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Health-Related Quality of Life and Its Drivers for Patients Living With Cystic Fibrosis in Five Key European Countries: a Systematic Review

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INTRODUCTION

- Cystic fibrosis (CF) is a progressive, inherited, and autosomal recessive genetic disorder that mainly affects the lungs, pancreas, and other organs.[1,2] On average, CF affects between 1 in 3,000 and 1 in 6,000 people from European decent[3]
- CF is a multisystem disorder involving an imbalance of salt transport in and out of cells. This leads to formation of thick and sticky mucus, affecting primarily the lungs but also the pancreas, gastrointestinal system, reproductive system, and other organs of the body[4]
- Mutations to the cystic fibrosis transmembrane conductance regulator (CTFR) protein causes CF. Patients with CF have a median survival age of 48.4 years, with many individuals living up to 50–60 years. Maintenance of CF requires aggressive symptomatic therapies from an early age, which adds to disease burden[5,6]
- We aimed to identify comprehensive evidence on the impact of CF on health-related quality of life (HRQoL) in five key European countries and the factors that are associated with poor quality of life (QoL)

OBJECTIVES

The objective of this systematic literature review (SLR) was to identify the HRQoL and its key drivers for children and adults living with CF in the UK, France, Germany, Italy and Spain

METHODS

- A systematic literature search to identify English-language articles published between 2013 and 2023 was performed in the MEDLINE® and Embase® databases, with pre-defined inclusion criteria (Table 1)
- Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA) guidelines were followed for reporting the SLR
- All the records retrieved from the literature search were screened against the pre-defined inclusion criteria, first based on the title and abstract and then on the full-text citations
- The eligibility of publications was assessed by two independent reviewers, with any discrepancy resolved by a third

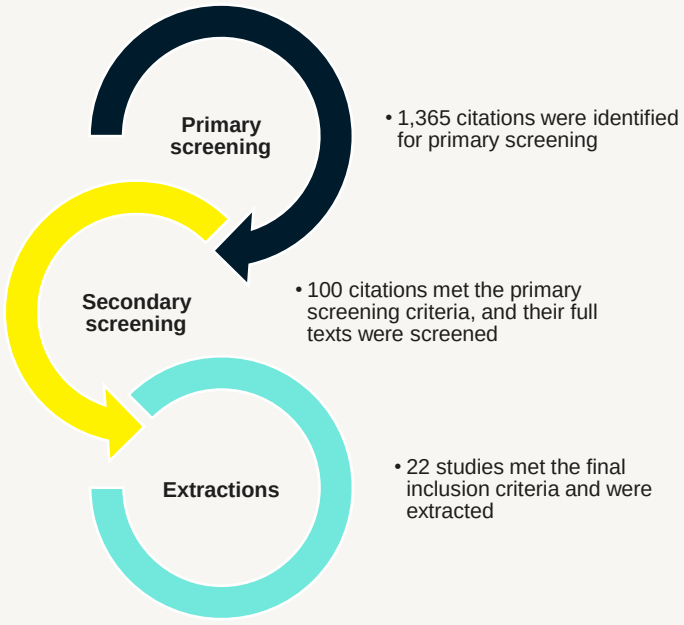
Table 1. Inclusion criteria

Language	English
Timeframe	2013–2023
Population	Adults and children with CF
Country	UK, France, Germany, Italy, and Spain
Outcomes	HRQoL Scales: Cystic Fibrosis Questionnaire-Revised (CFQ-R) and EQ-5D
Intervention and comparator	No restriction

RESULTS

- A total of 1,365 records were screened using the predefined Population, Intervention, Comparison, Outcomes and Study (PICOS)-based criteria; 22 studies were identified and included that evaluated the HRQoL of CF in the five countries (Figure 1)

Figure 1. Study flow diagram



- HRQoL data were reported for the UK in 14 studies, Germany in four studies, and in one study each for France, Italy, and Spain. HRQoL data for all five countries together were reported in one study
- The overall QoL of patients living with CF was significantly lowered by the following factors: increase in age; decrease in body mass index; reduced lung function; being female; presence of pulmonary exacerbations and their severity; disease duration; and associated infections/comorbidities
- Age, lung function and pulmonary exacerbations were the most significant and widely reported drivers of QoL in patients with CF (Table 2)

