



Intercept 

**PRACTICE**  **POLICY**®

Europe and Canada

# Innovating in PBC

SHARING BEST PRACTICE INSIGHTS  
FROM PRACTICE TO POLICY®

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# ABOUT PRACTICE TO POLICY®

The Practice to Policy® Health Awards Programme is designed to improve health outcomes for people living with primary biliary cholangitis (PBC) by providing a platform which encourages innovation and best practice sharing within the liver community.

Since its inception, we wanted to run this programme hand-in-hand with the community, as well as ensure that we are reinforcing good quality care as set out in the EASL guidelines for the treatment and management of PBC.

Launched back in 2016, the programme has funded over 30 projects in 9 countries, including learning platforms for physicians, peer-to-peer support analyses, cost-effective platforms to link patients through existing national frameworks, analyses of existing data to identify those undiagnosed, educational apps for the patient community, and many more!

The aim of this booklet is to share the key learnings from some of the projects we have supported. What makes this programme so valuable is that it supports local, innovative ideas, which form a basis for best practice principles which can be adopted more broadly. At its heart, Practice to Policy® is about sharing what worked well, what didn't, and what we can learn as an entire community to inform care practices.

We are delighted that we were able to give the initiatives in this booklet the means to innovate, push boundaries, explore new ideas to shape the PBC landscape and inspire change within the PBC community.

We would particularly like to thank our incredible judges. Their support has been invaluable to the programme, both in the judging itself, as well as the support to help formulate this booklet.

As existing projects come to life, we hope the key learnings outlined in this booklet will continue to inspire change and cultivate better experiences for people living with PBC.



**Lisa Bright**  
**President International, Intercept Pharmaceuticals, Inc.**

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The Europe and Canada Practice to Policy® Health Awards Programme is endorsed by The European Association for the Study of the Liver (EASL).

# PRACTICE TO POLICY®

## EXTERNAL JUDGES IN 2018\*

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### Hanns-Ulrich Marschall, MD, PhD, MSc

Hanns-Ulrich Marschall, MD, PhD, MSc, has been Professor of Clinical Hepatology at Gothenburg University since 2010. He was Professor of Medical Gastroenterology and Hepatology at the Karolinska Institute in Stockholm from 2007 to 2010. He is a leading expert in bile acid metabolism and his research involves animal and human studies with focus on the interaction between bile acids and the gut microbiota at the Wallenberg Laboratory. He is also actively involved in a large number of sponsored and investigator-initiated clinical trials focusing on the development of new treatment options for non-alcoholic fatty liver disease/steatohepatitis and cholestatic liver diseases such as primary biliary cholangitis (PBC), primary sclerosing cholangitis (PSC) and in particular, intrahepatic cholestasis of pregnancy (ICP).



## Achim Kautz (Leberhilfe Projekt gUG)

Achim Kautz is co-chair of the German liver disease project company “Leberhilfe Projekt gUG”, which focuses on public policy projects in liver disease. Achim has a degree in communications science, advertising and marketing and was the CEO of the Deutsche Leberhilfe e.V. before transferring into his role at Leberhilfe Projekt gUG. He is a founding member of the European Liver Patients’ Association (ELPA) and also held the position of Policy Director at ELPA. Additionally, Achim is a founding member of the World Hepatitis Alliance (WHA) and is one of its Special Consultants. He also works with the “National Strategy Planning Group”, an entity brought to life by the World Health Organization (WHO), and as an external expert for the Organisation for Economic Co-operation and Development (OECD).

\*The judging panel for the full Health Awards Programme comprises Intercept representatives from Corporate and Medical Affairs, alongside these external advisors. Any conflicts of interest are declared by the judges and said judge is excluded from any discussions which pertain to that conflict.



# PRACTICE TO POLICY®

## EXTERNAL JUDGES IN 2018\*

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### Marco Marzioni, MD (EASL)

Marco Marzioni, MD, is a clinical gastroenterologist and hepatologist, and Professor of Gastroenterology at the Clinic of Gastroenterology, Università Politecnica delle Marche - “Ospedali Riuniti” University Hospital of Ancona, Italy. He has taught there since 2005. From 2013 to 2016 he has been a member of the Associazione Italiana Studio Fegato (AISF) Governing Board; from 2014 to 2016 he served as Secretary General of the association. Since 2015, he has been advisor for gastroenterology and hepatology of the Italian Medicine Agency (AIFA). His research activity focuses on the pathophysiology of liver injury. In particular, his major interest is the study of the mechanism of regulation of cholangiocyte cell biology, injury, and repair. To date, his research activity has been published in 105 original articles in major hepatological and gastroenterological international journals. He is a member of the European Association for the Study of the Liver’s (EASL) medical board and acts as the EASL representative judge.





