PBC Management Among Clinicians: Gaps in Clinical Competence and Practice Performance – Survey Findings

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Introduction

- Primary biliary cirrhosis (PBC) is a chronic, progressive autoimmune cholestatic liver disease leading to the destruction of small interlobular bile ducts, progressive cholestasis, and eventually fibrosis and cirrhosis of the liver.\(^1\)\(^2\)

- Although considered rare, PBC nevertheless affects ~1 in 1000 women older than age 40.\(^3\)

- If left untreated, PBC results in shortened survival, with a median survival in untreated patients of ~9 to 10 years from presentation and the development of liver failure in up to 25%.\(^4\)

- Currently, the standard of care and the only FDA-approved therapy is ursodeoxycholic acid (UDCA).\(^5\) Although UDCA results in improved liver biochemistry and improved transplant-free and overall survival,\(^6\)\(^7\) as many as one third to one half of patients may not respond to the drug, leaving them with few treatment options.\(^8\)

- Diagnosis and treatment of PBC are complicated by the fact that most patients are asymptomatic at presentation;\(^9\) thus, many may go undiagnosed. Since prognosis improves dramatically in patients who receive treatment early in the course of disease,\(^10\) it is critical that physicians be able to detect and diagnose PBC in the absence of overt symptoms.

- An increased understanding of the etiology of PBC has led to the development of new agents targeting unique pathways in the pathophysiology of the disease.

- In light of the significant morbidity and mortality associated with PBC and the pending availability of emerging treatment options, it is even more important that clinicians be adequately trained in screening, diagnosing, treating, and monitoring this disease.

- To assess the competence/practice performance of clinicians involved in diagnosing and treating chronic PBC and to guide future educational programs, Projects In Knowledge, a continuing medical education (CME) provider certified by the Accreditation Council for CME (ACCMED), conducted an online survey of hepatologists and gastroenterologists (GIs) involved in the screening, diagnosis, and treatment of patients with PBC.

Methods

- Projects In Knowledge designed an online survey and emailed it on January 8, 2014, to a proprietary database of 6,000 US hepatologists and GIs involved in the care of patients with PBC.

- Clinicians surveyed were asked to self-assess whether they were highly, somewhat, or not at all competent with regard to specific topics, such as PBC risk factors/screening, criteria for response to treatment; mechanisms of action (MOAs), efficacy, and safety of current and emerging treatments; management of treatment-related side effects; and the link between cirrhosis, need for liver transplantation, and mortality.

- Using a four-point scale ranging from always to never, they were also asked about the degree to which they perform interventions in their practices (practice performance).

Results

- Survey response was capped for the purpose of this poster presentation analysis at 100 per specialty, for a total of 200 responses.

- Since hepatologists and GIs are the physicians who are presumably the most knowledgeable about the treatment of PBC, their responses to questions on competence and practice performance were considered particularly important in determining the current status of PBC treatment and in identifying gaps that should be addressed.
1. Risk Factors and Screening At-Risk Patients
   - Although 100% of the hepatologists and 59% of the GIs surveyed stated that they always/often screen for PBC in at-risk patients, much lower percentages felt confident in their ability to do so, with only 72% and 46%, respectively, rating themselves as highly competent in discussing these risk factors and in screening at-risk patients.

2. Importance of Early Diagnosis/Treatment
   - 80% of hepatologists and 65% of GIs felt highly competent regarding the importance of early diagnosis and treatment, and a large percentage (88% and 87%, respectively) stated that they always/often implement methods for diagnosing PBC after symptoms appear in at-risk patients.
   - 76% of hepatologists and 65% of GIs initiated PBC treatment or enrolled patients in a trial as soon as PBC was diagnosed.

3. Criteria for Response to Treatment in Patients with PBC
   - Despite the fact that 76% of hepatologists and 42% of GIs state that they always/often utilize criteria for response to PBC treatment (e.g., Paris criteria, Barcelona criteria) when monitoring patients on PBC treatment, much lower percentages (36% and 30%, respectively) felt highly competent regarding these criteria.
Effect of Untreated or Suboptimally Treated PBC on Disease Progression/Mortality

- A greater percentage of hepatologists than GIs felt highly competent regarding the effect of untreated or suboptimally treated PBC on disease progression/mortality (74% and 55%, respectively), but fewer physicians in each specialty stated they were highly competent in terms of the potential causes of suboptimal treatment (64% and 38%, respectively).

