INTRODUCTION

The Food and Drug Administration's (FDA) approval of the biologic[1] recombinant erythropoietin in June 1989 made available an important therapeutic advance for treating anemia associated with chronic renal failure.[2] By increasing the body's production of red blood cells, recombinant erythropoietin may correct anemia and reduce the need for blood transfusions, the most frequently used treatment for this condition.

Although recombinant erythropoietin has engendered excitement in the clinical community, it has also produced concern among policymakers because of its expense and the financial implications for the Medicare program. An annual supply of the product may cost approximately $5,000-$6,000 per treated patient. Because Medicare covers medical services for the elderly and disabled and for about 100,000 dialysis patients (156), it is by far the predominant payer for recombinant erythropoietin in the United States.[3]

Reflecting concern about increased Medicare expenditures, the House Committee on Ways and Means, Subcommittee on Health, requested the Office of Technology Assessment (OTA) to evaluate alternative payment policies for Medicare that might control expenditures related to recombinant erythropoietin without sacrificing the quality of care for beneficiaries.[4] This Special Report responds to that request.

This chapter first summarizes background material regarding recombinant erythropoietin and then identifies and analyzes options for Medicare payment of the biologic. Chapter 2 analyzes the clinical literature on its efficacy and safety; chapter 3 describes the economics of the recombinant erythropoietin marketplace; and chapter 4 reviews Medicare's current payment policies for services provided to patients with end-stage renal disease,[5] for other pharmaceuticals, and for recombinant erythropoietin administered in different health care facilities. The appendixes contain supporting material: appendix A describes the method used to conduct the study; appendixes B and C acknowledge the valuable assistance of workshop

1 FDA defines a biologic as any virus, therapeutic serum, toxin, antitoxin, or analogous product applicable to the prevention, treatment, or cure of disease or injuries to humans (21 CFR 600.3h).

2 Chronic renal failure is a degenerative condition that progresses from a predialysis phase, during which the kidneys still maintain some of their function, to a later phase, when a continuous course of dialysis or kidney transplantation is needed to maintain life. Anemia is characterized by a significant decrease in red blood cell mass and a decrease in the oxygen-carrying capacity of the bleed (23). Anemia is a common complication of chronic renal failure and, in patients with that condition, is caused primarily by an insufficient production of the hormone erythropoietin.

3 In February 1990, Medicare contractors processed claims for recombinant erythropoietin therapy from dialysis facilities for about 31,000 patients. These claims totaled $16.9 million, of which Medicare's share was 80 percent or $13.5 million (47).

4 This study was originally requested as part of a broader OTA project to evaluate alternative payment policies that Medicare could adopt for the outpatient prescription drug benefit added by the Medicare Catastrophic Coverage Act of 1988 (Public Law 100-360). After that benefit was repealed, OTA's Congressional Technology Assessment Board rescinded its approval of the broader study.

5 End-stage renal disease refers to permanent, chronic kidney disease requiring continuous dialysis or a kidney transplant to maintain life.

participants and other individuals; appendix D defines technical terms in a glossary; and appendix E describes the method that the Office of the Inspector General used to estimate the costs related to recombinant erythropoietin of Amgen Inc., currently the only manufacturer that FDA has approved to market the biologic.

SUMMARY

Clinical Significance of Recombinant erythropoietin

FDA evaluation of the safety and efficacy of recombinant erythropoietin was based on data from clinical trials conducted in the United States in anemic chronic renal failure patients. For predialysis and dialysis patients, efficacy data indicate that the biologic increases hematocrit levels and reduces blood transfusions in most patients. The rate of increase in hematocrit and the time required to increase it depend on the dose. The product appears to be efficacious by both the intravenous and subcutaneous routes of administration. The optimal level of initial and maintenance doses, however, still require investigation.

The quality of life of dialysis patients has been impaired because of a number of factors, including the symptoms of anemia (59). Studies assessing the effect of recombinant erythropoietin on the quality of life of chronic renal failure patients suggest that recombinant erythropoietin improves the well-being and ability to function of dialysis and predialysis patients. Future studies should determine long-term changes in the quality of life in elderly dialysis patients, the group projected to have the fastest rate of growth in the dialysis population in the near future, and the ability of dialysis patients to return to work. In addition, the relationship between the use of recombinant erythropoietin and the delayed need for dialysis in the predialysis population should be studied further.

Recombinant erythropoietin appears to be relatively safe. Hypertension is the most frequently occurring adverse reaction (160). Although seizures have been reported, they seem to occur at about the same rate in untreated patients. Information is not available, however, on whether the incidence of any adverse reaction is statistically different compared with untreated patients. Many of the side effects attributable to recombinant erythropoietin therapy, such as hypertension, may be the result of the natural progression of chronic renal failure.

The occurrence of hypertension in treated patients is a particularly important side effect, since the majority of chronic renal failure patients already have high blood pressure (38). The incidence of seizures, although not significantly different from untreated patients, appears to occur most frequently during the early stages of therapy as the hematocrit is increasing (160). Iron deficiency occurs because iron is necessary for erythropoiesis, the process of red blood cell formation (23).

Studies are underway to evaluate the use of recombinant erythropoietin for other anemias, including anemia associated with human immunodeficiency virus (HIV), rheumatoid arthritis, and cancer. The efficacy of recombinant erythropoietin in increasing the donation of autologous blood prior to elective surgery is also under investigation.

The Structure of the Recombinant erythropoietin Marketplace

Scientists have long recognized the medical importance of erythropoietin in regulating red blood cell production. erythropoietin was first purified from human urine in 1977; however, naturally-produced human erythropoietin was an unacceptable treatment alternative because of an inability to collect and adequately purify sufficient quantities for human administration (102).

In the mid 1980's, several biotechnology firms attempted to make erythropoietin for therapeutic use. Two of the manufacturers were Amgen Inc., of Thousand Oaks, CA and the Genetics Institute of Cambridge, MA. Amgen developed and patented genetic material that is an important component needed for the production of recombinant erythropoietin in Chinese hamster ovary (CHO) cells. Genetics Institute developed and patented a method to purify erythropoietin (6).

To market recombinant erythropoietin in the United States, each manufacturer entered into a licensing agreement with other manufacturers. Except for chronic renal failure patients on dialysis, Amgen Inc. licensed its domestic rights for recombinant erythropoietin to the Ortho Pharmaceutical Corporation of Raritan, NJ. Genetics Institute licensed its domestic rights to Chugai Pharmaceutical Company of Japan, which in turn licensed its U.S. rights to Chugai-Upjohn, Inc., of Rosemont, I1, a joint venture of the Chugai Pharmaceutical Company of Japan and the Upjohn Company of Kalamazoo, MI. Continuing disputes over patent rights between Amgen Inc. on the one hand and Genetics Institute and Chugai on the other, and over the licensing agreement between Ortho Pharmaceutical Corporation and Amgen Inc. have resulted in legal proceedings that are still unresolved. The results of these proceedings have major implications for the number of suppliers of recombinant erythropoietin that will be on the market.

The Orphan Drug Act of 1983 provides incentives for manufacturers to develop products for rare diseases, currently defined as conditions afflicting fewer than 200,000 individuals in the United States. When a sponsor files an application for FDA to approve a new product for marketing, the sponsor may also apply for FDA to designate the product an orphan. Several sponsors of the same product may receive orphan designations for the same rare condition, but FDA grants a 7-year period of market exclusivity for that condition only to the sponsor who first receives FDA approval to market the product. To date, FDA has approved only Amgen's Epoetin alfa and has granted 7-year market exclusivity only to Epoetin alfa for anemia associated with chronic renal failure.[6]

Ortho's product has orphan designation for the use of recombinant erythropoietin for anemia associated with HIV[7] and with preterm infancy (54 CFR 16295). A product may have orphan drug designation and obtain market exclusivity for multiple

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6 FDA will refer to recombinant erythropoietin in general as **Epoetin** and will add the suffix **alfa,** beta, or gamma, etc. for different recombinant erythropoietin (160). At the time of this Special Report, the United States Adopted Names Council, the organization charged by FDA with assigning names to new compounds, had assigned the name **Epoetin alfa** to **Amgen's** product and **Epoetin** beta to Chugai-Upjohn's product. FDA, however, which makes the final determination on names assigned to new products, had not assigned **Epoetin** beta to any product.

7 **Ortho** submitted a Product Licensing Application **(PLA)** to the FDA in February 1989 for this indication (l).

orphan conditions. Thus, if approved by FDA, Ortho's product could receive 7-years of market exclusivity for recombinant erythropoietin for these two conditions.

In addition, Ortho's and Chugai's products have each received orphan designation for anemia associated with ESRD. Structurally different products may receive 7 years of market exclusivity for the same orphan condition. If FDA finds either product structurally different from Amgen's, that company's product could theoretically be granted 7 years' exclusivity for anemia associated with ESRD or chronic renal failure.[8] By April 1990, FDA had not determined whether Chugai's or Ortho's product is different from Amgen's (142).

The existence of multiple patents, the licensing agreements made among the manufacturers, and the granting of exclusivity as orphan products to multiple brands of recombinant erythropoietin have the potential to increase the sources of supply of recombinant erythropoietin. Ortho's product, Eprex, and Chugai-Upjohn's product, Marogen, maybe on the market shortly, joining Amgen's product, Epogen. Although one might expect that the existence of competitors would lower the price of recombinant erythropoietin available to Medicare and its beneficiaries, lower prices have not necessarily followed the entry of additional manufacturers into the markets for other pharmaceuticals (100a).

Medicare payments currently dominate the domestic market for recombinant erythropoietin and constitute the primary source of revenue for Amgen, the sole manufacturer. The Medicare program will remain the predominant payer of recombinant erythropoietin for the near term, giving it substantial leverage in the marketplace, especially if there are multiple sources of supply.[9]

Medicare's Current Payment Policies

For covered beneficiaries, the Medicare program currently pays for recombinant erythropoietin administered to dialysis patients in dialysis facilities and to dialysis and predialysis patients in physicians' offices. Because the Social Security Act generally prohibits Medicare from covering pharmaceuticals that are self-administered, Medicare does not cover recombinant erythropoietin that patients administer to themselves. This restriction prevents Medicare from covering self-administration for patients who receive dialysis at home, who could number up to 18,000 beneficiaries (124).[10]

For recombinant erythropoietin administered in a dialysis facility, Medicare has set a rate of $40 for any dose under 10,000 units administered to increase a patient's hematocrit to a target level of 30-33

8 Amgen's original orphan product designation was for the use of recombinant erythropoietin for anemia associated with ESRD. Market exclusivity, however, was awarded to Amgen for the broader indication of chronic renal failure. Ortho and Chugai-Upjohn have tiled PLAs for chronic renal failure: it is not known whether their products will be approved for a broader indication or if the orphan drug designation will be expanded (142).

9 Since Medicare pays the medical expenses for approximately 93 percent of U.S. dialysis patients, it will continue to dominate payments for recombinant erythropoietin in this market. At present, Medicare also covers recombinant erythropoietin for elderly and disabled predialysis patients. FDA approval of the biologic for other indications under study, including anemia associated with HIV, infant prematurity, and cancer plus autologous blood donations, would add additional beneficiaries to Medicare's coverage.

10s. 2098 introduced in the Senate and H.R. 4247 introduced in the House of Representatives would extend Medicare coverage to self-administration of recombinant erythropoietin for dialysis patients.

percent, but no higher than 36 percent.[11] Medicare pays an additional *$30* for any dose over this amount needed to raise the hematocrit to the target level (154). The Health Care Financing Administration (HCFA) used an estimate of Amgen's costs along with other factors in setting the payment rate (see app. E).[12] Medicare pays for recombinant erythropoietin as a separate item in addition to the composite rate paid to dialysis facilities for a package of services and supplies that are commonly used during dialysis treatment (154). Medicare does not pay dialysis facilities separately for any additional staff time or supplies, such as needles and syringes, that are used to administer recombinant erythropoietin; Medicare considers these expenses to be covered by the composite rate.

For administration in a physician's office, Medicare pays for recombinant erythropoietin on a fee-for-service basis and sets approved charges based on customary, prevailing, and reasonable Charges.[13] Medicare makes a monthly capitated payment, which currently averages $173, to the physician supervising the patient's dialysis-related care. For recombinant erythropoietin and other pharmaceuticals, Medicare pays these physicians an additional amount only for the product and the supplies to administer it; it considers payment for staff time to administer the product to be covered by the monthly cavitation payment. If the physician administering recombinant erythropoietin is other than the patient's capitated physician, Medicare pays for the product and supplies, and that physician must obtain reimbursement for staff time from the capitated physician (155).

