



PATIENT GUIDE TO...

AASLD Clinical Practice Guidelines

Practice Guidance for Primary Biliary
Cholangitis and what it means for patients.



"Primary Biliary Cholangitis: 2018 Practice Guidance from the American Association for the Study of Liver Diseases"



foundation
support for life

In lay terms: what does it mean for the patient?

The original AASLD Guidance published in 2018 was an update of the previously published guidelines from 2009. The Guidance covers topics such as potential causes of PBC, diagnosis, treatment and how PBC presents itself to patients and providers. In a move away from traditional guidelines that make recommendations, with a strength of recommendation and a rating for the quality of evidence, the Guidance provides statements compiled, from a literature review, by a small panel of experts with their expert opinion built into the Guidance. The Guidance is a very positive step forward in the diagnosis and management of PBC, identifying preferred approaches to optimize patient care of PBC for patients, their support systems and providers.

This lay document is designed to make the AASLD Guidance more accessible to patients, and their support system, so as they can be more involved in the decision making processes within their own care, as an expert on the topic of "Living with PBC".

Here at the PBC Foundation, we believe that it is crucial that *patients*, who after all live with the consequences of any changes, and whether or not the Guidance is implemented in practice, are also made aware of the Guidance: to understand the content and how they can best utilize the information to optimize their treatment and prognosis.

Collette Thain MBE, CEO of the PBC Foundation, has this to say on the Guidance document: 'This Guidance is a significant step forward for a number of reasons. It brings a formal framework for current best practice and the latest available therapies. As an organization, we encounter and support too many patients who are not experiencing best practice, for example unnecessary liver biopsies. This Guidance can give the support and information patients need, written in black and white, to take to their clinician and ask for best practice, when it comes to their own individual care. The new Guidance is not the view of one person, or just one patient support group: it is rooted in scientific data and evidence-based medicine as well

as professional experience. Our duty now is to ensure everyone who could benefit from the Guidance has an opportunity to know its content.'

Hepatologist and PBC specialist, Dr. Robert Gish, agrees: 'The original Guidance was to inform clinicians; this lay version can educate and empower patients to become their own experts. Most patients in the US will be seen by clinicians for whom PBC is a rare disease so it is unlikely that they will have specialized knowledge of the condition. We recommend that the patient asks the provider to join them on their lifelong PBC journey where they can both exchange knowledge and information to ensure the best possible quality of life and outcomes for the patient. This lay document can provide a basis for that ongoing discussion.'

So here, health writer Isla Whitcroft, Dr. Robert Gish, and patient advocate Robert Mitchell-Thain have produced a patient guide to ensure that, by utilizing the AASLD Guidance, every PBC patient can receive the optimum treatment for their condition, no matter where or by whom they are treated.

At 26 pages, the Guidance may appear complex, so we have summarized the content in this stand-alone document specifically for the patient community to better understand the statements, what they mean for patients in everyday life and their care pathway in partnership with their Healthcare Providers. Essentially, from a patient perspective they can be divided into six sections:

- Diagnosis
- Treatment
- Symptoms
- General Advice
- PBC/AIH
- Transplant

We have also included useful questions throughout the sections for you to ask your doctor if you feel that your current treatment is not in line with the standards set by the Guidance.

DIAGNOSING PBC

'Although a lifelong disease, if diagnosed early and treated correctly, PBC has a very good outlook for most patients,' says Dr Gish. 'The Guidance points out that although there are more people being diagnosed with the condition than ever before, the response to medical treatment is very good with the number of liver transplant for PBC falling year on year. Therefore, it is extremely important that a diagnosis is made as soon as possible, with minimum discomfort to the patient.'

'Most cases of PBC can be diagnosed with a combination of blood liver (LT) tests - which provides information about how the liver is right now - and the Anti-Mitochondrial Antibody (AMA) test. Sometimes an equivalent of the AMA test will be carried out to look for different types of antibodies but that will make no difference to your overall diagnosis or prognosis. Liver function can also be assessed with blood tests like serum bilirubin.

The Guidance states that liver biopsy, which carries a risk of bleeding and other side effects, is no longer required for diagnosis in most patients and should not be used as a diagnostic tool expect except in a very few cases.

