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IV. CALIDAD DE VIDA RELACIONADA CON LA SALUD EN PACIENTES EN TRATAMIENTO RENAL SUSTITUTIVO

The CHOICE Study: A National Study of dialysis outcomes in the United States

N. R. Powe, M.D., M.P.H., M.B.A.[1,2] and N. E. Fink, M.P.H.[2]

[1]Johns Hopkings University School of Medicine, Baltimore, M.D. [2]Johns Hopkins University School of Hygiene and Public Health, Baltimore, M.D.

INTRODUCTION

Choices for Healthy Outcomes in Caring for ESRD (CHOICE) is a comprehensive study of the relationship between patient and provider choices, patient outcomes and health care costs for patients with ESRD who are undergoing dialysis. CHOICE is one of several Patient Outcomes Research Team (PORT) studies funded by the United States Agency for Health Care Policy and Research and the first one to focus on end-stage renal disease. PORTs comprise a multi-disciplinary group of investigators exploring important and sometimes controversial issues in medical practice. CHOICE investigators come from clinical medicine (in this case nephrology and internal medicine) and the fields of epidemiology, health services research, economics, statistics and survey research measurement (quality of life measurement, preference assessment and patient satisfaction measurement). [See Appendix for list of investigators and their affiliations]. The term «effectiveness research» rather than «outcomes research» may better characterize this type of research, since the aim is to understand what medical interventions «work» and «do not work» and the «circunstances under which they work»[1]. Effectiveness research is different from «efficacy research» in which medical practices are investigated under controlled conditions such as clinical trials in highly selected samples of physicians or medical centers. Under such conditions, variables which can influence treatment outcomes may only vary a little. For example, early clinical trials of new drugs or devices designed as efficacy studies often have these features. In documenting the effectiveness and cost-effectiveness of different treatment options, PORTs also carefully incorporate methods for assessing patient preference in decision making and provide information for formulation of clinical practice guidelines.

This article will review the impetus for and describe the projects that make up the CHOICE study.

BACKGROUND

In both the United States and the world, the approach to management of ESRD varies with regard to the percentage of patients undergoing peritoneal dialysis and the average prescribed and delivered dose of hemodialysis[2,3]. While the reasons for variation in clinical management are not clearly understood, it reflects choices made by providers and patients. Providers and patients often attempt to balance tradeoffs between one patient outcome and another and between patient outcomes and costs. Many of these clinical and economic outcomes are only beginning to be defined. For example, patients may prefer a shorter treatment session on dialysis, that is inherently less expensive for providers to deliver even though they may incur an early death or uremic complications. Patients may prefer peritoneal dialysis because of the freedom it affords and less frequent visits to a dialysis unit for treatment. Hemodialysis may be preferred because patients may be terrified by the prospect of peritonitis or because they prefer health professionals administering dialysis rather than themselves or their family. Dialysis providers may make choices that maximize operating efficiency and reflect training experiences of their physician, but that may lead to inefficiencies across the total spectrum of outpatient and inpatient care for ESRD patients.

In the United States, the cost of health care for dialysis patients is U.S. $44,187, eight to ten times the cost of the average elderly person who receives public insurance in the Medicare program[3]. In contrast, the annual mortality of nearly 20 percent compares poorly

Correspondencia: Neil R. Powe, M.D.
Johns Hopkins University School of Medicine
2024 E. Monument Street, Suite 2-600
Baltimore, MD 21205-2223

Appendix

CHOICE Investigators include:
- Neil R. Powe, M.D. M.P.H., M.B.A. (Principal Investigator), Johns Hopkins University School of Medicine and Johns Hopkins School of Public Health, Baltimore, MD.
- Gerard F. Anderson, Ph.D., Johns Hopkins School of Public Health, Baltimore, MD.
- Eric B. Bass, M.D., M.P.H., Johns Hopkins University School of Medicine, Baltimore, MD.
- William A. Briggs, M.D., Johns Hopkins University School of Medicine, Baltimore, MD.
- Ronald S. Brookmeyer Ph.D., Johns Hopkins School of Public Health, Baltimore, MD.
- Josef Coresh, M.D. Ph.D., Johns Hopkins School of Public Health, Baltimore, MD.
- Nancy Fink, M.P.H. (Project Director), Johns Hopkins School of Public Health, Baltimore, MD.
- Frederic Finkelstein M.D., New Haven, CAPD, New Haven, CT.
- Michael J. Klag, M.D., M.P.H. (Co-Principal Investigator), Johns Hopkins University School of Medicine, Baltimore, MD.
- Alan Kliger, M.D., New Haven, CAPD. New Haven, CT.
- Andrew S. Levey, M.D., New England Medical Center, Boston, MA.
- Nathan Levin, M.D., Renal Research Institute, New York, NY.
- Klemens B. Meyer, M.D., New England Medical Center, Boston, MA.
- Haya R. Rubin, M.D., Ph.D., Johns Hopkins University School of Medicine. Baltimore, MD.
- John H. Sadler, M.D., (Co-Principal Investigator) Independent Dialysis Foundation, Baltimore, MD.
- Albert W. Wu, M.D., Johns Hopkins School of Public Health, Baltimore, MD.