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## Robert Mitchell-Thain (PBC Foundation)

Robert Mitchell-Thain trained in counselling and coaching techniques and is the Head of Education and Development for the PBC Foundation, an international patient organisation based in the United Kingdom. The Foundation provides support for people living with primary biliary cholangitis (PBC), their families and friends. Robert is not only a strong advocate on behalf of all liver patients, but he also functions as an information provider to patients calling the helpline, medical professionals, politicians, industry members and to the general public. In 2003 he joined the Foundation, due to his mother being diagnosed with PBC in 1994. Since then he has presented all around the world to and on behalf of those affected by PBC. His motivation remains his experience with his mother, who had her life transformed once she had the information needed to come to terms with her condition.

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## FOREWORDS

PBC is a rare condition, yet it is a common rare condition. One of the features of PBC is that as it has become better known, it has also become better diagnosed. An early diagnosis can be important for a patient and their PBC journey, so it is vital that we each play a role in raising awareness of PBC.

Practice to Policy® has given us a wonderful opportunity to raise awareness of PBC, yes, but also to educate clinicians, patients, patient advocates and the general public about this rare condition.

Each project within the domain of disease awareness tackled the issue in a slightly different way, yet was successful in not only making an initial splash, but in making ripples that will extend into the wider community. Each one has sown a seed that can grow into something bigger: helping with diagnosis; with information after diagnosis; with easy to understand information for families, friends and carers; and destroying some of the previously-held myths that have held back PBC patients in the past.

Enjoy reading about the progress each project has made. I hope their example inspires you to take the next steps and to continue their good work in raising the profile of this condition and its patients, creating a much better understanding of PBC.



**Robert Mitchell-Thain**

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Is there a need to improve the diagnosis of PBC?

Recent guidelines allow us to diagnose PBC in patients that have elevated serum alkaline phosphatase and are positive for antimitochondrial antibodies (AMA). But what about those individuals that only tested AMA-positive once?

Do these individuals have PBC, where with proper diagnosis and management we might be able to prevent progression and severe complications? And could this also be the case in close relatives to PBC patients, considering the genetic background of PBC?

Practice to Policy<sup>®</sup> provides a platform for innovative forms of diagnosis. One of the key areas explored is the value of utilising existing patient data to identify patients at risk, and indeed in one study, 10% of subjects who tested positive for AMA in a non-hepatological setting were identified as PBC patients.

Furthermore, this platform also provided support for the value of screening for PBC in close relatives to a patient, as another project found clear indications of an increased risk for developing PBC in first-degree relatives.

The answer to the question above thus should be “yes”. These Practice to Policy<sup>®</sup> projects identify patients with previously unknown PBC already in small populations, by using simple approaches in populations with potential risk of PBC. Timely diagnoses could result in a beneficial outcome in the majority of them.



**Hanns-Ulrich Marshall**

# Clinical Re-Evaluation of Anti-Mitochondrial Antibody Positivity in a Monocentric Setting



## ORGANISATION NAME

Paracelsus Medical University,  
Salzburg

## COUNTRY

Austria

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## AWARD CYCLE

2016

## PROJECT

466 patients with positive anti-mitochondrial antibody (AMA) results within a non-hepatological setting were evaluated by using their hospital data or having been contacted specifically in order to evaluate their liver health, as it can act as a key marker for diseases such as PBC. The project recruited patients from non-hepatology settings: neurology, dermatology, rheumatology, haematology, and a combination of cardiology, pulmonology and nephrology.

## OBJECTIVES

The main objective for the team was to determine the liver health of AMA-positive subjects in a non-hepatological setting. In turn, this would then help to determine undiagnosed/mismanaged cases of PBC.

In addition, the team hoped to gain further insight into the implications of AMA-positivity if PBC criteria were not fulfilled at baseline.