Available data suggest that payments to dialysis facilities have been covering their costs. According to claims for dialysis patients processed through February 1990, the dose per treatment has averaged about 2,700 units, and Medicare's approved charge has averaged about $41 per treatment (47).[14] Based on a survey of selected dialysis facilities from November 1989 through March 1990, their product cost per treatment has averaged about $28 (slightly over $10 per 1,000 units) (85). According to one facility, its costs of labor, supplies, and financing amount to about $4 per treatment (43,90).1[5] If these non-product costs are representative of dialysis facilities generally, costs per treatment

l1 For doses under 10,000 units, Medicare's actual payment to the dialysis facility is $32 per administration, since the program covers 80 percent of the approved charge for medical semices under Part B, and patients pay the remaining 20 percent as cost sharing. At this payment rate, annual per patient costs for recombinant erythropoietin could total $6,240, 80 percent of which, or $4,992, would be paid by Medicare, and 20 percent, or $1,248, would be paid by the patient or another third-party.

l2 It was anticipated that an average of 5,000 units of recombinant erythropoietin would be administered at each of the 3 weekly dialysis sessions (129). Recent data indicate that dialysis patients are averaging 2,500 to 2,900 units per administration (47,117).

13 Determination of Medicare's approved charge is made by Medicare's contractors, known as intermediaries and carriers, based on guidelines developed by HCFA. In general, carriers make payments for outpatient services, and intermediaries make payments for inpatient services. Payment for services provided in a dialysis facility, however, are made by intermediaries, and payment for dialysis-related physician services are made by carriers. HCFA regulations define the approved charge as the lowest of 1) the physician's or supplier's customary charge for that service, 2) the prevailing charge for similar services in that locality, 3) the actual charge made by the physician or the supplier, or 4) the private business charge for comparable service (35). For injectable, Medicare advises its carriers to use prices from certain compendia of information on pharmaceutical prices to set the approved charge (155).

IL Through February 1990, HCFA contractors had processed claims submitted by about 1,400 dialysis facilities for about 31,000 patients (47).

I5'l%ese additional costs are based on current estimates for one dialysis facility in Michigan. The representativeness of this cost is not known. The facility was involved, over a 2-year period, in Amgen's clinical trials for recombinant erythropoietin. Therefore, their non-product costs are based on considerable experience in administering this biologic and may also incorporate practices continued after the clinical trials ended. Their figures did not include an allowance for fixed costs associated, for example, with building and equipment.

would total close to $32, and dialysis facilities would be averaging a profit of about $9 per treatment.

These statistics require certain caveats. Because of the different mix of patients at different facilities, a dialysis facility could be only breaking even or even incurring losses, if its patients required higher doses to respond. Furthermore, the data averaged from claims do not reflect the evolving nature of patient treatment and the dynamics of the patient population. Data from clinical studies suggest that the average dose for most patients may rise over time, at least during the initial phase of therapy. During the induction phase, before the target hematocrit was reached, about 55 percent of patients responded to doses equivalent to about 3,000 units per patient, but doses over 5,000 units were needed for 80 percent to respond (55) (see ch. 2). Although clinicians appear to be initiating therapy at low doses, the amounts may rise as substantial numbers of patients fail to respond. Doses required to maintain hematocrits at the target level could be much lower, however (see ch. 2).

At any time, the treated population consists of patients at various stages of therapy. At present, when diffusion of this therapy is progressing rapidly, new entrants would be expected to comprise a greater percentage of treated patients than during the later phases of diffusion, when most patients will be on a maintenance dose. Thus, it is possible that the average dose and the profits earned from current payment levels could change considerably over time. It is also possible that dosage levels have been influenced by the incentives of current payment methods to constrain use per treatment and to treat marginally anemic patients, as described below under option 3. Clarification of these patterns must await data on more long-term experience with therapy and Medicare claims.

DIMENSIONS FOR EVALUATING PAYMENT OPTIONS

Medicare coverage of medical services is intended to give beneficiaries financial access to medical care that can maintain and improve health or slow its deterioration. Medicare coverage of recombinant erythropoietin for anemic patients with chronic renal failure has improved financial access to a therapeutic breakthrough that is becoming the standard of care for this condition. In an analysis of the implications of alternative payment options, the likely effects on the quality of beneficiaries' care and on their financial access to care command primary attention. Especially in an era of Federal budget constraints, how a payment alternative is likely to affect Medicare expenditures and overall efficiency also weighs heavily in decisionmaking.

The payment options identified in this Special Report are evaluated according to their likely effects across these and other dimensions worthy of consideration: the quality of beneficiaries' medical care; access of beneficiaries to medical care; costs to the Medicare program, beneficiaries, and society plus overall efficiency; equity for beneficiaries and providers; technological innovation; and administrative feasibility. Payment methods that are effective in achieving some of these objectives may interfere with others. Highlighting these tradeoffs is an important part of the analysis in this chapter.

Quality of Care

By affecting incentives for providers and patients to use services, Medicare payment methods for recombinant erythropoietin may affect the quality of medical care that beneficiaries receive. The quality of care has many dimensions, reflecting the diversity of acceptable outcomes for patients, the complexity of the medical care process, and the multiple dimensions of patients' health.

Underlying evaluations of the appropriateness of care for a specific condition is knowledge about the efficacy and safety of a technology, such as recombinant erythropoietin, and its relationship to other technologies. Therapeutic technologies may bring about changes in length or quality of life, with effects on functional, physical, and psychological well-being. For patients with chronic renal failure, recombinant erythropoietin has been shown to correct anemia, reduce blood transfusions and improve functioning and well-being (see ch. 2). The risk of severe adverse events, such as seizures, appears to be minimal, and common side effects, such as hypertension, can usually be controlled.

Depending on the method and level of payment, Medicare policies may encourage providers to increase or decrease their use of recombinant erythropoietin and other services, with subsequent implications for patients' health. Similarly, through effects on patients' out-of-pocket expenses and access to care, payment policies may influence beneficiaries' decisions regarding the use of services and, ultimately, the quality of care received.

Access to Care

The concept of access refers to the ease with which a beneficiary can obtain medical care. Access relates to financial and physical barriers to obtaining a particular service. By affecting beneficiaries' and providers' costs, Medicare payment may influence both aspects of access.

Medicare beneficiaries directly bear the costs of recombinant erythropoietin and most other Part B services through an annual deductible and, for expenses greater than the deductible, through payment of 20-percent of Medicare's approved charge. If a physician's charge exceeds Medicare's approved charge, the physician may also bill the beneficiary for the balance. Given that treatment with recombinant erythropoietin can result in sizable out-of-pocket expenses for beneficiaries, in the range of $1,250 per year under the current payment method for dialysis patients, these direct financial liabilities may affect access to care. Although private supplementary insurance and Medicaid cover Medicare deductibles and copayments for many beneficiaries, financial access may still pose problems for some beneficiaries.[16] Therefore, payment methods that keep Medicare expenditures for recombinant erythropoietin at reasonable levels also afford greater financial access to beneficiaries.

16 According to a 1981 survey of ESRD patients, about 80 percent of Medicare patients receiving **hemodialysis** at home, 66 percent of patients receiving **hemodialysis** from a center, and 74 percent using continuous ambulatory peritoneal dialysis and continuous cycling peritoneal dialysis (both in-center and at home) had insurance coverage supplementary to Medicare's. No information was available, however, on the portion of Medicare deductibles and copayments that were covered. (94)

Medicare's payment policies may also affect how out-of-pocket expenses are distributed across beneficiaries. Payment methods that result in copayment extremes may be more harmful to access, particularly for those without Medicaid or other supplementary coverage, than methods that keep these direct beneficiary costs to more uniform levels. For example, under fee-for-service payment for recombinant erythropoietin, patients requiring high doses could incur out-of-pocket expenses many times the more uniform out-of-pocket expenses that beneficiaries now incur under the current per-treatment payment to dialysis facilities.[17]

In addition, Medicare policy may affect beneficiaries' access through restrictions on the settings in which services are covered. For example, because Title XVIII of the Social Security Act does not cover pharmaceuticals that patients administer to themselves, Medicare does not cover self administration of recombinant erythropoietin by dialysis patients in their homes. Especially for patients who receive dialysis at home, travel to physicians' offices or dialysis facilities to obtain the biologic could prove inconvenient.

Besides more direct effects on beneficiaries, financial incentives imparted by payment methods to the providers of recombinant erythropoietin may affect access. Depending on the payment method, providers may have a financial incentive to treat low-dose, less costly patients who would benefit only marginally from this biologic or to deny appropriate treatment to high-dose, more costly patients.

Costs *and Efficiency*

Costs and efficiency refer to the use of resources that are implied by a payment option, especially when measured against alternative uses to which those resources could be put. The costs of recombinant erythropoietin are directly borne by the Medicare program and, through deductibles and copayments, by beneficiaries. Indirectly, these costs are borne by society through taxes and by beneficiaries through Medicare premiums.

The total costs of recombinant erythropoietin to the Medicare program depend on the quantity consumed, Medicare's payment rate, and resulting effects on the use and cost of related medical services. Medicare's costs represent alternative uses of public and private resources and should be balanced against the health benefits gained from the biologic. Additional dollars spent on recombinant erythropoietin may be taken from other worthy areas, both public and private, to which society's limited resources may be allocated. Therefore, payment methods should encourage an allocation of resources between recombinant erythropoietin and other areas that is socially desirable.

Payment methods should also encourage distributors and providers to set prices that reflect the least-cost method of producing or providing a product and do not include a higher profit than is necessary to compensate for these activities. Higher prices

17 According to claims from dialysis facilities processed during November and December 1989, the dose per treatment with recombinant erythropoietin ranged from fewer than 1,500 units to over 10,000 units (47). If providers charged $10 for each 1,000 units, charges for the product alone would total $100 per treatment for patients receiving 10,000 units per treatment. Based on the 20-percent coinsurance for Medicare, annual out-of-pocket expenses for patients without supplementary insurance coverage could reach more than $3,000.

imply fewer health benefits from any Medicare dollar allocated to recombinant erythropoietin. By the same token, prices that are too low may discourage socially desirable investments and, depending on their pricing strategies, may induce distributors and providers to shift costs to other payers.

Historically, health insurance coverage and methods of payment have insulated providers and beneficiaries from the financial implications of their decisions to buy and use medical technologies. The literature clearly shows that use and total expenditures have been higher the lower beneficiary out-of-pocket expense when medical services are rendered (144). One would expect that the use of recombinant erythropoietin, like other expensive therapies, would be greater with Medicare coverage. Physicians would be more likely to prescribe and patients to use the biologic the lower patient cost-sharing.

Equity

Equity relates to who pays and who benefits from Medicare's policies. In public finance generally, equity is served by treating similarly people in similar circumstances and by treating differently people in different circumstances.

For recombinant erythropoietin, the issue is to what extent beneficiaries' out-of-pocket expenses should vary with their use of the biologic. For example, dialysis patients who require little or no recombinant erythropoietin may look unfavorably on a payment method that significantly increases their out-of-pocket expenses. Beneficiaries may feel that their direct payments for recombinant erythropoietin should be commensurate with their use. Historically, out-of-pocket expenses, whether for private insurance or for Medicare coverage, have varied with the use of services.

For providers of recombinant erythropoietin, equity is served if payments reflect any differences in their costs that are associated with operating in different markets. For example, some providers in small and geographically remote markets may incur higher unit costs for recombinant erythropoietin because of smaller purchases and costlier transportation expenses. Other providers of recombinant erythropoietin may serve patients who, on average, require larger doses of recombinant erythropoietin. Such market-related costs, if not incorporated into Medicare's payment rates, could adversely affect providers' finances and, in turn, beneficiaries' access to care. In addition to possible effects on beneficiaries, Government agencies have an obligation to treat providers, distributors, and manufacturers fairly, especially when the Government commands a predominant role in the market, as it does with recombinant erythropoietin.

Technological Change

Through its influence on the market, Medicare payment policies can shape the direction and extent of innovation in medical technologies. How Medicare and others pay for the services associated with a technology determines the total revenue and profitability of that product. Moreover, the market's response to a product or class of products sends a signal to potential innovators and investors in that field; successful ventures encourage future investments in similar undertakings, while failures retard their development.

Because Medicare promises to remain the primary payer of recombinant erythropoietin for the near future, the program's payment policies will have a substantial effect on the total market for the product. Over time, Medicare's policies are likely to influence investment in endeavors perceived as similar, namely research on innovative pharmaceuticals that employ biotechnology and on other products for which Medicare would be the dominant payer.

Especially when Medicare accounts for a substantial share of the market, as it does for recombinant erythropoietin, it is advisable that policymakers at least consider the implications of payment on the industry that develops, distributes, and administers it. More controversial is the responsibility of Medicare, as opposed to other Federal programs, to encourage worthwhile innovation. Even if one accepts that the Federal Government has some responsibility to foster worthwhile innovation, it is not clear that the Medicare program, as opposed to a Federal program charged specifically with that mission, should pursue that objective if it conflicts with Medicare's role as a prudent payer of medical care for its beneficiaries.

Administrative Feasibility

The final dimension for evaluating a payment option is the administrative feasibility of implementing the measure proposed. This aspect of the analysis questions how easily Medicare's administrative structure and the country's arrangements for producing, distributing, prescribing, and dispensing drugs could incorporate the proposed change.

A complex structure to support HCFA'S administration of the Medicare program is already in place. HCFA's intermediaries administer payment for inpatient and dialysis services, and its carriers administer payment for physician services and most outpatient services. End-stage renal disease networks and peer review organizations are responsible for reviewing the quality of care provided to beneficiaries.

