Testimony of
Leslie V. Norwalk, Esq.
Acting Administrator
Centers for Medicare & Medicaid Services
Before the
U.S. House of Representatives
Ways and Means Subcommittee on Health
on
"Payment, Safety and Quality Issues in Treatment of Patients with ESRD"

June 26, 2007

Good afternoon, Chairman Stark, Congressman Camp and distinguished Members of the Subcommittee. Thank you for inviting me to discuss important developments related to payment, safety, and quality issues in the treatment of patients with End-Stage Renal Disease (ESRD), Medicare's only disease-specific program. Roughly 400,000 Americans suffer from ESRD and require either kidney dialysis or transplantation to survive. In addition, an estimated 20 million Americans have Chronic Kidney Disease (CKD) from various causes, creating the potential for substantial growth in the number of patients with ESRD unless ways are found to mitigate the progression of CKD. ESRD-diagnosed individuals of all ages are entitled to Medicare coverage, and this population has been growing steadily through the years, placing increased resource demands on the Medicare program.

As I mentioned briefly when I testified before the Committee last December, the Centers for Medicare & Medicaid Services (CMS) has spent a great deal of time and focused significant attention on the development of a prospective payment system for ESRD treatment that bundles payment for separately paid drugs and other items. I would like to discuss this work today. Additionally, I would like to provide the Committee with an update on our efforts to monitor hematocrit levels among ESRD patients, and discuss our efforts to examine the use of Erythropoietin Stimulating Agents (ESAs) in certain patient populations.

Developing a Bundled Prospective Payment System for ESRD

Medicare provides coverage to an estimated 400,000 beneficiaries with ESRD and spends about $8.1 billion annually for ESRD services. Currently, ESRD services are paid under a blended model. Approximately 60 percent of total payments to ESRD facilities are paid under a composite rate that has a basic case-mix adjustment. The remaining 40 percent of payments to ESRD facilities represent separately billed services (primarily drugs and clinical lab tests). Payments for one drug used in particular for ESRD care, erythropoietin, represents about 60 percent of these separately billable services or 25 percent of the total payment for ESRD services.

Many have urged a shift from the current model of paying independently for dialysis treatments and separately billable drugs, to a system of bundled prospective payment. CMS is generally supportive of such reform, depending of course on the specifics of the proposal.

As required by Section 623(f) of the Medicare Modernization Act (MMA), CMS will be issuing a report to Congress that covers the elements and features for the design and implementation of a bundled prospective payment system for ESRD services. Research conducted by CMS and contract researchers at the University of Michigan was complex; as a result, it took longer to complete than we anticipated. However, it has allowed us to make significant progress in assessing key design elements I would like to discuss today.

(1)—Scope of Services: A prospective payment system needs to have a scope of services that is included in the bundled rate. A potential bundle of services for an ESRD prospective payment system could include the following: composite rate services; separately billed drugs; separately billed lab tests; and other separately billed dialysis services paid under Part B, such as supplies and blood products.

(2)—Unit of Payment: A prospective payment system needs a defined unit of payment. In some prospective payment systems, such as the current ESRD composite rate, the unit is per treatment. In other prospective payment systems, such as home health, the unit is a

period of time over which services may be received. Each potential payment unit type has advantages and disadvantages that must be fully vetted. For example, payment per treatment generally encourages adequate provision of services, but could discourage innovative treatment methods that could improve quality outcomes. Monthly payments generally give providers maximum treatment flexibility and create incentives to furnish services in the most efficient manner. However, a monthly payment also can provide incentives to underserve patients.

(3)—Case-Mix Adjustment: Payment units in prospective payment systems have case-mix adjustments in order to reflect the variation of resources for different kinds of patients. There are a number of potential case-mix adjustment factors that could be used in a bundled ESRD prospective payment system. Our research will examine an analytic approach using multiple data sources including: claims data covering both billings for composite rates as well as separate billings for drugs and lab tests (for 2002 through 2004); cost report data (for 2004 supplemented with 2005 data); and enrollment and patient characteristics.