'A liver biopsy is optional for patients with known or suspected PBC,' says Dr Gish. 'Currently fewer than 10% of patients with possible PBC need to undergo biopsy. If a liver biopsy is recommended it is for auto-antibody negative (AMA, ANA, etc.) suspected PBC patients, or patients with suspected AIH overlap with PBC or NASH or other overlap diseases.'

Sometimes a biopsy will help with diagnosis and management but your doctor should be able to justify specifically why this is necessary in your case.'

QUESTION TO ASK YOUR DOCTOR. My PBC has already been diagnosed with AMA and liver tests. The AASLD Guidance says that this is sufficient confirmation that I have the condition. Please can you explain why you are also offering me a biopsy?

Patient tip from the Foundation:

If a doctor is considering a medical procedure, any procedure, the first questions we hope that go through a patient's mind would be:

- 1) What is it we are trying to find out?
- 2) Why is that information helpful?
- 3) What is the best way to find that information?
- 4) What are the pros and cons (including risks and benefits) of each option?

Using these questions to inform your decisions will help you make the best decisions for your own care.

The key word in the suggestion above is "offering". You are not obliged to accept the biopsy. You can refuse if you feel it is not the best way forward for you. The Guidance is very clear re biopsy in the circumstances outlined above. Hopefully, the questions above will help you make the best decision for you.

Once your PBC has been confirmed, the Guidance states that non-invasive imaging is mandatory in the case of all patients with biochemical evidence of cholestasis, i.e. raised Alkaline Phosphatase results (also known as Alk Phos or ALP). Dr Gish reiterates, "A full upper abdominal ultrasound is an integral part of follow up to diagnosis for patients with known, or even suspected, PBC." Some insurances may not cover the procedure, but technology such as MRI may also be helpful.

QUESTION TO ASK YOUR DOCTOR. I have been diagnosed with PBC. The guidance says that I should have non-invasive imaging of my liver and I would like to have this carried out.

Patient tip:

It may feel bold to ask such a question but it may be entirely necessary. Gone are the days when medicine was practised at or to patients. In best practice, medicine is now very much practised **with** patients. If you feel you need more confidence, be sure to write down your questions in advance and take someone with you to ensure you have every question answered and to jot down those answers.

Remember, the Guidance highlights that liver imaging is mandatory for those with raised ALP. Just to be even more confusing, not all Alk Phos is from the liver. For example, a broken bone would cause raised Alk Phos.

The Guidance states that, if your liver tests are showing a potential positive for PBC but you have no PBC-specific antibodies (e.g. AMA) it is still possible that you have PBC. Around 10% of patients fall into this category. To formally diagnose PBC, another diagnostic tool is needed which, in this case, is a liver biopsy. This is one of the few occasions you might need a liver biopsy for diagnosis. As noted above, you may be offered a biopsy later on in your disease journey but that is for different reasons. Remember, a definite PBC diagnosis needs two positive pieces of information from three possible sources:

- 1) Biochemical evidence of cholestasis (raised Alk Phos)
- 2) Positive AMA (or other autoantibody markers specific to PBC)
- 3) Liver biopsy showing PBC physical evidence

The Guidance also notes that in some cases, a test result will show an AMA positive result while the liver blood tests comes back in the normal range. 'Sometimes people with another condition will have a blood test which shows that they carry the AMA, while a liver blood test will be returned as negative,' explains Dr Gish. 'The Guidance make this clear that in this case you do not have a diagnosis of PBC at this time and nor do you need a liver biopsy to rule out PBC. However, there is a slight chance that you may go on to develop PBC in the future and the AASLD Guidelines recommend following up with blood liver tests every year for 5 years and then every 2 -3 year until the age of 65 just to be sure.

QUESTION TO ASK YOUR DOCTOR: I carry AMA antibodies but I don't have PBC. I would still like to have a liver biochemistry test regularly just to check I have not developed the disease.

Patient tip:

This would make absolute sense in terms of minimising any risk to the patient later in life. As highlighted above, the earlier in the disease journey that a PBC patient is diagnosed, the better. This precautionary strategy would ensure that changes are picked up as early as possible. At this time, PBC would not be something to worry about as, in most cases, PBC does not develop.