Competence Regarding MOA, Efficacy, and Safety of Current and Emerging PBC Treatments

- Despite the fact that URSO is the only FDA-approved drug for PBC, only two thirds of hepatologists and just over half of GIs felt highly competent in terms of the MOA, efficacy, and safety of this treatment.

- Thus, it is not surprising that much smaller percentages of both hepatologists and GIs stated they were highly competent in terms of the MOAs, efficacy, and safety of various emerging therapies.

Treatment with FDA-Approved Therapy or Enrollment Into PBC Clinical Trials

- As might be expected, both hepatologists and GIs were most likely to treat their patients with URSO, the only FDA-approved drug for PBC, than to enter them into clinical trials with other therapies.
7 Monitoring Patients for Treatment Response, Side Effects, and Adherence

- There was a substantial disconnect between the percentages of hepatologists and GIs who always/often monitor their patients for treatment response, side effects, and adherence (83% and 69%, respectively), and the percentages that felt highly competent to do this (68% and 35%, respectively).

- 84% of hepatologists and 76% of GIs always/often educated their patients on the link between nonadherence and disease progression/mortality.

8 Implementing Treatment-Related Side Effect Strategies

- Although 80% of hepatologists and 61% of GIs stated that they always/often implement treatment-related side effect strategies, how competent they felt in doing this varied substantially depending on the treatment in question.

- Not surprisingly, clinicians were more likely to rate themselves as highly competent in managing side effects of the FDA-approved therapy, URSO, than the adverse events associated with emerging treatments.
  - Only two thirds of hepatologists and less than one half of GIs felt highly competent in managing URSO-related side effects.

9 Cirrhosis, Liver Transplant, and Mortality

- High percentages of both hepatologists (88%) and GIs (86%) always/often monitor cirrhosis and change in disease status.

- Although a similarly high percentage of hepatologists (84%) rated themselves as highly competent regarding the link between cirrhosis, need for liver transplant, and mortality, only 53% of GIs felt they were highly competent in this regard.
Challenges/Barriers in Treating PBC Patients

- Both hepatologists and GIs cited challenges in
  - Keeping up with current and emerging treatments for PBC.
  - Tips for improving patient adherence.
- GIs cited difficulties in
  - Screening and diagnosing PBC.
  - When to treat PBC.
  - Treatment-related side effect management.

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<tr>
<th>Challenges in Managing and Treating PBC Patients</th>
<th>HEP</th>
<th>GIs</th>
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<tr>
<td>Lack of knowledge about risk factors, screening, and diagnosis of PBC</td>
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</table>
| Early-Stage PBC
  - Awareness/ability to diagnosis | ✔ |
  - When best to treat | ✔ |
| Adherence
  - Convincing asymptomatic PBC patients to take meds/pay for meds/remain adherent | ✔ | ✔ |
  - Lack of patient understanding about the consequences of nonadherence | ✔ | ✔ |
| Lack of knowledge about current and emerging treatments for PBC | ✔ | ✔ |
| Treatment-related side effect management | ✔ |

Need for PBC-Related Educational Programs

- Consistent with the gaps identified in their self-assessment of competence, both hepatologists and GIs stated a need for further education regarding current and emerging therapies for PBC.
- GIs also cited a need for information on risk factors and screening for PBC.

Conclusions

- Both hepatologists and GIs demonstrated a substantial disconnect between clinical competence and practice performance, in a range of areas in the treatment of PBC—including screening, diagnosis, assessing response, efficacy/safety of current/emerging treatments, treating/entering patients into clinical trials, and monitoring patients.
- Given that physicians in these two specialties are presumably the most competent in the care of patients with PBC, these gaps have important implications for the quality of patient care and indicate the need for further education.
- Despite the limitations of clinician self-assessment surveys, they have proven helpful in determining gaps in competence/practice performance, such as those documented in this study, and can provide a useful guide for the development of further educational programs.

References