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WELCOME TO OUR NEXT NEWSLETTER

We're almost halfway through 2024 - and for ERN RARE-LIVER, it's time to look back, but also forward. Due to some personnel changes in the ERN office, we would like to start by introducing ourselves as a team. Alongside Hendrik Matthies and Caren Ramien, Nagham Issa, who had been supporting the team as a student assistant since April 2023, has now officially joined the team as a Project Manager. Additionally, we welcome Kari Klein and Jemina Schrodt both joining us as Project Managers. Sabina Bernhardt, our Communication Manager, whom we are very grateful for her contributions to the team during the past two years, will be leaving us in the summer.

We are looking forward to getting to know you better and connect with you on various levels. Do not hesitate to contact us for any inquiries or questions.

With the best regards
The ERN RARE-LIVER Office Team

[Contact the Team](#)

THE TEAM



HENDRIK MATTHIES

As a Project Manager with a background in physiotherapy and HR management, I oversee grant management, finances, meetings, and training activities in the ERN.



CAREN RAMIEN

As Scientific Officer, with a background in translational science, I support the research collaboration within the network. I am involved in the work on clinical practice guidelines with Aurélie Plessier, and keep an eye on additional funding options.



NAGHAM ISSA

As a Project Manager with a background in community nursing and public health, I oversee the R-LIVER registry and CPMS. My role involves communicating with our various partners, supporting different disease working groups and I am also part of the Patient Pathway Initiative.

THE TEAM



KARI KLEIN

Originally from Florida, USA, with a background in Psychology and Behavioural Health studies. I look forward to contributing to the team as a Project Manager with a focus on collaboration, high integrity and innovative solutions.



SABINA BERNHARDT

As a Marketing & Communications Manager with a background in journalism and PR, my main task is to increase the visibility of the ERN RARE-LIVER and to assist all members and partners with advice and creativity in order to identify and satisfy the respective needs.

JEMINA SCHRODT

As a Project Manager with a background in organisational development, I am mainly responsible for our patient representatives, CPMS and communication with our member centres.

MEETINGS/CONFERENCES 2024

ERN RARE-LIVER Academy 2024

In March, we had an excellent ERN RARE-LIVER Academy with the title “Growing up with liver disease”, which took place from 14-15 March in Copenhagen. We were able to bring together more than 20 young fellows with a special interest in paediatric and adult hepatology, exchange experience and network. The Academy also gave us the opportunity to report on our activities at ERN RARE-LIVER and to gain some new members in the Disease Working Groups. Thanks again to Marianne Hørby Jørgensen and Jon Nielsen (Rigahospitalet in Copenhagen, Denmark), as well as to Marianne Samyn (Kings College Hospital, London, UK) for the excellent organization, and to the whole faculty for their valuable contributions. There will be another ERN RARE-LIVER Academy in 2025, and we are calling for teams who wish to organize and host this event to apply to the office with their concepts and proposals by 15 July.

The call for the ERN RARE-LIVER EASL Academy 2025 is out! Apply for your centre if you are interested. The deadline for applications is 15 July 2024, and the organiser will be announced in September 2024. For more information click on the [link](#).



ESPGHAN 2024



In May, ERN RARE-LIVER was represented at ESPGHAN 2024 in Milan, Italy. At our booth in the exhibition area, we exchanged ideas with interested clinicians, patient associations and other networks. Two meetings in which ERN RARE-LIVER was involved at ESPGHAN were particularly enriching for us.

In our **ERN Paed Meeting** on 15 May we were able to discuss the structures and strategies of the ERN with a focus on paediatric impact. Accompanied by small impact presentations (e.g. about R-LIVER, projects in AIH, PSC and pALF), there was an extensive discussion about how paediatrics in ERN RARE-LIVER will continue to develop over the next 4 years.

Another highlight was the **ESPGHAN-ERN RARE-LIVER Symposium**. One focus of this meeting was the transition of young people with rare liver diseases from pediatric to adult care. In addition to a number of specialised presentations, Silas Sørensen spoke as a representative of the ERN RARE-LIVER Youth Panel and reported on his experience as a young patient. Thanks again to Silas for these moving and important 20 minutes! The voice of our young patients must and will be heard!

ERN Coordinators Meeting

Strategic considerations and analyses for all the 24 different ERNs were further discussed at the Coordinators Meeting 2024 in Luxembourg from 21-22 May. Every year, the coordinators of the 24 ERNs for rare diseases meet with DG Santé of the European Commission and representatives of the Board of Member States to evaluate the status quo of the ERNs and to discuss necessary next steps. Topics on the agenda included e.g. the future of the registries, further steps regarding Clinical Practice Guidelines (CPGs), possible ERN exchange programs and the different kind of ERN memberships. It is clear that much work remains to be done on the political, structural and scientific fronts, but all parties are united together with the same goal to improve the care of patients with rare and complex diseases. It is extremely encouraging to see that the EC, the national health ministries and the ERNs are united as they strive for better care for rare disease patients.