## KEY LEARNINGS

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The results demonstrated that regular and structured follow-ups for all AMA-positive subjects, including those from non-hepatology specialities, are important to monitor their liver health, with every two years being the most reasonable follow-up time. The project also identified that AMA-positivity may be lost over time for unidentified causes.

One of the challenges was that there was sometimes insufficient patient data at baseline or follow-up was difficult to obtain. This was not something that could be resolved, but something that required a work around.

This project provides the first data from Central Europe to support British and U.S. data regarding insufficiently treated PBC patients.

## Outcomes – What’s Next?

### RESULTS

The project identified 17 patients whom either did not know that they had PBC or were insufficiently treated. Of the AMA-positive patients who did not fulfil PBC criteria at baseline, 22/185 became AMA-negative, 56/185 remained AMA-positive and 12/185 had developed PBC.

### NEXT STEPS

The project is part of a broader university initiative which has been underway since 2006, seeking to determine the natural clinical course of all AMA-positive test subjects.

The preliminary results were presented at EASL 2017 and publication of the full manuscript is currently in progress.

### IMPACT

The results demonstrate that patients who test AMA-positive in non-hepatological settings could be living with undiagnosed PBC or the condition could be mismanaged. This demonstrates the importance of regular liver checks for all those who test positively for AMA within non-hepatological settings.

# Screening for PBC in First Degree Relatives of PBC Patients



H A M B U R G

## ORGANISATION NAME

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Hamburg-Eppendorf (UKE),  
Hamburg

## COUNTRY

Germany

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## AWARD CYCLE

2016

## PROJECT

The UKE team screened 100 first-degree relatives (FDRs) of PBC patients for clinical and laboratory signs of PBC, and early biomarkers pointing towards the early pathogenesis of this disease. Questionnaires were used to screen for risk factors and symptoms, cholestatic liver enzymes were analysed and blood was tested for PBC-specific antibodies.

FDRs who tested positive were further examined in a clinic in order to confirm or reject the diagnosis of PBC. The project is ongoing and aims to complete around 200 screenings for FDRs in total.

## OBJECTIVES

The main aim of the project is to define the risk for FDRs of patients with PBC to develop PBC themselves. This was achieved by screening peripheral blood to detect early biomarkers of PBC and any abnormal cholestatic liver enzymes and PBC-specific antibodies.

The project also aims to educate PBC patients and their FDRs about an increased risk of disease as well as risk factors and early symptoms of PBC. Through the project the research team hopes to be able to gain more insight into early disease stages.

## KEY LEARNINGS

FDRs of PBC patients are at an increased risk of developing PBC themselves and therefore need to be screened on a routine basis. This research helped raise awareness of this increased risk for FDRs and potentially supports a claim for screening to be included in PBC guidelines.

There is a strong solidarity and commitment throughout the PBC community to further evaluate the benefit of screening within risk groups. PBC patients also welcomed the opportunity to contribute to research by referring their FDRs for screening and felt empowered by the role they play in manifesting screening of FDRs as a routine practice.

The use of fluorospot-analysis to detect antigen-specific T cells in peripheral blood of FDRs was difficult to establish, as the frequency of these antigen-specific cells is relatively low in peripheral blood. However, the technique is still being optimised to help inform the results of the project.

## Outcomes – What’s Next?

### RESULTS

There were clear indications of an increased risk for FDRs developing PBC. The intensive screening effort has resulted in FDRs being newly diagnosed with PBC and offered timely treatment. Other FDRs who exhibited PBC biomarkers, but were not diagnosed with PBC have been educated and sensitised to their own risk of disease onset. The results of the study will be published.

### IMPACT

Results from this research may help to recommend which FDRs should be screened for PBC as clinical routine. This will help to identify PBC patients early in the course of their disease.

The screenings have helped raise awareness of increased risk for FDRs among PBC patients and their relatives, and the need to evaluate the value of screening for PBC.

### NEXT STEPS

The study will continue in order to successfully screen a total of 200 FDRs, after which the project will be expanded further in order to improve the impact of the results. In addition, all patients will be re-invited every two years after initial screening to confirm their results and to check whether PBC is likely to develop in the future.

By testing more FDRs, the group plans to establish whether PBC screening should be mandatory for FDRs of PBC patients.

