The Cost of Medical Care for Patients With Cystic Fibrosis in a Health Maintenance Organization

Tracy A. Lieu, MD, MPH*; G. Thomas Ray, MBA*; Gail Farmer, MS, RD‡; and Gregory F. Shay, MD‡

ABSTRACT. Background. Cystic fibrosis (CF) is the most common life-shortening genetic disorder among white individuals worldwide. Previous estimates of the costs of medical care have been based on expert opinion rather than observed costs. Accurate cost estimates are needed to enable evaluation of the cost-effectiveness of new interventions and prenatal genetic screening recommendations.

Objective. To evaluate the cost of medical care for patients (N = 136) served by a health maintenance organization with a CF center.

Methods. Retrospective analysis of data from computerized cost databases and the Cystic Fibrosis Foundation annual survey. Severity of disease was classified based on the percent predicted forced expiratory volume at 1 second.

Results. The annual cost of medical care in 1996 averaged $13 300 and ranged from $6200 among patients with mild disease to $43 300 among patients with severe disease. Of total costs, 47% were from hospitalization, 18% were from DNase (Pulmozyme), 12% were from clinic visits, and 10% were from outpatient antibiotics. When the observed costs were used to estimate the costs of medical care for the entire population of CF patients in the United States, these costs were estimated to be $314 million per year in 1996 dollars.

Conclusions. We conclude that the cost of medical care for CF varies greatly with severity but is substantial even among patients with mild disease. These findings underscore the need for strategies to ensure good health insurance coverage and high quality care for all individuals with this condition. Pediatrics 1999;103(6). URL: http://www.pediatrics.org/cgi/content/full/103/6/e72; cystic fibrosis, chronic conditions, cost, health services, managed care.

ABBREVIATION. CF, cystic fibrosis.

Cystic fibrosis (CF) is the most common life-shortening genetic disorder among white individuals worldwide.1 In the past decade, medical approaches to CF have undergone rapid change attributable to the introduction of inhaled antibiotics and inhaled enzyme therapy.2 The identification of the CF gene in 1989 has led to work on potential new therapies including gene therapy that are directed at the basic defect.

Data on the current costs of CF are important to evaluate the cost-effectiveness of new treatments. In addition, a consensus panel of the National Institutes of Health recently recommended that screening for CF mutations be offered routinely prenatally. The cost-effectiveness of prenatal screening depends greatly on the anticipated cost of care for CF patients that might be averted by screening.3

The Cystic Fibrosis Foundation estimated that the 1996 average annual costs of CF care were $45 000 (S.C. Fitzsimmons, unpublished data). However, this estimate was based largely on updated figures from a 1991 report that derived costs by asking experts to estimate the hospital and outpatient utilization of CF patients.4 The increasing reliance on managed care since that time has been associated with significant changes in the way in which care is actually delivered, including the increased use of intravenous antibiotics in the home setting.

This study’s aim was to describe the current costs of medical care for CF at a health maintenance organization with a recognized CF center and a representative patient group.

Methods

Study Population

The Kaiser Permanente Medical Care Plan is a nonprofit, group-model health maintenance organization that provides care to ~2.8 million patients in the Northern California region. Kaiser Permanente’s regional CF center identified all CF patients for the current analysis, which included those who had continuous health plan membership via commercial insurance during 1996. CF patients with Medicaid were excluded because their costs may not have been captured consistently by health plan databases.

Data Collection

Information on demographics, severity of illness, and other clinical information was collected from medical records by Kaiser Permanente’s CF center for the CF Foundation Annual Registry Database. Survey information was data-entered and shared with us by the CF Foundation. As suggested by the CF Foundation, severity of illness was defined based on the percent expected FEV₁ as mild (FEV₁ ≤70%), moderate (FEV₁ ≤40%–69%), or severe (FEV₁ <40%).

All utilization and costs for 1996 were identified using the health plan’s regional Cost Management Information System. This system captures all fixed, (eg, administrative overhead) and variable costs associated with hospital, laboratory, radiology, outpatient visit, and pharmacy databases. Fully allocated costs are assigned to units of service using step-down accounting methods. Costs for most events within the Kaiser Permanente system, such as hospitalization or clinic visits, are believed to be valid and reliable. When patients were hospitalized outside the health plan, the charges from these hospitalizations were counted as costs.
Home health nursing visits for intravenous antibiotic administration were not covered by the health plan, because parents or patients could either administer the medication themselves or visit the outpatient clinic for such services.

