The Benefits of Comprehensive Care of Hemophilia: A Five-Year Study of Outcomes

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Abstract: Eleven of 22 federally funded Comprehensive Hemophilia Centers have collected data on outcomes, before and after five years of this program's existence. Improved health, decreased hospitalization, decreased absenteeism, and a decrease in the unemployment rate from 36 per cent to 13 per cent were accompanied by decreased costs of care. In this model of a chronic handicapping illness, the early application of comprehensive care is preferable to the previous emphasis on end-stage rehabilitative efforts. (*Am J Public Health* 1984; 74:616–617.)

Introduction

About one in 5,000 males has hemophilia, congenitally lacking factor VIII (Hemophilia A or Classical Hemophilia) or factor IX (Hemophilia B or Christmas Disease). The inadequately treated hemophiliac can expect a shortened and anxiety-ridden life of pain, confinement, progressive crippling, and restricted opportunities.[1,2]

In 1975, Section 1131 of the Public Health Service Act established and funded a network of Hemophilia Diagnostic and Treatment Centers throughout the United States.[3] There are now 22 such regional centers providing comprehensive services to the hemophiliacs in their catchment areas. The present report is an analysis of health care outcomes and costs from 11 centers which voluntarily agreed to compare data on five full years of activity with available information from the year before inception of the program.

Description of the Centers

The minimum services provided by each center were:

- A coagulation laboratory of recognized high standards;
- A blood bank providing all of the blood components needed by hemophiliacs;
- A multidisciplinary hemophilia care team including a hematologist, an internist, a pediatrician, an orthopedic surgeon, a physical therapist, a dentist, a social worker, and a registered nurse;
- Formal linkages with mental health, genetic counseling, and rehabilitative services;
- A training course in self-therapy (home care) and updated hemophilia concepts for patients and family members;
- An outreach program to enable every hemophiliac within the area served to receive services of the program.

Every effort was made to use all existing resources for funding hemophilia treatment and, in many cases, centers worked closely with third party payers, such as Blue Cross/Blue Shield, to develop private funding for services.

Within the confines of 22 defined geographical areas in the United States, liaisons were established with any already existing state hemophilia programs. In sectors lacking any such programs, satellite or affiliate centers were set up or organized by staff of the primary federally funded centers. Currently we estimate about one-half of the hemophiliacs in the United States have access to such programs.[1]

Methods

Although no funding was available for data collection, one-half of the centers agreed to participate, representing a good cross section of the whole network in terms of geographic, socioeconomic, and age distributions. A standardized data collection form was developed.* Data were collected and tabulated at each center by the hemophilia nurse coordinator, the social worker, or both, and reviewed by the director. Overall costs of health care were obtained from the records each center kept of its blood product consumption for the year, and account for almost all of the cost of treatment.[4] The average costs of care per patient per year was calculated as previously described.[4] In addition, each patient was asked to estimate his out-of-pocket treatment expenses for the year.

Results

During the year prior to federal funding, the 11 participating centers and their affiliates saw 2,112 patients. Five years later (fiscal 1981), more than twice as many patients (4,742) were receiving comprehensive care through the centers. Eighty-six per cent of the patients served by the centers in 1981 had factor VIII deficiency, while 14 per cent had factor IX deficiency; 67 per cent had the severe form of the disease and 33 per cent were moderate or mild hemophiliacs, using the standard criteria described elsewhere.[4]

While initially only 514 patients were knowledgeable and skilled enough to treat themselves with appropriate doses of intravenous blood product, 2,001 had achieved this degree of proficiency by fiscal 1981. This "home care" spared them countless hours in transit and in waiting rooms, hence the morbidity from delayed treatment. Thirty-six per cent of the surveyed population were unemployed at the outset as compared to 12.8 per cent four years later.** (The oldest regional program, New England States, had less than 7 per cent unemployment, a figure approximately equal to that of healthy persons in 1981.) The number of days lost

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** Based on status at intake interview or annual visit during specified year divided by number of patients seen in that year.

from work or school decreased from 14.5 per year (9.4 of which were spent in the hospital) prior to funding to 4.3, with hospital treatment needed in only 1.8. The average patient, who could expect two hospitalizations per year before this program, required admission only once every three to four years, five years later.