# Raising Awareness of PBC in Finland



Munuais- ja maksaliitto  
NJUR- OCH LEVERFÖRBUNDET

## ORGANISATION NAME

Finnish Kidney and  
Liver Association

## COUNTRY

Finland

## CONTACT DETAILS

Sari Högström (Executive Manager)  
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## AWARD CYCLE

2016

## PROJECT

The Finnish Kidney and Liver Association team undertook a number of activities to raise awareness of PBC. A brochure for patients diagnosed with PBC was distributed to healthcare units and healthcare professionals across Finland and was also made available on request directly from the association.

When publishing the brochure, an interview style video was also developed alongside it, supporting further awareness around PBC. Brochures were also distributed during 'Liver Week' alongside public lectures and information events, organised by the association. In addition, on 'Rare Disease Day', the association shared a press release and organised multiple interviews.

## OBJECTIVES

The Finnish Kidney and Liver Association team hoped to raise and maintain awareness of PBC amongst patients, their families and healthcare professionals (HCPs), as well to provide basic understanding and awareness of PBC to the Finnish public. By targeting the media, the team aimed to bring attention to PBC.

As part of the initiative, the team also hoped to be able to provide newly-diagnosed patients with a deeper understanding of the condition to support their journey.

## KEY LEARNINGS

Readily available information about PBC is important to help newly-diagnosed patients become more involved in the management of their condition and better inform their decisions around disease management. As the needs and requirements of patients should be the focus of materials, it is important to involve the patients in the development phase.

Utilising different channels and different platforms to reach out to patients can increase awareness and engagement. By providing a range of newsletters and sharing various social media updates, it is possible to reach out to a wider patient group, which in turn increases the number of PBC patients reaching out to patient organisations.

However from a clinical setting, there were challenges in ensuring the brochure went to the right person at the right time in order for it to be utilised in health centres.

## Outcomes – What’s Next?

### RESULTS

There was a big increase of website views during and after the outreach activities (2,997 website views), with users staying on the site for a significant amount of time, highlighting their interest. After the distribution of approximately 3,000 brochures the overall feedback was ‘good’, with multiple professionals ordering the brochure for use in clinics. The project also received significant media coverage during ‘Rare Disease Day’ and ‘Liver Week’: 32 articles and 5 media and radio interviews.

### NEXT STEPS

The association plans to organise a PBC round table, with the aim to fully explore the needs of patients, the opportunities for support, and provide an opportunity for patients in Nordic countries to share experiences and practices. The association will continue to share their PBC brochure and raise awareness of PBC, whilst providing peer support to the already-enrolled PBC patients.

### IMPACT

All activities which make up this outreach project have a direct impact on newly-diagnosed patients, with patients who received the brochures highlighting how useful the document was. The impact on the wider general audience can also be noted with significant media coverage ensuring the wider public are aware of the condition and the impact it has on patients’ lives.

# CBP – (IN)FORMA RARA: Raising Awareness of PBC Pathology



**RARISSIMAS**  
Inovação  
Investigação  
Internacionalização

## ORGANISATION NAME

Raríssimas – Associação Nacional de Deficiências Mentais e Raras

## COUNTRY

Portugal

## CONTACT DETAILS

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## AWARD CYCLE

2016

## PROJECT

The Raríssimas team developed and used various materials and platforms to reach out to both healthcare professionals (HCPs) and the general population with information about PBC. Clinical charts, leaflets and posters were sent out to medical centres to be displayed; in addition, information leaflets were also directly shared with patients. To support the materials developed, workshops for HCPs were undertaken, as well as a workshop for both HCPs and patients. A short animation film was also developed as a creative and visual aid, to be shown at each workshop.

## OBJECTIVES

The main objective of all elements of this project was to increase knowledge about PBC and its intricate pathology amongst HCPs, patients and the general population, through scientifically validated information and eye-catching graphics.

In addition, the aim was to increase awareness of PBC to mass public audiences through media coverage.

## KEY LEARNINGS

The increased awareness of PBC was appreciated by patients and they were particularly grateful that the project helped them find a patient organisation for support.

The fact that the materials were validated and collaborated on with the medical community gave great value to the materials. However, in turn, this did mean that the initiative had to account for additional time to develop materials.