TREATMENT

‘My ultimate aim as a clinician is that all my PBC patients achieve a normal liver test (Alk Phos) result, which indicates a low amount of inflammation in the liver which, in turn, reduces the likelihood of developing cirrhosis.’ comments Dr Gish. UDCA (also known as Urso) is the first line therapy with the most information and the longest safety and effect record which clearly decreases progression, transplant and death in patients from the best studies.’

The Guidance clarifies that UDCA has proven efficacy in the treatment of PBC including reducing the likelihood of liver transplant, has minimal side effects and should be the first line treatment for anyone diagnosed with PBC. It also emphasises the important of taking the correct dosage: 13-15 mg/day per kilogram of weight has a close correlation with the best possible long term outcomes.

‘Studies have consistently shown better patient outcomes when UDCA is correctly taken according weight,’ says Dr Gish. ‘Monitoring your weight regularly and adjusting your dose accordingly is probably the single more important thing you can do to improve your disease progression risks. If you find taking multiple tablets difficult there are higher one dose versions which may make your life easier.

‘It is vital to remember that if your condition responds well to UDCA then you must continue on the therapy for the rest of your life. If your condition has improved it is because the UDCA is working and if you stop taking the UDCA your condition will deteriorate.’

QUESTION TO ASK YOUR DOCTOR: Is my UDCA being prescribed according to my weight at between 13- 15/mg/kg of my weight per day?

I wish to have a regular weight and dosage check added to my treatment regimen.

I am struggling with multiple tablets. Am I suitable for a higher dose, once a day dosing version of UDCA?

Patient tip:

PBC Foundation surveys in 2017, whilst unpublished, showed that much work needs to be done to ensure every patient is dosed correctly with their UDCA. The preliminary results tell us that 11% of PBC patients are not taking UDCA at all. Considering those taking UDCA, when directly measuring their dose to their weight as declared, 49% of participants were taking less than the AASLD recommended dose per day. We cannot emphasize enough the correlation between proper dosing of UDCA, UDCA response, and life expectancy. UDCA is available in a number of options, from tablets to capsules to liquid medicine, including 150mg, 250mg, 300mg and 500mg. In a small number of patients,

it is necessary to take very slightly over the recommended dose because of the strengths of the tablets available.

‘If you have an inadequate response to UDCA after one year then you should be considered for other treatments in combination with UDCA, or more rarely without UDCA,’ says Dr Gish. ‘There are a number of criteria that define non-response: ideally we would hope for an Alk Phos below 180 IU/L and for a normal bilirubin.’

QUESTION TO ASK YOUR DOCTOR: Am I a UDCA responder?

Patient tip:

UDCA response isn’t quite as simple as a one or a zero. There is a spectrum of response, and each case must be taken on its own merits. If you are not a UDCA responder, this means that you have a higher risk of progression. Now, more than ever, this higher risk is managed more closely with improved results for patients.

If you are a UDCA responder, then that, too, will drive your care pathway and ensure you receive the appropriate care for your condition. This pathway, while still being monitored and medicated for all of your life, involves much less risk of progression.

OCA, also known as Ocaliva or Obeticholic Acid, was approved by the Food and Drug Administration in May 2016 to be used in combination with UDCA in patients with PBC who have inadequate response to at least 1 year of treatment with UDCA, or as monotherapy for those patients who are intolerant to UDCA.

‘When taken with UDCA as a combination therapy, OCA has been shown to improve liver enzymes and liver function test in some UDCA-non-responders, although you can take OCA as a single therapy if you are intolerant to UDCA.’ say Dr Gish. ‘The guidelines emphasize the importance of adhering to the guidelines in terms of OCA dosage and OCA should only ever be taken as a mono therapy if the patient cannot tolerate the side effects of UDCA.

‘Fibrates are discussed as an option for PBC but are yet not approved as a treatment in PBC. Results have been from small scale studies and have highlighted potential benefits and risks, including liver and/or kidney toxicity.

QUESTION TO ASK YOUR DOCTOR: I am a UDCA non-responder. Can I be considered for Obeticholic Acid?

Patient tip:

Obeticholic Acid is the only licensed second line therapy for PBC. There is increased risk of itch, however this is reasonably well managed in most cases, with rifampicin being one of the preferred medication used for itch. In clinical trials, OCA was shown to improve liver biochemistry in the vast majority (87%) of cases and in the stringent classifications within the studies, it was found to help almost 50% of patients in the three measurable groups incorporated into the POISE study.

