

3 December 2025

ERN

GUARD-Heart

Gateway to Uncommon And Rare Diseases of the Heart

EUROPEAN REFERENCE NETWORKS
FOR RARE, LOW-PREVALENCE AND COMPLEX DISEASES

Share. Care. Cure.



ERN GUARD-HEART NEWSLETTER

YEAR 2025 NUMBER 5



The **19th ERN GUARD-Heart Board Meeting** will take place on **Thursday, 19 March 2026**, in **Bucharest** at the Courtyard by Marriott Floreasca Hotel. Mark your calendars, we look forward to seeing you there! ❤

Madrid Board Meeting Materials Online

We are pleased to inform you that all materials from the recent **Madrid Board Meeting** are now accessible in our **secure member area** on the website. This includes slides, notes and session recordings.

Access the Member Area: [Protected: Board Meeting Madrid - ERN GUARD-Heart](#)

To view the content, please **request the password** from the Coordinating Office via email: contact@guardheart.ern-net.eu ❤

AEPC & ERN GUARD-Heart webinar : A RyR2 variant in an aborted SCD case, how to act?

Wednesday 10-12-2025 17:00-18:30 CET

Moderator: Dr. P.G. Postema
(Amsterdam, Netherlands)

Speakers: Dr. C. van der Werf
& A. Bergeman
(Amsterdam,NL)

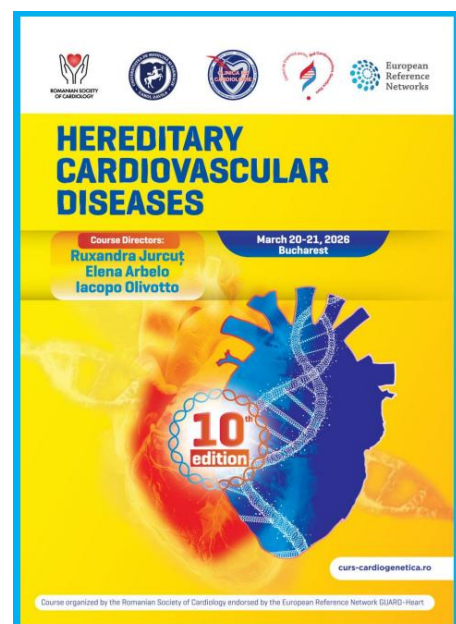
[More info](#)

[Register Now!](#)

AEPC WEBINAR SERIES

**AEPC HEART BEATS
FOR EDUCATION**

TEACHING WEBINARS ON PAEDIATRIC/CONGENITAL CARDIOLOGY &
CARDIAC SURGERY IN COLLABORATION WITH ERN GUARD-HEART



[Hereditary Cardiovascular Disease Course –
Hereditary Cardiovascular Disease Course](#)



New version -CPMS 2.0: Clinical Patient Management System

The Clinical Patient Management System (CPMS) 2.0 is a secure web-based application to support European Reference Networks in the diagnosis and treatment of rare or complex conditions across national borders.

CPMS 2.0 will realise one of the ERNs core tasks; *bringing expert specialised care to all patients in Europe.*

The **NEW version** of the Clinical Patient Management System (**CPMS 2.0**) introduces a range of improvements designed to make virtual collaboration easier, faster, and more secure for ERN members.

Key Features of CPMS 2.0:

- **secure and efficient collaboration:** CPMS 2.0 provides an even more secure environment for healthcare professionals to share patient information, discuss cases, and develop joint treatment plans.
- **enhanced patient care:** by facilitating efficient communication and information sharing, CPMS 2.0 helps to improve the quality and timeliness of patient care. It enables faster diagnoses, more accurate treatment plans, better patient outcomes and reduced patients' travel.
- **medical imaging:** CPMS 2.0 has specialised visualisers for DICOM and SVS medical images, allowing clinicians to view and discuss the relevant image details.
- **advanced data collection and reporting:** CPMS 2.0 promotes extensive anonymised data collection and reporting, which improves data quality and facilitates data analysis. This data can be used to identify trends, monitor disease progression, and evaluate the effectiveness of treatments.
- **security and data privacy:** CPMS 2.0 is fully compliant with the General Data Protection Regulation. All data processing operations are based on patient consent. Data are encrypted in transit and at rest and audio/video conferencing and texting are end-to-end encrypted. ❤️

[Login: European Reference Networks - Clinical Patient Management System](#)

[CPMS Supporting Documents and Instructions](#)

[CPMS2.0 Care ShortGuide v4.pdf](#)

CPMS 2.0 is an easy-to-use, secure, GDPR-compliant platform that supports cross-border collaboration and expert consultation across Europe. ❤️





Start of the CPMS Recurring Sessions for ERN GUARD-Heart 📢!!

The CPMS Recurring Meetings are an opportunity to collaborate across the network through virtual consultations — the core focus of CPMS.

Want to get familiar with CPMS 2.0 and ask questions directly to the ERN GUARD-Heart CPMS Support/Helpdesk?

Session led by **Dr. Malou van den Boogaard**, Amsterdam UMC, ERN GUARD-Heart CPMS expert.

Join our upcoming CPMS Sessions:

- **Thu, 18 Dec 2025 | 13:00–14:00 CET**
- **Tue, 13 Jan 2026 | 13:00–14:00 CET**

Help spread the word and invite your colleagues!

➔ [Register here](#)



CPMS 2.0 Online Training Sessions coming up in 2026

To help clinicians and admin users transition to CPMS 2.0, the general online training sessions are scheduled for **January–March 2026**. Each session lasts about one hour (30 min training + 30 min Q&A).