PBC patients and their families found it beneficial to interact with other PBC-affected families. It is key to note that the project has shown that patients find it useful to know other families' experience and clearly identify a patient organisation that could inform and advise them.

## Outcomes – What's Next?

### RESULTS

Raríssimas' helpline has observed a 186% increase in the number of PBC queries, which shows that activities have increased awareness of PBC. The Raríssimas Facebook page increased engagement, reaching approximately 22,000 people with the PBC animation film, which was also presented to over 200 HCPs at the Portuguese Society of Internal Medicine and shared on multiple social channels. The workshops had an average of 20 participants, who rated the workshops highly with an average score of 3.7/4 in positive experience.

### NEXT STEPS

Building on the workshop, Raríssimas intends to translate their PBC animation film into English to make it more accessible. Moving forward, Raríssimas would also like to promote their training programmes with a slightly different approach.

### IMPACT

The PBC patients who have engaged with the organisation since the project have all mentioned their excitement in having PBC highlighted by the media, but also in having identified a patient organisation that could help them get the information and support they need. More broadly, it demonstrates the high need to still educate and raise awareness of PBC, as it brings huge value to patients.

## FOREWORDS

The European Association for the Study of the Liver (EASL), in its guidelines for the management of primary biliary cholangitis (PBC), indicates with major relevance the identification of patient needs within the frame of the appropriate clinical care. In particular, proactivity is needed in the recognition of symptoms and their management.

With a chronic, long-lasting disease such as PBC, the integration of clinical findings and self-reported outcome is key for an optimal management. Hence, the patient her/himself is crucial for her/his possibility to achieve high value care. The skills of the doctor are mandatory, but not sufficient without the integration of appropriate information of the single patient needs.

There is thus a prominent need for projects aiming to disseminate the culture of self-assessment, to make patients aware of how to stratify their health state, what to do to improve it and how to communicate it to clinicians.



**Marco Marzioni**

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Patients living with PBC suffer twice from the disease: the disease itself, as well as the symptoms associated with it which they have to live with every day: pruritus, fatigue, dryness of mucous membranes or bone/wrist pain.

Symptom management is as critical as the clinical management of PBC itself. The impact on daily quality of life is enormous and cannot be comprehended by those not exposed to this condition. Several studies show that physician knowledge on how to manage symptoms or even classify or rate the severity is low. As a result, parallel studies are also showing how patients are starting to 'self-manage' to address it.

Practice to Policy® provides a unique opportunity to support pilot projects to shed light on the value of 'self-management' and the significant positive impact this can have.

The winning projects focus on improving quality of life, empowering patients and improving interaction between patients themselves as well their physicians. The projects that have been developed give patients the chance to have a better understanding of the disease, to be more adherent, to be supported by other patients and relatives, as well as to provide a new way of professional interdisciplinary support.

All the winning projects are complimentary and reflect the needs of the PBC patients and community. There is a great opportunity to learn from these excellent findings and to establish them in day-to-day practice for all patients around the world.



**Achim Kautz**

# Canadian PBC Society Peer-to-Peer Support Programme



## ORGANISATION NAME

Canadian PBC Society

## COUNTRY

Canada

## CONTACT DETAILS

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## AWARD CYCLE

2016

## PROJECT

The Canadian PBC Society hosted self-management workshops for people with PBC and HCPs in Canada. In addition to face-to-face workshops, a peer-to-peer support programme was offered to patients and family members, which assigned each participant a 'buddy' with whom they could share information and learnings through online platforms.

## OBJECTIVES

The Canadian PBC Society wanted to investigate the impact self-management workshops and peer-to-peer support can have on a PBC patient's quality of life. To evaluate the programme, participants filled in questionnaires to report on their quality of life before and after attending the workshops.

## KEY LEARNINGS

The project demonstrated clear success in collaborating and sharing best practice between organisations. The workshop series was based on an original programme developed by The PBC Foundation, who were very supportive in transferring knowledge and collaborating in the initial implementation.

This workshop series strengthened ties in key communities in British Columbia, Duncan, Alberta and Ontario, and precipitated additional educational meetings and dinners with HCPs as a result, demonstrating the significant appetite for this type of activity.

However, there were also challenges. For example, disease progression varies widely, and affects patients' ability to participate in various programmes such as this. In this instance, there were a number of 'drop-outs' due to poor health, which affected the assignment of 'buddies' in the peer-to-peer support element and the overall ability to statistically measure the success of the series.

