

## **Economic and social burden of cystic fibrosis in Poland. Estimates based on patients - reported data**

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### **Abstract**

Cystic fibrosis (CF) is a genetic disease inherited as an autosomal recessive disorder. With an incidence of 1:2500 to 1:3000 in white race population it can be regarded as the most common rare disease. Taking into account clinical characteristics of patients, CF may place a substantial burden on a population both from social and economic point of view. The aim of the study was to estimate the cost of CF treatment in Poland with a special emphasis on outpatient treatment. Selected medical and social aspects were also investigated. Survey study was conducted on the sample of 100 children and adolescents patients. Indirect costs linked to lost productivity and a cost of care could be 4 fold higher than direct costs. Medication and diet costs are main components of direct costs. Life satisfaction is deteriorated in the vast majority of studied CF patients.

*Key words: cystic fibrosis, costs, Poland, health economics, burden of disease, patient-reported costs*

### **Introduction**

Cystic fibrosis (CF) is a genetic disease inherited as an autosomal recessive disorder. The incidence of this disease in white race population varies from 1:2500 to 1:3000, but carriers of this genetic defect are many more - about 1 in 20 people (5%) [1]. A cause of the disease is a mutation of a gene responsible for synthesis of membrane chloride channel CFTR (cystic fibrosis transmembrane conductance regulator) which is located on the long arm of chromosome 7 [2]. There were identified about 1600 different mutations of the cystic fibrosis gene till September 2011. The mutation causes lesion of the transport of sodium and water by the cells of exocrine glands, which affects especially the respiratory

and digestive system [3,4]. Most of the patients develop multi-organ dysfunctions, but the quality and life expectancy is usually determined by the changes in the respiratory system [5,6]. Treatment of the disease is mainly supportive care and consists of diet (including fat-soluble vitamins supplementation [7]), pancreatic enzymes supplementation, physiotherapy [8,9,10,11,12]. Exacerbation of a broncho-pulmonary disease (one of CF's complication) usually needs inclusion of antibiotic, bronchodilators and mucolytic therapy.

### **Materials and Methods**

The aim of the study was to estimate the cost of cystic fibrosis (CF) treatment in Poland with a special emphasis on outpatient treatment. Selected medical and social aspects were also investigated including estimation of indirect costs. The inclusion criteria were: patients with diagnosed cystic fibrosis, aged 1-18 years old, treated in the two biggest paediatric centres in Warsaw, providing treatment for all Polish citizens.

Data on costs of main resource groups used in CF treatment were obtained from a survey study from patients or/ and their care-givers. Information was collected with a use of specifically designed, standardised and validated questionnaire during face to face interviews. Individual personal data were blinded, information about age and gender was further processed. Medical aspects covered: date of the disease diagnosis, parents' first reaction to diagnosis, main symptoms and influence on life quality of life.

As far as economics of CF outpatient treatment is concerned, methods of exacerbations' treatment and management were investigated, coupled with drugs, special diet, specialized equipment, health care professionals' consultations and self- education costs. Different sources of financing including state payer- the National Health Fund, social security funding and non- governmental organisations support was investigated, all from patients perspectives.

Direct non- medical costs covering hotel costs and transportation were also taken into account.

Productivity cost of productive and non- productive population being a consequence of care of an ill person was calculated with both working time and leisure time in focus. Influence of CF on working abilities of parents was also studied. Average earning lost in Mazovia district was used in calculations.

Alternative education opportunities for ill children were also looked upon.

Data collected in this study from the sample of 100 patients and their families were extrapolated to the whole CF population stored in the national patient register. Based on that, a total cost of outpatient treatment of CF in Poland was estimated.

### Study group

Survey study was conducted on the sample of 100 patients, of which 43 were female.

The questionnaire was answered either by patients or parents of children treated in two Warsaw hospitals: the Institute of Mother and Child and Children's Memorial Health Institute. The study was conducted from 30th October 2010 to 28th February 2011. Most of the patients were between 11 and 18 years old.

