COMMUNITY LIVER ALLIANCE ™

GLOBAL AWARENESS. LOCAL IMPACT.

PRIMARY BILIARY CHOLANGITIS (PBC)

PROVIDER TOOLKIT

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Welcome

The purpose of the CLA's Primary Biliary Cholangitis (PBC) Toolkit is to empower patients, caregivers, and healthcare professionals by providing comprehensive, accessible, and up-to-date information on PBC.

This guide aims to educate readers about the disease, treatment options, and possible side effects, while also offering practical advice on navigating the healthcare system, managing the emotional and financial impacts of a diagnosis, and enhancing quality of life through supportive resources and self-care strategies.

Our goal is to assist individuals affected by PBC in making informed decisions about their care and to support them in their journey toward recovery and well-being.

How to Use This Toolkit

This toolkit is available for download to provide you with instant access to valuable information. Additionally, hard copies are available upon request. Feel free to share this resource with family, friends, and healthcare providers to ensure everyone involved in your care is informed and supported.

We hope this toolkit serves as a helpful guide throughout your PBC journey. Remember, you are not alone, and there are many resources and support systems available to help you manage and overcome the challenges you may face. For further assistance, do not hesitate to reach out to our team or visit our website for additional information and support.

PBC Toolkit

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Understanding PBC

Overview

Primary Biliary Cholangitis (PBC) is a chronic, progressive autoimmune cholestatic liver disease. It primarily targets small and medium-sized intrahepatic bile ducts, leading to impaired bile flow, cholestasis, and, over time, fibrosis and cirrhosis.

Etiology and Pathophysiology

- Autoimmune basis: PBC occurs when the immune system mistakenly attacks the epithelial cells lining the small bile ducts.
- Key features:
  - Lymphocytic infiltrates around bile ducts
  - Progressive destruction of ducts
  - Resultant cholestasis and bile acid retention
  - Chronic inflammation and fibrosis that can progress to cirrhosis
- Autoantibodies: The hallmark is anti-mitochondrial antibodies (AMA), present in approximately 90–95% of cases. AMA targets components of the mitochondrial pyruvate dehydrogenase complex.

Epidemiology

- Strong female predominance (approximately 9:1)
- Most commonly diagnosed between ages 30 and 60
- Frequently associated with other autoimmune diseases, such as:
  - Sjögren's syndrome
  - Autoimmune thyroid disease
  - Rheumatoid arthritis
  - Systemic sclerosis

Early Disease

- Often asymptomatic or minimally symptomatic
- Frequently detected via abnormal liver tests, especially elevated alkaline phosphatase (ALP)

Symptomatic Disease

- Fatigue (often severe and out of proportion to findings)
- Pruritus (itching), often worse at night
- Sicca symptoms (dry eyes, dry mouth)
- Mild right upper quadrant discomfort

Advanced Disease and Complications

- Cirrhosis and complications of portal hypertension
- Hepatic decompensation (ascites, encephalopathy, variceal bleeding)
- Osteoporosis and osteopenia
- Fat-soluble vitamin deficiencies
- Increased risk of hepatocellular carcinoma (especially in advanced disease)

Natural History and Impact of Treatment

Before disease-modifying therapy, many patients progressed to cirrhosis and liver failure. Ursodeoxycholic acid (UDCA) has significantly changed the natural history of PBC by improving transplant-free survival in responders and slowing or halting progression in many patients.

Recognizing and Diagnosing PBC in Community and Primary Care Settings

Common Presentations

Asymptomatic with Abnormal Labs

- Incidentally discovered elevation in ALP (± GGT)
- Often mild or no elevation in AST/ALT

Symptomatic but Non-Specific

- Fatigue (often described as "bone-deep" tiredness)
- Pruritus, especially at night
- Sicca symptoms (dry eyes and mouth)
- Mild right upper quadrant discomfort

Advanced Disease Signs

- Jaundice
- Ascites, peripheral edema
- Easy bruising, muscle wasting
- Variceal bleeding or other decompensating features

Key Diagnostic Elements

Laboratory Evaluation

- Cholestatic pattern:
  - Elevated ALP is the central finding
  - GGT typically elevated
- Autoantibodies:
  - Anti-mitochondrial antibody (AMA) – hallmark of PBC
  - In AMA-negative cases, PBC-specific ANA (e.g., anti-sp100, anti-gp210) may be useful
- Additional labs:
  - AST, ALT, total and direct bilirubin
  - Albumin, INR
  - CBC, basic metabolic panel
  - Viral hepatitis panel