## Outcomes – What's Next?

### RESULTS

The workshops engaged a total of 170 PBC patients/family members in key PBC communities in Central and Western Canada and was successful in improving the quality of life of patients through learning and facilitating conversations with others in the community.

### IMPACT

There is huge value for people being able to meet groups of patients and their family and friends, making connections with others who are also going through the same experiences.

Through discussions, the biggest challenge identified was the nature of disease progression, which varies widely and can affect patients' ability to participate in various programmes.

### NEXT STEPS

The PBC self-management workshop will be continued and offered in multiple locations across Canada, including Halifax (Nova Scotia), Ottawa (Ontario), Toronto (Ontario), Winnipeg (Manitoba), St. John's (Newfoundland), Victoria (B.C.).

# PBC Foundation App: Putting Information At Patients' Fingertips



## ORGANISATION NAME

The PBC Foundation

## COUNTRY

United Kingdom

## CONTACT DETAILS

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of Education and Development)  
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## AWARD CYCLE

2016

## PROJECT

The PBC Foundation designed an app to make it easier for people living with PBC to receive accurate, up-to-date information about their disease, irrespective of their location.

External stakeholders such as physicians from across the world were consulted to ensure the content was best suited for patients and their needs. The app was launched on the iOS platform in September 2017 and for Android devices in October 2017.

## OBJECTIVES

The project's objective was to make the lives of patients living with PBC easier and provide them with a tool of self-empowerment, incorporating medical information, self-management tools and techniques, tools for tracking symptom frequency and severity, liver biochemistry results over time, and mood, as well as a reminder for medications and questions to ask clinicians. The app also includes information about local events to help break isolation affecting patients.



## KEY LEARNINGS

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App statistics show that patients have been engaged in using the app which helps them to self-manage, indicating that there is an appetite to do so within the patient community. The results also indicate that patients want to have an active role in their treatment journey. The app gives patients a feeling of being in control and thus helps them to deal with the emotional strains that come with PBC.

The cross-functional collaboration championed in this project to produce the most desirable outcomes should inspire the PBC community to work more closely together. For this project, stakeholders from different areas came together and worked towards a common goal.

## Outcomes – What’s Next?

### RESULTS

As of 01/01/18 there were 661 downloads of the app.

### IMPACT

The app development brought the PBC community closer together. Patients, advocates, treating physicians and researchers were collaborating to achieve one common goal. PBC patients are now able to track their health and disease-related biomarker levels themselves. The app functions as a tool for self-management and self-empowerment. Initial feedback shows that users are very satisfied with the app and respond positively to design and user experience.

### NEXT STEPS

The PBC Foundation aspires for the app to be a safe place for patients to store their disease-related data and make a contribution to research if they wish to do so.

# PBC Webinars: Providing Self-Management Workshops Regardless of Location



## ORGANISATION NAME

The PBC Foundation

## COUNTRY

United Kingdom

## CONTACT DETAILS

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of Education and Development)  
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## AWARD CYCLE

2016

## PROJECT

The PBC Foundation team produced a series of nine two-hour webinar sessions which provided educational information about the self-management of PBC to people around the world.

The seminars reached audiences from Turkey, Holland, Central and North America, along with those from the United Kingdom. The team also shared a questionnaire form with the participants to gain their insights into the series.

## OBJECTIVES

The primary aim of the project was to educate people around the world about PBC self-management, in particular those in locations without significant existing support.

In addition to the main objective of an increase in awareness, the project aimed to gain insights into how these webinars have helped participants and their understanding of PBC self-management.



## KEY LEARNINGS

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The webinars offered the ease for people to take part from the comfort of their own home. However, it is key to note that this in turn prevents some individuals from joining as they were intimidated and unfamiliar with the technology.

For those who did join, a ten-minute warm-up period at the beginning of each webinar was very useful in allowing participants to familiarise themselves with the technology.

One key area for improvement is timings. Trying to secure one time slot is difficult, mostly due to geographic time differences. It would be beneficial to split or repeat sessions to help include more people in this event series.

