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Original Article

Costs and health-related quality of life of patients with cystic fibrosis and their carers in France

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Abstract

Background: Our goal was to provide data on the economic burden and health-related quality of life (HRQoL) associated with cystic fibrosis (CF) in France.
Methods: A retrospective cross-sectional study was carried out on adults and children with CF, who completed an anonymous questionnaire regarding their socio-demographic characteristics, healthcare consumption and presence of a carer. Costs were calculated with a bottom-up approach, and HRQoL was assessed using EQ-5D.
Results: 82 adults and 158 children were included. The total average annual cost of CF was €29,746 per patient. Total costs were higher in adults than in children and increased with disease duration. The average utility was lower in adults (0.667 vs. 0.783 in children, $p = 0.0015$). The HRQoL of carers was also affected (0.742 and 0.765 for carers of adults and children with CF, respectively).
Conclusions: Our study highlights the burden of CF in terms of costs and decreased HRQoL for both patients and carers.

Keywords: Cystic fibrosis; Health-related quality of life; Costs and cost analysis; Economic burden

1. Background

The prevalence of cystic fibrosis (CF) is estimated at 12.6/100,000 in Europe [1], and in France, the neonatal screening programme reports a prevalence of 1 in 4,754 births for the period 2002–2012 (roughly 21/100,000), with great variations among regions [2].

As is often the case with rare diseases – defined by the European Commission as disorders affecting fewer than 5 in 10,000 people [3] – there is no curative treatment for CF. Patient lifespans are greatly shortened, with a life expectancy of roughly 40 years [4], and studies have found that quality of life is also affected [5–7]. Despite its low prevalence, CF may have a considerable impact on healthcare system expenditures in terms of direct healthcare costs and lost productivity [8–10], as orphan drugs are often very expensive and CF's symptoms often appear at a young age.

The project "Social economic burden and health-related quality of life (HRQoL) in patients with rare diseases in Europe" (BURQOL-RD) was carried out under the European Commission's 2nd Programme of Community Action in the

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Field of Public Health to explore these issues. Its goal was to develop and test a tool that could quantify the burden of different rare diseases. In order to do so, it examined the socio-economic burden and HRQoL of patients suffering from ten rare diseases – including CF – as well as their carers in eight European countries [11].

We present here the results of the BURQOL-RD project regarding the HRQoL of French patients with CF and their carers and the average cost of the disease, including direct healthcare costs, direct non-healthcare costs (both formal and informal) and indirect costs.

2. Methods

2.1. Patients

A retrospective cross-sectional study was carried out between September 2012 and May 2013. Patients with CF were recruited on a strictly voluntary basis through the CF reference centre of Nantes–Roscoff, the French CF Society and the patient association *Vaincre la Mucoviscidose*. Patients who consented to participate were directed to the BURQOL-RD website and asked to complete an anonymous questionnaire regarding their socio-demographic characteristics (age, sex, education level, marital status, employment etc.), healthcare consumption in the previous six months (drugs, medical devices, consultations, tests etc.), impact of the disease on their employment and presence of any formal or informal carers. For children under 18, the questionnaire could be completed by a parent or other carers. Responses were considered valid if at a minimum the questions regarding resource utilisation to compute direct healthcare costs were completed. The patient's principal carer was asked to respond to a separate questionnaire detailing their characteristics and time spent caring for the person with CF.

2.2. Costs

Costs were estimated from a societal perspective using a bottom-up approach and 2012 prices as a reference. The questionnaire included detailed information on the resources used by each patient within the previous six months (drug consumption, hospitalisations, consultations, hours of care needed by the patient etc.). Each resource was then valued with its unit cost (see below), and total costs were computed by multiplying each resource's cost by the number of units consumed. An annual average cost per patient was then calculated. It was only assessed for this one year, as it was not the goal of the study to extrapolate costs to a longer-term time horizon.

2.2.1. Direct healthcare costs

Direct healthcare costs consisted of drugs, medical tests, consultations, hospitalisations, medical devices and healthcare transport. The unit cost for drugs, medical devices and medical tests was taken from the Statutory Health Insurance (SHI) tariffs [12–15]. The cost of a consultation was calculated for each specialty, using an average consultation cost which accounted for extra-billing beyond the statutory tariffs by certain doctors. Regarding hospitalisation costs, a daily tariff was derived from the National Hospital Cost Study using weighted disease-related groups [16]. Finally distances for ground transport costs were approximated with the average distance for access to healthcare previously estimated in France [17], with variable costs per kilometre depending on the type of transport. Unit costs are presented in Appendix 2.

2.2.2. Direct non-healthcare formal costs

Direct non-healthcare formal costs included professional care (either at home by a professional carer or in institutions run by social services) and non-healthcare transport. Transport costs were calculated in a manner similar to healthcare transport costs (described above). Home-based care costs were valued using the French mean wage of a home carer, while average daily tariffs were used for services provided in institutions.

2.2.3. Direct non-healthcare informal costs

Informal carers were defined as people providing unpaid help to a family member or friend requiring care. We used the replacement cost method to value the hours dedicated to providing care, which assumes that a professional carer would have to be hired if the informal carer did not provide these services [18]. The amount of time informal carers spent helping the person with CF was recorded in the questionnaire, and we used the average hourly wage of a health aide to calculate the cost.

2.2.4. Indirect costs

We calculated the indirect costs for productivity loss and early retirement as a result of the disease. We took a human capital approach (HCA) to convert the data regarding time away from work into monetary units [19]. This method uses the average earnings of a worker as a proxy for labour productivity losses, and we based our estimations on the average annual French gross wages by gender provided by the National Institute of Statistics and Economics Studies (INSEE) [20]. While the friction cost approach (FCA) [21] which takes into account the compensation of a short-term absence by colleagues or the patient himself on his return or of a long-term absence by the hiring of a new employee could also have been used, HCA was preferred because it is more widely used in this context, and updated and country specific data are lacking for FCA. Finally, patients working in 'sheltered workshops' are paid on average 55% of the legal minimum wage, and their productivity loss cost was calculated using the French mean gross salary minus the wage received.

2.3. Health-related quality of life

The HRQoL of patients with CF and their carers was assessed using the EQ-5D-5L questionnaire [22] along with a visual analogue scale (VAS) to rate their perceived global health on that day (between 0 and 100). The EQ-5D is a generic tool intended for self-reporting adults which assesses five dimensions of health (mobility, self-care, everyday activities,