It is worth noting that OCA should not be considered in those with decompensated cirrhosis. If Obeticholic Acid is an option for you to consider but you have concerns, please

contact the PBC Foundation directly so we can assist you in this part of your journey.

QUESTION TO ASK YOUR DOCTOR: I am not intolerant to UDCA. Can you explain why you are stopping my UDCA treatment now I am on Obeticholic Acid?

Patient tip:

Obeticholic Acid has been recommended as a monotherapy **only** for patients who are intolerant of UDCA. The AASLD Guidance recommends combined therapy in all other cases. There is an explicit warning for patients with decompensated cirrhosis. It may be helpful to you to be aware of this before your conversation with your clinician.

SYMPTOMS

‘As well as minimising the risk of cirrhosis, it is just as important for clinicians to focus on achieving the best possible quality of life for the patient,’ says Dr Gish. ‘The Guidance reminds us that the symptoms of PBC, which include fatigue, itch (pruritus) and abdominal pain, along with dry eye and mouth (Sicca Syndrome) significantly impair quality of life and do not typically improve with treatment with UDCA or OCA, so much so that they warrant their own section on evaluation and treatment.’

QUESTION FOR YOUR DOCTOR: Are you as committed to managing my symptoms as you are to reducing my risk of cirrhosis?

‘If you feel that your symptoms are not being taken seriously enough or being ignored by your clinicians then you are not receiving the best quality treatment.

‘The main symptoms of PBC are fatigue, poor memory, itch and dry eyes and mouth and there are highly effective treatments for a number of these symptoms. Not every therapy used for symptom management is licensed for use in PBC, so it is important you are part of the decision making process to find the best answer for you.’

QUESTION FOR YOUR DOCTOR: I am suffering a symptom burden from my PBC. What can we do to make it more manageable?

Patient tip:

There are a number of symptoms associated with PBC. Many patients remain for a long time completely asymptomatic whereas some can be affected in a number of different ways. We thoroughly believe that health care starts with self-care. There will be symptoms where your doctor can help and there will be symptoms that you can affect with your life choices, too. An example would be fatigue: there is much a patient can do to alleviate their own fatigue and to improve their own quality of life. The key is informed decisions made in partnership with your clinician.

Once upon a time, what was called “brain fog” was dismissed as a symptom of PBC. Now we have an understanding that cognitive impairment can be a part of living with PBC and has a link with fatigue. There is research into more aspects of PBC such as Restless Leg Syndrome, etc. which will help us refine our understanding of what it means to live with PBC and how we can best help patients.

Your symptoms are yours, and you are best placed to discuss them with your clinician and to make informed decisions about your care. Using the PBC Foundation App will help you record your daily symptoms and open up that conversation with your clinician.

ITCH (PRURITUS): The Guidance recommends that the following treatments be used for itch

- i. Cholestyramine at a dose of 4g up to 16 mg per day.
- ii. Rifampicin at a dose of 150mg to 300mg twice daily, with monitoring of liver bloods 6 weeks and twelve weeks after the start of the course. You need to have your blood checked 2-4 weeks after starting on this drug.
- iii. An opioid antagonist naltrexone may be used at a dose up to 50 mg daily. Follow up liver tests are needed due to the risk of potential liver damage.
- iv. Sertraline (SSR) have been shown to work in some cases, taken at 75 to 100 mg daily.

‘Itching can have a huge effect on a person’s wellbeing. It affects sleep, energy and can cause such a life impact that life has little meaning,’ says Dr Gish. ‘However, there are over 14 remedies for itching alone- which can be used in varying combinations, so if the recommended guidelines don’t work then keep going back to your clinician. The key message is not to give up but to keep asking your clinician for help.’

QUESTION FOR YOUR DOCTOR: My itch has not improved with drug treatment. Can I be referred to a PBC specialist unit?

Patient tip:

“No,” is not an acceptable answer. If medication for itch escalates beyond cholestyramine, then there needs to be detailed monitoring of the patient. While the Guidance recommends 4-16mg per day, there have been successful protocols of using more than 16mg daily. This would be best done within a specialist clinician with a working expertise of PBC. Patients in the US have an ability to choose their providers: use this power to make sure you get the best possible treatment for your symptom management.

