Appendix E.—Case Studies of Medical Technologies

Introduction

The five case studies in this appendix are included as illustrative examples of the innovation process, including the development and adoption, of selected medical technologies. Of particular note are the effects which Federal research funding and regulatory and reimbursement policies have on the innovation process.

The case study on percutaneous transluminal coronary angioplasty (PTCA) was written by David Sawi, and that on hemodialysis and kidney transplant surgery by Katherine Jones. The other case studies on gastric freezing for the treatment of ulcers, hemodialysis for the treatment of schizophrenia, and maternal serum alpha-fetoprotein (MSAFP) were prepared by OTA staff.

Gastric Freezing

In the mid-1950's, a surgical leader in the United States, Owen Wangensteen (of the University of Minnesota Medical School), observed that iced saline solution lavaged into the stomach slowed gastrointestinal bleeding and that animal experiments showed reduction in stomach acid output following gastric cooling. From these observations, Wangensteen conceived of the idea of using gastric cooling for treatment of peptic ulcer disease (143).

In collaboration with a small refrigeration company, he developed a device to circulate alcohol at –15° C through a nasogastric tube to a balloon inserted into the stomach. After testing the device in dogs, he first tried it on one patient, then a dozen others (143).

In 1962, he reported his results in the ***Journal of the American Medical Association:*** no serious side effects, markedly reduced stomach acid output, immediate relief of ulcer pain, and radiographic evidence of ulcers healing (381). The ***1962*** report was extensively covered in the popular media. Wangensteen also sought to spread his procedure through professional meetings and publications, and the American College of Surgeons prepared an instructional film on the technique (143).

By the end of 1963, 1,000 devices had been sold and 10,000 to 15,000 procedures had been performed nationwide (143).

Beginning in ***1963,*** however, the efficacy and safety of gastric freezing began to be questioned. In 1964, there began to appear published reports concluding that acid suppression was limited or unrelated to pain relief, symptomatic improvement was short-lived or due to placebo effects, and important risks were present. Variations in the technique were used and became arguing points for valid use, but by 1966 the technique was rarely used. Furthermore, Wangensteen lost his support from the National Institutes of Health (NIH) for research in gastric freezing, although he still thought the procedure worthwhile and did not believe his earlier reports to be inaccurate (143).

Hemodialysis for Treatment of Schizophrenia

Schizophrenia, a disorder characterized by misinterpretation and retreat from reality, delusions, hallucinations, ambivalence, inappropriate affect, and withdrawn, bizarre, or regressive behavior, is estimated to afflict 2 to 3 percent of the population. Because of its relatively high prevalence **rate, onset in adolescence** and early adulthood, and lifelong chronicity, this disease imposes a major toll of disability and a great economic burden on our society. Although effective treatment for the disease could help large numbers of people, ineffective new interventions pose the prospective risk of wasting large sums of money. Schizophrenia's unpredictable course in individual patients, coupled with the difficulty of assessing the health status of patients, objectively enhances the risk that ineffective or unproved modes of treatment will be adopted, perhaps even widely, in the management of schizophrenic patients (50).

In 1977, Wagemaker and Cade ***(379)*** created intense interest by claiming, in a report published in the ***American Journal of Psychiatry, dramatic*** improvement in five physically healthy schizophrenic patients treated with weekly dialyses for Up to ***16*** weeks. Since then, Wagemaker and Cade have continued this treatment. Wagemaker has dialyzed an additional 15 patients, reporting 100 percent success in 7 women and 3 good successes, 3 partial successes, and 2 failures in 8 men (109).

Scattered reports in the literature between 1925 and 1960 had claimed improvement in schizophrenic patients given blood transfusions either from remitted schizophrenics or from healthy volunteers, but Feer, et al. (139), apparently were the first to use dialysis. These investigators reported in 1960 that three out of five schizophrenics improved after only one or two hemodialyses (139).

There is some evidence that hemodialysis may remove a circulatory psychotogen, as Palmour and Ervin (285) reported a substance characterized as a beta-endorphin in the dialysate of the patients treated by

Wagemaker and Cade. A 100-fold decrease in the substance, corresponding to improved clinical functioning, was found in 14 of 16 treated patients. This claim has not been duplicated and reported by others.

Because of the positive report by Wagemaker and Cade, the Clinical Research Branch of the National Institute of Mental Health (NIMH) funded three research projects using a double-blind design. Dr. Wagemaker, of the University of Louisville (Kentucky), developed the first project beginning in September 1978 through his "own initiative with considerable institute staff consultation to develop an acceptable protocol. " This study is near completion but has not been reported. The other two projects are being conducted at the University of Maryland (Baltimore) and the University of Washington (Seattle), both starting in September 1979. In addition, the NIMH Intramural Program conducted a small study, the results of which were published in ***Science*** in March 1981 (338). Of eight chronic schizophrenics, none of the patients improved during active dialysis, and four patients worsened.

In September 1980, the National Center for Health Care Technology (NCHCT) issued a memorandum at the request of the Health Care Financing Administration (HCFA) for a recommendation regarding the use of hemodialysis in the treatment of schizophrenia. NCHCT concluded that the evidence on its safety and efficacy was inconclusive and recommended that the procedure not be covered under Medicare (109).

NCHCT later commissioned an analysis to estimate the economic effect of the decision not to reimburse for the procedure. The study found that under the central estimate, by which 1 percent (4,000 patients) of Medicare-age schizophrenics would receive dialysis in 1984, annual costs would reach a peak of $15.4 million in 1983, and the present value of total costs over a 7-year life of this practice would total $29.3 million. High and low assumptions, under which a minimum of 0.25 percent (1,000 patients) or 5 percent (20,000 patients) of the pool of schizophrenics are dialyzed, result in peak annual costs of $3,9 million to $82.1 million and total costs of $7.7 million to $149.9 million.

If similar fractions of the entire schizophrenic population were to be treated, the total costs of treating schizophrenia with dialysis in this larger pool would be five times higher, or $39 million to $75O million. While funds for patients not treated under Medicare would be provided through Blue Cross/Blue Shield, insurance companies, and private individuals, a portion of this cost would be assumed by the Federal Government as diminished tax revenues due to increased medical expenditures (50).

This case study characterizes NCHCT's former role in responding to HCFA's Medicare coverage issues. It also suggests the utility and need for a continued systematic coverage evaluation process within the Public Health Service. Expenditures for dialysis in the Medicare population alone could have created substantial cost burdens in the absence of a clear Federal policy. In view of the unproved effectiveness of the treatment, NCHCT's recommendation was based on medical/scientific grounds. As this case illustrates, however, it is very difficult to separate cost implications from such decisions.

Percutaneous Transluminal Coronary Angioplasty*

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Introduction

On January 16, 1964, Charles T. Dotter and M. P. Judkins performed the first percutaneous transluminal angioplasty (PTA) (6,27). The patient, an 83-year-old woman, was referred to the University of Oregon Medical School Hospital with a disorder of the left leg. She had a 6-month history of pain and infection of the left foot and toes, and a gangrenous appearance in three toes had occurred within the previous 3 months. Angiographic examination revealed a 0.5 cm long atherosclerotic obstruction of the left superficial femoral artery at the level of the adductor hiatus. The patient was considered unsuitable for vascular surgery owing to her age, poor cardiac condition, and bad runoff. Because of her advanced gangrene, low thigh amputation was advised, which the patient refused.

It was then decided to attempt catheter dilation. Treatment, using a coaxial double catheter, lasted a short time and gave excellent results. The patient's pain disappeared within hours, and the patient was ambulatory within weeks. Repeated followup angigraphy confirmed the patency of the treated artery. The patient continued to walk without difficulty up to her death at the age of 86 years.

With the increase in lifespan over the past few decades, more patients now need surgical vascular reconstruction. This need increased the demand for more centers specializing in vascular surgery and equipped with intensive care facilities (30). Additionally, surgical measures were not always successful when applied to smaller arteries and faced limiting factors such as technical difficulty and operative trauma (6).

● NOTE: Reference citations for David Sawi's case study on PTCA appear on p. 174.

Description of Procedure

PTA is the noninvasive, mechanical treatment of vascular obstructions with the use of catheters (5). The procedure is done by one of two methods, depending on the nature of the lesion.

The first method, called transluminal dilation, is correcting a stenotic lesion by enlarging the diameter in the constricted lumen (or passage). For example, in Dotter's initial procedure ***(6),*** a tapered, radiopaque, Teflon dilating catheter of approximately 0.1-inch outer diameter was slipped over a coil-spring catheter guide of about 0.05-inch outer diameter. The catheter guide had been passed down the lumen until its tip had traversed the stenosis. The passage of the dilating catheter over the catheter guide enlarged the stenotic lesion by exerting outward pressure on the lumen. This method was refined by the introduction in 1974 of a catheter with a distensible (balloon) tip, which, when inflated, exerted outward pressure. on the lumen (12).

The second method, called transluminal recanalization, is used to correct a vascular occlusion by creating an artificial lumen through an occluded segment. The catheter guide is passed through the occluded segment, and then the passage is enlarged by the introduction of the dilating catheter over the catheter guide ***(27).*** This approach is possible because the atheroma causing the obstruction consists of a low-density fatty material which has the characteristic of inelastic compressibility (12,30).

The above procedures are often done in conjunction with anticoagulants and platelet aggregation inhibitors, which seem to improve outcome (30).

There are basically two classes of transluminal angioplasty: peripheral angioplasty (PTA) and coronary angioplasty (PTCA). The technique performed by Dotter and Judkins in 1964 (6) was peripheral angioplasty. In September 1977, Andreas Gruntzig performed the first nonoperative transluminal angioplast y of coronary arteries in a human being (12).

