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Economic Burden of Cystic Fibrosis in the United States: A Systematic Review

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INTRODUCTION

- Cystic fibrosis is a progressive, inherited autosomal recessive genetic disorder caused by mutations in the *CTFR* gene.[1] It affects more than 40,000 people in the US[2] and is one of the most common genetic disorders in white populations[3]
- Cystic fibrosis is a multisystem disorder that affects salt transport in and out of cells, creating an imbalance that causes thick and sticky mucus to form. This primarily affects the lungs, but also affects the pancreas, gastrointestinal system, reproductive system, and other organs[4]
- Patients with cystic fibrosis have a median survival age of 48.4 years, with many individuals living up to 50–60 years. Maintenance of cystic fibrosis requires aggressive symptomatic therapies from an early age, which adds to disease burden[5,6]

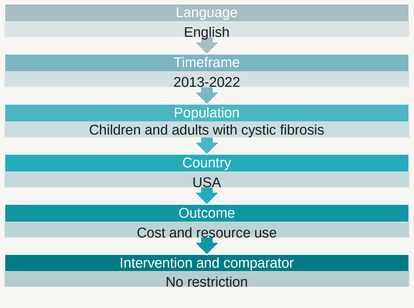
OBJECTIVES

- The objective of this systematic literature review (SLR) was to identify the economic burden (cost and resource use) of cystic fibrosis, in children and adults, in the US

METHODS

- A systemic literature search to identify English-language articles published between January 2013–December 2022 was performed in the MEDLINE® and Embase® databases, with a pre-defined inclusion criterion (Figure 1)
- Preferred Reporting Items for Systematic Reviews and Meta-Analyses 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

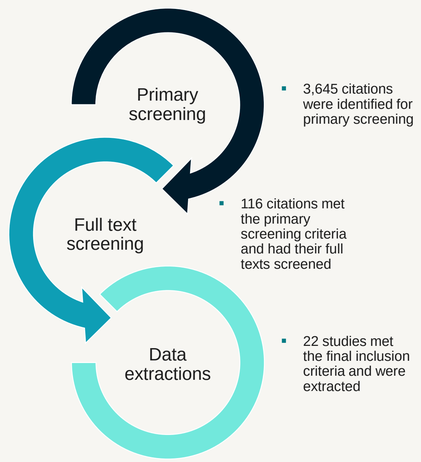
Figure 1. Inclusion criteria



RESULTS

- A total of 3,645 records were screened using the predefined population, intervention, comparison, outcomes and study (PICOS)-based criteria. Twenty-two studies that evaluated the economic burden of cystic fibrosis in the US were included (Figure 2)

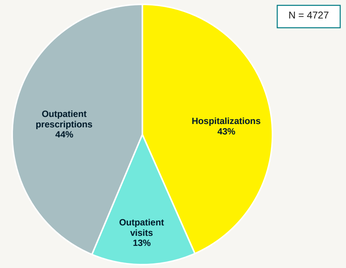
Figure 2. Study flow diagram



- 3,645 citations were identified for primary screening
- 116 citations met the primary screening criteria and had their full texts screened
- 22 studies met the final inclusion criteria and were extracted

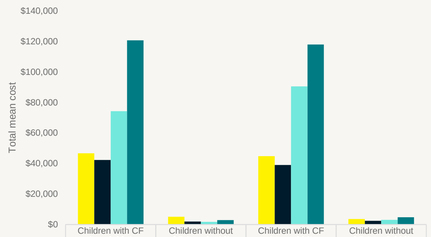
- The mean annual healthcare cost (cost year 2018) for commercially and Medicaid-insured children with cystic fibrosis (< 17 years; N = 4,727) was considerably higher ($82,096) than those without cystic fibrosis (N = 14,181; $3,165). Key cost drivers for children with cystic fibrosis were hospitalizations ($35,617), outpatient visits ($10,634), and outpatient prescriptions ($35,845). For children who were hospitalized, those with cystic fibrosis had significantly longer mean length of stays ($p < 0.05$) than those without cystic fibrosis (Figures 3 and 4)
- The mean (standard deviation) hospital costs for children (N = 3,412) identified from the Kids' Inpatient Database (KID) was $26,249 ($40,593). For adults (≥ 21 years, N = 10,258) identified from the National Inpatient Sample (NIS), the mean (standard deviation) hospital costs were $21,601 ($31,997)[7]

Figure 3. Key cost drivers of cystic fibrosis



Source: Thorat et al. (2021).