FATIGUE: ‘This is major symptom of PBC and one that often causes a huge amount of distress and disruption to the patient,’ say Dr Gish. ‘Often it is overlooked by clinicians, but the AASLD Guidance recognizes that fatigue must be addressed and treated where possible.

‘It is important to understand that there can be other potential reasons for your fatigue including hypothyroidism, depression, anemia, and sleep disorders such as sleep apnea. Once these causes have been investigated and discounted then it is time for the patient and the clinician to work together to find ways of managing and reducing the patient’s issues with fatigue. Every single patient is unique, both in the way in which fatigue affects them and how they will respond to various treatments, which is why a good partnership is vital. I liken it to a pilot and co-pilot situation where one cannot operate effectively without the other.

'Poor sleep is a noted symptom of PBC and will add greatly to the fatigue burden. Ways of tackling this can include taking therapies such as melatonin (Rozerem,) Doxepin, which also is an anti-itch and anti-depressant. A second line treatment could be the SSR groups.

'Non-therapeutic strategies for coping with fatigue include being self-aware during stressful or active times and taking breaks to allow the body time to recover from activities.

'Although it can be hard, it is important for your wellbeing to try to keep at least moderately active, enjoy social events and if you can, continue to work. PBC is linked with an increased rate of depression and being isolated or inactive can make this worse.

QUESTION FOR YOUR DOCTOR: Have you explored every other possibility for my fatigue apart from PBC including under active thyroid, anaemia etc? Can you help to manage my poor sleep quality?

Patient tip:

As Dr Gish states, the best way to manage fatigue is to find the cause and treat that cause, particularly if other conditions are the answer. It is important to remember that none of the therapies listed above in the fatigue section are licensed for use in PBC.

In our many years as a Foundation, we have found that emotional, psychological and physical self-management can have a profound effect on fatigue: both for the negative and the positive. There is much we can do about fatigue and help to improve quality of life on a day-to-day basis living with PBC.

There is a cycle that incorporates our physical health, our mental state and our behaviors. It is all too easy to let our fatigue impact upon our emotional state, which then leads to unhelpful behaviors. We, as patients, can take control and begin to make the best possible decisions for ourselves and break this negative cycle. We can start, just one decision at a time, to make the best decision for us: eat the healthiest meal we can, do even just a little exercise, get the best night's sleep we can. Improve one thing at a time and you will see the positive impact it can have on your fatigue.

We, at the Foundation, have a proven track record in helping those affected by fatigue improve their own quality of life by empowering active self-management. We are always open to helping, including using technology such as Skype, WhatsApp and Facetime to facilitate free calls and still be able to help a patient directly.

Sicca Syndrome

'Dry eyes can cause misery for patient and destroy vision,' says Dr Gish. 'Likewise, dry mouth can be deeply unpleasant and can result in teeth decay. As with itch, if the recommended treatments you should continue to ask for help as there are a number of treatments that can be tried. In any case, you should have your eyes checked once a year in case of damage and the same goes for your oral care.'

QUESTION FOR YOUR DOCTOR: My dry eyes and mouth has not improved with drug treatments. I would like to be referred to a specialist, rheumatology clinic, eye or dental specialist for further assessment.

Patient tip:

Again, no is not an acceptable answer. You know how each symptom affects you on a day-to-day basis. You can use the PBC Foundation app, or pen and pad, to record the impact your symptoms have on a daily basis and use this information to advocate for your own care.

Hopefully, your clinician will have already tried first line therapies, as recommended in the Guidance, such as: artificial tears or artificial saliva; pilocarpine or cevimiline can be used if symptoms are persistent; oral hygiene advice should be given to those at risk of dental cavities; vaginal moisturizers may help but use of oestrogen creams should only be directed by primary care or specialist gynaecological services.

GENERAL ADVICE

Periodic monitoring of a range of blood markers, symptom assessment, and a physical exam are all crucial parts of the PBC treatment journey,' says Dr Gish. 'It allows us to detect any changes which may indicate a deterioration in the condition or a need to adjust medication. It also allows us to keep a close eye on whether or not the patient is taking the correct therapy dosage'

The guidelines recommend that for everyone with PBC:

Liver blood tests should be carried out every 3 – 6 months
Thyroid tests once a year

For those with Cirrhosis:

Liver imaging every six months

An upper endoscopy at least every three years to check for varices

Regular surveillance for hepatocellular Cancer (HCC)

Osteoporosis

'The Guidance recognises that most patients with PBC are at risk of osteoporosis and that screening is essential,' say Dr Gish. A baseline screening should be carried out on diagnosis followed by bone mineral density testing surveillance every two years.

