

# February 2024



European  
Reference  
Networks



## Newsletter #14

### Welcome to our next newsletter!



**Dear members and friends of the ERN RARE-LIVER,**

It was fantastic to have so many of you attending the Online Members' Meeting on 8 February and to hear of so many activities of the various working groups. The network has been thriving, is thriving, and will continue to thrive! That is great.

The past few months have very much been dominated by the many adaptations of our new grant application that the European Commission required. I will not bother you with the details, but I want you to at least have some feeling for the complicated procedure that is taking place in order to receive continued funding. I understand that certain rules need to be followed and that the political landscape as well as the legal restrictions especially concerning the cooperation with

Ukraine, can be challenging, but the amount of detail required, and most importantly the delays caused by all these steps is really worrying.

Our bridge grant, which was meant to carry the ERN into the next funding period, ended originally in August, was then extended, without extra money, until the end of September, and thus since 1 October the ERN RARE-LIVER and all the other 23 ERNs are without one cent of EC money, and are depending on the pre-financing by the hosting medical centre, i.e. for us the University Medical Centre Hamburg-Eppendorf, to continue paying the salary of our staff and all other running expenses, because the final grant agreement and the subsequent transfer of funds has been so much delayed.

**Or maybe better more positively:** I am extremely grateful to my hospital leadership for the continued support, and to the ERN office team for the hard work to keep ERN RARE-LIVER running despite all these uncertainties.

This, I can assure you, is the end of critical comments in this newsletter, and now we will move forward again:

- the **new grant** will come, and it will give us some security up until the autumn of 2027. It will probably be easier to manage, as it will include all activities in one single grant, including the basic registry funding.

- the new grant will not all go to the central management team in Hamburg, but will also fund specific work packages at other member sites: Ruth de Bruyne will be responsible for transition and patient empowerment and funding will go to Ghent University; Juan Carlos García-Pagán will be responsible for training and funding will go Barcelona; Aurélie Plessier will be responsible for guidelines and funding will go to Paris; and Marianne Hørby Jørgensen will continue to be responsible for paediatric co-leadership and funding will go to Copenhagen. I am very grateful to these colleagues for taking up these responsibilities and helping to move ERN RARE-LIVER forward.

- the **Hamburg office team will grow**, with the help of some additional local funding. As of 1 March Jemina Schrodtt, who as a student already worked in the nephrology ERN ERKNet, after completing her master's degree is now joining our team, and while we will lose Juliane Gaenge to a consulting company, we will be joined by Kari Klein in June, an experienced project manager who has worked in other rare diseases in both the US and Australia in the past. I am delighted to see these new members of our team.

Even though the Hamburg team is primarily busy with the everyday running of the network, and the many management and reporting issues with the EC, they are also here for you - as the ERN RARE-LIVER is all of you. We move forward on the basis of your ideas, activities and projects. Do not hesitate to contact us - we need you, all of you, to fulfil our ambitious mission: improved patient care for ALL rare liver disease patients in Europe.

Yours with best wishes  
Ansgar

[Contact Office Team](#)

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# New release:

## ERN RARE-LIVER registry video for patients

ERN RARE-LIVER has established a multicentre European patient registry called R-LIVER. The registry addresses a major issue with rare liver diseases which is the lack of data and the fact that data are fragmented and scattered across different countries.

This new video not only introduces the registry and its benefits, it also explains to patients how it works and what advantages their participation in the registry entails.



[Download on YouTube](#)

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## CPMS Clinical Patient Management System

# DO YOU NEED ASSISTANCE OR SUPPORT WHEN USING CPMS?

The team is happy to help. You can contact us by email:  
[ERN.RareLiver@uke.de](mailto:ERN.RareLiver@uke.de)

University Medical Centre Hamburg-Eppendorf: *Marcial Sebode, Juliane Gaenge, Gustav Buescher, Ida Schregel*  
Radboudumc, Nijmegen: *Romée Snijders, Renée Duijzer*  
Copenhagen University Hospital - Rigshospitalet: *Mikkel Malham*



ERN RARE-LIVER was always leading all ERNs in the use of the CPMS patient consultation system. However, a negative trend worries us and is probably also observed by the EC: it seems that our leading position with regard to CPMS activity is in danger. Although our network is growing in number of centres and individuals, we detect a constant negative trend of active CPMS users and rate of active CPMS panels per month in the previous 6-12 months.