Clinical Liaison Committee Members:
- Thomas Depner, M.D. DCI Sacramento, CA.
- H. Keith Johnson, M.D. DCI Corporate Headquarters.
- K. Shashi Kant, M.D. DCI Cincinnati, OH.
- Klemens Meyer, M.D. DCI Boston, MA
- Edward Schroeder, M.D. DCI Syracuse, NY
- Richard Sherman, M.D. DCI North Brunswick.
- Pradip Teradesai, M.D. DCI Aliquippa, PA.
- John Van Stone, M.D. DCI Columbia, MO
- Alan Wasserstein, M.D. DCI Philadelphia, PA (HUP)
- Lucius Wright, M.D. DCI Jackson, TN
- Jackson Yium, M.D. DCI Chattanooga, TN.
- Philip Zager, M.D. (Chair). DCI Alburquerque, NM.

to many other diseases. The variation in dialysis practices which also results in variation in the cost of care, may reflect either appropriate practice that takes into account the individual needs or patients or care that is not optimal. Strong evidence about practices might help providers improve the outcomes of their patients as well as reduce the cost of care. Patient outcomes research in renal disease attempts to provide information for optimal decision making by physicians, patients and health policymakers.

CHOICE PROJECTS

CHOICE involves five complimentary research projects. These five projects include: 1) a prospective cohort study, 2) a study of patient preferences for dialysis, 3) a study of how cost-quality tradeoffs are made by nephrologists and dialysis facility administrators, 4) a study of the relation between resource use and outcomes in dialysis care using data from the U.S. ESRD registry 5) a decision and cost-effectiveness analysis of dialysis delivered by different modalities and using different doses. A few of the projects have included development or application of new techniques for assessing patient outcomes. These projects and techniques are described bellow.

Prospective Cohort Study of Clinical Outcomes

The CHOICE cohort study was initiated in october 1995 to examine the impact of dialysis modality (hemodialysis vs. peritoneal dialysis) and dose on a broad array of outcome measures. The outcomes to be assessed include mortality, morbidity, symptoms, quality of life, patient satisfaction and costs of care. The study is a concurrent prospective study of incident cases of end-stage disease. Figure 1 shows the design of the study and figure 2 shows the design

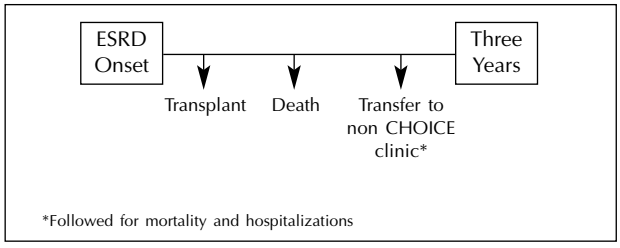

Fig. 1.—Design of the CHOICE Cohort Study.

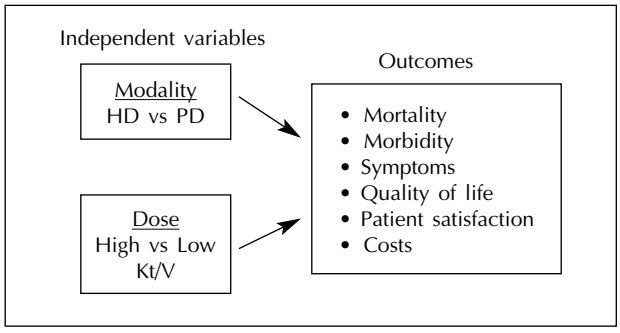

Fig. 2.—Analysis of the CHOICE Cohort Study.

of the planned analyses. Patient inclusion criteria include new onset of chronic renal replacement therapy in the three months before enrollment, informed consent, age 18 years or older and English or Spanish speaking. Home hemodialysis and hospice patients are excluded.

The clinical sites for data collection are located in 19 states across the U.S. and are afilliated with Dialysis Clinic, Inc. (DCI) (79 clinics) and Beth Israel Medical Systems (2 clinics). Figure 3 shows the location of the clinics in the U.S. The population of dialysis patients in these clinics is broadly representative of both community and academic medical center clinics in the U.S.