Imaging

- Abdominal ultrasound:
  - First-line to rule out extrahepatic biliary obstruction (stones, mass, strictures)
  - Evaluate liver texture and signs of portal hypertension
- MRCP or CT:
  - Consider when there is suspicion for primary sclerosing cholangitis (PSC), malignancy, or other structural disease

Liver Biopsy

- Not always required, but consider biopsy when:
  - AMA-negative cholestatic pattern
  - Suspicion of overlap (e.g., PBC–autoimmune hepatitis)
  - Staging or diagnostic uncertainty

Simplified Diagnostic Criteria

PBC is typically diagnosed when two of the following three are present:

1. Biochemical evidence of cholestasis (ALP elevation)
2. Presence of AMA (or PBC-specific ANA)
3. Liver histology consistent with nonsuppurative destructive cholangitis and destruction of interlobular bile ducts

Management and Monitoring: Treatment, Follow-Up, and Safety

First-Line Therapy: Ursodeoxycholic Acid (UDCA)

- Standard of care for all patients with PBC, regardless of symptoms
- Dose: 13–15 mg/kg/day (often divided twice daily)
- Benefits:
  - Improves cholestasis
  - Delays histologic progression
  - Improves transplant-free survival in responders

Monitoring Response

- Assess biochemical response at 12 months after initiation (and periodically before):
  - ALP and bilirubin trends are key markers
  - Normalization or significant reduction in ALP and stabilization of bilirubin are favorable
- Suboptimal biochemical response (persistent ALP elevation, rising bilirubin) indicates higher risk and should prompt hepatology involvement for treatment escalation.

Second-Line and Adjunctive Therapies

Obeticholic Acid (OCA)

- Approved for patients with inadequate response to UDCA or intolerance to UDCA
- Typically initiated and monitored by hepatology
- Requires caution or avoidance in patients with advanced cirrhosis or decompensated disease

Other / Emerging Agents

- Fibrates and other investigational therapies may be considered in select cases under specialist guidance.

Symptom Management

Pruritus

- First-line: bile acid sequestrants (e.g., cholestyramine)
- Second-line/adjunctive (often specialist-guided): rifampin, naltrexone, sertraline

Fatigue

- Rule out other causes: anemia, thyroid disease, sleep disorders, depression
- Encourage gentle, regular physical activity and address co-morbid contributors

Bone Health

- High risk of osteoporosis and fractures
- Baseline and periodic DEXA scans
- Ensure adequate vitamin D and calcium intake
- Consider bisphosphonates when indicated

Follow-Up and Safety

- Liver function tests: every 3–6 months
- Ultrasound and HCC surveillance: in patients with cirrhosis, per guidelines
- Variceal screening: via upper endoscopy as recommended for cirrhotic patients

Monitor for:

- Decompensation: ascites, hepatic encephalopathy, variceal bleeding, worsening jaundice
- Medication-specific side effects, particularly with second-line agents

Supporting the Patient: Education, Quality of Life, and Self-Management

Key Education Points

- PBC is a chronic but manageable autoimmune liver disease.
- PBC is not caused by alcohol or lifestyle choices; this can help reduce stigma and guilt.
- UDCA is a long-term medication that helps protect the liver, even if the patient feels well.
- Regular follow-up visits, labs, and imaging are essential for monitoring and early detection of progression or complications.

Lifestyle and Self-Management

Diet

- Encourage a balanced, heart-healthy diet.
- Recommend avoiding heavy alcohol use; ideally, patients with PBC should abstain.
- Consider smaller, more frequent meals in the setting of fatigue or nausea.

Physical Activity

- Gentle, regular exercise can improve fatigue, mood, bone health, and overall function.
- Tailor recommendations to the patient's capacity; advise against over-exertion.

Sleep and Energy Management

- Discuss pacing strategies: alternating activity with rest periods.
- Consider screening for sleep disorders when fatigue is severe or persistent.

Mental Health and Coping

- Normalize emotional responses to chronic illness.
- Screen for depression and anxiety.
- Offer referrals to counseling or mental health services when appropriate.