CPMS activity is essential for every ERN and is an important indicator for the success of our ERN, which is also measured by the EC. Therefore, we want to urge you to become more active, and in addition to yourselves to identify fellows at your centre who will be responsible for your CPMS activity.

The CPMS helpdesk offers help and can organize online-trainings how to get a CPMS account and how to set up a panel to discuss a case.

Please contact the ERN office if you would like to get in contact with the CPMS helpdesk.

[Contact Office](#)

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**In memoriam:  
Hanns-Ulrich Marschall (1954-2023)**



One of the first supporters of the ERN RARE-LIVER, and a most sincere and knowledgeable expert in many rare liver diseases and in the specific challenges of liver diseases in pregnancy as well as pregnancy in liver disease, **Hanns-Ulrich Marschall**, who was also the ERN representative of Sahlgrenska University Hospital in Gothenburg, is dearly missed after losing his long fight against cancer last summer.

He will be remembered by many of us, and we will miss him.

The Journal of Hepatology published a worthy obituary.

[Read obituary](#)

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## SAVE THE DATE: DINNER AT EASL CONGRESS IN MILAN



Once again this year, we would like to invite our members and friends to a special dinner meeting on **6 June at 7:00 pm** as part of the EASL conference in Milan to have time for dialogue within our network.



The exact location has not yet been finalised. But we will of course keep you informed.

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## New Members of our Network:

### 3 new Collaborative Partners approved: Madrid, Amsterdam and Moldova

At the Online Members' Meeting on 8 February, three new medical centres that had applied to become Collaborative Partners were approved by a vote of the 115 members present at the meeting.



The **Hospital General Universitario Gregorio Marañón** in Madrid, Spain, was approved with Rafael Bañares as lead doctor. The clinical practice area of this University Hospital covers adult care.

[Visit Hospital Website](#)



The **Amsterdam University Medical Centre** in Amsterdam, the Netherlands, has also been accredited with Joost Drenth as lead doctor and adult care as clinical practice area.

[Visit Hospital Website](#)



We are also pleased to welcome **Nicolae Testemițanu State University of Medicine and Pharmacy** in Chișinău, Molodva, as a Collaborative Partner and to be able to support a centre outside Europe. This centre covers both areas adult and paediatric care with lead doctor Adela Turcanu.

[Visit Hospital Website](#)

## MEMBERS' NEWS

### **Fredrik Åberg is the new ERN RARE-LIVER representative for the Helsinki Member Centre in Finland**

After several successful years of joint work, **Martti Färkkilä** from Helsinki University Central Hospital stepped back and handed over his position as Representative for the Helsinki Members Centre to **Fredrik Åberg** whom we warmly welcome.



"I am from Helsinki, Finland. I have studied and received my degree in medicine at University of Helsinki and I am working as a hepatologist at the Transplantation and Liver Surgery unit at Helsinki University Hospital. I used to work 3 years at the Transplant Institute of Sahlgrenska University Hospital in Gothenburg, Sweden, before I returned to Helsinki 3,5 year ago.

I was officially appointed our hospital's representative in ERN RARE-LIVER just before Christmas in December 2023.

As a transplant hepatologist, I see many types of rare liver diseases in my practice. Personally, my main interest in the field of rare liver diseases is

vascular liver disease.

My research interest includes short- and long-term outcomes of liver transplantation, liver epidemiology and risk prediction models, and we have also published on rare diseases like portal vein thrombosis, Budd Chiari syndrome, and Wilson's disease.