Most pharmaceutical products are readily available to patients and health care professionals through multiple distribution outlets across the nation. Traditionally, pharmaceutical products are shipped from the manufacturer, often through a wholesaler, to a point of distribution or administration to patients, such as physicians' offices, hospitals, pharmacies, and dialysis facilities.

Some of the potential payment options would require changes in the product and financial flows or in procedures for setting payment rates and assessing the quality of care. The extent to which these changes would pose a burden to beneficiaries, providers, distributors, manufacturers, or Government administrators represents an important aspect of an option's implications.

A further administrative consideration relates to updating payment arrangements in response to dynamic changes in the market for recombinant erythropoietin. As market conditions (e.g., the number of manufacturers, the medical conditions approved by FDA and Medicare's prominence in the market) evolve, it will be necessary for HCFA to reassess the appropriateness of the level and perhaps even the method of payment.

OPTIONS FOR MEDICARE PAYMENT

This Special Report analyzes nine options regarding Medicare payment of recombinant erythropoietin. Two general options discuss Medicare coverage of self-administered recombinant erythropoietin and Medicare payment to encourage needed research on the biologic. The other options relate to methods of paying providers or to methods of paying for the product (see table l-l). Each option is evaluated across the six dimensions described above; table 1-2 summarizes the findings for each option.

The implications of each option depend not only on its inherent qualities but also on the market circumstances under which it is applied. The market for recombinant erythropoietin is dynamic, with substantial changes likely in the next few years in the number of manufacturers and in Medicare's share of the market. In the near term it seems likely that between one and three firms will supply the market. It also seems that Medicare will, for some time, be the dominant payer for the use of this biologic.

With a single manufacturer of recombinant erythropoietin, Medicare's options for setting payment rates would be more limited. For example, using a competitive approach to determine payment rates for the product would not be feasible. Medicare would have to rely on an alternative method, such as setting a rate based on

Table l-l-Options for Congress to Address Medicare Payment Related to Recombinant erythropoietin

General Options	
Option 1:	Amend the Social Security Act to allow Medicare coverage of recombinant erythropoietin self-administered by patients.
Option 2:	Mandate the Medicare program to set different payment rates for providers who participate in approved clinical trials of recombinant erythropoietin.
Provider Payment Options	
Option 3:	Mandate the Medicare program to set a fixed rate per recombinant erythropoietin treatment.
Option 4:	Mandate the Medicare program to include payment for recombinant erythropoietin in the composite rate paid dialysis facilities and the monthly cavitation rate paid physicians for dialysis patients.
Option 5:	Mandate the Medicare program to pay for recombinant erythropoietin on the basis of customary, prevailing, and reasonable charges (CPR).
Option 6:	Mandate the Medicare program to pay for recombinant erythropoietin according to a fee schedule.
Product Payment Options	
Option 7:	Mandate the Medicare program to base payment rates for recombinant erythropoietin on manufacturer costs.
Option 8:	Mandate the Medicare program to set the payment rate at the lowest price for recombinant erythropoietin listed in the Federal Supply Schedule.
Option 9:	Mandate the Medicare program to set payment for recombinant erythropoietin through competitive bidding.

SOURCE: Office of Technology Assessment, 1990.

Table l-2-Summary of Analysis of Options for Medicare Payment for Recombinant Erythropoietin

Options	Dimensions for evaluation: Costs and efficiency	Access	Quality	Equity	Technological innovation	Administrative feasibility
GENERAL OPTIONS:						
Option 1: Coverage of self-administration	Slight to moderate increase in use end higher coats to Medicare. Decreased Medicare and beneficiary costs for patients otherwise administered product in physicians offices, depending on payment rate.	Improved access for beneficiaries on home dialysis.	Improved because of better access.	Equity Improved for home dialysis patients.	Higher revenues for manufacturers and perhaps greater incentive for innovation.	Little administrative change needed.
Option 2: Different payment for providers in clinlcal trials	Increased short-term coats to Medicare. May transfer research costs from manufacturers to Medicare. Over time, knowledge gained may reduce dose and associated expenditures.	Improved if option speeds FDA approval and access for indications not yet approved.	Improved knowledge about appropriate dose gained more quickly.	Improved for beneficiaries if knowledge gained expedites FDA approval for other indications.	Spur to innovation, if overall use rose and option was used for other technologies.	Administration of studies would increase Medicare costs moderately.
PROVIDER PAYMENT OPTIONS:						
Option3: Fixed rate par rHuEPO treatment	Incentive to reduce and to treat low-dose cases. Moderate costs for Medicare, depending on payment level. Moderate and fairly uniform out-of-pocket costs for beneficiaries.	Moderate financial access for beneficiaries.	Incentive to reduce dose below clinically appropriate levels and to treat low-dose cases.	May be moderately to highly inequitable for beneficiaries end providers.	Moderate stimulus for technological innovation.	Need to differentiate and update provider payments. Moderate to strong need for peer review to assess overuse and underuse.
Option4: Payment for rHuEPO treatment in conposite rate	Strong incentive for providers to skimp on use. Low coats for Medicare, depending on payment level. Uniform coats for beneficiaries.	High financial access for beneficiaries. Incentive for providers to deny access.'	Major incentive for providers to reduce dose below clinically appropriate level.	May be highly inequitable for beneficiaries and providers.	Reasonable threat of insufficient stimulus for technological innovation.	Need to differentiate and update provider Payments. Strong need for peer review to counter underuse.
Option5: CPR payment according to units of rHuEPO used	Strong incentive for providers to increase dose and to raise charges to Medicare. High coats to Medicare. High and variable out-of-pocket costs to beneficiaries, with strong likelihood of extremes.	Cost-sharing higher and financial access lower for high-dose patients. No incentive for providers to deny access.	Incentive for providers to increase use above clinically appropriate level.	Moderately equitable for beneficiaries and providers.	Likely to stimulate excessively innovation.	Administratively complex. Strong need for peer review to counter overuse.

Table 1-2--Summary of Analysis of congressional Options for Medicare Payment for Recombinant Erythropoietin-continud

Opt ions	Dimensions for evaluation: Costs and efficiency	Access	Quality	Equity	Technological innovation	Administrative feasibility
Option 6: Fee schedule according to units of rHuEPO used	Incentive for providers to increase dose, depending on payment rates. Moderate to high costs to Medicare, depending on payment level. Moderate and variable out-of-pocket costs to beneficiaries, with possible extremes.	Moderate financial access for beneficiaries. Little or no incentive for providers to deny access.	Incentive for providers to increase dose above clinically appropriate level, depending on payment level. Little or no incentive to reduce dose below this level.	Highly equitable for beneficiaries. Moderately equitable for providers.	Moderate stimulus for technological change.	Need to differentiate and update payments. Moderate need for peer review to counter overuse and underuse.
PRODUCT PAYMENT OPTIONS:						
option 7: Based on manufacturer costs	Risk that product price may be set too high or too low. Low price implies lower costs to Medicare and beneficiaries and cost shifting to other markets and products. High price implies higher cost to Medicare and beneficiaries and possible substitution of less effective therapies.	Low product price implies greater access for beneficiaries, and high price reduced access.	High product price may result in use below clinically appropriate levels. Low price per se should not affect use.	No implications for beneficiary or provider equity.	Low price may discourage technological innovation, while high price may provide excessive stimulus.	Calculation of an appropriate price is very difficult administratively. Logistics of implementation may be difficult.
Option 8: Buy from federal Supply Schedule	Substantial risk that product price will be set too high. Little or no risk it will be too low. High price implies high costs to Medicare and beneficiaries and potential substitution of less effective therapies.	High product price implies reduced access for beneficiaries.	High product price may result in use below clinically appropriate levels.	No implications for beneficiary and provider equity.	High product price may excessively stimulate technological innovation.	Logistics of distribution and financial flows may pose moderate administrative problems.
Option 9: Competitive bidding among manufacturers	Risk that product price will be set too low. Little or no risk it will be too high. Low prices imply low costs to Medicare and beneficiaries, but also may result in cost shifting to other markets and products or exit of new or small firms.	Low product price implies greater access for beneficiaries.	Low product price may stimulate overuse.	No implications for beneficiary and provider equity.	Low product price may discourage technological innovation.	Manufacturers/ participation uncertain. Logistics of dividing market and financial flows may pose difficult administrative problems.

KEY: rHuEPO = recombinant erythropoietin

SOURCE: Office of Technology Assessment, 1990.

its assessment of the manufacturer's costs. The presence of a single manufacturer would affect provider payment options as well. If separate providers face a monopolist manufacturer of recombinant erythropoietin, they may not have sufficient market leverage to influence the prices that they must pay for the product. As a consequence, using methods of paying providers to encourage them to more prudently purchase the biologic would be ineffective and might impair beneficiaries' access and the quality of their care. Therefore, under this market scenario, a payment option that placed less risk on the provider might be more appropriate. Also implied here is the stronger need for Medicare to apply directly its market leverage to set a payment rate for the product.

The presence of multiple manufacturers of recombinant erythropoietin would pose a contrasting market situation with different implications for payment options. Although Medicare could then apply a more competitive approach for obtaining a lower payment rate for the product, there would be less need for Medicare to use its market leverage to achieve this objective. With multiple manufacturers, provider payment methods that encouraged prudent purchasing might be capable of achieving significantly lower rates for the product. Indeed, whether it is desirable for Medicare to set a rate that it pays manufacturers for recombinant erythropoietin depends on whether any provider payment method, by itself, would be sufficiently effective across the range of dimensions to be considered.

The options considered below are not mutually exclusive. Options 1 and 2, the general options on coverage and research, could be implemented with any of the other options. Any of the options for paying providers of recombinant erythropoietin (options 3, 4, 5, and 6) could be combined with any of the options for paying for the product (options 7,8, and 9). Even within the provider options and the product options, more than one alternative could be adopted. Furthermore, these payment options are not limited to anemia associated with chronic renal failure, the only condition that Medicare currently covers; these options and the analysis of their implications apply to other conditions for which the biologic may be covered in the future.

General Options

Option 1: Amend the Social Security Act to allow Medicare coverage of recombinant erythropoietin self-administered by patients.

Title XVIII of the Social Security Act prohibits Medicare coverage of most pharmaceuticals, including recombinant erythropoietin, insulin, and most prescription drugs, that beneficiaries administer to themselves. Although these patients manage to administer dialysis treatments and related medical services at home, they must travel to their supervising dialysis facilities or physicians' offices to receive recombinant erythropoietin covered by Medicare. The time and inconvenience required may pose significant physical and financial barriers for many patients. As FDA approves and Medicare covers more indications, these restrictions will inconvenience more beneficiaries.

Under this option, Congress would amend the Social Security Act to allow Medicare to cover recombinant erythropoietin when self-administered by patients.

In regulations implementing the amendment, the Medicare program could specify the conditions under which use would be covered, such as for indications approved by FDA and for a certain level of anemia. The expanded coverage could be restricted to patients who receive dialysis at home. In fact, legislation pending in the Senate (S. 2098) and the House of Representatives (H.R. 4247) would mandate Medicare coverage for home dialysis patients.

Dialysis patients could obtain their recombinant erythropoietin and related supplies from their dialysis centers or dialysis distributors, which would both be responsible for billing Medicare. Options 3,4,5, and 6 discuss different methods that could be used to set payment rates for providers. If legislation covered self-administered recombinant erythropoietin for all FDA-approved conditions, these provider payment options could also apply to dialysis distributors and pharmacies.

Overall Medicare expenditures would increase, if this option was implemented. Easing financial and physical barriers to access typically increases use and expenditures. Physicians would be more likely to prescribe recombinant erythropoietin, especially for patients who receive dialysis at home or who have difficulty traveling. Patients' use would rise because of greater convenience and reduced costs related to travel and perhaps work loss.

The effect on program costs of beneficiaries who shift to self-administration from administration in other settings is less straightforward. Medicare pays supervising physicians who receive monthly cavitation payments for dialysis patients an additional amount for the product, but not for administering it. Medicare also pays non-supervising physicians who administer recombinant erythropoietin to dialysis patients only for the product; these physicians must bill the supervising physicians for administering the biologic. Therefore, the shift from physician to self-administration resulting from this option would not save the Medicare program any expenditures associated with administering the product.

Medicare's approved charge for the product to physicians administering recombinant erythropoietin in their offices, however, may exceed the amount that Medicare currently pays dialysis facilities ($40 per 10,000 units or fewer of the biologic). If, as specified in S.2098 and H.R. 4247, home dialysis patients were required to obtain the biologic from their supervising dialysis facilities or dialysis distributors and these providers were paid the same lower rate, Medicare's per patient expenditures could be lower. If the option applied to patients other than those on dialysis, Medicare could restrict the payment rate to the lowest paid in any setting.

