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Specialty Pharmacy

The impact of elexacaftor/tezacaftor/ivacaftor adherence on hospital outcomes in cystic fibrosis patients

Allison Watson, PharmD, CSP | Sarah Bukhgalter, PharmD, CSP | Misty Rombach, PharmD | Thomas Pouliot

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Background

- Cystic fibrosis (CF) is a chronic, genetic disease associated with substantial healthcare utilization due to its multisystem involvement.
- Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) modulators work at the cellular level targeting the mutation causing CF and are now standard-of-care.
- In October 2019, the FDA approved elexacaftor/tezacaftor/ivacaftor (ETI), a triple-combination modulator targeting the most common CF mutation — yet real-world evidence on the impact of adherence remains limited.
- Understanding the relationship between adherence and hospitalizations may help inform strategies to improve CF outcomes.
- Centene claims data were used to retrospectively evaluate ETI adherence and its impact on hospitalization.

Objective

Does staying adherent to ETI decrease occurrence of hospitalizations in CF patients?

Methods

Retrospective claims analysis of Centene, a major U.S. managed care organization, covering patients with an initial ETI dispense from October 2019 through March 2025.

Adherence tracked for 1 year after ETI initiation using Proportion of Days Covered (PDC); at least two dispenses required for a valid PDC, per pharmacy quality measures.

Patients split into two cohorts: adherent (PDC ≥80%) and non-adherent (PDC <80%).

1-year hospitalization rates were compared by risk difference (RD), relative risk (RR), and 95% confidence intervals (CI), tested by chi-square (statistical significance at $P < 0.05$).

Results

- Of 1,918 patients, 1,241 were adherent (PDC ≥80%) and 677 were non-adherent (PDC <80%). Within one year of starting ETI, 37% of adherent patients were hospitalized versus 44% of non-adherent patients.
- Patients achieving PDC ≥ 80% demonstrated a 7-percentage point lower hospitalization rate (RD: -7%; 95% CI: -11.6% to -2.4%; p<0.001) and a 16% relative reduction in hospitalization risk (RR: 0.84; 95% CI: 0.75–0.94; p<0.001).

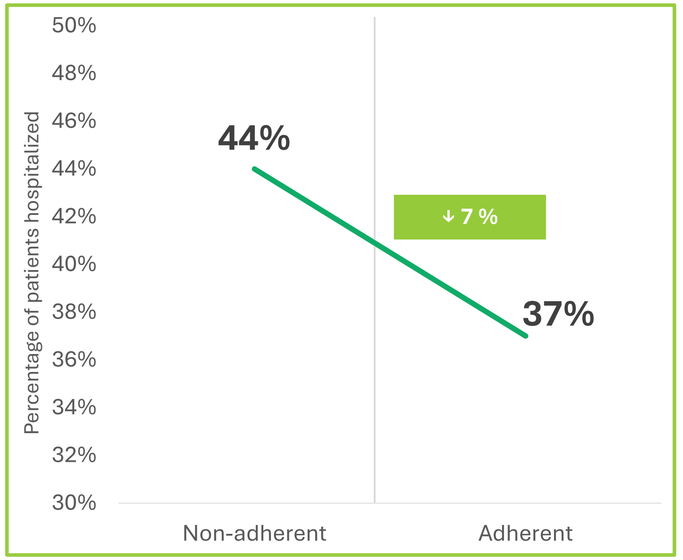
Conclusion

- Adherent patients were hospitalized significantly less often than non-adherent patients in the year after starting ETI.
- Given the high treatment burden and lifelong therapy requirements in CF, maintaining adherence remains a recognized challenge with clinical implications.
- These findings highlight the value of supporting sustained ETI adherence in clinical practice to optimize patient outcomes.

Table 1. Hospitalization Rates by PDC Group

Group	N	Hospitalized Within One Year
PDC < 80%	677	44%
PDC ≥ 80%	1,241	37%

Figure 1. Hospitalization by PDC Group Within One Year



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