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GAO

United States
General Accounting Office
Washington, D.C. 20548

Human Resources Division

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October 13, 1993

The Honorable Fortney H. (Pete) Stark
Chairman, Subcommittee on Health
Committee on Ways and Means
House of Representatives

Dear Mr. Chairman:

This letter responds to your request that we evaluate the methodology and data used by the Health Care Financing Administration (HCFA) to establish Medicare's payment rate for erythropoietin, a drug used to treat anemia in dialysis patients with chronic renal failure, also known as end-stage renal disease (ESRD). The Omnibus Budget Reconciliation Act of 1989 required the Secretary of Health and Human Services (HHS) to report on the methodology and rationale used by HCFA to establish Medicare's payment rate for erythropoietin, as well as the method HCFA planned to use to assess future rates.

You expressed concern that the Secretary's report, issued in July 1991, did not include critical information needed to evaluate the appropriateness of the methodology used to develop the payment rate. As agreed with the Subcommittee staff, we reviewed the methods and data used to develop the payment rate for erythropoietin, as well as the problems that HCFA encountered in obtaining the information necessary to set a payment rate for erythropoietin.

BACKGROUND

In 1989, the Food and Drug Administration (FDA) approved the use of recombinant human erythropoietin to treat anemia associated with ESRD. This drug, a genetically engineered version of a natural kidney hormone, stimulates the body's production of red blood cells to help combat anemia, which is common in dialysis patients, and reduce the need for blood transfusions.

Since June 1989, Medicare has paid for recombinant human erythropoietin administered to dialysis patients in dialysis facilities and physicians' offices. Because Medicare covers medical services for about 150,000 ESRD

patients, it is the primary payer for erythropoietin in the United States.

Amgen Incorporated, a biotechnology company, is the sole U.S. marketer of erythropoietin for use by dialysis patients with anemia caused by chronic renal failure. In 1989, FDA granted Amgen 7 years of market exclusivity for Epogen (EPO), Amgen's trademark name for its erythropoietin, under the Orphan Drug Act of 1983. Sales of EPO, constitute the primary source of revenue for Amgen.

HCFA determined Medicare's initial reimbursement rate to providers for the use of EPO. The HHS Office of Inspector General (OIG) assisted HCFA by developing a market pricing model. Amgen was given an opportunity to evaluate the OIG model. Although Amgen did not question the model's cost components, Amgen provided the OIG with additional data regarding the estimates the OIG proposed using for the model's components.

After the OIG evaluated the additional data provided by Amgen, HCFA set the initial payment rate for EPO at $40 for any dose under 10,000 units and $70 for any dose over 10,000 units. In 1990, however, the OIG recommended changing the rate to one based more closely on the number of units of EPO administered. The Congress subsequently changed the rate to $11 per 1,000 units effective January 1, 1991, and $10 per 1,000 units effective January 1, 1994.

RESULTS IN BRIEF

The estimates used to set the initial payment rate for EPO were not close to HCFA's actual experience. This occurred for two reasons. First, there was no information available on the actual number of patients that would use EPO or the quantity of EPO that would be administered during each dialysis treatment. Second, HCFA was denied access to other information it requested to set EPO's payment rate, such as data on the cost of producing EPO.

OIG MODEL INCLUDES NECESSARY COST COMPONENTS

According to the OIG, neither it nor HCFA had prior experience in developing a payment rate for a new drug. Because Medicare was the primary payer of EPO, HCFA wanted to control the payment rate by using available financial information and allowing for a reasonable rate of return and profit based on Amgen's investment. The resulting rate

was intended to be below the average wholesale price, which is generally the base upon which Medicare sets drug reimbursement rates.

HCFA encountered several problems in obtaining information to determine an appropriate payment rate for EPO. First, because Amgen is the only supplier of EPO in the United States, HCFA could not look at market costs or pricing to assist it in determining a rate. Second, because EPO was Amgen's only product, there was no historical cost or utilization data that HCFA could use to develop its model.

At HCFA's request, the OIG reviewed and analyzed limited financial information supplied by Amgen, as well as financial filings with the Securities and Exchange Commission from Amgen and other drug manufacturers. The OIG also reviewed financial forecasts and projections developed by security investment brokers. The OIG developed its model based on estimates because Amgen provided HHS with limited financial information. According to Amgen, some of the information requested by the OIG was not provided because it was proprietary and could, if disclosed, harm Amgen's competitive position.