In order to standardize data collection throughout the 81 participating clinical sites, several quality assurance measures were developed. A manual of forms and procedures was developed and training sessions for clinical coordinators were held at the initiation of the study. Day long site visits were made to the clinics over the course of recruitment to encourage enrollment of patients and assure quality data collection. A data collection schedule was developed for longitudinal assessment. Figure 4 details

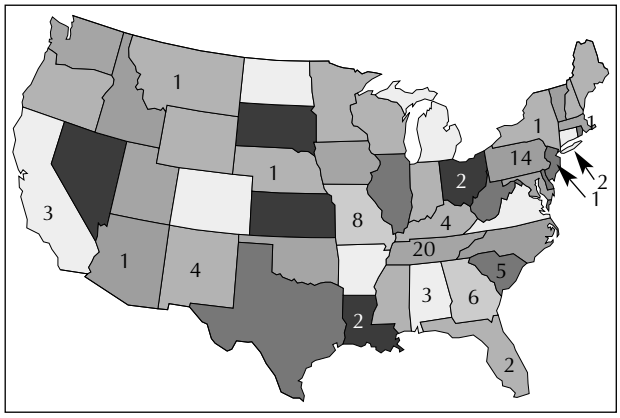

Fig. 3.—Location of clinical sites in the CHOICE Cohort Study.

Assessment	Base line	3 months	6 months	9 months	12 months
Kinetic modeling	X	X	X	X	X
Residual renal function	X	X	X	X	X
Comorbidity	X				X
Quality of Life	X	X	X		X
Preferences	X				X
Satisfaction	X				X

Fig. 4.—Longitudinal assessments Made in the CHOICE Cohort Study.

the data collection schedule for the first year of study and the data elements collected. Form packets are computer-generated and sent to each clinic. Data are tracked from completion of data forms to entry into the CHOICE database. Measurements of dose of dialysis are standardized.

Recruitment began in october 1995 and ended in june 1998. A total of 1,067 patients (73% HD and 27% peritoneal dialysis) were enrolled. Table I details the baseline characteristics of the 1,067 participants. Preliminary unadjusted analysis of demographic data shows the hemodialysis patients different from peritoneal dialysis patients with respect to race, age, marital status, education, employment, smoking and alcohol con-

Table I. Baseline Characteristics of Patients in Choice Cohort Study

	Hemodialysis n = 783	Peritoneal n = 284
Modality, %	73	27
Mean age, years	59	54
Gender, % male	54	55
Race, % white	65	78
Education (% hs grad)	66	81
Employed, %	8	28
Married, %	56	66
Travel to facility > 30 miles, %	8	28
Current smoker, %	28	19
Current alcohol use, %	12	25
Patient-reported Co-morbidity		
Diabetes mellitus, %	52	51
Myocardial infarction, %	19	15
Coronary artery by-pass graft surgery, %	18	19
Congestive heart failure, %	30	25
Stroke, %	14	9
Chronic obstructive pulmonary disease, %	7	2
Cancer, %	10	9
Producing 250 cc urine/day, %	81	88
Amount of time before first dialysis nephrologist was first seen, mos.	8	17

sumption. Follow-up of the cohort is continuing until june 1999, at which time the mean follow-up per patient will be approximately 2 years. This cohort represents one of the largest cohorts of incident dialysis patients ever actively followed in the United States.

Since CHOICE is an observational study, it is essential to assess patient factors that influence outcomes. Comorbidity and disease severity is one such factor. In addition to asking patients about comorbid diseases, comorbidity and severity is assessed in CHOICE using the Index of Coexistent Disease (ICED). The ICED is a comorbidity classification system that measures the severity of each of 19 diseases/conditions (ischemic heart disease, congestive heart failure, arrhythmia, other heart disease, hypertension, cerebrovascular disease, peripheral vascular disease, diabetes mellitus, musculoskeletal disease, nervous system disease, respiratory disease, gastrointestinal disease, hepatobiliary disease, urinary tract disorders, malignancy, HIV/AIDS, ophthalmologic disease, hematologic disease, and anticoagulation) and takes into account the impact of the disease on the patient's physical function in 11 body areas (ambulation, circulation, fecal, feeding, hearing mental status, neurologic, respiratory, speech, urinary and vision). The ICED was developed to be used on data extracted from medical records and has been validated in ESRD populations[4]. ICED scores range from 0 (no disease) to 3 (severe disease). In CHOICE, an experienced dialysis nurse reviews the patient charts and assigns the ICED score at baseline and annually thereafter. Few patients in the CHOICE cohort at baseline are scored at 0. Approximately, one third of the patients are in each of the other three ICED severity categories.

Quality of life is being assessed using the CHOICE Health Experience Questionnaire (CHEQ), a patient-centered, disease and treatment specific measure. The questionnaire includes the generic Medical Outcomes Study Short Form-36 (SF-36) and disease-specific questions focused on aspects of quality of life potentially related to modality and dose of dialysis (fig. 4). The CHEQ also contains questions about patient preferences and satisfaction with care[5]. This instrument went through a rigorous development process which included a structured literature review, focus groups of patients and providers and pilot testing. The instrument was also translated into Spanish.