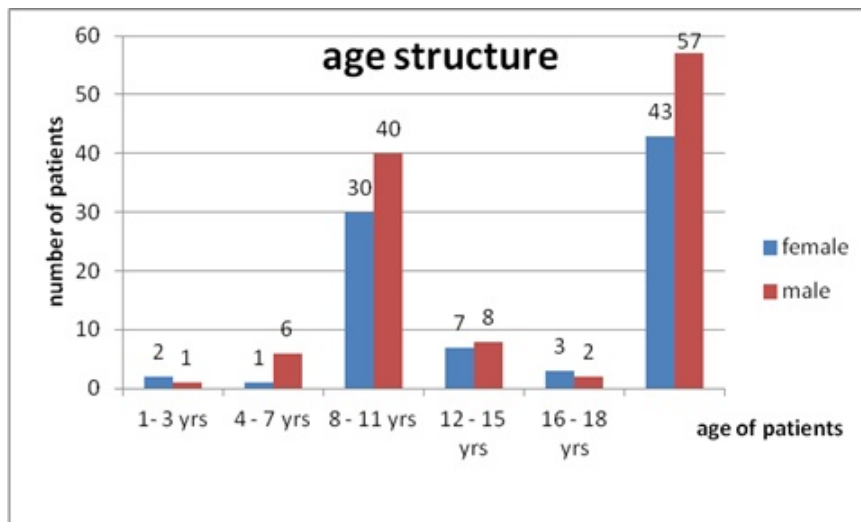


Figure 1. The age structure of patients enrolled

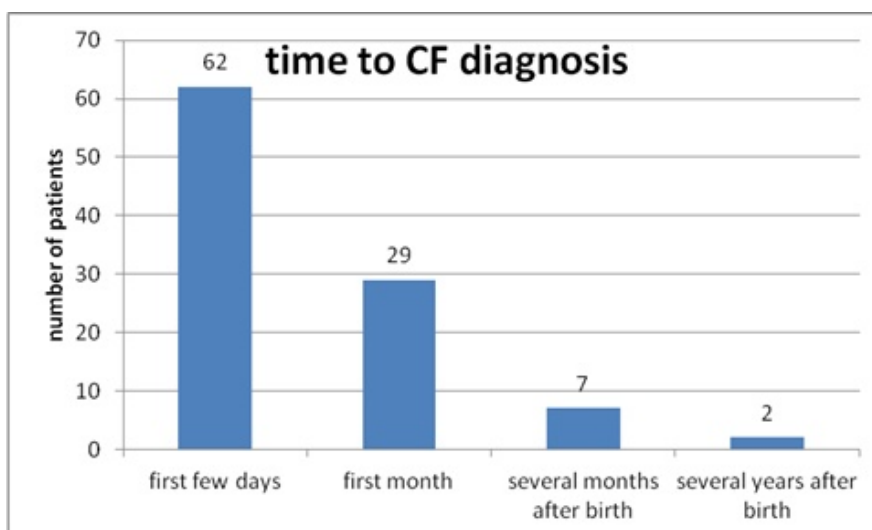


Figure 2. Time to first CF diagnosis in enrolled group of patients  
The data was not divided by gender.

## Results

Collected data indicates that first diagnosis of CF in this study group was made in 91% of patients within first month of life (fig.2).

### Cost of CF treatment

According to the results of the survey all of patients experiencing exacerbations of the disease used pharmacotherapy and physiotherapy.

The expenses on prescription drugs from parents'/care-takers' perspective ("out of pocket") are presented below (fig.3).

The survey results were not presented by age and gender - differences, if any, relating to these aspects could not be reported.

Another identified, significant cost from parents' perspective concerned a special diet. The level of monthly expenditure on food supplements ranged from 100 to 1800 PLN (fig.4).

