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

Nicole M. Trask

University of Massachusetts Medical School

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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
- Although largely asymptomatic, NASH can progress to cirrhosis, liver failure, and liver cancer, and is projected to become the most common indication for liver transplantation between 2020 and 2025.1
- 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.2
- Several agents are currently in development for NASH, of which obeticholic acid and elafibranor are being tested in Phase III registration trials.3,4
- Given limited treatment options for NASH, the clinical interest in using novel therapies may be great once they become available.

METHODS

- A literature 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.3
- 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 clinical 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>Mechanism of action</td>
<td>Dual PPAR-α/δ agonist</td>
<td>FXR ligand</td>
</tr>
<tr>
<td>Key clinical trial</td>
<td>Phase II GOLDEN-505 study*</td>
<td>Phase II FIUENT study*</td>
</tr>
<tr>
<td>Study population</td>
<td>N=274; adults with NASH (NAS≥3) without cirrhosis</td>
<td>N=283; adults with NASH (NAS≥4) without cirrhosis</td>
</tr>
<tr>
<td>Primary endpoint</td>
<td>Proportion of patients achieving resolution of NASH without worsening of fibrosis</td>
<td>Proportion of patients achieving ≥2 point reduction in NAS without worsening of fibrosis</td>
</tr>
<tr>
<td>Intervention</td>
<td>Elafibranor 80 mg or 120 mg orally once daily or placebo for 52 weeks</td>
<td>OCA 25 mg orally once-daily or placebo for 72 weeks</td>
</tr>
</tbody>
</table>

Note: Results may vary depending on the indication and specific population.

Economic Assessment

- Estimated annual drug cost: $74,351
- Estimated prevalence of NASH: 3.3% in U.S.1,5,6
- An estimated 5% of patients who have NASH have been diagnosed.1,5
- Approximately 850,000 individuals in the U.S. may have the diagnosis and be eligible for treatment.1,5
- NASH is a chronic condition and treatment is continued until progression to cirrhosis or (at which time a liver transplant may be required) or until resolution.

Budget Impact

- Medicaid plan of 100,000 covered lives:
  - If low (10%) uptake
    - $1.3 to $1.9 million per year
  - If all approved patients treated
    - $13 to $18.6 million per year

DISCUSSION

- NASH is associated with significant morbidity and mortality and if left untreated, may progress to liver transplantation.7
- The FDA-approval of elafibranor and obeticholic acid for NASH may present opportunities for innovative cost-containment strategies.
- Several assumptions were made in estimating budget impact:
  - Presumed prevalence in the Medicaid plan
  - Number of members diagnosed
  - Number of members that would seek treatment
  - Cost of the agents
  - The current analysis did not utilize medical claims data to determine the prevalence of NASH in a specific population.
- The projected budget impact of elafibranor and obeticholic acid highlights the need for innovative cost-containment strategies.

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 that would seek treatment
  - Cost of the agents
  - The current analysis did not utilize medical claims data to determine the prevalence of NASH in a 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.

REFERENCES


Figure 1: Projected Pharmacy Budget Impact in Year One

- 100,000 Covered Lives
- 3,500-5,000 Patients with NASH
- 175-250 Patients diagnosed may require treatment