Indications and Alternatives

Indications for PTA are disabling claudication (limping, cramp-like pain due to inadequate blood supply), salvage effort prior to amputation, short stenosis in a large caliber, accessible artery, and little likelihood of success with reconstructive surgery (31).

Indications for PTCA are more restrictive. The patient should have a short history of angina] pain and should be experiencing disabling angina. The obstruction should be within a single vessel to minimize risk should complications occur. Grüntzig estimates that of patients with coronary heart disease, 3 to 5 percent of the older medical population and 10 to 15 percent of the younger population are suitable for PTCA (12).

Alternatives to PTA appear varied. At times, PTA is done as an alternative to surgery, whereas in other instances, it is done when surgery is contraindicated due to high risk and little chance of success (i.e., in elderly patients with prior heart problems) (5). Also, PTA can be an alternative to amputation.

PTCA is an alternative to coronary artery bypass graft (CABG) surgery, but only for a small percentage of patients. As of 1979, it appeared that the 5-year survival rate for PTCA was comparable to that of bypass surgery. However, improved blood flow to ischemic segments was also noted (23).

Historical Development

The development of PTA can be viewed as havin$_g$ four stages: 1) the technical development leading to Dotter and Judkin's initial peripheral angioplasty, 2) the period thereafter during which clinical tests reaffirmed the efficacy of the procedure and began to establish clear-cut indications, ***3)*** the modification of the method by different types of catheters for the dilation technique, which lowered the rate of complications, and 4) the extension of the technique to coronar$_y$ angioplasty (PTCA).

The technological development leading up to Dotter's initial procedure primarily related to the refinement of the catheter. Seidlinger (Sweden) first introduced the flexible catheter in 1953 (25). Meanwhile, Dotter (U.S.) had been refining a catheter for use in occlusion angiography (1958) and diagnostic angiography (1951). In 1962, at about the time Dotter commenced post mortem investigations using a coaxial dilation catheter, Nordenstrom introduced five types of balloon catheters for percutaneous insertion using a modified Seidlinger technique (22). Prior to Nordenstrom, balloon catheters had been introduced into the vessel via an incision. In February 1963, Fogarty (England) used **a** balloon catheter for the extraction of distal thrombosis (8). In the early 1970's, Postmann's (Germany) caged balloon catheter and Wholey's (U. S.) balloon catheters preceded Güntzig's (Sweden) development of a viable and effective balloon-tipped catheter in 1974. It appears as though subsequent development of the catheter instrument has been primaril$_y$ a refinement of Grüntzig's model.

It is clear from this brief overview that researchers in a number of different countries were essentiall$_y$ working on the same problem simultaneously. Two primary dynamics in the catheter development were the desire to minimize the possibility of embolism as

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a result of PTA and the desire to improve the patency rate on followup studies. In regard to this second point, Zeitler reports that between 1968 and 1980, he conducted a randomized study of 1,217 procedures. Treatment conditions included different types of catheters and different drug regimens (29). Zeitler found the patency rate of a newer, improved single Teflon catheter and the Grüntzig balloon catheter to be three times that of the coaxial dilating set.

Numerous other clinical trials have been conducted in addition to Zeitler's study (see table E-l). Two points should be made regarding these studies. One is that the outcomes seem to substantiate the claim that PTA is a viable alternative to incisive treatment and, in fact, can succeed where surgery might fail. The second point is that the trials in table E-1 are not, strictly speaking, comparable with each other. A number of flaws prevent this comparison:

- In later years, better patient selection increased the probability of successful treatment.
- The catheters were constantly being improved, especially in Grüntzig's balloon catheter.
- Criteria for a "successful outcome" are not consistent across trials.
- The skills of the physicians differed.

Additionally, the trials were not ***randomized*** clinical trials, in which patients are randomly assigned to treatment/no treatment conditions, or to treatment A (PTA) /treatment B (e.g., surgery) conditions. The relative efficacy of PTA was determined by comparing treatment outcome v. historical data on outcome using alternative methods. In spite of these studies' methodological flaws mentioned above, their results were sufficiently positive to encourage continued research.

Grüntzig's balloon catheter (1974) is generally credited with being the key to PTA's rapid diffusion (3,4). Grüntzig received research support to develop the balloon catheter from a European firm named Snyder. This seems to have been a significant variable. Dotter had been attempting to refine the balloon catheter for a number of years prior to Grüntzig's success. Dotter's lack of funding support hampered his efforts (4).

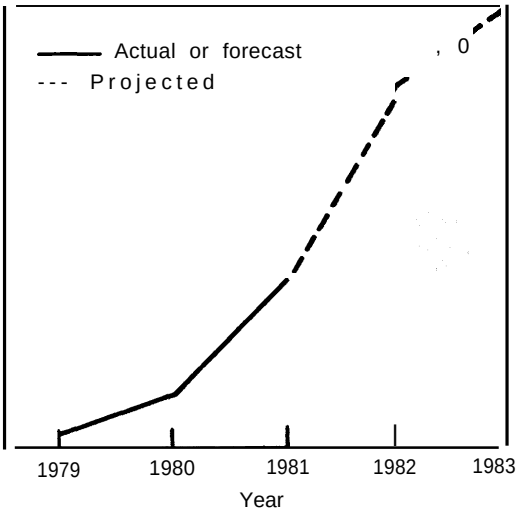
Notwithstanding a decision by Medicare to discontinue reimbursement of PTA, substantial sales growth (in units) is expected for the next few years (see fig. E-l). Based on figures provided by Cook, Inc., the size of the PTA catheter market for 1981 was approximately 100,000 units. This was expected to increase to near-

Table E-1.—Reported Clinical Trials of Peripheral Transluminal Angioplasty

Publication date	Trial dates	Observations [a]	+ %[b]	Comments [c]
1964	1964	15	>50%	Dotter, and Judkins: F,P,I
1966	1/64-9/65	113	~50+ %	Dotter, et al.
1968	"	153	71 "/0	Dotter, et al.: F
1969	"	59	***640/o*** (WA)[d]	Brahme, et al. (Sweden): F
1971	"	161	70%	Zeitler (Germany): F,P,I
1973	"	100	71-890/o	Wierny, et al.: F
1973	"	237	80-900/0	Dotter: F
1973	"	25	840/o	Grüntzig (Switzerland):
1974	"	43	81 0/0	Dotter: 1, with ballon catheter
1975	"	210	***780/o***	Zeitler: 1, with balloon catheter
1975	1969-75	534	740/0 (WA)[d]	Zeitler, et al.:F,P
1978	"	69	"	Zeitler: randomized catheter
1978	9177-8178	61	510!0	Grüntzig: PTCA
1978	11/68-12/73	138	***75 "/0 (500/0)***	Schoop, et al.: I
1978	"	1,184	***74%***	German study with
		206	***920/o***	numerous contributors: F,I
1979	3178-4179	64	"	
	1971-3/78	188	***86°/0*** (balloon)	Grüntzig and Kumpe
		48	64°/0 (coax)	
		300		
1979	"	43	96%	Katzen: balloon catheter
1980	1978-80	172	80%	Colapin, et al. (Canada)
1980	1968-80	1.217	"	Zeitler

Key: "Unstated or unclear.
[a] Indicates number of Procedures performed. Later reports undoubtedly include outcomes of early studies
[b] Generally stated as patency rate within 2 weeks of procedure.
[c] F = femoral," P = popliteal; I - iliac.
[d] WA indicates weighted average primary success rate across various trial conditions,

Figure E-1.–Cook, Inc., Annual PTA Catheter Sales (actual and projected)



Cook, Inc 65 to 75 percent market share, $70,000 per unit
SOURCE M Kanne. Cook, Inc , Bloomington, Ind

ly ***300,000*** units by 1983. After that, growth should level off to an annual rate of 10 to 15 percent (17).

Innovation of PTCA

In 1977', Gruntzig performed the first PTCA (see table E-2). There appear to be two major factors leading to the innovation of PTCA:

- PTCA is, essentially, an extension of the same concept, methodology, and technology as that used in PTA; to extend the concept to coronary arteries was nearly inevitable.
- Gruntzig's refinement of the balloon catheter provided the technology necessary to safely perform the procedure.

Diffusion of PTCA

At present, three factors are driving the diffusion of PTCA. The first factor is Gruntzig himself. Gruntzig's impact has been felt through the instruments he developed to perform the technique; his contribution to the literature (see table E- 2); his refinement of the procedure, with meticulous recording of numerous clinical trials; and the training he has provided to other physicians, both in Zurich and other sites to supervise initial operations.

A second factor is the Food and Drug Administration's (FDA's) decision in late 1980 to release the PTCA catheter device from investigational device exemption (IDE) status (19). An IDE status limits a company's ability to market a product by placing strict guidelines on the product's distribution and use. Each purchasing institution has to be designated as an investigator by FDA, and this involves considerable documentation and takes 2 to 3 months for approval. With removal of IDE status, the restrictions are removed, and marketing and purchasing of the catheter are simplified. USCI, one of the two manufacturers, claims that since its catheter received premarketing approval status, the number of centers which **have** ordered equipment to perform PTCA has doubled (9).

The third factor is the Interim Registry of PTCA at the National Heart, Lung, and Blood Institute. This registry receives voluntary information from centers performing PTCA within the United States and reports on successes, measurements, complications, and followups. The availability from the Interim Registry of information showing outcomes which are consistently comparable with alternative, invasive procedures nevertheless has a powerful influence in predisposing physicians to accept PTCA. It also creates pressures on third-party reimbursers to extend coverage to a procedure whose efficacy does not appear to be in doubt.

