e., CMS's Coverage and Analysis Group) as to whether an item or service is covered nationally by Medicare. Essentially, an

Medicare's proposals to consider cost-effectiveness in the coverage process (cont.)

including direct medical costs or savings and indirect costs.

- Consider unquantifiable factors.

According to the proposed rule, cost-effectiveness would not always be used in the coverage process. For example, if a breakthrough technology had no comparable alternative, there would be no comparative analysis to other available technologies since none existed (Health Care Financing Administration 1989).

Stakeholders, including medical providers and the medical device industry, argued that (1) cost had no role in the coverage process, (2) CMS could not use criteria for coverage that extended beyond what medical experts thought was reasonable and necessary

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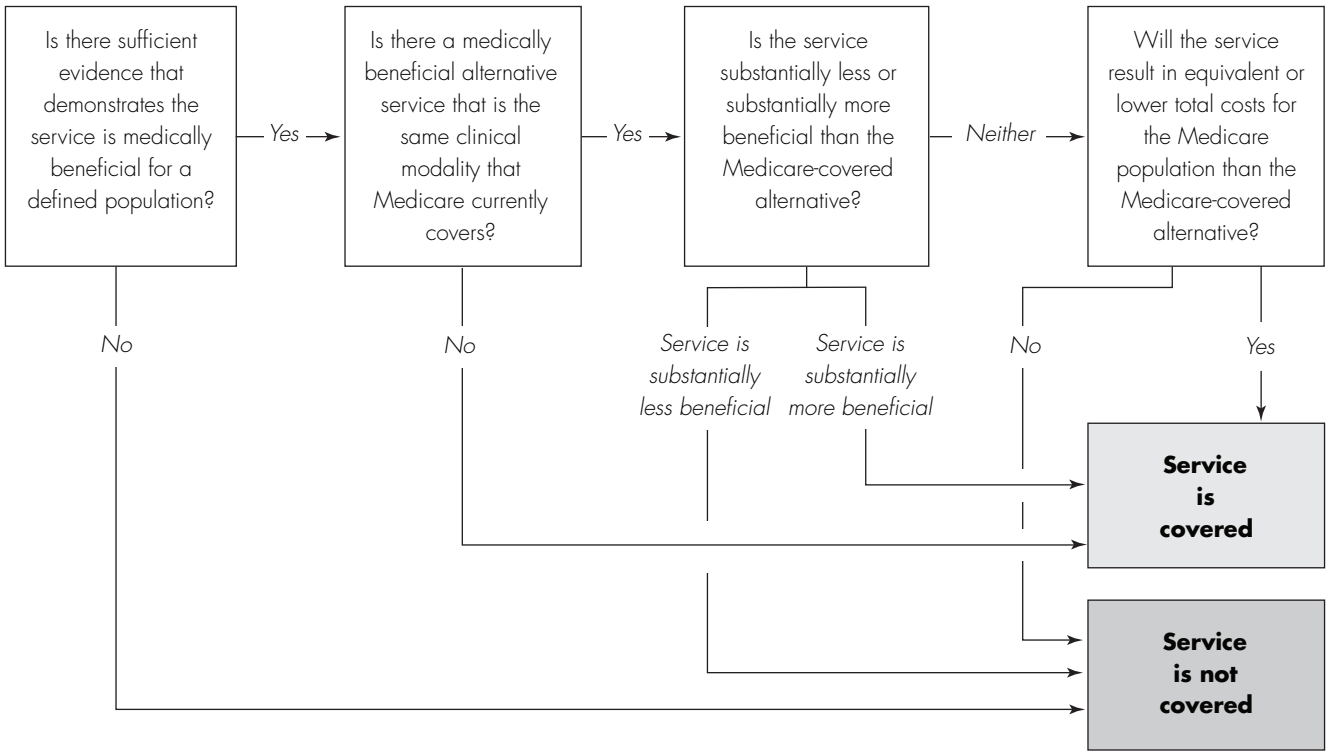
FIGURE 10–1

2000 NOI proposed criteria for making coverage decisions



Note: NOI (notice of intent).

Source: MedPAC analysis of notice of intent to publish a proposed rule on criteria for making coverage decisions, Health Care Financing Administration 2000.

NCD is a policy statement that allows Medicare coverage of a particular service with or without clinical conditions (including coverage with evidence development); leaves the determination to the discretion of the MACs; or precludes Medicare coverage.

Because NCDs are developed by CMS, they do not vary from region to region and are thus binding on all of the program's contractors, including MACs, durable medical equipment regional contractors, quality improvement organizations, program safeguard contractors, and

Medicare's proposals to consider cost-effectiveness in the coverage process (cont.)

for an individual's medical need, and (3) the statute did not permit the agency to deny coverage based on whether a service was or was not cost-effective (Pear 1991). Some stakeholders feared the use of cost-effectiveness was a move toward rationing health care (Neumann 2005). CMS did not finalize the proposed rule, which was formally withdrawn in the late 1990s.

The 2000 notice of intent to consider the notion of *added value* in the coverage process

In May 2000, CMS released an NOI that sought public comments on criteria to determine whether a service was reasonable and necessary under the coverage process if it met the following criteria: The service had to demonstrate medical benefit, and the service had to demonstrate added value to beneficiaries. According to the NOI, cost would be considered in the coverage process in certain circumstances to determine whether a service demonstrated "added value." As shown in Figure 10-1, consideration of cost would have been limited to instances in which two services had equivalent health outcomes and were of the same clinical modality.

CMS provided the following examples of situations in which a service, compared with the current mix of services, would add value and be covered:

- a medically beneficial breakthrough technology;
- a medically beneficial service if no other medical alternative exists;
- a medically beneficial service that is different in clinical modality from the existing item or service;
- a medically beneficial service, even if a less expensive alternative exists but is not included in a Medicare benefit; and
- a medically beneficial item or service that is the same clinical modality as a Medicare-covered alternative and has equal or lower total costs for the Medicare population.

Under the NOI, a service that has equivalent health outcomes and the same clinical modality but is more expensive than a Medicare-covered alternative would not be covered (Figure 10-1).[1] In determining coverage under these criteria, CMS would not compare an item or service that falls within a statutory benefit category with one that is outside the scope of the Medicare program.

The NOI also discussed coverage of a new service that is "substitutable" for a Medicare-covered alternative. The agency sought comments about whether, if the substitutable service has greater total costs to the Medicare program, it should deny coverage but allow the requestor through the reconsideration process to alter the request to seek a positive coverage decision. Another option would be to cover the new service but reduce the payment rate to the same rate as the Medicare-covered alternative (i.e., a least costly alternative policy). Finally, the NOI said that the Medicare program should move toward measuring "quality of life outcomes," and requested public comment on the metric that should be used in the coverage process to quantify this measurement, such as quality-adjusted life years and disability-adjusted life years.

Like the 1989 proposed regulation, stakeholders raised concerns about the NOI, and CMS did not release it as a proposed rule (Foote 2002). While the NOI did not explicitly include cost-effectiveness as a criterion for coverage, some stakeholders perceived that the added-value criterion implied such an analysis (Foote 2002). ■

administrative law judges during the claim appeal process. Since October 2001, NCDs have been binding for MA plans. NCDs take precedence over LCDs that exist on the same clinical topic.

Generally, substantive changes to Medicare policy (e.g., changes in payment policy) are required to go through the notice and comment rule-making procedures. However, NCDs have a separate process to get public

TABLE 10–2 **Total number of NCDs considered by CMS, by fiscal year, 2006–2016**

	Fiscal year										
	2006	2007	2008[a]	2009	2010	2011	2012	2013	2014	2015	2016
New NCD, covered[b]	1	0	2	3	2	4	5	1	1	3	2
New NCD, noncovered	4	3	3	5	3	0	0	0	0	0	0
New NCD, coverage linked to clinical trial or registry	1	0	0	0	0	1	0	0	1	1	0
New NCD, contractor discretion	0	0	2	0	0	1	0	0	0	0	0
Reconsideration	11	9	6	1	7	5	0	5	3	1	2
Total	17	12	13	9	12	11	5	6	5	5	4
Days elapsed until NCD implementation[c]	81 days	114 days	126 days	127 days	118 days	72 days	81 days	132 days	160 days	245 days	301 days

Note: NCD (national coverage determination). In fiscal year 2007, one NCD did not meet the benefit category definition of durable medical equipment. "Days elapsed until NCD implementation" is an average.
[a] In 2008, CMS completed a national coverage analysis for one service, but the agency determined that no NCD was appropriate at the time.
[b] Includes NCDs that specified the clinical conditions for which a service is covered, NCDs that are based on existing LCDs, and NCDs that maintained current covered clinical indications.
[c] Days elapsed from date of final NCD posted on CMS website (i.e., policy effective date) to date of published implementation instructions.

Source: Commission analysis of information from CMS's reports to Congress on national coverage determinations between fiscal year 2006 and fiscal year 2016.

comments. The MMA requires that CMS provide a 30-day public comment period after a proposed determination is published. In most instances, CMS also provides opportunities for public input when the NCD process begins.

The NCD process is used less frequently than the local coverage process. As shown in Table 10-2, between fiscal years 2006 and 2016, the number of NCDs that CMS considered ranged from 4 to 17 in a given year. In August 2017, CMS's website listed roughly 300 active NCDs in its database. By contrast, there were nearly 1,000 final LCDs in Medicare's online database.[2] CMS makes fewer NCDs than LCDs because:

- most services do not meet the criteria for CMS to initiate an NCD;
- limited resources can affect CMS's ability to initiate more NCDs; and
- manufacturers and providers may be apprehensive about requesting an NCD because they perceive that the decision could result in an "all or nothing" scenario in terms of their ability to obtain Medicare payment, and thus they are more likely to pursue LCDs.

A negative NCD can be especially problematic for providers and manufacturers of a service for which Medicare constitutes a large share of the market. However, NCDs are often written for a specific clinical indication of an item or service and can be modified once new clinical information is available.

The NCD process A new NCD is triggered by a request from an external party, including beneficiaries, manufacturers, clinicians, or medical associations; from one of Medicare's administrative contractors; or by CMS staff. Circumstances that can prompt the agency to initiate an NCD include the following:

- Practitioners, patients, or other members of the public have raised significant questions about the outcomes attributable to the use of items or services for beneficiaries.
- New evidence or reinterpretation of existing evidence indicates that an NCD may be warranted.
- LCDs for a particular item or service vary among the MACs.
- The technology represents a substantial clinical advance and is likely to result in a significant improvement in outcomes or positive impact on the Medicare program.
- Rapid diffusion of an item or service is anticipated, and the evidence does not adequately address questions about the impact on beneficiaries.

NCDs are most commonly requested by manufacturers or individuals who are interested in expanding existing coverage (Tunis et al. 2011). After initiating an NCD, CMS releases a tracking sheet on its website that describes the issue being considered and the actions that have been completed. The agency also opens an initial 30-day public comment period on the topic. After conducting a formal review of the evidence, CMS posts a proposed decision memorandum that provides the agency's evaluation of the service and opens a second 30-day request for public comments. CMS's evidence review can be informed by a technology assessment—a systematic analysis of the performance characteristics, safety, effectiveness, outcomes, and appropriateness of a service—from an external entity such as the Agency for Healthcare Research and Quality (AHRQ).[3] In addition, CMS can consult with the Medicare Evidence Development & Coverage Advisory Committee (MEDCAC), an advisory group that was established by the Secretary in 1998 to supplement the agency's clinical expertise and allow for public input and participation.[4] MEDCAC consists of experts in clinical and administrative medicine, biologic and physical sciences, public health administration, patient advocacy, health care data and information management and analysis, health care economics, and medical ethics.

CMS posts final NCDs online in the agency's NCD manual along with a decision memorandum that summarizes public comments and CMS's responses to those comments and the scientific basis for the coverage determination (e.g., an analysis and summary of the evidence considered). Under the time frames that the MMA established for developing NCDs, CMS must:

- issue a proposed NCD within 6 months of the request date for an NCD that does not require a technology assessment from an outside entity or deliberation from MEDCAC or within 9 months for a policy that does require an assessment or deliberation from MEDCAC and
- issue a final NCD 60 days after the end of the public comment period.

Researchers have raised concerns about the lack of high-quality evidence that is available when Medicare develops coverage determinations (Chambers et al. 2015b, Foote et al. 2004, Mohr 2012, Neumann et al. 2008, Redberg 2007). For example, between 2009 and 2013, the evidence considered in NCDs was judged by CMS to be "fair" or "poor" for 81 percent of the services evaluated and "good" for only 19 percent of the services evaluated (Chambers et al. 2015b). These researchers did not identify any changes in the quality of evidence that the agency considered in the NCD process during three time periods analyzed (1999 to 2003, 2004 to 2008, and 2009 to 2013). These researchers also found that, between 1999 and 2013, NCDs were more likely to cite the lack of relevant outcomes and the lack of applicability of study results to the Medicare population as limitations of the supporting evidence.

Reconsideration and challenge of an NCD CMS can internally open a reconsideration of an NCD because of new evidence that could support a material change in coverage, for which the agency would seek public comment on relevant questions. In addition, any individual or entity may request that CMS reconsider any provision of an NCD. As shown in Table 10-2, between 2006 and 2016, the number of NCD reconsiderations ranged from 11 in 2006 to 0 in 2012. Of the 11 reconsiderations implemented between 2012 and 2016 (the 5 most recent years available), all but 1 were initiated by an external party requesting a coverage expansion (data not shown). Nine of the 11 reconsiderations expanded national coverage for the service under consideration (e.g., by expanding the covered population or clinical conditions), 1 turned over

coverage to the local coverage process (i.e., MACs' medical directors), and 1 maintained the national coverage policy.

The Benefits Improvement and Protection Act of 2000 (BIPA) created a process to challenge NCDs that is available to certain beneficiaries, referred to as "aggrieved parties," a category that includes an FFS or MA beneficiary or the estate of a Medicare beneficiary. An aggrieved party can file a complaint concerning an NCD, which is reviewed by the Department of Health and Human Services (HHS) Departmental Appeals Board (DAB). Outcomes of an NCD challenge include the agency conducting a reconsideration of the NCD or the DAB issuing a decision (which constitutes final agency action). This challenge is separate from the process of appealing a MAC's decision on individual claims.

Expedited process to remove NCDs Because clinical science and technology evolve, in 2013, CMS adopted (through rulemaking) an expedited process to evaluate the continued need for older NCDs (that have not been reviewed in 10 years) that meet certain criteria, such as NCDs that no longer contain clinically pertinent and current information and that involve services that beneficiaries use infrequently.[5] CMS expects that removing an NCD will be quicker using the expedited process compared with the reconsideration process. In November 2013, CMS posted 10 NCDs for possible removal and subsequently announced (after a 30-day public comment period) that it would rescind 7 NCDs and retain 3 NCDs (Centers for Medicare & Medicaid Services 2014a). MACs have the discretion to determine coverage for the services specified in a rescinded NCD.

Coverage with evidence development Since 1995, Medicare has linked coverage to the collection of clinical evidence. In making coverage decisions involving CED, CMS (as part of the NCD process) can decide, after a formal review of the medical literature, to cover a service only in the context of an approved prospective clinical study or when additional clinical data are collected to assess the appropriateness of an item or service for use with a particular beneficiary. CMS adopted CED in 2006.

CED is an approach for Medicare to cover potentially beneficial items and services that lack clear evidence showing their clinical effectiveness in specific patient populations. Under CED, beneficiaries have access to medical services while clinical evidence is being collected in prospective clinical studies and registries. Because CED provides Medicare the opportunity to generate clinical evidence that otherwise might not have been collected, it enables the program to ultimately develop better, more evidence-based policies.

CED also provides an opportunity to collect clinical evidence for groups that are often underrepresented in clinical trials, including older beneficiaries and minorities. For example, researchers have reported that older adults are underrepresented in cancer and cardiovascular clinical trials (Dhruva and Redberg 2008, Singh et al. 2017, Talarico et al. 2004). In addition, through CED, Medicare can collect evidence on long-term outcomes and effectiveness in different practice settings that are not always collected in clinical trials (Daniel et al. 2013). However, CED does not duplicate or replace the FDA's authority in assuring the safety and efficacy of drugs, biologics, and devices, and it does not assume the role of the National Institutes of Health in sponsoring clinical trials.

As of April 2018, there were roughly 20 active NCDs that included a CED policy. The design of each CED effort has varied, depending on the service and circumstance leading to the CED policy. A CED cycle is considered "completed" when CMS completes a reconsideration of the coverage determination and removes the CED requirement as a condition of coverage. Since Medicare has linked coverage to the collection of clinical evidence, we are aware of at least three NCDs that have been revised based on the collected evidence:

- In 2003, CMS revised the NCD for lung volume reduction surgery to cover all patients who matched the characteristics of patients in the clinical trial who experienced a survival or quality-of-life benefit.
- In 2013, CMS ended the CED requirement for oncologic uses of fluorodeoxyglucose–positron emission tomography (FDG–PET).
- In 2018, CMS published a coverage decision that ended the CED requirement for the use of MRIs for beneficiaries with implanted pacemakers and other selected implantable devices.

Medicare's statutory justification to apply CED has shifted over time. The agency's earlier CED decisions were made under the Secretary's authority to cover items and services

that are reasonable and necessary (i.e., Section 1862(a)(1)(A) of the statute) (Centers for Medicare & Medicaid Services 2014b). NCDs issued more recently (since 2006) rely on the Secretary's authority under the statute's Section 1862(a)(1)(E) that allows Medicare payment for services determined by AHRQ to reflect the research needs and priorities of the Medicare program.[6] According to CMS, AHRQ reviews and approves the CED questions and general standards for CED studies issued under Section 1862(a)(1)(E). When CED under this section is required, it is because there are outstanding questions about the service's health benefit in the Medicare population. As such, the service is covered only in the context of a study that requires patient monitoring, data collection, and an open presentation of results. When CED under Section 1862(a)(1)(A) is required, it is because additional clinical information is needed to ensure the appropriate use of the service in the Medicare population to facilitate accurate claims processing and payment (Centers for Medicare & Medicaid Services 2014b).

Because Medicare's statutory foundation to apply CED is unclear, some researchers argue that Medicare's use of CED has been hampered and is limited (Daniel et al. 2013, Mohr and Tunis 2010). Mohr and Tunis argued that the agency's lack of clear statutory authority has affected the research questions and study design of the CED effort and the clinical evidence that was collected as well as Medicare's ability to develop a proactive mechanism to identify potential CED topics. Daniel and colleagues also noted the challenges in Medicare's use of CED, citing the lack of well-defined funding sources to conduct such studies, a shared data and research infrastructure, and predictable criteria and methods for conducting studies (Daniel et al. 2013). To improve Medicare's ability to apply CED, Tunis and colleagues proposed a statutory change that would give CMS explicit authority to apply CED to promising technologies that are particularly important to the Medicare population and require better evidence to answer important questions about their clinical effectiveness (Tunis et al. 2011). Daniel and colleagues called for developing an infrastructure for more routine use of electronic health data (compiled into longitudinal clinical registries) that could support CED and quality measurement and suggested that such an effort be supported by payers, physician groups, and other organizations (Daniel et al. 2013).

Requirement that facilities meet safety requirements Medicare also issues NCDs that require facilities furnishing certain services and procedures—including lung cancer screening, ventricular assist devices as destination therapy, and carotid artery stenting—meet certain minimum standards to ensure beneficiary safety. Facilities are sometimes required to participate in a registry that is separate from the CED process. For example, the NCD on lung cancer screening also requires that facilities participate in a registry that collects administrative and clinical information.

Coverage of services furnished in clinical trials In addition to CED, there are two other coverage policies relating to clinical trials: the Clinical Trial Policy and the Investigational Device Exemption (IDE) Policy. Implemented in 2000, the Clinical Trial Policy was first issued through an executive memorandum. CMS subsequently issued an NCD that explains Medicare's coverage of the routine costs associated with qualifying clinical trials, as well as services that treat or diagnose complications that may arise from participation in a clinical trial.

Regarding the IDE Policy, under the MMA, Medicare pays for the routine costs of care furnished to beneficiaries in certain categories of IDE studies. For Category A (experimental) devices—those for which "absolute risk" has not been established and the FDA is unsure of the device's safety and efficacy—Medicare covers the cost of routine care items and services furnished in trials. For Category B devices (nonexperimental/investigational)—where incremental risk is in question or it is known that the device type can be safe and effective—Medicare covers routine care costs as well as the cost of the device.

FDA–CMS Parallel Review Program The FDA–CMS Parallel Review Program, which began as a pilot in 2011, permits a manufacturer to request a concurrent review of clinical evidence for premarket medical devices by the FDA and CMS. The program's goal is to reduce the time between FDA marketing approval and an NCD (Food and Drug Administration and Centers for Medicare & Medicaid Services 2016). In 2013, both agencies permanently extended the program, which accepts five candidates per year and gives priority to devices that will have the largest impact on Medicare beneficiaries (Food and Drug Administration and Centers for Medicare & Medicaid Services 2016).

Under the program, both agencies provide the manufacturer with feedback about the design and analysis of the device's pivotal clinical trial and concurrently and independently review the clinical trial evidence and

communicate (as necessary) with the manufacturer during their respective reviews. CMS opens the NCD process on FDA approval. Although an FDA marketing approval does not guarantee a favorable coverage decision by Medicare, the two technologies that have undergone this process have been covered by the program.

Since 2011, CMS has accepted two tests—Cologuard, a colorectal cancer screening test, in 2014, and FoundationOne CDx, a next-generation sequencing test in 2017—into the Parallel Review Program and issued NCDs concerning their coverage.[7] CMS released the proposed NCD for both tests on the same day that FDA approved the technology, and CMS finalized coverage within four months of the proposed NCD.

The experience to date under the Parallel Review Program shows its potential to expedite the NCD process. Some stakeholders assert that the Parallel Review Program increases collaboration between manufacturers, FDA, and CMS, and it provides beneficiaries with timely and innovative medical devices. However, some stakeholders contend that the program has had a limited impact because it affects few devices and does not address all difficulties that some manufacturers encounter when bringing a device to the U.S. market, such as the timeliness and ease of acquiring a billing code (Podemska-Mikluch 2016). Finally, some stakeholders contend that the program does not address the different evidentiary standards used by FDA and CMS. A device must be "safe and effective" to gain FDA approval, while it must be "reasonable and necessary" to gain CMS approval.

Off-label coverage of anticancer chemotherapy drugs and biologics Effective January 1, 1994, the Omnibus Budget Reconciliation Act of 1993 provided coverage when the indication for an off-label cancer drug is included in third-party drug compendia (privately owned reference guides), which include the American Hospital Formulary Service's *Drug Information*, National Comprehensive Cancer Network's *Drugs and Biologics Compendium*, Micromedex's *DRUGDEX, Clinical Pharmacology*, and *Lexi-Drugs*. The MACs have discretion to ensure that such off-label use is reasonable and necessary. In addition, the medical directors may also identify off-label uses that are supported by clinical research published in peer-reviewed literature.

According to some researchers, there is limited transparency about how compendia are assembled and the conflicts of interest on the part of their contributors, and there are substantial inconsistences both among and within these resources (Green et al. 2016). In addition, there is also concern that the quality of evidence cited in compendia for off-label cancer drug use is less rigorous than the standards supporting FDA-approved drugs (Abernethy et al. 2009).[8]

Local coverage determination process

MACs review claims for services furnished by providers and pay for only those services that meet Medicare's coverage requirements.[9] Consequently, contractors play an important role in protecting the integrity of the Medicare program. The LCD, created by BIPA, is a determination by a MAC's medical director as to whether an item or service is reasonable and necessary.[10] LCDs (1) specify the circumstances (based on clinical conditions, prerequisite treatments, or other factors) in which a service is considered reasonable and necessary; (2) must be consistent with all statutes, regulations, rulings, and national coverage determinations as well as payment and coding policies; and (3) apply only to services provided in the contractor's regional (multistate) jurisdiction.

Each medical director develops and manages LCDs according to the requirements set forth in the *Medicare Program Integrity Manual*. Medical directors can develop an LCD based on requests from external parties (e.g., beneficiaries, providers, or manufacturers) in their jurisdiction. According to the manual, MACs must develop LCDs when they have identified a service that is never covered (under certain circumstances) and want to establish automated reviews. Other circumstances for which medical directors have the option to either develop new or revise existing LCDs include:

- a validated, widespread problem demonstrating a significant risk to the Medicare Trust Funds, identified as potentially high-dollar or high-volume services;
- the need to ensure beneficiary access to care;
- frequent denials being issued or anticipated; and
- the contractor's efforts to create uniform LCDs across multiple jurisdictions.

In addition, LCDs can provide more specific information about an item or service addressed in an NCD. The existence of one or more LCDs does not preclude CMS from making an NCD.