Reductions in blood transfusions and other services for anemic patients would partly offset any increase in program expenses from improving beneficiaries' access to recombinant erythropoietin. One study estimated total annual savings for blood and related services at about $1,600 per transfusion-dependent patient and savings for androgens at about $900 per patient receiving them (68). Savings might also arise from reductions in untoward consequences of transfusions, such as therapy for hepatitis contracted through transfusion. In addition, transfusions may induce antibodies that lower the likelihood of successful kidney transplantation. Since

costs to Medicare of patients with successful transplants are substantially less than those remaining on dialysis, over time Medicare may reap additional savings for averted transfusions.

If Medicare restricted coverage under this option to recombinant erythropoietin obtained from a dialysis facility or distributor, out-of-pocket expenses for patients on home dialysis would be the same as those for patients receiving dialysis in facilities. Under current Medicare payment to dialysis facilities, patient out-of-pocket costs for up to 10,000 units are limited to $8 (20 percent of $40) per treatment with recombinant erythropoietin. If Medicare extended coverage under this option to indications other than dialysis and limited payment to the rate paid dialysis facilities, beneficiaries would incur the same level of out-of-pocket expenses.

Patients who self-administer the biologic would save other direct costs relating to time and travel to physicians' offices and dialysis facilities. Beneficiaries who would otherwise not have received the product would now incur the related out-of-pocket costs, but they would also gain whatever health benefits resulted from taking recombinant erythropoietin.

In response to a query from the Senate Finance Committee, the Congressional Budget Office (CBO) during the process of budget reconciliation in fall 1989 estimated the effect on Federal expenditures of covering self-administration of recombinant erythropoietin for dialysis patients (71). Assuming that half of an estimated 24,000 home dialysis patients would opt for self-administration, CBO concluded that coverage for dialysis patients would raise Federal outlays about $40 million for fiscal year 1990.[18] This estimate made no allowance for reductions in blood transfusions and other services or for patients who are currently receiving recombinant erythropoietin from a dialysis facility or physician's office. Using updated figures on home dialysis patients, CBO is re-estimating the budgetary implications of covering self-administration.

The net effect of this option on the quality of care would combine positive health benefits from alleviating anemia in newly treated beneficiaries with any negative effects associated with self-administration. How Medicare administered the benefit could greatly influence the quality of care. Medicare has already instructed its intermediaries to restrict payment to claims demonstrating hematocrit levels in a certain range and could apply those restrictions to self-administered use as well. If safety was a concern, Medicare could require that patients obtain recombinant erythropoietin from a medical provider during the induction phase and stipulate that the program would cover self-administration only after a maintenance dose is achieved. Peer review organizations (PROS) or end-stage renal disease (ESRD) networks could also assess the appropriateness of recombinant erythropoietin use, whether the drug was administered by a patient or a medical provider (see ch. 4).

The literature contains limited information on self-administration of recombinant erythropoietin. A few clinicians

18 Since coverage would have begun on Jan. 1, 1990, expenses related to only part of the fiscal year. CBO's original estimate included about $5 million additional expense for fiscal year 1990 as a result of Medicare payments associated with limits on beneficiaries' liability that were related to provisions of the Medicare Catastrophic Coverage Act of 1988 (71). Those provisions no longer apply because that Act has been repealed.

have reported that patients on home dialysis administered the biologic intravenously or subcutaneously with no unusual safety problems (see ch. 2).

To the extent that expanded coverage of recombinant erythropoietin increased the market for the product, further innovation in this and related fields would be stimulated. In addition, implementation of this option would be possible within current administrative structures. To determine payment rates for the product, Medicare could combine the techniques and information used to set payment rates for recombinant erythropoietin in other settings. As noted, under S.2098 and H.R. 4247, home-dialysis patients self-administering this biologic would be required to obtain it either from their dialysis facilities or distributors, both of which would be paid according to the same method. Applying existing procedures, PROS and ESRD networks could review the quality of care for home dialysis as well as for other patients taking recombinant erythropoietin.

Option 2: Mandate the Medicare program to set different payment rates for providers who participate in approved clinical trials of recombinant erythropoietin.

Although clinical trials have shown recombinant erythropoietin to be efficacious in correcting anemia among patients with chronic renal failure, some important clinical questions remain unanswered. Clinicians require clarification mainly about appropriate dosing regimens, both for intravenous and subcutaneous administration, and also about the safety and effectiveness of patients' self-administering the biologic. Beyond these immediate needs is information on the efficacy and safety of the product for other indications, including autologous blood transfusions and anemia associated with HIV.

Resolution of these outstanding questions could result in cost savings for the Medicare program. It is not uncommon for the effective dose of a drug eventually to be found to be substantially lower than the amount originally approved by FDA. For example, doses about half those first approved have been shown to be effective for treating acquired immunodeficiency syndrome (AIDS) with zidovudine (162). Many clinicians are now starting their dialysis patients on doses of recombinant erythropoietin that are much lower than those approved by FDA (see ch. 2).

Despite the potential advantages from improved information, providers are often reluctant to participate in clinical trials for an unapproved technology. Not only do participants incur higher costs associated with recordkeeping and protection of human subjects, but also third-party payers, such as Medicare, may not pay for the technology or associated services. In the case of recombinant erythropoietin, which FDA has approved for patients with chronic renal failure and Medicare covers for the approved indication, providers caring for patients with this condition may have no financial incentive to participate in trials.

Under this option, Congress would mandate the Medicare program to use different payment arrangements to encourage providers' participation in approved proto-

cols to refine clinical information on recombinant erythropoietin.*9 Working with clinicians, manufacturers, and FDA, Medicare could identify the specific information desired, with priority to research questions that had implications for improving patients' health and moderating Medicare's costs. These research questions could pertain to chronic renal failure or to other indications not yet approved by FDA and covered by Medicare. For treating medical conditions not approved by FDA, Medicare could make payment to providers under this option conditional on their participation in a research protocol that had been approved by the FDA.

For patients with medical conditions not approved by FDA, Medicare could offer to pay for recombinant erythropoietin on the same basis that it pays for patients with chronic renal failure. Since Medicare already covers recombinant erythropoietin for chronic renal failure, however, Medicare would have to offer a higher payment rate or a payment method more desirable to providers in order to entice their participation in clinical trials. To determine payment, Medicare could use any of the methods discussed in options 3 through 6.

The immediate effect on Medicare costs would be to increase expenditures by the amount of the demonstration plus payments for conditions not approved by FDA and whatever additional payments resulted from paying providers higher rates for patients with chronic renal failure. Over time, however, the information gathered could influence Medicare's payment rates and expenditures on recombinant erythropoietin. Although the dosing regimens that will eventually be considered appropriate are uncertain, many clinicians have been treating dialysis patients at substantially lower doses than those recommended in the labeling approved by FDA (11,17,47). Although not substantiated, it has also been suggested that subcutaneous administration requires lower and less frequent use than intravenous administration (see ch. 2). Conducting more research more quickly on the efficacy of lower doses could provide a more informed basis for setting payment rates, especially for nondialysis patients who receive the biologic through subcutaneous administration in physicians' offices. Any savings for chronic renal patients might be offset by additional expenditures for treating anemia associated with other conditions. If the research data generated by this option led to more rapid approval by FDA of recombinant erythropoietin for other conditions, Medicare might experience an earlier rise in expenditures for these additional conditions.

The net effect on Medicare expenditures would thus depend on changes in the payment rate for recombinant erythropoietin, which would reflect the level and frequency of dosing; the increase in use for beneficiaries with covered conditions; and any reductions in expenditures from correcting anemia, such as fewer blood transfusions. The effect on beneficiaries' costs would parallel changes in Medicare expenditures and depend on the specific method and level of payment that Medicare adopted.

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19 The Social Security Amendments of 1983 (Public Law 98-21) gave the Secretary of Health and Human Services the authority to pay for research and experimentation related to Medicare's prospective payment system (21 USC 1395y(1)(D)). HCFA could use this authority to pay providers who agreed to gather needed information in the context of clinical trials.

On the other hand, this option might only transfer the costs of further research from the manufacturers of recombinant erythropoietin to the Medicare program and its beneficiaries. At least three manufacturers are conducting studies pertaining to safety for chronic renal failure and to efficacy and safety for certain conditions not approved by FDA. In addition, at least one manufacturer is studying different dosage levels administered subcutaneously in predialysis patients. The manufacturers bear the full cost of this research, including the cost of the biologic; physician, testing, and other clinical services; and administrative services associated with the research. Several completed studies are also being prepared for publication. Moreover, none of the manufacturers has reported difficulty in finding researchers, clinicians, or patients to participate.

The information developed from this option might improve beneficiaries' access to recombinant erythropoietin. If data were collected and other conditions were approved by FDA more quickly, beneficiaries with these conditions would gain improved financial access to the product. Also in the direction of better financial access, any reductions in Medicare's payment rates for recombinant erythropoietin because of lower dosing regimens would reduce beneficiaries' cost-sharing.

A major advantage of this option lies in its potential to improve the quality of care received by Medicare beneficiaries. Encouraging providers to participate in research protocols could be a quick and efficient way to gather data to refine appropriate dosing regimens for intravenous and subcutaneous administration and to develop information on efficacy and safety for conditions besides chronic renal failure. The quality of beneficiaries' care would clearly benefit from better information on efficacy and safety. Clinicians would have a more valid basis on which to prescribe recombinant erythropoietin, and the Medicare program would have a more valid basis by which to evaluate appropriate use. The outstanding question, however, is whether this option would produce the desired information more quickly than the manufacturers' own testing, and if so, whether the benefits would be worth the extra cost to the Federal Government.

Developing better information on efficacy and safety more quickly could improve equity among beneficiaries. As that information led to decisions about FDA approval and Medicare coverage for other conditions, use by beneficiaries with conditions that, like chronic renal failure, would benefit from recombinant erythropoietin would also be covered. One would expect equity among providers to improve, as the new information enabled Medicare to reimburse providers for efficacious and safe uses of recombinant erythropoietin and to withhold payment for other uses.

It is not clear how the research results on balance would affect the size of the market for recombinant erythropoietin and consequent incentives for future technological innovation. Other medical conditions might receive FDA approval more quickly, but the appropriate dose might prove to be lower. More important than the market size for this particular product would be the potential for using this

mechanism to stimulate assessments of new technologies. Patient groups, clinicians, third-party payers, researchers, and manufacturers have lamented the financial obstacles to producing valid information on new technologies or on new uses of existing technologies. Using this mechanism to generate research on recombinant erythropoietin would be viewed as a test case for a possible model to develop the information needed for assessments.

Although this option would require that HCFA and its contractors establish some new procedures, much of the required administrative apparatus is already in place. FDA and Institutional Review Boards already approve in advance the design of clinical trials on human subjects for biologics seeking approval for new conditions or changes in existing labeling. HCFA could notify its carriers and intermediaries that dialysis facilities and physicians engaged in approved trials were eligible for different payment rates, and the contractors would have to institute procedures to identify these providers. A necessary element not yet in place, however, is a locus for synthesizing the research results and applying them to refine Medicare policy. Such a role would be consistent with the mandate of the recently created Agency for Health Care Policy and Research, which is charged with developing information on the effectiveness of medical technologies and, in concert with the medical community, with setting guidelines for clinical practice.

Payment to Providers

At present, dialysis facilities are the principal providers of recombinant erythropoietin to beneficiaries, a situation that reflects FDA approval and Medicare coverage only for chronic renal failure. Beneficiaries with chronic renal failure, whether in the predialysis or dialysis phase, may also receive the biologic from physicians' offices. Hospitals' provision of recombinant erythropoietin to inpatients is covered by payments that are fixed by diagnosis-related group, while provision of the biologic by health maintenance organizations (HMOs) and other competitive medical plans is covered by Medicare's monthly cavitation payment. Amgen has been selling Epogen exclusively to wholesalers, who in turn sell it to dialysis facilities, physicians' offices, and others who provide the biologic to patients. Wholesalers may also sell to other intermediate suppliers, such as pharmacies. If FDA approves recombinant erythropoietin for other conditions or if legislation extends coverage to self-administration, as described in option 1, dialysis distributors and pharmacies could also provide the product to beneficiaries. If additional manufacturers enter the U.S. market, they may choose to sell directly to other intermediate suppliers or to dialysis facilities and other providers.

Options 3, 4, 5, and 6, respectively, pertain to methods that Medicare might adopt to set rates paid to providers: payment per recombinant erythropoietin treatment; inclusion of payment for recombinant erythropoietin in the composite rate for dialysis facilities and the cavitation rate for supervising physicians; payment based on customary, prevailing, and reasonable charges; and payment according to a fee schedule. All of these options could be applied to dialysis facilities, physician providers, and dialysis distributors. The set of

feasible options for pharmacies is more limited. Option 4 does not apply to pharmacies and, although option 3 is theoretically possible, options 5 and 6 would be most practical for pharmacies. Options for hospitals, competitive medical plans, and nursing homes are not considered separately in this report.

Medicare's payment to dialysis facilities and physicians would include compensation for several components: the biologic itself; any associated supplies or services; and the physician's or other health professional's services to administer the product to a patient. If Medicare coverage was extended to self-administration, as described in option 1, payment to dialysis distributors and pharmacies would compensate for the biologic, any associated supplies or services; and any professional counseling.