Table E-2.—Reported Clinical Trials of PTCA

Publication date	Trial dates	Observations	+ %	Comments
1977	—	—	—	Gruntzig: animals and post mortem studies
1978	—	—	—	3 post mortem and 1 animal study reported[a]
1978	9/77-12/77	7	86 %	Gruntzig
1978	1977-78	29	70%	Gruntzig, et al.
1979	•	65	600/0	Gruntzig, et al. United States and Switzerland, 5 centers
1979	1/78-7179	50	680/0	Gruntzig, et al.
1979	9/77-10/79	163	620/o	Gruntzig

Key: 'Unstated or unclear.
[a] *Circulation* 57 (suppl 2):80, 1978

Considerable uncertainty exists over the completeness of the registry's data base. The cumulative number of procedures reported to the registry is shown in table E-3, As of May 1981, the registry had 100 centers reporting to it (19); however, USCI and Advanced Catheter Systems (ACS), the two manufacturers of the catheters used to perform MCA, estimate the number of purchasers (i.e., centers) to be between 300 and 400. FDA's release of the catheter from IDE status in late 1980 made it available to users other than reporting centers. According to the manufacturers, therefore, the registry's data undercount the number of procedures actually performed.

Third-Party Reimbursement

It was initially thought that PTCA was not reimbursed by third-party carriers. This is widely believed by numerous professionals in the field. However, subsequent information indicated that this was not the case and that the situation is as follows.

First, Medicare, MediCal, Blue Cross, and Blue Shield do not reimburse for PTCA. The procedure is viewed **as** being investigational, and the primary reservation is the lack of data on clinical effectiveness. Data coming out of the Interim Registry and FDA's decision are viewed as important precursors to recognition from the aforementioned carriers (7,20).

Second, grants and free service have covered the costs in some institutions. For example, at Stanford University Hospital, PTCA has been recognized as a "research procedure. " Thus, either research grants have covered the costs, or the service has been provided free (l). In 1981, PTCA was being reviewed to determine whether it could be called a "billable procedure, " in which case third-party reimbursement would be sought to the extent that it is available.

Third, most private carriers* reimburse for PTCA to some extent, either knowingly or otherwise. In some cases, the charges for PTCA are "buried" in other, chargeable, items, such as catheter laboratory charges, coronary angiograms, etc. (7,19,26). More often, it seems, the procedure is openly identified as PTCA and charged accordingly.

The latter is the procedure used, for example, by Steven Myler of San Francisco, who has an active practice in PTCA. * The patient is billed and pays for the procedure. The patient is then reimbursed for expenses by the insurance carrier to the extent designated within the policy. Insurance coverage appears to have begun in 1980 or 1981, though it is difficult to determine exactly when. St. Mary's Hospital, in which Myler does his work, indicates that third-parties have not refused payment, though some have questioned the new procedure, The only difficulty is in being reimbursed for the procedure itself. The hospital visits are reimbursed without question.

A number of major health insurance carriers were contacted. One carrier, New York Life Insurance, said "anything ordered by an M.D. is covered, unless specifically excluded" (21). A second carrier, Mutual of Omaha, stated that their major catastrophic insurance covered "treatment by a physician or surgeon" and "service by a radiologist for diagnosis of treatment" (20).

The fact that PTA is a familiar procedure with proven efficacy makes PTCA more acceptable to carriers. (HCFA has approved payment for the PTA procedure when used in lower extremities.)

Additionally, FDA's decision to release the catheter from the investigational devices list is viewed as important. With various restrictions due to an IDE status, health care facilities are reticent to do PTCAs During the investigational stage, physicians keep informed of the development of the procedure. Once it is cleared by FDA, acceptance is fairly rapid (see fig. E-2) (20).

Market Factors

Of the approximately 100,000 CABG procedures performed each year, approximately 10 percent of these could be replaced with PTCA. Thus, an estimate of the maximum annual demand of the primary market for PTCA is: 100,000 X 10 percent = 10,000 procedures per year. Additional demand could be realized if indications for the procedure were broadened.

Currently, there are two manufacturers of the special catheter used in PTCA: USCI of Massachusetts and ACS of Santa Clara, Calif. It seems unlikely that additional companies will enter the field. The market is limited in size. Also, the technological barriers to entry are substantial, By 1981, USCI's catheter had

● Excluding Blue Cross and Blue Shield.

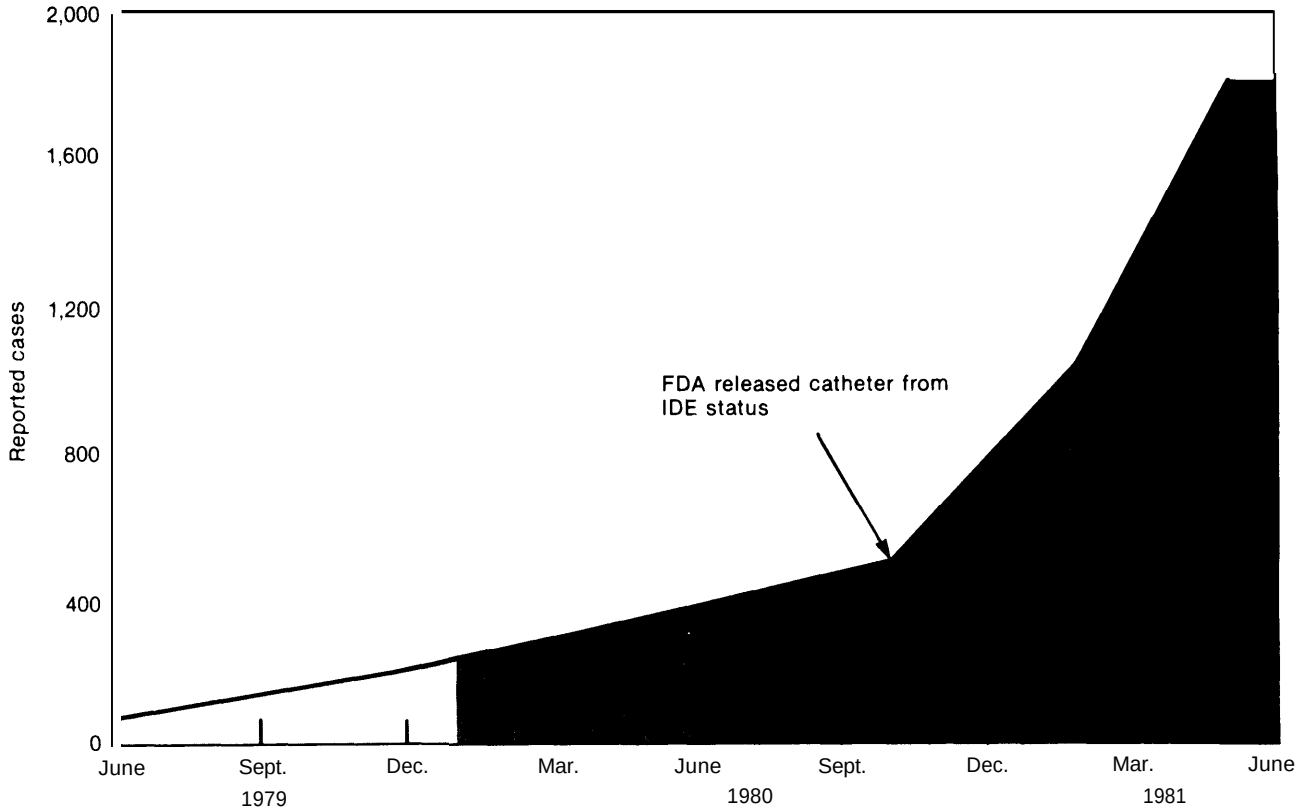
Table E-3.– Reported Use of PTCA

Date	Cumulative number of Procedures reported	Number of centers
June 1979 ....., . . .	61	5
December 1979	200	—
June 1980		60
Fall 1980—FDA released catheter from list of investigational devices		
October 1980	504	—
February 1981	1,016	—
May 1981	1,800	100

SOURCE: S. Mull in, Interim Registry of PTCA, Cardiac Disease Branch, National Heart, Lung, and Blood Institute.

● Ann, of Dr. Myler's office, personal communication.

Figure E-2.—Reported Cases of PTCA



SOURCE: S. Mullin, Interim Registry of PTCA, Cardiac Disease Branch, National Heart, Lung, and Blood Institute.

been released from IDE status, whereas ACS's catheter had not. ACS expected to receive premarketing approval within 6 months.

The price to the patient of a CABG is quoted as $20,000 at Stanford University Hospital. The price of PTCA is ***$3,000 (see*** table E-4). The source of the cost of PTCA was unable to state unequivocally whether the price included a backup team for CABG. However, the patient would be charged $3,000.

Some percent of those patients undergoing PTCA eventually have CABG performed anyway. A true cost comparison should factor this in (see table E-5). The cost savings are such that it would be necessary for 85 percent of the patients who receive PTCA to ***also*** have CABG before the cost savings advantage of PTCA would be nullified.

Table E-4.—Cost Comparison: PTCA, CABG

CABG	$20,000
PTCA:	
Supplies:	
Catheter	$387
Other catheters	
Medical supplies	500
Other	93
	$980
Personnel: Clerical, technician, two cardiac cath lab nurses (3 hours for procedure)	$74
Benefits	15
	$89
Coronary hemodynamic (partial charge)	$75
Indirect costs, departments and hospital	137
	$1,281
Adjusted for inflation, 15°/0	1.15
	$1,473
Profit markup, 100/0	1.10
	$1,620
Physician fees	1,275
Total cost (price to patient)	$2,895

SOURCE: P Berry, Cardiac/EKG Department, Stanford University, 1981.

