A Budget Impact Model for Two Investigational Agents for the Treatment of Nonalcoholic Steatohepatitis

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A Budget Impact Model for Two Investigational Agents for the Treatment of Nonalcoholic Steatohepatitis (NASH)

INTRODUCTION
- NASH is a type of nonalcoholic fatty liver disease (NAFLD) that affects approximately 15 million adults in the U.S.1,2
- Although largely asymptomatic, NASH can progress to cirrhosis, liver failure, and cancer, and is projected to become the most common indication for liver transplantation between 2020 and 2025.1,2
- There are no Food and Drug Administration (FDA)-approved therapies for NASH. The American Association for the Study of Liver Diseases (AASLD) recommends pioglitazone and vitamin E as options for select patients.3
- Several agents are currently in development for NASH, of which obeticholic acid and elafibranor are being tested in Phase III registration trials.1,4
- Given limited treatment options for NASH, the clinical interest in using novel therapies may be great once they become available.

METHODS
- A Medline search was conducted (timeframe: Jan. 1, 1995 to Oct. 30, 2017) to identify all published Phase II and Phase III clinical trials of elafibranor and obeticholic acid for NASH.
- Conference abstracts, manufacturer press releases, and value assessments evaluating elafibranor and obeticholic acid for NASH during the same timeframe were also reviewed.
- A clinical and economic assessment was performed to determine the budget impact.

OBJECTIVE
To describe the pharmacy budget impact of elafibranor and obeticholic acid on a sample state Medicaid plan in the first year following their FDA-approval for the treatment of NASH.

RESULTS

Table 1: Evaluation of Elafibranor and Obeticholic Acid

<table>
<thead>
<tr>
<th>Mechanism of action</th>
<th>Elafibranor</th>
<th>Obeticholic acid (OCA)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Key considerations for market uptake</td>
<td>Positive effects on lipids and glucose</td>
<td>FXR ligand</td>
</tr>
<tr>
<td>Resolution of NASH: 19% for OCA vs 12% for placebo (P&lt;0.045)</td>
<td>Phase II FLINT study*</td>
<td></td>
</tr>
<tr>
<td>Key clinical trial</td>
<td>Phase II GOLDEN-505 study*</td>
<td></td>
</tr>
<tr>
<td>Study population</td>
<td>N=274; adults with NASH (NAS≥3) without cirrhosis</td>
<td></td>
</tr>
<tr>
<td>Primary endpoint</td>
<td>Proportion of patients achieving resolution of NASH without worsening of fibrosis</td>
<td></td>
</tr>
<tr>
<td>Intervention</td>
<td>Elafibranor 80 mg or 120 mg orally once daily or placebo for 52 weeks</td>
<td></td>
</tr>
<tr>
<td>Results</td>
<td>Improved liver histology: 45% for OCA vs 21% for placebo (P=0.0002)</td>
<td></td>
</tr>
<tr>
<td>*Randomized, placebo-controlled</td>
<td></td>
<td></td>
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<tr>
<td>†Projected market entry: 2019</td>
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</tbody>
</table>

Economic Assessment
- Estimated annual drug cost: $14,951
- Estimated prevalence of NASH: 3.3% to 6.1%
- An estimated 5–10% of patients who have NASH have been diagnosed.3
- Approximately 350,000–570,000 individuals in the U.S. may have the disease and be diagosed for treatment.2
- NASH is a chronic condition and treatment is continued until progression to cirrhosis or (in those with a liver transplant may be required) or until resolution.

Budget Impact

Medicaid plan of 100,000 covered lives:

<table>
<thead>
<tr>
<th>If low (10%) uptake</th>
<th>If high (25%) uptake</th>
</tr>
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<tr>
<td>$1.3 to $1.9 million per year</td>
<td>$3 to $4.7 million per year</td>
</tr>
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</table>

Figure 1: Projected Pharmacy Budget Impact in Year One

DISCUSSION

NASH is associated with significant morbidity and mortality and if left untreated, may progress to liver transplantation.1

Based on available peer-reviewed literature, elafibranor and obeticholic acid may offer clinical advantages over currently available non-FDA approved therapies.3

Despite the treatment advancements these agents may offer, only ~20% of patients responded to therapy in clinical trials.1,4

The FDA-approval of elafibranor and obeticholic acid for NASH may present opportunities for innovative cost-containment strategies.

- Supplemental rebate: Selection of a preferred NASH agent
- Value-based contracts: Payment contingent on improved clinical outcomes
- Adherence monitoring: Promotion of appropriate medication use

Plan-specific projections may be made by utilizing medical claims data to determine the exact prevalence of NASH within the plan membership.

LIMITATIONS
- Clinical impact was based on Phase II trial data which assessed surrogate endpoints; Phase III trials are ongoing.
- Several assumptions were made in estimating budget impact:
  - Prevalence of disease in the Medicaid plan
  - Number of members diagnosed
  - Number of members who would seek treatment
  - Cost of the agents
  - The current analysis did not utilize medical claims data to determine the prevalence of NASH in the specific population.
  - Uptake of new therapies is difficult to assess due to the many variables that may influence it.

CONCLUSIONS
- New agents for the treatment of NASH are likely to have a significant impact on state Medicaid program budgets.
- The projected budget impact of elafibranor and obeticholic acid highlights the need for innovative cost-containment strategies.
- Proactive pipeline monitoring and high-level budget impact modeling may assist state Medicaid programs in preparing for high-cost specialty medications that are likely to have significant cost implications.

FUTURE STUDIES
Continuous review and adjustment to assumptions made in this budget impact model are necessary as more clinical and economic data become available.

DISCLOSURES/ACKNOWLEDGMENTS
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REFERENCES