

THE LIFE WITH CYSTIC FIBROSIS: SOCIOECONOMIC IMPACT OF THE DISEASE ON PATIENTS AND THEIR CAREGIVERS IN THE CZECH REPUBLIC

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Background

Cystic fibrosis (CF) is a rare genetic disease with a chronic, progressive, and multisystemic nature that negatively affects patients and caregivers. However, its socioeconomic burden has been only scarcely investigated in the literature.(1)

Objectives

The objective of our study was to explore the socioeconomic burden of CF in the Czech Republic.

Methods

A cross-sectional questionnaire-based study was performed among the Czech CF patient organization members (<https://klubcf.cz/>). We collected demographic, clinical, and healthcare resource use data, out-of-pocket and social transfer costs, and responses to validated specific questionnaires: WPAI (Work productivity and activity impairment; range 0% = no work/activity impairment and 100% = full work/activity impairment), EQ-5D, and the Zarit burden interview (ZBI; range min-max: 0-88) describing the caregiver burden.

Productivity loss/costs were assessed using a human capital approach (2,3) with an assumed life expectancy of 45 years (4) (accounting for premature death/lost productivity), caregiver retirement age assumed equal to 64 years (5), and discounted by 3% (6). Productivity loss was monetized using average wage as of Q3/2021 including all levies (i.e., (super) gross wage = costs of work for employer) equal to €2,038/month.(7) The calculations reflect work impairment in working patients and caregivers (Table 5). In patients with a disability pension, the assumed work impairment is equal to 42% (level 1), 60% (level 2) and 85% (level 3) as defined by Act No. 306/2008 Coll., § 39 (8). The weighted average of work impairment due to disability in our study sample reached 63% (proportions of disability pension levels are shown in Table 4). In unemployed patients and caregivers, 100% work impairment was assumed. However, only excessive unemployment above the general population was considered (i.e. 13% unemployed patients and 31% unemployed caregivers and 3.2% unemployment in the general population (9)). The productivity costs related to premature death were calculated in the base case analysis assuming expected survival of 45 years and 100% decrease in work productivity (4). The costs were extrapolated for the whole Czech cohort using data from the registry of cystic fibrosis (RECYF) with 687 patients in total (347 adults and 340 paediatric patients) (10).

All costs were recalculated from Czech Crowns (CZK) to EUR (€) using the average exchange rate in 01/2022-04/2022 equal to 24.604 CZK/€.(11)

Results

In total, 257 patients completed the questionnaires (37% of the Czech CF population). The mean age was 17 years; most were females (59%), and the mean FEV1 was 81.4% (SD:25.4%) (Table 1).

Nearly half of the patients (i.e., 107 patients) had caregiver(s) with a mean age of 39 years. The study revealed a significant time burden for caregivers (an extra 4.6 hours/day). ZBI scores were equal to 25.4 (Figure 1), comparable with advanced cancer, dementia, or Duchenne muscular dystrophy (12-14).

Table 2 shows activities and corresponding time burdens for both patients and their caregivers.

Total out-of-pocket family costs related to CF were €278/month, mainly related to medicines, supplements and medical devices (€105; the sum of prescribed and over-the-counter (OTC)), foods (€73), and transport (€59) (Table 3). A significant proportion of patients received a disability pension (25%) or other social security benefits (18%) (Table 4). The proportion of unemployed caregivers was ≈10-times higher than the general population (31% vs. 3.2%) (9).

The quality of life (QoL) measured by EQ-5D was decreased in both patients (0.802) and their caregivers (0.869). Patients with caregiver(s) had lower QoL values probably due to higher disease burden. With increasing disease severity (defined by FEV1%), the QoL decreased and reached 0.852, 0.823 and 0.747 in mild (≥70%), moderate (40-69%) and severe disease (<40%), respectively. The work impairment of employed patients and caregivers was 25% and 15%, respectively, with the majority accounting for presenteeism (19% and 11%, respectively). The EQ-5D and WPAI results are shown in Table 5.

Total lifetime discounted productivity costs extrapolated to the whole Czech CF cohort (N=687) and their caregivers (N=255) were €155,181,286 (€225,883 per patient including caregivers). Detailed results of productivity costs are shown in Table 6.

Conclusion

Our study revealed a significant societal burden imposed on CF patients and their caregivers. We, therefore, believe that these high societal costs should not be omitted while assessing innovative, effective CF treatments. This study will be utilised during future administrative proceedings of orphan drugs in the Czech Republic as the societal burden has been recently incorporated in the assessment. A detailed description of the new reimbursement system for orphans can be found elsewhere (15).