LCDs have a moderate impact on coverage of Part B services. The Office of Inspector General (OIG) estimated that, in 2011, over half (59 percent) of Part B billing codes (for medical procedures, imaging services, evaluation and management visits, drugs, and tests) were subject to an LCD in one or more states, representing about one-quarter of total allowed charges billed for all Part B services (Office of Inspector General 2014).[11]

The LCD process The process for developing an LCD includes drafting language based on a review of medical literature, the contractor's understanding of local practices, the advice of local medical societies and medical consultants, public comments, and comments from the provider community. Contractors are required to provide open meetings to discuss draft LCDs, during which interested parties can make presentations of information related to draft policies. In addition, contractors are required to establish carrier advisory committees (CACs) in each state to provide a forum for information exchange between the contractors and medical professionals (physicians and representatives of other medical organizations) and a beneficiary representative. CACs meet at least three times per year and are composed of physicians, a beneficiary representative, and representatives of other medical organizations. Contractors are required to present draft LCD policies to the CAC (after the meeting with the public).

Contractors must provide a comment period of at least 45 calendar days for all new LCDs and revised LCDs that restrict existing LCDs or make a substantive correction. In addition, contractors must provide a 45-day notice period before the final LCD's effective date. Revised LCDs, for which comment and notice periods are not needed, include policies that liberalize an existing LCD; correct typographical or grammatical errors; add information that clarifies the LCD but does not restrict it; and update a coding issue. All final LCDs are posted on the contractor's website and on Medicare's coverage database.

LCD reconsiderations and challenges Similar to the NCD process, there is a reconsideration process for final LCDs that contractors or interested parties can initiate.[12] BIPA also created a process to challenge LCDs, available to an "aggrieved party"—a Medicare FFS or MA beneficiary or the estate of a Medicare beneficiary. Under this process, which is distinct from the existing appeal rights, an aggrieved party can file a challenge either 6 months before receiving the service or 120 days after receiving the service. The challenge is first reviewed by an administrative law judge, and if complainants are unsatisfied, they can subsequently seek review by the DAB (which would constitute final HHS action). Contractors can initiate a reconsideration process for challenged LCDs.

Variation in LCDs across contractors In contrast to NCDs, LCDs apply only in the contractor's jurisdiction—with one exception: In 2006, CMS required the four regional contractors for durable medical equipment, prosthetic devices, prosthetics, orthotics, and supplies (DMEPOS) to jointly develop and use a single set of coverage policies. Consequently, coverage policies for *non*-DMEPOS services can vary across regions because each contractor sets policies within its specified multistate jurisdiction. CMS encourages a contractor operating in two or more states to develop uniform local coverage policies across all jurisdictions to the extent possible and has taken steps to promote consistency among contractors. For example, one MAC develops coverage, coding, and pricing policies for molecular diagnostic tests and other molecular pathology services under the Molecular Diagnostic Program, which are applied in 28 states.

In two recent evaluations of the LCD process, OIG found variations in local coverage policies and recommended that CMS take steps to reduce this variability to ensure beneficiaries' access to care. Specifically, OIG found:

- In 2011, over half of Part B billing codes were subject to an LCD in one or more states, and LCDs affected coverage for these services differently across states; LCDs defined similar clinical topics inconsistently; and there was no correlation between the number of states with LCDs for services and the unit cost or utilization rate of those services. CMS has taken steps to increase consistency among LCDs but lacks a plan to evaluate LCDs for national coverage, which the MMA required (Office of Inspector General 2014). OIG recommended that CMS continue efforts to increase consistency among existing LCDs and consider requiring MACs to jointly develop a single set of coverage policies. CMS concurred with these recommendations.
- In 2012, MACs varied in the methods and sources used to make coverage determinations for Part B drugs and in the use of payment edits and medical reviews (Office of Inspector General 2016). OIG recommended that CMS assign a single entity to assist

MACs with making coverage determinations and evaluate the cost-effectiveness of edits and medical reviews that are designed to ensure appropriate payments for Part B drug claims. CMS concurred with the second recommendation but not with the first (because a single entity would not capture regional differences, which the agency considers to be a fundamental characteristic of local coverage).

The Government Accountability Office also reported that, due to variations in LCDs, there were coverage inequities for beneficiaries with similar medical conditions and recommended that CMS replace LCDs with NCDs (Government Accountability Office 2003). However, some providers and manufacturers support a regional coverage approach, arguing that it is more responsive to local innovations in medical care than a national approach.

The MMA addressed the variability of LCDs by requiring the Secretary to determine which new LCDs should be adopted nationally and the extent to which greater consistency can be achieved among existing LCDs. To comply with the MMA requirement, CMS convenes workgroups and facilitates communication among the contractor medical directors. For example, CMS convenes face-to-face meetings with the contractors' medical directors multiple times a year to engage in collaborative learning on effective approaches to coverage, address at least one coverage decision topic in a unified manner at each meeting, and develop standardized processes and criteria for coverage decisions when appropriate (Office of Inspector General 2014).

Coverage policies implemented in program manuals

Coverage policies also can be implemented through publication in Medicare's program manuals, memorandums, and rule-making process. Program manuals (including the *Medicare Benefits Policy Manual* and Medicare claims processing manuals) and program memorandums contain operating instructions, policies, and procedures based on statutes, regulations, and directives to further define when and under what circumstances items or services may be covered. For example:

- According to the *Medicare Benefits Policy Manual*, Medicare pays end-stage renal disease (ESRD) facilities furnishing dialysis in a facility or in a patient's home a maximum of 13 treatments during a 30-day month and 14 treatments during a 31-day month unless there is medical justification for additional treatments. CMS reiterated this policy in the final rule for the 2015 ESRD prospective payment system (Centers for Medicare & Medicaid Services 2014c).
- In April 2016, CMS issued a program memo that provided an overview of Medicare's coverage of inpatient and outpatient services for the treatment of substance abuse, which included a summary of available services.

These policies are developed by CMS staff and are binding on all MACs. The number of coverage policies implemented in this manner is unknown.

Medicare's coding process

CMS's coding requirements may implicitly affect the coverage of new services. Medicare's payment systems are organized around standard sets of codes that describe the services furnished by providers to beneficiaries. All services must be appropriately coded for providers to receive payment from Medicare. Two entities are responsible for assigning new codes. The Current Procedural Terminology (CPT) Editorial Panel of the American Medical Association annually updates codes for procedures and other physician services—CPT codes. The Healthcare Common Procedure Coding System (HCPCS) National Panel, which is composed of CMS and insurer representatives, annually updates codes for medical devices and other products—HCPCS Level II codes. Because the code sets maintained by the American Medical Association CPT Editorial Panel and HCPCS National Panel are designed to serve multiple health insurers, not all of the codes are for services or items covered by Medicare.

Appeals process for Part A and Part B services

Beneficiaries and providers have the opportunity to appeal the denial of an individual claim for coverage for services that contractors believe do not fall within a Medicare benefit category, are not reasonable and necessary, or are otherwise excluded by statute or regulation. Under the current process, if dissatisfied with the outcome, the beneficiary, provider, or representative can appeal the determination. Medicare's five levels in the Part A and Part B appeal process are (1) redetermination by the responsible MAC, (2) reconsideration by a qualified independent contractor, (3) hearing by an administrative law judge, (4) review by the Medicare Appeals Council

within the Departmental Appeals Board, and (5) judicial review in the U.S. District Court. The process for appealing an individual claim is distinct from challenging national and local coverage determinations.

Medicare coverage policy rules as they apply to Medicare Advantage plans

MA plans are required to provide the same set of benefits under Medicare Part A and Part B that are available to Medicare beneficiaries in the Medicare FFS program, except that FFS Medicare covers hospice care and covers certain services associated with clinical trials under Medicare's Clinical Trials Policy for MA enrollees. MA plans must use Medicare-certified providers for the provision of all covered services. An additional service that MA can cover, which is treated as a Medicare-covered service under MA, is skilled nursing facility care without a previous three-day hospital stay (at the option of the MA plan).

MA plans must adhere to NCDs and LCDs applicable in their service areas, with two exceptions. One exception applies to regional preferred provider organization (PPO) plans, which cover wide geographic areas spanning multiple Medicare FFS MAC areas. A regional PPO can choose LCDs of one of those MACs and apply them, exclusively and uniformly, throughout the regional PPO's service area. An additional exception applies to local MA plans that include multiple MAC areas. A local MA plan may choose to apply the LCD that is most generous to the beneficiary (as determined by the Secretary) throughout its entire service area.

The MA plan functions like a MAC in that the plan is responsible for deciding whether coverage of an item or service meets Medicare's reasonable and necessary criterion, using "coverage criteria no more restrictive than original Medicare's national and local coverage policies" (as stated in CMS manual provisions). The plan decision can be appealed, and the plan's reconsidered decision can be appealed to an outside independent review entity. The review entity's decision can be further appealed to an ALJ and subsequent appellate levels if the claim meets the minimum dollar threshold for appeals (currently $160 for an appeal to an ALJ and $1,560 for judicial review—the same standard as for appeals in FFS).

Plans are permitted to use tools such as requiring providers to seek prior authorization for certain (typically, expensive) services to have a service covered. Also, plans have leeway in controlling utilization through cost sharing. MA cost sharing can differ from the cost-sharing structure of FFS Medicare and can be either higher or lower than FFS for particular services (for example, by imposing cost sharing for Medicare-covered home health care). There is an overall limit under which the total expected average actuarial value of cost sharing must be less than or equal to the actuarial value of Medicare FFS cost sharing. By statute, certain specified services may not have cost sharing that exceeds the Medicare FFS level—including, for example, renal dialysis services, chemotherapy administration, and "such other services that the Secretary determines appropriate (including services that the Secretary determines require a high level of predictability and transparency for beneficiaries)" (Section 1852(a)(1)(B)(iv)(IV)). Plans cannot impose cost sharing on preventive services that have no cost sharing in FFS.

MA plans can have tiered cost sharing based on the provider an enrollee chooses "as an incentive to encourage enrollees to seek care from providers the plan identifies based on efficiency and quality data," as stated in CMS manual provisions.

Medicare coverage for Part D drugs

Part D is a voluntary prescription drug benefit created by the MMA and implemented on January 1, 2006. Under the Part D program, Medicare contracts with private plans to deliver drug benefits to enrollees. To obtain the drug benefit, Medicare beneficiaries must enroll in a stand-alone prescription drug plan or in a Medicare Advantage–Prescription Drug plan.

Plan sponsors are responsible for creating and managing formularies, which are lists of drugs their plans cover. Part D law and regulations place some constraints on which drugs plan sponsors may cover and how they operate their formularies.

Part D drug definition

To be eligible for coverage under the Part D program, a drug must be approved by the FDA for use and sale in the United States and be prescribed and used for a medically accepted indication. Part D drugs include most outpatient prescription drugs dispensed by retail pharmacies, including self-injectable biological products such as insulin, medical supplies associated with the injection of insulin, and vaccines that are not covered under Part B (42 CFR § 423.100).

There are certain types of drugs that Part D plans are generally not allowed to cover under the basic benefit.

The definition of a Part D drug excludes certain drugs and biological products covered under Medicare Part A or Part B as well as certain drugs or classes of drugs that are not covered under the Medicaid program.[13] Plan sponsors may, however, cover some of these excluded drugs as part of an enhanced Part D plan's supplemental benefits, but enrollees must pay the full premium cost for those additional benefits.

Formulary requirements

Law and regulations lay out requirements for Part D plan formularies. Plan sponsors must have a pharmacy and therapeutics (P&T) committee composed of members who meet certain requirements regarding background (physicians and pharmacists) and conflicts of interest. P&T committees develop and review their formulary's structure, exceptions policies, and protocols for prior authorization and other forms of utilization management. In making decisions about plan coverage and formulary design, P&T committees must take into consideration the strength of scientific evidence and standards of practice.

CMS reviews and approves each plan's formulary to "ensure inclusion of a range of drugs in a broad distribution of therapeutic categories and classes" so that it would not substantially discourage enrollment by any group of eligible individuals, such as those with certain conditions (Centers for Medicare & Medicaid Services 2010).

Plan sponsors must include coverage of the types of drugs most commonly needed by Medicare beneficiaries as recognized in national treatment guidelines. For most drug classes, plans must include two distinct drugs that are not therapeutically equivalent or bioequivalent. In addition, CMS requires that "all or substantially all drugs" in six protected classes be included in Part D plan formularies—anticonvulsants, antidepressants, antineoplastics, antipsychotics, antiretrovirals, and immunosuppressants for the treatment of transplant rejection.

Coverage determinations and appeals

CMS requires Part D plan sponsors to have an appeal process through which enrollees can challenge a denial of drug coverage (a negative coverage determination) in a timely manner. The goal is to ensure that plan formularies do not impede access to needed medications. However, the burden associated with navigating these processes varies from plan to plan.

A coverage determination is issued by a plan, for example, when an enrollee requests coverage of a drug that is not on the plan's formulary (formulary exception) or when an enrollee asks that a drug he or she needs that is on a higher cost-sharing tier be assigned to a lower cost-sharing tier because alternative drugs on the plan's lower cost-sharing tier would not be as effective for the individual (tiering exception).

An appeals request begins with a denied request for a formulary exception or lower cost-sharing amount. To initiate an appeals request, an enrollee, the enrollee's prescribing physician, or the enrollee's authorized representative must request a redetermination from the plan. If dissatisfied with the outcome of the redetermination, the enrollee can ask for reconsideration—a review from an independent review entity. If the enrollee remains dissatisfied, he or she may appeal to an ALJ, then to the Medicare Appeals Council, and finally to federal district court.[14]

Part D requires quicker adjudication time frames for exceptions than for MA medical benefits because "the majority of Part D coverage requests involve prescription drugs an enrollee has not yet received, which increases the risk of adverse clinical outcomes if access to the drug is delayed" (Centers for Medicare & Medicaid Services 2016a). For example, plan sponsors must make a decision about exceptions and coverage determination within 72 hours of a request or within 24 hours for expedited requests.

Evidence of low-value care

Low-value care is the provision of a service that has little or no clinical benefit or care in which the risk of harm from the service outweighs its potential benefit (Chan et al. 2013, Kale et al. 2013). In addition to increasing health care spending, low-value care has the potential to harm patients by exposing them to the risks of injury from inappropriate tests or procedures and may lead to a cascade of additional services that contain risks but provide little or no benefit (Keyhani et al. 2013, Korenstein et al. 2012). For our analysis of low-value care, we reviewed the literature on the prevalence of low-value care in Medicare, Medicaid, and commercial plans; examined selected low-value services in Medicare using 31 measures; and examined case studies of three services or items paid for by Medicare that are potentially low value because they lack evidence of comparative clinical effectiveness.

Review of the literature on low-value care

Potentially inappropriate use of health care services can take three forms: underuse, misuse, or overuse (Chan et al. 2013). Underuse is the failure to provide a service to a patient when the potential therapeutic benefit of a test or treatment outweighs the risks (e.g., not using aspirin for patients with coronary disease) (Kale et al. 2013). Misuse is the delivery of the wrong care (e.g., prescribing the wrong medication to a patient given her clinically established diagnosis) (Kale et al. 2013, Korenstein et al. 2012). Overuse is providing either a service that has little or no clinical benefit or a service in which the risk of harm outweighs its potential benefit (e.g., using an antibiotic to treat a viral infection or repeating a diagnostic test more frequently than necessary) (Chan et al. 2013, Kale et al. 2013). Another term for overuse is *low-value care* (Schwartz et al. 2014). Some researchers contend that reducing or eliminating low-value services would both improve quality and reduce health care spending, though they acknowledge that it may be difficult to precisely identify such services in clinical practice (Colla et al. 2015).

The medical community's most significant attempt to identify services that represent overuse or low-value care is the "Choosing Wisely" campaign, an initiative of the American Board of Internal Medicine (ABIM) Foundation that is supported by the Robert Wood Johnson Foundation. In the latest iteration of this ongoing effort, over 80 medical specialty societies have identified more than 520 tests and procedures that are often overused (ABIM Foundation 2016). The goal of Choosing Wisely is to promote and inform conversations between clinicians and their patients about appropriate tests and treatments. Evaluations of the effects of Choosing Wisely have shown a small decline in some of the services the initiative targets (Hong et al. 2017, Rosenberg et al. 2015). However, the extent to which these reductions can be directly attributed to the campaign or other interventions that address low-value care is unclear.

There is evidence of substantial use of low-value care in FFS Medicare. A team of researchers developed several measures of low-value care drawn from evidence-based lists (such as Choosing Wisely), recommendations by the United States Preventive Services Task Force (USPSTF), and the medical literature, which they applied to Medicare claims data (Schwartz et al. 2015, Schwartz et al. 2014). It is challenging to reliably identify low-value care with claims data because they may not have enough clinical detail to distinguish appropriate from inappropriate use. Thus, a key feature of these measures is that they are designed to allow for explicit trade-offs between the sensitivity and specificity of each measure. Increasing the sensitivity of a measure captures more potentially inappropriate use but is also more likely to misclassify some appropriate use as inappropriate. Increasing a measure's specificity leads to less misclassification of appropriate use as inappropriate, at the expense of potentially missing some instances of inappropriate use. The authors developed two versions of each measure: a broader one with higher sensitivity (and lower specificity) and a narrower one with lower sensitivity (and higher specificity).

In their first article, which used 26 measures, Schwartz and colleagues found the following based on 2009 data:

- Based on the broader versions of the measures, there were 80 instances of low-value care per 100 Medicare beneficiaries, and 42 percent of beneficiaries received at least one low-value service. Total Medicare spending for these services was $8.5 billion.
- Based on the narrower versions of the measures, there were 33 instances of low-value care per 100 beneficiaries, and 25 percent of beneficiaries received at least one low-value service. Total Medicare spending for these services was $1.9 billion (Schwartz et al. 2014).

The researchers also found that regional spending on low-value care (using the narrower version of each measure) ranged from $227 per beneficiary in the 5th percentile (in spending) of hospital referral regions (HRRs) to $416 per beneficiary in the 95th percentile.

The authors grouped the 26 measures into 6 larger clinical categories. Imaging, cancer screening, and diagnostic and preventive testing accounted for most of the volume of low-value care, while imaging and cardiovascular testing and procedures accounted for most of the spending (the sixth category was preoperative testing).

In a second study, Schwartz and colleagues compared the use of low-value services between two groups of beneficiaries: beneficiaries attributed to Medicare Pioneer accountable care organizations (ACOs) and beneficiaries attributed to other health care providers (the control group) (Schwartz et al. 2015). They used the 26 measures of low-

value care from the first study plus 5 new measures. The study compared the change in the use of low-value care between the two beneficiary groups, using the periods before and after the ACO contracts went into effect.[15] The authors found a significant reduction in both volume (–1.9 percent) and spending (–4.5 percent) for low-value services in the ACO group relative to the control group.[16]

There is also evidence that delivery of low-value care exists among payers other than Medicare. A study that included patients ages 18 to 64, across all payer types, found that 19 percent of patient encounters with a health care provider included a low-value service (Barnett et al. 2017). This study used nationally representative data from the National Ambulatory Medical Care Survey (NAMCS) and the National Hospital Ambulatory Medical Care Survey (NHAMCS).

Two studies used data from all payers to examine the use of low-value care in Virginia and Minnesota. A study of Virginia claims data for 5.5 million patients in 2014 found that about 1 in 5 patients received at least 1 low-value service and that $586 million was spent on these low-value services, accounting for 2.1 percent of Virginia's total health care spending (Mafi et al. 2017). This study examined 44 services determined to be of low value based on Choosing Wisely, the USPSTF, Healthcare Effectiveness Data and Information Set® (HEDIS®) measures, and clinical guidelines.

A study of Minnesota claims data from all payers examined the prevalence of 18 low-value services in the categories of imaging, disease screening, and preoperative tests in 2014 (Minnesota Department of Health 2017). The rate of low-value imaging ranged from 1.1 percent (thorax computed tomography (CT) scan with and without contrast) to 35.5 percent (CT scan for suspected appendicitis without prior ultrasound). The rate of low-value screening ranged from 0.4 percent (colorectal cancer screening for adults ages 85 and over) to 18.9 percent (prostate-specific antigen (PSA) screening for men age 75 and over). The rate of low-value preoperative tests ranged from 0.5 percent (preoperative pulmonary function test) to 5.5 percent (preoperative chest X-ray).[17] The low-value measures were based on Choosing Wisely, the USPSTF, and the United Kingdom's National Institute for Health and Care Excellence.

Two studies compared the use of low-value care among commercially insured patients with Medicaid or Medicare patients. Charlesworth and colleagues compared the rate of low-value care in Medicaid patients with commercially insured individuals in Oregon in 2013 (Charlesworth et al. 2016). This study found that 15 percent of Medicaid patients received a low-value service compared with 11 percent of commercially insured patients. The authors also found that the amount of low-value care appeared to be influenced by local practice patterns. For most measures, Medicaid patients had a higher probability of receiving a low-value service if they lived in a region where commercially insured patients had higher rates of low-value care.

Colla and colleagues used data from 2009 to 2011 to compare the prevalence of seven Choosing Wisely services between commercially insured patients and Medicare FFS beneficiaries (Colla et al. 2017b).[18] The authors found little difference in rates of cardiac screening in low-risk, asymptomatic patients; use of dual-energy X-ray absorptiometry (DXA) scans; opioid use in migraine patients; and cervical cancer screening for women over age 65.[19] Imaging for low back pain was more prevalent among the commercially insured population (29 percent) than Medicare beneficiaries (23 percent), while preoperative cardiac testing was more common among Medicare beneficiaries (46 percent) than commercially insured patients (26 percent). The prevalence of low-value care in HRRs appeared to be largely independent of payer type and instead was likely related to local practice patterns, which is consistent with findings from the study by Charlesworth and colleagues and our analysis of PSA testing among men ages 70 and older in FFS Medicare and MA (see text box on examining a measure of low-value care in MA compared with FFS Medicare, pp. 318–321) (Charlesworth et al. 2016).

Reid and colleagues analyzed low-value care and spending using claims data for patients ages 18 to 64 from a large national commercial plan (UnitedHealthcare) (Reid et al. 2016). They used 28 previously published low-value care measures and found that 7.8 percent of patients received at least one low-value service in 2013, accounting for 0.5 percent of total spending. The most common low-value services were triiodothyronine (T_3) measurement in hypothyroidism, imaging for nonspecific low back pain, and imaging for uncomplicated headache.[20]

Another type of low-value care is inappropriate drug use, which can harm patients by causing adverse drug events (Landro 2016, Opondo et al. 2012). In addition, the overprescribing of antibiotics can lead to the formation of antibiotic-resistant infections. Adults ages 60 and over are particularly at risk for inappropriate drug use (Morin

et al. 2016). One systematic review of the prevalence of inappropriate prescriptions to adults ages 65 and over found that one in five prescriptions in the primary care setting was inappropriate (Opondo et al. 2012). Another study found that 20 percent of veterans ages 65 and over had been prescribed at least one potentially inappropriate medication, according to a 2006 HEDIS quality measure (Pugh et al. 2006).[21] A study that used data from the NAMCS and the NHAMCS on patients of all ages found that one in three prescriptions for oral antibiotics in ambulatory settings was inappropriate, and almost 20 percent of antibiotic prescriptions for patients ages 65 and older were inappropriate (Fleming-Dutra et al. 2016).

Although the studies we reviewed differed in their measures of low-value care and the populations they examined, some common themes emerge from the literature. At least some low-value services can be identified with claims data, and low-value care is prevalent across FFS Medicare, Medicaid, and commercial insurance plans. In addition, the amount of low-value care in a geographic area appears to be more a function of local practice patterns than payer type.

Use of selected low-value services in FFS Medicare based on 31 claims-based measures

In a previous analysis examining the use of low-value care in FFS Medicare, the Commission contracted with Schwartz and one of his co-authors (McWilliams) to obtain the algorithms for the 31 measures they developed, which we applied to 100 percent of Medicare claims data from 2012 to 2014 (Schwartz et al. 2015, Schwartz et al. 2014) (see online Appendix 10-A, available at http://www.medpac.gov, for a list of the measures and their sources).[22] We also performed a separate analysis comparing the prevalence of one low-value service in FFS Medicare and MA—the rate of PSA testing among older men, for whom testing is not recommended (see text box on examining a measure of low-value care in MA compared with FFS Medicare, pp. 318–321). For our analysis of the 31 measures in FFS Medicare, we used 2 versions of each measure based on the original studies: a broader version (more sensitive, less specific) and a narrower version (less sensitive, more specific). For each version, we calculated the number of low-value services per 100 FFS beneficiaries, the share of FFS beneficiaries who received at least one low-value service, and total spending across all FFS beneficiaries for each service.

Even though these measures do not include all low-value services, our results show substantial use of low-value care in FFS Medicare in 2014. Based on the measures' broader versions, our analysis found about 72 instances of low-value care per 100 beneficiaries, and more than 37 percent of beneficiaries received at least 1 low-value service (Table 10-3, p. 314). Medicare spending for these services was over $6.5 billion, or 2.0 percent of FFS Medicare spending for the beneficiaries in our sample. Based on the measures' narrower versions, our analysis showed about 34 instances of low-value care per 100 beneficiaries, and almost 23 percent of beneficiaries received at least 1 low-value service. Medicare spending for these services totaled over $2.4 billion, or 0.7 percent of FFS Medicare spending for the beneficiaries in our sample. Between 2012 and 2014, there was a modest decline in volume and spending on low-value services (data not shown).

