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) rates 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) has demonstrated statistically significant absolute improvements in FEV1, as well as reductions in PEx rates, hospitalizations, and use of intravenous antibiotics.2
• 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.3 4

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

METHODS
This retrospective, observational cohort study utilized pharmacy and medical claims and prior authorization data.

Enrollment
• Members of one state’s fee-for-service (FFS) and managed Medicaid plan with ≥ 1 pharmacy claim for LUM/IVA between July 2, 2015 (Food and Drug Administration-approval date) and September 30, 2016.

Inclusion criteria:
• Age ≥ 6 years
• Diagnosis of CF and homozygous for the F508del mutation
• At least one hospitalization or emergency room visit for cystic fibrosis
• No prior LUM/IVA or ivacaftor therapy
• Data was collected six months pre- and post-index date.

RESULTS
Among all members, the annualized rate of PEx decreased in the post-LUM/IVA period compared to the pre-LUM/IVA period. Concomitantly, PEx associated with at least one ER visit or hospitalization decreased during this period.

DISCUSSION
There was no statistically significant difference in the annualized rate of PEx and days of PEx per member in the pre-LUM/IVA period compared to the post-LUM/IVA period.
• Among adherent members, the annualized rate of PEx decreased in the post-LUM/IVA period compared to the pre-LUM/IVA period. Annualized days of PEx per member marginally increased. However, these changes were not considered to be significant.

Type of PEx associated with antibiotic courses (only) increased in the post-LUM/IVA period compared to the pre-LUM/IVA period. Concomitantly, PEx associated with at least one ER visit or hospitalization decreased during this period.

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 important medication administration parameters, such as pulmonary function data, were not available.

CONCLUSIONS
• This claims analysis did not find a statistically significant difference in the rate of PEx after initiation of LUM/IVA in a real-world cohort of CF patients in a Medicaid program, although numerical improvement was observed in a subset of adherent members.
• 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 its members.

REFERENCES

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