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

Mark A. Tesell
University of Massachusetts Medical School

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

**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) led to 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. This retrospective, observational cohort study utilized pharmacy and medical claims and prior authorization data. To compare CF PEx rates pre- and post-initiation of LUM/IVA in one state’s Medicaid program.

**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 hospitalization for PEx during the study period
- Medicaid was secondary payer
- Any break in Medicaid coverage during the study period

**Outcomes**

- The data of the first pharmacy claim for LUM/IVA was defined as the index date.
- Data was collected in 6 months pre- and post-index date.
- Demographic data collected included gender, age, baseline CF medications, and complications of CF.
- The primary outcome was annualized rate of PEx per member pre- and post-index date.
- For members who were on LUM/IVA but not during the study period (either before or after), PEx rates were calculated as any combination of claims for the following events:
  - CF PEx or respiratory infection (CD-10 code related to 1) or an emergency room (ER) visit or (2) outpatient hospitalization or (3) pharmacy claim for an oral or intravenous antibiotic (excluding macrolides).

**RESULTS**

<table>
<thead>
<tr>
<th>Member Demographics</th>
<th>Gender (% female)</th>
<th>Age, in years, at treatment initiation (mean, range)</th>
<th>Number of CF medications at baseline (mean, standard deviation (SD))</th>
<th>Members receiving respiratory medications, excluding respiratory antibiotics (%)</th>
<th>Members receiving respiratory antibiotics (%)</th>
<th>Members receiving gastrointestinal medications (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>N=21</td>
<td>&lt;11*</td>
<td>20.1, 12-51</td>
<td>3.5, 2.1</td>
<td>81.8</td>
<td>59.1</td>
<td>68.2</td>
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**Annualized PEx Rate**

<table>
<thead>
<tr>
<th></th>
<th>All members</th>
<th>Adherent members</th>
<th>Post-LUM/IVA</th>
<th>P=0.69</th>
<th>P=0.41</th>
<th>All members</th>
<th>Adherent members</th>
<th>Post-LUM/IVA</th>
<th>P=0.05</th>
<th>P=0.68</th>
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**Number of type of events corresponding to each PEx**

<table>
<thead>
<tr>
<th></th>
<th>All members</th>
<th>Adherent members</th>
<th>Post-LUM/IVA</th>
<th>P=0.08</th>
<th>P=0.08</th>
<th>All members</th>
<th>Adherent members</th>
<th>Post-LUM/IVA</th>
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**DISCUSSION**

There was no statistically significant difference in the annualized rate of PEs and days of PEs per member in the pre-LUM/IVA period compared to the post-LUM/IVA period.

- Among adherent members, the annualized rate of PEs decreased in the post-LUM/IVA period compared to the pre-LUM/IVA period. Annualized days of PEs per member marginally increased; however, these changes were not considered to be significant.

**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.
- Clinical parameters, as well as pulmonary function data, were not available.

**CONCLUSIONS**

- This study analysis did not find a statistically significant difference in the rate of PEs 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 suggest that interventions to improve adherence to CF treatments may represent a strategy for a payer to improve health outcomes for their members.

**REFERENCES**

5. *Author opinions and views are those of the authors and do not necessarily reflect the views of the Centers for Disease Control and Prevention.*

**DISCLOSURE/ACKNOWLEDGMENTS**

The authors have no financial disclosures.