## Outcomes – What’s Next?

### RESULTS

65 people from the United Kingdom, Canada, USA, Netherlands and Turkey attended the webinar sessions with 98% of them agreeing/strongly agreeing that they are now better able to manage their symptoms as well as being able to make better decisions about their PBC and treatment. 75% of attendees also agreed/strongly agreed that they will now feel less alone, with some patients forming new friendships with other attendees.

### NEXT STEPS

There are plans to repeat the project in 2018, using multiple sessions to enable participants from different time zones to join at a convenient time. In addition, promotion of the project will be done further in advance, allowing people to familiarise themselves with the necessary technology and reduce confusion.

### IMPACT

The impact of the seminars can be seen directly by those participants who took part and the questionnaires that they completed. The results showed that the educational information shared within the workshops greatly helped those attending, and will ultimately affect the way they manage their condition. Providing these types of platforms is essential to ensuring patients worldwide are supported throughout their journey.

# Development of an Innovative Guide for Effective Pruritic Treatment in PBC



## ORGANISATION NAME

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## COUNTRY

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## CONTACT DETAILS

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## AWARD CYCLE

2016

## PROJECT

The team from Friedrich-Alexander University Erlangen-Nürnberg developed a short questionnaire to help clinicians to easily identify PBC patients requiring support in the management of pruritus and other symptoms such as fatigue, dryness of mucous membranes and bone/wrist pain. The questionnaire was taken by the patients twice within a week period to validate the answers they gave.

In addition to this, a brochure was developed to provide information and guidance on managing pruritus, which was handed out to PBC patients who participated in the questionnaire. These patients finally answered a survey to determine how useful they found the brochure.

## OBJECTIVES

The main aims of this project were to:

- Support clinicians in the identification of patients experiencing debilitating symptoms as a result of their PBC
- Create an easy-to-understand leaflet to support PBC patients in managing pruritus and thereby increase their quality of life

## KEY LEARNINGS

Pruritus remains a major unmet clinical need in PBC patients – pruritus affects approximately 50% of PBC patients and is inadequately treated in the majority of these cases. Thus, it became apparent that it is important to raise awareness of this symptom and to give a guideline-based approach to efficiently manage pruritus and other symptoms for patients and doctors.

Within the project itself, there was some difficulty in finding patients to participate in the initial questionnaire and the subsequent validation iteration. Whilst the target number of patients was reached, this took longer than anticipated.

## Outcomes – What’s Next?

### RESULTS

Patients were asked to rate the usefulness of the PBC information brochure: ‘very useful’, ‘useful’, ‘undetermined’, ‘less useful’, or ‘not useful at all’. Out of 40 pruritic patients, 22 rated it ‘very useful’. Those who have read the brochure, both pruritic and non-pruritic patients, are thus now able to effectively identify the symptom and take steps to manage it.

### NEXT STEPS

The project group aims to publish the patient questionnaire and brochure and distribute it to patient organisations, university hospitals and local hepatologists as well as communicate their findings via social media and the internet.

They will also apply for further funding for other educational materials (e.g. brochure for GPs & GI specialists). Another future project may be to develop a ‘Special Itch’ mobile phone app for PBC patients.

### IMPACT

Chronic itch can dramatically reduce quality of life, cause sleep deprivation, depressive mood and may even induce suicidal ideation in those patients most affected. This brochure addresses an important unmet need for pruritus as a symptom of PBC and contributes to an increase in quality of life for PBC patients.

# APEMAD – Pilot Study of a Model of Therapeutic Education



## ORGANISATION NAME

APEMAD, CHU de Montpellier

## COUNTRY

France

## CONTACT DETAILS

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## AWARD CYCLE

2016

## PROJECT

The team from CHU de Montpellier developed a pilot therapeutic education model to support PBC patients' adherence to treatment. A multidisciplinary approach was used, with the project directed by a hepatologist and coordinated by a specialised nurse. The nurse's job during a visit every 12th week was to ensure the patient knew the disease and the side effects of treatment. The study was conducted in new patients, as well as those already receiving treatment.

## OBJECTIVES

The main aims of this project were to:

- Investigate if therapeutic education would improve adherence to the treatment of PBC
- Evaluate the quality of life of people with PBC
- Define the reasons for insufficient adherence

## KEY LEARNINGS

It has been largely observed that adherence to treatment is a key issue among patients with PBC. One of the reasons is the side effects of treatment, particularly pruritus. One of the key insights from this project was that patients feel isolated and sometimes lack quality information regarding their treatment options and the potential side effects, which can result in them simply stopping to take their treatment.