Table E-5.—Expected Cost and Break-Even

Expected cost
Assume: 10 to 15°/0 of PTCAs are followed up with CABGs.
Expected cost per patient:
(3,000) (1.00) + (20,000) (0.10) = $5,000
(3,000) (1.00) + (20,000) (0.15) = $6,000
Expected cost per patient = $5,000 to $6,000.
Break-even:
$3,000 + **$20,000 (x) = $20,000**
20,000 (x) = 17,000
X = 0.85
where X = percent of patients having PTCA who also need CABG.

Sawi's References

1. Berry, P., Business Manager, Cardiac/EKG Department, Stanford University Hospital, personal communication, June 1, 1981.
2. Brahme, F., et al, "Evaluation of Transluminal Recanalisation of the Femoral Artery," *Acta Chir. Scand.* 135:679, 1969.
3. Colapin, R. F., et al., "Percutaneous Transluminal Angioplasty of Peripheral Vascular Experience: A Two Year Experience," in ***Cardiovascular and Interventional Radiology,*** E. Zeitler (cd.) (Berlin, Heidelberg: Springer-Verlag, 1980).
4. Dotter, C. T., Oregon State University, Portland, Oreg., personal communication, Apr. 24, 1981.
5. Dotter, C. T., "Transluminal Angioplasty," in *Angiography,* vol. 2, 3d cd., H. L. Abrams (cd.) (Boston: Little Brown & Co., 1980).
6. Dotter, C. T., and Judkins, M. P., "Transluminal Treatment of Arteriosclerotic Obstruction," ***Circulation,*** 30:654, ***1964.***
7. Finklestein, S., Alfred P. Sloan School of Management, Massachusetts Institute of Technology, personal communication, May *29, 1981.*
8. Fogarty, T. J., et al., "A Method for Extraction of Arterial Emboli and Thrombi," *Surg. & Gynec. Obstet.* 116:241, 1963.
9. Grossman, N., Product Manager, Cardiac Catheters, USCI, Billerica, Mass., personal communication, May 29, 1981.
10. Grüntzig, A. R., "Coronary Percutaneous Transluminal Angioplasty: Preliminary Results," ***Circulation 58*** (Suppl. 2):56, *1978.*
11. Grüntzig, A. R., "Coronary Transluminal Angigraphy," ***Circulation 56*** (Suppl. 3):84, 1977.
12. Grüntzig, A. R., "Percutaneous Transluminal Coronary Angioplasty," unpublished document obtained from H. Abrams, Stanford University, April 1981.
13. Grüntzig, A. R., "Percutaneous Transluminal Coronary Angioplasty—Present State of the Art," ***Circulation*** 60:1031, ***1979.***
14. Grüntzig, A. R., "Transluminal Dilatation of Coronary Artery Stenosis," letter, *Lancet* 1:263, 1978.
15 Grüntzig, A. R., et al., "Nonoperative Dilatation of Coronary Artery Stenosis: Percutaneous Transluminal Coronary Angioplasty," N. *Eng. J. Med.* 301:61, ***1979.***
16 Grüntzig, A. R., et al., "Percutaneous Transluminal Coronary Angioplasty: A Cooperative Study," Am. *J. Cardiol.* 43:384, ***1979.***
17. Kanne, M., Executive Vice-President and Sales Manager, Cook, Inc., Bloomington, Ind., personal communication, May *29, 1981.*
18. Katzen, B. T., and Chang, J., "Percutaneous Transluminal Coronary Angioplasty With Grüntzig Balloon Catheter," ***Radiology*** 130:623, ***1979.***
19. Mullin, S., Interim Registry of PTCA, Cardiac Disease Branch, National Heart, Lung, and Blood Institute, Bethesda, Md., personal communication, May 14, 1981.
20. Mutual of Omaha, Medical Coverage Representative, personal communication, May***29, 1981.***
21. *New* York Life Insurance, Coverage Representative, personal communication, May 29, 1981.
22. Nordenström, B., "Balloon Catheters for Percutaneous Insertion Into the Vascular System," *Acta Radiologica* 57:411, ***1962.***
23, Rapaport, E., "Percutaneous Transluminal Coronary Angioplasty—Editorial," ***Circulation*** 60(5):969, ***1979.***
24, Schoop, W., et al., "Early and Late Results of PTD in Iliaca Stenosis," in ***Percutaneous Vascular Recanalization: Technique, Application, Clinical Results,*** E. Zeitler, et al. (eds.) (Berlin, Heidelberg: Springer-Verlag, 1978).
25. Seidlinger, S. I., "Catheter Replacement of the Needle in Percutaneous Angiography," *Acta* ***Radiological*** 39:368, ***1953.***
26, Simpson, C., Advanced Catheter Systems, Santa Clara, Calif., personal communication, May 22, 1981.
27. Van Andel, G. J., ***Percutaneous Transluminal Angioplasty: The Dotter Procedure***(Amsterdam-Oxford: Excerpta Medica, 1976).
28. Wierny, et al., "Long-Term Results in 100 Consecutive Patients Treated by Transluminal Angioplasty" ***Radiology*** 112:543, ***1974.***
29. Zeitler, E., "Percutaneous Dilatation and Recanalization of Iliac and Femoral Arteries," in ***Cardiovascular and Interventional Radiology,*** E. Zeitler (cd.) (Berlin, Heidelberg: Springer-Verlag, 1980).
30. Zeitler, E., et al. (eds.), ***Percutaneous Vascular Reconstruction: Technique, Application, Clinical*** *Results* (Berlin, Heidelberg: Springer-Verlag, 1978).
31. Zeitler, E., et al., "Treatment of Occlusive Arterial Disease by Transluminal Catheter Angioplasty," ***Radiology*** 99:19, 1971.

Maternal Serum Alpha= Fetoprotein

The Federal involvement with MSAFP for detection of fetal neural tube defects is a case study that illustrates successful technology assessment monitoring and interagency coordination The determination of the level of alpha-fetoprotein in maternal serum is the first step in a sequence of diagnostic tests used to screen and diagnose fetal neural tube defects. It aids in detecting two types of defects, anencephalia (absent or undeveloped brain) and open spina bifida (failure of the spine and overlying skin to close over the spinal cord), which together affect ***3,000*** to ***6,000*** newborns in the United States each year. Followup procedures, for use

when results of screening are abnormal, include repeat serum testing, ultrasonography, and amniocentesis (108).

In late 1978/early 1979, FDA was on the verge of approving a 2-year interim period for widespread usage of MSAFP. However, a special interest group, the spina bifida parents, questioned the quality of the FDA data and the impending diffusion of MSAFP. The Centers for Disease Control (CDC) also became concerned over manufacturers' ability to consistently produce quality components for a marketable kit, as well as over physicians' readiness to work in close cooperation with their patients. Compounded further by ethical and reimbursement issues, CDC sent a formal memorandum to the Office of Health Research, Statistics, and Technology Director, enumerating the concerns with the technology. As a result, the MSAFP screening test was discussed at a meeting of the Technology Coordinating Committee of the Department of Health and Human Services; and NCHCT was asked, through the committee, to coordinate development of departmental policy (108,315).

In November of 1979, former Secretary Harris was briefed, at her request, on departmental activities relating to MSAFP. NCHCT coordinated the briefing. It was agreed that the Public Health Service (PHS), through CDC, should conduct **a** controlled epidemiologic field study to obtain needed clinical data on MSAFP and to supplement FDA's postmarketing surveillance and data collection program. CDC's protocol was subsequently reviewed by the Technology Coordinating Committee. In July of 1980, NCHCT and FDA cosponsored a national educational conference on MSAFP. By November of 1980, regulatory proposals had been readied for publication **(119).**

In the November ***7, 1980,*** issue of the Federal Register, FDA published proposed regulations to restrict the sale, distribution, and use of alpha-fetoprotein test kits used in detecting fetal neural tube defects. Also, in that same edition of the Federal Register, CDC and HCFA published jointly proposed regulations pertaining to quality control and proficiency testing for clinical laboratories engaged in alpha-fetoprotein testing. Public hearings on these regulatory proposals were held on January 15-16, 1981. Testimony was presented by some **40** to **50** individuals at these hearings, and approximately ***650*** written comments were received by FDA and CDC/HCFA subsequent to the publication of the proposed rules. These responses were analyzed and assessed by FDA and CDC/HCFA. FDA found that it had several options in regard to the restricted or unrestricted release for marketing of the alpha-fetoprotein test kits; these were under review as of January 1982 by Arthur Hayes, the FDA Commissioner (120).

Hemodialysis and Kidney Transplant Surgery*

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Background Information

Hemodialysis and kidney transplant surgery are two alternative forms of therapy for chronic renal failure. Permanent or chronic or end-stage renal disease (ESRD) occurs when an individual irreversibly loses a sufficient amount of kidney function so that life cannot be sustained without treatment intervention. Chronic renal failure may be caused by any of a number of separate diseases (glomerulonephritis, pyelonephritis, polycystic kidney disease, hypertension, diabetes mellitus, and others), but the uremic or ESRD state is their final outcome.