The differences between the measures' broader and narrower versions demonstrate that the amount of low-value care detected varies substantially based on the measures' clinical specificity. For example, the broader measure of imaging for low back pain included any back imaging for low back pain and therefore captured more inappropriate use but also probably some appropriate use. The narrower version of this measure excluded certain diagnoses and was limited to imaging provided during the first six weeks of the diagnosis of low back pain; consequently, it counted less than one-third as many cases as inappropriate compared with the broader measure (Table 10-3, p. 314).

The measures we used excluded many low-value services (e.g., imaging for pulmonary embolism without moderate or high pretest probability) because it was difficult to distinguish inappropriate from appropriate use of these services with claims data (Schwartz et al. 2014). Therefore, our analysis likely represents a conservative estimate of the number of low-value services in Medicare. In addition, we did not estimate the downstream cost of low-value services because we could not determine through claims data whether a specific low-value service led directly to a downstream service (e.g., a follow-up test or procedure). Consequently, our spending estimates probably understate spending on low-value care.

Among the measures' broader versions, measures with the highest volume in 2014 were imaging for nonspecific low back pain (12.0 per 100 beneficiaries), PSA screening for men ages 75 and over (9.0), and colon cancer screening for older adults (8.0) (Table 10-3, p. 314).[23] Measures with the highest aggregate Medicare spending were percutaneous coronary intervention with balloon angioplasty or stent placement for stable coronary disease (almost $1.3

TABLE 10–3

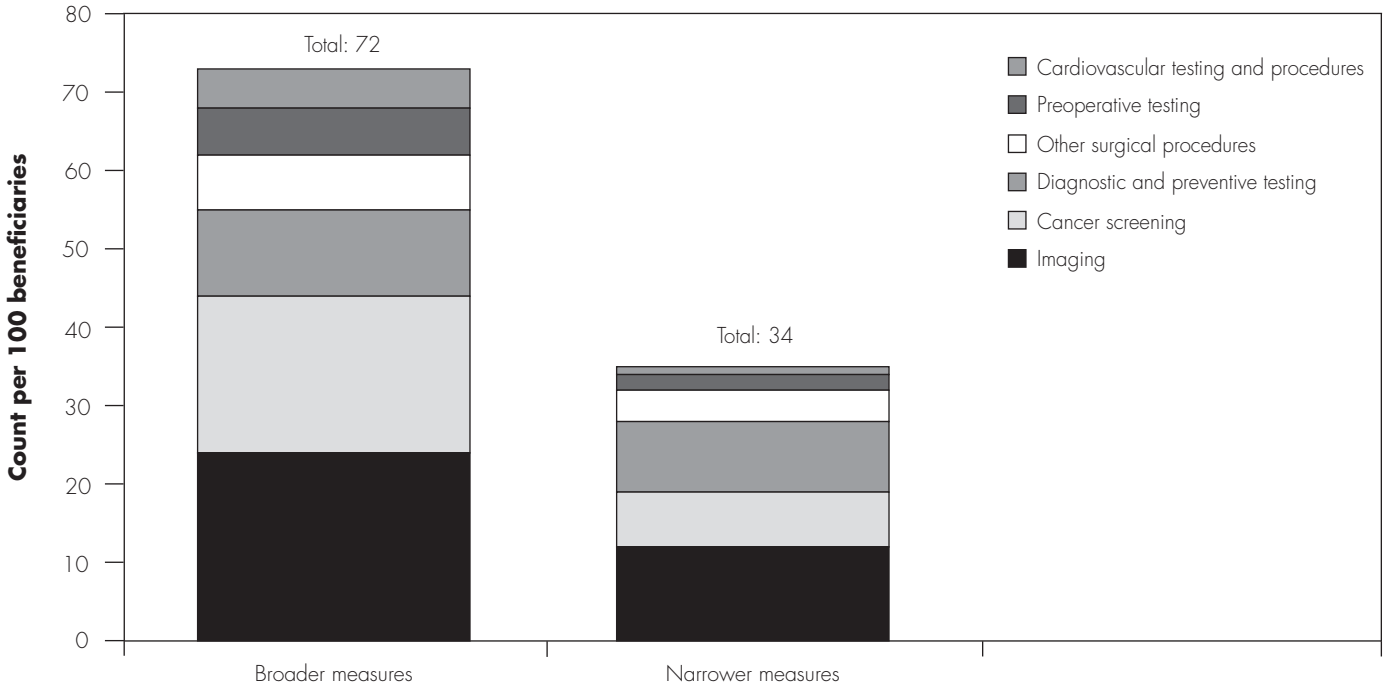
Between 34 and 72 low-value services provided per 100 FFS beneficiaries in 2014; Medicare spent between $2.4 billion and $6.5 billion on these services

	Broader version of measure			Narrower version of measure		
Measure	**Count per 100 beneficiaries**	**Share of beneficiaries affected**	**Spending (millions)**	**Count per 100 beneficiaries**	**Share of beneficiaries affected**	**Spending (millions)**
Imaging for nonspecific low back pain	12.0	8.9%	$232	3.4	3.1%	$66
PSA screening at age ≥ 75 years	9.0	6.2	79	5.1	4.2	44
Colon cancer screening for older adults	8.0	7.5	405	0.3	0.3	3
Spinal injection for low back pain	6.6	3.3	1,261	3.4	2.0	643
Carotid artery disease screening in asymptomatic adults	5.1	4.6	268	4.2	3.8	221
Preoperative chest radiography	4.6	4.1	67	1.1	1.1	17
PTH testing in early CKD	4.5	2.6	83	3.9	2.3	71
Stress testing for stable coronary disease	4.3	4.1	1,198	0.5	0.5	137
T_3 level testing for patients with hypothyroidism	3.8	2.2	23	3.8	2.2	23
Head imaging for headache	3.6	3.3	242	2.4	2.2	160
Cervical cancer screening at age > 65 years	2.2	2.2	44	1.9	1.9	39
Homocysteine testing in cardiovascular disease	1.5	1.2	12	0.4	0.3	3
Head imaging for syncope	1.2	1.1	78	0.8	0.7	51
Preoperative echocardiography	0.8	0.8	62	0.2	0.2	19
Preoperative stress testing	0.6	0.6	177	0.2	0.2	60
Screening for carotid artery disease for syncope	0.6	0.6	33	0.4	0.4	23
CT for rhinosinusitis	0.6	0.5	39	0.2	0.2	17
Vitamin D testing in absence of hypercalcemia or decreased kidney function	0.5	0.4	8	0.5	0.4	8
Imaging for plantar fasciitis	0.5	0.4	9	0.4	0.3	6
BMD testing at frequent intervals	0.4	0.4	9	0.3	0.3	6
Cancer screening for patients with CKD on dialysis	0.4	0.3	9	0.1	0.1	1
PCI/stenting for stable coronary disease	0.3	0.3	1,284	0.1	0.1	216
Arthroscopic surgery for knee osteoarthritis	0.2	0.2	204	0.1	0.1	108
Vertebroplasty	0.2	0.2	338	0.2	0.2	327
Preoperative PFT	0.2	0.2	2	0.1	0.1	1
Hypercoagulability testing after DVT	0.2	0.1	5	0.1	0.1	2
IVC filter placement	0.1	0.1	33	0.1	0.1	33
Carotid endarterectomy for asymptomatic patients	0.1	0.1	165	0.03	0.03	66
EEG for headache	0.1	0.1	4	0.04	0.04	2
Renal artery stenting	0.1	0.1	152	0.02	0.02	51
Pulmonary artery catheterization in ICU	0.01	0.01	0.2	0.01	0.01	0.2
Total	72.2	37.4	6,526	34.2	22.5	2,425

Note: FFS (fee-for-service) PSA (prostate-specific antigen), PTH (parathyroid hormone), CKD (chronic kidney disease), CT (computed tomography), BMD (bone mineral density), PCI (percutaneous coronary intervention), PFT (pulmonary function test), DVT (deep vein thrombosis), IVC (inferior vena cava), EEG (electroencephalography), ICU (intensive care unit). "Count" refers to the number of unique services. The total for share of beneficiaries affected does not equal the column sum because some beneficiaries received services covered by multiple measures. "Spending" includes Medicare Part A and Part B program spending and beneficiary cost sharing for services detected by measures of low-value care. Spending is based on a standardized price for each service from 2009 that has been updated to 2014. See online Appendix 10-A, available at http://www.medpac.gov, for the sources for the measures.

Source: MedPAC analysis of 100 percent of Medicare claims using measures developed by Schwartz and colleagues (Schwartz et al. 2015, Schwartz et al. 2014).

FIGURE 10–2 **Between 34 and 72 low-value services provided per 100 FFS beneficiaries in 2014**



Note: FFS (fee-for-service). "Count" refers to the number of unique services provided to FFS Medicare beneficiaries. See online Appendix 10-A, available at http://www.medpac.gov, for a list of the measures and their sources.

Source: MedPAC analysis of 100 percent of Medicare claims using measures developed by Schwartz and colleagues (Schwartz et al. 2015, Schwartz et al. 2014).

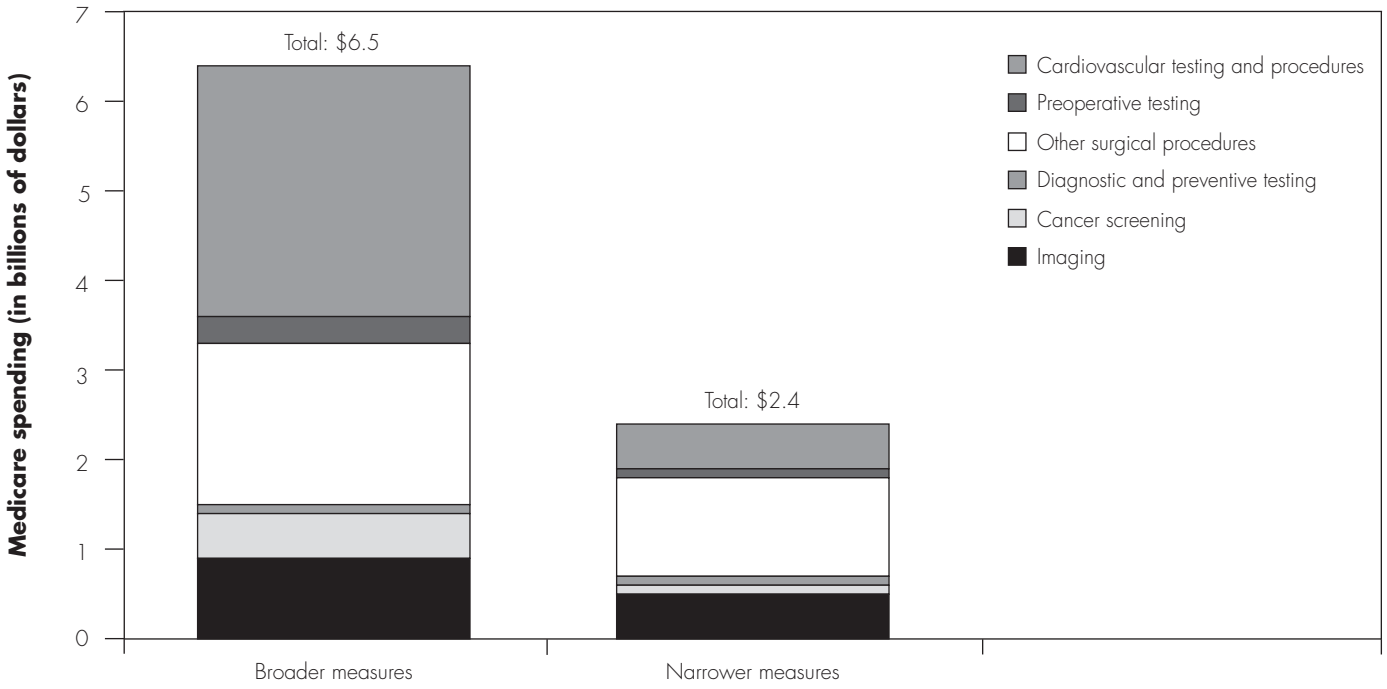
billion), spinal injection for low back pain (almost $1.3 billion), and stress testing for stable coronary disease (almost $1.2 billion).

Among the measures' narrower versions, measures with the highest volume in 2014 were PSA screening for men ages 75 and over (5.1 per 100 beneficiaries), screening for carotid artery disease in asymptomatic adults (4.2), and parathyroid hormone measurement for patients with early chronic kidney disease (3.9) (Table 10-3).[24] The measures with the highest Medicare spending were spinal injection for low back pain ($643 million), vertebroplasty or kyphoplasty for osteoporotic vertebral fractures ($327 million), and screening for carotid artery disease in asymptomatic adults ($221 million).

After grouping the 31 measures into 6 larger clinical categories, we found that imaging and cancer screening measures in 2014 accounted for 44 low-value services per 100 beneficiaries using the measures' broader versions, or 60 percent of the total number of low-value services (Figure 10-2) (see online Appendix 10-A, available at http://www.medpac.gov, for a list of the clinical categories and the measures assigned to each one). In contrast, cardiovascular testing and procedures and other surgical procedures constituted $4.6 billion in spending, or 71 percent of total spending (Figure 10-3, p. 316). Among the measures' narrower versions, imaging and diagnostic and preventive testing accounted for 21 low-value services per 100 beneficiaries (61 percent of the total number of low-value services), while spending on other surgical procedures and imaging was $1.6 billion (67 percent of total spending) (Figure 10-2, this page, and Figure 10-3, p. 316).

We also examined geographic variation in the use of low-value services, using a model developed by Schwartz and colleagues that adjusted for geographic differences in demographic characteristics and comorbidities that

FIGURE 10–3 **Medicare spent between $2.4 billion and $6.5 billion on low-value care in 2014**



Note: "Spending" includes Medicare Part A and Part B program spending and beneficiary cost sharing for services detected by measures of low-value care. To estimate spending, we used standardized prices to adjust for regional differences in payment rates. The standardized price is the median payment amount per service in 2009, adjusted for the increase in payment rates between 2009 and 2014. This method was developed by Schwartz and colleagues. See online Appendix 10-A, available at http://www.medpac.gov, for a list of the measures and their sources.

Source: MedPAC analysis of 100 percent of Medicare claims using measures developed by Schwartz and colleagues (Schwartz et al. 2015, Schwartz et al. 2014).

could affect the use of low-value services.[25] Even after adjusting for these factors, we found substantial variation in the use of low-value care. For example, the adjusted number of low-value services per 100 beneficiaries in 2014 was 61 percent higher in the geographic area at the 90th percentile (of use) compared with the area at the 10th percentile (data not shown).[26] Of the 10 geographic areas with the highest adjusted number of low-value services, 5 were in Florida (Table 10-4). Because we adjusted for differences in beneficiaries' demographic characteristics and chronic conditions, variation in the use of low-value care could reflect such factors as geographic differences in physician practice patterns, entrepreneurial behavior, and beneficiaries' preferences for care.

We also explored the relationship between use of low-value services and overall Medicare service use (which includes all Part A and Part B services) among geographic units in 2014. Our measure of overall service use adjusted for regional differences in input prices, special payments to certain providers, and beneficiaries' demographic characteristics and health status. We ran a regression with overall service use per beneficiary as the dependent variable and the adjusted number of low-value services per beneficiary as the explanatory variable. This regression produced a coefficient for the number of low-value services of 0.77 and an R^2 of 0.29. This result indicates a modest positive relationship between low-value care and overall service use, which is not surprising. Beneficiaries who receive more services in general are more likely to receive services classified as low value. In addition, higher use of low-value care and higher overall service use could be driven by similar factors, such as more aggressive practice patterns, patient preferences for more tests and procedures, and a greater supply of providers.

TABLE 10–4 **Geographic areas with the highest adjusted number of low-value services, 2014**

Geographic area	Adjusted number of low-value services per 100 FFS beneficiaries
Yuma, AZ	56
Punta Gorda, FL	53
Miami–Ft. Lauderdale–W. Palm Beach, FL	51
Ocala, FL	51
Sebastian–Vero Beach, FL	51
Naples–Immokalee–Marco Island, FL	49
Beaumont–Port Arthur, TX	48
Hammond, LA	47
New York–Newark–Jersey City, NY/NJ	47
Sumter, SC	46

Note: FFS (fee-for-service). Geographic areas are defined as the metropolitan statistical areas (MSAs) of the core-based statistical areas. If an MSA crosses state borders, the MSA is divided into multiple areas based on state borders. The number of an area's low-value services is adjusted for the demographic characteristics and comorbidities of the area's beneficiaries. This table is based on the narrower versions of the measures of low-value services (instead of the broader versions) because they represent a more conservative estimate of low-value care. See online Appendix 10-A, available at http://www.medpac.gov, for a list of the measures and their sources. The national average number of low-value services per 100 beneficiaries is 32.1.

Source: MedPAC analysis of 100 percent of Medicare claims using measures developed by Schwartz and colleagues (Schwartz et al. 2015, Schwartz et al. 2014).

Case studies of potentially low-value services

We examined three case studies of services that lack evidence of comparative clinical effectiveness and are therefore potentially low value. The services examined in these case studies are early dialysis for end-stage renal disease, proton beam therapy, and H.P. Acthar Gel® (Acthar, a drug covered under Part D).

Case study 1: Trend in starting dialysis earlier in the course of chronic kidney disease

The timing of starting dialysis for end-stage renal disease (ESRD) is a matter of clinical judgment, guided by values of residual kidney function and symptoms and comorbidities present in affected patients. Data from the mid-1990s through 2010 suggest a trend toward initiating dialysis earlier in the course of chronic kidney disease (CKD). The proportion of new dialysis patients with higher levels of residual kidney function steadily increased between 1996 and 2010, from 13 percent to 44 percent (Figure 10-4, p. 322). (An estimated glomerular filtration rate (eGFR)—a measure of residual kidney function—above 10 mL/min/1.73 m^2 is considered a higher level of residual kidney function. Lower values of this measure suggest comparatively less residual kidney function.)

While the share of patients initiating dialysis earlier in the course of CKD decreased modestly between 2011 and 2016 (from 43 percent to 40 percent, respectively), the share remains three times higher than in 1996. The trend of earlier dialysis initiation is seen in other countries, but U.S. dialysis patients are initiated at a higher mean eGFR level than most other countries (Robinson et al. 2014).

Researchers have questioned this early initiation of dialysis in those with late-stage CKD, concluding that it is not associated with improved survival or clinical outcomes (Cooper et al. 2010, Evans et al. 2011, Kazmi et al. 2005, Stel et al. 2009, Traynor et al. 2002). Of the few randomized controlled trials (RCTs) on this topic, the most influential RCT found that survival is similar between patients for whom dialysis is initiated early (with an eGFR equal to 10.0 mL/min/1.73 m^2 to 14.0 mL/min/1.73 m^2) and those for whom dialysis is electively delayed (with an eGFR equal to 5.0 mL/min/1.73 m^2 to 7.0 mL/min/1.73 m^2) and concluded that dialysis can be delayed for some patients until the eGFR drops below 7.0 mL/min/1.73 m^2 or until more traditional clinical indicators for the initiation of dialysis are present (Cooper et al. 2010). Since publication of this RCT in 2010, the share of early dialysis starts has begun to level off, but it has not yet returned to its earlier levels (Figure 10-4, p. 322). Furthermore, one study

Examining a measure of low-value care in Medicare Advantage compared with fee-for-service Medicare

For the past three years, Medicare Advantage (MA) plans have been reporting the rates of use of a specific low-value service through the Healthcare Effectiveness Data and Information Set® (HEDIS®): the rate of prostate-specific antigen (PSA) testing among men ages 70 and older for whom testing is not recommended (which is different from the age 75 cut-off for other analyses). Unlike measures reported through HEDIS that are based on medical record sampling (411 records per contract), for this measure, plans use administrative or claims and encounter data to report a rate. For this reason, and because the measure applies to a large segment of the population, the measure lends itself to comparison with the Medicare fee-for-service (FFS) population. For the comparison, we computed FFS PSA testing rates using FFS claims data and applying the HEDIS measure specifications.

Because this measure applies to a large number of beneficiaries, we were able to examine MA and FFS results for this measure by metropolitan areas. That is, we were able to do a market-level analysis using a geographic unit that the Commission has recommended as a possible geographic unit for quality reporting in MA and FFS. The PSA testing measure shows wide variation among MA plans across metropolitan areas—the rate at the 90th percentile is 2.1 times that of the rate at the 10th percentile of metropolitan areas (compared, for example, with the MA breast cancer screening HEDIS measure, which has a 90th-to-10th percentile ratio of 1.2 across metropolitan areas). The data also permit us to analyze variation within markets.

In our analysis, we used data from MA HMO plans on the assumption that HMO plans are more likely to be able to control the use of low-value care and should be expected to perform better than FFS in a given market area. We included only metropolitan statistical areas (MSAs) in which there were at least 1,000 HMO enrollees included in the denominator of the measure (excluding Puerto Rico). Of the 408 metro

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TABLE 10–5

Metropolitan areas with the highest rates of nonrecommended PSA testing among Medicare Advantage HMOs

	MA			Percentile rank	
MSA/metro division name	Male enrollees ages 70 and older	Number receiving nonrecommended PSA test	Rate	MA	FFS
Miami–Miami Beach–Kendall, FL	45,052	31,176	69%	100	100
Fort Lauderdale–Pompano Beach–Deerfield Beach, FL	23,540	14,637	62	95	93
McAllen–Edinburg–Mission, TX	4,201	2,462	59	93	43
West Palm Beach–Boca Raton–Delray Beach, FL	13,062	7,438	57	92	99
Beaumont–Port Arthur, TX	2,881	1,516	53	91	95
Knoxville, TN	11,848	6,066	51	90	91
Corpus Christi, TX	5,155	2,635	51	89	88
Jacksonville, FL	5,678	2,899	51	88	52

Note: PSA (prostate-specific antigen), MA (Medicare Advantage), MSA (metropolitan statistical area), FFS (fee-for-service). The denominator used to calculate the rate includes all men ages 70 or over, with certain exclusions (such as prostate cancer diagnosis, dysplasia of the prostate, or prior elevated PSA finding). The exclusions could not be applied to the FFS data, and there may be coding differences between the MA and FFS data, limiting our ability to make a direct comparison of actual MA and FFS rates.

Source: MedPAC analysis of 2017 Healthcare Effectiveness Data and Information Set® data and 2015 FFS claims data.

Examining a measure of low-value care in Medicare Advantage compared with fee-for-service Medicare (cont.)

areas for which we have data reported in the most recent HEDIS reporting period, 113 metro areas (MSAs and metropolitan divisions of large MSAs) met the criterion. The total number of enrollees in the MA denominator for our analysis of the 113 areas was 1.7 million (out of 1.9 million enrollees across all 408 metro areas). The MA results are based on the 2017 HEDIS results for "measurement year" 2016. Our claims-based FFS results are based on claims from 2015.

We found that high rates of nonrecommended PSA testing were common to both MA and FFS in many metropolitan areas. Table 10-5 reports the rates for the metro areas with the highest MA PSA testing rates, along with the percentile ranking across metropolitan areas for MA and for FFS. Table 10-5 shows that the Miami metropolitan area had the highest relative level of PSA testing among men ages 70 and older for both MA and FFS. (The 100 percentile ranking means that Miami is at the 100th percentile of metro areas.) Among the metropolitan areas shown in Table 10-5, two metropolitan areas show substantially better performance in FFS than in MA: In relation to FFS PSA testing levels across all the 113 metropolitan areas, both the Jacksonville, FL, and McAllen, TX, metro areas have lower FFS rates of PSA testing relative to other areas, while their MA testing rates are very high. (The correlation coefficient of the percentile rankings of the MSAs we examined showed a moderate correlation of 0.60 between an area's ranking for MA rates and FFS rates.)

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TABLE 10–6

Metropolitan areas with the lowest rates of nonrecommended PSA testing among Medicare Advantage HMOs

	MA			Percentile rank	
MSA/metro division name	Male enrollees ages 70 and older	Number receiving nonrecommended PSA test	Rate	MA	FFS
Oakland–Hayward–Berkeley, CA	34,649	5,681	16%	0	46
San Francisco–Redwood City–South San Francisco, CA	15,971	2,660	17	1	11
Sacramento–Roseville–Arden-Arcade, CA	30,600	5,363	18	2	27
Santa Rosa, CA	7,824	1,425	18	3	9
Portland–Vancouver–Hillsboro, OR–WA	29,553	5,804	20	3	19
Denver–Aurora–Lakewood, CO	30,714	6,091	20	4	49
San Jose–Sunnyvale–Santa Clara, CA	20,636	4,100	20	5	54
Salem, OR	5,450	1,084	20	6	16
Seattle–Bellevue–Everett, WA	26,488	5,370	20	7	15
Urban Honolulu, HI	6,600	1,340	20	8	71
Minneapolis–St. Paul–Bloomington, MN–WI	17,851	3,982	22	8	3
Albuquerque, NM	12,388	2,775	22	9	14

Note: PSA (prostate-specific antigen), MA (Medicare Advantage), MSA (metropolitan statistical area), FFS (fee-for-service). The denominator used to calculate the rate includes all men ages 70 and over, with certain exclusions (such as prostate cancer diagnosis, dysplasia of the prostate, or prior elevated PSA finding). The exclusions could not be applied to the FFS data, and there may be coding differences between the MA and FFS data, limiting our ability to make a direct comparison of actual MA and FFS rates.