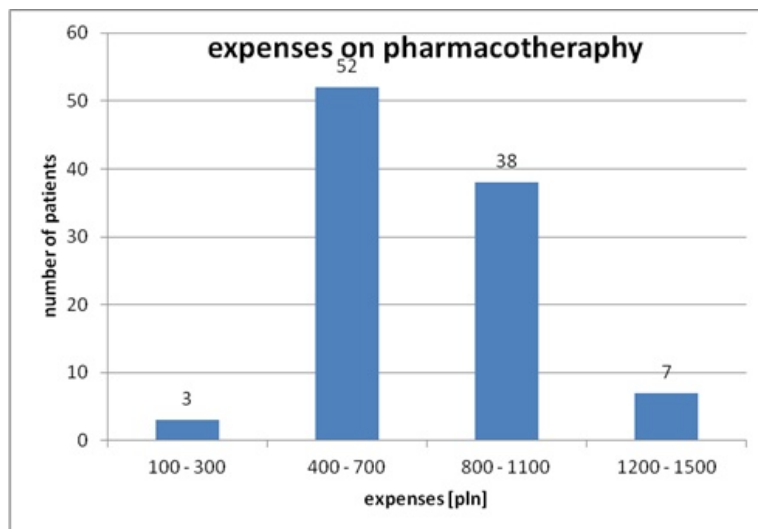


Figure 3. Annual expenses on pharmacotherapy from caretakers' perspective

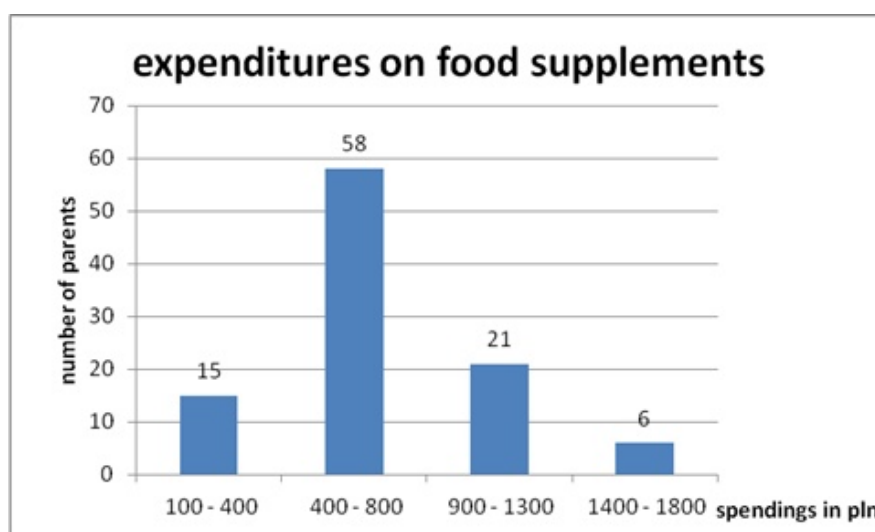
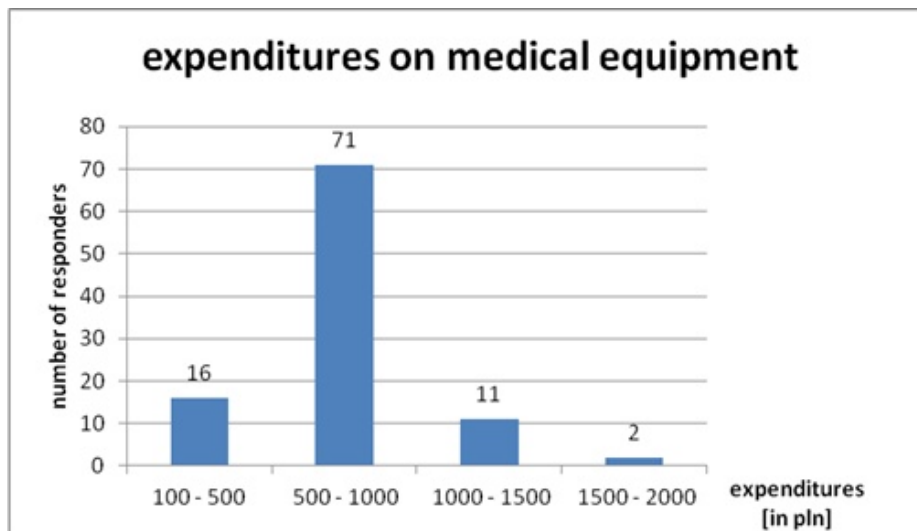
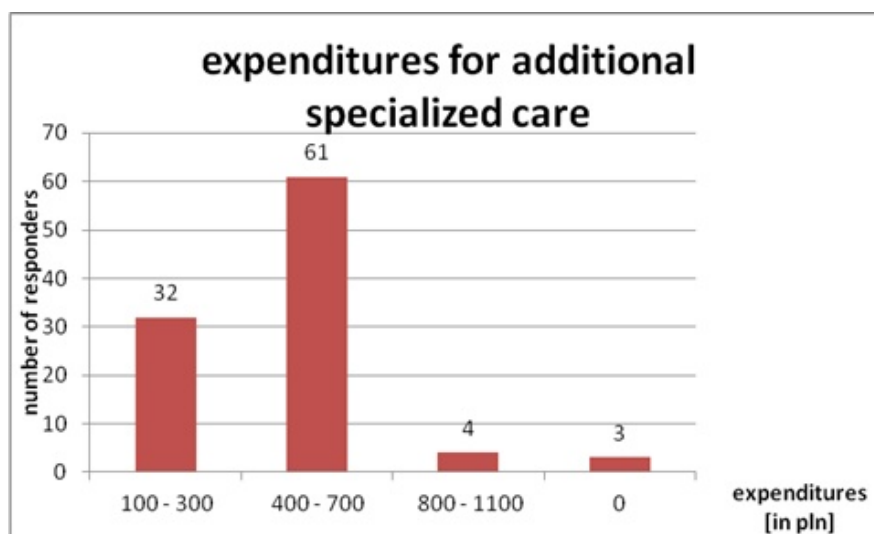


