

October 2024



**European
Reference
Networks**



Newsletter #17

Welcome to our next newsletter!



Dear members and friends of the ERN RARE-LIVER,

Two weeks ago we held our successful annual Online Members Meeting, the second one in 2024. At this point, we would like to take this opportunity to thank all of you who attended.

In addition to lots of input and presentations, we said a virtual farewell to José Willemse, who has held a leading position in the ERN RARE-LIVER ePAGs

(European Patient Advocacy Group) for many years, helping to make the patients views seen and voices heard in the ERN and beyond.

To everything we have looked back on together, we are looking forward to the coming weeks. Alongside the launch of CPMS 2.0 within this year, we will welcome new Collaborative Partners and thus expand our network. We are excited!

With the best regards

The ERN RARE-LIVER Office Team

Meetings/Conferences 2025

[ERN RARE-LIVER Members Meeting 2025](#)

We want to draw your attention to our **Members Meeting in Barcelona, Spain on 23 + 24 January 2025.**

Participation is limited to two representatives per member centre, including affiliated partners. One of the participants can be funded per centre, according to EC regulations (EU unit costs).

Additionally, we will fund one lead of each disease working group to attend and present on behalf of their group.

The deadline for registration is 4 November.

We are looking forward to seeing you in Barcelona.



[ERN RARE-LIVER Academy 2025](#)



The university hospital of Heidelberg is dedicated to advancing the understanding and treatment of rare liver diseases and the complex

The ERN RARE-LIVER EASL ACADEMY 2025 will be hosted on 10-11 April by the university hospital of Heidelberg and will be on rare liver diseases with a special focus on translating clinical practice guidelines in real-life care.

challenges in real-life care for patients with rare liver diseases.

Registration is open until 13 November.

For more information on the focus, preliminary programme and speakers, please visit our website to stay up to date: ERN RARE-LIVER Academy.

Meetings/Conferences 2024

ERN RARE-LIVER Online Members Meeting 2024

We had a very constructive and enjoyable Members Meeting with you and thank you very much for your attention and participation. In addition to presentations on R-LIVER, the European Patient Advocates (including the Youth Panel), CPMS, some of the ERN Disease Working Groups spoke about their projects and meetings.

The virtual meeting ties in with our Members Meeting on 23-24 January 2025 and closes our circle for the year. Many thanks to all participating speakers.

During the meeting, five new collaborative applications were also presented to the members, which have now been chosen by the majority of votes by our full and affiliated partners.

These five new Collaborative Partners of ERN RARE-LIVER are:

- *Medical University of Innsbruck, Austria*
- *Ukrainian Centre of Maternity and Childhood, Ukraine*
- *University of Florence - Clinical Gastroenterology Careggi, Italy*
- *University hospital Brussels, Belgium*
- *Marques de Valdecilla University Hospital, Santander, Spain*

Virtual Meeting of the ERNs Coordinators Group

On 21 and 22 October 2024, the 21st meeting of the Coordination Group of



the European Reference Networks (ERNs) took place as well as the 29th meeting of the ERNs Board of Member States. The main topics discussed included the collaboration with different types of members, updates on CPMS 2.0 as well as the ERN registries.

Many of these issues are more complex than one would wish and think, but it is good to see that the commitment to developing ERNs successfully is very strong on all sides, including the European Commission itself and its board of member states.



ePAG Lead/Board Representatives Meeting in Paris 2024

On 24 and 25 October, the ERN RARE-LIVER ePAG team (Leads and Board Representatives) met to discuss future projects and structuring of the patient representatives. The main topics included participation, transparency in communication, the exchange between the ERN Office and the patient representatives as well as the patient representatives amongst each other.

CPMS



CPMS 2.0

Clinical Patient Management System

Following further delay of transitioning between the current CPMS system to CPMS 2.0, we have been informed that the 3rd release will be between November and December 2024.

We will restructure the CPMS page on the website, including the new changes. One focus will be on the CPMS Live Discussions that we are establishing. Also, the CPMS 2.0 upcoming online training dates will be available online.

In this context, we are also pleased to announce the following new CPMS Helpdesk Members:

- **Valeria Perez Campuzano**, Hospital Clínic de Barcelona (Spain)
- **Salvatore Fasano**, Hospital Beaujon Paris (France)
- **Eliezer Zahra Bianco**, Mater Dei Hospital (Malta)
- **Mikkel Malham**, Rigshospitalet University of Copenhagen (Denmark)

Next to the current members of the CPMS Helpdesk, Marcial Sebode, Katharina Moll, Ida Schregel, Nagham Issa and Jemina Schrodt (University Medical Center Hamburg-Eppendorf) the team is thereby complete.

At the Members Meeting in January, the ERN RARE-LIVER CPMS Helpdesk will be available to provide assistance and to answer questions in person concerning CPMS 2.0.

New Webinars

We are pleased to announce the next 3 webinars at ERN RARE-LIVER, one of them in December this year, the other two are scheduled for January and February 2025:

- **Contraception & Fertility in Rare Liver Diseases** (17 December 2024, 5pm (CET)) – Leona Dold, Willy Visser, The ERN RARE-LIVER Youth Panel
- **Hemochromatosis: update on diagnosis and patient management starting from the EASL CPG** (18 January 2025, (CET)) – Heinz Zoller, Elena Corradini, Antonello Pietrangelo
- **Novel developments in alpha1-antitrypsin deficiency-associated liver disease** (18 February 2025, (CET)) – Pavel Strnad, Malin Fromme, Piotr Socha

If you are interested to host a Webinar or contribute a talk in a Webinar, please talk to Juan Carlos García-Pagán from the Hospital Clínic de Barcelona (JCGARCIA@clinic.cat).