'A further recommendation is that all perimenopausal and post-menopausal women take calcium and Vitamin D supplements and that patients with advanced PBC should undergo Vitamin D level testing annually.

As a clinician I would also recommend lifestyle changes such as taking up weight bearing exercises and giving up smoking.

QUESTION FOR YOUR DOCTOR: Do I have osteoporosis? If not, I would like a baseline scan for future reference.

Patient tip:

Osteoporosis, and osteopenia, are very manageable: with or without PBC. However, if you don't know you are affected, you cannot manage the condition. Again, this is where you can easily ask informed questions to ensure you have access to the best possible care for you. The guidelines recommend that every patient should be considered for risk assessment for osteoporosis. That includes you.

Fat Soluble Vitamin Malabsorption

The guidelines note that this condition does occur in patients with PBC, but only those with elevated bilirubin or jaundice and therefore these are the only patients that need to be regularly monitored.

'You can greatly reduce the likelihood of experiencing this condition by simply taking a good quality supplement of vitamin A, D and E,' says Dr Gish.

High Cholesterol:

'Cholesterol can be elevated in patients with PBC, but it is usually good cholesterol which on its own does not indicate risk of heart disease,' says Dr Gish. 'Raised 'bad' cholesterol should be treated in exactly the same way as if it were an individual condition'.

QUESTION FOR YOUR DOCTOR: If I have high cholesterol: is it bad or good cholesterol?

Family Members

'PBC does have a small hereditary link, partially between females such as sisters and daughters,' says Dr Gish. 'It therefore makes sense to have first degree relatives screened for raised Alk Phos and the Guidance recommends that this is done in the form of blood tests from the age of thirty and every five years after that.'

Patient Tip

There are many views on this and the key is coming to the right decision for you and your family. Studies suggest a first degree relative (daughter, sister, son, etc.) has approx. a 4% chance of also developing PBC. That amounts to a 1 in 25 chance. This is important to balance out risks of developing disease with the costs of screening and other implications for your family.

Pregnancy and PBC

It is extremely rare for cirrhosis to be present in a woman of child bearing age. However, if this is the case, the guidelines state that special monitoring will be required throughout pregnancy.'

PBC/AIH Overlap

The Guidance notes that PBC can occur alongside another auto immune condition (an overlap) the most common example being Autoimmune Hepatitis (AIH), which is treated with immunosuppression and corticosteroids. AIH is usually indicated by a high ALT and IgG liver blood reading.

'AIH overlap with PBC actually occurs rarely: the Guidance says between 1 and 10 % of all patients,' says Dr Gish. 'It is therefore very important that the correct tests are carried out before AIH is confirmed and treatment started as some AIH treatments can substantially decrease your quality of life. This is one situation where a liver biopsy is worth considering, to confirm or rule out AIH'

QUESTION FOR YOUR DOCTOR: You say I have AIH (or PBC/AIH Overlap) but can you explain exactly how you have come to this conclusion? Is there any doubt in this conclusion and should I be considered for a biopsy to confirm or rule out AIH?

Patient tip:

PBC is a condition that has a very successful diagnostic tool in AMA, which is present in approximately 95% of PBC patients. AIH (and thus PBC/AIH overlap) has no such diagnostic tool currently and so is dependent on liver biopsy for diagnosis. There are liver tests which are suggestive of AIH as a possibility, but they are still not diagnostic.

The treatment of AIH also doesn't benefit from simple, clear guidelines such as those for PBC. Treatment of AIH is not consistent, either within the disease or within the community, and may need to be tweaked as changes happen to the patient. These tweaks in care are very much dependent on the expertise of the clinician.

Under these circumstance, and bearing in mind true AIH is rare with PBC, we need to be absolutely sure of the diagnosis before starting treatment.

LIVER TRANSPLANT

'The Guidance is quite clear that despite the rate of PBC diagnosis increasing, the number of liver transplants associated with disease is falling,' says Dr Gish.