Hemodialysis is a treatment that involves using a machine—the artificial kidney—to achieve the vital functions previously performed by the kidneys. A patient undergoes the treatment from 4 to 6 hours a day, two or three times a week. The treatment can be given in hospital-based dialysis units, freestanding units (for-profit or not-for-profit), or in the patient's home. A fast growing substitute for hemodialysis is continuous ambulatory peritoneal dialysis (CAPD), a technique that uses the peritoneum (lining of the abdominal cavity) to cleanse the blood of its impurities.

The alternative treatment to dialysis is kidney transplantation, which is performed with kidneys from either living related donors or cadaveric donors. The best kidney survival rates are achieved with living related donors, especially siblings.

DEVELOPMENT OF THE TECHNOLOGY

Development of the hemodialysis technology began as early as 1913, when Abel, Rowntree, and Turner performed the first dialysis in animals at the Johns Hopkins Medical School **(8,27).** This team built their own dialyzing system and coined the expression "artificial kidney." On February 28, 1926, Haas performed the first hemodialysis in a human being, using Hirudin (prepared from leech heads) as the anticoagulant (9). The development of the anticoagulant Heparin greatly enhanced the dialysis procedure, as did the marketing of cellophane for use as the artificial dialyzing membrane. Because of an inability to repeatedly access the bloodstream of people suffering from chronic renal failure, however, the use of hemodialysis

● NOTE: Reference citations for Katherine Jones' case study on hemodialysis and kidney transplant surgery appear on p. 183.

was limited to patients with acute, reversible kidney failure.

In 1943, Kolff developed the first practical model for human hemodialysis, building an artificial kidney using a rotating drum (27). It took 2 more years before Kolff achieved his first success in treating acute renal failure patients with hemodialysis. Independently of Kolff, Alwall in Sweden and Murray in Canada were also developing types of hemodialysis machines, and all three published their experiences at about the same time ***(9,27).***

Use of the artificial kidney for patients with acute renal failure continued until 1960. In that year, a critical technological advance was made when Quinton and Scribner reported the use of a subcutaneous arteriovenous shunt (a plastic tube connected to an artery and a vein in the arm or leg), which allowed repeated access to the circulatory system and thus permitted continuous dialysis treatments (27). After this important development, technological advances were made in the areas of improved blood access devices, dialyzing membranes, dialysis machines, and types of artificial kidneys ***(8,9).***

The first kidney transplant was performed at Harvard by Hufnagal and Hume in 1947 (27). A cadaver kidney was transplanted into the antecubital fossa (area in front of the elbow) of a young woman dying from acute renal failure. The kidney functioned for 2 days, lasting long enough for the patient to regain her own renal function. From ***1951*** to ***1953,*** Thorn and Merrill referred several patients to Hume, who performed several transplants. They watched patients experience a reversal of their uremic state only to experience a rejection of the kidney in a few days ***(27).***

In February of 1953, the first success was achieved by Hume and his associates. A person survived with a functioning kidney transplant for **5** months and 25 days and demonstrated the potential of the procedure (21). This case also marked the end of experimental transplantation without the use of immunosuppression to prevent graft rejection (21,27).

In 1954, the first transplant between monozygotic twins was performed by Murray and his associates in Boston (2). This case demonstrated that monozygotic twins were, indeed, immunologically identical. Within 5 years, this group had performed eight transplants between twins and had perfected the surgical technique of retroperitoneal iliac fossa (groin area) placement of the graft (2). The initial transplant recipient lived for 8 years before dying of myocardial infarction (21,27).

Clinical and animal experiments with different forms of immunosuppression occurred in 1958 and 1959. Total body irradiation usually proved to be fatal and was soon abandoned (2). Schwartz next introduced drug-induced immunological tolerance, first using mercaptopurine, then switching to a superior derivative called Imuran, a drug still in use today (14,27). Steroids joined Imuran in treatment of graft rejection in ***1962 (2).*** In 1963, Terasaki began using serotyping techniques to select immunologically favorable kidney donors (2). New tissue-matching techniques, organ acquisition and preservation procedures, and immunosuppressive drugs have since been introduced, although improvement is still needed in long-term organ survival rates (21,27).

DIFFUSION OF THE TECHNOLOGY

In the early 1960's, hemodialysis and kidney transplant became accepted as life-extending therapies for victims of chronic renal failure. In America, much of the research was initially supported by the John A. Hartford Foundation and later by the Artificial Kidney / Chronic Uremia Program (AKCUP) of the National Institutes of Health (NIH). AKCUP in the National Institute of Arthritis, Metabolism, and Digestive Diseases (NIAMDD) was founded in 1965 with a contract research program to build a better artificial kidney. This program was mandated by the House and Senate Appropriations Committees 1 year after the artificial heart program, although the artificial kidney was more developed at the time ***(27).***

The development of hemodialysis as a mode of therapy posed complications for NIH. NIH found it difficult to support Scribners development of clinical applications of the artificial kidney and was never prepared to do so on the scale he requested (2). The NIH orientation toward biological and biochemical processes led to a preference to fund research on kidney disease etiology, not the clinical applications of that research. Funding for transplant research was not as problematic, since those involved with immunological research were well known as basic researchers ***(27).***

In 1964, the Senate Appropriations Committee stated that PHS had the authority to provide demonstration and training funds for artificial kidney programs. In 1965, PHS established the Kidney Disease Control Program (KDCP), which funded 14 community treatment centers around the country and demonstrated the organizational feasibility of dialysis in various settings. Although these contracts were gradually phased out beginning in 1968, many PHS-funded dialysis centers became nationally prominent hemodialysis provider institutions (27).

In 1969, KDCP became part of the Regional Medical Program (RMP), and the emphasis shifted from demonstrations of feasibility to the building of dialysis

capacity (27). This was accomplished through centralized funding and policy control in Washington, and decentralized funding of facilities through the RMP agencies ***(27).***

A very significant role in the diffusion of dialysis technology was played by the Veterans Administration (VA). In ***1963, 2*** years before the initiation of AKCUP and KDCP, VA announced its intention to establish dialysis centers in ***30*** VA hospitals (27). It proceeded to do so over the next several years. Dialysis and transplantation were provided for all qualified veterans with chronic kidney failure, whether or not service-connected. Public Law 89-785 (Nov. 7, 1966) provided that nonveterans could also receive such services from VA hospitals if facilities were available, but that VA had to be reimbursed the full cost of services rendered under agreements with other hospitals (27,31).

By 1971, VA had initiated a home dialysis program, had opened its first home training unit, and had also initiated satellite dialysis. By 1972, VA was dialyzing 25 percent (979 patients) of the Nation's dialysis patients in 44 treatment centers and another 26 patients in branch centers. As of March 1973, the VA system had ***501*** dialysis beds (including ***123*** for home training) and 10 hospitals that were operating branch dialysis centers. VA had also provided backup medical services for 766 patients being dialyzed at home. Thirty-three VA hospitals reported a total of ***327*** transplants performed in ***1972.***

The activity in PHS and VA related to hemodialysis and transplantation prompted the Government to conduct a high-level policy review of the situation. The Bureau of the Budget established the Gottschalk committee in 1965 to review the implications of therapy for ESRD for the entire Nation (27). In 1967, the Gottschalk committee (12) declared that hemodialysis and transplantation were acceptable forms of therapy and recommended that a national treatment benefit program be established by amending title XVIII of the Social Security Act. No legislation was enacted as a result of this report, partly because of the simultaneous release of a PHS report that emphasized research and prevention rather than treatment.

Gottschalk (12) estimated that in 1962, one out of every five patients dying from chronic uremia was medically suitable for dialysis and transplantation. Of the ***7,000*** *new* renal patients in 1968 who ***would*** be suitable for treatment, transplants were available for approximately 450 and chronic dialysis for approximately 550 (12). The treatment technology was available but was prohibitively expensive, In 1965, the costs for dialysis and transplant were as follows (12):

- ***home dialysis:*** average $6,000 per year, range $3,750 to $9,800;
- ***in-center/hospital dialysis:*** average $10,000 per year, range **$8,400** to $21,000; and
- ***transplant:*** average $13,300 surgery and recovery plus $200 to $1,000 per year, range $10,000 to $22,000.

The eight primary sources of funding for dialysis and transplantation prior to the establishment of the ESRD program were the following (7):

1. ***Medicare.*** —This program helped finance treatments for persons 65 and over (but few patients this age were selected for treatment).
2. ***Medicaid.*** —Under this program administered by the Social and Rehabilitation Service, the States could assist in paying for kidney disease treatment. Eligible individuals were those who received public assistance under certain titles of the Social Security Act, or those persons in certain States whose income and resources were insufficient to meet medical needs. Services varied among the States.
3. ***Vocational Rehabilitation Act.*** —Using Federal grants to the States, this program helped 300 to 400 individuals with kidney disease annually at a cost of about $1 million.
4. ***Comprehensive Health Planning (authorization expired June*** 30, ***1974).*** —According to the Department of Health, Education, and Welfare, RMP of the Health Resources Administration was, as of April 1975, investing $4.8 million in grants to develop chronic kidney disease treatment services reimbursable under the Medicare program. Funds were primarily aimed at startup costs of cadaver kidney procurement systems and specialized laboratory services.
5. ***Military.*** —The Army, Navy, and Air Force provided a limited number of dialysis machines for home and center programs through the Civilian Health and Medical Program of the Uniformed Services.
6. ***Research,*** —NIAMDD was the NIH component primarily responsible for supporting kidney disease research. The National Institute of Allergy and Infectious Diseases (NIAID) of NIH supported research on the infectious and immunological aspects of kidney disease and was the major source of funds for transplantation.
7. ***State and Private Involvement. —A*** General Accounting Office (GAO) survey of 14 States in 1973 found 8 States that were appropriating funds to directly assist patients with kidney disease. Wide variation existed between the States: One

State legislature appropriated about $4,400 per 100,000 population, while another appropriated about $11,000 per 100,000 population. Two of the States operated their own dialysis facilities.