Figure 4. Monthly expenditures on food supplements



**Figure 5.** Annual expenditures on medical equipment



**Figure 6.** Annual expenditures for additional specialized care

The next cost group from the survey concerned medical equipment. Responders indicated that the annual spending on medical equipment range from 100 to 2000 PLN (fig.5).

Majority of responders indicated the need for additional specialized care (97 % of responders).

These included, among others, paid doctor's and psychologist's advices. These annual costs ranged from 0 to 1100 PLN (fig. 6).

From parents' perspective travel expenses appear to be significant. These were understood as the cost of commuting to health centers and accommodation in hotels (fig.7).

All of parents indicated that they were constantly improving their knowledge about CF. On textbooks, brochures etc. most of the parents spent from 20 to 50 PLN monthly (62 % of responders), all the rest did not spend money as they made use of internet resources.

91 % of parents due to high cost of care benefited from foundations, both care and rehabilitation allowance. These additional sources of funding reduced expenses by 50% (decelerated by 53 % of responders), more than 50 % (27 % of responders) or less than 50 % (11 % of declarations).

Additionally data concerning time spent on child's care was collected (fig.8).

According to gathered data, due to CF all children required care from parents. The vast majority of children requires at least 4 hours of parents' care per day (declared by 98% of the study group). That was probably the main reason of reducing a number of working hours by at least one of the parents (declared by 87 % in the study group, fig.9).

These data indicate that 83% of parents need to reduce working hours significantly (understood as a reduction of at least 4 hours per day). In 13 % of cases parents were able to manage the child's care without reducing working time.