To our knowledge, this is one of the few studies providing such an extensive description of the societal burden of CF in patients and their caregivers together with other important aspects of life with CF.(1)

References

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Table 1. Baseline characteristics of patients (full set N=257)

Demographic characteristics	
Adults (N, %)	113 (44%)
Children (N, %)	144 (56%)
Male (N, %)	105 (41%)
Female (N, %)	152 (59%)
Clinical information	
BMI in adults (kg/m ²) (N=113)	21.8
FEV1% during last medical visit (% , SD) (N=204)	81.4 (25.4)
Mild ≥70% (N, %)	144 (71%)
Moderate 40-69% (N, %)	42 (21%)
Severe <40% (N, %)	18 (9%)
Lung transplant (N, %)	12 (5%)
Unplanned medical visit during last 12 months (N=254) (N, %)	115 (45%)
Number of unplanned visits during last 12 months (N=115)	3.3 visits
Emergency visits during last 12 months (N=255) (N, %)	39 (15%)
Number of emergency visits during last 12 months (N=39)	1.7 visits
Hospitalizations during last 12 months (N=255) (N, %)	70 (28%)
Number of hospitalization visits (N=70)	1.9 visits
Average length of hospitalization (N=70)	10.5 days
Pulmonary exacerbations during last 12 months (N=255) (N, %)	98 (38%)
Number of pulmonary exacerbations (12 months) (N=98)	2.5 exacerbations
Hospitalizations due to pulmonary exacerbations (12 months) (N=98) (N, %)	40 (41%)
Infections in last 12 months (N=256) (N, %)	200 (78%)
Comorbidities (N=256)* (N, %)	96 (38%)
Causal CF treatment (N=256)† (N, %)	256 (26%)
Antibiotic therapy in last 12 months (N=254) (N, %)	202 (80%)
Employment status (≥15 years of age) (N=132; multiple answers)	
Full-time (N, %)	26 (20%)
Part-time (N, %)	23 (17%)
Casual contract (N, %)	15 (11%)
Freelance contract (N, %)	6 (5%)
Disability pension (N, %)	47 (36%)
Student (N, %)	41 (31%)
Unemployed (N, %)	17 (13%)

*The average number of comorbidities in those who have them was equal to 3.3. The largest proportion of comorbidities accounted for (those with n≥10): diabetes (n=46), pancreatic insufficiency (n=39), other (n=37), nasal polyps (n=35), bone metabolism problems (n=18), gastroesophageal reflux (n=17), rhinosinusitis (n=14), infertility (n=13), anxiety (n=11). †Such as ivacaftor, tezacaftor, elxacaftor, lumacaftor etc.

Figure 1. Caregiver burden measured by Zarit Burden Interview (ZBI) score (N=95)

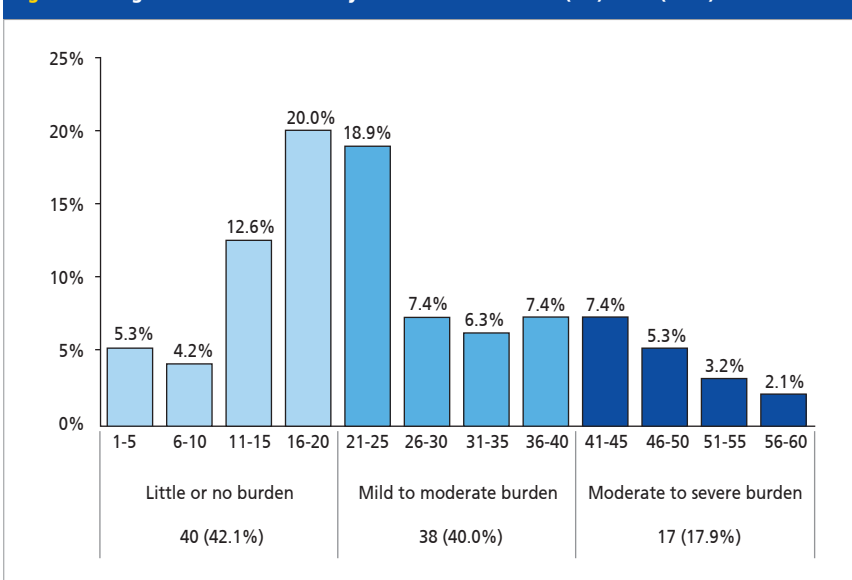


Table 2. Activities related to cystic fibrosis: patients (N=257) and caregivers (N=107)

Activity	N (%)	Duration of activity* (frequency)
Patients		
Inhalation (per day)	250 (97.3%)	18 mins (3.2/day)
Drug and medical device preparation (per day)	232 (90.3%)	19 mins
Exercise, rehabilitation, physiotherapy	230 (89.5%)	17 mins (2.3/day)*
Sterilization of medical devices (per day)	225 (87.5%)	23 mins
Cleaning, hygiene (beyond normal, per day)	195 (75.9%)	28 mins
Intravenous drug application (last 3 months)	20 (7.8%)	54 hours
Intravenous port (last 3 months)	20 (7.8%)	62 hours
PEG administration of enteral feeds (per day)	2 (0.8%)	63 mins
Oxygenotherapy (per week)	2 (0.8%)	960 mins (7/week)
Special food preparation (per day)	34 (13.3%)	91 mins
Travelling to medical examination (per year)	217 (85.1%)	83 mins (4.9/year)
Other travelling due to CF (per month)	98 (38.4%)	100 mins (5.7/month)
Travelling to specialized CF centre (total time)	180 (70.6%)	145 mins
Other activities (education, other medical examinations etc.) (per day)	34 (13.3%)	48 mins
Caregivers		
Total time burden	107 (100%)	276 mins**
Inhalation	88 (82.2%)	36 mins
Drug and medical device preparation	95 (88.8%)	20 mins
Exercise, rehabilitation, physiotherapy	93 (86.9%)	33 mins
Sterilization of medical devices	100 (93.5%)	24 mins
Cleaning, hygiene (beyond normal, per day)	104 (97.2%)	34 mins
Intravenous drug application (last 3 months)	4 (3.7%)	104 mins
Accompany a patient	52 (48.6%)	196 mins
Patient surveillance	88 (82.2%)	142 mins
Patient transport	68 (63.9%)	64 mins
Other activities	17 (15.9%)	110 mins