Source: MedPAC analysis of 2017 Healthcare Effectiveness Data and Information Set® data and 2015 FFS claims data.

Examining a measure of low-value care in Medicare Advantage compared with fee-for-service Medicare (cont.)

Table 10-6 (p. 319) shows the metro areas at the other end of the spectrum—where MA nonrecommended PSA testing rates are low relative to other metro areas (the Oakland, CA, area, at the 0 percentile rank for MA, has the lowest PSA testing rate for MA among the 113 metro areas). Many of the areas (such as the San Francisco area and Minneapolis) have low PSA testing rates in both MA and FFS.

We note that Kaiser Foundation Health Plan (Kaiser) figures prominently in the areas with low use of nonrecommended PSA testing. Except for Albuquerque and Minneapolis, Kaiser has significant MA enrollment in each of the areas listed in Table 10-6 (p. 319). As we noted, the data permit an intramarket analysis, allowing us to look more closely at the different MA plans operating in high-performing markets (Table 10-7). Table 10-7 illustrates what is true for all of the California MSAs shown in Table 10-6 (p. 319), which is that Kaiser is primarily responsible for the area's good performance relative to other market areas (using Sacramento to illustrate the California situation because of the large number of enrollees of other organizations in that MSA). Other HMOs in the same market do not perform as well as Kaiser. This contrast is not surprising in that Kaiser is a group-model HMO of salaried physicians providing services only to its enrollees, with the health plan (and the Permanente Medical Group) being better able to determine standards of utilization for all their physicians. (The correlation coefficient of the MA and FFS percentile rankings rises to 0.69 if we exclude the MSAs with large Kaiser enrollment.)

Inferences drawn from our analysis of nonrecommended PSA testing in Medicare FFS and MA

Many geographic areas have high levels of PSA testing among MA plans, considering this low-value care measure has been in place for three years. Plans have a financial incentive to control the frequency of this service to reduce costs of the test itself and subsequent tests and services that could be of questionable value. An additional consideration is the incentive of addressing quality of care concerns for a plan and for patients who may be subjected to a battery of tests

TABLE 10–7

Within-market nonrecommended PSA testing rates among MA HMOs in the Sacramento–Roseville–Arden-Arcade, CA MSA

	MA HMO		
Parent organization	**Male enrollees ages 70 and older**	**Number receiving nonrecommended PSA test**	**Rate**
Kaiser Foundation Health Plan	21,534	2,627	12%
Centene Corporation	2,176	606	28
UnitedHealth Group	4,419	1,248	28
Anthem	386	130	34
Humana	517	195	38
California Physicians' Service	1,190	477	40

Note: PSA (prostate-specific antigen), MA (Medicare Advantage), MSA (metropolitan statistical area). The denominator used to calculate the rate includes all men ages 70 and over, with certain exclusions (such as prostate cancer diagnosis, dysplasia of the prostate, or prior elevated PSA finding). The overall MA PSA testing rate for the Sacramento MSA for all enrollees in all plans serving the MSA, shown in Table 10-4 (p. 317), is 18 percent.

Source: MedPAC analysis of 2017 Healthcare Effectiveness Data and Information Set® data and 2015 fee-for-service Medicare claims data.

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Examining a measure of low-value care in Medicare Advantage compared with fee-for-service Medicare (cont.)

and procedures that are unwarranted. The incentives for plans to control PSA testing may not translate into an incentive for individual physicians to be judicious in the use of this service, particularly if a plan pays physicians on a fee-for-service basis without any financial risk for physicians tied to their utilization. The high rates among some MA plans suggest that if CMS wishes to see reductions in the use of this low-value service, the PSA testing measure could be included as a star measure in the quality bonus program. For example, the HEDIS MA measure of whether adult body mass index is recorded rose from an average rate of 46 percent in 2012, when first included in the star rating system, to the current average rate of 95 percent across MA plans. Though physicians may be paid on a fee-for-service basis without shared risk, some MA sponsors use star rewards programs to provide annual bonuses that are tied to performance on HEDIS measures that are included as star ratings.

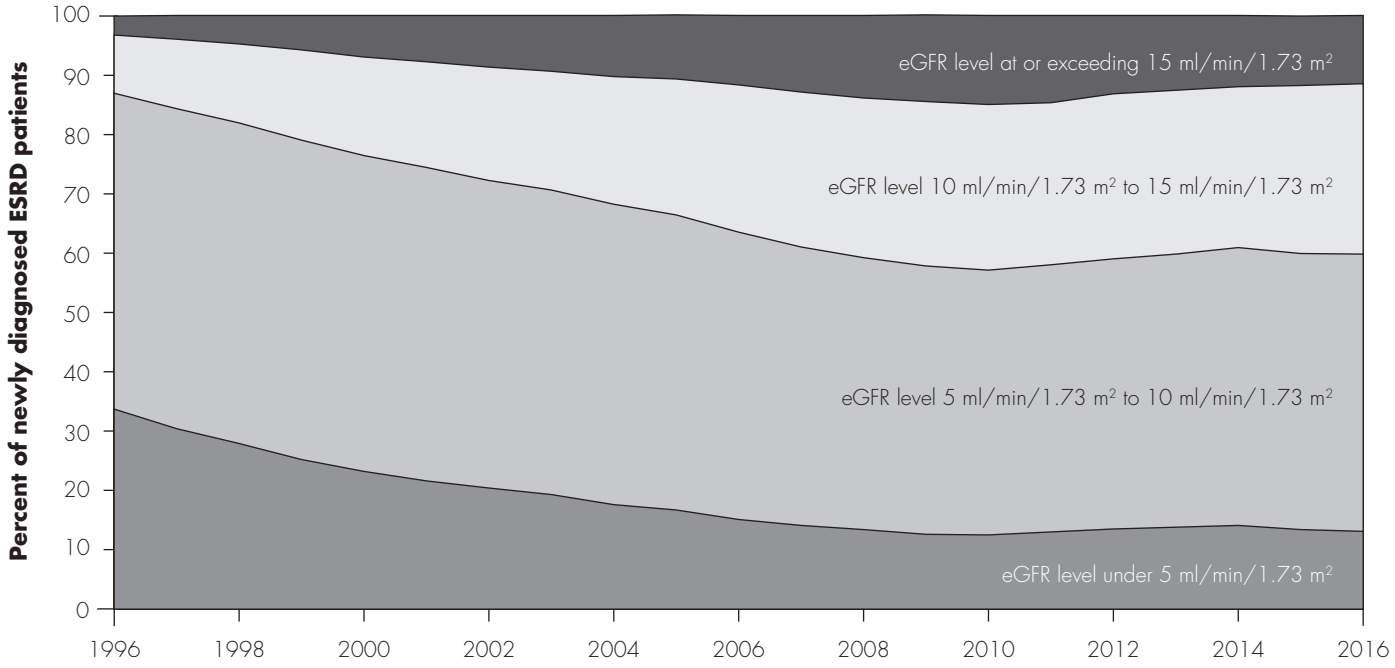
The results also speak to the issue of whether there is "spillover" in care patterns between MA and FFS. A beneficial spillover effect would be that, in areas where MA plans have low rates of PSA testing, the conservative use of the measure would spill over into FFS and reduce overutilization of the service in FFS. The PSA testing data are inconclusive in this respect. In areas such as Albuquerque and Minneapolis, for example (where Kaiser is not present in the market), is the good performance in both MA and FFS (Table 10-6, p. 319) due to the influence of health plans? Or is it a reflection of the practice patterns of the area's physician community—in the same way that, in Miami, the high testing rates in both MA and FFS are likely to reflect the community standard of care?

One further observation, given the Commission's interest in being able to compare quality between MA and FFS, is that the PSA measure is almost exceptional as a measure allowing MA-to-FFS comparisons with the data currently available. The PSA measure has a denominator of 3.3 million across all MA plans, and the measure can be compared with FFS using claims data. For other MA measures, aside from the breast cancer screening measure (a denominator of 3.5 million) and the hospital readmission measure (2.5 million), other HEDIS measures used in the MA star rating system have relatively small denominators (500,000 or fewer—down to 108,000, across MA, for the osteoporosis management measure). For this reason, and because of issues with risk adjustment (for the readmission measure), more work is needed before we are able to do more MA-to-FFS comparisons. ■

reviewing medical records of Department of Veterans Affairs (VA) patients between 2000 and 2009 found that eGFR at dialysis initiation increased, but clinical indicators did not simultaneously increase, indicating that clinical acuity was likely not driving the increase in earlier dialysis initiation (Wong et al. 2016). Moving forward, it will be important to continue monitoring factors that can affect dialysis initiation to ensure that patients receive the most effective and efficient dialysis care.

Our analysis of data on the clinical and demographic characteristics of all patients who started dialysis between 1996 and 2016 found that patients who started dialysis with higher levels of residual function were more likely to be older, male, white, and insured; have certain comorbidities such as congestive heart failure, diabetes, and cerebrovascular disease; and be unable to ambulate or transfer, be institutionalized, and need assistance with daily activities (Table 10-8, pp. 324–325). Dialysis facility characteristics, including profit status and chain status (data not shown), have a relatively small effect on dialysis timing. Our results are generally consistent with other researchers', as summarized in the text box on factors influencing the timing of dialysis initiation (pp. 327–329) (Kausz et al. 2000, Li et al. 2017, O'Hare et al. 2011, Slinin et al. 2014). We estimate that Medicare dialysis spending in 2016 for FFS beneficiaries who initiated treatment with higher levels of kidney function ranged from $500 million to $1.4 billion.

FIGURE 10–4 **Dialysis has been initiated with higher levels of residual kidney function since 1996**



Note: ESRD (end-stage renal disease), eGFR (estimated glomerular filtration rate). "Higher levels of residual kidney function" refers to patients with an eGFR (a measure of residual kidney function) above 10 milliliters per minute per 1.73 square meters. (Lower values of this measure suggest reduced residual kidney function.) Population includes only patients newly diagnosed with CMS Form 2728.

Source: MedPAC analysis of Medicare's medical evidence form (Form 2728) submitted by dialysis providers to CMS.

Since 2010, improved comparative clinical effectiveness evidence has moderated the trend of early dialysis The trend of earlier dialysis initiation began in part because older studies—none of which were RCTs—indicated that beginning patients on dialysis at higher levels of renal function would allow them to preserve residual kidney function, prevent or reverse nutritional deterioration, and increase survival rates (CANUSA 1996, Hakim and Lazarus 1995, Lin and Zuo 2015, Owen et al. 1993, Rosansky et al. 2011). Based on this research, multiple national and international nephrology groups began releasing clinical guidelines in the late 1990s that promoted dialysis initiation at progressively higher eGFR values (Lin and Zuo 2015, O'Hare et al. 2011). Although these guidelines were intended to assist providers in making decisions, the circumstances in which patients initiate dialysis are often complicated by additional factors that may not be fully addressed in the guidelines (e.g., eGFR trajectory over time, acute illnesses, and preferences of patients and providers).

The tendency to initiate dialysis early began to shift in the late 2000s as more studies comparing the outcomes of patients starting dialysis at different times found that beginning dialysis at earlier levels of kidney function provided no advantage over starting dialysis later and, in some cases, led to worse patient outcomes (e.g., mortality) (Beddhu et al. 2003, Rosansky et al. 2009, Wright et al. 2010).

Few RCTs comparing patient outcomes based on dialysis start time have been published (Cooper et al. 2010). The most influential RCT, the Initiating Dialysis Early and Late (IDEAL) RCT, assigned patients to one of two groups: the early-start group (eGFR 10–14 mL/min/1.73 m^2) or the late-start group (eGFR 5–7 mL/min/1.73 m^2). While patients were supposed to begin dialysis based on the group that they had been assigned to, clinicians were not to delay dialysis if they believed the patient required it. In the end, the late-start group initiated at a higher eGFR than originally anticipated, which provided a smaller between-group difference. Between the two groups, researchers found no significant

differences in survival rates, cardiovascular or infectious events, or quality of life (Table 10-9, p. 326) (Cooper et al. 2010, Harris et al. 2011). IDEAL therefore challenged the previous notion that an earlier start to dialysis led to better patient outcomes.

Some researchers have raised concerns about IDEAL's design and study population. Regarding the timing of patients beginning dialysis, the mean eGFR at dialysis initiation for the late-start group was higher than originally planned, which could minimize potential differences between the two groups (Lin and Zuo 2015). Because the study took place in Australia and New Zealand, some question the generalizability of its results for a U.S. patient population, which is more diverse and has a higher prevalence of comorbidities (Rivara and Mehrotra 2017). Additionally, IDEAL participants had lower use of catheters and in-center hemodialysis than the general U.S. dialysis population.

Recent retrospective studies (that are not RCTs) since 2010 have generally confirmed IDEAL's findings that early initiation of dialysis relative to later initiation does not improve patient outcomes, and for some patients it can lead to worse outcomes (Rivara and Mehrotra 2017, Susantitaphong et al. 2012). Because no clear time frame for dialysis initiation has emerged in the literature, recent studies and the most current clinical guidelines advocate for an individualized approach to initiation based on patient signs and symptoms indicating kidney failure (Lin and Zuo 2015, National Kidney Foundation 2015, Rosansky et al. 2011).

Costs associated with early dialysis initiation We estimate that dialysis spending in 2016 for FFS Medicare beneficiaries who initiated treatment with higher levels of kidney function ranged from $500 million to $1.4 billion. The first estimate is based on the additional number of FFS beneficiaries who initiated early treatment (with an eGFR of 10 ml/min/1.73 m^2 or more) in 2016 relative to 1996. The second estimate is based on the research finding that dialysis began five months earlier in 2007 compared with 1997, which we applied to the number of new FFS Medicare dialysis beneficiaries in 2016.[27]

Case study 2: Proton beam therapy

Proton beam therapy is a type of external beam radiation therapy used primarily for cancer treatment. Although it was initially a treatment for pediatric cancers and rare adult cancers, its use has expanded in recent years to include more common conditions, such as prostate and lung cancer. However, there is a lack of evidence that it offers a clinical advantage over alternative treatments for these types of cancer. Nevertheless, the number of proton beam centers in the United States has increased rapidly since 2009. Medicare's payment rates are substantially higher for proton beam therapy than other types of radiation therapy, and Medicare has few coverage restrictions on this treatment. Spending and volume for proton beam therapy in FFS Medicare grew rapidly from 2010 to 2016, driven by the sharp increase in the number of centers and Medicare's relatively broad coverage. Prostate cancer was by far the most common condition treated by proton beam therapy in Medicare, accounting for almost half of total spending and volume.

Compared with other types of radiation therapy, proton beam therapy delivers a more focused beam of radiation to the tumor and no "exit" dose that irradiates tissue beyond the tumor (Massachusetts General Hospital Cancer Center 2013). It delivers the majority of radiation to the target site with less scattering of radiation to adjacent normal tissues. Initially, proton beam therapy was used primarily for rare conditions for which it is very important to spare sensitive normal tissues adjacent to the tumor, such as cancers of the brain stem, eye, or spinal cord (Ollendorf et al. 2014). It was also used for many pediatric tumors because low-dose irradiation of normal tissue in pediatric patients can cause acute and long-term toxicity. Recently, however, proton beam therapy has been expanded to treat more common cancers such as prostate, lung, liver, and breast cancer because of its ability to spare adjacent tissues from excess radiation (Ollendorf et al. 2014). Despite growth in the use of proton beam therapy for more common cancers, there are uncertainties about its effects on deep-seated tumors such as prostate tumors; about whether there is more scattering of the beam to adjacent tissues than originally estimated; and about the effects of the neutrons that are produced by proton beams on the radiation dose to the patient (Ollendorf et al. 2014).

The Institute for Clinical and Economic Review (ICER) evaluated the evidence of the overall net health benefit (which takes into account clinical effectiveness and potential harms) of proton beam therapy in comparison with its major treatment alternatives for various types of cancer (Ollendorf et al. 2014).[28] ICER concluded that proton beam therapy has superior net health benefit for ocular tumors and incremental net health benefit for adult brain and spinal tumors and pediatric cancers.[29] ICER judged that proton beam therapy is comparable with alternative treatments for prostate, lung, and liver

TABLE 10–8

Mean levels of residual kidney function by patients' characteristics and site of care, 1996–2016

	Mean eGFR				
	1996	**2004**	**2012**	**2015**	**2016**
All new dialysis patients	**6.9**	**9.2**	**10.2**	**9.9**	**9.9**
Age					
≥18 and ≤45 years	6.3	8.2	9.3	9.1	9.0
>45 and ≤65 years	6.8	9.0	10.1	9.7	9.7
>65 and ≤75 years	7.1	9.5	10.5	10.1	10.1
>75 years	7.1	9.8	10.6	10.3	10.3
Gender					
Male	7.1	9.6	10.5	10.2	10.2
Female	6.6	8.8	9.7	9.5	9.5
Race					
White	7.1	9.5	10.4	10.1	10.1
Nonwhite	6.8	8.8	9.7	9.5	9.5
Beneficiary place of residence					
Rural	6.9	9.2	10.2	9.9	9.8
Urban	7.0	9.5	10.3	10.1	10.0
Nephrologist care before dialysis					
0 to 6 months	N/A	N/A	10.4	10.2	10.1
6 to 12 months	N/A	N/A	10.2	9.9	9.8
12 or more months	N/A	N/A	9.8	9.5	9.5
None	N/A	N/A	10.1	9.9	9.8
Insurance					
MA	N/A	N/A	10.5	10.2	10.2
Dual eligible (Medicare and Medicaid)	7.2	9.8	10.7	10.3	10.3
VA	6.7	9.1	9.6	9.3	9.6
Medicare	7.2	9.7	10.6	10.3	10.3
EGHI or other coverage	6.6	8.6	9.5	9.2	9.2
Medicaid only	6.8	9.1	10.0	9.6	9.6
None	5.9	7.9	8.6	8.3	8.2
Inability to ambulate or transfer					
No	6.8	9.2	10.0	9.7	9.7
Yes	8.2	11.0	12.3	11.9	11.8

Note: eGFR (estimated glomerular filtration), N/A (not available), MA (Medicare Advantage), VA (Department of Veterans Affairs), EGHI (employer group health insurance), CHF (congestive heart failure). Lower values of eGFR suggest less residual kidney function. This analysis includes dialysis patients 18 years of age and older who initiated dialysis in 1996, 2004, 2012, 2015, or 2016. We assigned patients to seven mutually exclusive insurance categories (reported at dialysis initiation) according to the following hierarchy: (1) MA, (2) dually eligible for Medicare and Medicaid, (3) VA, (4) Medicare with or without EGHI, (5) EGHI with or without other coverage; (6) Medicaid only, and (7) none. The presence of comorbid conditions (cerebrovascular disease, CHF, diabetes) includes conditions present at the dialysis initiation or during the 10 years before treatment. "Facility type" refers to the facility at which the patient received dialysis at treatment initiation. "Facility capacity" was measured by assessing the total number of Medicare treatments furnished in the given year; small facilities furnished fewer than 6,500 treatments, while larger facilities furnished 6,500 treatments or more.

Source: MedPAC analysis of Medicare Form 2728 and claims submitted to CMS.

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TABLE 10–8

Mean levels of residual kidney function by patients' characteristics and site of care, 1996–2016 (cont.)

	Mean eGFR				
	1996	2004	2012	2015	2016
Institutionalized					
No	N/A	N/A	10.0	9.7	9.7
Yes	N/A	N/A	12.2	11.9	11.8
Needs help with daily activities					
No	N/A	N/A	10.0	9.7	9.7
Yes	N/A	N/A	11.6	11.3	11.2
Cerebrovascular disease					
No	6.8	9.2	10.1	9.8	9.8
Yes	7.5	9.9	10.7	10.4	10.4
CHF					
No	6.4	8.7	9.6	9.3	9.3
Yes	7.8	10.4	11.6	11.3	11.3
Diabetes					
No	6.3	8.6	9.7	9.3	9.3
Yes	7.6	10.0	10.6	10.3	10.3
Facility type					
Freestanding	7.0	9.3	10.2	9.9	9.9
Hospital based	6.7	8.9	10.2	9.9	9.9
Facility capacity					
Small	N/A	9.2	10.0	9.7	9.7
Large	N/A	9.6	10.6	10.2	10.2

Note: eGFR (estimated glomerular filtration), N/A (not available), MA (Medicare Advantage), VA (Department of Veterans Affairs), EGHI (employer group health insurance), CHF (congestive heart failure). Lower values of eGFR suggest less residual kidney function. This analysis includes dialysis patients 18 years of age and older who initiated dialysis in 1996, 2004, 2012, 2015, or 2016. We assigned patients to seven mutually exclusive insurance categories (reported at dialysis initiation) according to the following hierarchy: (1) MA, (2) dually eligible for Medicare and Medicaid, (3) VA, (4) Medicare with or without EGHI, (5) EGHI with or without other coverage; (6) Medicaid only, and (7) none. The presence of comorbid conditions (cerebrovascular disease, CHF, diabetes) includes conditions present at the dialysis initiation or during the 10 years before treatment. "Facility type" refers to the facility at which the patient received dialysis at treatment initiation. "Facility capacity" was measured by assessing the total number of Medicare treatments furnished in the given year; small facilities furnished fewer than 6,500 treatments, while larger facilities furnished 6,500 treatments or more.

Source: MedPAC analysis of Medicare Form 2728 and claims submitted to CMS.

cancer, although the strength of evidence was low for these conditions.[30] For example, there was only one RCT comparing proton beam therapy for prostate cancer with an alternative radiation treatment, which found that most patient outcomes for the two treatments were similar. ICER determined that the evidence base for other conditions (including breast and gastrointestinal cancer) was insufficient to determine the net health benefit.

Under a contract with the Agency for Healthcare Research and Quality (AHRQ), the ECRI Institute–Penn Medicine Evidence-based Practice Center reviewed evidence of various treatments for clinically localized prostate cancer, including proton beam therapy (Sun et al. 2014). The report found that the evidence for most treatment comparisons is inadequate to determine the comparative risks and benefits of treatments for prostate cancer.

TABLE 10–9

Outcomes of a randomized controlled trial comparing early and late initiation of dialysis

	Late initiation of dialysis	Early initiation of dialysis
Mean eGFR at dialysis initiation (mL/min/1.73 m^2)	9.8	12.0
All-cause mortality (number of events per 100 patient-years)	9.8	10.2
Cardiovascular events (number of events per 100 patient-years)	8.8	10.9
Infectious events (number of events per 100 patient-years)	14.3	12.4
Quality of life (quality-adjusted life-years)	2.1	2.0
Dialysis cost (per patient)	$96,763	$117,163

Note: eGFR (estimated glomerular filtration rate). The Initiating Dialysis Early and Late (IDEAL) study randomized patients to one of two groups: planned early dialysis initiation or planned late dialysis initiation. The last two outcomes (quality of life, dialysis cost per patient) came from Harris and colleagues (2011) and used a slightly smaller group of patients from the IDEAL cohort than were used for the analysis in the first four outcomes, which came from Cooper and colleagues (2010). The cost of dialysis per patient is the only category that significantly differed between the two groups.

Sources: Cooper et al. 2010, Harris et al. 2011.

The report called for more RCTs and better designed observational studies to evaluate the alternative therapies.

Although it is expensive to construct a proton beam facility, the expansion of proton beam therapy to more common cancers has spurred substantial growth in the number of these facilities. A large facility with multiple treatment rooms typically costs between $150 million and $200 million (Ollendorf et al. 2014). However, a new, compact proton system with one treatment room costs between $25 million and $30 million (Beck 2015). As of 2009, there were only six proton beam facilities in the United States. Since then, 21 facilities have opened, 10 facilities are under construction, and 4 facilities are in the planning stage (Particle Therapy Co-Operative Group 2018).[31]

Medicare's payment rates are higher for proton beam therapy than for other types of radiation therapy

Medicare's payment rates are substantially higher for proton beam therapy than for other types of external beam radiation therapy, such as intensity-modulated radiation therapy (IMRT). IMRT uses thin beams of radiation that are aimed at the tumor from many angles, which reduces the damage to healthy tissue near the tumor. Both proton beam therapy and IMRT receive a separate payment for each session of treatment, although treatment for most cancers involves many sessions over multiple weeks. For example, proton beam therapy for prostate cancer involves seven to nine weeks of daily treatment (Yu et al. 2013). When radiation therapy is delivered in a hospital outpatient department, it is paid under the hospital outpatient prospective payment system (OPPS). In 2016, the national OPPS rate for the most common proton beam therapy Healthcare Common Procedure Coding System (HCPCS) codes was $1,151 per treatment session, compared with $506 for IMRT.[32] When radiation therapy is delivered in a freestanding facility, it is paid under Medicare's fee schedule for physicians and other health professionals, commonly called the fee schedule. CMS sets national payment rates for most fee schedule services. Services that do not have a national payment rate, such as proton beam therapy, receive payment amounts that are determined separately by each MAC (these are called carrier-priced codes). Because there is no national payment rate for proton beam therapy under the fee schedule, we used claims data to calculate the mean and median payment amount per treatment session for proton beam therapy services in 2016. The mean payment was $988, and the median payment was $1,010. By comparison, the national payment rate for IMRT under the fee schedule in 2016 ranged from $346 to $348, depending on the code.