In the next section, options 7, 8, and 9 discuss methods that Medicare could use to set the rate that it pays for the product itself. If the payment rate for the product is set by Medicare through an agreement with a manufacturer or manufacturers, then it would be logical to incorporate that rate into the calculation of provider payment levels. On the other hand, if Medicare does not set the rate for the product or if a manufacturer conveys price concessions directly to Medicare rather than to providers, then some alternative basis for determining provider payment levels would be" necessary. To set current payment rates for dialysis facilities, HCFA estimated providers' costs of obtaining recombinant erythropoietin based on HCFA's assessment of Amgen's costs of producing the biologic and other factors affecting the costs of providing the service, such as expected dosage levels.

Other methods are also possible for estimating providers' costs for the product as an ingredient in setting Medicare payment rates to providers. For example, Medicare might use the average wholesale price (AWP) for the product. Some Medicare carriers may be using the AWP to derive an approved charge for physicians who administer recombinant erythropoietin in their offices. Average wholesale prices, however, are usually list prices instead of the transaction prices that providers actually pay for pharmaceuticals. Although the level of Medicare payment to providers has major importance, the options presented here are structured according to methods of payment and do not consider in depth how to calculate the level of payment.

A general issue that applies to providers of recombinant erythropoietin is whether they should be required to accept assignment. Under assignment, a provider agrees to accept a beneficiary's rights to benefits, to bill the Medicare carrier instead of the patient, and to accept Medicare's payment rate as full payment for the service rendered. Current law requires providers to accept assignment for patients of dialysis facilities and dialysis distributors and for inpatients in hospitals. Furthermore, in the context of mandating transition to Medicare payment for physician services according to a fee schedule, the Omnibus Budget Reconciliation Act of 1989 (Public Law 101-239) established limits on the extent that physicians who do not accept assignment may bill beneficiaries in excess of Medicare's set rate. If pharmacies become providers in the future, Medicare could require them to accept assignment or could restrict the extent to which their charges to beneficiaries may exceed Medicare's approved rate.

Another consideration in setting provider payment rates concerns the choice between uniform and differentiated payments. Under the latter approach, payment levels would vary to reflect fundamental differences among providers. Providers of recombinant erythropoietin may serve different markets and consequently, may incur different costs of providing this service. Differentiated payments are generally more equitable, especially if they afford more even access to beneficiaries (84).

Financial incentives inherent in different payment methods influence providers' and patients' decisions about using medical services, such as recombinant erythropoietin. Payment methods that place greater financial risk on providers contain stronger incentives for them to constrain use and could result in underprovision of the product and poorer quality care. Such methods would also more strongly encourage providers to prudently purchase recombinant erythropoietin. On the other hand, payment methods that place providers at less financial risk contain stronger incentives for greater use and perhaps overprovision of the product and poorer quality care. Generally, the financial risks to providers are stronger the larger the units on which payment is based. For example, payment per treatment with recombinant erythropoietin places more financial risk on the provider than payment per unit of the product.

Levels of payment also affect use and the quality of care received by beneficiaries. For any given payment method, lower payment levels are likely to discourage use, while higher levels encourage greater use. The extent to which use varies with payment levels also depends on the incentives inherent to each payment method. For example, when the amount of payment does not vary with the volume of service, higher payment levels are less likely to result in more use than when the amount of payment does vary with volume.

As noted above, options 3, 4, 5, and 6 are not mutually exclusive; Medicare can and does use different methods to pay providers in different settings. On grounds of efficiency and equity, however, it is preferable that Medicare pay the same amount for the same service, regardless of the setting in which it is provided. Paying a higher amount in one setting, such as a physician's office, provides a financial incentive for a provider to administer the service in the most lucrative setting, regardless of where the service could be most effectively and efficiently provided. Paying different amounts for different settings may also be inequitable, if beneficiaries and providers in similar circumstances are treated differently.

Option 3: Mandate the Medicare program to set a fixed rate per recombinant erythropoietin treatment.

Medicare currently pays dialysis facilities a fixed amount of $40 per recombinant erythropoietin treatment, which increases to $70 if the dosage level exceeds 10,000 units (see ch. 4). S. 2098 and H.R. 4247 would apply this payment method to dialysis distributors for recombinant erythropoietin self-administered by home dialysis patients. This method could also be applied to physicians, but would be least practical for pharmacies.

Since under this option the amount reimbursed would not vary with the quantity of recombinant erythropoietin

administered in each treatment up to a threshold, this payment method contains a financial incentive for providers to control and even skimp on use. The fixed payment also encourages providers to make prudent purchases of the product.

Providers would also have a financial incentive to treat patients who would require especially low doses but who would gain little marginal benefit from treatment. The costs of such cases would be substantially below the payment per treatment. The consequence of this behavior would be greater numbers of beneficiaries receiving treatment and higher costs to the Medicare program. ESRD networks and PROS could monitor use for appropriateness, but PROS have had little experience in the outpatient arena.

There is some financial incentive to provide higher than the clinically appropriate dosage levels to cases just below the 10,000 unit threshold, especially if medical consequences are minor. Slight increases in dosage could nearly double the payment per treatment. Depending on the strength of this financial incentive and the proportion of cases near the threshold, costs to the Medicare program could increase significantly. Review of these claims by PROS or ESRD networks might counter overuse near the threshold.

Providers may have a financial incentive under this payment method to deny access, that is, not to administer recombinant erythropoietin when it is medically appropriate. This would not apply to patients requiring smaller dosage levels, who are likely to cost less than the payment amount. Denial of treatment might occur for patients requiring doses that are below, but closer to, the 10,000 unit threshold. Medical ethics may constrain such behavior. Although denial of access would reduce the costs of recombinant erythropoietin to the Medicare program, the costs of alternative medical services, such as blood transfusions, might increase.

If financial incentives of this option led providers to skimp on use, reduced quality of care could result, if doses fell below clinically appropriate levels. Again, this tendency would most likely depend on the seriousness of the medical consequences and the effectiveness of peer review.

It should be noted that at present, appropriate dosage levels are unclear. Dialysis facilities paid by Medicare through February 1990 averaged about 2,700 units of recombinant erythropoietin per patient per treatment, and facilities surveyed by Amgen from mid-December 1989 to mid-January 1990 averaged about 2,900 units per treatment (47,117). These rates are much lower than those recommended in the FDA-approved labeling or the 5,000-unit mean dose expected by HCFA when it set the present payment rate (5,85). These doses are also much below the mean dose that clinical trials found necessary for a response (55) (see ch. 2). Administering lower doses is consistent with the incentives of this payment method to skimp on the quantity used and to treat patients only marginally anemic. That initial doses are apparently much lower than expected, however, cannot be attributed entirely to financial incentives inherent in this payment method. Although clinical trials have shown that some patients need much greater doses of recombinant erythropoietin to respond, at present clinicians cannot determine a priori the effective dose. Consistent with usual medical practice in the face of such uncertainty,

clinicians appear to be starting with lower doses and, presumably, will raise the dose for poor responders. Over time, however, the average dose will combine the effects of newly treated patients in the induction phase, poor responders with increased doses in the induction phase, and other patients on a maintenance dose.

Under this payment method, out-of-pocket costs to most beneficiaries are fixed at $8 per treatment ($1,248 per year with 3 treatments per week). For those with doses in excess of 10,000 units, out-of-pocket costs are fixed at $14 per treatment ($2,184 per year with 3 treatments per week). Some beneficiaries may consider this distribution of out-of-pocket costs to be inequitable. For example, a beneficiary requiring very modest treatment with recombinant erythropoietin might view a $1,248 increment in out-of-pocket costs as quite unfair. This inequity would be somewhat remedied if payments to providers under this option varied to reflect differences in patient characteristics, such as weight, that affected dosing levels.

Equitable compensation of providers requires that payments be differentiated to reflect market-related differences in their costs. Some providers may treat cases who, on average, require higher dosage levels. Other providers may, because of geographic location, pay higher wages or incur higher acquisition costs for recombinant erythropoietin. Because of markups of wholesalers and other intermediate suppliers, differences in providers' acquisition costs may occur even if, as under the options in the next section, Medicare sets the price of the product with the manufacturer.[20] Under these circumstances, uniform payments might lead some providers to reduce doses below clinically appropriate levels, or to treat patients with only marginal anemia. Therefore, equity for both providers and patients and access by beneficiaries would be improved if payments were differentiated to reflect case mix and other market-related differences in cost.

This option may be less appropriate for physician providers of recombinant erythropoietin than for dialysis facilities. Because physicians treat smaller numbers of patients, they face greater financial risk from a few patients who require high doses. Since payment does not generally vary with dosage level under this option, physicians may experience considerably more incongruity between payments and costs for this service. For all providers, adjusting for patient characteristics predictive of high use is likely to prove difficult, as exemplified by problems in adjusting cavitation payment to competitive medical plans and DRG payments to hospitals. Paying pharmacies under this option would raise similar problems.

This option might affect technological innovation. If providers' incentives to lower dosage levels led to lower purchases and considerably lower revenues for manufacturers than expected, incentives to further develop this and other products

20 The wholesaler markup usually accounts for a small fraction of provider acquisition costs. According to a survey by the Office of the Inspector General conducted between November 1989 and March 1990, dialysis facilities are paying about $41 for 4,000 units of recombinant erythropoietin (85). During this period, Amgen's price to wholesalers was $10 per 1,000 units (117).

used extensively by Medicare might be dampened. The opposing financial incentive for providers to treat more low-dose cases, however, would somewhat mitigate these effect.

The administrative difficulty of this payment alternative depends on what refinements are introduced. Under the current method, payments are fairly uniform and, consequently, administratively simple. Administrative difficulty could increase substantially if payments were differentiated. Given its experience with the prospective payment system for reimbursing hospital operating expenses, the Medicare program is well aware of such difficulties. Differentiated payments, if feasible, may nevertheless be necessary to reduce the negative effects of payment methods that encourage providers to be more cost conscious. Because payment rates would be set prospectively under this option, it would also be necessary continuously to update payment levels in response to dynamic changes in the market for recombinant erythropoietin or changes in clinically appropriate dosage levels.

Option 4: Mandate the Medicare program to include payment for recombinant erythropoietin in the composite rate paid dialysis facilities and dialysis distributors and the monthly cavitation rate paid physicians for dialysis patients.

This option applies only to the payment of recombinant erythropoietin provided to dialysis patients by dialysis facilities, dialysis distributors, and physicians. Dialysis facilities and dialysis distributors are currently paid a prospective amount per dialysis treatment, which varies according to factors such as area wage costs.[21] This covers nearly all services relating to dialysis (see ch. 4).[22] Physicians treating dialysis patients are paid a monthly cavitation payment for services directly relating to this condition (34). This amount applies to patients receiving dialysis at home as well as those in facilities.

Under this alternative, the composite rate for dialysis facilities and dialysis distributors and the physician cavitation for home dialysis patients would be increased to cover the costs of recombinant erythropoietin. For dialysis facilities and dialysis distributors, the increase in the composite rate would be based on an estimate of the average amount of the product used during each dialysis session. For physicians, the increase in the cavitation amount would be based on an estimate of the average number of patients administered recombinant erythropoietin per month and the average dosage.

The principal difference between this option and option 3 is that payment would not depend on whether recombinant erythropoietin is administered. Because payment under this option depends neither on the administration of recombinant erythropoietin nor on its dosage, there are no financial incentives to treat more cases or to provide larger doses of this biologic than is clinically appropriate. This option contains stronger incentives than option 3,

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21 Consideration of the rate paid for dialysis treatment lies outside the scope of this OTA study. The Institute of Medicine Committee To Study the Medicare End Stage Renal Disease (ESRD) Program is addressing certain aspects of the rate-setting process, but is not conducting a full-scale rate-setting study (118).

22 In addition to recombinant erythropoietin, other items such as the whole bled used in transfusions are paid separately.

however, to skimp on use. Providers would also have a strong incentive to make prudent purchases of recombinant erythropoietin. The significance of this incentive is greater if Medicare does not set the price paid by providers for the product.[23] Because of the strong incentives for economy under this option, costs to the Medicare program and direct costs to beneficiaries would be kept at fixed levels. Financial access for beneficiaries might also be greatly improved because out-of-pocket extremes would not be possible.[24]

Access may be adversely affected in other ways under this alternative. Since payment is independent of treatment, providers may have a strong financial incentive to deny recombinant erythropoietin to some patients for whom its application would be clinically appropriate. Because medical consequences would be less serious, this behavior is more likely to occur with patients who are only slightly anemic. Although peer review may address this problem, inappropriate decisions regarding such patients would be difficult to detect. The financial incentives to deny access are stronger here than under the other options for provider payment. By denying access under this method, providers would save the full cost of treatment. Under option 3 they would reap only the difference between the cost and the payment per treatment.

There may also be a strong financial incentive for providers to administer doses of recombinant erythropoietin that are below clinically appropriate levels. By doing so, they would increase net revenues or reduce losses. Again, such behavior would most likely depend on the seriousness of medical consequences and the effectiveness of peer review. Under option 3 providers have the opportunity to improve net revenues by treating more low-dose patients. Since revenue does not rise with treatment under this option, there might be a greater tendency to reduce dosage levels.