RESULTS

Demographics

There were 182 patients initially identified by the CF center. Of these patients, 42 were excluded because they did not have continuous Kaiser Permanente membership in 1996, and 4 were excluded because they were insured at least in part by Medicaid, leaving 136 in the eligible population. Of the eligible patients, 52% were male, and 93% were white. Their ages ranged from 9 months to 56 years (mean: 16.6, median: 13). Among the 44 patients who were 18 years, all had graduated from high school or had attended college, and 50% were employed.

Clinical Characteristics and Utilization

Of the eligible population, 41% had mild disease, 31% had moderate disease, and 15% had severe disease. Data on the severity of illness based on FEV\textsubscript{1} were not available for 12% of the eligible population. As Table 1 shows, during the year, patients with mild disease experienced a mean of .82 hospital days (median 0), and in contrast, those with severe disease experienced a mean of 18 hospital days (median 11). Of all the patients, 89% used pancreatic enzymes, 18% used home intravenous antibiotics at least once during the year, and 49% used DNase (Pulmozyme). One patient had a heart-lung transplant that was performed at a university hospital outside of the health plan.

TABLE 1. Medical Utilization and Costs of Patients With Cystic Fibrosis, Northern California Kaiser Permanente, 1996

<table>
<thead>
<tr>
<th>Subgroup by Disease Severity</th>
<th>Mild N = 56</th>
<th>Moderate N = 43</th>
<th>Severe N = 21</th>
<th>Unknown N = 16</th>
<th>All Patients N = 136</th>
</tr>
</thead>
<tbody>
<tr>
<td>Utilization</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Hospitalizations</td>
<td>2 ± .6</td>
<td>.4 ± .9</td>
<td>1.7 ± 1.9</td>
<td>.2 ± .4</td>
<td>.5 ± 1.1</td>
</tr>
<tr>
<td>Hospital visits</td>
<td>6</td>
<td>0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Hospital days</td>
<td>8 ± 3.4</td>
<td>2.5 ± 9.1</td>
<td>18 ± 23</td>
<td>8 ± 2.5</td>
<td>18 ± 12</td>
</tr>
<tr>
<td>Outpatient hospitalizations*</td>
<td>6 ± 1.3</td>
<td>.3 ± 1.1</td>
<td>1.4 ± 3.0</td>
<td>.1 ± .3</td>
<td>.4 ± 1.4</td>
</tr>
<tr>
<td>Clinic visits</td>
<td>7 ± 6.4</td>
<td>7 ± 5.5</td>
<td>10 ± 7.4</td>
<td>6 ± 4.5</td>
<td>7 ± 6.2</td>
</tr>
<tr>
<td>Days of home</td>
<td>6 ± 8</td>
<td>6</td>
<td>8</td>
<td>5</td>
<td>6</td>
</tr>
<tr>
<td>IV antibiotics</td>
<td>1.4 ± 8.3</td>
<td>7.0 ± 12.4</td>
<td>24 ± 46</td>
<td>0 ± 0</td>
<td>6.5 ± 21.3</td>
</tr>
<tr>
<td>Utilization of:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Pancreatic enzymes†</td>
<td>89</td>
<td>84</td>
<td>95</td>
<td>100</td>
<td>89</td>
</tr>
<tr>
<td>Aminoglycosides‡</td>
<td>20</td>
<td>49</td>
<td>71</td>
<td>13</td>
<td>36</td>
</tr>
<tr>
<td>DNase‡</td>
<td>36</td>
<td>72</td>
<td>71</td>
<td>6</td>
<td>49</td>
</tr>
</tbody>
</table>

Costs

As Table 1 shows, the mean cost per patient was $13,300, and the median cost per patient was $5,300. Among all patients, 47% of total costs were attributable to hospitalization. The remainder of costs were attributable to DNase (Pulmozyme) (18%), clinic visits (12%), outpatient antibiotics (10%), and other medications (13%). Figure 1 shows that the relative

Abbreviation: IV, intravenous.

* Outpatient hospitalizations included admissions to short-stay units and day surgery and would usually have had length of stay of ≤1 day.

† Based on reports by clinicians in the Cystic Fibrosis Foundation annual survey.

‡ Based on information from the Kaiser Permanente Pharmacy Information Management System. Aminoglycosides included inhaled tobramycin as well as intravenously administered aminoglycosides.
contributions of hospitalization and other services to total cost varied according to the severity of disease. For the heart-lung transplant, total charges exceeded $500 000, although the actual costs to the health plan were less than this amount under an existing contractual arrangement.