8. ***Private Sources.*** —Private sources included out-of-pocket payments, savings withdrawals, private health insurance, the National Kidney Foundation, local community organizations and fund drives, and employer contributions.

ESRD Program

From the mid-1960's until 1972, a long policy debate occurred over who should be responsible for funding treatment of patients with chronic renal failure. The issue was publicized in the media, and the existence of "death committees" (groups of physicians or health professionals who decided who would be dialyzed and who would be allowed to die) became well known ***(18).*** There was mounting pressure for the Federal Government to take action that would relieve the patients and other payers of the expensive burden of this lifesaving technology.

The debate culminated in 1972 with the passage of section 2991 of Public Law **92-603** of the Social Security Amendments of 1972, a law that extended coverage for renal disease treatment to over **90** percent of the population **(30).** Medicare eligibility began in the third month after the month in which a course of dialysis was initiated and ended in the 12th month after the month in which a person had a functioning kidney transplant. Factors that led to the congressional decision to pay for ESRD treatment included a recognition that the alternative to life sustainment by dialysis was death, that ESRD treatment was very expensive, and that there occurred 7,000 to 10,000 uremic deaths a year because of the limited availability of dialysis facilities.

HCFA assumed responsibility for the ESRD program in ***1978.*** HCFA prescribes standards for treatment by its regulations and approves payments for services through local insurance companies or other intermediary agencies. HCFA is also responsible for the quality of care that patients receive and exercises that responsibility through existing National, regional, and State agencies.

CHANGES IN THE PATIENT POPULATION

There have been changes in the number of patients receiving transplants and the proportion of patients receiving home dialysis since institution of the ESRD program.

Transplants.—Between 1963 and 1972, the number of kidney transplants had been increasing steadily, going from **163** to 1,993 a year (7). After 1972, the number grew at a slower pace, and it plateaued in 1975 (10,26) (see table E-6).

The reasons for the decline in transplants included lack of improvement in graft success rates (although patient survival rates ***had*** improved), decreased donor pool due to smaller families, and financial disincentives in the Medicare regulations. Benefits ended the 12th month after transplant surgery. If a person lost his or her kidney after this time period, that person had to undergo another 3-month waiting period before Medicare began paying for the resumed dialysis treatments. The patient also had to pay the costs associated with transplant failure, In 1975, GAO (7) recommended that the waiting period and associated disincentives for transplant, a less costly treatment modality, be eliminated from the law. These recommendations were implemented in ***1978.***

Hemodialysis.—The number of patients receiving hemodialysis grew rapidly after 1970. From 11,000 dialysis patients in 1973, the program expanded to about 50,000 dialysis patients in 1980 (see table E-7). This growth occurred primarily as a result of changes in patient selection criteria. Originally, selection was limited to patients 15 to 45 years of age who were in good health apart from their renal disease. Today, there is essentially one criterion for acceptance—

Table E-6.—Number of Kidney Transplants Performed in the United States, 1951.79

Year	Number[a]	Percentage change
1951 -62	75	
1963	163	117.3"/0
1964	239	46.6
1965	305	27.6
1966	338	10.8
1967	448	32.5
1968	676	50.9
1969	838	24.0
1970	1,091 (1,460)	30.2
1971	1,616 (2,909)	48.1
1972	1,993 (2,852)	23.3
1973 (Medicare coverage)	3,017	51.4
1974	3,190	5.7
1975	3,730	16,9
1976	3,504	–6.1
1977	3,973	13.4
1978	3,949	–0.6
1979	4,271	8.2

[a]Numbers in parentheses reflect discrepancies in the literature.

SOURCES: Comptroller General of the United States, *Treatment of Chronic Kidney Failures: Dialysis, Transplant Costs, and the Need for More Vigorous Efforts, 1975;* Office of Special Programs, Office of End-Stage Renal Disease, Health Care Financing Administration, *End-Stage Renal Disease Second Annual Report to Congress, Fiscal Year 1980,* 1980; and E. A. Freedman, et al., "Pragmatic Realities in Uremia Therapy," *N. Eng J. Med.* 298:368, *1978*

Table E-7.–ERSD Patient Population, 1972.80

Year	Number of hemodialysis patients	Percentage of patients on home dialysis[a]	Number of patients on CAPD[b]	Percentage of patients over 65 years	Number of dialysis facilities
1972 ., ...	10,000	400/o	—	NA	NA
1973	11,000	35.90/o	—	NA	NA
1974	18,875	32.70/o	—	50/0	664
1975	22,000	280/o	—	11%	NA
1976	30,131	23.70/o (13°/0)	—	17 "/0	700
1977	32,435	11.56% (20%)	—	20%	761
1978	36,463	12.42 '10	8	19%	850
1979	45,565	130/o (10%)	1,800	NA	950
1980	50,000	NA	3,000	NA	1,000

[a]Numbers in Parenthesis reflect conflicting reports in the literature.
[b]Medicare coverage began in 1978
NA = not available.

SOURCES' Office of Special Programs, Office of End-Stage Renal Disease, Health Care Financing Administration, End-Stage *Renal Disease Second Annual Report to* Congress, *Fiscal* Year 1980, 1980; R. Rettig, *Implementing the End-Stage Renal Disease Program of Medicare, 1980;* and Health Services Administration, *End-Stage Renal Disease Medical /formation System–Facility Report Number 1, 1976.*

disabling uremia. A large proportion of the dialysis population is either very young or very old and suffers from other serious diseases, such as liver disease, cancer, and diabetes. Table E-7 also shows that there has been a fairly rapid *decrease* in the proportion of home dialysis patients since the beginning of the ESRD program.

When the Social Security Amendments of 1972 were passed, 40 patients per million were receiving long-term hemodialysis treatment in the United States, almost entirely under the auspices of nonprofit organizations (24). The number of patients now receiving dialysis treatments in the United States exceeds 200 per million population, an eightfold increase, and is the highest in the world ***(24).***

The composition of patients receiving dialysis treatment in the United States has also changed since 1972. The average age of the maintenance dialysis population has increased, The mean age of dialysis patients rose from ***42*** in 1970 to 50 in ***1977 (18,31).*** Americans well past retirement age may be placed on dialysis. A survey 01 European dialysis centers in 1977 revealed that 70 percent had no age limit to dialysis; 22 percent as a general rule excluded patients over 65; and 8 percent excluded patients over age 55 (l).

CHANGES IN THE USE OF THE TECHNOLOGY

The changes in the treated renal patient population have occurred mainly as a result of increased availability of funding for dialysis and transplantation. The increasing number of new patients presenting each year, increasing average age of new patients, and increasing number of patients with serious complications have contributed to the decreasing use of home dialysis and transplantation and to an overall rise in morbidity and mortality.

There has been much national concern expressed over the declining proportion of home dialysis patients, since significantly lower costs are associated with home treatment, especially after the first year. The Medicare regulations themselves included disincentives for home dialysis (3,7). For example, the Medicare regulations did not require centers to provide training programs for home dialysis (7). Instead, they required more out-of-pocket costs for home dialysis supplies and equipment and did not provide reimbursement for the services of a home dialysis assistant nor for the effort involved in renting equipment, ordering supplies, and other bookkeeping requirements. Home patients incurred additional costs for home modification and higher electric and water bills (13,19).

Some of the movement back to facility dialysis was the result of the stresses on family life caused by home dialysis. Opponents of home dialysis have stated that many patients were initially placed on home dialysis solely because of limited funds for facility dialysis treatment and that Medicare has removed these financial barriers to the preferred treatment setting. Other factors in the home dialysis / facility dialysis controversy include the personal philosophy of the physician or hospital treating the patient, increased age and morbidity of dialysis patients that reduce their suitability for home treatment, and the impact of facility proprietary-status on the location decision outcome.

Proponents of home dialysis have argued that the costs of home dialysis are lower than those for facility dialysis, that quality of life is improved as patients are more independent and able to work, and that morbidity and mortality rates are lower (5). Using data from 1972, the NIH National Dialysis Registry reported a home dialysis 3-year mortality rate of 21.4 percent and a facility dialysis 3-year mortality rate of 28.6 percent (13). Using 1976 cumulative data, it re-

ported the following annual death rates: home dialysis, 6.7 percent; freestanding-unit dialysis, 7.5 percent; hospital-based-unit dialysis, 10 percent.

GAO in **1977** reported that mortality rates for dialysis patients were unavailable to it and that GAO was therefore unable to compare mortality rates of home-treated v, center-treated dialysis patients (6). Neither the Medicare billing system nor the ESRD medical information system recorded morbidity data (4). The ESRD amendments of 1978 mandated HCFA to collect such data, and information on hospital admissions, average length of stay by disease category, survival rates, and program costs associated with alternative treatments of ESRD was to be reported in 1981.

GAO ***was*** able to collect cost information and in 1975 reported the following data based on 1972-73 cost or charge information (7):

Type of dlalysis	*Average yearly cost*	*Range*	*Per treatment cost*
Home	**$14,900-first year**	**$9,300-$22,200**	**$96**
	$7,000 -following years	**$3,900-$10,300**	**$46**
Freestanding	**$27,600**	**$16,440-$41,003**	**$203**
Hospital-based	**$30,500**	**$11,500-$49,100**	**$202**

NIH cost estimates (excluding physician fees) based on 1973 cost data (5 dialysis centers) were as follows (10):

Type of dialysis	*Average yearly cost*	*Per treatment cost*
Home	$15,000-first year	$33-$66
	$6,500-following years	
Freestanding	$16,520	$100-$116
Hospital-based.	$24,738	$146-$259

In 1973, the Medicare reimbursement rate was **$150** for center dialysis and $50 for home dialysis, both including physicians' fees and assuming 156 treatments a year.