EASL 2024



We are very pleased to be exhibitors at EASL 2024 from 5-8 June in Milan. Please visit us at our booth/reception. For more information, contact our team or follow our social media!

CPMS



European
Commission

CPMS 2.0

Clinical Patient Management System

At the Coordinators Meeting the new version of the **CPMS** system was discussed, and we would like to give you a brief update on this development. CPMS is one of our tools to support clinicians in treating patients in a case-specific and accurate manner. Currently the platform is in transition to a more user-friendly system, **CPMS 2.0**. CPMS 2.0 will be easier to access, easier to use and more flexible. The trial versions of CPMS 2.0 will be launched soon, and by November 2024 there will also be a Mobile App available to facilitate collaborative working on panels.

Irrespective of the system changes, we want to continue to emphasize the benefits of CPMS for those within our network who are not yet using CPMS. Over the past few months, dozens of new panels have been created, were discussed and 'solved' by experts within hepatology all across Europe, providing the highest quality cross-border health care to our patients. Those of you who have not yet used the CPMS system, you are missing an important tool. The start of CPMS 2.0 should be your start into this consultation platform!

If you have any questions regarding the use of CPMS, please contact our CPMS Helpdesk Team, which consists of Marcial Sebode, Naghm Issa, Ida Schregel, Katharina Moll and Jemina Schrodt. We especially welcome our hepatology fellow Katharina Moll to our CPMS Helpdesk, who will be the successor of Gustav Buescher. Gustav Buescher will leave the team at the end of June – a special gratitude and thanks to him for all his hard work and commitment to CPMS.

Moving from the present CPMS to CPMS 2.0 will require some preparation on our side: all open panels will need to be completed and closed, and once that has been completed, we will move to the new system – we will keep you posted on this in a separate message as soon as we are ready.

CPMS ERN RARE-LIVER

NEW WEBINARS

We are pleased to announce specific dates for 3 webinars in the coming weeks and months:

- **Liver cyst infection - the feared complication of cystic liver disease** (25 June 2024, 5pm (CET)) - Marten Lantinga, Joost Drenth, Pavel Strnad, Anouar Azou
- Webinar on **EASL Clinical Practice Guidelines on the management of liver diseases in pregnancy** (9 July 2024, 5pm (CET)) - Willy Visser, Catherine Williamson, Jan Philipp Weltzsch, Leona Dold
- **Transition of healthcare: Communication webinar** (17 September 2024, 5pm (CET)) – Janne Suykens, Jemma Day

Please let us know, if you are interested to host a Webinar or contribute a talk in a Webinar – we will be pleased to review your application and include it in our educational programme.

[See Past/Upcoming Webinars](#)

SCIENTIFIC NEWS/PUBLICATIONS



The NEW ENGLAND
JOURNAL of MEDICINE

Efficacy and Safety of Elafibranor in Primary Biliary Cholangitis

In a multinational, phase 3, double-blind, placebo-controlled trial 161 patients with primary biliary cholangitis who had an inadequate response to ursodeoxycholic acid were randomly assigned to receive elafibranor or placebo (2:1 ratio). Elafibranor, a dual PPAR α and δ agonist, significantly improved biochemical markers of cholestasis (primary endpoint) compared to placebo, with 51% of patients achieving a biochemical response versus 4% in the placebo group. Normalization of alkaline phosphatase levels occurred in 15% of the elafibranor group versus none in the placebo group. However, no significant difference in pruritus intensity change was observed between the two groups. Adverse events such as abdominal pain, diarrhea, nausea, and vomiting were more common with elafibranor. This trial suggests elafibranor's potential as a treatment option for primary biliary cholangitis.

A Phase 3 Trial of Seladelpar in Primary Biliary Cholangitis

In a phase 3 double-blind, placebo-controlled trial 193 patients with primary biliary cholangitis who had an inadequate response to ursodeoxycholic acid were randomly assigned to receive seladelpar or placebo (2:1 ratio). Seladelpar, a peroxisome proliferator-activated receptor delta agonist, significantly increased the percentage of patients achieving a biochemical response compared to placebo (61.7% vs. 20.0%). Normalization of alkaline phosphatase levels also occurred more frequently with seladelpar (25.0% vs. 0%). Seladelpar demonstrated a greater reduction in pruritus intensity among patients with moderate-to-severe pruritus at baseline. Adverse events were reported at similar rates between the seladelpar and placebo groups.