The plan right now is to try to increase the activity of HUS (Helsinki University Hospital) within the ERN RARE-LIVER network by getting more colleagues involved in the various working groups. I think this is the first important step to build a stronger commitment and expertise within the rare liver disease field. I am hoping we can contribute more actively to the network within the coming years."

The ERN RARE-LIVER community is happy to welcome you to our network.

Info Helsinki University Hospital

## News from the working groups

### New patient leaflet:

### Tips on how to take medication



The image shows a patient leaflet with a light blue background. On the left is the ERN RARE-LIVER logo, which includes the text 'European Reference Network on Hepatological Diseases'. The main title is 'Reasons why to take medication regularly' in a large, bold, dark blue font. Below the title is the subtitle 'Tips for young patients'. At the bottom, there is a white box containing the text 'TIPS TO INTEGRATE THE MEDICATION IN DAILY LIFE' and 'Routines' followed by two bullet points: '• Try to take your pills at the same times every day.' and '• Try to associate taking your medication with an activity of your daily routine. For example: when you get out of bed, or when you start cooking.'

In cooperation with the **Wilson's working group** and the **ERN Youth Panel**, our ERN RARE-LIVER members **Aida Regi Cosculluela** from the Spanish Wilson Association and **Dr. Wiebke Papenthin** from Morbus Wilson e.V. in Berlin developed a patient-focused guide how to take medication regularly and how to integrate medication into daily life.



The guide offers help by suggesting that the patients get into the habit of establishing a certain routine, setting up reminders and coming up with their personalised reward system.

**You will soon be able to download this leaflet on our website.**

A big thank you for this valuable initiative.

[Our Working Groups](#)

## UPCOMING EVENTS

### **Workshop on cross-border Healthcare, Patients' Rights and Rare Disease on 29 February in Valetta, Malta**



In this workshop, the Ministry for Health of Malta invites a wide audience including people from Ministries, NGOs, National contact points on cross-border healthcare, European Reference Networks (ERNs) and beyond. The objective of this event is to raise awareness of patients' rights on cross-border health, ERNs, and rare diseases.

This workshop is being organised in collaboration with the European Commission and is being held under the auspices of the President of the Republic of Malta, Dr George Vella. Foreign and local speakers, including individuals with rare diseases, will be coming together to share their personal experiences and provide updates on these important, under-recognised aspects of health care.

**Registration is free of charge.**

[Register here](#)

# Conference on Fontan-associated liver disease

## FONTAN ASSOCIATED LIVER DISEASE

7th March 2024, 15.00-17.00 CET

Organizes: Paediatric Liver Transplant Unit, Hospital Vall d'Hebron, Barcelona, Spain.

### **Physiopathology related to univentricular heart disease.**

**\* Dr. J. Sanchez de Toledo, Assistant professor of critical care medicine and pediatrics, University of Pittsburgh.**

### **Liver disease in patients undergoing Fontan's Procedure.**

**\* Dr. J. Quintero, Responsable Hepatologia Pediàtrica, Hospital de la Vall d'Hebron, Barcelona.**

### **Screening, Management and Transplant Consideracions.**

**\* Dr. Juliet Emamaullee, Associate Professor of Surgery and Transplant Surgeon, University of Southern California, Children's Hospital-LA LA (USA).**

Videoconference available on : [click here](#) (code 648234)



On **7 March 2024**, 15:00 – 17:00 CET, a panel of international speakers will show different perspectives (senior intensivist, cardiologist and surgeon) on Fontan-associated liver disease. This conference is organised by the Paediatric Liver Transplant Unit of the Hospital Vall d'Hebron in Barcelona, Spain.

Fontan-type surgery is the final step in the palliative surgical treatment of infants born with a single heart ventricle. It carries long-term haemodynamic changes resulting in liver damage, leading to Fontan-associated liver disease (FALD). It is estimated that 1/10.000 children are born with this condition expecting that more than 70 million people worldwide will be affected in 2023. It translates into a real challenge for patients and physicians, often leading to a multidisciplinary approach in highly specialized healthcare institutions.