Severity of illness was an important determinant of costs; the mean costs were $6300 among patients with mild CF, $11 400 among patients with moderate CF, and $43 300 among patients with severe CF. Costs also varied according to the patient’s age. Patients who were from 0 to <11 years old (N = 49) had a mean cost of $8700; those who were from 11 to <18 years old (N = 41) had a mean cost of $17 000, and those who were ≥18 years old (N = 46) had a mean cost of $15 000. In this relatively small sample, there was no statistically significant difference in costs between males and females.

The severity-adjusted costs that we observed were applied to the 23 000 patients who have been diagnosed with CF nationally. Among all CF patients in the United States, 56% have mild disease, 28% have moderate disease, and 16% have severe disease (S.C. Fitzsimmons, unpublished data). If the costs of the HMO of this study were used to generate estimates for the entire US population, the total estimated cost of medical care for CF in 1996 would be $314 million; the mean cost per patient would be $13 650.

**DISCUSSION**

The annual cost of medical care for CF among patients in this study averaged $13 300, and ranged from $6200 in mild patients to $43 300 in severe patients during 1996. This study is unique because it provides a breakdown of CF-related costs by severity of disease as well as by source. For example, hospitalization accounted for approximately half of the total costs, and DNase (Pulmozyme) accounted for nearly half of all medication costs. The costs described here provide information for evaluating the cost-effectiveness of future therapies for CF, and the utilization patterns described should be useful in efforts to monitor their clinical and cost impact.

The mean cost of CF care in the current study was $13 300, less than the amount in a previous report by the CF Foundation (S.C. Fitzsimmons, unpublished data) but similar to the mean cost of $14 377 in 1992 dollars that was reported in a Washington Medicaid population of children with CF.6 The CF Foundation cost estimate of $45 000 may be higher, in part, because it was based on 1989 estimates of hospital, outpatient, and medication use that have been updated to account for inflation.4 During the last decade, the preferred setting for administering intravenous antibiotics has shifted largely from the inpatient to the outpatient setting. Although this shift is probably more pronounced in capitated environments than in fee-for-service environments, we believe that this trend in care is generalizable. In addition, it is possible that advances in CF care, including inhaled antibiotics and DNase (Pulmozyme), may have reduced hospitalization. However, it is important to note that the current study did not address the question of whether giving treatments actually reduced hospital costs compared with not giving these treatments.

Our study may also have observed lower costs than other studies because we estimated actual costs (the amounts invested to provide services and goods) rather than charges (the amounts that providers bill to payers). For example, the Washington State study used Medicaid expenditures that reflect payment rates in a fee-for-service setting.5 Costs are believed to present a more consistent picture of the economic effects of health services utilization, because they are not affected by profit mark-up or cost-shifting.6 Third-party payers and patients will likely face charges that are higher than the costs reported here.

The costs for CF patients are far higher than the annual medical costs of $955 for average children between the ages of 0 to 18 years in the Washington State population.3
Medicaid population. Children with CF or other chronic illnesses may be especially vulnerable in competitive health care environments because of the cost containment pressures. The high costs that are observed here highlight the need for health policy strategies that support insurability and high quality services for these children.

The population that we studied was relatively representative of CF patients in the United States. The proportion of CF patients who used DNase in this population (49%) was similar to the percent that is cited in the country’s CF national registry (44%; S.C. Fitzsimmons, unpublished data). However, in this study setting, home health nursing was probably used less frequently than in other settings, because it was not a covered benefit.

This study was limited to direct medical costs. Nonmedical costs such as child care, transportation expenses, and missed work by parents who take time off to care for children with illness may have an economic impact that is similar to or greater than that of medical service costs. In the future, medical costs for CF could increase if inhaled antibiotics, eg, tobramycin, are recommended more widely. The trend toward increased use of DNase by patients with milder disease could also increase costs.

CONCLUSION

We conclude that the cost of medical care for CF varies greatly with the severity of the disease but is substantial even among patients with mild disease.

These findings underscore the need for strategies to ensure adequate insurance and health services for children with this condition or other special health care needs.

ACKNOWLEDGMENTS

We thank Stacey Fitzsimmons, MPH, of the Cystic Fibrosis Foundation, for sharing data from the Foundation’s annual survey and for advising this work, and the patient who received the heart-lung transplant for his efforts in data collection.

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