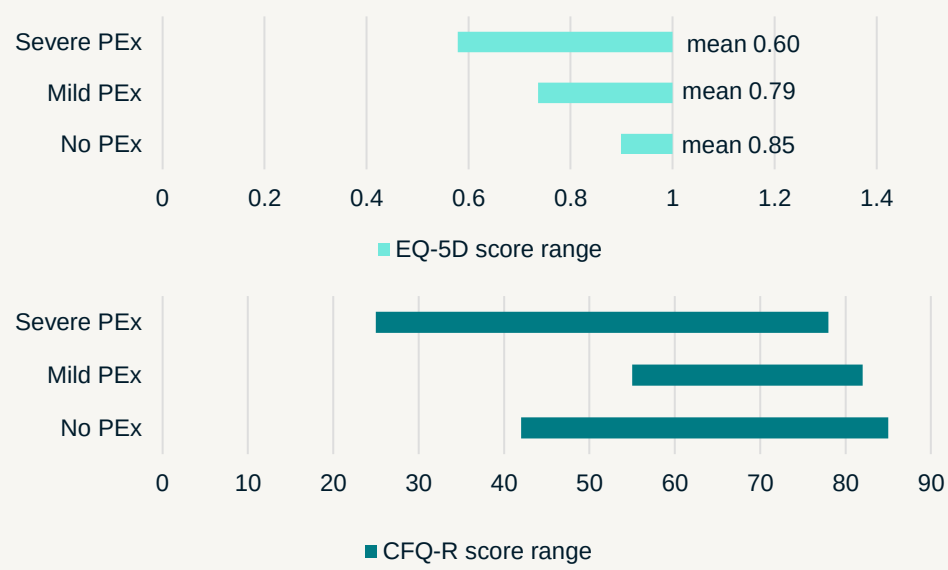
Table 2. Number of studies reporting key drivers

Key drivers	CFQ-R					EQ-5D™
	Physical functioning	Emotional functioning	Treatment burden	Respiratory symptoms	Health perceptions	
Age	2	1		1	1	1
Gender		1				
BMI		1		1	1	
Lung function*	4	2	1	1	1	1
PEx	2	1	1	2	1	1
Disease duration						2
Infections/ comorbidities	1	1	1	1	1	

Key: BMI, body max index; CFQ-R, Cystic Fibrosis Questionnaire-Revised; PEx, pulmonary exacerbation.
Notes: *Lung function as assessed by FEV1%. FEV1%: This represents the percentage of your lung capacity you can expel in 1 second

- Mean EQ-5D utility values for participants were: 0.667 (France), 0.783 (Germany), 0.820 (Italy), 0.870 (Spain) and 0.640 (UK).[7] In the UK, the EQ-5D scores for the CF population (mean 0.64) were lower than general population (mean 0.93; age adjusted)[8]
- Severity of pulmonary exacerbations also significantly affects QoL. EQ-5D and CFQ-R data showed that the more severe the pulmonary exacerbation (PEx), the poorer the HRQoL[8] (Figure 2)

Figure 2. EQ-5D and CFQ-R score ranges as per severity of pulmonary exacerbation



Key: CFQ-R, Cystic Fibrosis Questionnaire-Revised; PEx, pulmonary exacerbation.

- CFTR modulators have shown improvements in disease progression and QoL. In a study conducted in Germany, elexacaftor/tezacaftor/ivacaftor combination improved CFQ-R respiratory domain score by 27.9 (interquartile range: 5.6, 47.2; $p < 0.001$)[9]

CONCLUSIONS

- CF is a life-limiting condition with a significant impact on overall quality of life of patients
- Age, gender, lung functions, pulmonary exacerbation ,and associated infections and comorbidities were the key factors that influence overall QoL
- Appropriate treatment and management of the disease can improve the HRQoL of patients living with CF

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