In 1977, GAO estimated the following costs for home dialysis (6):

First year	
Equipment	$6,800
Training—24 treatments @$158	3,792
Physician fees (training)	500
Physician fees (supervision)–	
12 months @$140 ... ., ..., ...	1,680
Backup dialysis–16 treatments @$138	2,208
Supplies and equipment–116 treatments @$55.	6,380
Reasonable charges covered by Medicare	$21,360
Second year	
Backup dialysis–19 treatments @$138 . . .	$2,622
Physician fees–12 months @$140	1,680
Supplies and equipment–137 treatments @$55.	7,535
Reasonable charges covered by Medicare	$11,837

A study by Roberts, et al. (29), estimated the following costs for dialysis and transplantation in 1980:

Home dialysis—first year	.$22,760
Home dialysis–remaining years (per year) .,	13,237
In-center dialysis (per year)	24,800
Cadaver transplant–first year	23,400
Cadaver transplant—second year .	3,000
Cadaver transplant—third year	1,500
Cadaver transplant–remaining years (per year)	750
Related donor transplant—first year	20,700
Related donor transplant—second year	1,500
Related donor transplant–remaining years (per year)	500
Graft rejection (per year)	9,000

For the years 1976 through 1979, the ESRD program (23) estimated the following kidney acquisition costs (donor surgery costs):

Year	*Average*	*Low*	*High*
1976.	$4,223	$1,003	$13,197
1977	4,690	1,000	15,000
1978	5,790	1,000	12,683
1979	5,906	1,000	15,000

Average transplant charges in 1973 were $12,800, with a range of $5,500 to $20,500. Average transplant charges in 1979 were $23,000, and cadaveric kidney transplants averaged $25,000 in 1978 (30).

The above cost per year figures show that substantial savings could be achieved by shifting more patients to treatment by home dialysis or transplant (30). Roberts and his associates reported in 1980 that living, related donor transplants were the least costly treatment modality and had the greatest survival time; center dialysis (hospital-based) was the least cost effective (29). Shifts to either home dialysis or cadaveric donor transplantation would save from **$7,000** to $8,000 per life year or $284 million per year for the existing ESRD program (29).

GROWTH OF PROPRIETARY FACILITIES

More than 20 percent of the Nation's hemodialysis patients are now dialyzed in for-profit units. Proprietary facilities present difficult ethical problems for the medical community (20). Physicians who render care to a patient also share in the profitability of that function. For-profit dialysis units tend to prefer maintaining patients in an outpatient setting rather than a home setting. There are many cost disincentives to home hemodialysis. Since home dialysis means less profit, profit may influence the choice of mode of care (20). On the other hand, proprietary facilities filled a need when university hospitals and the Government could not or would not expand facilities. Such facilities are extremely cost effective, and all function within the Medicare reimbursement screen. Freestanding units can deal with Medicare billing, private insurers, and other funding sources more efficiently than hospital billing departments (3). They also have financial incentives to maintain a high patient census and to provide short, highly efficient dialysis using ultrafiltration technology in order to maximize the business efficiency of the facility. Physician reimbursement for services also gives for-profit units an incentive to encourage facility dialysis, since home dialysis generates fees at the office visit level only (3).

Function and Structure of the ESRD Program

REIMBURSEMENT FOR PHYSICIANS' SERVICES

Hemodialysis.—Under the ESRD program, there are two methods of reimbursement for physicians' services: initial method and alternative payment method. By the ***initial method,*** physicians are paid directly by the facility for their supervisory services during dialysis. For other nonroutine services required by the patient, physicians bill on a fee-for-service basis. The average payment rate to the physician for supervisory services during dialysis is part of the overall dialysis charge and averages $13 per treatment ($12 for freestanding units). By the ***alternative reimbursement method,*** physicians are paid a monthly fee for each patient for the full renal care of that patient. Such care includes supervisory services during dialysis plus all other related services furnished during a particular month. Prior to July 1, 1978, alternative monthly allowances for physician services to patients dialyzing in facilities ranged from a minimum level of $160 to a maximum level of $240. Allowances for physician services for treatment of patients dialyzing at home ranged from a minimum of $112 to a maximum of $168. These amounts are subject to Medicare Part B coinsurance and remained constant from time of their implementation in 1974 to July 1, 1978.

The monthly allowances were increased on July 1, 1978, to reflect changes in the customary and prevailing charges for internists and routine followup office visits, but were not to exceed the increase in the medical care index, which rose ***20.9*** percent from July 1975 to July 1978. The resulting revised monthly payments to the 957 physicians using the alternative method of payment now range from $180 to $260 before coinsurance for facility patients and from $126 to $182 for home patients. The price index adjustment resulted in an arithmetic mean payment before coinsurance of ***$220*** a month for facility patients and $154 a month for home patients.

Transplant.—All physicians' fees are covered as follows:

- Ž 1978-79—Excise surgeon (donor kidney): $350 for 1 kidney, $700 for two kidneys.
- 1979—Transplant surgeon: $1,600 to $2,500, depending on number of services provided.
- 1979—Transplant surgeon: $1,690 to $2,730, depending on number of services provided.

ESRD NETWORKS

ESRD networks in 32 geographic areas covering the United States were established by regulations published on June 30, 1976. Their role and function were restated in section 1881 of Public Law 95-292. In 1977, Federal funding policies were changed to encourage network organizational efforts. The year 1978 was devoted to the establishment of viable organizations and the conduct of multiple functions: inviting Medicare-approved ESRD facilities to join the network coordinating councils, developing operating rules and procedures, and hiring professional and technical staff personnel, All 32 networks had secured the services of an executive director by September 1978.

Medical review boards began in 1978 to fulfill their regulatory functions (14): 1) monitoring the effect of long-term programs by assessing appropriateness of patients for proposed treatment procedures, 2) reviewing the comparative performance of facilities and physicians for areas of patient care, 3) conducting medical care evaluation studies, and 4) performin$_g$ other studies as needed.

Network coordinating councils review and recommend approval/disapproval of applications for new or expanded dialysis facilities, a process that has led to charges of conflict of interest. HCFA regional offices consider the network recommendations together with recommendations from the health systems agency and State health planning agencies in making their facility certification decision. In many networks, 75 to 80 percent of the facility review committee may have a direct proprietary interest in the decision being made.

The network coordinating councils were requested by HCFA in November/December 1978 to develop goals relating to self-dialysis and transplantation. In mid-January, they were asked to submit interim statements of 1979 goals to HCFA. As of March 15, 1977, 12 of the 32 networks had submitted statements. According to the 1980 annual report of the ESRD program, 24 of the networks had established goals for self-dialysis training, home dialysis, or for self-dialysis programs and kidney transplantation (23). Eight networks refused or evaded the HCFA operating guidelines: Network 3 (Northern California), 6 (Arizona and New Mexico), 15 (Illinois), 23 (Washington, D.C.), 24 (Delaware), 27 (Connecticut), 28 (Maine, Vermont, New Hampshire, Massachusetts, and Rhode Island), and 32 (New Jersey).

DIALYSIS PAYMENT RATES (22,23)

In 1979, the average payment rate was $149 per treatment. This is a combined weighted average of payments to hospital-based and non-hospital-based units. Hospital facilities were paid the lesser of their costs or a national payment limit (the screen), and the average payment was $159 per treatment. Independent

facilities were paid the lesser of their charges or the national payment limit, and the average payment was $138 per treatment.

Any ESRD facility desiring a payment rate above the national limit had to request a reimbursement exception and submit documentation of its higher costs. In 1978, HCFA approved 208 ESRD facility requests for reimbursement exceptions. Some of the primary reasons for approval included:

- treatment of an unusually ill population;
- treatment of an unusual patient population (e.g., children);
- location in a high-cost or low-utilization area;
- recent approval and continuous experience of low utilization;
- demonstrated low utilization due to sporadic workload (referral hospital).

The number and range of payments approved in excess of program payment screens in 1978 and 1979 were as follows:

Range	1978	1979	Home training, 1979	Peritoneal
Up to $150	28	"27	o	0
$151-$170	63	80	8	0
$171 -$190	69	62	15	1
$191 -$210	30	35	20	4
$211 +	208	221	52	5

According to the 1980 ESRD annual report, however, HCFA actually approved 278 reimbursement exception requests (in full or in part) in 1979, while denying or returning another **23** requests (23).

GROWTH IN ESRD PROGRAM COSTS

The cost of the ESRD program grew from ***$250*** million in 1974 to over $1 billion in 1979, greatly exceeding original congressional estimates of potential costs (29). However, the number of patients receiving treatment also exceeded estimates. According to Kolata (18), when inflation is taken into account, the costs of dialysis ***per patient*** have decreased since the program began. (But total published costs underestimate true costs to the Government because a substantial number of patients collect Federal disability payments (16).)

In March 1977, the average weekly payment for the ESRD program was $6 million; in July 1978, it was $10 million a week; and by August 1978, it had reached $12 million a week (20). Between 1973 and 1977, the annual cost per patient had increased at less than half the annual rate of inflation. From 1977 to 1978, the per capita payment increased from $15,295 to $16,300, a 6.5-percent increase.