'So the good news is that if patients are diagnosed early and treated correctly with the optimum amount of UDCA according to weight, most people with PBC will not go on to develop cirrhosis and even fewer will need a liver transplant.'

The Guidance notes that some UDCA non-responders may be at increased risk of cirrhosis and, as already discussed, this can be diagnosed, for example with a fibroscan or ELF and tests (not a liver biopsy). Thereafter the Guidance recommend that you should be offered a regular ultrasound to check on activity and endoscopy to check on the blood vessels around the liver.

QUESTION FOR YOUR DOCTOR: Do I need screening for cirrhosis complications and would it be useful for me to have a fibroscan?

Patient tip:

It is important to recognize the increased risk of UDCA non-responders, length of time diagnosed, and age/sex of the patient when looking at a particular care pathway.

If we can help you in this conversation with your clinician, then do contact us to discuss your particular circumstances and how you can best move forward with your PBC care.

The Guidelines confirm that liver transplants are usually considered for patients whose bilirubin is 100 µmol/L or 6mg/dl or above at any point.

‘If this number is reached this is a simple rule that should be a trigger for referral to a liver transplant unit,’ says Dr Gish. ‘In addition, the MELD score, can be used to stimulate a liver transplant referral if over 15. Most patients who have a liver transplant do very well although a small number can develop PBC again. They can be treated as per the guidelines above as if they had just been diagnosed.’

QUESTION FOR YOUR DOCTOR: Do I have a bilirubin level of over 100 µmol/L or 6 mg/dl? If so I wish to be referred to a liver transplant unit immediately.

Patient tip:

Usually with PBC, bilirubin tends to rise in a gradual way. If you are not responding to UDCA and your counts, for example Alk Phos and bilirubin, are rising, we would hope you have been referred to a specialist liver unit already. However, if this has not happened and your bilirubin does reach 6 mg/dl, this is an important trigger to being referred to a specialist liver transplant unit.

Dr Gish summarizes, ‘for the **majority of patient living with PBC, early diagnosis and response to treatment will result in no reduction in life expectancy.** For other patients, there is a risk of a higher symptom burden or potential disease progression. This Guidance was designed to help patients on their own particular PBC journey and to give them the information and confidence to have a more engaged partnership with their clinician.

‘When patients are supported, and informed, they can take much greater control of their own lives. Removing isolation also has a beneficial impact. Patient support organizations, such as the PBC Foundation, are enormously helpful in the patient experience, as you will see on the back page. Most importantly, the Foundation provides its service users all around the world with high quality information and support, empowering patients to manage their own condition.

‘The best person to manage you is you. Hopefully, this Guidance, and the support of the Foundation, will equip you to live your best life on your PBC journey, ensuring you receive the best possible care.’

QUESTION FOR YOUR DOCTOR. Can I give you a link to the AASLD PBC Guidance to download?

<https://www.aasld.org/sites/default/files/2019-06/PracticeGuidelines-PBC-November2018.pdf>

Patient tip:

This document is designed to stand alone and to bring you some of the most important aspects of the AASLD guidelines in a way that helps you advocate for your own best care within your medical system. We hope that this empowers you to ask, in a respectful way, to be an active partner and to ensure you encounter best practice wherever possible.

You can offer your doctor the link to these guidelines. You can be an informed part of the decision making process when it comes to your care. You can be an active part of your own care, not only by self-managing but by ensuring you are involved.

If we can help you in any way, do contact us and we shall do our utmost to assist you in any way we can.

The PBC Foundation Self-Management App is free to download and use. It is available in both android and apple formats. It can help you record your symptoms, your test results, and your quality of life. You can also use it to take part in surveys to ensure your experience helps us drive the patient care pathway, and to ensure documents such as this are followed.

The PBC Foundation supports patients in 76 countries around the world, with our information available in 19 languages. You can find our information at: www.pbcfoundation.org.uk

Wherever in the world you are, you have the right to free, accurate and up-to-date information with regards to PBC. We hope this document helps you in achieving that.