According to a study by Yu and colleagues, the median amount paid by Medicare for a course of radiation therapy

Summary of factors influencing the timing of dialysis initiation

While the optimal timing for dialysis initiation is still unknown, we conducted a literature review to better understand the factors that influence the decision to initiate. Most often, the timing is decided by the nephrologist of end-stage renal disease (ESRD) patients and is based on clinical judgment that is guided by values of residual kidney function and the patient's signs and symptoms of kidney failure (e.g., fluid overload, fatigue), including those related to comorbidity (Li et al. 2017, Rosansky et al. 2009).[33] Clinical guidelines also impact dialysis timing, and some practitioners have based dialysis initiation on estimated glomerular filtration rate (eGFR) values specified in earlier clinical guidelines (O'Hare et al. 2011, Robinson et al. 2014). In addition to the level of residual kidney function and clinical guidelines, patient-level and provider-level factors can also impact the decision to begin dialysis, including:

- patients' clinical characteristics,
- patients' demographics,
- nephrologists' training and experience,
- the availability of nephrology care before dialysis initiation, and
- potential financial motivation of dialysis providers and nephrologists.

Clinical guidelines

Clinical guidelines have played an influential role in the timing of dialysis initiation over the past two decades. In 1997, the National Kidney Foundation (NKF) Kidney Disease Outcomes Quality Initiative (KDOQI) released its first set of guidelines regarding the treatment of chronic kidney disease (CKD), which recommended that dialysis be initiated when the eGFR fell below 10.5 mL/min/1.73 m^2 (National Kidney Foundation 1997).[34] The 1997 NKF KDOQI guidelines were based on a literature review, which included the Canada–USA Peritoneal Dialysis Study Group (CANUSA) study, an observational study recommending a potential survival benefit for patients who began dialysis between 9 mL/min/1.73 m^2 and 14 mL/min/1.73 m^2 (CANUSA 1996, Lin and Zuo 2015). After the release of the NKF guidelines, other nephrology groups followed suit and began specifying levels of kidney function at which time dialysis should begin (or specific levels of function at which time providers should closely monitor patients). In 2006, NKF revised its guidelines and recommended that once patients reached 15 mL/min/1.73 m^2, "nephrologists should evaluate the benefits, risks, and disadvantages of beginning kidney replacement therapy" (National Kidney Foundation 2006). The revised guidelines were a product of additional studies establishing a link between level of residual kidney function at dialysis initiation and improved nutrition and survival (Rosansky et al. 2011, Shemin et al. 2001, Suda et al. 2000, Termorshuizen et al. 2004). According to researchers, the increased focus that guidelines placed on eGFR values likely contributed to the corresponding rise in eGFR at dialysis initiation (Lin and Zuo 2015, O'Hare et al. 2011).

As more recent literature has indicated that dialysis initiation should not be initiated solely based on calculated kidney function, the content of clinical guidelines has shifted (Rivara and Mehrotra 2017). Multiple national and international nephrology and CKD-focused groups have published updated guidelines regarding initiation of renal replacement therapy, many of which no longer advocate for specific levels of eGFR at which to begin dialysis (e.g., NKF KDOQI 2015 guidelines, the United Kingdom Renal

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for prostate cancer was $32,428 for proton beam therapy patients and $18,575 for IMRT patients (Yu et al. 2013). This estimate used claims data from 2008 and 2009 and included all payments for radiation treatment and treatment planning during a three-month period after the start of treatment.[35]

Rapid growth in spending for and volume of proton beam therapy in Medicare Spending for and volume of proton beam therapy in FFS Medicare grew rapidly from 2010 to 2016 (Figure 10-5, p. 330). Key drivers of this growth include the rapid increase in the number of proton beam centers since 2009 and Medicare's relatively broad

Summary of factors influencing the timing of dialysis initiation (cont.)

Association 2013 guidelines). While the guidelines differ in a few areas, threads of similarity run between the newest versions. Specifically, many include an increased focus on individualized initiation of renal replacement therapy based on patient signs and symptoms of renal failure, while simultaneously moving away from basing initiation solely on calculated levels of kidney function (i.e., eGFR). Shared decision-making between providers and patients has also received increased focus (e.g., the 2010 Renal Physicians Association's "Shared Decision-Making in the Appropriate Initiation of and Withdrawal from Dialysis" guidelines), as has a trend toward safely delaying dialysis when possible.

Patients' clinical characteristics

As guidelines from nephrology groups have shifted away from focusing predominantly on eGFR levels, the emphasis has been on initiating dialysis based on patient-specific signs and symptoms indicating kidney failure and comorbidities. According to recent guidelines, signs and symptoms that could indicate kidney failure and trigger initiation include volume overload and evidence of uremia (e.g., nausea or vomiting, fatigue). Certain comorbidities (e.g., diabetes) have also been noted in the clinical guidelines as a factor to consider when beginning dialysis. Few studies have systematically evaluated the full breadth of signs and symptoms and comorbidities present at dialysis initiation, but the available literature indicates that individuals with certain comorbidities (e.g., diabetes, congestive heart failure) tend to begin dialysis earlier (Lin and Zuo 2015, O'Hare et al. 2011). Another study indicated wide variation in the signs and symptoms reported at the time of dialysis initiation, with patients beginning dialysis with an average of five different signs and symptoms of kidney failure (Rivara and Mehrotra 2017).[36] According to the literature, this wide variation in signs and symptoms present in patients beginning dialysis—in addition to a lack of understanding regarding the optimal timing of dialysis—has contributed to the trend of individualized approaches for dialysis initiation.

Patients' demographic characteristics

Research indicates that demographic characteristics, including gender and age, may also influence the timing of dialysis initiation (Kausz et al. 2000, Li et al. 2017). Specifically, individuals who are older or male tend to start dialysis earlier than individuals who are younger or female, regardless of clinical severity (Lassalle et al. 2010, Li et al. 2017, O'Hare et al. 2011, Wilson et al. 2007).

Employment and insurance level have also been linked to dialysis start, with individuals who are insured and unemployed starting dialysis at higher levels of kidney function (Kausz et al. 2000, Li et al. 2017). Race can also impact dialysis timing, although these findings are mixed (Li et al. 2017, Streja et al. 2013). Some data also indicate that geography can impact when patients begin dialysis; according to the United States Renal Data System data from 2017, patients living in hospital service areas in the North and Midwest began dialysis at higher eGFRs than individuals living elsewhere (United States Renal Data System 2017). One study reported that decline in eGFR before dialysis initiation occurred more rapidly in younger versus older patients, in African American patients, and in patients with diabetes, but otherwise was similar across patient subgroups (O'Hare et al. 2011).

Nephrologists' training and experience

Nephrologist characteristics have also been linked to the timing of dialysis initiation. For instance, one study found that nephrologists who were less experienced (defined as zero to eight years of experience) or foreign medical graduates were more likely to begin patients on dialysis earlier (Slinin et al. 2014). According to another study, the number of nephrology providers available in a given state does not impact the timing of dialysis initiation (i.e., a greater number of nephrology providers does not lead to more or earlier dialysis initiations) (Ku et al. 2015). One study found that, while patient-level factors accounted for more of the variation in patients' eGFR at dialysis initiation, provider-level factors still affected when a patient began dialysis (Li et al. 2017). Understanding provider

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Summary of factors influencing the timing of dialysis initiation (cont.)

characteristics that might impact dialysis initiation is important, especially because providers continue to be predominantly responsible for making the final decision regarding when dialysis will begin (Wong et al. 2016).

The availability of nephrology care before dialysis initiation

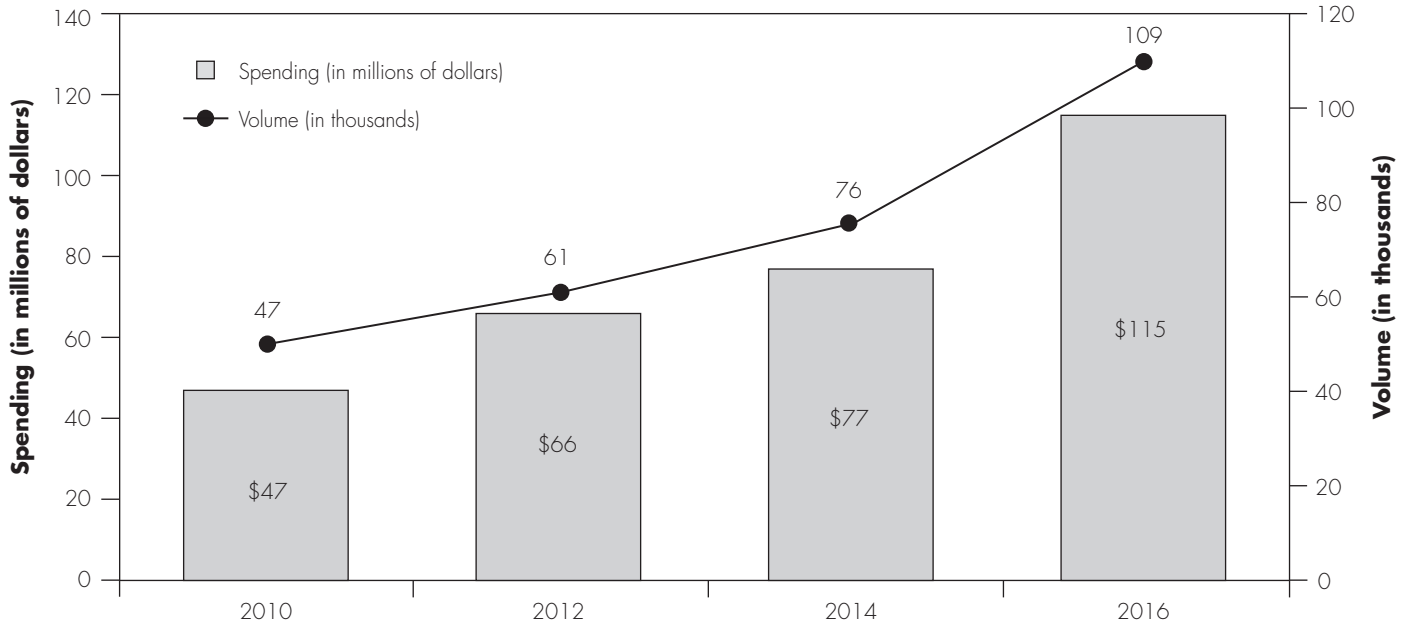
The care a patient receives before renal replacement therapy can impact the timing of dialysis initiation, although the research is mixed as to how timing is affected. While it is believed that patients should be under the care of a nephrologist before beginning dialysis to prevent "crashing" onto dialysis (i.e., an unplanned dialysis start), some research indicates that prior nephrology care can lead to earlier dialysis initiation (Li et al. 2017, Slinin et al. 2014). This literature is mixed, however, with other studies finding that individuals with predialysis nephrology care have lower eGFRs at dialysis initiation than those without predialysis nephrology care (Nee et al. 2017, Slinin et al. 2014). The data also suggest, though, that while predialysis care from a nephrologist might lead to earlier initiation, this relationship decreases the longer a patient receives care from a provider; specifically, individuals who receive care for a year or more before dialysis initiation have lower rates of early initiation (comparable with individuals with no nephrology care) than those who had less than a year of prior care (Slinin et al. 2014).[37] Additionally, individuals who have obtained permanent access (i.e., those who have undergone surgery to receive an arteriovenous graft or fistula) have been found to start dialysis earlier than those who have not obtained permanent access (Slinin et al. 2014, Wong et al. 2016).

Potential financial motivation of dialysis providers and nephrologists

Some researchers speculate that dialysis facilities and nephrologists might have a financial incentive to encourage earlier dialysis use (Slinin and Ishani 2014). For example, nephrologists could benefit from initiating dialysis earlier directly through higher physician fees or co-ownership of dialysis facilities or, less directly, through medical directorships of dialysis facilities or greater convenience and efficiency—that is, by being able to see more patients while rounding in the same dialysis unit (Ramanathan and Winkelmayer 2015). There is a paucity of research in this area, even as some have called attention to how most research on dialysis initiation ignores potential financial motivations (Senekjian 2011). In response, a few recent studies have begun to examine financial motivation with respect to dialysis. One study compared dialysis initiation for veterans who began dialysis in a Department of Veterans Affairs (VA) setting versus a setting outside the VA, and veterans who had their dialysis paid for by the VA versus those who did not (Yu et al. 2015). Differences by setting and payer emerged for the timing of dialysis initiation, with veterans whose dialysis was paid for by the VA—where physicians are salaried and do not handle insurance billing—and administered in VA clinics having the lowest eGFR at dialysis initiation. These findings indicate that the type of health system in which dialysis is begun could impact earlier versus later initiation. This study also found that the differences between groups became more pronounced over the decade-long study period. Additionally, average eGFR at initiation did increase throughout the study period for the entire VA population, indicating that financial incentives may not have been the only factor driving the increase in earlier initiation.

Other studies have argued against financial incentives contributing to differences in eGFR at dialysis initiation. One group examined the difference between for-profit dialysis facilities and nonprofit facilities, expecting that for-profit facilities might have an incentive to start patients early. They found, however, that eGFR at dialysis initiation was fairly similar between the two types of facilities (Rosansky et al. 2009). Additionally, it is unknown whether nephrologist ownership of facilities influences the timing of dialysis initiation, largely because of a lack of available information regarding physician ownership of facilities (Medicare Payment Advisory Commission 2009). In general, research examining financial incentives for beginning dialysis is still in the early phase and has not yet provided conclusive evidence indicating that financial motivation affects the timing of dialysis initiation. ■

FIGURE 10–5 **Spending and volume for proton beam therapy in Medicare grew rapidly, 2010–2016**



Source: MedPAC analysis of claims data for 100 percent of Medicare beneficiaries.

coverage of this treatment. During this period, spending rose from $47 million to $115 million (cumulative growth of 144 percent). Spending growth was driven by a 130 percent cumulative increase in volume, as measured by the number of treatment sessions, which increased from 47,420 to 108,960. The number of beneficiaries who received proton beam therapy during this period rose from 1,553 to 3,951 (cumulative growth of 154 percent) (data not shown). The share of volume provided in freestanding centers (vs. hospital outpatient departments) increased from 61 percent to 71 percent (data not shown). Prostate cancer was by far the most common condition treated by proton beam therapy, accounting for 44 percent of total spending in 2016 and 46 percent of total volume. About 1,500 beneficiaries with prostate cancer were treated with proton beam therapy in 2016 (comprising 38 percent of the beneficiaries who received this treatment).

Coverage of proton beam therapy by Medicare and other payers There is no national coverage determination for proton beam therapy in Medicare, but four MACs have LCDs for this treatment. MACs that do not have LCDs for proton beam therapy cover it as long as it is reasonable and necessary. Three MACs—Cahaba Government Benefit Administrators, CGS Administrators, and First Coast Service Options—have similar LCDs that divide indications for proton beam therapy into two groups and place conditions on coverage for indications in the second group (Centers for Medicare & Medicaid Services 2016b, Centers for Medicare & Medicaid Services 2015a, Centers for Medicare & Medicaid Services 2015c).[38] Under Cahaba's LCD, for example, Group 1 includes conditions for which proton beam therapy is considered medically reasonable and necessary, such as certain tumors of the central nervous system, tumors located at the base of the skull, and intraocular melanomas (Centers for Medicare & Medicaid Services 2015c). Group 2 includes conditions for which proton beam therapy is still under investigation, such as certain lung cancers, breast tumors, liver tumors, and nonmetastatic prostate cancer. Proton beam therapy is covered for these conditions when the intent of treatment is curative (for primary lesions) or life expectancy is greater than two years (for metastatic disease). In addition, the patient must be enrolled in a clinical trial or enrolled

in a national or regional clinical registry.[39] Conditions that are not listed for Group 1 or Group 2 are not covered. A fourth MAC—National Government Services—also has an LCD that divides indications for proton beam therapy into two groups but does not require that patients treated for conditions in Group 2 be enrolled in a clinical trial or registry or treated in a protocol designed for evidence development (Centers for Medicare & Medicaid Services 2015b).[40]

In 2006, the Medicare carrier for Virginia (TrailBlazer Health Enterprises) proposed an LCD that contained a least costly alternative (LCA) policy for proton beam therapy that would have paid for this treatment at the same payment rate as IMRT for some conditions (including prostate cancer) and the same rate as conventional radiation for other conditions (TrailBlazer Health Enterprises 2006).[41] Under an LCA policy, comparative clinical effectiveness evidence is used to determine the payment of alternative treatment options (assigned to separate billing codes) based on the rate of the lowest cost service. TrailBlazer did not implement the LCA.

Unlike Medicare's relatively broad coverage of proton beam therapy, Washington State has more limited coverage of this treatment for state government health insurance programs. The state covers proton beam therapy for ocular cancers, pediatric cancers, and central nervous system tumors, but covers it for other nonmetastatic cancers only at the state agency's discretion and only if the patient has had prior radiation in the expected treatment field with contraindication to all other forms of therapy (Washington State Health Care Authority 2014). Washington State has a unique health technology assessment program to determine which services will be covered for state employees, FFS Medicaid beneficiaries, and workers-compensation claimants. An independent clinical committee of health care practitioners—the Health Technology Clinical Committee (HTCC)—reviews evidence-based reports about whether certain medical devices, procedures, and tests are safe and effective to determine whether the state should pay for the technology. The HTCC bases its decisions on the safety, effectiveness, and cost-effectiveness of the technology. The state used this process to determine coverage for proton beam therapy.

Two national commercial insurers (Aetna and Anthem) cover proton beam therapy for certain conditions but not prostate cancer. Aetna considers it medically necessary for chordomas or chondrosarcomas at the base of the skull or cervical spine, cancer in children, and uveal melanomas (cancers of the eye) (Aetna 2014). The insurer considers it not medically necessary for localized prostate cancer because it has not been proven to be more effective than other types of radiation. Aetna considers it experimental and investigational for all other indications. Anthem covers proton beam therapy for the same conditions as Aetna, plus a few others (e.g., central nervous system lesions) (Anthem 2018). Anthem considers it investigational and not medically necessary for all other indications, including localized prostate cancer.

Case study 3: H.P. Acthar Gel®

H.P. Acthar Gel (Acthar) is an older, Part D-covered drug that has experienced rapid growth in prices and Medicare spending over the last several years, despite weak evidence that it is effective for adult indications. Between 2001 and 2017, the average price per vial increased from $748 to $38,000. Between 2011 and 2015, Medicare spending for Acthar increased from $49 million to $504 million. Fewer than 2,000 clinicians prescribed Acthar to beneficiaries in 2015, and 71 percent of them received at least one nonresearch payment from the manufacturer of Acthar related to the drug. Two-thirds of the total payments were compensation for services other than consulting, such as promotional speaking fees. These financial relationships raise questions about conflicts of interest among prescribers of Acthar.

Acthar is an injectable biologic that was approved by the FDA in 1952 and is indicated for the treatment of infantile spasms in children and eight other immunologic diseases or conditions, such as exacerbations of multiple sclerosis (MS) in adults (Food and Drug Administration 2015, Shakil and Redberg 2017).[42] When the drug was approved, the FDA did not require clinical trials to demonstrate its effectiveness (Morgenson 2014).

The evidence that Acthar is effective for adult conditions is weak (Shakil and Redberg 2017). Most of the studies of Acthar for adult conditions are small, retrospective or prospective observational studies that do not compare Acthar with other drugs or placebo. Two small, prospective randomized trials from the 1980s compared Acthar with intravenous methylprednisolone (a cheaper drug) for patients with acute relapse of MS (Barnes et al. 1985, Thompson et al. 1989). Both studies found that, three months after treatment started, both drugs produced comparable clinical benefits. A randomized trial conducted in the 1960s used several clinical measures

TABLE 10–10 **Medicare Part D spending and volume for H.P. Acthar Gel® grew rapidly, 2011–2015**

	2011	2012	2013	2014	2015	Percent change, 2011–2015
Gross spending (millions)	$49	$141	$263	$391	$504	919%
Number of prescriptions	1,471	3,387	6,752	9,611	11,209	662
Spending per prescription	$33,621	$41,763	$38,889	$40,702	$44,964	34
Number of beneficiaries who filled a prescription	853	1,583	2,431	2,932	3,104	264
Spending per beneficiary	$57,980	$89,357	$108,014	$133,421	$162,371	180
Number of prescriptions per beneficiary	1.7	2.1	2.8	3.3	3.6	109

Note: Gross spending does not reflect manufacturers' rebates.

Source: MedPAC analysis of Medicare drug spending data from CMS.

to compare Acthar with a placebo for patients with an acute exacerbation of MS (Rose et al. 1970). Four weeks after treatment began, patients who received Acthar were statistically more likely to improve than patients who received placebo according to some measures but not others.[43] However, the differences between Acthar and placebo were generally modest, and the study had a relatively short observation period.

Even though Acthar has been on the market since 1952, its price has increased rapidly since 2001, when the drug was acquired by Questcor (Shakil and Redberg 2017). Between 2001 and 2014, the average price per vial increased from $748 to $34,034 (Robinson 2017). In 2014, Acthar was acquired by Mallinckrodt, which raised the price per vial in 2017 to $38,000 (Lopez 2017).

The manufacturers of Acthar have been able to sustain a high price for the drug in part because there is no generic version. Although Acthar's patent has expired, it received orphan drug status from the FDA in 2010 for treatment of infantile spasms. Orphan drug status conveyed market exclusivity (sole marketing rights) to the manufacturer for seven years, which ended in October 2017. In 2013, Questcor acquired the U.S. rights to a synthetic version of Acthar called Synacthen Depot. The Federal Trade Commission filed a complaint alleging that Questcor acquired the competing drug to prevent another company from purchasing it and selling it in the United States, which enabled Questcor to preserve its monopoly over Acthar and maintain very high prices (Federal Trade Commission 2017b). Mallinckrodt, which had purchased the rights to Acthar, settled the charges in 2017 and agreed to license the rights to develop and market Synacthen Depot in the United States to another company (Federal Trade Commission 2017a). However, Synacthen Depot is not yet on the market. A separate manufacturer (ANI Pharmaceuticals) is also developing a generic competitor to Acthar that is not yet on the market (PRNewswire 2018).

Between 2011 and 2015, Medicare spending for Acthar under Part D increased from $49 million to $504 million (cumulative growth of 919 percent), driven by 264 percent growth in the number of beneficiaries who received the drug and 180 percent growth in spending per beneficiary (Table 10-10).[44] Although a very small number of beneficiaries receive Acthar, spending per beneficiary is remarkably high. From 2011 to 2015, the number of beneficiaries prescribed the drug rose from 853 to 3,104, while spending per beneficiary increased from almost $58,000 to over $162,000. At the same time, the average number of prescriptions per beneficiary grew from 1.7 to 3.6, and spending per prescription rose from almost

TABLE 10–11

Payments by manufacturer of H.P. Acthar Gel® to physicians who prescribed it to Medicare beneficiaries, by payment category, 2015

	Payments		Physicians		Payments per physician	
	Amount (in thousands)	Share of total	Number*	Share of all physicians who received a payment**	Mean	Median
Compensation for services other than consulting	$3,295	67%	211	17%	$15,617	$9,950
Travel and lodging	869	18	207	17	4,198	1,846
Consulting fee	470	10	162	13	2,901	2,700
Food and beverage	267	5	1,233	100	217	120
Education	7	<1	220	18	31	6
Total	**4,908**	**100**	**1,235**		**3,974**	**127**

Note: Table excludes research payments and ownership interests. "Compensation for services other than consulting" includes payments for speaking, training, and educational engagements that are not related to continuing education.
*There were 1,235 unique physicians who received at least one payment from the manufacturer. This column does not sum to 1,235 because a physician could have received payments in multiple categories.
**This column indicates the share of physicians who received a payment in each category from the manufacturer. Because a single physician could have received payments in multiple categories, this column does not sum to 100 percent.

Source: MedPAC analysis of Medicare Part D prescription drug event data from CMS and Open Payments data (general payments file) from CMS.