Patient input in pilots is critical to success. It was proposed that the effectiveness of the programme should be assessed via a questionnaire. However the original questionnaire reviewed by a panel of patients supporting the pilot outlined the need for it to be revised in order for it to be of most value. Collaboration with patients in the evaluation of a project is as valuable as their collaboration in its design; it ensures that all the key learnings identified can be transferred into best practice learnings.

## Outcomes – What’s Next?

### RESULTS

The study was undertaken in 30 patients and showed that in the majority of cases there was improved adherence.

In terms of patients engagement in the programme, the preliminary results show:

- Very good participation of the patients whatever the stage of disease (>90%)
- Very good adherence to scheduled visits and treatment

Patients were more likely to ask their nurse for information versus their doctor due to shyness or due to the perception that doctors are not available.

### IMPACT

The provision of education had a significant impact in improving medicine adherence, and patients engaged positively with a programme which helped to facilitate this.

Overall, the perception was that this information was more readily available from a nurse versus a doctor.

### NEXT STEPS

As part of the study, data will continue to be collected as part of the 48-week follow-up to determine how the programme affected medicine adherence.

Once completed, future opportunities for publication will be identified.

# 2016 WINNING PROJECTS\*

## Full list

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Pilot Study of a Model of Therapeutic Education

**APEMAD - CHU Montpellier, France**

PBC Peer-to-Peer Support Programme

**Canadian PBC Society, Canada**

ELPA PBC Education Day

**European Liver Patients' Association (ELPA), Europe**

PBC-Community-App

**European Liver Patients' Association (ELPA), Europe**

Development of an Innovative Guide for Effective Pruritus Treatment

**Friedrich-Alexander-University of Erlangen, Germany**

Peer-to-Peer Support and Education

**Liver4Life, United Kingdom**

Clinical Re-Evaluation of AMA-Positivity in a Monocentric Setting

**Paracelsus Medical University Salzburg, Austria**

CBP - (IN)FORMA RARA

**Raríssimas - Associação Nacional de Deficiências Mentais e Raras, Portugal**

Characterising Linkage for the Patient with PBC from Primary to Secondary Care in South England

**Royal Surrey County Hospital, United Kingdom**

PBC Awareness in Finland

**The Finnish Kidney and Liver Association, Finland**

PBC Foundation App: Putting Information at Patients' Fingertips

**The PBC Foundation, United Kingdom**

PBC Webinars: Providing Self-Management Workshops Regardless of Location

**The PBC Foundation, United Kingdom**

Raising the Bar: A Cross-Platform Approach to Raising Awareness in PBC

**Toronto General Hospital, Canada**

Screening for Primary Biliary Cholangitis (PBC) in First Degree Relatives of PBC Patients: Prevalence of PBC and Screening for Early Disease Biomarkers

**University Medical Centre Hamburg-Eppendorf, Germany**

# UPCOMING PROJECTS

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PBC Alive - What You Need to Know

**Albi Association, France**

A Cholestatic Symptoms Clinic for the East of England

**Cambridge University Hospitals NHS Foundation Trust, United Kingdom**

PBC Self-Management Programme

**Canadian PBC Society, Canada**

Patient Empowerment Through An Online Road Map

**EpaC Onlus, Italy**

Patient Education for Primary Biliary Cholangitis

**Grenoble Alpes University Hospital (CHUGA), France**

Linking Patients Into NHS England Operational Delivery Networks For PBC Therapy

**Nottingham University Hospitals NHS Trust, United Kingdom**

Cidadania Rara

**Raríssimas - Associação Nacional de Deficiências Mentais e Raras, Portugal**

Creation Of 2 Innovative Group Workshops On Fatigue And Chronic Disease

**REVHEPAT, France**

Breaking Down Barriers To Information: PBC Resource Translation

**The PBC Foundation, United Kingdom**

\*Please note, not all projects listed are featured in this booklet as they are waiting on the assessment of key outcomes before sharing the final results.

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The Europe and Canada Practice to Policy® Health Awards Programme is endorsed by The European Association for the Study of the Liver (EASL).

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