The current ESRD basic case-mix adjusted system includes adjustments to the facility's composite rate for five age groupings, body surface area, and low body mass index (an indicator of patients who are malnourished). Other prospective payment systems have different case mix adjustments based on other factors such as comorbid conditions or other clinical factors.

(4)—Geographic Adjustment: Prospective payment systems often entail some type of geographic adjustment to reflect relative differences in resource costs among geographic areas. The current ESRD payment system adjusts a portion of the composite rate for geographical differences in wages, similar to other prospective payment systems.

(5)—Other Payment Adjustments: Prospective payment systems often have special adjustments such as for outlier cases to account for very costly cases, or special characteristics of facilities, e.g., rural location.

(6)—Special Design Issues for ESRD: Prospective payment systems often have special design and implementation issues unique to the particular type of service. In the case of ESRD services, these special issues may include (a) whether there should be separate rates for hospital based and independent facilities or a consolidated single rate for all facilities; (b) treatment of oral Part D covered versions of Part B covered intravenous drugs; (c) billings for clinical laboratory tests furnished by independent laboratories; (d) payment for home dialysis including peritoneal dialysis; (e) treatment of currently-approved composite rate exceptions for pediatric facilities; (f) costs for self-dialysis patient training; and (g) application of beneficiary coinsurance under a bundled rate.

(7)—Setting and Updating Initial Rates: Prospective payment systems involve setting the initial payment rates, and a process for considering future changes and updates to these initial payment rates. Initial payment rates under prospective payment systems are often based on expenditures that would be projected to occur in the absence of the prospective system.

In the case of ESRD, questions have been raised about both the use and pricing of erythropoietin, particularly since payments for erythropoietin account for about 25 percent of total ESRD payments (this includes both payments for composite rate and separately billed items in 2005). The Department of Health and Human Services' Inspector General has found that acquisition costs for the ESRD facilities owned or managed by the largest providers is lower than the acquisition costs for other providers. Thus, questions have been raised about whether setting initial ESRD prospective payment system rates based on expenditures that incorporate recent use and pricing of erythropoietin would set such initial rates too high.

Prospective payment systems usually entail processes for consideration of updates. The current ESRD payment system does not provide automatic payment updates. Other prospective payment systems have updates based on a market basket and other factors. Since the statute requires the report to contain a methodology for appropriate updates under an ESRD prospective payment system, we will analyze the development of an ESRD

market basket for a bundled set of services. A market basket can be a useful starting point for determining an appropriate update mechanism. The market basket is a standardized assessment of the inputs involved with furnishing services. Thus, the market basket rate of increase is therefore a standardized measure of changes in input prices.

However, any update mechanism could take a number of other factors into account, such as productivity changes, changes in efficiency, changes in real and measured case mix, and any other variables that may determine appropriate changes to payment rates. For example, an ESRD prospective payment system could provide incentives to achieve efficiencies that would reduce costs, e.g., a movement to subcutaneous administration of erythropoietin. Such efficiencies could be considered in the context of an update. In addition, given that erythropoietin currently accounts for 25 percent of total spending on ESRD services, it presents an issue regarding what assumptions should be made for pricing growth. Finally, a market basket update could be considered in the context of pay-for-performance approaches, e.g., an update could be provided based on performance on quality measures.

(8)—Quality: Prospective payment systems encourage providers to efficiently furnish services. The larger the bundle the more opportunities exist for a provider to achieve efficiency. However, a bundled prospective payment also raises concerns that some providers may furnish fewer services than might be medically needed. An important feature of an ESRD prospective payment system is ensuring the quality of services furnished to beneficiaries, particularly that they receive all medically necessary services. This is especially important for this vulnerable patient population.

For the past 10 years, CMS has been working on quality measures for the quality of care furnished to ESRD beneficiaries. As required by the Balanced Budget Act of 1997, in 1998, CMS developed ESRD Clinical Performance Measures (CPMs) based on the National Kidney Foundation's Kidney Disease Quality Initiative Clinical Practice Guidelines. Sixteen CPMs were developed to measure and report the quality of dialysis services provided under Medicare in the areas of adequacy of hemodialysis and peritoneal dialysis; anemia management; and vascular access management (see

http://www.cms.hhs.gov/ESRDQualityImproveInit/03_Quality%20Measures.asp for more details).