$34,000 to almost $45,000.[45] Based on our analysis of Medicare Part D prescription drug event data, 1,743 clinicians prescribed Acthar in 2015 (data not shown). The top decile of Acthar prescribers accounted for 41 percent of total Acthar prescriptions and 40 percent of total spending. In Medicare Part D, the most frequent prescribers of Acthar are rheumatologists, neurologists, and nephrologists (Hartung et al. 2017).

In 2017, most Part D plans did not cover Acthar, and those that covered it used utilization management tools to control its use. Less than 6 percent of stand-alone prescription drug plans and about one-quarter of Medicare Advantage–Prescription Drug plans included Acthar on their formularies (these figures are not weighted by the number of enrollees in each plan). All of the plans that listed Acthar on their formularies required prior authorization for it, and a small number of these plans also had quantity limits. We do not have information on whether Acthar was included in formularies or subject to prior authorization in previous years.

We linked Medicare data from 2015 on Acthar prescribers to data from 2015 on payments to physicians from drug and device manufacturers, which we obtained from CMS's Open Payments system. Under Open Payments, manufacturers report to CMS information about certain payments and other transfers of value to physicians and teaching hospitals (Centers for Medicare & Medicaid Services 2017a, Medicare Payment Advisory Commission 2017). We found that 71 percent of clinicians (1,235) who prescribed Acthar to Medicare beneficiaries in 2015 received at least one nonresearch payment from the manufacturer related to the drug. The collective value of these payments was $4.9 million. On average, each physician received $3,974 in payments (median of $127). Of the total payments for Acthar, 44 percent were received by neurosurgeons, 25 percent by rheumatologists, 14 percent by nephrologists, and 11 percent by neurologists (data not shown).

Two-thirds of the total payment amount was compensation for services other than consulting, 18 percent was for travel and lodging, 10 percent was for consulting fees, and 5 percent was for food and beverage (Table 10-11). Compensation for services other than consulting includes payments for speaking, training, and educational engagements that are not related to continuing education

(e.g., a manufacturer pays a physician to talk about a drug to other physicians at a restaurant) (Centers for Medicare & Medicaid Services 2017c). About 200 physicians (17 percent of Acthar prescribers who received payments from the manufacturer) received compensation for services other than consulting, with each physician receiving $15,617, on average (median of $9,950). Almost all Acthar prescribers who received payments from the manufacturer received food and beverage; the average value per physician was $217 (median of $120).

We also examined manufacturer payments received by the top 10 percent and the bottom 10 percent of Acthar prescribers in 2015.[46] Eighty-six percent (149) of the highest prescribing physicians received at least one nonresearch payment related to Acthar, compared with 62 percent (108) of the lowest prescribing physicians. The top 10 percent of prescribers received a total of $1.8 million in payments with a per physician average of $11,759 (median of $286). By contrast, the bottom 10 percent of prescribers received a total of $270,000 in payments with a per physician average of $2,498 (median of $107).

The financial relationships between Acthar's manufacturer and physicians who prescribe it raise questions about potential conflicts between physicians' obligations to act in the best interest of their patients and the commercial interests of the manufacturer. Studies have shown that physicians' financial interactions with drug manufacturers are associated with greater willingness to prescribe more expensive drugs (Watkins et al. 2003, Wazana 2000). A recent study found that physicians who received meals related to the promotion of specific brand-name medications had a higher rate of prescribing those medications to Medicare beneficiaries (DeJong et al. 2016).

Tools for addressing low-value care

There are various tools available to payers to reduce the use of low-value services. The tools that are appropriate for a given service depend on the strength of the evidence for the service's value (including its comparative clinical effectiveness), the availability of clinical information to determine the service's value, and the service's cost. Administrative tools such as coverage determinations, prior authorization, and changes to beneficiary cost sharing may be appropriate when there is strong evidence that a service is low value for certain patients or settings, payers have access to clinical information to determine when the service is provided in a low-value circumstance, and the service is costly. For example, there is robust evidence that imaging for nonspecific low back pain does not improve patient outcomes, and MRI scans of the lower back region receive high Medicare payment rates (Chou et al. 2011). In addition, a payer could obtain information about a patient's diagnoses and symptoms to determine whether an imaging study for back pain is low value from claims or by requiring the provider to submit additional information (e.g., through an online system). For example, a diagnosis of cancer, trauma, or neurological impairment could indicate that the imaging study is not low value.

Another tool is an LCA policy, in which payers set a single payment rate for a group of clinically similar services based on the lowest cost item. This policy may be suitable for a service that is much more expensive than a comparable service but there is no evidence that the costlier service is clinically superior to the cheaper one. For example, Medicare pays higher rates for proton beam therapy than IMRT, but there is a lack of evidence that proton beam therapy offers a clinical advantage over IMRT for prostate cancer.

New payment models, such as models that hold providers accountable for the cost and quality of care, may be appropriate for services for which it is more difficult to distinguish low value from high value. For example, the timing of initiation of dialysis depends on a host of factors, such as the values of residual kidney function and the patient's clinical characteristics. One approach is to give providers clinical discretion on when to initiate dialysis if they participate in a model that holds them accountable for total spending and outcomes. Compared with administrative tools such as coverage policies that determine when dialysis may be initiated, this approach would give providers more discretion and may be easier to implement. In addition, payment systems in which providers take responsibility for spending and outcomes could be effective at reducing the use of multiple low-value services. In these models, providers have an incentive to reduce the use of services that do not improve quality or outcomes; have more access to clinical information for determining value than payers; and can decide which low-value services to target based on their prevalence, potential savings, and the cost of interventions to reduce the use of low-value care.

We describe six tools Medicare could consider employing to address the use of low-value care:

- requiring prior authorization for certain types of services
- implementing clinician decision support and provider education
- altering beneficiary cost sharing
- establishing new payment models that foster delivery system reform
- revisiting coverage determinations on an ongoing basis
- linking FFS coverage and payment to clinical comparative effectiveness and cost-effectiveness information

Prior authorization

CMS has adopted prior authorization to reduce the unnecessary use of certain types of durable medical equipment (DME) and other services. Under prior authorization, a provider must obtain approval from a plan or payer for a product or service before delivering it. CMS has tested prior authorization in a variety of demonstrations since 2012, one of which led to the establishment of a national prior authorization process for some types of DME. The Secretary's authority to conduct these demonstrations and implement a national process comes from a variety of sources, including amendments to the Social Security Act and the statutory authority of CMS's Center for Medicare & Medicaid Innovation (CMMI) to test innovative payment and delivery reform models.

In 2011, the Commission recommended that the Congress direct the Secretary to establish a prior authorization program for clinicians who order substantially more advanced diagnostic imaging services (MRI, CT, and nuclear medicine) than their peers (Medicare Payment Advisory Commission 2011). The goal of this approach was to ensure that clinicians who order more of these services than other clinicians use them appropriately. This recommendation has not been adopted.

Prior Authorization of Power Mobility Device Demonstration

On September 1, 2012, CMS launched the first of its prior authorization demonstrations, the Medicare Prior Authorization of Power Mobility Device Demonstration. The demonstration relies on the Secretary's authority to conduct demonstrations to investigate and prosecute fraud in the Medicare program, as laid out in Section 402(a)(1)(J) of the Social Security Amendments of 1967 (Centers for Medicare & Medicaid Services 2012). Originally applied to FFS beneficiaries in seven states, the demonstration established a prior authorization process for certain power mobility devices (PMDs) (i.e., power wheelchairs) for parts of the country especially prone to fraud and errors. In its first year (September 2012 to September 2013), the demonstration decreased monthly expenditures from $12 million to $3 million without impacting beneficiary access to medically necessary items (Centers for Medicare & Medicaid Services 2014e). The demonstration was later extended until August 31, 2018, and expanded to include a total of 19 states.[47] The PMD Demonstration also led to the development of two new prior authorization demonstrations: the Prior Authorization of Repetitive, Scheduled Non-emergent Ambulance Transport Model and the Prior Authorization Model for Non-Emergent Hyperbaric Oxygen (HBO) Therapy.

Since the PMD Demonstration, CMS has also established a national prior authorization process for certain DMEPOS products (Centers for Medicare & Medicaid Services 2014e). Under Section 1834 of the Social Security Act, CMS is authorized to develop and maintain a master list of DMEPOS products that are frequently used unnecessarily and to establish a prior authorization process for items on the list. As of July 2017, two power wheelchair products were subject to the national prior authorization process (Centers for Medicare & Medicaid Services 2015e).

Prior Authorization of Repetitive, Scheduled Non-Emergent Ambulance Transport Model

The Medicare Prior Authorization of Repetitive, Scheduled Non-Emergent Ambulance Transport (RSNAT) Model began on December 1, 2014. The model is a joint effort between CMMI and the Center for Program Integrity. It originally applied to transports occurring within three states, which were chosen because they had high incidences of improper payment for these services (Centers for Medicare & Medicaid Services 2014d, Medicare Payment Advisory Commission 2013). Under this model, a repetitive ambulance service is defined as "a medically necessary ambulance transportation that is furnished in 3 or more round trips during a 10-day period, or at least 1 round trip per week for at least 3 weeks" (Centers for Medicare & Medicaid Services 2015d). For the trip to be medically necessary, the beneficiary must be bed confined or medically required to be transported by ambulance; for example, a trip would not be covered if the beneficiary could be transported by another

method but another method is unavailable. A common example of a covered RSNAT would be a bed-confined beneficiary needing transport to a dialysis appointment. Because of promising early results, the demonstration was expanded through the Medicare Authorization and CHIP Reauthorization Act of 2015 to five additional states and the District of Columbia (DC), beginning in 2016 (Centers for Medicare & Medicaid Services 2015d).[48]

According to its first interim evaluation, the model reduced RSNAT service use and expenditures for ESRD beneficiaries across the 8 model states and DC in 2015 and 2016, with an estimated average reduction of 2.5 RSNAT trips and $432 in RSNAT expenditures per ESRD beneficiary per quarter (Asher et al. 2017).[49] In addition, our analysis shows a national decline from 2013 to 2016 in nonemergent ambulance trips to dialysis facilities for ESRD beneficiaries. Although prior authorization likely contributed to the decrease in payments and use of RSNAT services, in October 2013, CMS reduced payment rates by 10 percent for nonemergency basic life support trips to dialysis facilities for ESRD beneficiaries. This payment decrease, which was based, in part, on a previous Commission recommendation, may have contributed to the reported savings (Medicare Payment Advisory Commission 2013).[50]

Quality of care according to the evaluation was mixed, with quantitative analyses evaluating outcomes and access (e.g., mortality, dialysis services) showing little to no change because of the model. The evaluation found an increase in the number of emergency dialysis treatments, but there was not an increase in hospitalizations or emergency department utilization (Asher et al. 2017). Qualitative analysis (e.g., discussions with dialysis facilities, providers, and beneficiaries) suggests that the model may have resulted in some beneficiaries delaying or missing treatment. In the Commission's 2013 mandated report on ambulance services, we suggested that dialysis facilities should be allowed to provide transportation services to their patients by creating exceptions to the anti-kickback statute and the civil monetary penalty law prohibiting inducements to Medicare and Medicaid beneficiaries (Medicare Payment Advisory Commission 2013). Allowing facilities to transport patients to dialysis sessions would ensure that patients do not miss dialysis treatments because of a lack of transportation. Facilities would not be required to offer this service to their patients, and the cost of operating it would not be factored into the bundled payment for dialysis facilities. Later evaluations from CMS will continue to monitor the success of the demonstration. These evaluations should consider the impact of the 23 percent payment reduction beginning on October 1, 2018, for nonemergency ESRD ambulance transports mandated by the Bipartisan Budget Act of 2018.

Prior Authorization Model for Non-Emergent Hyperbaric Oxygen Therapy

The Prior Authorization Model for Non-Emergent Hyperbaric Oxygen Therapy began on March 1, 2015, and ended on March 1, 2018. Hyperbaric oxygen (HBO) therapy is a treatment that exposes the entire body to oxygen under increased atmospheric pressure and can be provided in an outpatient facility or hospital (Centers for Medicare & Medicaid Services 2016d). The model is a joint effort between CMMI and the Center for Program Integrity and applies to FFS beneficiaries who receive HBO therapy in a hospital outpatient facility, have one of five conditions (e.g., osteoradionecrosis), and reside in one of three model states.[51] According to preliminary data released by CMS, the model slightly decreased expenditures for nonemergent HBO therapy in model states by approximately $5.33 million over the first 13 months of the model (Centers for Medicare & Medicaid Services 2016c). A formal evaluation of the model is currently under way.

Clinician decision support and provider education

Another set of tools that Medicare could use to reduce the use of low-value care is clinician decision support (CDS) and provider education. According to the literature, interventions that include CDS and performance feedback have the potential to address low-value services, and provider education paired with other strategies also shows promise (Colla et al. 2017a). A related tool is shared decision-making, in which providers communicate information to patients about the outcomes, probabilities, and uncertainties of treatment options, and patients communicate their values and the relative importance they place on benefits and harms (Medicare Payment Advisory Commission 2010).

A 2009 study aimed at reducing inappropriate prescribing of fluoroquinolones, a commonly prescribed antibiotic in ambulatory care and emergency department visits, found that combining provider education with CDS could decrease prescribing of these antibiotics by 30 percent (Wong-Beringer et al. 2009). Additionally, the study showed improved patient outcomes. Another study

examined the impact of combining computerized reviews with clinician education on antibiotic use in a VA hospital (Feucht and Rice 2003). This effort reduced unnecessary intravenous antibiotic use by 26 percent and inappropriate prescriptions of more than five days by 16 percent.

A study by Meeker and colleagues analyzed the effects of behavioral interventions on inappropriate antibiotic prescriptions by primary care clinicians in Boston and Los Angeles (Meeker et al. 2016). Providers were randomly assigned to one of three interventions: suggested alternatives, accountable justification, and peer comparison. This study found that accountable justification (the clinician was prompted to enter free-text justifications for prescribing an antibiotic in the patient's electronic health record) and peer comparison (clinicians were sent emails that compared their antibiotic prescribing rate with those of other providers) were the most effective at lowering inappropriate antibiotic prescribing. Accountable justification decreased prescribing by 18.1 percent, while peer comparison decreased prescribing by 16.3 percent (Meeker et al. 2016).

CMS is developing the Appropriate Use Criteria (AUC) Program that will require clinicians to use CDS when ordering advanced diagnostic imaging services for Medicare beneficiaries (Centers for Medicare & Medicaid Services 2017b).[52] Under this program, clinicians who order these services will need to consult with CDS software and obtain feedback on whether the services adhere to AUC developed by medical societies or other provider-led entities. Clinicians will be required to use CDS software that is certified by CMS based on certain requirements. CMS is in the process of developing this program, which is scheduled to begin on January 1, 2020. However, a prior demonstration of this approach raises questions about its effectiveness. Under the Medicare Imaging Demonstration (2011 to 2013), physicians who ordered certain advanced imaging studies received feedback about the appropriateness of their orders through CDS software (Timbie et al. 2014).[53] An evaluation of this demonstration found that 65 percent of the orders could not be rated for appropriateness because they could not be linked to a clinical guideline used by the CDS systems. This result occurred because the information entered by physicians was not sufficiently precise to match a guideline or a guideline did not exist for the specific clinical scenario. CMS is using the experiences from this demonstration to develop the AUC Program (Centers for Medicare & Medicaid Services 2015f).

The goal of shared decision-making is to improve patients' knowledge of their condition and alternative treatments so they can arrive at treatment decisions with their clinicians that reflect their values and preferences (Medicare Payment Advisory Commission 2010). Information is often conveyed through patient decision aids that give patients evidence-based, objective information on treatment options for a given condition. Shared decision-making programs often focus on preference-sensitive care (i.e., care that depends on patient preferences when two or more options exist). Several low-value services are preference sensitive, such as cancer screening for older adults, imaging for nonspecific low back pain, spinal injection for low back pain, and arthroscopic surgery for knee osteoarthritis (Schwartz et al. 2015). By conveying evidence-based information to patients about the benefits and risks of treatment options, these programs could help reduce the use of low-value care. The American Cancer Society's recommendation for prostate cancer screening states that men should make an informed decision with their provider about whether to be screened after receiving information about the uncertainties, risks, and potential benefits of screening (American Cancer Society 2016). Studies of shared decision-making programs have found that they reduced invasive treatments without adverse effects on health outcomes (O'Connor et al. 2009, O'Connor et al. 2004).

Altering beneficiary cost sharing

Altering beneficiary cost sharing for certain services is another potential tool to address low-value care. Reducing cost sharing for high-value services should encourage consumers to seek these services. Conversely, increasing cost sharing for services that are deemed low value should discourage patients from obtaining these services. Among the Commission's recommended changes to the benefit design of FFS Medicare is that the Congress should give the Secretary authority to alter or eliminate cost sharing based on evidence of the value of services (Medicare Payment Advisory Commission 2012). Although CMS does not adjust cost sharing in FFS Medicare based on the clinical value of services, CMMI is testing a model that allows MA plans in several states to offer reduced cost sharing or additional benefits to enrollees with certain chronic conditions (Centers for Medicare & Medicaid Services 2018). However, this model does not allow plans to increase cost sharing for low-value services.

Outside of Medicare, some plans and payers adjust cost sharing for different services based on evidence

of their clinical benefits (Chernew et al. 2007). A 2016 study evaluated the impact of such an approach—called value-based insurance design (VBID)—by a large public employer in Oregon (Gruber et al. 2016). The program increased cost sharing for services that were deemed low value: sleep studies, endoscopies, advanced imaging, and surgery for low back pain. The analysis found that the VBID program significantly reduced utilization of the targeted services. However, further evaluations of these types of interventions are needed (Colla et al. 2017a).

New payment models that foster delivery system reform

Medicare could also use new payment models that encourage delivery system reform to reduce low-value care. Payment models that hold providers accountable for the cost and quality of care may create incentives for the efficient delivery of care, including decreased use of low-value services (Colla et al. 2017a).

One such model is the accountable care organization (ACO), in which a group of providers takes responsibility for the cost and quality of care for a group of patients. If an ACO is successful in controlling (or decreasing) costs while maintaining or increasing quality, it may be eligible to share savings with the plan or payer. ACOs that are at one-sided risk are eligible to share savings but are not at risk for losses, while ACOs at two-sided risk share in both savings and losses. One way for ACOs to constrain costs without reducing quality is to reduce the use of low-value services. Preliminary evidence indicates that ACOs at two-sided risk were able to significantly reduce low-value services during their first performance year, which suggests that strong financial incentives can motivate ACOs to target low-value care.

A study by Schwartz and colleagues analyzed the use of 31 low-value services during the first year of Medicare's Pioneer ACO demonstration, a two-sided-risk model (Schwartz et al. 2015). The researchers compared the change in the use of low-value care in the ACO model with the change in a control group, using the periods before and after the ACO contracts went into effect. The authors found a significant reduction in both volume and spending for low-value services in the ACO group relative to the control group. Another study examined the performance of ACOs in the Medicare Shared Savings Program (MSSP) in their first year of operation and found that these ACOs did not achieve significant reductions in the use of low-value services relative to the control group (McWilliams et al. 2016). At the time of the evaluation, all MSSP ACOs were at one-sided risk. Because these two studies examined only the first year of each ACO model and the models were different in ways other than their type of risk, the evidence is too limited to conclude that one-sided-risk ACOs are unable to reduce the use of low-value care.[54]

Under a payment model similar to ACOs, ESRD Seamless Care Organizations (ESCOs) take responsibility for cost and quality for a group of beneficiaries on dialysis. Large ESCOs are required to accept two-sided risk, but smaller ESCOs may choose either two-sided or one-sided risk.[55] The ESCO model decreased costs in the first year of operation: Most of the savings resulted from lower inpatient and post-acute care spending rather than significant reductions in unnecessary readmissions or emergency department use (Marrufo et al. 2017). Researchers have not evaluated the impact of the ESCO model on specific low-value services.

Revisiting coverage determinations on an ongoing basis

Revisiting NCDs on an ongoing basis has the potential to reduce low-value care. Even though the majority of determinations are established with "fair" or "poor" evidence, Medicare infrequently revisits its national coverage decisions (see Table 10-2, p. 302). Moreover, nearly all of the reconsiderations that Medicare opened over the past five years have been at the request of external parties (e.g., manufacturers, physicians, medical associations) and have resulted in expanding coverage for the service under consideration. Researchers have raised concerns about the lack of high-quality evidence needed when Medicare develops coverage determinations (Chambers et al. 2015b, Foote et al. 2004, Neumann et al. 2008, Redberg 2007).

In addition, there is concern that services shown to be of high value for the clinical conditions covered in an NCD might be furnished to beneficiaries who do not meet the NCD's clinical criteria (and thus result in low-value care). Huo and colleagues used the National Health Interview Survey to examine the age and smoking history of a sample of individuals who said they had undergone lung cancer screening with low-dose CT. These researchers found that individuals undergoing this screening may not meet the criterion for smoking history specified in Medicare's NCD for this service (Huo et al. 2017).[56]

Some policymakers contend that the Secretary could be more preemptive and establish criteria that would identify NCDs for reconsideration on an ongoing basis. The

criteria could consider the rigor of the clinical evidence that Medicare considered when establishing the NCD. For example, NCDs that were implemented with "fair" or "poor" clinical evidence or without comparative clinical effectiveness evidence could be revisited on an ongoing basis. Criteria could also consider the service's impact on the Medicare Trust Funds and the rate at which the service diffuses among the Medicare population. On an ongoing basis, the Secretary could assess whether the beneficiary population receiving a service covered under an NCD meets the clinical criteria specified in the NCD. Such an ongoing, preemptive process could ultimately lead to the development of more rigorous clinical evidence and decrease the use of low-value services.

Linking FFS coverage and payment to clinical comparative effectiveness and cost-effectiveness information

Comparative clinical effectiveness—which compares the clinical effectiveness of two or more treatment options for the same condition—serves as the foundation for cost-effectiveness analysis, which compares costs and clinical outcomes of two or more treatment alternatives. Linking information about the comparative clinical effectiveness and cost-effectiveness of health care services to FFS policies has the potential to improve value in Medicare spending.

Over the past decade, policymakers have recognized the importance of comparative clinical effectiveness evidence. In June 2007, we recommended that the Congress establish an independent entity to sponsor credible research on comparative effectiveness and disseminate this information to patients, providers, and payers (Medicare Payment Advisory Commission 2007). The Patient Protection and Affordable Care Act of 2010 (PPACA) established an independent nonprofit entity, the Patient-Centered Outcomes Research Institute (PCORI), to fund and disseminate comparative clinical effectiveness research.

Medicare considers comparative clinical effectiveness evidence in the coverage process when it is available, but such evidence is not required. The program generally does not consider comparative clinical effectiveness evidence in its rate-setting processes and lacks explicit statutory authority to consider a service's cost-effectiveness when making coverage decisions or setting payment rates. In addition, the use of cost-effectiveness analysis is constrained because PPACA prohibits the Secretary from using certain outcome measures that cost-effectiveness studies use—quality-adjusted life years or similar measures—to determine coverage or payment. Federal agencies and researchers have supported the use of comparative clinical effectiveness and cost-effectiveness information by Medicare. Some payers, including risk-bearing Medicare providers and purchasers, have the flexibility to use cost-effectiveness evidence for medical and pharmacy management. We examined case studies describing two organizations—PCORI and ICER—that generate information on the value of medical services that has the potential to improve value in Medicare spending.

FFS Medicare generally does not use comparative clinical effectiveness information in coverage and payment policies

Under the local and national coverage processes, a formal review of the medical, technical, and scientific evidence is conducted to evaluate the relevance, usefulness, and medical benefits of an item or service to Medicare beneficiaries. Medicare's coverage process has the flexibility to consider comparative clinical effectiveness evidence when such evidence is available. However, coverage is generally determined without any requirement for evidence demonstrating that the service in question is equally or more effective than other available, covered treatment options (Pearson and Bach 2010).

The statute includes several constraints in Medicare's use of comparative clinical effectiveness evidence. For example, Medicare cannot use comparative clinical effectiveness evidence that AHRQ produces under MMA's Section 1013 to withhold coverage of prescription drugs. Since 2010, PPACA imposes constraints on Medicare's use of comparative clinical effectiveness research conducted by PCORI when making coverage decisions and setting payment rates. When such evidence is available, the program:

- must use an iterative and transparent process (which includes public comment and consideration of the effect on subpopulations) in formulating coverage decisions;
- cannot use the evidence as the sole source of information to deny coverage;
- cannot use evidence in determining coverage, payment, or incentive programs that treats extending the life of an elderly, disabled, or terminally ill individual as of lower value than extending the life of an individual who is younger, nondisabled, or not terminally ill; and

- cannot use evidence in determining coverage, payment, or incentive programs in a manner that precludes or discourages an individual from choosing a treatment based on how the individual values the trade-off between extending the length of her life and the risk of disability.

Medicare's payment systems are determined by statutory provisions that generally do not consider a service's comparative clinical effectiveness. For example, the Part B fee schedule does not consider comparative clinical effectiveness evidence. Payment rates for new services are based on the relative costliness of the inputs used to provide the service: work, practice expenses, and professional liability insurance expenses. Consequently, a new service might be paid at a higher rate than clinically similar treatment options.