The original design of the cohort study was enhanced by the addition of a specimen bank. Serum, plasma, DNA, red blood cells and urine from 910 CHOICE participants are being banked at baseline and over the course of follow-up. An average of about 1,500 vials are stored each month. Several studies related to biologic risk factors of cardiovascular disease and vascular access complications are underway using the specimen bank.

Table II. Domains Assessed in Quality of Life Instruments used in CHOICE Study

Generic measure Short Form-36	Disease-specific measure: CHOICE Health Experience Questionnaire (CHEQ)
• Physical function	• Time
• Role-physical	• Freedom
• Bodily pain	• Cognitive functioning
• General health	• Financial
• Vitality	• Restrictions on diet
• Social function	• Recreation
• Role-emotional	• Work
• Mental health	• Body image
	• Sex
	• Sleep
	• Access/catheter problems
	• Symptoms
	• General Quality of Life

Patient Preferences for Treatment Options

We are assessing patient preferences for dialysis treatment options. We have developed an instrument to explicity assess patient preferences for modality, dose and other attributes of dialysis. One hundred eighty eight dialysis patients in Maryland and Massachusetts have been interviewed using the instrument at baseline-109 hemodialysis patients and 79 peritoneal dialysis patients. Patients who were alive and on dialysis at one year after the baseline interview received a follow-up interview. Data on sociodemographic, clinical and treatment characteristics has been collected. The study uses a time-tradeoff methodology to assess how patients rate their current health on their current dialysis treatment. Patients are then asked to rate hypothetical treatments with regard to modality and dose that they might receive in the future. In these hypothetical examples, patients' rating of tradeoffs between a higher dose of dialysis and survival are explored. Consistency has been examined between the time-tradeoff method and more traditional standard gamble methods. The reproducibility of preferences measured with the time-tradeoff method has also been assessed. This project will improve our understanding of how patients feel about features of different dialysis treatment regimens and how they value various outcomes of different regimens. We hope to develop better clinical tools for providing information on initial and subsequent treatment options that are available to patients.

Decision Regarding Cost-Quality Tradeoffs in Dialysis Care

We have performed a national survey of a random sample of U.S. physicians (n = 271) in order to better understand the attitudes, beliefs, training experience and environmental barriers that shape current treatment practices.

We have also examined the factors which may prevent delivery of adequate dialysis. Because facility administrators are involved in decision making, we have also performed a national survey of a random sample of dialysis facility administrators (n = 157). We have asked administrators about how they handle cost-quality tradeoffs in everyday treatment decisions. The analysis of these surveys will permit providers of dialysis care to design strategies for changing practice in ways that lead to the improvement of patient outcomes.

Relation Between Dialysis Care Resource Use and Patient Outcomes

A declining reimbursement rate in real terms has led to concerns about the quality of dialysis care in the U.S. The cost-quality relationship in dialysis care is not clear. We are examining the relation between the resource inputs for dialysis care and outputs of health. Resource inputs include labor, capital and supplies. This project is using nationwide data from the U.S. ESRD registry (Medicare) on nearly 40,000 patients with new onset ESRD. We will determine whether different levels of resource consumption in the delivery of dialysis care are associated with differences in survival, hospitalization and the cost of care. These analyses will be useful to public insurance plans that must decide on how much to pay for dialysis care.

Relative Cost-effectiveness of Different Dialysis Prescriptions

Health policymarkers are seeking information on both cost and outcomes of care. We are performing cost-effectiveness analysis of different dialysis prescriptions using decision analyses with Markov modeling. These analyses are being done from the perspectives of patients and from a societal perspective. Different dialysis management strategies are being compared with regard to both outcomes and cost. The decision models include data on disease incidence and prevalence, effectiveness of therapy, patient preferences for health outcomes, health status transitions made by patients and resource utilization. Much of this data is derived from the four other CHOICE projects described above.

SUMMATION

CHOICE is a study of the effectiveness (versus efficacy) of different dialysis treatments (modality and dose) in the United States. The study is being conducted a variety of routine clinical practice settings which include academic and non-academic practices. There are few exclusion criteria. A broad array of clinical and economic outcomes variables are being measured longitudinally including patient-centered data on health-related quality of life, patient preferences and patient satisfaction. CHOICE is an observational study, so a great deal of attention is being paid to characterization and adjustment for confounders, particularly case-mix and disease severity. The goal of the study is to provide complimentary evidence to experimental and other ongoing studies on the impact of dialysis modality and dose on health outcomes[6,7]. We seek to provide data that can be used to guide and improve dialysis management while taking into account effectiveness of different prescriptions, patient and provider preferences and cost-effectiveness.

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