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Understanding the Costs of Care for Cystic Fibrosis: An Analysis by Age and Health State

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ABSTRACT

Objectives: Cystic fibrosis (CF) is an inherited disease that requires more intensive treatments as the disease progresses. Recent medical advancements have improved survival but have also increased costs. Our lack of understanding on the relationship between disease severity and lifetime health care costs is a major impediment to the timely economic assessment of new treatments. **Methods:** Using data from three waves of the Australian Cystic Fibrosis Australia Data Registry, we estimate the annual costs of CF care by age and health state. We define health states on the basis of annual lung-function scores and patient's organ transplant status. We exploit the longitudinal nature of the data to model disease progression, and we use this to estimate lifetime health care costs. **Results:** The mean annual health care cost for treating CF is US $15,571. Costs for patients with mild, moderate, and severe disease are US $10,151, US $25,647, and US $33,691, respectively. Lifetime health care costs are approximately US $306,332 (3.5% discount rate). The majority of costs are accounted for by hospital inpatients (58%), followed by pharmaceuticals (29%), medical services (10%), complications (2%), and diagnostic tests (1%). **Conclusions:** Our study is the first of its kind using the Australian Cystic Fibrosis Data Registry, and demonstrates the utility of longitudinal registry data for the purpose of economic analysis. Our results can be used as an input to future economic evaluations by providing analysts with a better understanding of the long-term cost impact when new treatments are developed.

Keywords: Australia, cost of illness, cystic fibrosis, registry data.

Introduction

Cystic fibrosis (CF) is the most common life-shortening genetic disease, with an incidence of 1 in 2500 and carrier frequency of 1 in 25, among Caucasians [1]. With recent advances in treatment, most children with CF now can expect to survive into adulthood and life expectancy has improved considerably. CF is a progressive disease that affects many organ systems. As the disease progresses, patients require more intensive health care that includes home-based care, medications, more frequent and prolonged hospital admissions, and, in around half of all cases, lung transplantation [2,3]. With the advent of new and improved treatment options, the patterns of care have changed and this has had an impact on both health care costs and patient outcomes. For example, two of the key medications developed in the last 15 years, dornase alfa (Pulmozyme) and tobramycin (Tobi), cost around US $10,000 per annum and more sophisticated technology, such as gene-based treatments, may be even more expensive. Ever increasing pressures on health care budgets mean that health care decision makers are expected to require evidence on the cost-effectiveness of new treatments before funding is approved.

Cost-effectiveness analysis requires a sound understanding of the long-term costs of care for CF. In particular, information on how treatment costs are altered when disease progression is slowed would be a valuable input to future economic analysis. The aim of this study was to provide researchers with estimates of the long-term costs and consequences of CF progression. Such estimates will be of particular value to policymakers and evaluators who wish to examine the potential cost impact of new technologies. The results reported in this study will allow short-term clinical trial results to be extrapolated over the longer term.

Previous Literature

There have been a small number of studies that examined the cost of care associated with CF. A systematic review identified eight cost-of-illness studies, but only five of these were based on individual patient care data, with the other three based on cost estimates provided by clinical experts [4]. We have identified four further studies that were published after the Krauth et al. [4] review. Table 1 summarizes each of the studies that were based on patient data. The average annual health care cost ranged from US $8,148 to US $50,723 (in constant 2009 dollars). Some studies found considerable cost variation among patients with CF depending on the patient's lung function, whereas others found no significant effect. For example, the study by Lieu et al. [10] found that the average annual health care cost for a patient with

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Table 1 – Summary of previous studies—health care cost of treating cystic fibrosis.

Source	Study year	Study country	Patients (n)	Mean age (y)	Age range (y)	Mean annual cost* (US $)
Robson et al. [5]	1990	UK	119	21	16–44	21,533
Wildhagen et al. [6]	1991	The Netherlands	81	14	0–37	22,737
Ireys et al. [7]	1993	USA	204		0–18	20,147
Baumann et al. [8]	1996	Germany	138		0–18	33,039
Johnson et al. [9]	1996	Canada	303	18		8,148
Lieu et al. [10]	1996	USA	136	17	0–56	17,546
Heimeshoff et al. [11]	2004	Germany	212	20	0– adult	50,723
Horvais et al. [12]	2001	France	65			21,830
Eidt-Koch et al. [13]	2006	Germany	301			27,999
DeWitt et al. [14]	2008	USA	352	14.6	5– adult	40,037

* All originally reported national currencies converted to US $ at 2009 price levels applying OECD PPP conversion rates and using the CCEMG – EPPI-Centre Cost Converter (see http://eppi.ioe.ac.uk/costconversion/default.aspx); blank values, not stated.

poor lung function was seven times greater than for a person with relative good lung function status. A more recent study by DeWitt et al. [14] found that baseline lung function score was not a significant predictor of health care costs.

These studies have been limited by their data. The small sample size of all studies (ranging from 65 to 352) limits their ability to examine differences in the cost among different population groups. For example, the study by Baumann et al. [8] relied on children's resource use data to estimate costs of adult patients. Other studies were limited by the scope of the data collection. For example, Horvais et al. [12] included out-of-hospital costs but did not have data on inpatient treatments and Heimeshoff et al. [11] focused on patients treated in one center. Consequently, these studies are limited in their ability to provide an overall assessment of the lifetime health care costs for the entire CF population.

Our study is based on the Australian Cystic Fibrosis Data Registry (ACFDR). The longitudinal nature of this data allows us to estimate the rate of CF progression, and its large sample size allows us to calculate the rate of disease progression for various age groups and health states as well as the health care costs associated with treating CF. The results from the transition and cost analysis are then combined to arrive at the estimated lifetime costs of CF.

Methods

Data

Three years of data from the ACFDR were used (2003, 2004, and 2005), with de-identified data from participants that can be linked across these years. The ACFDR includes information on clinical measures, mortality, demographics, complications, and health care resource use. For more information on the ACFDR, including descriptive analysis and data items, see the reports published by Cystic Fibrosis Australia [15,16].

Estimating Transitional Probabilities to Model Disease Progression

The ACFDR contains data on the forced expiratory volume in 1 second as a percentage of predicted volume (FEV_1%)—a standard measure of lung function. Severity of lung disease is the key to the quality and length of life [17] of patients with CF. The best recorded FEV_1% measure in each year was used to classify health states. We chose the FEV_1% cutoff scores for severity states 1, 2, and 3 on the basis that these were consistent with a previous US study that examined the cost of CF care [10]. We generated a separate category for patients who had received a lung transplant because these patients require medications and health care services that are in addition to standard CF care [18]. For this reason, transplant patients were assumed to remain in health state 4 unless a death was recorded. Death is the fifth and absorbing health state. Patients can therefore progress through five health states, defined as follows:

- Health state 1—mild disease where $FEV_1\% \geq 70$
- Health state 2—moderate disease where $40 \leq FEV_1\% < 70$
- Health state 3—severe disease where $FEV_1\% < 40$
- Health state 4—where a patient has received a heart and/or lung transplant
- Health state 5—where a patient has died.

Patients in the CF registry with FEV_1% observations in at least two separate years were included in estimating the transition probabilities. Those with fewer than two health state