However, growing concern has been expressed over the large costs for a program benefiting a rather small number of people (29). In 1979, benefit payments for ESRD exceeded 5 percent of total Medicare expenditures, and were fully 10 percent of expenditures from the Supplemental Medical Insurance fund (Part B) of Medicare, although renal patients comprise only ***0.2*** percent of the Medicare population ***(4,29).*** In addition, fully one-third of ESRD beneficiaries were eligible for Social Security monthly disability benefits. More specifically, 10 percent of Part B funds went to ***50,000*** ESRD beneficiaries, while ***90*** percent of the funds went to ***23*** million elderly enrollees; as of 1977, the ESRD patients received $13,555 per capita, while the elderly enrollees received $218.37 per capita (28). The Government set a fee of about ***$28,000*** per year for each patient in an outpatient facility in 1979. Medicare paid 80 percent and the rest was covered by the States and private insurance carriers or was absorbed by the centers. Table E-8 shows the growth in program costs since the beginning of the ESRD program.

Quality of Life on Dialysis

Now that physicians no longer have to make painful choices regarding who gets selected for dialysis, the question has been raised whether too many people in the United States are now being dialyzed. Blagg and Scribner (4) believe that for an ever-increasing proportion of dialysis patients, the quality of life is unacceptable and increasingly costly. Many patients now being accepted into dialysis programs have such severe complicating illnesses that they may be unable to live at home. Known examples include a blind diabetic with severe angina and a nursing home patient who gets transported by ambulance twice weekly to the dialysis center (18). Quality-of-life issues also relate to the "sick room" atmosphere of hospital-based dialysis units (9). Freeman (11) recommends removing dialysis units from hospitals to better designed, less expensive, more cheerful, freestanding units, with the hope that the "mass production line" approach to dialysis can be avoided.

There are now ***55,000*** patients on hemodialysis. An informal survey of 21 dialysis centers found that 44

Table E-8.—Growth in ESRD Costs, 1974-80

Year	Per capita costs	Program costs (millions)[a]
1974		$250 (283)
1975	$11,000	330 (450)
1976	12,300	450 (598)
1977	16,800	600 (722)
1978	17,300	737 (947) (1.1 billion)
1979	23,500	850.5 (1.2 billion)
1980	28,000	NA

[a]Numbers in parentheses reflect discrepancies in the literature.
NA = indicates not available.

SOURCE: R. Rettig, *Implementing the End-Stage Renal Disease Program of Medicare, 1980.*

percent of the dialysis patients were not working and that more than 50 percent of this population were probably too sick to work. Twenty percent of the non-diabetic patients were unable to care for themselves completely, and 50 percent of the diabetic patients were unable to care for themselves completely. Skewed incentives may have encouraged insufficient discrimination in the selection of candidates for dialysis (15).

In Britain, many physicians decide not to refer certain types of patients for hemodialysis because of their belief that it is inappropriate treatment for the situation. "In the absence of personal financial incentives to treat more patients with dialysis, the NHS [National Health Service] doctor is more free to decide that these extraordinary procedures for prolonging life do not confer a good enough quality of life to make them suitable for all patients dying of renal failure. Not to treat may be kinder and wiser." (1).

Generalizations About the ESRD Program

According to Rettig (28), most of the problems of the ESRD program have arisen from its administrative system, the planning and operational stages of its implementation, and the substance of reimbursement and medical issues. The most important reimbursement policy has been the screen, or de facto ceiling, on the per treatment reimbursement of outpatient maintenance dialysis. This screen has provided a strong incentive to cost containment. On the other hand, the financial disincentives to home dialysis have been one of the last defensible aspects of reimbursement **(28).** Rennie (26) has stated that all the problems with the ESRD program can be attributed to inappropriate economic incentives and inappropriate economic deterrents in the present law and regulation.

Blagg and Scribner (3) have identified several problems with respect to the ESRD program's implementation: lack of effective leadership, no continuity in administrative policy, slow and haphazard implementation; increased vulnerability to political lobbying, piecemeal regulations, and lack of meaningful data. The absence of data has made it impossible to assess and compare the quality of care and patient outcomes. It has been impossible to pull out of HCFA's computers data such as percentages of patients dialyzed at home and at centers and such patients' relative mortality rates, ages, and illness levels.

Three important innovations have been introduced by the ESRD ***program (28).*** One is the screen on facility reimbursement, which creates strong incentives for delivering outpatient dialysis. The second is the process of reimbursing physicians indirectly by a monthly cavitation method, which departs from the traditional fee-for-service reimbursement. The third innovation is the facility certification process, which permits the number and capacity of treatment facilities to increase in reasonable relation to the growth in the patient population.

Jones' References

1. Anonymous, "Selection of Patients for Dialysis and Transplantation," ***Br. Med. J.*** 6150:1449, 1978.
2. Bergan, J. H., "History of Renal Transplant Registry," in NIAID, *U.S. Kidney Transplant Fact Book* (Washington, D. C.: Department of Health, Education, and Welfare, 1972).
3. Blagg, C. R., and Scribner, B., "Long-Term Dialysis: Current Problems and Future Prospects," *Amer. J. **Med.*** 68:633, *1980.*
4. Blagg, C. R., and Scribner, B., "Medicare End-stage Renal Disease Program: More Than A Billion Dollar Question, " ***Ann. Inter. Med.*** 93:501, 1980.
5. Cestero, R., "A Regional End-Stage Renal Disease Program: Twelve Years' Experience," ***Ann.*** *Inter.Med.* 93:494, ***1980.***
6. Comptroller General of the United States, ***Request To Update Information in the 1975 GAO Report*** (Washington, D. C.: General Accounting Office, 1977).
7. Comptroller General of the United States, ***Treatment of Chronic Kidney Failure: Dialysis, Transplant Costs, and the*** *Need for More Vigorous* ***Efforts*** (Washington, D. C.: General Accounting Office, 1975).
8. Czaczkes, J. W., and De-Nour, A. K., ***Chronic*** *Hemodialysis as a Way of Life (****New*** York: Brunner/Mazel, 1978).
9. Drukker, W., et al. (eds.), ***Replacement of*** *Renal* ***Function by Dialysis*** (The Hague: Mortinus Nijhaff Medical Division, 1978).
10. Freedman, E. A., et al. "Pragmatic Realities in Uremia Therapy," N. *Eng. J. **Med.*** 298:368, ***1978.***
11. Freeman, R., 'The Social Impact of Chronic Maintenance Hemodialysis," in ***Replacement of Renal Function by Dialysis,*** W. Drukker, et al. (eds.) (The Hague: Mortinus Nijhaff Medical Division, 1978).
12. Gottschalk, C. W., ***Report of*** *the* ***Committee on Chronic Kidney Disease*** (Washington, D. C.: Bureau of the Budget, 1967).
13. Greenberg, D., "Renal Politics," N. *Eng. J. **Med.*** 298:1427, 1978.
14. Gutch, C. K., and Stoner, M., ***Review of*** *Hemodialysis* ***for Nurses and Technicians (****New*** York: C. V. Mosby, 1979).
15. Gutman, R., et al. "Physical Activity and Employment Status of Patients on Maintenance Dialysis," N. *Eng. J. **Med.*** 304:309, ***1981.***
16. Hampers, C., and Hager, E., "The Delivery of Dialysis Services on a Nationwide Basis," ***Dialysis& Transplant.*** 8:417, 1979.
17. Health Services Administration, *End-Stage Renal* ***Disease Medical*** *Information* ***System,*** *Facility* ***Report Number*** *1* (Washington, D. C.: Department of Health, Education, and Welfare, 1976).
18. Kolata, G. B., "Dialysis After Nearly a Decade," ***Science*** 208:473, ***1980.***

19 Kolata, G. B., "NMC Thrives Selling Dialysis, " *Science* 208:379. *1980.*
20 Moore, F. D., "End-Stage Renal Disease and the Government," *Dialysis & Transplant.* 7:258, 1978.
21 Moore, F. D., *Transplant: The Give and Take of Tissue Transplantation (New* York: Simon & Schuster, 1972).
22. Office of End-Stage Renal Disease, Health Care Financing Administration, *End-Stage Renal Disease Program Annual Report to Congress, 1979* (Washington, D. C.: Department of Health, Education, and Welfare, 1979).
23. Office of End-Stage Renal Disease, Health Care Financing Administration, *End-Stage Renal Disease Program Second Annual Report to Congress, 1980* (Washington, D. C.: Department of Health and Human Services, 1980).
24. Relman, A., "The New Medical-Industrial Complex," N. *Eng. J, Med.* 303:963, 1980.
25. Rennie, D., "Home Dialysis and the Costs of Uremia, " N. *Eng. J. Med.* 298:393, 1978.
26. Rennie, D., "Renal Rehabilitation—Where Are the Data?" N. *Eng. J. Med.* 304:351, 1981.
27. Rettig, R., "End-Stage Renal Disease and the "Cost" of Medical Technology," in *Medical Technology: The Culprit Behind Health Care Costs? S.* Altman and R. Blendon (eds.) (Washington, D. C.: Department of Health, Education, and Welfare, 1977).
28. Rettig, R., *Implementing the End-Stage Renal Disease Program of Medicare* (Santa Monica, Calif.: Rand Corp., 1980).
29. Roberts, S., et al., "Cost-Effective Care of End-Stage Renal Disease," N. *Eng. J. Med.* 92:243, *1980.*
30. Stange, P., and Summer, A., "Predicting Costs and Life Expectancy for End-Stage Renal Disease, " N. *Eng. J. Med.* 298:372, *1978.*
31. Steinman, T. I., letter to the editor, N. *Eng. J. Med.* 298:1086, 1978.