The payment rates under the outpatient and inpatient hospital prospective payment systems (PPSs) are generally based on the hospitals' reported charges converted to costs. However, under Medicare law, CMS considers clinical evidence to encourage the early adoption of cost-increasing, quality-improving technologies. For certain new technologies, the agency considers whether they provide a "substantial clinical improvement" compared with existing technologies to determine whether they qualify for temporary (two to three years) pass-through payments under the outpatient hospital PPS and add-on payments under the inpatient hospital PPS. To qualify for the additional payment, new devices in the outpatient setting and new services and technologies in the inpatient setting must meet a cost threshold and must demonstrate that they provide a substantial clinical improvement compared with treatment alternatives.

Before 2010, CMS linked available comparative clinical effectiveness information in the rate-setting process for certain items and services not covered under a PPS. Referred to as the LCA and functional equivalence policies, Medicare set a single payment rate for a group of clinically similar Part B drugs assigned to separate payment codes based on the lowest cost item. For example:

- Under the LCA policy, Medicare used the prevailing payment policy (which, in accordance with the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA), has been the average sales price plus 6 percent since 2005) to determine the payment rate for luteinizing hormone-releasing hormone (LHRH) agonists for prostate cancer (assigned to separate billing codes) in a drug class and then set the payment rate for all the clinically comparable drugs (in that class) based on the least costly one. LCA policies were implemented in LCDs in which the MACs decided to cover a particular product in its geographic jurisdiction. As a result of two federal court rulings, Medicare has not used LCA policies since 2010.[57]
- Medicare applied an LCA-type policy—referred to as the functional equivalence policy—on the national level to set the payment rate for anti-anemia drugs paid for under the outpatient hospital PPS. Medicare used the functional equivalence standard in 2004 and 2005. After the enactment of the MMA, the payment rate for each biologic was set based on 106 percent of its average sales price beginning in 2006. In addition, the MMA prohibited the use of the functional equivalence standard for drugs and biologics in the hospital outpatient setting.

The policies' rationale is that beneficiaries, Medicare, and taxpayers should not pay more for a service when a similar service can be used to treat the same condition and produce the same outcome but at a lower cost.

Other federal agencies have estimated that expanded use of LCA policies would result in savings for beneficiaries and taxpayers. The Congressional Budget Office (CBO) included the use of LCA for Part B drugs in its 2008 budget options related to health care (Congressional Budget Office 2008). The Office of Inspector General (OIG) has twice recommended that the Secretary apply LCA policies to LHRH agonists (Office of Inspector General 2004). Most recently, OIG, in a 2012 report, recommended that CMS seek legislative authority to implement LCA policies for "certain clinically comparable products under circumstances it deems appropriate" (Office of Inspector General 2012). In this report, OIG determined that if LCA policies for the LHRH agonists had not been rescinded, Medicare spending would have been reduced by $33 million, from $264 million to $231 million, over one year (between June 2010 and June 2011).

Some researchers have proposed linking information about the comparative clinical effectiveness of health care services to FFS payment policies to improve value in Medicare spending. For example, Pearson and Bach

proposed that Medicare adopt a "dynamic pricing policy" that would base payment for a new service on the usual statutory formulas, but, after three years, the service's payment rate would be reduced if comparative clinical effectiveness information did not show that it offered clinical advantages compared with its alternatives (Pearson and Bach 2010).

Some commercial payers link evidence of comparative clinical effectiveness to coverage and payment. For example, one commercial payer concluded that, among drugs in a particular therapeutic class (targeted immune modulators), there is a lack of reliable evidence that any one agent is superior to other agents. Consequently, the payer considers the more costly drugs medically necessary only if the patient has a contraindication, intolerance, or incomplete response to the less costly agents (Aetna 2018).

FFS Medicare generally does not consider cost-effectiveness information in coverage and payment policies

Although the Medicare coverage process for Part A and Part B services considers clinical effectiveness evidence, it generally does not explicitly consider evidence on either cost-effectiveness or cost. Only for preventive services (including vaccinations and colorectal cancer screen tests), and based on legislative requests and statutory directives, has Medicare explicitly considered the cost-effectiveness of a service when making a national coverage decision.

Pneumococcal vaccine, the first preventive service added to the Medicare benefits package, in 1981, was based on a congressionally requested cost-effectiveness analysis, which showed it to be cost saving (Chambers et al. 2015a). Since then, the program has considered the cost-effectiveness of other preventive services, including colorectal cancer screening, breast and cervical cancer screening, and other preventive services. For example, the Omnibus Budget Reconciliation Act of 1987 included a provision requiring the Secretary to conduct a demonstration project to determine the influenza vaccine's cost-effectiveness. More recently, Medicare considered cost-effectiveness evidence in the NCDs for preventive services, including screening for HIV infection in 2009 and counseling to prevent tobacco use in 2010. Both NCDs cited the provision in the Medicare Improvements for Patients and Providers Act of 2008 that the Secretary, in making determinations for preventive services, "may conduct an assessment of the relation between predicted outcomes and the expenditures for such service and may take into account the results of such assessment in making such determination."

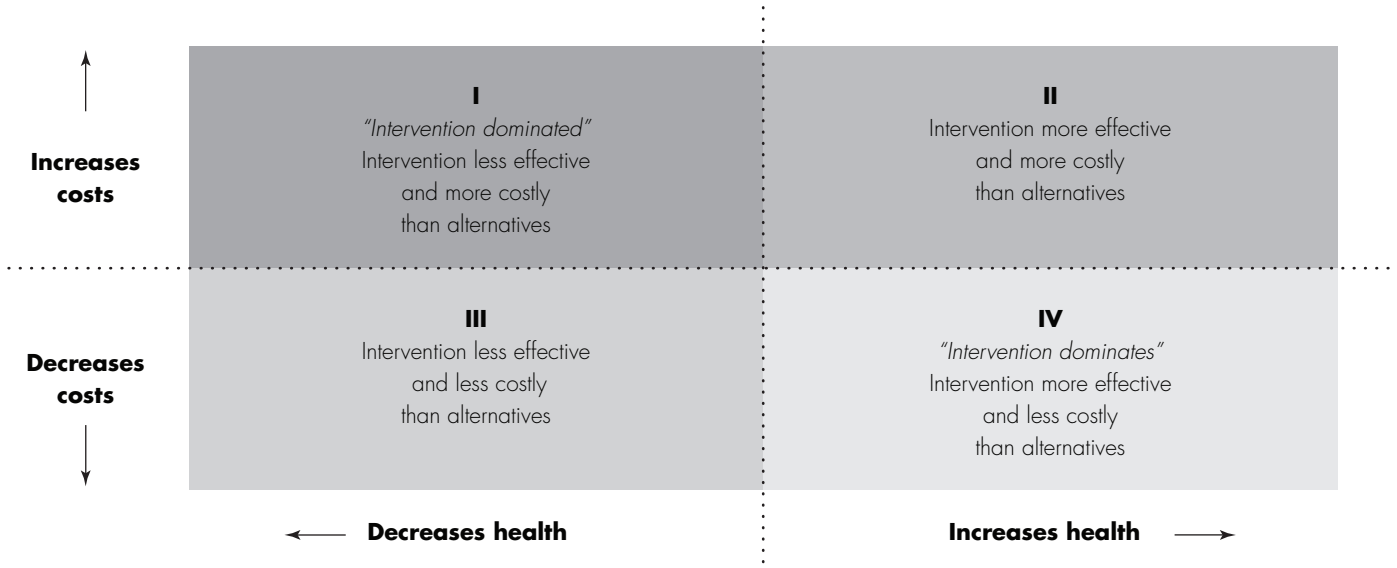
What is cost-effectiveness analysis?

Cost-effectiveness analysis compares the incremental cost in dollars of one intervention with another in creating one unit of health outcome. It has been used to assess a wide range of interventions, including vaccination against pneumococcal pneumonia, bypass surgery for coronary artery disease, and diabetes prevention programs. The results of cost-effectiveness analyses are typically summarized in a series of incremental cost-effectiveness ratios that show, for one intervention compared with another, the cost of achieving an additional unit of health (outcome). To estimate expected health effects and costs, cost-effectiveness analyses require data on each treatment's clinical effectiveness (including comparative clinical effectiveness evidence, if available), health outcomes, and health care resource use and costs.

All cost-effectiveness analyses require that researchers measure the effect (outcome) of a medical intervention on the quantity of health gained. Some cost-effectiveness analyses express health benefits in terms of outcomes specific to the treatment and disease under investigation, such as the number of cancer cases prevented or the number of cancer-related hospital admissions prevented. While this approach is advantageous in that it focuses narrowly on the disease under consideration, the results of such cost-effectiveness studies cannot be compared with the cost-effectiveness of treatments for other conditions. Alternatively, some cost-effectiveness analyses express health benefits in terms of the number of years of life gained. Although the results of such studies can be compared across different treatments and conditions, the outcome measure—increased survival—does not account for the quality of the additional time that is gained due to a medical intervention. Thus, an added month of life with disability or pain is valued the same as an added month without disability or pain.

Expert panels have recommended that cost-effectiveness analyses use outcome measures that integrate both quantity-of-life and quality-of-life effects (Drummond et al. 2015, Gold et al. 1996, Neumann et al. 2017). For example, the quality-adjusted life year (QALY) estimates the gains from improved morbidity (quality gains) and improved survival (quantity gains) into a single metric. QALYs provide a common currency to assess the extent of the benefits that patients gain from a variety

FIGURE 10–6 The impact of medical interventions on outcomes and costs



Note: The figure (often referred to as "the cost-effectiveness plane") evaluates the impact of medical interventions in terms of their net outcomes and net costs as a grid, with four quadrants showing the impact of interventions as either increasing or decreasing health and costs.

Source: Drummond et al. 2015.

of services in terms of health-related quality of life and survival. To calculate QALYs, weights (ranging from 0 to 1) are assigned to each time period that corresponds to the quality of life during that period. The measure is the arithmetic product of life expectancy and a measure of the quality of the remaining life years. Alternatives to QALYs include healthy-years equivalents, saved young life equivalents, and disability-adjusted life years. Each measure has its own limitations and is subject to debate. Economic evaluations that value increases in survival time and changes in quality of life into one measure are sometimes referred to as *cost-utility analyses*.

By providing estimates of costs (in the numerator) and outcomes (in the denominator), cost-effectiveness analysis shows the tradeoffs involved in choosing among alternative interventions. Researchers commonly think of the value of alternative medical interventions—in terms of their net outcomes and net costs—as a grid, with four quadrants showing the impact of services as either increasing or decreasing health and either increasing or decreasing costs (Figure 10-6) (Drummond et al. 2015). Researchers refer to this grid as the "cost-effectiveness plane."

In Figure 10-6, an intervention that falls into Quadrant IV "dominates" because it is more effective and less costly than its alternative. In contrast, an intervention that falls into Quadrant I is "dominated" because it is less effective and more costly than its alternative. An intervention that is more costly and more effective than its alternative falls into Quadrant II, while an intervention that is less costly and less effective than its alternative falls into Quadrant III. Although a new, high-priced innovation may be cost-effective (i.e., have a lower incremental cost-effectiveness ratio) compared with an existing high-priced treatment option, there can be significant financial implications for beneficiaries and taxpayers (Bach 2015).

The number of cost-effectiveness analyses has grown steadily over time. Between 1990 to 1999, the number of published cost-effectiveness analyses averaged 34 per year; by contrast, between 2010 to 2014, the number of published studies averaged more than 500 per year (Baumgardner and Neumann 2017). However, the

application of cost-effectiveness analysis is not equivalent across medical interventions. Between 2010 and 2012, researchers found that nearly half (46 percent) of studies evaluated pharmaceuticals (Neumann and Cohen 2015). Between 1990 and 2012, pharmaceutical manufacturers sponsored an increasing proportion of cost-utility analyses (Neumann and Cohen 2015). During this period, an increasing share of cost-utility studies evaluated oncology interventions while a decreasing share evaluated cardiovascular studies.[58]

The availability of efficacy data on drugs from FDA clinical trials partly accounts for the higher proportion of published studies assessing drugs. In addition, as one component of their pricing strategy, manufacturers may need to show the value of a new drug to formulary committees and other purchasers. Manufacturers also use cost-effectiveness analysis to predict the price that purchasers will be willing to pay for a new drug (Neumann 2005).

Designing a cost-effectiveness analysis When measuring the outcomes and costs of alternative medical interventions, researchers must construct a conceptual model. Such models range from the simple (such as decision trees) to the complex (such as Markov models).

Recognizing the complexity of cost-effectiveness analysis, several panels have endorsed guidelines designed to ensure and improve the quality of such analyses (Gold et al. 1996, Neumann et al. 2017). Recommendations for conducting cost-effectiveness analyses have also been issued by health care organizations, including the World Health Organization and the International Society for Pharmacoeconomics and Health Outcomes, and physician groups, including the American College of Cardiology and the American Heart Association.

A cost-effectiveness analysis typically addresses the following methodological issues:

- ***The method of defining costs.*** Costs include direct medical (e.g., cost of medical services to payers and patients), direct nonmedical (e.g., transportation costs), and non–health care costs (also referred to as indirect costs) (e.g., value of lost productivity due to illness or death). For example, lost productivity is a measure of the costs associated with impaired ability to work or engage in leisure activities and lost economic productivity due to death.
- ***The perspective of the analysis.*** The findings of a cost-effectiveness analysis vary depending on the researcher's point of view. A cost-effectiveness analysis from a societal perspective includes everyone who is affected by the service; all health outcomes; and costs borne by insurers and patients, other medical costs, and nonmedical costs. By contrast, a cost-effectiveness analysis from a health care purchaser's viewpoint would include only those outcomes and costs that affect the purchaser. Some researchers recommend that cost-effectiveness analyses report a reference case based on both the health care perspective and the societal perspective (Neumann et al. 2017).
- ***The sources of clinical effectiveness data.*** Researchers use data from numerous sources, including FDA clinical trials and practical clinical trials, patients' medical records, health care claims submitted to insurers, and health surveys.
- ***The selection of alternative interventions.*** Some researchers recommend that the complete range of available interventions that are likely to be considered by providers and other decision makers should be included, such as existing practice and no treatment (as appropriate) (Drummond et al. 2015, Neumann et al. 2017). Omission of relevant comparators can produce misleading results. For example, researchers may overestimate the cost-effectiveness of an intervention (and underestimate its incremental cost-effectiveness ratio) because an intervention has not been compared with more cost-effective alternatives that are available (Drummond et al. 2015).
- ***The time horizon.*** Researchers must choose the period of time to measure a service's costs and outcomes. The time horizon of the analysis should extend far enough into the future to capture important health effects, and the choice of a time horizon should not bias the analysis in favor of one intervention over another (Drummond et al. 2015). Analyses with a societal perspective often follow patients over their lifetime, while analyses with a health care purchaser's perspective often use a shorter time period (e.g., five years).
- ***The discounting of costs and outcomes.*** When the time horizon of the analysis extends into the future, researchers often convert future costs and future health outcomes to present value. In doing so, researchers

adjust the cost-effectiveness ratios for the different timing of costs and outcomes.

- ***The uncertainty of the clinical events, costs, and outcomes.*** Sensitivity analyses vary the assumptions of the clinical, cost, and outcome data to test for the robustness of the results, to identify the data elements to which the results are particularly sensitive, and to test the point at which one intervention becomes more costly or more effective than another.

- ***The measurement of outcomes.*** Outcomes can be measured in terms of the quantity of health gained, such as number of life-years gained, number of hospital admissions avoided, and number of cases of a particular illness prevented. Alternatively, researchers use measures that combine both the quantity and quality of health gained, such as QALYs, which are widely used in economic evaluations (Drummond et al. 2015). Consensus panels, researchers, and organizations have endorsed using QALYs because the metric reflects effects on both morbidity and mortality and provides a basis for broad comparisons of the health effects of various interventions and policies (Drummond et al. 2015, Gold et al. 1996, Neumann et al. 2017). Even though QALYs are widely used in economic evaluations, the measure has attracted several criticisms, as described in the text box on concerns about QALYs. PPACA prohibits the Secretary from using QALYs (or similar measures) as a threshold to determine Medicare coverage or reimbursement.

Issues and concerns surrounding the use of cost-effectiveness analysis by payers and purchasers

Over the years, numerous stakeholders—drug and device manufacturers, providers, patients, and health economists—have raised issues and concerns about the use of cost-effectiveness information by Medicare and other public and private payers and purchasers.

- ***Some stakeholders mistrust the methods used to conduct cost-effectiveness studies.*** Researchers have noted that methodological approaches vary from study to study. Evaluations of the same services and diseases can sometimes have different results (Eddy 2005, Neumann 2005). The lack of clear reporting on methods has led to concerns from some stakeholders that cost-effectiveness analysis is not transparent. The desire for comparability led the original U.S. Panel on Cost-effectiveness in Health and Medicine to seek standardization in the conduct and reporting of cost-effectiveness analyses through the creation of a reference case. Since the publication of the original panel's recommendations in 1996, more studies are adhering to the guidelines of the panel (Neumann 2009, Neumann et al. 2005).

  Some stakeholders are also concerned that analyses contain the biases of the sponsors who fund the studies and the researchers who conduct them. For example, studies funded by the pharmaceutical industry tend to report more favorable results (Bell et al. 2006, John-Baptiste and Bell 2010, Lane et al. 2016).

- ***Cost-effectiveness analysis might slow innovation.*** Some stakeholders are concerned that payers' use of cost-effectiveness in the coverage process might reduce manufacturers' incentives for innovation by creating a hurdle to launch medical services (Neumann 2005). For example, manufacturers have noted that a negative NCD by Medicare has an enormous (negative) effect on manufacturers' revenues. In contrast, some observers argue that there is an inherent need to strike a balance between incentives for innovation and access to high-value services and that the use of cost-effectiveness analysis might stimulate manufacturers to bring more cost-effective products to market. Others argue that payers do not have to use information on cost-effectiveness analysis rigidly. For example, payers could use information from cost-effectiveness analyses to prioritize quality initiatives.

- ***Affinity for new technology could bias the public against use of cost-effectiveness in coverage decisions.*** Some researchers contend that stakeholders' resistance to the use of cost-effectiveness analysis might stem from the affinity for new medical technology in the United States. Research using survey data found that 9 of 10 adults agree that there is a strong link between being able to get the most advanced technology and receiving high-quality health care and that Americans expressed more interest in new medical discoveries than survey participants from European countries (Schur and Berk 2008).

- ***Cost-effectiveness analysis might interfere with the clinician–patient relationship.*** Some clinicians contend that using cost-effectiveness analysis could affect their advocacy duties and the trust necessary for

Concerns about using QALYs in economic evaluations

Quality-adjusted life years (QALYs) are widely used in economic evaluations and have been endorsed by several research panels (Gold et al. 1996, Neumann et al. 2017). Among the measure's strengths:

- QALYs can account for gains in both the quantity and quality of health gained. By contrast, assessing only the quantity of health gained, such as life-years gained or number of strokes avoided, does not consider changes in an individual's disease symptoms, functional capacity, and well-being (i.e., quality of life).
- QALYs can be used across a wide variety of diseases and treatments, enabling the comparison of interventions both within and across disease and treatment categories. For example, health losses associated with treatments for myocardial infarctions can be expressed commensurately with health losses associated with pneumonia.

Nonetheless, there is debate among researchers and others about their use (Drummond et al. 2015, Gold et al. 1996). The debate about QALYs centers on the techniques and methods used to develop QALYs and concerns that QALYs may not reflect societal values and may be biased against certain populations, including the elderly and the disabled. In addition, some stakeholders contend that the measure is in contrast to the movement toward personalized medicine and patient-centered care (Partnership to Improve Patient Care 2018).

The Patient Protection and Affordable Care Act of 2010 prohibits the Secretary from using QALYs (or similar measures) as a threshold to determine coverage, reimbursement, or incentive programs under Title XVIII of the Social Security Act (Medicare and Medicaid). According to the statute:

- "The Secretary shall not utilize such an adjusted life year (or such a similar measure) as a threshold to determine coverage, reimbursement, or incentive programs under title XVIII."
- "The Secretary shall not use evidence or findings from clinical comparative effectiveness research . . . in determining coverage, reimbursement, or incentive programs . . . in a manner that treats extending the life of an elderly, disabled, or terminally ill person as of lower value than extending the life of an individual who is younger, nondisabled, or not terminally ill." ■

good relationships with their patients by interfering with their ability to prescribe clinically necessary care (Neumann 2004).

- ***Cost-effectiveness analysis might impair beneficiaries' access to certain services and might lead to rationing.*** Some stakeholders are concerned that payers' use of cost-effectiveness analyses, particularly in the coverage process, might affect access to care. For example, a policy that covers only those services that have cost-effectiveness ratios below a specific threshold would result in patients not having access to all services. Some stakeholders are concerned that payers and purchasers will use cost-effectiveness information for cost-containment purposes only, not for promoting appropriate care. Researchers who conducted focus groups have countered that, when members of the lay public are presented with cost-effectiveness information in a systematic way, they may be willing to use such information to inform priorities for coverage (Gold and Taylor 2007, Gold et al. 2007).[59] Researchers found that 75 percent of focus group participants felt "somewhat" or "very" comfortable with the use of cost-effectiveness analysis to inform Medicare coverage of new treatments, while 10 percent said that it should "never" be used.

Use of cost-effectiveness by other public and private entities There is no exhaustive research on the use of cost-effectiveness analysis by commercial payers, pharmacy benefit managers (PBMs), or other purchasers. Nonetheless, reports in the lay press suggest an increasing interest in determining the value of medical interventions, including examining information on comparative clinical effectiveness and cost-effectiveness. In particular, there appears to be increased interest in determining the clinical effectiveness and value of pharmaceuticals to inform formulary decisions. Medicare organizations that take on financial risk, including MA plans and ACOs, have flexibility in using cost-effectiveness in the design of their medical and pharmacy management programs.

A recent analysis found that 14 of 17 commercial payers considered cost-effectiveness analyses in an average of 14 percent of their coverage policies; 3 payers did not report reviewing information on cost-effectiveness (Chambers et al. 2016).[60] In workshops on cost-effectiveness analysis, about 75 percent of California health care leaders (of public and private health care organizations) who participated said that such analysis should be a factor in decisions by commercial payers (Bryan et al. 2009).[61] The three most frequently cited barriers in using such cost-effectiveness information were:

- the risk of litigation if the organization denies access to treatments that are known to be medically effective but do not demonstrate long-term cost-effectiveness,
- the disconnect between the long-term perspective of cost-effectiveness analysis and the short-term horizons of the payers' decisions, and
- concern about result bias in cost-effectiveness studies with commercial sponsorship (Bryan et al. 2009).

Methodological concerns were not a major theme of the potential barriers to using information on cost-effectiveness.

Over the past few years, there has been increasing interest by commercial payers, purchasers, and PBMs in value-based arrangements. The extent to which these arrangements assess cost-effectiveness is unknown. In addition, the fact that ICER and other organizations have launched value frameworks over the past decade suggests the growing acceptance of value and cost-effectiveness assessments. Value frameworks have also been introduced by medical professional groups and provider organizations, including Memorial Sloan Kettering Cancer Center, the American Society of Clinical Oncology, the American College of Cardiology, and the American Heart Association.

Data are limited on the extent to which commercial entities use results generated from ICER's and other organizations' frameworks. However, the sponsorship of ICER by commercial payers, purchasers, and PBMs suggests that these organizations are seeking information on the cost-effectiveness of health care services. For example, in 2016, Prime Therapeutics, a PBM, joined ICER as a flagship member (Prime Therapeutics 2016). According to ICER, pharmacy benefit managers, insurers, and government agencies have used ICER reports in negotiating pricing and preferred formulary placements with manufacturers. Nearly half of all published cost-effectiveness studies evaluated pharmaceuticals, and, between 1990 and 2012, pharmaceutical manufacturers sponsored an increasing proportion of such studies. The move toward value-based payment and outcomes-based payment among private entities that include payers and pharmaceutical manufacturers is ostensibly oriented toward assessing the cost-effectiveness of medical interventions.

The VA uses cost-effectiveness analysis to inform drug formulary decisions (Al et al. 2004). In 2017, ICER announced a collaboration with the VA's Pharmacy Benefits Management Services Office to incorporate the use of ICER drug assessment reports in drug coverage and price negotiations with the pharmaceutical industry (Institute for Clinical and Economic Review 2017).

Other countries use cost-effectiveness analysis in their decisions to cover drugs and in their negotiations with drug companies. For example, the United Kingdom's National Institute for Health and Care Excellence (NICE), founded in 1989, includes cost-effectiveness analyses in its guidance on pharmaceuticals, medical devices, and other medical services.[62] However, the use of cost-effectiveness evidence has not proceeded without some debate. For example, in 2009, to address growing concern about access to new cancer drugs, NICE introduced additional flexibility when appraising treatments that extend survival in patients with short life expectancy (Dillon and Landells 2018).