The OIG model has several components--research and development costs; cost of goods sold; selling, general and administrative costs; and profit and taxes. Amgen said it could not provide HCFA with specific research and development costs for EPO because the company did not segregate these costs for each of the products under development. Therefore, HCFA had to estimate the amount Amgen spent on the research and development of EPO by determining the percentage of Amgen's total sales that were attributable to EPO, and then applying this percentage to Amgen's total research and development costs. Cost of goods sold was based on the estimated cost per unit calculated from Amgen's financial budget projections. Because Amgen was in the process of establishing a sales force at the time HCFA was developing the reimbursement rate, HCFA used Amgen's projections to estimate the costs attributable to marketing and sales. To estimate the markup that would generate a reasonable profit margin for Amgen, HCFA analyzed profit data from 19 companies involved in drug sales to determine the average profit margin. HCFA used a 40-percent tax rate to estimate Amgen's income tax liability.

HCFA then used these estimates plus a return on investment to calculate Amgen's total allocated costs. These costs were then divided by the estimated number of ESRD patients who were sufficiently anemic to benefit from EPO to determine the annual cost per dialysis patient. The annual cost was then divided by the number of dialysis sessions per year, plus wholesaler markup, to arrive at the HCFA reimbursement rate per treatment.

Based on our review of the OIG model and discussions with OIG officials, we believe the model includes the appropriate cost components for establishing a Medicare payment rate. However, we did not evaluate the rate. Amgen, who provided information to HCFA under a pledge of confidentiality, refused the OIG's request that we be given access to the data HCFA used to determine the rate.

ESTIMATES USED IN HCFA MODEL HAVE CHANGED

HCFA made several assumptions to determine EPO's initial reimbursement rate, including the average dose of EPO per treatment and the number of dialysis patients that would benefit from EPO. HCFA estimated that the average dose per patient would be 5,000 units, the same dosage used during clinical trials. It also estimated that initially about 20,000 ESRD patients would be sufficiently anemic to benefit from EPO, and that this number would increase over time as the benefits of EPO to dialysis patients became better known.

In September 1990, the OIG reported that the average dose per administration was approximately 2,700 units, about half the initial estimate of 5,000 units. In addition, the OIG found that the actual market penetration for EPO was approximately 50,000 patients in comparison with the initial estimate of 20,000 patients. The OIG reported that the rise in patients using EPO could increase Medicare costs for EPO from $125 million to $330 million, of which 20 percent will be the liability of the beneficiary.

Although by 1992, actual market penetration was about 70,000 patients and the average dose per treatment was 3,500 units, HCFA has not recomputed the EPO reimbursement rate to reflect these changes. According to OIG officials, it was not necessary to recompute the EPO payment rate because the Congress, in 1991, established a new reimbursement rate of $11 per 1,000 units.

OIG RECOMMENDS LOWER EPO PAYMENT RATE

In February 1993, the OIG issued a report on a review of the prices dialysis facilities paid for EPO. The study showed that, for a random sample of 30 dialysis facilities, the cost of EPO was between $10 and $10.10 per 1,000 units administered. In addition, some facilities also received year-end manufacturer rebates or free EPO from Amgen depending upon the volume purchased. Based on these findings, the OIG recommended that HCFA consider reducing the reimbursement rate not to exceed $10.10 per 1,000 units. According to the OIG study, this would effectuate savings of $27.5 million to the Medicare program and $6.9 million to the beneficiaries. The OIG also recommended that, for a long-term solution, HCFA enter into negotiations with Amgen to determine a rate that takes into account rebates to the Medicare program based on the volume of EPO purchased and used to treat Medicare beneficiaries.

The Omnibus Budget Reconciliation Act of 1993 reduced Medicare payments for EPO to dialysis facilities from $11 to $10 per 1,000 units for services furnished on or after January 1, 1994. Payment for EPO provided in a physician's office would remain unchanged.

There may be additional opportunities to reduce the amount Medicare spends on EPO when the market exclusivity period Amgen received under the Orphan Drug Act for EPO expires in 1996. At that time, other pharmaceutical companies may manufacture and market generic versions of erythropoietin for ESRD-related anemia that may be available to dialysis facilities at lower costs than Amgen's EPO.

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We provided a copy of a draft of this letter to HCFA. HCFA officials told us that the draft was a fair characterization of how they established the payment rate for erythropoietin. We will send copies of this letter to HCFA and Amgen and make copies available to other

interested parties. Please call me at (202) 512-7104 if you have any questions about the information discussed.

Sincerely yours,

Leslie G. Aronovitz
Associate Director,
Health Financing Issues

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