Practical Tools

Encourage patients to use tools such as:

- Medication trackers to support adherence
- Symptom diaries to monitor fatigue, itch, mood, sleep, and triggers
- Emergency contact lists
- Educational materials and links to trusted liver health organizations

Coordinating Care: Multidisciplinary Collaboration and Referral Pathways

Core Roles in Care

Primary Care Providers

- Identify abnormal liver tests and potential PBC symptoms
- Initiate basic diagnostic workup and timely referral
- Manage co-morbid conditions (cardiovascular risk, diabetes, mental health)
- Support immunizations and general preventive care

Gastroenterology / Hepatology

- Confirm diagnosis and assess disease stage
- Guide initiation, adjustment, and monitoring of disease-specific therapy
- Monitor for progression and complications
- Evaluate for transplantation when needed

Other Specialists and Services

- Rheumatology for associated autoimmune conditions
- Endocrinology for thyroid disease or metabolic bone disease
- Nutrition for dietary counseling
- Physical therapy for mobility and conditioning
- Mental health providers for psychological support
- Social work / case management for insurance, disability, and resource navigation

Communication and Information Sharing

- Provide clear, concise referral notes that include:
  - Key lab trends (ALP, bilirubin, AST/ALT)
  - Autoantibody results (especially AMA)
  - Imaging findings
  - Current medications and doses
  - Symptoms and functional status
- Request explicit guidance on:
  - Follow-up intervals
  - Monitoring parameters between specialist visits
  - Criteria for re-referral or urgent evaluation

Navigating Systems and Barriers

- Recognize that UDCA and OCA may require prior authorization.
- Support patients with:
  - Documentation for patient assistance programs
  - Letters of medical necessity
  - Referrals to financial counselors or liver advocacy organizations when needed

Research and Clinical Trials: What Providers Should Know

Why Clinical Trials Matter in PBC

- A subset of patients do not respond adequately to current therapies.
- New therapies aim to:
  - Improve bile acid metabolism
  - Reduce inflammation and fibrosis
  - Improve symptoms and quality of life

Talking With Patients About Trials

- Normalize clinical research as part of advancing knowledge and care.
- Clarify that:
  - Participation is voluntary and requires informed consent.
  - Trials provide potential access to new therapies, but also have risks and unknowns.
  - Patients can withdraw at any time without losing standard care.

Where to Find Trial Information

- ClinicalTrials.gov – search "Primary Biliary Cholangitis" and filter for "Recruiting" or "Active, not recruiting."
- Regional academic medical centers and liver specialty clinics.
- PBC-focused organizations and liver foundations, which may list active or upcoming studies.

Provider Role in Research

- Identify patients who may be candidates for trials (e.g., partial/non-responders to UDCA).
- Encourage conversations between patients and their hepatology teams regarding research opportunities.
- Support follow-up and basic monitoring if a patient participates in a trial.

Continuing Education and Key Resources for Providers

Clinical Guidelines

- AASLD (American Association for the Study of Liver Diseases) Guidelines for PBC
- EASL (European Association for the Study of the Liver) Guidelines

These provide evidence-based recommendations on:

- Diagnosis and staging
- Treatment thresholds and algorithms
- Surveillance for complications
- Criteria for transplant referral

Professional Education Opportunities

- CME-accredited modules on autoimmune liver diseases
- Liver-focused conferences and symposia (national and regional)
- Webinars, case conferences, and grand rounds from liver centers and advocacy groups

Key Organizations and Resources

- Community Liver Alliance – education, support, regional programs, and patient materials
- PBC Foundation – international PBC-specific information and support
- Professional societies (AASLD, EASL) – guidelines, updates, educational resources

Quick Access Tools

Keep convenient access to:

- A one-page PBC care algorithm (screening → diagnosis → first-line therapy → referral triggers)
- A plain-language patient handout on PBC
- Links or QR codes to trusted educational sites for patient use
- Local hepatology referral contacts or clinic directories

Summary Insights for Providers

Section 1

- PBC is not rare in everyday practice. Any adult with persistent cholestatic liver tests deserves evaluation.
- Early treatment matters. Starting UDCA early improves outcomes and slows disease progression.
- PBC is not alcohol-related liver disease. Clarifying this with patients reduces stigma and improves engagement.
- Think of PBC as part of an autoimmune cluster. If you diagnose PBC, consider screening for other autoimmune conditions, and vice versa.

Section 2

- Any persistent ALP elevation warrants a clear explanation. Avoid dismissing it as "mild" without evaluation.
- A positive AMA in a patient with cholestasis is usually PBC until proven otherwise.
- Imaging is primarily to rule out obstruction and other structural disease, not to confirm PBC.
- In AMA-negative cholestatic patients, consider other cholangiopathies but remember that PBC can still be confirmed with PBC-specific ANA and/or biopsy.
- Early referral to hepatology is appropriate when there is diagnostic uncertainty or suspicion of overlap syndromes.

Section 3

- Start UDCA early and ensure correct weight-based dosing. Underdosing diminishes benefit.
- Biochemical response to UDCA is prognostic; track ALP and bilirubin trends.
- Ask about itching and fatigue at every visit. These symptoms often go under-reported but significantly impact quality of life.
- Involve hepatology early for non-responders, advanced disease, or when considering OCA or other advanced therapies.
- Always incorporate bone health into long-term management of PBC.