Overview of the Patient-Centered Outcomes Research Institute

PCORI is a public-private entity established by PPACA and tasked with identifying comparative effectiveness

research (CER) priorities, funding CER efforts, and disseminating CER findings. The statute authorizing the agency prohibits the use of QALYs, specifically stating:

> The Patient-Centered Outcomes Research Institute established under section 1181(b)(1) shall not develop or employ a dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual's disability) as a threshold to establish what type of health care is cost effective or recommended.

Because of this stipulation, the organization states it does not consider cost or cost-effectiveness to be an outcome of direct importance to patients (Patient-Centered Outcomes Research Institute 2017).

PCORI is governed by a 21-member board of governors who are appointed by the Comptroller General of the United States. The board must include the directors of the National Institutes of Health and AHRQ (or their designees) and 19 other members (including 7 clinicians, 3 patient representatives, 3 drug and device industry representatives, 3 private-payer representatives, 1 quality improvement or health services researcher, and 2 representatives from the federal and state governments) with expertise in clinical health sciences research. There is also a 17-member methodology committee whose members are also appointed by the comptroller general of the United States, which sets methodology standards for the organization's research.

The Patient-Centered Outcomes Research Trust Fund

PPACA created the Patient-Centered Outcomes Research Trust Fund (PCORTF) to fund CER efforts between fiscal year 2010 and fiscal year 2019 from three funding streams: appropriations from the general Treasury, transfers from the Medicare Trust Funds, and a fee assessed on private insurance and self-insured health plans.[63] On an annual basis, PCORI receives 80 percent of PCORTF's funds, and HHS receives the remainder. The majority of HHS's funding goes to AHRQ and supports CER dissemination and research capacity–building efforts. Unless reauthorized by the Congress, PCORTF's funding will expire on September 30, 2019. Between fiscal years 2010 and 2017 (the most recent year available), PCORTF funding has totaled $2.4 billion from all revenue streams (the general Treasury, Medicare Trust Funds, private insurance and self-insured health plans, and earned interest).

PCORI's research process Per PPACA, PCORI established, with public comment, five broad national research priorities (and funding allocations) in 2012 to guide the organization's funding of CER efforts:

- assessment of prevention, diagnosis, and treatment options (40 percent of funding)
- improving health care systems (20 percent of funding)
- communication and dissemination of research (10 percent of funding)
- addressing disparities (10 percent of funding)
- accelerating patient-centered outcomes research and methodological research (20 percent of funding)

In addition to these five broad national priorities, PCORI established nine research criteria to identify how each priority would be addressed.[64]

PCORI's most studied conditions include mental/behavioral health disorders, cancer, cardiovascular diseases, neurological disorders, nutritional and metabolic disorders, and trauma/injury. PCORI's top three populations of interest are racial/ethnic minorities, individuals of low socioeconomic status, and older adults.[65] In 2015, PCORI began funding pragmatic clinical trials—observational studies that compare two or more alternatives for preventing, diagnosing, treating, or managing a particular clinical condition. PCORI has stated that $5 million to $15 million in funding will be available for these trials (Patient-Centered Outcomes Research Institute 2013).

In 2014, PCORI created the National Patient-Centered Clinical Research Network (PCORnet), whose goal is to improve the national infrastructure for comparative effectiveness research by using large amounts of health data to address patients' and clinicians' health-related questions. Researchers can access large sets of health and health care data through electronic medical records and claims data gathered in real-world settings (e.g., clinics and hospitals) (National Patient-Centered Research Clinical Network 2018). This network currently includes 128 million individuals' data that can be used for randomized clinical trials, large observational studies, and other research (Government Accountability Office 2018).

PPACA mandated that the Government Accountability Office (GAO) release two reports evaluating activities funded by PCORI. In its first report, GAO assessed the organization's financial statements and concluded that PCORI was operating in line with the expectations that PPACA laid out (Government Accountability Office 2015). In its second report, GAO found that PCORI funded some 600 research-related infrastructure and methods projects for roughly $2 billion. Of the total funding, PCORI awarded 79 percent to fund comparative-effectiveness research projects, 16 percent to create PCORnet, and the remaining 5 percent to fund projects related to methods development and dissemination (Government Accountability Office 2018).

PCORI's dissemination process presents research findings on its website—one for consumers and patient audiences and a more technical version for medical professionals—within 90 days after the results are finalized. In fiscal year 2016, 190 articles associated with PCORI-funded projects were published, an increase from 56 articles in fiscal year 2014. With respect to its use in clinical care, PCORI reports that two of its studies on prostate cancer were included in medical resource software used by academic medical centers (Government Accountability Office 2018).

Concerns about PCORI Patient advocacy groups like the Partnership to Improve Patient Care have commented on PCORI's commitment to supporting patient-centered research that engages patients and aids in their health care decisions (Schulte 2015). Additionally, some researchers have perceived the positive effects of its mission. For example, the PCORI board has worked to ensure transparency, credibility, and access, holding open board meetings every other month in various cities across the United States (Washington and Lipstein 2011).

However, some organizations and researchers from different political perspectives have raised concerns about PCORI. Mazur and colleagues noted that less than one-third of PCORI studies involve or are relevant to primary care—the largest patient care platform in the United States (Mazur et al. 2016). Researchers from the Center for American Progress raised concerns that PCORI was not adequately funding comparative clinical effectiveness research (Emanuel et al. 2016).

In interviews that GAO conducted with stakeholders (including health policy experts and PCORI contractors), some interviewees expressed concern that PCORI's research priorities are broad and lack specificity (Government Accountability Office 2015). According to GAO, payer representatives noted limitations to the usefulness of PCORI's research findings because they do not take treatment costs into account (Government Accountability Office 2018). Some have recommended that the organization strategically plan its agenda to address research questions that comparative-effectiveness research can answer quickly and decisively (Sox 2012). The findings of such studies would ideally make their way into everyday medical practice and demonstrate PCORI's ability to fund important transformative comparative-effectiveness research.

Overview of the Institute for Clinical and Economic Review

ICER is an independent nonprofit organization founded in 2005 with the goal of providing independent analysis of evidence on the value and effectiveness of medical interventions, including drugs, medical devices, tests, and delivery system innovations. Nonprofit foundations provide 78 percent of the organization's funding. Their largest individual source of funding comes from the Laura and John Arnold Foundation. The remaining 22 percent of their support comes from other nonprofit organizations, pharmaceutical manufacturers, health plans, and pharmacy benefit management companies.[66] ICER does not accept funding from manufacturers or private insurers to perform reviews of specific technologies (Institute for Clinical and Economic Review 2018b).

ICER's evaluations include a systematic review of the clinical and economic literature on a given intervention and an analysis of the cost-effectiveness and potential budget impact associated with the intervention. As part of its comparative clinical effectiveness assessment, the analyses provide sources of evidence, the strengths and limitations of individual studies, and an evaluation of the net health benefit of the treatment options being considered. ICER's analyses apply evidence of a treatment's comparative clinical effectiveness to determine its cost-effectiveness, usually over the lifetime of patients (when feasible). ICER also assesses the potential budget impact of a new drug over a five-year period, taking into account assumptions about the treatment's projected uptake. ICER calculates cost-effectiveness from the health system perspective as its base case but performs a scenario analysis to include work productivity when feasible. The primary measure of ICER's cost-effectiveness analysis is the QALY; other measures, such as the cost per life-year gained and cost per avoided event (e.g., stroke), are also reported.

According to ICER, the organization aims to make its research and methodology process as transparent and public as possible. According to ICER's patient and manufacturer engagement guide, there are both formal and informal opportunities for patients, manufacturers, and other stakeholders to provide input and comment during the report development process. ICER recently announced that its executable versions of draft cost-effectiveness models will be shared with relevant manufacturers during the evidence review process. In addition, the organization recently updated its value assessment framework and provided opportunities for public comment from stakeholders. ICER's new value framework seeks to inform decisions that are aimed at achieving sustainable access to high-value care for all patients. Long-term value is the primary anchor for ICER's framework, but the organization also considers short-term affordability in its assessments.

For example, a recent ICER report on the comparative clinical effectiveness and value of chimeric antigen receptor T-cell therapies (tisagenlecleucel and axicabtagene ciloleucel) for treatment of two types of B-cell cancers concluded that each product was cost-effective (with incremental long-term cost-effectiveness ratios below or within $50,000 per QALY and $150,000 per QALY gained) (Institute for Clinical and Economic Review 2018a). However, ICER also concluded that the potential short-term budget impact for one of the products, axicabtagene ciloleucel, would exceed ICER's annual $915 million annual budget impact threshold at the product's current price.[67] According to ICER, the added cost of a new service that exceeds its annual budget impact threshold may be difficult for a payer to absorb over the short term without displacing other needed services or contributing to a rapid growth in insurance costs, which might affect patients' access to high-value care. Other examples of its completed and current analyses include reports evaluating the CER and value of drug treatments for hepatitis C, prostate cancer, hemophilia type A, migraines, osteoporosis, and cystic fibrosis and of nondrug treatments of low back pain.

Concerns about ICER Some stakeholders have argued that ICER fills a necessary void in the U.S. health care system. Many see the impact that ICER could have on shaping health care and assessing the value of treatments. With the rise of prescription drug prices in the United States, some researchers have called for the need to assess the benefits and value of drugs and other interventions (Neumann and Cohen 2015). Since there is no federal government organization that performs research similar to ICER, some stakeholders have said that the organization will have a valuable and growing influence on the health care system (Silverman 2016).

Other stakeholders assert that ICER's evaluations of the affordability of drugs favor insurance companies. In addition, representatives of pharmaceutical and medical device manufacturers and other health care organizations have raised many concerns about ICER. For example, these stakeholders have (1) asserted that ICER's models used to assess a therapy's value are not sufficiently transparent to the public; (2) taken issue with the methods used to assess value (e.g., the overreliance on data from randomized clinical trials and the use of QALYs to assess cost-effectiveness); (3) asserted that patients, patient groups, family caregivers, and others have not been sufficiently engaged in the analytical process.

Two evaluations criticized the five-year time horizon ICER uses in its budgetary evaluations: "ICER's approach is problematic because it penalizes high-value new technologies, treats all drugs the same regardless of the severity of the underlying condition, encourages a myopic view (overweighting upfront costs and ignoring savings and health benefits that occur after 5 years), downplays existing waste and inefficiency in the system, and provides disincentives to companies developing a drug with broad public health impacts" (Lakdawalla and Neumann 2016, Neumann and Cohen 2017). Neumann and Cohen further criticized ICER's use of cost-effectiveness thresholds ($50,000 per QALY to $175,000 per QALY gained), arguing that these judgments should be made by payers and their enrollees, and argued that ICER should assess a treatment's cost-effectiveness from the societal perspective, not solely from the health system perspective.[68]

Conclusion

FFS Medicare's coverage process allows many new services to disseminate quickly into routine medical care without evidence that they are superior to existing treatments. In addition, there is substantial use of low-value care. A very conservative estimate of Medicare spending on low-value services ranges from $2.4 billion to $6.5 billion per year. There is additional spending on

potentially low-value services such as early initiation of dialysis, proton beam therapy, and H.P. Acthar Gel. The spending estimates do not reflect the downstream cost of low-value services (e.g., follow-up tests and procedures). Because other payers also cover low-value services, payers may want to coordinate their efforts to identify and reduce low-value care. There are many policy tools that Medicare could consider adopting to reduce the use of low-value services, such as prior authorization, clinician decision support and provider education, altering beneficiary cost sharing based on the clinical value of a service, new payment models, revisiting coverage determinations on an ongoing basis, and linking FFS coverage and payment to clinical comparative effectiveness and cost-effectiveness information. CMS has developed early experience with some of these tools, such as prior authorization and new payment models. ■

Endnotes

1 CMS explained that, since it anticipated limiting the application of cost considerations to a "narrow situation when two services have equivalent health outcomes and are of the same clinical modality," it would have needed to conduct a simple cost analysis in such cases.

2 The Commission's estimate of the number of LCDs does not take into account the duplication of LCDs within a given region.

3 Instances in which CMS may request an external technology assessment include the following: (1) the evidence to review is extensive, making it difficult to complete an internal technology assessment within the statutory time frame; (2) there are significant differences in opinion among experts concerning the relevant evidence; and (3) the review requires clinical or methodological expertise not available among CMS staff at the time of the review.

4 The MMA requires that CMS consult with outside clinical experts if the MEDCAC is not convened.

5 Other factors that CMS considers for removing NCDs under the expedited process include the following: local contractor discretion would better serve the needs of the program, the technology is obsolete and no longer marketed, and the NCD has been superseded by subsequent Medicare policy.

6 Under Section 1862(a)(1)(E) of the statute, the Secretary has the authority to "conduct and support research through the AHRQ administrator with respect to the outcomes, effectiveness, and appropriateness of health care services and procedures in order to identify the manner in which diseases, disorders, and other health conditions can most effectively and appropriately be prevented, diagnosed, treated, and managed clinically."

7 The Symplicity renal denervation system for treatment-resistant hypertension is the only other device known to be accepted into the Parallel Review Program, according to its manufacturer (Medtronic 2013). The device's parallel review process, which began in 2013, was discontinued in 2014 after the manufacturer announced that the device did not achieve its primary efficacy endpoint in a clinical trial (Gafney 2014).

8 For example, Medicare covers off-label use of bevacizumab for metastatic breast cancer despite the FDA's removal of this clinical indication from the biologic's label in 2011. In 2016, Medicare's Part B spending for bevacizumab for breast cancer totaled $17 million, which represents 2 percent of the biologic's FFS Medicare spending.

9 Currently, there are 4 MACs that process durable medical equipment claims and 12 MACs that process all other Part A and Part B claims.

10 Before BIPA, Medicare's contractors developed local medical review policies (LMRPs). The difference between an LMRP and LCD is that an LCD is a determination as to whether an item or service is reasonable and necessary, while LMRPs may also contain benefit category and statutory exclusion provisions.

11 OIG's estimates are based on a review of LCDs for Part B services (excluding durable medical equipment items) in effect during a one-week period in 2011.

12 Interested parties include beneficiaries residing or receiving care in a contractor's jurisdiction, providers doing business in a contractor's jurisdiction, and any interested party doing business in a contractor's jurisdiction.

13 Under law, drugs or classes of drugs or their medical uses that may be excluded from coverage or otherwise restricted under Medicaid under Sections 1927(d)(2) or (d)(3) of the Social Security Act (except for smoking cessation agents) are excluded from the definition of a Part D drug (42 CFR § 423.100). Examples of excluded drugs include drugs for weight loss or gain, drugs for erectile dysfunction, drugs for relief of cough and colds, nonprescription drugs, drugs used for cosmetic purposes or hair growth, drugs used to promote fertility, and prescription vitamins and minerals, except prenatal vitamins and fluoride preparation products.

14 The amount in controversy must be greater than the specified dollar thresholds.

15 The authors adjusted for differences in beneficiaries' sociodemographic and clinical characteristics and geographic location.

16 The study adjusted for changes in each group's sociodemographic and clinical characteristics (e.g., the presence of specific chronic conditions and the total number of conditions) between the precontract period and postcontract period.

17 Both of these measures were limited to low-risk, noncardiac surgery.

18 The study used data on patients with commercial insurance from the Health Care Cost Institute, which maintains a database of claims on individuals who are under age 65 with employer-sponsored insurance from Aetna, Humana, Kaiser Permanente, and UnitedHealthcare.

19 The DXA scan measures bone mineral density.

20 The T_3 service is a lab test that measures the level of T_3 in the blood. The test is used to evaluate and manage thyroid dysfunction.

21 The HEDIS measure for high-risk medication is described as the share of Medicare members ages 66 and older who had at least one dispensing event for a high-risk medication, or the share of Medicare members ages 66 and older who had at least two dispensing events for the same high-risk medication.

22 Schwartz and colleagues published a study that used 26 of their measures to calculate the amount of low-value care in FFS Medicare in 2009 (Schwartz et al. 2014).

23 The broad version of the PSA screening measure includes all PSA tests for men ages 75 and over. It includes both screening and diagnostic billing codes.

24 The narrow version of the PSA screening measure includes PSA tests for men ages 75 and over who do not have a history of prostate cancer. It includes screening (but not diagnostic) billing codes.

25 For each geographic area, the model included demographic variables (e.g., age, race, sex, and Medicaid enrollment), clinical variables (e.g., the presence of specific chronic conditions and the total number of conditions), and a dummy variable.

26 We used the narrow versions of the measures for this analysis because they represent a more conservative estimate of low-value care.

27 Researchers estimated that, among patients of all insurance types, dialysis was initiated at a mean of 147 days earlier in 2007 compared with 1997 (O'Hare et al. 2011).

28 The study was produced for the Washington State Health Technology Assessment Program.

29 *Superior net health benefit* means that the evidence suggests a moderate-to-large net health benefit versus comparators. *Incremental net health benefit* means that the evidence suggests a small net health benefit versus comparators.

30 *Comparable net health benefit* means the evidence suggests that, while there may be trade-offs in effectiveness or harms, overall net health benefit is comparable with comparators.

31 One facility (Indiana University Health Particle Therapy Center) closed in 2014 and is not included in these numbers.

32 There are four HCPCS codes for proton beam therapy. Code 77520 is in ambulatory payment classification (APC) group 5623, which had a payment rate of $506 in 2016. This code accounted for only 1 percent of the volume of proton beam therapy codes paid under the OPPS in 2016. HCPCS codes 77522, 77523, and 77525 are in APC group 5625, which had a rate of $1,151 in 2016. These codes accounted for the remaining 99 percent of volume.

33 Providers may also consider a patient's trajectory of kidney failure (i.e., the rate of decline in eGFR levels) when considering when to begin dialysis.

34 The NKF KDOQI guidelines are the most commonly used clinical guidelines in the United States. The NKF does accept financial support from the industry.

35 The authors adjusted for selection bias by matching proton beam therapy patients with IMRT patients with similar clinical and demographic characteristics.

36 As described earlier, these signs and symptoms of kidney failure could fall under the larger symptom categories of fluid overload or evidence of uremia. The specific signs and symptoms examined in this study included lower extremity edema, pulmonary edema, pericarditis, shortness of breath, cognitive dysfunction, pruritus, nausea or vomiting, anorexia, diarrhea, constipation, fatigue, muscle cramps, pain, sleep disturbance, sexual dysfunction, depressive symptoms or anxiety, altered taste, muscle weakness, hiccups, or dizziness.

37 The relationship between predialysis nephrology care and earlier initiation could also partially be explained by individuals who "crash" onto dialysis having much lower eGFRs, and thus bring down the average for those receiving no nephrology care (Slinin et al. 2014).

38 Cahaba Government Benefit Administrators covers providers in Alabama, Georgia, and Tennessee. CGS Administrators covers Kentucky and Ohio. First Coast Service Options covers Florida, Puerto Rico, and the Virgin Islands. Of the 27 proton beam facilities in the United States as of April 2018, 8 are located in states covered by these 3 MACs.

39 The clinical trial must be approved by an institutional review board and meet the standards of scientific integrity and relevance to the Medicare population as described in the *Medicare National Coverage Determinations Manual*. The clinical registry must be compliant with the principles established in AHRQ's *Registries for Evaluating Patient Outcomes: A User's Guide*.

40 National Government Services covers providers in Connecticut, Illinois, Maine, Massachusetts, Minnesota, New Hampshire, New York, Rhode Island, Vermont, and Wisconsin. Its LCD limits coverage of proton beam therapy

for conditions in Group 2 to providers who have demonstrated experience in data collection and analysis with a history of publication in the medical literature.

41 Carriers were the Medicare contractors who processed and paid claims for Part B services before Medicare established the MACs.

42 The other indications are rheumatic, collagen, dermatologic, allergic, ophthalmic, respiratory, and edematous states.

43 For example, 65 percent of patients who received Acthar improved on the Disability Status Scale after four weeks, compared with 48 percent of patients who received placebo (the difference was significant at $p < 0.05$). According to a measure of each patient's overall condition, 82 percent of patients who received Acthar improved, compared with 69 percent of patients who received placebo, but the difference was not statistically significant ($p < 0.05$).

44 At the time this report was prepared, CMS had not yet released Part D data from 2016.

45 Spending per prescription is higher than the average price per vial because spending per prescription does not reflect manufacturers' rebates, and some prescriptions are for more than one vial.

46 There were 174 prescribers in each decile.

47 The model originally applied to California, Florida, Illinois, Michigan, New York, North Carolina, and Texas. The 12 additional states added in the extension included Arizona, Georgia, Indiana, Kentucky, Louisiana, Maryland, Missouri, New Jersey, Ohio, Pennsylvania, Tennessee, and Washington.

48 The three original states included in the demonstration were South Carolina, New Jersey, and Pennsylvania. Through the expansion, Delaware, the District of Columbia, Maryland, North Carolina, Virginia, and West Virginia were added.

49 The evaluation included only ESRD beneficiaries because they constitute about 75 percent of Medicare RSNAT claims.

50 The payment reduction was mandated by statute. It began before the demonstration started and may have influenced ambulance provider behavior during the demonstration evaluation period. However, the evaluation used a differences-in-differences study design to control for external changes that occurred during the demonstration.

51 The three states included in the model were Illinois, Michigan, and New Jersey.

52 This program was mandated by the Protecting Access to Medicare Act of 2014.

53 The demonstration was mandated by the Medicare Improvements for Patients and Providers Act of 2008 and applied to 12 common imaging services. Participation by physicians was voluntary.

54 One of the other ways in which the models were different was their beneficiary attribution method. ACOs in the Pioneer ACO Model had beneficiaries attributed to them prospectively (at the beginning of the year), while MSSP ACOs had beneficiaries attributed to them retrospectively (at the end of the year).

55 Large dialysis organizations (those with 200 or more dialysis facilities) that participate in the ESCO model are required to be at two-sided risk, while small dialysis organizations (those with fewer than 200 dialysis facilities) have the option to choose between one-sided and two-sided risk.

56 Medicare's NCD for lung cancer screening covers low-dose CT once per year for beneficiaries between the ages of 55 and 77, who do not have symptoms of lung cancer, who have a history of smoking at least one pack per day for 30 years, and who either are current smokers or have quit smoking within the last 15 years. The NCD also requires that beneficiaries have an office visit (before the screening visit) that is devoted to counseling and shared decision-making on tobacco-related issues and the relative harms and benefits of lung cancer screening.

57 The courts asserted that the statute's provision for Part B drugs based on its average sales price precludes Medicare from applying LCA policies. More information about this topic can be found in the Commission's June 2010 report to the Congress (Medicare Payment Advisory Commission 2010).

58 In terms of diseases studied, cost-utility studies evaluated cardiovascular diseases (18 percent of the studies overall), oncology (15 percent), and infectious diseases (15 percent).

59 The focus group participants received information about methods used to conduct cost-effectiveness analyses and information about the effectiveness of treatments for 14 conditions. They were asked to prioritize the coverage of these 14 treatments under assumptions of a constrained Medicare budget. They were then given cost-effectiveness information to revisit and discuss their rankings. Provision of cost-effectiveness information influenced their priorities.

60 Across the 14 payers, cost-effectiveness was factored into between 8 percent to 43 percent of policies.

61 A total of 58 people participated in the workshops on cost-effectiveness analysis sponsored by the California Health Care Foundation.

62 NICE uses QALYs in its cost-effectiveness analyses.

63 The fee equaled $1 per insured person in fiscal year 2013 and $2 per insured person in fiscal year 2014; thereafter, the fee is increased by the percentage increase in the projected per capita amount of National Health Expenditures, as most recently published by the Secretary before the beginning of the fiscal year.

64 These research criteria are (1) impact on health of individuals and populations, (2) probability of improvability through research, (3) inclusiveness of different populations, (4) the ability to address current gaps in knowledge/variations in care, (5) impact on health care system performance, (6) potential to influence decision-making, (7) patient centeredness, (8) rigorous research methods, and (9) efficient use of research resources.

65 This finding is based on the number of projects (out of a total of 365). A project may study more than one condition or population of interest.

66 Other nonprofit and for-profit entities that fund ICER include Blue Cross Blue Shield of Massachusetts, Blue Shield of California Foundation, the California Health Care Foundation, Express Scripts, Genentech, Johnson & Johnson, and Kaiser Permanente.

67 ICER's short-term budgetary impact analysis includes an estimate of the share of patients who could be treated at selected prices without crossing a budget impact threshold that is aligned with overall growth in the U.S. economy. Factors included in the calculation of ICER's budget threshold include growth in U.S. gross domestic product, total personal medical health care spending, contribution of drug spending to total health care spending, and average annual number of new molecular entity approvals.

68 A cost-effectiveness analysis with a societal perspective incorporates direct medical, direct nonmedical (e.g., transportation), and indirect costs (e.g., lost productivity), regardless of who incurs the costs (Gold et al. 1996). By contrast, an analysis with a payer perspective may incorporate only direct medical costs incurred by the payer.

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