Patient advocacy, coordinated efforts between hemophilia centers and various agencies, and the backing of the federal government resulted in third party health care coverage of 93 per cent of the patients (usually considerably more comprehensive), as compared to 74 per cent before funding.

A summary of outcomes and the average cost of services for each patient is shown in Table 1.

Discussion

The outcome parameters listed translate cogently into a quality of care which is both equitable for the individual and fiscally sound for society. They demonstrate that the hemophiliac, optimally looked after, is as capable of educating and supporting himself as any other member of society.

Because participation in the study was voluntary, it is possible that these data are biased in favor of comprehensive care. We believe this unlikely in view of the fact that all the funded centers had to compete for the federal grants and thus represented areas where the quality of services was above average even at the outset. The findings reported here are in agreement with previous reports, which have shown that an actual cost savings occurred[5–7] despite the increased use and expense of concentrate in one center. As to quality of health, the effect of state-of-the-art care on the knee—the most commonly affected joint in hemophilia—has recently been reported suggesting that with proper early management of hemarthroses, expensive surgical interventions to restore or replace joints may decrease in the future.[8] Since comprehensive centers are currently surgically rehabilitating a backlog of already severely damaged joints, we may expect overall medical costs to further decrease in the future.

Hemophilia may well serve as a model for other lifelong crippling diseases, in which the shifting of federal funds from end-stage rehabilitation programs toward comprehensive early intervention with an emphasis on home care will also be worthy of trial.

TABLE 1—Outcome Data in 11 of 22 Federally Funded Comprehensive Hemophilia Diagnostic and Treatment Centers

Outcome Data	Year before Program	Fifth Year of Program
No. patients seen at primary centers	1783	3705
No. patients seen at affiliate centers	329	1037
No. patients receiving regular comprehensive care	1333	4682
No. patients on self-infusion ("home care")	514	2001
Average days/year lost from work or school	14.5	4.3
Average hospital admission/year	1.9	0.26
Average days/year spent as inpatient	9.4	1.8
Per cent patients with third party coverage	74	93
Out-of-pocket expense/patient/year	$ 850.*	$ 342.
Overall costs of care/patient/year	$15,800.*	$5,932.
Per cent unemployed adults**	36	12.8

*These figures represent retrospective estimates from small samples, in the case of most of the centers.

**See Results section of text.

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2. **Gulf States Centers:** W. Keith Hoots, MD, *Houston, TX*.
3. **Michigan-Indiana Center:** Jeanne M. Lusher, MD, *Detroit, MI*; John A. Penner, MD, *Lansing, MI*; Ralph A. Gruppo, MD, *Cincinnati, OH*; Douglas A. Triplett, MD, *Muncie, IN*; Robert M. Weetman, MD, *Indianapolis, IN*; Leo Zelkowitz, MD, *Kalamazoo, MI*.
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5. **New England Comprehensive Center:** Peter H. Levine, MD, *Worcester, MA*; Louis Bove, MD, *Portland, ME*; Stanley Burns, MD, *Burlington, VT*; Cornelius Cornell, MD, *Hanover, NH*; Peter S. Smith, MD, *Providence, RI*; Frederick Rickles, MD, *Farmington, CT*.
6. **New York Centers:** Margaret W. Hilgartner, MD, Louis M. Aledort, MD, and Richard Lipton, MD, *New York City*; Mary M. Gooley, NHF, *Rochester, NY*.
7. **North Carolina Centers** (Two centers): Philip M. Blatt, MD, *Chapel Hill, NC*; Christine A. Johnson, MD, *Winston-Salem, NC*.
8. **Pennsylvania:** M. Elaine Eyster, MD, *Hershey, PA*.
9. **Tennessee:** Marion Dugdale, MD, *Memphis, TN*.

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