Data on these 16 CPMs are collected on a national random sample of adult in-center hemodialysis patients, all in-center hemodialysis patients less than 18 years of age, and a national random sample of adult peritoneal dialysis patients. Thirteen of the CPMs are calculated, and released in the Department of Health and Human Services Annual Report of the ESRD Clinical Performance Measures Project.

CPM data are not currently collected in numbers sufficient for calculating dialysis facility-specific rates. Right now, they are collected on a 5 percent national sample by paper or electronic forms. However, CMS is currently implementing a system, referred to as the CROWN/Web system, that we expect will allow all ESRD facilities to report CPMs for all patients on or about February 1, 2009. Under this system, ESRD facilities would submit administrative and quality data electronically via the Internet. The CROWN/Web system will allow for the more timely, accurate, and efficient use of data to support administration of the ESRD program. This reporting requirement was included in the proposed rule updating the Conditions for Coverage for ESRD Facilities.

Currently, CMS calculates facility-specific measures using Medicare administrative data and reports these measures on the Dialysis Facility Compare location on www.medicare.gov. The three measures publicly-reported are (a) the percent of Medicare hemodialysis patients treated in the facility that received adequate dialysis treatments (e.g., treatments removing a sufficient amount of waste from the patient's system); (b) the percent of Medicare patients treated in the facility whose anemia was adequately managed; and (c) patient survival categories are reported as expected, better than expected, and worse than expected. These three measures are updated annually on Dialysis Facility Compare, using one year of data for the adequacy and anemia measure and four years of data for the patient survival measure.

Twenty-two measures are scheduled to be considered for endorsement by the National Quality Forum (NQF), a not-for profit membership organization that endorses voluntary consensus standards using agreed upon procedures. The NQF has a formal process by which it achieves consensus on standards or measures that it endorses. The endorsement process for these measures is scheduled to be completed by December 2007. Once endorsed by NQF, these measures would be required to be reported by facilities through the CROWN/Web system beginning in February 2009 if the proposal in the Conditions for Coverage for ESRD Facilities proposed rule were finalized.

(9)—Operational and Administrative Issues: A prospective payment system involves numerous operational, administrative, and systems issues. System changes generally take a minimum of five months to implement, and the considerable changes required for a new payment system could take significantly longer to complete. In addition, successful implementation of a new prospective payment system requires extensive provider education and it is likely that level of provider education would be needed for an ESRD prospective payment system. This timeframe for systems changes begins after a change request is written, which occurs only after final rulemaking, and that can happen only after the policy development needed for rulemaking is completed.

In the case of ESRD, operational and systems changes will likely be needed to expand data elements reported on the claim, and to implement consolidated billing (bundling) requirements. In addition, new payment systems often involve transitions between the old and new systems. While transitions allow facilities to adjust to new payment systems, they often involve administrative complexity.

(10)—Effective Date: The effective date for implementation of an ESRD prospective payment system involves consideration of a number of issues as indicated earlier. First, policy development and rulemaking would be involved. Second, systems changes are needed to ensure that accurate payments are made under the new payment system. All told, it is likely that 2 to 3 years from the date of enactment of authority to implement a prospective payment system would be involved in these activities.

We are also considering how potential changes to the ESRD payment system would interact with the statutorily required demonstration. The process of clearing a solicitation, obtaining and reviewing applications, selecting demonstration sites, and obtaining clearance for the demonstration award typically takes a minimum of 12 months to complete. The statute requires a 3-year demonstration. The final report for the evaluation of a demonstration is typically completed 1 year after the conclusion of the demonstration. Thus, if the demonstration is to be conducted first, before implementing an ESRD prospective payment system, about 5 years would pass before the new payment system could begin to be put into place. A demonstration could shorten somewhat the time required to implement a new payment system, but a new payment system may involve operational issues that the demonstration did not deal with. An alternative to a demonstration that could serve the same purpose would be to monitor and analyze the experience of patients and providers under the new system as it is being implemented.

