Impact of CFTR Modulator Initiation on Direct Medical Costs for People with Cystic Fibrosis

EE527

University of Maryland School of Pharmacy

Dominique Seo, MPH[1], T. Joseph Mattingly II, PharmD, MBA, PhD, C. Daniel Mullins, PhD[1], Kristine A. Parbuoni, PharmD, BCPPS[1], Roumen M. Vesselinov, PhD[3], David C. Young, PharmD[2] Eberechukwu Onukwugha, MSc, PhD[1]

[1.] University of Maryland School of Pharmacy, [2.] University of Utah College of Pharmacy, [3.] University of Maryland School of Medicine

Purpose

- CFTR modulators (CFTRm) have transformed treatment and improved quality of life for people with Cystic Fibrosis (pwCF)[1]
- The high cost of treatment, which has a list price that exceeds $300,000 per year can create significant financial strain[2]
- Cost-effectiveness analyses (CEAs) have consistently found CFTRm not cost-effective[3], yet they continue to be covered by payers
- There is limited evidence on cost impacts across healthcare settings, which provides important context for value assessment
- This analysis evaluates the impact of CFTRm initiation on direct medical costs overall and across healthcare settings

Methods

- This study used a 25% random sample of the IQVIA PharMetrics® Plus for Academics data; a database composed of fully adjudicated insurance claims data and enrollment information for mostly commercial individuals
- We included patients aged less than 65 who initiated a CFTRm between 2012-2021 and were continuously enrolled in a health plan for 12 months before and after treatment initiation.
- A pre-post design was used to compare direct medical costs in the year before and after treatment.
- We calculated total, inpatient, outpatient, emergency department (ED), and pharmacy costs in the 12 months before and after treatment initiation.
- Regression models adjusted for age, sex, census region, primary payer, and plan type.
- We report incremental costs, estimated using a generalized linear model (GLM).
- Statistical significance was determined at $\alpha = 0.05$

Results

- The sample consisted of 134 pwCF (Table 1), of this 89 (66%) are adults and sex distribution is nearly balanced (52.2% female, 47.8% male)
- Most individuals were commercially insured (85.1%), with PPO plans being the most common (47%).

Table 1: Demographics and Payer Characteristics of included pwCF

Variable	N	Percent
AGE GROUP		
Pediatric	45	33.6
Adult	89	66.4
SEX		
Female	70	52.2
Male	64	47.8
REGION		
Northeast	21	15.7
South	31	23.1
Midwest	46	34.3
West / Unknown	36	26.8
PAYER TYPE		
Commercial	114	85.1
Public / Unknown[a]	20	14.9
PRODUCT TYPE		
HMO	40	29.9
PPO	63	47.0
Other[b]	31	23.1

Footnotes:
HMO: Health Maintenance Organization, PPO: Preferred Provider Organization
a. Includes: State Children's Health Insurance Program (SCHIP), Medicaid, Medicare Advantage, Self-insured
b. Includes: Indemnity/ traditional, Point of Service, Consumer Directed Health Care, Health Savings Account (HSA)

Results

- Compared to the year before CFTRm initiation, in the year following treatment initiation the average **total** costs increased 3.2x or by $261,056 (Table 2)

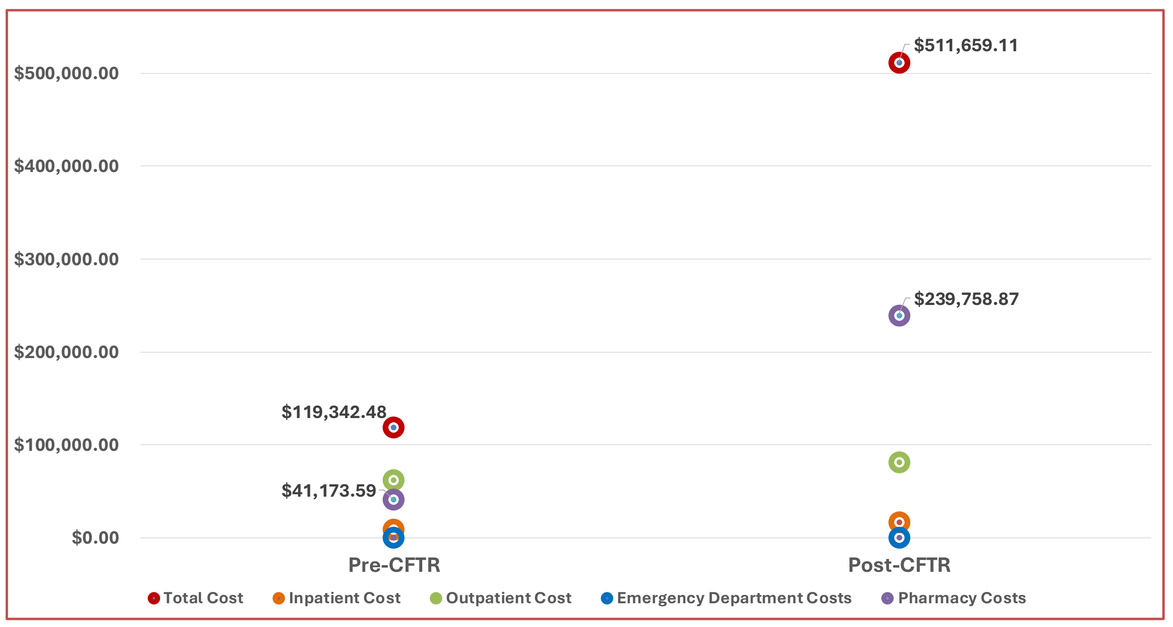
Table 2: Average Adjusted Direct Medical Costs Per Patient Pre- and Post-CFTR Modulator Therapy Initiation

Cost Category	Pre-CFTR Modulator Initiation	Post-CFTR Modulator Initiation	Incremental Change
Total Cost	$119,342	$511,659	$392,317
Inpatient Cost	$8,962	$16,986	$8,026
Outpatient Cost	$61,8450	$81,598	$19,748
Emergency Department Cost	$312	$258	($54)
Pharmacy Cost	$41,174	$239,759	$198,585

Note: All values represent mean costs per patient. Negative values in parentheses indicate cost reduction.

- Regression estimates showed a 5.8x increase in **pharmacy** costs from $49,542 to $289,313 ($p < 0.01$)
- ED costs dropped about 25% (from $805 to $599), but this change wasn't statistically significant (p=0.43); similarly, inpatient and outpatient cost differences showed no significant changes

Figure 1: Change in Direct Medical Costs Per Patient Pre- and Post-CFTR Modulator Therapy Initiation



Strengths and Limitations

- Administrative Claims Data may not fully capture true healthcare costs or utilization; excludes over-the-counter and uncovered medical expenses
- This study has an extended follow-up time that tracks outcomes over 12 months, allowing assessment of sustained impact of CFTR modulators
- Missing most public insurance programs and have limited information on key health outcomes
- There are no external controls, so observed changes may be influenced by external factors rather than the intervention alone

Conclusion

- Pharmacy costs are a major driver of rising healthcare costs for pwCF
- The use of real-world data captures total costs, utilization, and economic footprint, providing insights for clinicians, payers, and policymakers
- Use of regression models, rarely applied in CF research, provides important methodological strength to the analysis
- These findings highlight the need for value-based pricing and robust coverage to sustain high-cost therapies

References

References available upon request.

Contact Information: Dominique Seo, MPH

Department of Practice, Sciences, and Health Outcomes Research

University of Maryland School of Pharmacy, 220 Arch Street, 12th Floor, Room 01-409, Baltimore, MD 21201

(e-mail: dseo@umaryland.edu).