Section 4

- Explicitly tell patients: "This is not your fault." It can meaningfully reduce shame and distress.
- Patients may be overwhelmed at diagnosis; repetition of key messages is often necessary.
- Quality of life and symptom burden may not correlate with lab values—ask about daily function.
- Encourage support groups and peer networks, which can reduce isolation and increase engagement.

Section 5

- PBC management is best approached as shared care, not isolated care.
- A focused, structured referral note facilitates efficient specialist evaluation and decision-making.
- Ask for and document clear follow-up plans from hepatology so roles are defined.
- Remember to address non-hepatic issues: cardiovascular risk, bone health, mental health, and overlapping autoimmune conditions.

Summary Insights for Providers

Section 6

- You do not need to run trials yourself to meaningfully support research—simply knowing where to direct interested patients is valuable.
- Consider clinical trials especially for patients with suboptimal response or advanced but compensated disease who still meet eligibility.
- Framing research positively—"New therapies are coming, and you may have opportunities to access them"—can provide hope while remaining honest.

Section 7

- You don't need to memorize every detail; knowing where to find trusted guidance quickly is more important.
- Proactively sharing reliable patient resources helps combat misinformation and supports patient engagement.
- Create a small "PBC mini-toolkit" in your EMR or desktop with:

1. Local hepatology contact information
2. Guideline links
3. Standard lab panels and follow-up intervals
4. Printable patient education materials

Tips for Family and Caregivers

Caring for someone with PBC is an act of love and commitment. As a caregiver or family member, your support plays a vital role in your loved one's health and quality of life.

Emotional Support

- Be patient and understanding: Living with a chronic illness can bring fatigue, frustration, or emotional ups and downs. Offer empathy, listen without judgment, and encourage open communication.
- Celebrate small victories: Recognize progress and positive moments — even small steps forward matter.
- Stay informed: Learn about PBC so you can better understand symptoms, treatments, and what your loved one may be experiencing.

Practical Help

- Assist with daily tasks: Help with chores, meal prep, or errands when your loved one is feeling tired.
- Transportation: Offer rides to medical appointments, support groups, or lab visits — this can ease stress and ensure consistent care.
- Organization: Help track medications, appointments, and test results using a Medication Tracker or Symptom Diary from the PBC Practical Tools Toolkit.
- Advocate together: Encourage your loved one to ask questions and express their needs during appointments — or help take notes if needed.

Managing Caregiver Stress

Taking care of yourself is not selfish — it's essential.

Self-Care Strategies

- Prioritize your own health: Eat well, exercise, and make time for rest. You can't pour from an empty cup.
- Set realistic limits: You don't have to do everything. Ask for help and delegate when possible.
- Take breaks: Short moments of downtime — even 10 minutes of quiet — can help reset your energy.
- Stay connected: Keep in touch with friends, hobbies, and interests that bring you joy.

Support for Caregivers

- Join caregiver support groups: Sharing experiences with others who understand can provide relief, advice, and encouragement.
  - Family Caregiver Alliance – Education, support, and advocacy.
  - Well Spouse Association – Peer support for spouses and partners.
- Seek professional help: Talking to a counselor or therapist who understands chronic illness can help manage stress and prevent burnout.

Remember: You're not alone — support is available for caregivers, too.

Support Information

The suggestions below may be a useful prompt when people say, "Let me know if you need anything."

Although family and friends are often eager to help, many people struggle to request and accept support. During treatment and while managing side effects, having a supportive network can be incredibly beneficial.

Family and friends generally welcome the opportunity to offer support, as it makes them feel helpful.

However, not everyone has family or friends who can provide the needed assistance. In such cases, numerous professional support resources are available to help.

People are often willing to help if they know what you need. Family and friends can support you in different ways. Some people will be able to talk about the diagnosis and comfort you if you are upset.

Other people may prefer to offer practical support. If you have a partner or another person providing most of your care, an important role for other family members and friends may be to support that caregiver.

Offering Practical Support

- Preparing meals
- Performing household chores
- Running errands and grocery shopping
- Driving to appointments
- Managing after-school schedules
- Assisting with exercise routines

Facilitating Communication

- Screening calls and emails
- Serving as the primary contact for family and friends
- Coordinating incoming support offers
- Managing updates on social media

Providing Companionship

- Spending time with you
- Listening empathetically without offering solutions
- Helping you stay active and social
- Engaging in conversations about topics other than cancer

Some people like to use an app on their smartphone or computer, such as Liver Education Advocates or Caringbridge. These resources allow you to choose activities that match your needs.