Promoting Patient Safety and Appropriate Payment through Hematocrit Monitoring

As I indicated in December, nearly all ESRD patients suffer from debilitating anemia – much of which can be managed through drug therapy such as treatment with erythropoietin, an anemia-controlling compound, as an alternative to receiving blood transfusions. To promote appropriate erythropoietin usage, CMS' monitoring policy considers both hematocrit and erythropoietin dosage levels. The monitoring policy indicates that providers should adhere to the Food and Drug Administration (FDA) label instructions for erythropoietin, and not seek to achieve (or "target") a hemoglobin level in excess of 12 g/dL (a value that generally correlates with a hematocrit level of 36.0 percent). The instruction to carriers to initiate monitoring when the hematocrit exceeds 39.0 percent is not a new policy; rather, it establishes a marker at which payment must be reduced because the reported hematocrit was not maintained at levels consistent with FDA labeling.

While patients' therapeutic hematocrit targets are appropriately left to the clinical judgment of their physicians, the monitoring policy recognizes the difficulty of maintaining the hematocrit in the narrow clinical range of 33.0 to 36.0 percent, which is the target range set

forth in current kidney disease clinical guidelines. Because factors such as nutritional status, infection, and bleeding may cause the hematocrit to fluctuate, it is not easy to manage patients to this narrow target range. Some patients might be above (or below) the target in one month, for example, but below (or above) it in others. If frequent and significant changes in doses of anemia management drugs occur on top of these existing hematocrit fluctuations, such hematocrit fluctuations can become even more variable and difficult to interpret and manage, particularly within the narrow target range of 33.0 to 36.0 percent.

Accordingly, the monitoring policy does not immediately cut-off payment for a single reading that fluctuates above or below the 'guideline' value. However, the monitoring policy sets in motion a payment reduction when the hematocrit level exceeds 39.0 percent, and if the provider has not responded by reducing the ESA dosage as FDA labeling and national clinical guidelines indicate.

A provider submitting a claim for ESAs furnished to an ESRD patient with a hematocrit above 39.0 percent may indicate that a dose reduction has occurred, despite the continued high hematocrit, using a modifier on the claim form. If the provider fails to include the modifier, then Medicare will apply an automatic 25 percent reduction in amount of payment for ESAs.

We are in the process of analyzing the impact of this monitoring policy, looking specifically at the percent of ESRD patients for whom the reported hematocrit was above 39.0 percent since the monitoring policy went into effect. We are comparing these data to data for the same measure for periods before the new monitoring policy was in effect. This analysis will reveal whether the monitoring policy has resulted in a reduction in the percent of patients with hematocrits above 39.0 percent. Based on what these data show, we are prepared to consider potential revisions to the monitoring policy.

As mentioned above, the monitoring policy is based on data submitted on the claim form. A key limitation of this approach is that the base period is one during which a prior

monitoring policy was in effect. While this aggregate assessment of the monitoring policy can be done with existing data, it may not be possible to attribute changes to the monitoring policy. We are also assessing the aggregate number of units of erythropoietin that Medicare pays for per beneficiary each month. Here too while this is a macro assessment of erythropoietin use, from a research methodological perspective, it may not be possible to attribute changes to the monitoring policy.

For the longer term, a more detailed study would examine the hematocrits and erythropoietin use for specific beneficiaries; such approach has more potential to hold constant other intervening variables. We are currently developing the methodology for such a study. However, since the human physiologic response to erythropoietin is not immediate, and the effect of a given dosage on the hematocrit of a given individual can vary widely, even analysis of data for the same patient over time may make it difficult to attribute changes in the hematocrit to erythropoietin use.

As I mentioned in December, one possible approach is to collect data, such as the dosage of erythropoietin actually administered or additional hematocrit measurements, through clinical trials. Another approach might be to create registries of data submitted by hospitals and other facilities. Such registries could be a robust data collection mechanism, pursuing elements beyond what can be collected on the claim form. Before such an approach could be adopted, however, CMS must assess potential restrictions to requiring hospitals and facilities to report information to a registry. Provider burden also would be an important consideration.