Under this payment method, beneficiary out-of-pocket costs would be totally unrelated to recombinant erythropoietin use. Even patients who are not treated with this biologic would incur out-of-pocket expenses relating to its costs. For this reason, beneficiaries are likely to view this payment alternative as being far less equitable than option 3.

This payment method is likely to be even more inequitable to providers than option 3. The adequacy of compensation not only continues to vary with average dosage levels, but also varies with the proportion of dialysis cases given recombinant erythropoietin. As with option 3, inequitable compensation could also result if providers, because of different markets, incur different acquisition costs for recombinant erythropoietin, labor, or other inputs. These compensation inequities could be addressed by differentiating payments to reflect these differences among providers.

This option is even less appropriate for physician providers than option 3. Because payment is affected by neither the adminis-

23 Even if the manufacturer's price were set, providers would still have a strong incentive, under this option, to shop for the lowest wholesaler markups.

24 All dialysis patients would incur the same increase in out-of-pocket costs under this option. The increment would be 2(I percent of the increase in the composite rate or physician cavitation. The increase in out-of-pocket coats per beneficiary would also be lower under this option than under option 3, since the costs of recombinant erythropoietin would be spread across all dialysis patients rather than only those treated with this biologic.

tration of recombinant erythropoietin nor its dosage level, physicians are likely to experience even greater incongruity between revenues and costs over time. Adverse effects on patient quality and access could also be greater. The same difficulty regarding an adjustment to physician payments for relevant patient characteristics also applies here.

Given incentives for providers to skimp on use, this option could adversely affect technological innovation. If the total demand for recombinant erythropoietin fell substantially below manufacturer expectations, manufacturers could be discouraged from investing in similar therapies or in any therapies for which Medicare is a dominant payer. Because incentives to underprovide recombinant erythropoietin are stronger under this payment alternative than under option 3, the threat to technological innovation is also greater. Since higher payment levels would have little effect on use under this option and since all of its inherent incentives are for economy, there is no possibility for an excessive stimulus to technological innovation.

As with option 3, the administrative difficulty of this payment method depends on whether payment rates are differentiated to reflect fundamental differences among providers and on the extent of these refinements. Also like option 3, there is the added administrative burden of updating payment levels in response to dynamic changes in the market for recombinant erythropoietin or changes affecting appropriate dosage levels. Lastly, because of its very strong incentives for economy, it would be necessary under this option to reinforce peer review to better ensure against underprovision.

Option 5: Mandate the Medicare program to pay providers of recombinant erythropoietin on the basis of customary, prevailing, and reasonable charges (CPR).

Under this option, Medicare payment to a provider would vary according to the number of units of recombinant erythropoietin administered to a patient. The CPR method, which Medicare currently uses to pay physicians, would pay each provider an amount for the therapy that is the lesser of the actual charge, the customary charge based on the provider's previous billings, and the prevailing charge for the service by comparable other providers. Medicare could continue to permit providers who do not accept assignment to bill patients for amounts in excess of Medicare's approved charges, or Medicare could restrict providers' additional billing. As noted in option 1, physicians who receive monthly cavitation payments for supervising dialysis patients and other physicians administering the biologic to dialysis patients may charge only for the product and related supplies, not for administering it. The CPR method could also be used to reimburse dialysis facilities, dialysis distributors, and pharmacies. For these providers and for physicians administering the biologic to other than dialysis patients, payments under this option would compensate for administration or dispensing services and supplies as well as the product.

This option gives providers and patients the weakest incentives to constrain utilization and prudently purchase recombinant erythropoietin. The main difference between this method and reimbursement based on actual charges is that a ceiling is placed on the amount that Medicare will

pay. This ceiling, however, is not very effective. What is considered customary, prevailing, and reasonable is based on actual charges that with a lag determine Medicare's approved rates. Therefore, knowing that their inflated bills will increase CPR ceilings and the amounts that Medicare will pay in the future, providers have an incentive to inflate charges. The only constraints on providing and charging too much is the risk that patients will not pay their bills or, over the longer term, will seek lower cost providers.

As a type of fee-for-service payment, the CPR method gives providers a financial incentive to increase use, through higher dosage levels and treatment for greater numbers of patients, as long as the payment per unit of service exceeds the provider's unit costs. The strength of this incentive depends on the extent to which payment levels exceed costs. Overprovision can take the form of doses of recombinant erythropoietin that are in excess of clinically appropriate levels and treatment of marginal patients for whom this therapy is inappropriate. If payment just equals cost, providers experience no financial gain from exceeding clinically appropriate dosage levels or inappropriately treating patients. There may still be overprovision in an economic sense, however. The clinically optimal level of recombinant erythropoietin is not necessarily equivalent to the economically efficient level. One more unit of the biologic may have a clinical benefit, but this benefit may be insufficient to warrant the additional cost. Medicare dollars might be better used elsewhere. Thus, even if providers gain nothing financially, they may still have an incentive to overprovide. They incur no net costs from doing so and the additional costs to the beneficiary are limited by the 20-percent coinsurance rate.

Under this option, providers would have little or no incentive to shop for a lower price for recombinant erythropoietin. The weakness of this incentive is especially significant if Medicare does not set the rate at which providers may purchase the product. Because of these generally weak incentives, this option is likely to result in higher costs to both the Medicare program and beneficiaries, well above those likely under options 3, 4, and 6. Higher costs to beneficiaries mean less financial access. Financial access might also be diminished because of the greater likelihood of out-of-pocket extremes under this option.

The weakness of the CPR method is well recognized. Recent amendments to Title XVIII of the Social Security Act (Public Law 101-239) require that, after a phase-in period ending in 1996, Medicare end the current CPR method of paying for physician services and implement payment according to a fee schedule. Under the CPR method, providers would have a financial incentive to exceed clinically appropriate dosage levels and even to administer the biologic to patients for whom it is unnecessary. Given the possibility of adverse events, harmful effects from overuse are certainly possible.

The preceding discussion of provider incentives applies only to physicians and dialysis facilities that administer recombinant erythropoietin. Financial incentives to overuse pertain less to pharmacies and dialysis distributors, which do not prescribe treatment and dosage levels. The lack of incentive under this option to shop for a low price for the product, however, would still be an important factor in evaluating its appropriateness for these providers.

Because out-of-pocket costs vary with the quantity of service, this payment method may be perceived by beneficiaries

as more equitable than options 3 and 4. There may still be some inequity, however, because out-of-pocket expenses would continue to be affected by differences in charges among providers for the same service.

Since payments are related to providers' charges up to Medicare ceilings, providers incurring market-related differences in costs are far more likely to receive commensurate payments. This method is also neutral with respect to patient characteristics that affect use.

The incentives for overuse under this option may result in higher revenues and profits accruing for manufacturers and an excessive stimulus to technological innovation, especially for similar therapies for which Medicare is a dominant payer. Excessive stimulation could draw into related research and development additional resources that would have greater social value if used elsewhere. There is little possibility that this payment method would provide an inadequate stimulus for technological change.

Although Medicare carriers and fiscal intermediaries are already familiar with the workings of this payment method, the administrative burden is nonetheless substantial. Determination of the customary, prevailing, and reasonable charge is a complicated procedure that must be applied for each provider. Unlike options 3 and 4, payment differentiation occurs automatically and is irrelevant as a potentially necessary refinement .25 Also, unlike options 3 and 4, payment rates are not prospectively determined, eliminating the need to update them in response to dynamic changes in the market for recombinant erythropoietin. Lastly, although underprovision is not a problem under this option, there is still a considerable need for peer review because of the strong incentive for overprovision and the potential for reduced quality.

Option 6: Mandate the Medicare program to pay providers of recombinant erythropoietin according to a fee schedule.

Under this option Medicare would set in advance of the period in which they were to apply a schedule of fees that it would pay per unit of recombinant erythropoietin. Unit amounts would apply to the product, related supplies, and services to administer or dispense it. The fees paid could be uniform, or they could vary to reflect market-related differences in providers' cost. As noted in option 5, after a phase-in period, Medicare will pay for all physician services according to a fee schedule. Separate fee schedules could be developed for dialysis facilities, physicians, dialysis distributors, and pharmacies.

In comparison with options 3 and 4, the fee-schedule method places less financial risk on providers and patients and consequently, creates weaker incentives to constrain use and to prudently purchase recombinant erythropoietin. Like fee-for-service payment generally, if the payment rate exceeds unit cost, physicians and dialysis facilities would have a financial incentive to provide additional units of the product, especially if there are few or no adverse consequences from doing so. Although total payments would vary directly with the quantity of recombinant

25 Difficulties have arisen, however, in rationalizing payment differences between urban and rural physicians.

erythropoietin provided, a fee-schedule approach has other advantages over the CPR method. One advantage is that Medicare can control the amount paid per unit of the service, whereas Medicare passively processes providers' billings under CPR. In addition, Medicare can encourage or discourage the use of a particular service by raising or lowering payment rates.

Because of the above incentives, costs to the Medicare program and to beneficiaries would likely be higher under this option than under options 3 and 4. Higher out-of-pocket costs for beneficiaries imply less financial access. Financial access might also be less under a fee-schedule approach than under options 3 and 4, because out-of-pocket extremes are more likely. This payment method, however, would probably result in lower costs to the Medicare program and beneficiaries than the CPR discussed under option 5. If the payment is less than unit cost, providers may have a strong financial incentive both to reduce amounts of recombinant erythropoietin below clinically appropriate levels and to deny access.

Not all of the above incentives apply to pharmacies and dialysis distributors. Since pharmacies and dialysis distributors do not make decisions regarding dosage, these providers have less influence than physicians and dialysis facilities over use and cost to Medicare and its beneficiaries. In contrast to payment based on charges billed, a fee-schedule approach would encourage all providers to be prudent purchasers of the product. This situation would be beneficial to Medicare to the extent that providers' actual acquisition costs enter into the calculation of fee schedules.

Incentives to overprovide or underprovide recombinant erythropoietin would also be affected by whether payments were uniform or differentiated. Differentiated payments would be appropriate, for example, if providers faced market-related differences in wage rates and in the acquisition costs for the product. Financial gains and losses would then be smaller, and incentives to both overprovide and underprovide the service would be weaker.

A fee schedule may be the most equitable payment method from the beneficiary's perspective. Out-of-pocket costs would vary directly with and depend only on the quantity of the product used. Differentiated payments, to account for market-related differences in costs among providers, might reduce rather than improve beneficiary equity. Such adjustments would cause out-of-pocket costs to vary also with provider unit costs and might be viewed as unfair by beneficiaries.

A fee schedule is a more equitable payment method for providers than options 3 and 4. Since payments vary directly with the quantity of recombinant erythropoietin used, differences among patients would not result in uneven compensation. Uneven compensation due to market-related differences in acquisition costs for the product and other service costs, however, would still exist. The compensation imbalances under this option would be remedied if unit amounts are differentiated to reflect market-related differences in cost.

Unless payment amounts were generally inadequate and well below unit costs, this alternative should not adversely affect industry incentives for technological innovation. Utilization levels should be

sufficiently high to satisfy industry sales requirements. On the other hand, the relatively weak constraints on use inherent in this payment method could result in overuse of recombinant erythropoietin. This could give the industry an excessive stimulus for investment.

This option would be less burdensome to Medicare carriers and fiscal intermediaries than option 5, because determination of the appropriate payment for each provider would be considerably easier. Like options 3 and 4, however, this option requires the calculation of prospective rates and their periodic updating in response to dynamic changes in the market for recombinant erythropoietin. Also, like options 3 and 4, there is a potential need to differentiate these rates, which further adds to the administrative burden. Lastly, because of the potential for both overprovision and underprovision and the resulting diminutions in the quality of care relating to each, peer review is no less necessary under this option than under the other provider payment options.

Payment for the Product

This section reviews three methods that Congress could require Medicare to use to determine the rate that it will pay for the product recombinant erythropoietin. Setting a payment rate for the product, in addition to setting rates for providers, may enhance Medicare's overall ability to control the costs of this therapy. Better control of costs implies more effective use of limited Medicare resources and, therefore, more potential benefits to patients.

An important consideration in implementing payment for the product component of recombinant erythropoietin therapy is the mechanism through which a payment rate for the product would be realized by Medicare. The product flows from the manufacturer through one or more wholesalers or other intermediate suppliers before it reaches the ultimate providers. A rate agreement between Medicare and manufacturers and the consequent financial flows may or may not involve intermediate suppliers.

One possibility for handling the financial flow is that the manufacturer or manufacturers of recombinant erythropoietin pay rebates directly to Medicare. Rebates could be based on a specific amount per unit sold to Medicare providers. Volume information could be obtained from copies of claims submitted to Medicare carriers and fiscal intermediaries. If there is more than one manufacturer, specific volumes would have to be verified for each. This should not pose a problem, since each manufacturer's brand of recombinant erythropoietin could be identified from a code appearing on each claim.

