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Impact of Lumacaftor/Ivacaftor on Pulmonary Exacerbation Rates in Members with Cystic Fibrosis in a Medicaid Population

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BACKGROUND

Although cystic fibrosis (CF) affects multiple organ systems, throughout the body, pulmonary disease is the leading cause of morbidity and mortality among patients with CF. It has been shown that forced expiratory volume in one-second (FEV1) levels and pulmonary exacerbation (PEx) rate are predictors of survival and thus remain important targets when evaluating the benefit of new CF therapies.1

In randomized trials, lumacaftor/ivacaftor (LUM/IVA) had statistically significant absolute improvements in FEV1, as well as reductions in PEx rates, hospitalizations, and use of intravenous antibiotics2,3.

Two observational studies demonstrated the real-world effectiveness of LUM/IVA in improving pulmonary outcomes; however, higher rates of adverse events and discontinuation rates occurred compared with randomized trials. To our knowledge, there is no published data evaluating real-world outcomes for Medicaid patients receiving this therapy.4,5

OBJECTIVE

To compare CF PEx rates pre- and post-initiation of LUM/IVA in a one-state Medicaid program.

METHODS

This retrospective, observational cohort study utilized pharmacy and medical claims and prior authorizations data from December 1, 2014, through September 30, 2016. Inclusion criteria were members age ≥ 6 years with at least one ER visit or hospitalization related to CF from July 2, 2015 (Food and Drug Administration-approval date) through September 30, 2016.

Inclusion criteria:
- Age ≥ 6 years
- Diagnosis of CF and homozygous for the F508del mutation
- Medication history
- Medication use during the study period

Outcomes

The primary outcome measure was the number of PEx events per member during the study period. PEx was defined as any combination of claims for the following conditions or events:
- CF exacerbation requiring hospitalization or emergency room visit
- CF-related respiratory infection
- CF-related exacerbation

The primary outcome was the annualized rate of PEx per member.

RESULTS

In total, 645 Medicaid members were included in the analysis, of which 457 members (71.1%) were included in the post-LUM/IVA period. The annualized rate of PEx decreased in the post-LUM/IVA period compared to the pre-LUM/IVA period (P=0.41).

LIMITATIONS

- Using claims data to define PEx is not validated.
- Due to its small sample size, this study was not powered to show a difference in the primary endpoint.
- Pharmacy claims are not a true measure of patient adherence to a medication in the outpatient setting, and data was not available to identify inpatient medication administration.
- Clinical parameters, such as pulmonary function data, were not available.

CONCLUSIONS

This claims analysis did not find a statistically significant difference in the rate of PEs between the pre- and post-LUM/IVA periods. Further investigation is warranted to better understand LUM/IVA medication use patterns in this population and impact on disease state.

Our findings support that interventions to improve adherence to CF treatments may represent a strategy for a payer to improve health outcomes for their members.

REFERENCES


DISCLOSURE/SUPPORTING INFORMATION

The authors have no financial disclosures.