Transforming Rare Disease Care Through Data-driven, Patient-centered Medical Education: Outcomes from a Multimodal Educational Initiative in Primary Biliary Cholangitis

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Introduction

Despite recent therapeutic advances, significant gaps remain in recognizing primary biliary cholangitis (PBC), assessing disease burden (particularly pruritus), and selecting evidence-based treatment. To address these needs, a continuing medical education (CME) initiative incorporated healthcare provider (HCP) practice-pattern data and patient perspectives to emphasize earlier diagnosis, patient-centered symptom assessment, and practical integration of emerging therapies into clinical care.

Methodology

Educational interventions

- Two 45-minute CME activities for gastroenterologists, hepatologists, primary care clinicians, and advanced practice providers
- Targeted 1- to 4-minute microlearning videos distributed via social media to NPI-verified gastroenterologists
- Practice-pattern survey of 50 gastroenterologists and hepatologists to inform the content
- Patient/caregiver (P/C) educational program developed in collaboration with National Organization for Rare Disorders (NORD) and American Liver Foundation (ALF) to collect insights on the patient journey and experiences with care

Measurements

- HCPs: pre-/post-test comparisons of knowledge (MCQ) and competence (Likert scale), intent to change practice, and perceived barriers to implementing change
- P/Cs: self-reported changes and ratings on a 5-point scale
- Chi square for pre vs post comparison

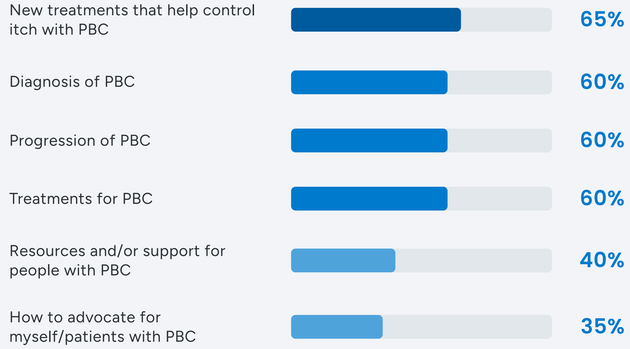
Results

P/Cs Journey and Experience with Care

553
Education views

Gender
60% Female 40% Male

Reasons for participating (to learn more about…) (select all that apply)



Patients/caregivers

Likely to discuss new PBC treatment options with their HCP

Satisfied with current PBC treatment

Satisfied with current PBC treatment in addressing the itch

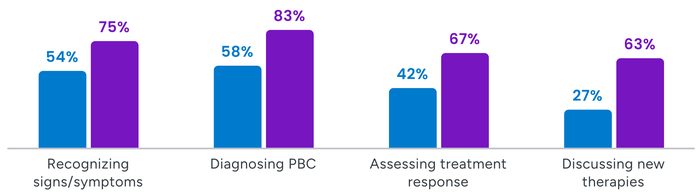
There was high satisfaction with treatment in general, but lower satisfaction with how itch is managed. This may suggest that patients have reached a status quo in expectations of treatment and are looking for additional options.

Practice-Pattern Survey

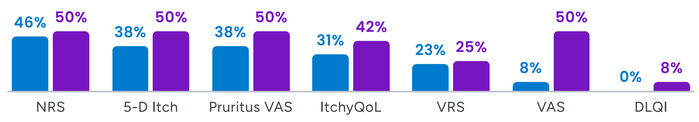
Gastroenterologists trail hepatologists by 15–36 points on every metric.

Gastroenterologists (n=26) Hepatologists (n=24)

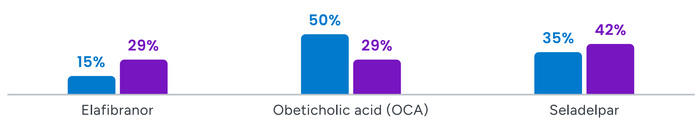
HCPs confidence by specialty



Itch severity assessment tool use



Management of PBC with inadequate response to UDCA, ursodiol



Insights from practice-pattern survey: Compared to gastroenterologists, hepatologists report considerably greater confidence recognizing signs and symptoms of PBC, making a PBC diagnosis, assessing response to treatment, and discussing new therapies. Hepatologists are more likely to prescribe seladelpar or elafibranor for patients who have not responded to UDCA or ursodiol, as gastroenterologists tend toward prescribing obeticholic acid.

HCPs Educational Insights

Demographics

- 4,764 total participants (1,111 CME enduring + 3,653 microlearning)
- Degree: 88% MDs/DOs; 6% NPs/PAs; 3% RNs; 3% other
- Specialty: 84% gastroenterology; 7% primary care; 9% other
- 58% identified as current treaters who see an average of 6 patients with PBC each month

Believe their patients are satisfied with their PBC treatment
n=232

Confident recognizing PBC
n=173

Confident managing pruritus in patients with PBC
n=173

Knowledge: pre- vs post-test

First attempt, post-test data

Pre-test (n=233) Post-test (n=181)

		Pre	Post
Janice is a 35-year-old patient recently diagnosed with PBC based on elevated liver enzymes, serum anti-mitochondrial antibodies (anti-AMA), and characteristic findings on liver biopsy. Which autoantibody in her blood would further confirm the diagnosis and indicate a worse prognosis of PBC (including faster progression to hepatic insufficiency and higher mortality)?	Anti-gp210	37%	58%
When severe, which of the following can be considered a justifiable indication for liver transplantation in patients with PBC?	Pruritus	27%	60%
Which treatment for PBC works by binding selectively to peroxisome proliferator–activated receptor gamma (PPAR-δ)?	Seladelpar	25%	49%
In clinical trials of agents to treat PBC, what alkaline phosphatase (ALP) level is considered a surrogate endpoint for assessing response to treatment?	ALP <1.67 times ULN	43%	60%

Pre-test (n=130) Post-test (n=95)

		Pre	Post
Which of the following findings on liver biopsy is considered pathognomonic for primary biliary cholangitis (PBC)?	Florid duct lesion	47%	96%
Which of the following is associated with pruritus in PBC?	Early stage disease	13%	62%
Diana is a 32-year-old woman who was diagnosed with PBC 1 year ago. She started ursodeoxycholic acid (UDCA) at a dose of 6.9 mg/kg/d, with an increase after 6 months to 13.9 mg/kg/d to further reduce ALP. She is experiencing pruritus (5/10 on the itch numeric rating scale [NRS]), and her ALP is 325 IU/L. What would be the next best step in treatment?	Add seladelpar	32%	63%

79% Overall, 79% of HCPs reported their intent to make changes in patient-management practices after participation, including in the following areas:

Theme	% of Total
Treatment selection and management	28%
Diagnosis, recognition, and screening	26%
General awareness/knowledge gained	19%
Pruritus-specific symptom management	10%
Referral and specialist collaboration	8%
Patient education and team communication	5%
Monitoring and follow-up testing	4%

Conclusion

A data-driven educational strategy that included serial CME activities, targeted microlearning, and integrated clinician practice-pattern data and patient perspectives successfully improved clinician knowledge and competence and was associated with intended practice changes related to PBC management. Importantly, the practice-pattern survey identified specialty-specific educational gaps that enabled the content to be tailored to target audiences. The survey also highlighted the need for tailored education, such as practical support and updates for gastroenterologists and more advanced, innovation-focused content for hepatologists. Overall, this model demonstrates how combining real-world clinician data with patient-centered educational designs can advance rare-disease education and accelerate dissemination of evolving evidence into clinical practice.

Supporter statement

This activity was supported by an independent educational grant from Gilead Sciences, Inc.