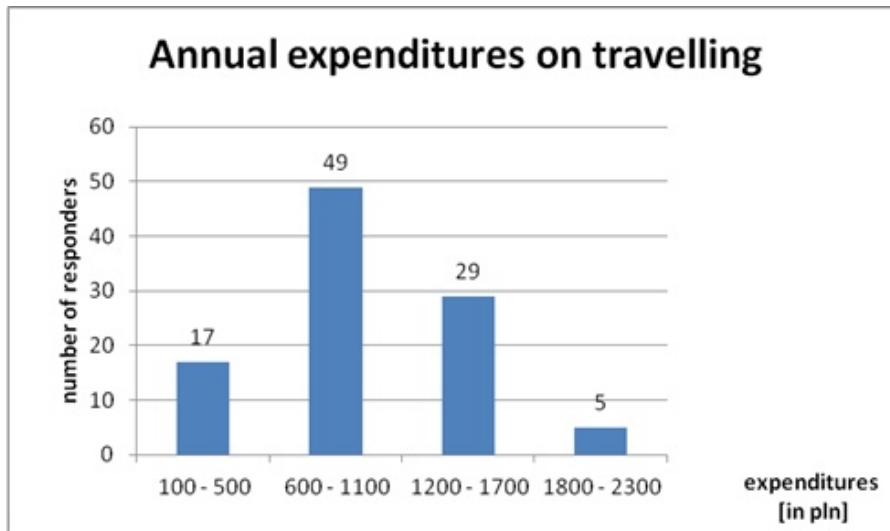


Figure 7. Annual expenditures on traveling from responders' perspective

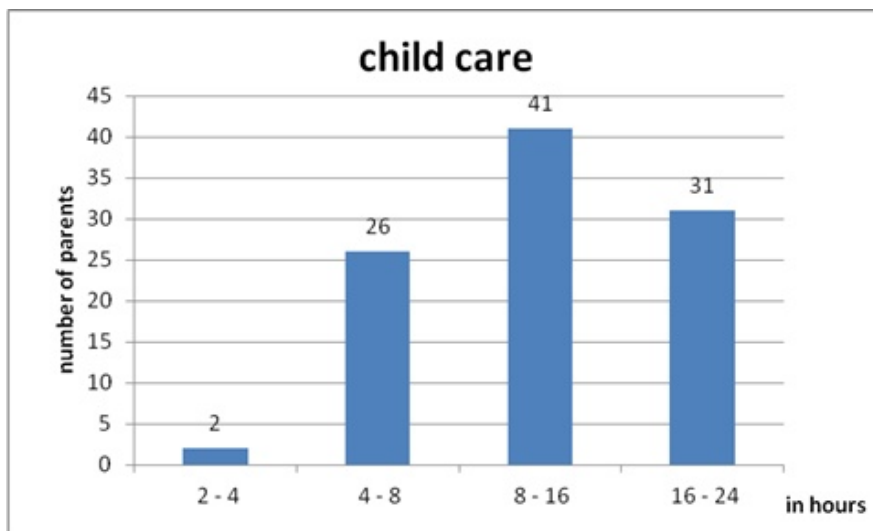


Figure 8. Time spent on child's care per day

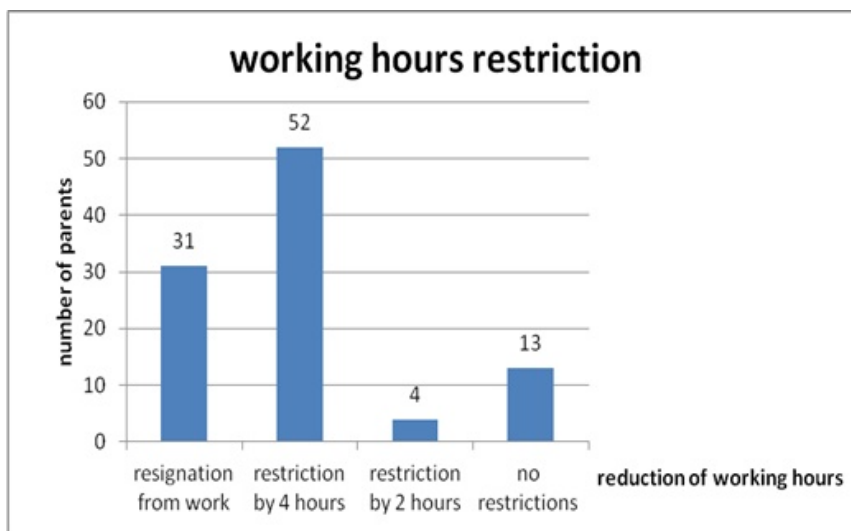


Figure 9. Working hours restrictions caused by providing care to a child

## Quality of life

According to the survey's data, 91 % of responders assessed that the quality of life of children suffering from CF deteriorated. Only 3 % assessed that the quality of life was not worsened (fig.10).