We would also like to draw your attention to an EASL DeepDive webinar happening next week:

Please register [here](#).

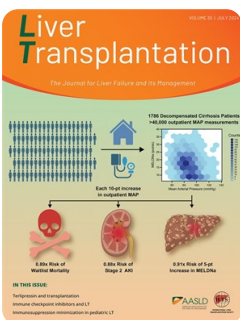
See past/upcoming webinars

Scientific News/Publications



Rare primary liver cancers: An EASL position paper

The diagnosis and treatment of very and ultra-rare primary liver malignancies can be challenging. A group of international experts have worked together to gather available knowledge on these tumours. They have written a position paper with clear advice from diagnostic and therapeutic points of view. The combined EASL and ERN Rare Liver effort can be found in the Journal of Hepatology. You can access the paper also via the [ERN Website](#).



Immunosuppression minimization is safe and associated with good long-term success in pediatric recipients of liver transplants

In this single-centre retrospective study from the US the authors analyzed the success rate and predictive markers for the successful reduction of immunosuppression in 28 eligible pediatric patients after liver transplantation. Immunosuppression was reduced from twice-daily to once-daily calcineurin inhibitor dosing. About 60% of patients had a long-term success after reduction of immunosuppression with a median follow-up of about 5 years. Predictive for the success were lower AST levels within normal range prior to reduction of immunosuppression. Those patients who failed and showed increasing liver enzymes during follow-up, returned to twice-daily dosing of calcineurin inhibitor dosing

and no serious events occurred. This study supports that reduction of immunosuppression is safe in pediatric patients after liver transplantation, but an international, multi-centre registry is needed to better identify eligible patients and predictive biomarkers. Thereby individualised approaches for immunosuppression after liver transplantation with less side effects can be offered to our patients.



Porto-sinusoidal vascular liver disorder with portal hypertension: Natural history and long-term outcome

Porto-sinusoidal vascular liver disorder (PSVD) is a rare liver condition that often leads to severe complications from portal hypertension and can reduce life expectancy. This study, which involved several ERN-Rare Liver centers and the largest cohort of PSVD patients to date, has identified that although PSVD typically has a favorable prognosis, factors such as the severity of the underlying associated condition, presence of ascites, age, and levels of bilirubin, albumin, and creatinine are linked to poorer outcomes. By integrating these parameters, we developed a nomogram that effectively predicts prognosis with strong discrimination and calibration capabilities.

Other Announcements

Ocaliva for PBC - FDA Advisory Panel Voting

After the European Medicines Agency (EMA) recommended revoking Ocaliva's marketing authorization on 28 June 2024, a medicine provisionally authorised since 2016 for the treatment of adults with primary biliary cholangitis (PBC). On 13 September, the FDA Advisory Panel held a hearing on the role of Ocaliva in PBC.

The Gastrointestinal Drug Advisory Committee voted 13 to 1 with no abstentions that the benefits of Ocaliva on clinical outcomes in patients with PBC could not be verified with the available data. They also voted 10 to 1 with 3 abstentions

that obeticholic acid (OCA) did not have a favorable benefit-risk assessment for use as a second-line treatment in the United States.

These votes are advisory and not binding. The formal decision on the status of Ocaliva by the FDA is pending.

For more detail you can find the detailed material of the meeting [here](#) or rewatch the Expert Discussion Panel on [YouTube](#).



ERN RARE-LIVER Study - Survey for people with PSC, PBC or AIH

Social determinants of health (SDOH) are the conditions in which people are born, grow up, work and age. Studies suggest that SDOH influence the course of liver disease. However, this has not yet been studied in patients with autoimmune liver disease. With this study in cooperation with centers of the European Reference Network for Rare Liver Diseases (ERN RARE-LIVER) **the connections between SDOH and autoimmune liver diseases** are to be investigated. The survey is available in 9 different languages.

Please distribute the link for the study amongst your patients: [EUSurvey - Survey](#).



Save The Date – Scientific Liver Meeting 2025
**7th International Conference
On Pediatric Liver Disease**

20.-21.02.2025

Registration available soon!
www.cpld-conference.com

University Transplant Center
Department of Pediatrics
Department of Visceral Transplantation
Department of Pediatric Surgery

Save The Date – Scientific Liver Meeting 2025 **7th International Conference On Pediatric Liver Disease**

The University Medical Center Hamburg-Eppendorf will host its 7th International Conference on Paediatric Liver Disease on 20-21 February 2025. This year's symposium will focus biliary atresia, primary and secondary sclerosing cholangitis, Wilson's disease and liver transplantation. Interactive case discussions will contribute to an exciting program.

The programme can be viewed [online](#) and **registration is open now.**

ERN Webinar online - How to communicate with adolescents and young adults

Following our successful webinar on 17 September, the video is now available on [EASL Campus](#) and can be viewed there. Involved were Janne Suykens (Ghent University Hospital), Jemma Day (King's College, London), Dr n. med. Maciej Janik (Medical University of Warsaw) and Prof. Dr Ruth De Bruyne (Ghent University Hospital), as well as the ERN RARE-LIVER Youth Panel.



Visit us on our website



ERN RARE-LIVER

University Medical Centre Hamburg-Eppendorf, Martinistraße 52, 20251, Hamburg
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