Figure 4. Average healthcare cost for children with cystic fibrosis



	COM		MED	
0-2 y	$46,661	$5,075	$44,751	$3,545
2-6 y	$42,283	$1,939	$38,962	$2,410
6-12 y	$74,185	$1,654	$90,497	$3,034
12-17 y	$120,605	$2,836	$117,927	$4,716

Key: CF, cystic fibrosis; COM, commercially insured; MED, Medicaid-insured.
Source: Thorat et al. (2021).

- Inpatient hospitalization and emergency department utilization add a significant amount of economic burden to cystic fibrosis care in adults: the costs of care with inpatient hospitalization were $57,264, compared with $11,473 for care with ambulatory care (patients without emergency department visits and hospitalization) (cost year 2018)[8]. NIS adults had a lower mean length of hospital stay (8.54 days) compared with KID children (9.79 days)[7]
- Cystic-fibrosis-associated comorbidities like cirrhosis, infection complication, and mental health disorders were found to be major contributors to healthcare utilization in children (Figure 5)
- The median hospital charge for cystic-fibrosis-associated cirrhosis was $66,543, compared with $53,365 with no cirrhosis ($p < 0.001$)[10]
- Infectious complications impose a mean hospital charge of $72,292 ($54,069 with no infectious complication) and a mean length of stay of 11.5 days (8.6 days for no infectious complications)[11]
- Mental health disorders – including depression, anxiety, attention deficit hyperactivity disorder, substance use disorder, and adjustment disorder – impose a median hospital charge of $42,797 (versus $34,197 for patients with no mental health disorders) and a median length of stay of 7.7 days (versus 6.2 days for patients with no mental health disorders)[12]

Figure 5. Healthcare cost of cystic fibrosis associated with co-morbidities

Key: CF, cystic fibrosis; IC, infection complications; MHD, mental health disorder.
Source: A: Shaheen et al., 2018; B: Godara et al., 2019; C: Martinez-Schlurmann et al., 2015.

KEY TAKEAWAYS

- Cystic fibrosis is a life-limiting condition with a significant economic burden
- The burden of cystic fibrosis increases with the associated co-morbidities and complications
- Children with cystic fibrosis are associated with a considerable level of HCRU across all age groups
- Hospitalization contributes significantly to the economic burden of cystic fibrosis

CONCLUSIONS

- Hospitalizations, drug utilization, and complications were key cost and resource use drivers in patients with cystic fibrosis. Treatment costs increase with disease severity
- Further measures are needed to enhance health security for patients with cystic fibrosis and relieve the economic burden

REFERENCES

1. Patel et al. *Nutrients*. 2022; 14(5). **2.** About cystic fibrosis | Cystic fibrosis foundation. 2023. https://www.cff.org/intro-cf/about-cystic-fibrosis (accessed April 17, 2023). **3.** Cystic fibrosis: MedlinePlus Genetics. 2023. https://medlineplus.gov/genetics/condition/cystic-fibrosis/ (accessed April 13, 2023) **4.** Dickinson et al. *Pediatr Rev*. 2021; 42(4). **5.** Bresnick et al. *BMJ Open Respir Res*. 2021; 8(1). **6.** Mcbennett et al. *Pediatr Pulmonol*. 2022; 57(Suppl1). **7.** Vadagam et al. *Hosp Pract*. 2018; 46 (Issue4). **8.** Perkins et al. *Pediatr Pulmonol*. 2021; 56(5). **9.** Thorat et al. *Pediatr Pulmonol*. 2021; 56(9). **10.** Shaheen et al. *Can Liver J*. 2018; 1(1). **11.** Martinez-Schlurmann et al. *Crit Care Med*. 2015; 43(12). **12.** Godara et al. *Am J Respir Crit Care Med*. 2019; 199(9).

An electronic version of the poster can be viewed by scanning the QR code.

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