Advocacy and Involvement

Get Involved
Your voice and actions can help raise awareness, support research, and empower others living with PBC.

Advocacy Efforts:

- Join national or local awareness campaigns such as Delivering Hope or PBC Day.
- Share educational posts on social media to spread awareness about liver health.
- Participate in fundraising events that support research, patient education, and advocacy.

Volunteering:

- Partner with organizations focused on PBC and liver wellness:
  - Community Liver Alliance
  - PBC Foundation
- Volunteer to help with community outreach, patient education, or event coordination.

Amplify your impact:

- Encourage your workplace or community to host a liver health awareness event.
- Share your story — personal experiences inspire others and highlight the importance of research and early diagnosis.

PBC research is moving forward every day — bringing new hope, improved treatments, and better understanding of this complex disease. By staying informed, advocating for awareness, and getting involved, you help shape a brighter future for everyone affected by PBC.

Additional Resources

https://www.hepmag.com/basics/liver-health/caregivers

Practical Tools

Living well with PBC involves more than medical appointments — it's about staying organized, tracking changes, and having key information at your fingertips.

These tools can help you take control of your care and make communication with your healthcare team easier.

Staying organized is one of the most powerful ways to take charge of your health. By using tools like medication trackers, symptom diaries, and emergency contact lists, you can better understand your condition, communicate effectively with your care team, and feel more confident in your day-to-day life with PBC.

Medication Tracker - You can use a paper chart, a phone app, or a printed checklist — whichever makes it easiest for you to stay consistent.

What to Include:

- Medication name and dose
- Time and frequency (morning, evening, with meals, etc.)
- Start and refill dates
- Notes for side effects or reminders

A Symptom Diary - Bring your diary to appointments to give your doctor valuable insight into how your treatment is working.

Track daily:

- Quality of life (energy, activity, overall mood)
- Itching (pruritus) intensity and when it occurs
- Fatigue – note when you feel most tired
- Sleep quality and hours of rest
- Dietary or lifestyle changes – new foods, supplements, or activities
- New or changing symptoms – note anything unusual

Emergency Contacts - Keep a printed list in your wallet, phone, or near your medications.

Include contact details for:

- Family and Caregivers – people who can help in an emergency
- Healthcare Providers – hepatologist, primary care physician, and pharmacist
- Local and Online Support Groups – for guidance and encouragement
- Emergency Services – local emergency number (911 in the U.S.)
- Poison Control: 1-800-222-1222 (U.S.)

Tip: Save "ICE" (In Case of Emergency) contacts in your phone — paramedics and hospitals often look for these first.

Daily Journal

DATE

MOOD

YOUR THOUGHTS

GOALS

TO DO

Clinical Trials

Clinical trials serve as the cornerstone for validating the effectiveness and safety of emerging treatments in securing regulatory approval. However, it has become increasingly evident that the underrepresentation of diverse patient populations in research studies has significant implications for health outcomes.

Advancing Health Equity with Clinical Trial Diversity

Clinical trials are currently the gold standard for proving the efficacy and safety of new treatments for regulatory approval. Data has revealed that underrepresented patient populations in research have impacted health outcomes.

Goals of the Clinical Trials Page

- To aid individuals in making informed decisions regarding participation in clinical trials.
- To educate the provider community about clinical guidance for their patients.
- To provide access to credible and up-to-date clinical trial opportunities.
- To promote links to member partners clinical trial opportunities.
- To promote health equity with clinical trial diversity.

Clinical trial diversity is a critical component of public health.

Whether researchers submit a new drug to the FDA or providers use data for medical decision-making, data collection from a participant population that reflects the entire patient population is very important.

The CLA Clinical Trial website page hosts an informational video developed to educate patients and providers about the basic purpose and process of clinical trials.

The Community Liver Alliance strives to unite patients, providers and the health care industry.

We influence the best possible care practices and develop new, scalable solutions for the fight against liver disease.

Additionally, downloadable Clinical Trials Educational Guides geared towards patients and a separate guide for providers as another resource.

For More Information on Clinical Trials Check Out

www.clinicaltrials.gov
https://communityliveralliance.org/clinical-trials/
https://communityliveralliance.org/wp-content/uploads/2023/06/Clinical-Guidelines-Patient.pdf
https://communityliveralliance.org/wp-content/uploads/2023/06/Clinical-Guidelines-Provider.pdf
https://communityliveralliance.org/wp-content/uploads/2023/09/Clinical-Trials-Spanish.pdf

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