The expenditures presented below were aggregated to present an average cost of CF in Poland from patient's /caregiver's perspective (fig.11).

## Discussion and Conclusions

In the study group time to first diagnosis differs from other scientific reports indicating that in Poland the average age of patients when being CF diagnosed is between 3.5 to 5 years [13]. It may indicate that the severity of CF in investigated population was greater than in the whole CF population observed in the national patient register. The consequence might be that costs calculated in this study were overestimated.

The age structure of the patient groups in this study is characterized by an overwhelming majority of patients aged 11-18 years, who constituted 70% of the study population - both male and female. This in comparison with data from a survey pointing out that in 91% of patients diagnosis of CF was raised within first month of age, may indicate that during first decade of life it comes

to a significant deterioration in the health status of people with CF in Poland. It may point out to urgent need to strengthen patient care from the very moment of diagnosis. Due to absence of information, in the presented results of the study, on the severity of CF in the studied population this hypothesis is difficult to be verified.

Presented results indicate a significant reduction of average patient's age when the diagnosis of CF is made from about 3-5 years to one month. This may indicate the effectiveness of the screening program for CF introduced in Poland in stages from September 2000.

In Poland the average annual cost of treating a child with CF aged 1-18 years from the perspective of a caretaker was estimated to be 108 948 pln. That cost is mainly composed of indirect expenditures.

While presented structure of medical expenses incurred by parents is reliable, the level of the National Health Found spending should be treated with caution. Verification of these data should be the next step in the process of estimating the costs of treating CF in Poland. The calculations based on national or hospital registries should also provide more accurate and reliable data on a real resources consumption level. Such an approach would assure external validation of the results obtained in this study.

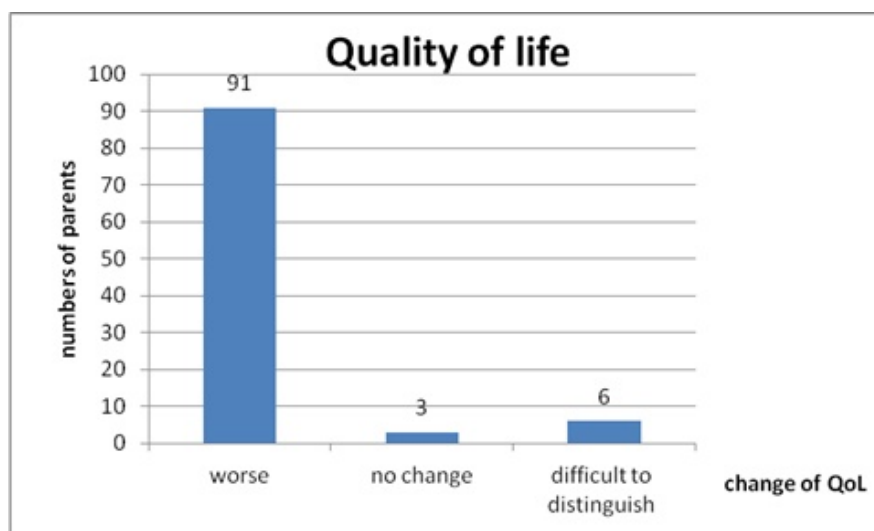


Figure 10. QoL deterioration

cost category		
<b>indirect</b>	child care	58250
	decrease in productivity	30624
	<b>total</b>	<b>88874</b>
<b>direct - medical</b>	medicines	8 970
	diet supplements	8550
	medical equipment	753
	additional specialized care	789
<b>direct non-medical</b>	education	22
	transportation	990
	<b>total</b>	<b>20074</b>
<b>total</b>		<b>108984</b>

**Figure 11.** Annual average cost per patient [in PLN] of CF in Poland

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