# Adherence to Cystic Fibrosis Transmembrane Regulator Modulator therapies, hospitalizations, and medical costs in patients with Cystic Fibrosis using MarketScan commercial claims and encounters database

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## BACKGROUND

Cystic fibrosis (CF) is a genetic disease resulting from the mutation of the cystic fibrosis transmembrane conductance regulator (CFTR) gene located on chromosome 7. CF causes a buildup of thick, sticky mucus in the lungs, pancreas, and exocrine glands, and primarily affects the respiratory and digestive systems. The treatment of CF is mainly directed toward relieving symptoms that result from the disease and treating the underlying cause - the genetic defect (antibiotics, mucolytics, pancreatic enzymes, and CFTR modulator(CFTRm)).[1-5]

There has been research that has demonstrated that poor adherence is linked to worse health outcomes. New CFTRm continue to come onto the market and improve health in patients with CF. Many patients with CF take a CFTRm that corrects the underlying defect of their particular disease. CFTRm therapy adherence and clinical outcomes are increasingly more important to address.

## OBJECTIVES

The goal of this research study was to examine the association between CFTRm therapy adherence and the outcomes of hospitalization and medical costs in CF patients using MarketScan commercial claims from 2019.

## METHODS

MarketScan commercial claims from 2019 were used to calculate proportion of days covered (PDC) in this retrospective study. For outcome comparisons, patients were defined as adherent if they had a PDC ≥ 80%.

Patients were eligible for PDC calculation if they were adults (≥18 years), prescribed one of three CFTRm therapies (Kalydeco, Orkambi, Symdeko, and Trikafta was not included since it wasn't approved until October 2019), had at least one-year continuous enrollment in a health plan, ≥2 medical claims with a diagnoses for CF (ICD-9 / ICD-10 CM (Clinical Modification)) occurring ≥30 days apart during study period.

Outcomes examined in CF patients were bed days (for those hospitalized), comorbidities (using The Charlson Comorbidity Index (CCI) to assess comorbidity level by combining the number and severity of 19 pre-defined conditions), CF-associated medical costs, CFTRm medication costs, and non-CFTRm medication costs.

Independent *t-tests* were used for continuous outcome variables and chi-square tests were used for categorial outcome variables. A threshold of $p < .05$ was used as our criteria for judging differences as statistically significant.

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## RESULTS

A total of 598 patients met criteria for the study. Patients with CFTRm therapy were younger (mean (*M)* = 32 years). Majority of patients were male (57%). Region of residence was most commonly south (40%), followed by north central (26%), northeast (21%), west (12%) and unknown(1%).

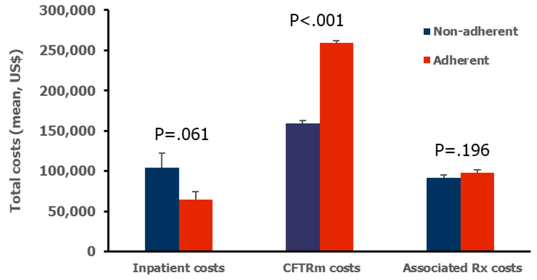
**Table 1. Adherence to CFTRm therapy in patients with cystic fibrosis**

| Medications | *N* | Adherent (n, %) | PDC (Mean ± S.E.) |
|---|---|---|---|
| Kalydeco (ivacaftor) | 132 | 75 (57%) | 76.2% ± 1.9 |
| Orkambi (lumacaftor/ivacaftor) | 112 | 34 (30%) | 59.6% ± 2.5 |
| Symdeko (tezacaftor/ivacaftor and ivacaftor) | 386 | 200 (52%) | 73.8% ± 1.0 |

**Table 2. CFTRm therapy adherence and hospitalization**

| Outcomes | Non-adherent | | Adherent | | Effect |
|---|---|---|---|---|---|
| | Mean | S.E. | Mean | S.E. | P value |
| CFTRm patients (n) | 290 | | 308 | | |
| Hospitalized patients (n) | 109 | | 68 | | |
| % of CFTRm patients hospitalized(%) | 37.59 | | 22.08 | | p < .001 |
| Days stay (d) | 15.7 | 1.9 | 13.3 | 2.5 | p = .434 |
| Comorbidity (CCI scores) | 0.982 | 0.089 | 1.176 | 0.162 | p = .295 |

**Figure 1. CFTRm therapy adherence and medical costs**




## RESULTS CONTINUED

The mean PDC for CF patients was 74.1%. With regard to specific CFTRm therapies, the highest mean adherence was for Kalydeco (*M* = 76.2%), followed by Symdeko (*M* = 73.8%), and Orkambi (*M*= 59.6%) in **Table 1**.

Adherent patients (22.1%) were significantly less likely to be hospitalized during the time period than non-adherent patients (37.6%, $p < .001$). Bed days of hospitalized adherent patients (*M* = 13.3) were also lower than that of non-adherent patients (*M* =15.7); however, the difference was non-significant ($p = .434$). Adherent patients had higher CCI scores than that of non-adherent patients, but the difference was not significant ($p = .295$; **Table 2**).

Total inpatient costs for adherent patients (*M* = $64,736) were lower than non-adherent patients (*M* = $104,085), but there was no significant difference in total inpatient costs between two groups ($p = 0.061$; **Figure 1**).

Finally, CFTRm pharmacy costs (*M* = $259,188) for adherent patients were higher than for non-adherent patients (*M* = $159,440, $p < .001$), but there was no difference between non-adherent (*M* = $91,316) and adherent groups (*M* = $97,832, $p = .196$) for associated medication pharmacy costs in **Figure 1**.

## DISCUSSION / CONCLUSIONS

Given the recency of their availability, this study sought to investigate adherence and health outcomes associated with the new CFTRm therapies. The use of a robust claims database provided an opportunity to examine the association between adherence and hospitalization and medical costs in CF patients with CFTRm therapies. Unfortunately, there were a relatively small number of sampled patients who met study criteria. Future research should utilize a larger sample powered to detect differences in inpatient costs and bed days of hospitalized patients.

The results of this study suggest that adherence to CFTRm therapies may lower hospitalization rates and inpatient costs. Specialty pharmacies should consider implementing clinical patient support programs to aid in improving adherence.

## REFERENCES

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