For further information, please contact:
PBC Foundation (UK) Ltd
6 Hill Street, Edinburgh, EH2 3JZ
Telephone: +44 (0)131 556 6811
Fax: +44 (0)131 556 8488
Email: info@pbcfoundation.org.uk
Website: www.pbcfoundation.org.uk

Access to PBC Foundation services improves patient self-management and mental wellbeing

Robert Mitchell-Thain¹, Jessica Leighton², Vinod S Hegade².

1-Dept of Education and Development, PBC Foundation, Edinburgh, 2-Liver Unit, Freeman Hospital, Newcastle upon Tyne, UK.

INTRODUCTION

The PBC Foundation has existed since 1996, providing information and support to patients with primary biliary cholangitis (PBC). It is the biggest PBC-specific patient support organisation in the world; a community of over 13,000 registered service users in 76 countries around the world.

Feedback from service users highlights feelings of being 'lost' and 'alone' before accessing PBC Foundation services. The same service users spoke of being "more knowledgeable", "more empowered" and "positive" afterwards.

International guidelines on PBC recommend PBC patients should be signposted to a patient support organisation.

AIM

We aimed to determine if the PBC Foundation services were able to meet the EASL recommendation of supporting people with PBC and to explore members' experiences.

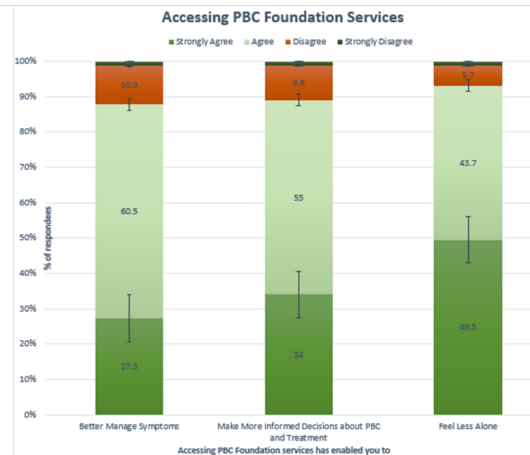
MATERIAL & METHODS

The online questionnaire was approved by 40 core PBC patients. The 32-question survey captured information ranging from clinical experience to patient support needs. This poster discusses three key questions with a four point Likert scale ('strongly agree', 'agree', 'disagree' and 'strongly disagree').

Patients were also invited to list specific needs from support organisations in a free-text question.

RESULTS

727 (n) patients (96% females) within the PBC Foundation Network were surveyed over 5 months. 609 (83.7%) were identified as members of the PBC Foundation with 32% from outside the UK.



Of 693 patients who responded to the statement 'Accessing PBC Foundation services has enabled you to better manage your symptoms', **27.2%** (188) strongly agreed and 60.4% (443) agreed. Similarly, **34%** (236) 'strongly agreed' and 54.9% (380) 'agreed' to the statement 'Accessing PBC Foundation services has enabled you to make more informed decisions about your PBC and its treatment'. Of the 707 patients who responded to the statement 'Accessing the PBC Foundation services has made you feel less alone' **49.5%** (350) strongly agreed and 43.7% (309) agreed.

Overall, the proportion of positive responses ('strongly agree' or 'agree') to above three statements was **~90%**, suggesting a significant positive impact. For PBC patients, the three priority needs from a support organisation were: **information** (beyond that given by medical practitioners), **contact and support** (with other PBC patients) and **discussion and sharing experiences** (exchanging information and advice with other PBC patients).

CONCLUSION

This survey of PBC patients highlights the importance of access to patient support organisations and consequential improvement to quality of life. Large proportions of PBC patients accessing the PBC Foundation reported improvement in their disease self-management, decision making abilities, and social and mental wellbeing. The study suggests high level of satisfaction with the PBC Foundation, which is well placed to provide patient support as per the EASL guidelines.

ACKNOWLEDGEMENTS

Thank you to the PBC foundation members who contributed to this research.

DISCLOSURES

PBC Foundation funding is found here: <https://www.pbcfoundation.org.uk/support-us/our-funding-partners>

The survey received funding from Intercept Pharma UK and Ireland Ltd who also provided input into the questionnaire design and analysis of results.

Dr Vinod Hegade and Jessica Leighton have no conflicts of interest to declare.



Contact information

robert@pbcfoundation.org.uk

j.leighton@newcastle.ac.uk

vinod.hegade@newcastle.ac.uk