SCIENTIFIC NEWS/PUBLICATIONS



Recurrent splanchnic and extrasplanchnic thrombotic events in patients with non-cirrhotic portal vein thrombosis associated with local factors

This study examines factors linked to new thrombotic events in patients with recent non-cirrhotic portal vein thrombosis (NCPVT) due to local factors, and the effect of stopping anticoagulation. In 154 patients, 53.9% had prothrombotic factors at baseline, with 32.5% high-risk. Over a median follow-up of 52 months, 17 new thrombotic events were observed. High-risk factors increased the likelihood of new events, while recanalization and anticoagulation decreased it. Long-term anticoagulation proved beneficial, particularly for high-risk patients, reducing new thrombotic events significantly. The study highlights the need for systematic screening for prothrombotic factors to support long-term anticoagulation therapy in NCPVT.



Deleterious variants in TNFAIP3 are associated with type II and seronegative pediatric autoimmune hepatitis

Pathogenic variants in the TNFAIP3 gene, which encodes the A20 protein, are linked to an early-onset, autosomal dominant autoinflammatory disease. This study describes four pediatric patients from three families, all presenting with autoimmune hepatitis (AIH) and immune activation profiles due to TNFAIP3 mutations. The clinical features included mucosal ulcerations, rash, uveitis, and polyarthrititis. Genetic mutations were identified through exome sequencing, and RNA analysis showed elevated type I interferon response genes during active disease phases. The cases varied from acute liver failure to chronic hepatitis, and treatment with corticosteroids and immunosuppressants led to biochemical remission. However, extrahepatic symptoms persisted, prompting the use of JAK inhibitors, which proved to be effective. This study points to mechanisms of pathogenesis in autoimmune hepatitis and opens up new therapeutic avenues. TNFAIP3 genetic testing in children with seronegative or type II AIH should be performed. Tailored treatment approaches may be necessary for managing both hepatic and extrahepatic symptoms of such cases.

FUNDING OPPORTUNITIES

Funding opportunity for clinical exchange!

The UEG (United European Gastroenterology) is offering clinical visiting fellowships to young doctors. You can apply to visit a European centre of excellence from a given list of hosts including a number of ERN RARE-LIVER members. This programme allows fellows to establish new avenues for clinical and scientific collaboration, develop additional professional skills, and expand their knowledge.

UEG will grant 30 fellowships of 1250 € to contribute to travel and accommodation costs for two week visits and allows for additional funding. The application for clinical visits in 2025 is open until June 27th, 2024. For more information and the list of host centres, check the [UEG website](#).

OTHER ANNOUNCEMENTS

Acceptance of the COST ACTION EURO-VALDI-NET

Congratulations to Pierre-Emmanuel Rautou and colleagues from the vascular liver disease group for their successful COST Action application!

134 researchers from universities, hospitals, companies, NGOs and patient organizations in 27 countries put together the EURO-VALDI-NET project on vascular liver diseases. The project including many ERN colleagues, but also colleagues from Israel, Turkey, Egypt and Lebanon, will now be funded for 4 years.

EURO-VALDI-NET aims to create a pan-European multidisciplinary co-operative network of stakeholders, bringing together scientists, clinicians, industry partners, and patients associations. Through the creation of shared data registries on main relevant basic and clinical aspects, conference calls, meetings, workshops, as well as training schools, this Action will coordinate efforts aiming at advancing the understanding of vascular liver diseases to translate basic research and preclinical findings into clinical practice. Nine working groups will work together in order to increase knowledge on vascular liver diseases and improve recognition and management of affected patients.

Change of ERN Representatives

Birmingham Children's Hospital, UK

We would like to announce that **Girish Gupte** has taken on the role of Centre Representative at Birmingham Children's Hospital, one of our Collaborative Partners. Many thanks to former Centre Representative Deidre Kelly for her fantastic long-time support and co-operation. We will miss her – but will be sure, she will continue to support our endeavour with her enormous knowledge and energy, her sharp thinking and her warm heart!

Institute for Clinical and Experimental Medicine and Motol University Hospital Prague, Czech Republic

At our member centre in the Czech Republic, Institute for Clinical and Experimental Medicine and Motol University Hospital Prague, there has also been a change of the Centre Representatives. We are delighted that **Sona Frankova** will be taking the place of Milan Jirsa and would like to take this opportunity to thank him very much for his work with us to date.

New Release: A video about parents with a young child with a liver disease

Nederlandse
Leverpatiënten
Vereniging



We are very pleased to present a video from the Nederlandse Leverpatiënten Vereniging (NLV). They say: 'The NLV is incredibly proud of this video and especially of the parents who participated before and behind the scenes. We dedicate this video to all parents with a child with liver disease. Please feel free to share the link to the video!'

[Link to Video](#)

OTHER ANNOUNCEMENTS

Understand your Diagnosis

New disease leaflets are available on our website: [Pregnancy and breastfeeding among women with Wilson disease.](#)

The available versions can be downloaded in German, Spanish, Maltese, English, French and Italian.

Visit us on our website



European Reference Network for Hepatological Diseases
ERN RARE-LIVER

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