A more important difficulty with this approach would arise if some manufacturers of recombinant erythropoietin did not have a rate agreement with Medicare. Since the providers of recombinant erythropoietin would not benefit from the rebates, they would have no incentive to purchase recombinant erythropoietin from Medicare-designated manufacturers. This follows from the fact that rebates paid by manufacturers to Medicare need have no direct bearing on the prices that manufacturers would charge to providers. The total cost savings to Medicare would, therefore, be more limited and would depend on the portion of Medicare providers who chose, for whatever reason, to purchase from

Medicare-designated manufacturers. As a remedy, Medicare could lower payments to providers who failed to purchase from Medicare-designated manufacturers or deny them payment altogether.

Another possibility to address this problem would be for the manufacturer to provide rebates to Medicare providers of recombinant erythropoietin rather than to the Medicare program. Providers would also be identifiable from claims. Under this alternative, providers would have a financial incentive to purchase recombinant erythropoietin produced by Medicare-designated manufacturers, because Medicare's payments to all providers would be based on the low prices negotiated with manufacturers. A major difficulty, however, is that manufacturers would be burdened with the task and cost of periodically providing rebates to thousands of dialysis facilities, physicians, dialysis distributors, and perhaps pharmacies. Alternatively, rebates could flow from the manufacturer to Medicare carriers and fiscal intermediaries which, in turn, could transfer them to providers. Medicare carriers and fiscal intermediaries already directly deal with providers on a regular basis. Since this new responsibility would raise the costs of carriers and intermediaries, it might be necessary for Medicare to raise payments to these contractors.

Option 7: Mandate the Medicare program to base payment rates for recombinant erythropoietin on manufacturer costs.

Under this approach Medicare would determine a price for recombinant erythropoietin based on a thorough review of manufacturer costs. This alternative is most applicable to a market with a single manufacturer. Although it could also be used for multiple manufacturers, the complexities involved in determining an appropriate payment rate would make it impractical relative to other alternatives.

If it wished to obtain an explicit rate agreement from the manufacturer, Medicare could use its calculated rate as a target toward which to negotiate. What actual rate emerged from negotiation, and how closely it approached the target rate, would depend on the strength of Medicare's market position relative to that of the manufacturer. Alternatively, Medicare could simply use the target rate as an input in calculating payments to providers of recombinant erythropoietin. Medicare employed a variant of this method to set the current payment rate to dialysis facilities. This latter alternative, however, would be less effective in controlling product costs, since Medicare would have no direct influence over the rates charged by manufacturers.

Calculation of a payment rate for recombinant erythropoietin on the basis of manufacturer costs poses certain difficulties. First, since manufacturers are usually developing and producing many products, it is quite difficult to allot common costs, such as basic research and development and overhead expenses, to the product in question. Common costs are costs that cannot be traced to specific products. It is typical in the pharmaceutical industry that multiple discoveries emerge from the same basic research (70). Although measurement of common costs is difficult, their allocation to specific products is more so.

Accounting methods, such as the allocation of common costs according to the projected sales volumes of the related products, are unlikely to result in appropriate payment rates (1976). In the pharmaceutical industry, the related products sold by a firm usually differ in therapeutic significance. Products emerging from the same basic research and development process are further developed and marketed, if sufficient revenues to cover incremental costs are expected. This often yields a hierarchy of related products in terms of therapeutic significance and strength of market demand (31). Larger shares of common cost are efficiently allocated to products with stronger market demands (128).[26] A product has a strong market demand if it can command a high price and if the quantity purchased is largely insensitive to price. Therefore, to allocate efficiently common costs to recombinant erythropoietin, it is necessary to estimate the strength of its market demand relative to that of related products produced by the firm or firms in question. This determination is further complicated by the fact that demand is measured over time and common costs must also be apportioned according to the expected market life of each product.

A second issue complicating this payment option concerns the determination of an appropriate profit rate for the manufacturers of recombinant erythropoietin. The average profit rate for the pharmaceutical industry may be inappropriate if common costs are allocated using accounting methods.[27] Accounting methods would allocate too small a portion of common research and development and other expenses to products with stronger market demands. Consequently, the application of the average industry profit rate to the investment base for these products would yield profits that were too low. Profit rates for individual pharmaceutical products, when calculated using an accounting allocation of common costs, have been shown to vary widely, with many being very low or negative (78). Therapeutic breakthroughs, such as recombinant erythropoietin, generally have high accounting profits. If common costs were appropriately allocated among related products, profit rates would be more uniform.

It has been argued that large accounting profits on successful products are necessary to offset accounting losses on unsuccessful ones, and that only through these can firms earn an adequate overall rate of return (169). Therefore, if accounting methods are used to allocate common costs, and they may be the only practical methods to use, it maybe more appropriate to apply to recombinant erythropoietin the average profit rate for significant therapeutic breakthroughs rather than the average rate for the industry. Actual profit rates, whether for specific classes of products or for the pharmaceutical industry as a whole, are appropriate for the rate calculation in this option only if competition in the industry is sufficient to keep overall profits at reasonable levels.

26 The efficient allocation of common costs is essentially equivalent to pricing according to what the market will bear. Therefore, pharmaceutical firms automatically achieve this objective in their pursuit of profits. Although this may lead to product prices that are efficient relative to one another, absolute prices may still result in excessive profits if firms possess considerable market power overall.

27 Accounting methods would tend to allocate common costs on the basis of the projected volumes for each product and would not take into account the product's value to consumers and their sensitivity to price.

Despite considerable research (3,32,36, 39,1 15,169), the degree of competition in the pharmaceutical industry is still unclear. Consequently, it is difficult to know whether industry profit rates are acceptable for calculating a product rate under this option. An analysis of the average profit rate for the industry might reveal something about the degree of competition. Interindustry comparisons of profit rates are often used in such evaluations. Extreme caution, however, should be applied in making comparisons. Differences could be justified by differences in risk and the timing of returns. Also, profit rates in the pharmaceutical industry should be carefully interpreted and compared with those in other industries, because they are very sensitive to accounting practices and other assumptions made in their calculation (9,22,30,135).

A third complication affecting this payment option concerns inefficient uses of resources. In addition to price competition, some pharmaceutical firms may compete in other ways that are wasteful. Such behavior is possible in an industry that is not purely competitive but is characterized by the imperfect competition associated with brand names and product differentiation. Inefficiency arises if products are marketable at prices that cover incremental costs, only because of "persuasive" promotion. Persuasive promotion is distinct from "informative" promotion, which serves the important function of educating potential users regarding the merits and possible side effects of a product. Persuasive promotion goes beyond conveying to potential buyers the information necessary for making rational purchasing decisions (88) and attempts to encourage purchase by distorting information or by offering benefits unrelated to the product's price. In addition to encouraging imprudent purchases, expenditures on persuasive promotion are in themselves wasteful of resources. Studies have shown persuasive promotion to be a significant factor in the pharmaceutical industry (73,74). To the extent that this behavior applies to the manufacturers of recombinant erythropoietin, price determinations under this option might limit allowance for promotion and other expenditures relating to products marketed in this manner.

The implications of this option depend on whether Medicare succeeded in calculating a payment rate that reflected the costs of efficient production, including a normal profit. Whether this result would occur, however, is not predictable. If the calculated rate was substantially higher or lower than the rate that reflected efficient production, a number of problems could arise. A high rate would mean fewer benefits per dollar allocated to recombinant erythropoietin and higher costs to the Medicare program. Depending on how Medicare set payment rates for providers, it might also result in 1) the substitution of less effective therapies, such as blood transfusions, with perhaps deleterious effects on patients' health, 2) higher out-of-pocket costs for beneficiaries and consequently, less financial access and lower quality of care, and 3) an excessive stimulus to the pharmaceutical industry for technological innovation. A low rate might be harmful, because it could also distort the selection of therapies and provide an inadequate stimulus for technological innovation. In addition, a low rate could cause the manufacturer of recombinant erythro-

poietin to shift costs to other markets or products, depending on how these prices were determined.

A principal drawback of this option is the difficulty of calculating a payment rate that compensates fairly and encourages the efficient use of resources. Although this consideration is crucial, it also adds significantly to administrative difficulty. Other factors contributing to the administrative costs of this option include the staff resources needed to obtain and update information for periodically recalculating the payment rate for the product. Recalculations would be needed to reflect changes in product volumes, input costs, and other factors that in turn affect the costs of producing and distributing the biologic.

Option 8: Mandate the Medicare program to set the payment rate at the lowest price for recombinant erythropoietin listed in the Federal Supply Schedule.

The Federal Supply Schedule (FSS) is a catalog of single- and multiple-source products that are available from various manufacturers to the health care facilities of certain agencies of the Federal Government, such as the Department of Veterans Affairs (VA), the Department of Defense, the Public Health Service, and the Centers for Disease Control. The FSS is distinct from products directly purchased by the VA and distributed to facilities through its depot system. Administrative responsibility for the FSS has been delegated by the General Services Administration to the Department of Veterans Affairs Marketing Center (107,120).

The FSS represents prices negotiated with manufacturers.[28] Federal Government medical centers may buy products at FSS prices directly from these manufacturers. The prices listed on the supply schedule are less than or equal to the lowest prices charged to the same class of trade in non-government transactions. Each manufacturer wishing to list on the FSS must provide the VA Marketing Center with complete and confidential information on the prices charged to other customers. The final price is arrived at after negotiation between the VA Marketing Center and the manufacturer (107,120).

Federal Government medical centers are ordinarily required to purchase the lowest priced item on the FSS that meets their needs. Product orders are placed directly with and are shipped from the manufacturer. The Federal Government does not ordinarily guarantee that any specific volume of the product will be purchased by Government medical centers from the manufacturers listing in the FSS. In addition, facilities may purchase from suppliers not on the FSS if the prices charged by these are lower than the lowest priced products on the FSS (107,120).

As a payment option, Medicare dialysis facilities and perhaps other providers of recombinant erythropoietin could be allowed to purchase this product at the price listed in the FSS. This approach, of course, assumes that at least one recom-

28 FSS prices now include delivery to Government medical centers. A different arrangement could be negotiated for recombinant erythropoietin and Medicare dialysis facilities.

binant erythropoietin product is listed.[29] The FSS approach has the advantage of applying the weight of the Federal Government's purchasing power. In this respect it is superior to option 7, in which Medicare would negotiate independently with the manufacturer or manufacturers of recombinant erythropoietin. Nevertheless, the FSS approach may still be a weak method for obtaining the best possible prices from manufacturers.

There seems to be no strong incentive for manufacturers of recombinant erythropoietin either to participate in the FSS or to offer the lowest prices that they will accept, if they do choose to participate. Since manufacturers can later reduce prices and since the Government ordinarily makes no sales commitments to low bidders, the best strategy for a manufacturer may be to offer an FSS price that is considerably higher than the lowest price that the company would accept. High FSS prices give manufacturers the option of either sticking to those prices or selectively offering prices lower than the FSS ones, if competitive pressures warrant. At present, the Government, chiefly through Medicare beneficiaries, accounts for most of the U.S. market for recombinant erythropoietin (see ch. 3). It does not seem that, under such circumstances, a manufacturer would list in the FSS at a significantly discounted price, unless compelled to do so by the threat of lost sales. This situation may change if the non-Medicare market expands.

In any case, there may be some advantages to manufacturers of recombinant erythropoietin from appearing on the FSS as relatively low-priced sellers. Because of wide exposure, it could significantly reduce the need for direct marketing. It could also build good will with both the Government and providers.

Another difficulty that applies specifically to recombinant erythropoietin is the limited information on prices paid by comparable non-government purchasers. For the only indication that the FDA has approved to date, chronic renal failure, the Federal Government is by far the dominant domestic payer. Also, because of difficulties relating to the translation of foreign currencies into U.S. dollars and because of foreign government regulation of prices for recombinant erythropoietin, foreign prices may not be appropriate for comparison (see ch. 4 for foreign prices of recombinant erythropoietin adjusted for purchasing power parities among foreign currencies). Therefore, adequate reference prices from which to negotiate Government price concessions may not be available. This limitation, however, should be eased as more indications for recombinant erythropoietin receive FDA approval.

There appears to be little or no possibility under this option for FSS prices for recombinant erythropoietin to be too low. As argued, however, incentives are such that they could be well above the lowest prices that manufacturers would accept.[30]

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29 Effective Jan. 1, 1990, Amgen listed recombinant erythropoietin on the FSS. The Federal Government was given a 2-percent discount off Amgen's list price of $20 per 2,000-unit vial, $40 per 4,000-unit vial, and $100 per 10,000-unit vial (117,139).

30 Pharmaceuticals listed in the FSS average 41 percent below the average wholesale price (AWP) for single-source products and 67 percent for multiple source ones (138). The AWP is an inappropriate benchmark, however, since it is a list price and is not usually charged to any purchaser. Moreover, recombinant erythropoietin is a recent therapeutic breakthrough, and the above discounts may not apply to such products.

As discussed in option 7, higher prices mean fewer benefits per dollar allocated to recombinant erythropoietin and higher costs to the Medicare program and beneficiaries. Higher beneficiary costs may reduce access to this product and result in lower quality care. High prices may also provide a socially inappropriate stimulus to technological change.