You can find the registration link on our website.

[More Information on the Website](#)

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## 3rd Wilson Aarhus Symposium 2-5 May 2024



# Wilson Aarhus

We would like to draw your attention on the **3rd Wilson Aarhus symposium** for a review of the latest clinical and translational research in Wilson disease. The Wilson Aarhus 2024 symposium will be an up-to-date, evidence-based, clinically orientated symposium with distinguished international faculty. Basic, clinical and translational research work by junior investigators will also be presented.

As part of the Aarhus Wilson Meeting, **2-5 May 2024**, a discussion about the registries in Wilson disease will be included. The key question will be to define the core data that should be included in every registry and how to maintain the registry for the long run.

The ERN RARE-LIVER will contribute in the discussion about registries' legal issues and practical problems.

**Please note:**

The symposium is for active researchers and clinicians working on Wilson disease, and there is a limited number of seats.

You can find the programme and the registration form on our website.

[More Information on the Website](#)

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## 5th Autoimmune Hepatitis Research Workshop on 27 & 28 June 2024

ERN RARE-  
LIVER

### 5th Autoimmune Hepatitis Research Workshop

Where: Hannover, Germany,  
When: 27 and 28 June 2024

Find more information on our [website](#).

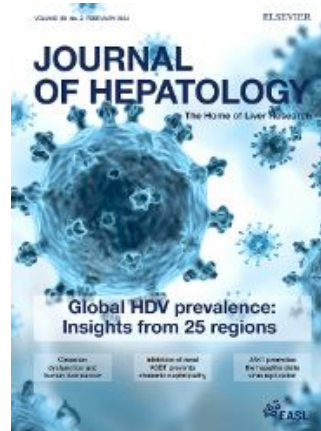
This in-person workshop will focus on advancing research in AIH, strengthening existing networks and building new ones. Thematically, the workshop will continue previous paths with updates on ongoing clinical projects, emphasis on diagnostic and predictive improvements, and the understanding of AIH pathogenesis.

Following the successful initiation during the previous workshop in Maastricht 2022, a dedicated session will focus on the patient's perspective. To learn from researchers with a background other than autoimmune liver diseases, there will also be keynote lectures and a dedicated workshop focusing on data curation and AI approaches.

[More Information on the Website](#)

## Scientific News

### **An open-label randomised-controlled trial of azathioprine vs. mycophenolate mofetil for the induction of remission in treatment-naive autoimmune hepatitis**



The findings of the CAMARO trial, now published in the Journal of Hepatology, present a randomised-controlled trial directly comparing azathioprine and mycophenolate mofetil, both administered with prednisolone, in the context of inducing biochemical remission among seventy treatment-naive patients with autoimmune hepatitis.

Patients assigned to the mycophenolate mofetil group reached biochemical remission at 24 weeks more often (56.4% vs. 29.0%,  $p = 0.022$ ). Moreover, azathioprine use was associated with more (serious) adverse events, resulting in treatment discontinuation for 1 out of 4 patients ( $p = 0.018$ ).