*Duration of activity or time burden is calculated only in patients/caregivers carrying out the given activity; all values are means. *Exercise only. **The total time burden is not equal to the sum of given activities as it is calculated only in patients carrying out the activity. The total time burden is calculated as a weighted average of patients carrying out the activity and their time burden.

Table 3. Direct monthly costs per family (N=254)

Direct monthly costs per family	Mean in €*
Transport using own car (N=219)	€58
Transport to specialized examinations	€20
Transport to other activities related to CF	€34
Parking	€3
Transport other than by car (related to CF) (N=43)	€1
Costs of specialised foods related to CF (N=254)	€73
Rehabilitation + exercise + physiotherapy (N=133)	€10
Rehabilitation	€4
Exercise	€4
Physiotherapy	€2
Over-the-counter drugs/supplements (N=253)	€60
Prescription drugs/medical devices (N=252)	€45
Drugs	€19
Inhalators	€10
Filters	€6
Liquid clinical nutrition	€5
Other	€4
Other medical costs related to CF (N=252)	€31
Disinfection	€15
Toiletries	€11
Other	€5
Total direct monthly costs of CF/month/family	€278

*Mean costs are recalculated to the whole cohort of 254 patients, non-responders to given items have costs equal to 0.

Table 4. Social security costs of patients and caregivers

Social security type in patients (N=254)	
Disability pension (N, %)	89 (35%)
1 st level* (N, %)	32 (36%)
2 nd level* (N, %)	20 (23%)
3 rd level* (N, %)	35 (39%)
Disability benefits (N, %)	32 (13%)
Sick leave in last 12 months (N, %)	8 (3%)
Other social security benefits (N, %)	5 (2%)
Foundation contribution (N, %)	91 (36%)
None (N, %)	63 (25%)
Social security costs in patients (N=254)	Costs per month €
Disability pension (N=89)	€382
Mobility allowance (N=29)	€31
Allowance for special aids (n=1)	€134
Other social security (N=4)	€308
Social security costs in caregivers (N=108)	Costs per month €
Care allowance (N=53)	€195
Child benefits (N=1)	€41
Housing allowance (N=2)	€230
Parental allowance (N=5)	€342

*Act No. 306/2008 Coll., § 39 defines levels of disability as follows: 1st level (35% to 49% reduction in work productivity), 2nd level (50-69% reduction in work productivity), 3rd level (≥70% reduction in work productivity).

Table 5. EQ-5D and WPAI in patients and caregivers

EQ-5D	Mean (SD)
Caregivers (N=88)	0.869 (0.194)
Patients (N=223)*	0.802 (0.231)
Patients without caregiver (N=125)	0.850 (0.168)
Patients with caregiver (N=98)	0.740 (0.282)
WPAI in adult patients	
Absenteeism (N=45)	10.5% (21.8%)
Presenteeism (N=44)	19.3% (20.8%)
Work impairment (N=44)	24.6% (25.7%)
Activity impairment (N=95)	26.6% (25.1%)
WPAI in caregivers	
Absenteeism (N=36)	7.6% (18.4%)
Presenteeism (N=35)	11.4% (18.5%)
Work impairment (N=35)	14.5% (22.0%)
Activity impairment (N=90)	12.6% (19.6%)

*EQ-5D decreased with increased disease severity measured by FEV1%: 0.852 (mild), 0.823 (moderate) and 0.747 (severe).

Table 6. Productivity costs of the whole CF cohort in Czechia (patients and caregivers)

	Number of patients in cohort	Productivity costs/patient	Productivity costs/cohort
Adult patients with CF in Czechia (N=347) (10)			
Disability pension	124 (35.6 %)	€428,692	€52,957,140
Unemployment	45 (12.9 %)	€413,780	€18,522,018
Students	66 (19.1 %)	-	-
Employed	113 (32.4 %)	€302,850	€34,084,638
Total	347 (100%)	-	€105,563,795
Paediatric patients with CF in Czechia (N=340) (10) and their caregivers (N=255)			
Unemployed caregivers	79 (31 %)	€493,481	€39,009,652
Employed caregivers	176 (69 %)	€60,289	€10,607,838
Total	255 (100 %)	-	€49,617,491
Total number of patients with CF in Czechia (N=687) (10)			
Costs of adult patients	347	-	€105,563,795
Costs of caregivers of paediatric patients	255	-	€49,617,493
Total costs	-	€225,882*	€155,181,286

*Calculated as total costs divided by the total number of patients (N=687).