WHEN TREATMENT IS NO LONGER AVAILABLE

Frequently Asked Questions About Current Changes in PBC Treatment

Disclaimer: This information is provided for general educational purposes only and is not intended as medical advice. Always consult your physician or qualified healthcare provider regarding your specific medical condition, treatment options, and any questions you may have.

Medications for rare liver diseases can sometimes become unavailable due to safety updates, manufacturing changes, or new treatment options. While this can feel uncertain, understanding these changes is critical so you can make informed decisions about your care and work closely with you healthcare team.

This resource uses the recent withdrawal of Ocaliva® (obeticholic acid) for primary biliary cholangitis (PBC) as an example to answer your questions, guide you on next steps, and help you feel supported and confident about your treatment options.

Why do I have to stop Ocaliva®?

Ocaliva is being permanently withdrawn from the U.S. market for primary biliary cholangitis (PBC) after the Food and Drug Administration (FDA) determined that the risks of the medicine outweigh its benefits. Intercept Pharmaceuticals has agreed to voluntarily remove it following the FDA's decision and worked closely with the agency and patient representatives to ensure that patients and healthcare providers have time to discuss next steps.

This means OCALIVA will no longer be available in the U.S., and all patients currently taking it will need to stop, under the guidance of their healthcare team, to safely transition to other treatment options.

When a therapy is suddenly unavailable, what should I do as a patient?

Make an immediate appointment to see your GI/liver specialist or Primary Care (PCP). If you cannot get in to see your doctor, let the office know this is a **“TIME SENSITIVE AND AN URGENT NEED”**. Continue requesting an appointment within 4 weeks, and if needed, seek support from the PBC community. Look for help from patient advocacy groups, industry support lines, and/or your local liver coalition(s).

For PBC patients, Intercept has notified all prescribers about the withdrawal, so doctors should be prepared to discuss next steps. In the meantime, Intercept’s free Interconnect program can help with insurance and care coordination. **Visit interconnectsupport.com or call 1-844-622-4278 for assistance.**

Do I finish my current supply of Ocaliva® first, or stop immediately even if I have pills left?

Always consult your liver specialist before starting or stopping any treatment. Ocaliva will be available only until **November 14 in the U.S.**, and refills will not be available after that date. Since the FDA has withdrawn the medication from the market and no compassionate use program is in place, you will no longer be able to access it after November 14.

Am I at higher risk for complications because I've been on Ocaliva® for years, or does stopping now eliminate those concerns?

There is no evidence to suggest that people who have taken the medication longer will be at a higher risk if they safely transition to another second-line therapy.

Do I need to taper off Ocaliva® before starting a new medication?

There are no specific recommendations to slowly reduce Ocaliva. Studies have shown that it is safe to stop Ocaliva immediately when preparing to start a new treatment.

Will my insurance cover remaining refills on my prescription or provide an emergency supply while I figure out my next steps?

Insurance is on a case-by-case basis. Many patients have reported receiving written and verbal communication that the medication will no longer be covered.

What do I do with my remaining Ocaliva® medication, can I return it, or should I dispose of it?

Dispose of it through a Drug Take Back Program. Some pharmacies provide prepaid services to receive the medication. Find one near you at http:/www.safe.pharmacy/drug-disposal/

Now that I know that I must stop taking Ocaliva®, what are the first things I might experience?

You will continue taking **Ursodiol** if you are already taking this medication. While on your first line therapy, you may experience improvement in itch if you had it while on Ocaliva. You may experience a gradual rise in alkaline phosphatase levels in 8-12 weeks. Significant changes in liver function are uncommon during the transition to a new second-line treatment.

Will my liver disease or symptoms get worse during the transition period?

If you experience itch while taking Ocaliva, it may improve once you stop taking it. Some patients may experience worsening of fatigue if it has improved on therapy.

What do I do if I have a history of not tolerating treatment interruptions?

PBC is a slowly progressive disorder. Even if the delay is beyond three months, you should be ok. This transition should not affect your long term outcome.

What are the signs that I might notice that my liver disease is getting worse?

Developing fluid in the belly (ascites), turning yellow (jaundice), gastrointestinal bleeding, or biochemical evidence of worsening liver function (such as elevated INR or low albumin).

Should I be documenting anything specific during this transition for my medical records?

Any worsening of symptoms should be documented and discussed with your physician. It's Important to keep monitoring your alkaline phosphatase and bilirubin levels.

Are there any other medications on the market that I can use now that Ocaliva® is no longer a choice?

The good news is that there are two FDA approved second line therapy treatments that can replace Ocaliva. Patients should talk to their providers. If you qualify, seladelpar (Livdelzi®) and elafibranor (IQIRVO®) are two medications that are tolerated very well.

Will I need prior authorization for alternative medications, and how long does that take?

Yes, you will need prior authorization, and it can take anywhere from 4-6 weeks; sometimes it will be appealed. HOWEVER, if the transition is due to FDA recalls, there is a chance that the prior authorization will be expedited. Both seladelpar (Livdelzi®) and elafibranor (IQIRVO®) have a bridge program to assist patients with this transition. They will offer 30 days at no cost/low cost and expedited to assist patients through this transition.

What if I can't afford the alternative medication my doctor recommends?

Each medication has a patient assistance program and a copay assistance program to help underwrite some of the costs.
Find copay assistance for:

- seladelpar (Lidelzi): https://www.mysupportpath.com/co-pay
- elafibranor (IQIRVO) here: https://www.iqirvohcp.com/access-and-resources/access-support

What happens if the alternative medication also gets pulled from the market in the future?

Both medications currently available have Accelerated Approval and are required to show long-term benefit according to the FDA. It is possible that they could get pulled from the market if they do not show benefit or if they show harm. The good news is that the drugs appear to be safe for those with advanced liver diseases.

How can I, as a patient, help keep these medications from being pulled off the market?

By getting involved! Research, advocacy, awareness, and education all make a difference. Connect with patient advocacy organizations to learn how you can take part in research or share your story to help others.

CONTINUE TO KEEP YOUR LIVER HEALTHY!

1. Limit alcohol use

2. Keep up healthy habits and maintain a healthy weight

3. Exercise often

4. Eat a diet in rich in micronutrients (calcium and vitamins A, D, E, K)

This resource was created with the support of patient advocacy organizations dedicated to helping you navigate PBC, whether you're adjusting to a new medication or simply looking for someone to talk to. Reach out today and get connected.

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Global Liver Institute (GLI) is a 501(c)3 nonprofit organization founded in the belief that liver health must take its place on the global public health agenda commensurate with the prevalence and impact of liver illness. GLI promotes innovation, encourages collaboration, and supports the scaling of optimal approaches to help eradicate liver diseases. Operating globally, GLI is committed to solving the problems that matter to liver patients and equipping advocates to improve the lives of individuals and families impacted by liver disease. GLI holds Platinum Transparency with Candid/GuideStar, is a member of the National Health Council and NORD, and serves as a Healthy People 2030 Champion. Follow GLI on Facebook, Instagram, LinkedIn, and YouTube or visit www.globalliver.org.