It should also be noted that an ESRD bundled prospective payment system would focus on appropriate delivery of the full range of ESRD services included in the bundle for a beneficiary. In contrast, the current system, which separately pays for ESAs, encourages their use. An ESRD bundled prospective payment system would change incentives for use of ESAs.

Examining the Use of Erythropoietin Stimulating Agents (ESAs) in Certain Patient Populations

CMS pays close attention to FDA Black Box warnings because the safety of Medicare beneficiaries is paramount. Upon being advised of the March 9, 2007 Black Box warning for use of erythropoiesis stimulating agents (ESAs) in multiple clinical settings, CMS immediately began a dialogue with FDA. FDA conveyed serious concerns about potential dangers with the use of ESAs in some types of cancer/oncology management.

In wanting to protect Medicare beneficiaries from potential avoidable risks, CMS promptly opened a national coverage decision to assess whether there is sufficient evidence to conclude that ESA treatment is not reasonable and necessary for beneficiaries with certain clinical conditions, either because of a deleterious effect of the ESA on their underlying disease or because the underlying disease increases their risk of adverse effects related to ESA use.

Following the opening of this national coverage decision on March 14, 2007, CMS staff reviewed over 500 peer-reviewed articles (which are cited in the proposed national coverage decision) and consulted with FDA staff and other healthcare subject experts in this topic. The FDA held an Oncology Drug Advisory Panel (ODAC) meeting on May 10, 2007 to discuss the safety of recombinant ESAs and to inform possible further revisions to the labeling of these drugs. In addition, representatives of various cancer patient groups provided testimony expressing their concern about safe use of ESAs for treatment of anemia related to cancer. On May 14, 2007, CMS posted a "proposed" or "draft" coverage decision.

The national coverage decision process specifically involves the solicitation of public comment. Like all proposed national coverage decisions, public comment is solicited over a 30-day period following its publication. Ultimately, CMS uses the public comments received to inform its final decision, responding in detail to the public comments when issuing the final decision memorandum. The comment period for this national coverage decision closed on June 13.

We have received input from interested public parties on all sides of this issue, including the physician community, patient groups, and manufacturers. CMS is now in the process of reviewing all of these comments. Some of the comments suggested that ESA use not be restricted for specific conditions or situations as proposed. Many of the critical comments focused on a few specific conditions, e.g., our proposal that ESAs are not reasonable and necessary when used in conjunction with treating anemia of myelodysplasia (MDS) (which is an off-label indication of ESA usage). CMS also received many favorable comments that supported the approach in the proposed national coverage decision. Our physicians are carefully reviewing all of these comments and we will take them into account in developing a final national coverage decision.

At the same time, we are continuing to examine whether similar action is warranted with regard to the use of ESAs to treat patients with non-cancer conditions, namely ESRD patients. We have begun preliminary discussions with the National Institutes of Health about the possibility of collaborating on a large clinical trial to examine the effect of ESA treatment in ESRD patients. Further, we are awaiting the findings of the FDA's Cardiac and Renal Drug Advisory Committee, which will be meeting later this year to specifically examine the use of ESAs in treating the renal patient population.

Conclusion

CMS is committed to establishing and maintaining policies in all areas of the Medicare program that promote efficient and appropriate use of medical interventions, protect beneficiaries, and enable providers to furnish high quality care. As highlighted today, we have made significant progress in the research to develop a bundled prospective payment system for ESRD services, we continue to improve and refine our monitoring policy to promote appropriate erythropoietin usage, and we took prompt action with respect to Medicare coverage of ESAs following the FDA's issuance of a Black Box warning. As Congress considers ESRD payment reform and examines patient safety concerns, we look forward to continuing to work with this Committee on these important issues. At this stage we are continuing to devote significant resources to the substantial analytical and actuarial

development necessary to design a robust and accurate payment system. The development of a new payment system is a significant endeavor that merits careful consideration and analysis.