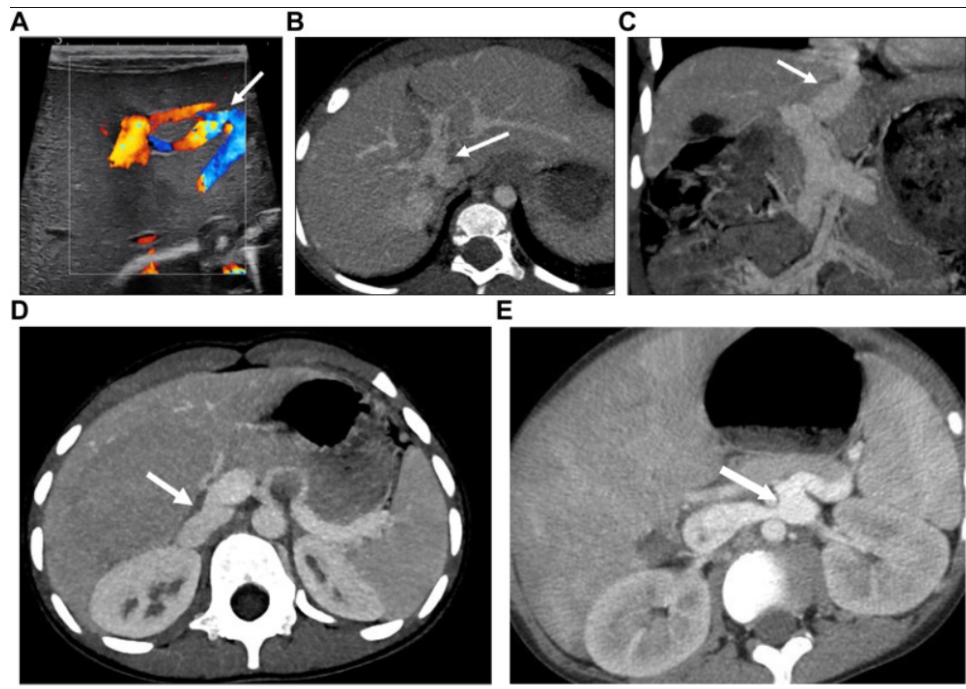
This trial provides important insights, and raises at the same time many new questions, as commented in the accompanying editorial by Gideon Hirschfield and Ansgar W. Lohse.

[Read Publication](#)

[Read Editorial](#)

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## New Position Paper on Congenital Portosystemic Shunts



Congenital portosystemic shunts are often associated with systemic complications, the most challenging of which are pulmonary hypertension, endocrine abnormalities, and neurocognitive dysfunction.

In this new position paper, expert clinical guidance is provided on the management of these systemic complications and recommendations are made regarding shunt closure and follow-up.

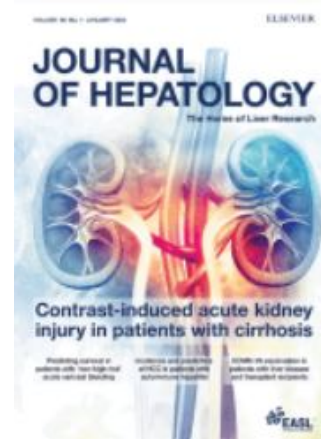
The ERN RARE-LIVER Community thank her external expert **Valérie Anne McLin** who is the first author of this valuable paper.



## Incidence and predictors of hepatocellular carcinoma in patients with autoimmune hepatitis

Autoimmune hepatitis (AIH) is a rare chronic liver disease of unknown aetiology; the risk of hepatocellular carcinoma (HCC) remains unclear and risk factors are not well-defined.

The authors aimed to investigate the risk of HCC across a multicentre AIH cohort and to identify predictive factors.

[Read Publication](#)

The results of this study were discussed in an interesting editorial by our Aarhus ERN representatives Drs. L and H. Grønbæk.

[Read Editorial](#)

## PD-1/PD-L1 immune checkpoint therapy demonstrates favorable safety profile in patients with autoimmune and cholestatic liver disease



Immunotherapy with immune checkpoint inhibitors has become an important therapeutic pillar in many malignant diseases. However, the safety profile of

checkpoint inhibitors for patients with concomitant autoimmune and cholestatic liver diseases (PSC, PBC, AIH) has not been analysed yet.

In a joint effort by multiple ERN RARE-LIVER members, the tolerability of these treatments was demonstrated in a multicenter retrospective study.

Future prospective work will focus on patients with autoimmune hepatitis to clarify remaining uncertainties regarding the safety of these therapies.

[Read Publication](#)

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**European Reference Network for hepatological diseases  
ERN RARE-LIVER**

**Universitätsklinikum Hamburg-Eppendorf, I. Medizinische Klinik  
und Poliklinik Zentrum für Innere Medizin, Hamburg**

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