Although this option has the advantage of the FSS' already being in place, it may still pose some administrative difficulties. These difficulties apply less to dialysis facilities and distributors than to other providers of recombinant erythropoietin. The Government facilities currently purchasing from the FSS are relatively large and few in number. Therefore, the logistics of distributing products to these facilities at FSS prices are manageable. Also, because these facilities serve government-related personnel only, there is little risk to manufacturers that products purchased at FSS prices will be used for non-government purposes. If additional indications for recombinant erythropoietin are approved and Medicare coverage is broadened, very large numbers of physicians and retail pharmacies could be involved. The distribution logistics implied may be far more complicated than those for existing FSS purchases. In addition, the above providers of recombinant erythropoietin serve other than government-related beneficiaries. This could significantly increase the risk to manufacturers that recombinant erythropoietin purchased at FSS prices would be used for unintended purposes.

Both of the above problems, however, would be considerably reduced if the FSS approach were applied only to dialysis facilities and distributors. In 1989, there were about 1,800 dialysis facilities that served primarily beneficiaries of government programs (see ch. 4) (156).

Option 9: Mandate the Medicare Program to set payment rates for recombinant erythropoietin through competitive bidding.

Under this option prices for recombinant erythropoietin would be obtained through a bidding process established by Medicare. Although competitive bidding could take place with as few as two suppliers, its effectiveness generally increases with the number of bidders. Medicare could set the rules and payoffs of the bidding process, and these would influence how closely price offerings approach the lowest price that each manufacturer would accept. A crucial requirement of the competitive bidding approach is that awards be clear and irrevocable. This means that Medicare must guarantee, through contract, recombinant erythropoietin volumes to the winning bidder or bidders. Otherwise, as with the FSS, suppliers would have little incentive to offer their lowest acceptable prices.

Two basic bidding approaches have been identified and evaluated (95). Under one approach, manufacturers of recombinant erythropoietin would openly quote prices to Medicare with the freedom of making reductions in response to each other's bids. Since bidders are unlikely initially to know the lowest acceptable prices of their rivals, prices would be lowered through successive rounds of bidding. Each bidder, for fear of losing, would have an incentive to gravitate toward its lowest acceptable price, and each bidder, except the winner, would eventually be compelled

to reveal this price. The winner would have to bid only slightly below the previous bid in order to win. Therefore, the timing bid could exceed the lowest price that the winner was willing to accept by some unknown amount.

Under a second approach, manufacturers of recombinant erythropoietin would offer sealed bids to Medicare. The principal difference here is that manufacturers would not be able to adjust offers in response to the observed bids of rivals. If bidders have little or no information regarding each other's lowest acceptable prices, bids would reflect each manufacturer's tradeoff between the probability of winning and winning with a price that, in retrospect, is unnecessarily low. The more severe the consequences of losing Medicare sales, the closer will bids be to each manufacturer's lowest acceptable price.

Without additional information, it is unclear which bidding approach would be more advantageous to Medicare. Open bidding would yield a price slightly below the lowest acceptable price of the second-lowest bidder. Depending on the financial consequences faced by manufacturers, sealed bidding would yield a price that is either higher or lower than the above price. Sealed bidding would be more advantageous to Medicare if the manufacturers of recombinant erythropoietin would incur major financial losses from not winning a contract. Manufacturers would probably be very averse to losing Medicare sales if Medicare accounted for the dominant share of the market for recombinant erythropoietin and if this biologic accounted for a large portion of each firm's total sales. Alternatively, if the Medicare market was of considerably less importance, manufacturers' aversion to losing Medicare sales might also be less. Under these circumstances, open bidding might be superior. Under either approach, a larger number of bidders (manufacturers of recombinant erythropoietin) would be advantageous to Medicare, because it is more likely to result in the winning bid's being closer to the winner's lowest acceptable price.[31]

The issue of single or multiple winners should also be considered. Multiple winners are possible even if there are only two manufacturers of recombinant erythropoietin. Although the price would be set at the lowest bid, guaranteed sales to the lowest bidder should be significantly greater. This approach is necessary to maintain manufacturers' incentives to reveal their lowest acceptable prices and to discourage collusive behavior. Although a single winner may provide maximum incentives, this approach could be very harmful to losers, the market for recombinant erythropoietin, and the industry. If Medicare accounts for all or nearly all of the market, exclusion of losers might result in their permanent elimination. This would make the market less competitive and could result in higher prices for recombinant erythropoietin in the long run.

A possible disadvantage of multiple winners pertains to the logistics of dividing the Medicare market among manufac-

31 For open bidding, the difference between the winner's lowest acceptable price and that of the preceding bidder would most likely diminish as the number of bidders increased. For sealed bidding, a larger number of bidders would reduce the probability that each would win with any given bid. This should induce manufacturers to lower their bids, putting them closer to their lowest acceptable prices.

turers. A relatively simple approach would be geographically to divide the Medicare market for recombinant erythropoietin. The lowest bidder, for example, could be guaranteed the largest portion (percentage of sales) of the Medicare market and the freedom to choose which geographic areas would be included in this share. The remainder of the Medicare market could be divided in a similar manner, with the second-lowest bidder getting the next largest share, and so forth. Medicare would require all participating manufacturers to sell the biologic at the winning price bid. To receive payment, Medicare could require providers in each geographic area to purchase the brand of recombinant erythropoietin that Medicare designated for that area.

Difficulties might arise, however, if one organization had dialysis facilities in areas designated for different areas. Such an organization might be faced with obtaining recombinant erythropoietin from more than one source, a situation that could reduce the organization's ability to negotiate a lower price from suppliers. Another complication would arise if the brands of recombinant erythropoietin are not therapeutically equivalent and if these differences are protected by patent. This implies that for some patients, the different brands would not be interchangeable. In that case, totally excluding a brand from a geographic area would not be feasible. As a solution, physicians could be required to justify a specific brand for those patients for whom substitution would be clinically inappropriate e. Manufacturers being awarded geographic contracts should be allowed to produce and distribute all versions of the product within legal limits.

As long as manufacturers of recombinant erythropoietin do not refuse to participate in a Medicare bidding process, this option would appear to be an effective method for obtaining competitive prices. There is little reason to believe that resulting prices would be too high. It is possible, however, that prices could be too low. For example, if Medicare's market position was very strong and a single timer was specified, manufacturers might make bids that were below the costs of efficient resource use.

Any price that would at least cover the incremental costs of producing and distributing recombinant erythropoietin could emerge under this option. Such a price, however, might contribute little or nothing to common costs, that is, the costs of resources that are used by more than one product. As argued, this is inefficient for a product, such as recombinant erythropoietin, that would face a strong demand under normal market circumstances. Medicare can prevent manufacturers from bidding prices that are too low by reducing the risks from not doing so. Risks to manufacturers would be reduced if multiple awards were made and if the differences among awards were smaller.

Prices that are too low can provide inadequate incentives for technological innovation, both for the class of products in which recombinant erythropoietin is included and for all pharmaceuticals for which Medicare is a dominant payer. Low prices may also cause manufacturers of recombinant erythropoietin to shift costs to other markets and products.

Competitive bidding has been used by State and local governments to set payment

rates for health care services, but the results have been mixed (96). Although public and private organizations that deliver health care have obtained certain services or products through competitive bidding, the results of similar attempts by governments acting as third-party payers have been disappointing. Despite the potential, it is not clear that these arrangements have resulted in lower prices or lower expenditures for the programs.

In some cases, manufacturers or suppliers have refused to participate. For example, brand-name manufacturers did not offer bids in response to a solicitation from the Kansas Medicaid program regarding pharmaceutical products (9a). Compared with this situation, however, the Medicare program represents a different market with different incentives for manufacturers. With the Kansas Medicaid program, manufacturers had to weigh the possibility of lost sales to that program against the possibility of much larger revenue losses if price concessions had to be shared with other State Medicaid programs. Given Medicare's current predominance as a payer of recombinant erythropoietin therapy, the possibility of lost payments from Medicare would most likely outweigh negative effects on other markets.

Quality problems that have plagued some other competitive bidding programs would be less likely to apply to recombinant erythropoietin under Medicare. Past difficulties seemed to have stemmed in large part from an inability to define precisely the service. Recombinant erythropoietin, however, is a more specific product whose quality is already controlled by FDA requirements.

The administrative responsibilities of conducting a competitive bidding process, monitoring the contracts, and distributing rebates from manufacturers would entail additional costs for HCFA. Also unique to this option are administrative difficulties regarding the division of the Medicare market, if multiple winners are specified. Medicare has not previously negotiated a price for an intermediate product that is used by medical providers rendering services to beneficiaries. In many cases, a demonstration project within a limited geographical area enables HCFA to evaluate the feasibility of an innovation, but such a demonstration project would not provide a fair test of this option. If the option applied only to a given region, manufacturers would have less incentive to participate and to tender low bids. It would be more reasonable initially to implement the option for dialysis facilities, which currently treat most of the beneficiaries receiving recombinant erythropoietin. Administrative procedures regarding rebates, for example, would be more manageable for the smaller number of dialysis facilities than if physicians' offices were also included. If successful, the option could subsequently be expanded to physicians.

CONCLUSION

Selecting payment options for Medicare payment of recombinant erythropoietin requires balancing desirable and undesirable implications. The most important tradeoffs relate to improving access to and quality of care for beneficiaries vs. constraining costs to Medicare and its beneficiaries.

Of all the options analyzed, option 1 (extending Medicare coverage to self-administration of the biologic) would most improve access to care, especially for home

dialysis patients. Such an extension of coverage would reduce beneficiaries' expenses, but raise those of the Medicare program. Option 2 (setting payment rates to encourage providers to engage in further research) has the potential to improve substantially the quality of care that beneficiaries receive over time. However, this option might merely transfer costs from manufacturers to the Medicare program.

Among options for paying providers of the biologic, option 4 (including payment for recombinant erythropoietin in the composite rate paid to dialysis facilities and in the cavitation rate paid physicians for dialysis patients) has the greatest potential to constrain Medicare expenditures and beneficiaries' out-of-pocket expenses. This option, however, also contains the strongest incentive for providers to skimp on use, which could damage the quality of care that beneficiaries receive. Along with option 4, option 5 (basing Medicare payment on customary, prevailing, and reasonable charges (CPR)) has the worst implications for the quality of care, but from a different direction. The CPR method threatens the quality of care by rewarding overuse of the biologic and at the same time has the greatest potential to fuel inflation in Medicare expenditures and beneficiaries' cost-sharing. Option 3 (paying a fixed rate per recombinant erythropoietin treatment), the present method, is likely to produce moderate expenditures for Medicare and its beneficiaries. This option moderately rewards providers who skimp on dosage, a practice that is subject to quality review. Option 6 (paying according to a fee schedule) may contain moderate incentives encouraging use, with implications for expenditures and the quality of care. These drawbacks can be addressed, however, by judiciously setting payment levels and by monitoring use. Adoption of this option would apply to recombinant erythropoietin the same payment method that the Omnibus Budget Reconciliation Act of 1989 recently mandated for Medicare payment of physician services generally.

Under present policy, Medicare varies the level and method of payment for recombinant erythropoietin therapy according to the setting in which it is provided. Equity among beneficiaries and providers and incentives for efficient use of medical services would argue for paying the same amount for the same service, regardless of where it was provided.

If Congress adopted an option for paying for the product itself, the resulting payment rate for the product could be incorporated into the level of payment for providers. Of the product payment options, option 9 (setting payment for the product through competitive bidding) has the potential in the short term to result in the lowest price for Medicare and the lowest expenditures for the program and its beneficiaries. Less clear, however, are its feasibility and the likely effects over time on the viability of companies heavily dependent on Medicare revenue and hence on the competitiveness of the industry.

The viability and advisability of the particular options for product payment must be considered within the dynamic context of the market for recombinant erythropoietin. With only one manufacturer about to enter the market, HCFA used option 7 (basing product payment on manufacturer costs) to set current payment rates for providers, but the impracticality of this option

increases with the number of manufacturers. Given Medicare's predominant position as a payer of recombinant erythropoietin therapy, it is unlikely that, under option 8 (using the Federal Supply Schedule), manufacturers would give substantial price concessions. To be viable, option 9, which calls for competitive bidding, requires at least two manufacturers. Indeed, any contractual agreement between Medicare and a manufacturer would have to take into account the stability of market conditions and the effect on the long-term competitiveness of the industry. If additional manufacturers were poised to enter the market, for example, Medicare would probably benefit from delaying its contracts or limiting them to a short period.

Whatever payment options are adopted, HCFA will have to be able to exercise flexibility in monitoring and responding to changing market conditions. In this dynamic market, the number of manufacturers, FDA-approved medical indications for use, and, eventually, Medicare's predominance are likely to evolve over time. The appropriate level and perhaps even the method of payment may well change with market conditions. HCFA's responsiveness to continuing changes promises to influence the quality of care, Medicare and beneficiary expenditures, and the positions of manufacturers and providers.