Clinicians – CPMS 2.0 Care Application & Mobile:

- **Jan 20, Feb 17, Mar 17 (10:00 CET)**

Topics: Sign-up flow, patient records, discussions, scheduling, chat, mobile version.

Admins & Local Helpdesks:

- **Jan 22, Feb 19 (10:00 CET), Mar 19 (15:00 CET)**

Topics: Access requests, user management, groups, reporting.

➔ **Indicate your interest [here](#)**

to receive login details and further information.





Who are our ePAG's? Gaelle MARGUIN- French patient association Petit Cœur de Beurre



*'I am **Gaelle MARGUIN**, co-founder of the French patient association Petit Cœur de Beurre. We support patients born with **congenital heart disease** and their families. I am myself the mother of a boy born in 2014 with a transposition of the great arteries, and it was following our healthcare journey that we decided to create the French association.*

Our patient association, Petit Cœur de Beurre, now has nearly 400 members and about 90 volunteers who provide support to patients and their families, both in and outside the hospital.

Since 2017, I have been a salaried employee of the association (we currently have eight employees). I recently completed a University Diploma in Health Democracy, and I am also trained as a patient-partner and trainer.

My role is to oversee the implementation of the association's activities. What I love most is networking and co-constructing with healthcare professionals, patients, and families.

I also enjoy working on advocacy initiatives to change how patients are cared for and to defend their rights.

Petit Cœur de Beurre works closely with healthcare providers and patients. We produce accessible information tools for patients, available on our website and distributed by cardiologists. We regularly host webinars to keep our patients well-informed and, together with cardiologists and nurses, we create therapeutic education workshops.



Gaelle MARGUIN, co-founder Petit Cœur de Beurre

We are also committed to addressing cross-cutting topics such as promoting physical activity, preventing risk factors, and, more recently, our project on neurodevelopmental disorders in children born with congenital heart disease. We developed a kit including an information booklet and a developmental wheel to help identify possible developmental delays in children by age group.

We also fund and create projects within hospital settings: well-being workshops for caregivers, ward decoration projects, and reclining chairs/beds so that parents can sleep next to their child at night — all with the goal of making hospitalization a gentler experience.

We work closely with researchers, participating from the early stages of study design to the dissemination of results, particularly in projects related to the human and social sciences.

I feel very fortunate today to have a job that I am truly passionate about.

The world of patient associations is a true collective wealth !

"Never doubt that a small group of thoughtful, committed citizens can change the world; indeed, it's the only thing that ever has." Margaret Mead ♥





ERN Summer School in Padua: Biopsy-proven pediatric and adult myocarditis from diagnosis to tailored aetiology

Padua, 11-2025

The 'Summer' School on *"Biopsy-proven pediatric and adult myocarditis from diagnosis to tailored aetiology-directed therapy"*,

organized by **Prof. Alida LP Caforio**, Leader of Thematic Area 5, from Cardiology and by **Prof- Cristina Basso**, from Cardiac Pathology, Padua, Italy, took place on **November 7th in Cardiology and on November 8th in Cardiac Pathology**. The participants were cardiologists and cardiac pathologists from various ERN-GUARD-Heart members or affiliated centres. The first day was dedicated to the new ESC myocarditis and pericarditis guidelines, the various technical approaches to obtain an endomyocardial biopsy, and the interactive and multidisciplinary discussion of personalized biopsy-guided etiology-directed therapy in real clinical cases from the Padua Cardioimmunology clinic.



The second day was dedicated to the new pathologic classification and report of myocarditis at endomyocardial biopsy and at autopsy, the modern immunohistochemical, molecular tools, as well genetic testing and "live" demonstration of explanted hearts and digital microscopic examples of myocarditis cases (see Picture). ❤

ERN Summer School in Prague : CRT in Paediatric and Congenital Heart Disease



Prague, 11-2025

On **14–15 November 2025** ERN GUARD-Heart hosted its 'Summer' School in Prague at the Children's Heart Centre, University Hospital Motol. The course, led by **Prof. Jan Janoušek**, focused on **Cardiac Resynchronization Therapy (CRT)** in paediatric and congenital heart disease, offering a practical guide to diagnosis, indication, and implementation. The program included expert lectures on heart failure in children, ventricular dyssynchrony, advanced imaging techniques, conduction system pacing, and case-based interactive sessions. ❤



CALL FOR EXPERTS: EMA PANEL ON PAEDIATRICS AND RARE DISEASES

The European Medicines Agency (EMA) is seeking experts for its newly created expert panel on Paediatrics and Rare Diseases in the field of medical devices.



LEARN MORE AND APPLY
VIA THE EMA WEBSITE:

Medical Device Expert Panels – EMA

Call for Experts: New EMA Panel on Paediatrics and Rare Diseases

Amsterdam, November 2025

The **European Medicines Agency (EMA)** has launched a call for expression of interest for experts to join the newly created **expert panel on Paediatrics and Rare Diseases in the field of medical devices**.

A corrigendum to the [Call for expression of interest](#) for medical devices and in vitro diagnostic (IVD) medical devices experts has been published. Experts wishing to be considered for the new Paediatrics and Rare diseases panel are welcome to send their application. Experts wishing to be considered for the [Central list](#) may also apply at any time.

- Application guidance is available in [Annex I](#) of the published call.
- When applying, ensure you tick (at least) **“Paediatrics and Rare Diseases”** as a panel that would best fit their expertise.
- For technical issues, contact EMA IT Service Desk: support.ema.europa.eu or call +31 (0)88 781 8520.

Learn more and apply via the EMA website:

[Medical Device Expert Panels – EMA](#)

Experts from ERN GUARD-Heart are strongly encouraged to apply and share their expertise. ❤️

European Commission survey on HCPs and ERN Registries

Deadline extended 12 December!

Your feedback matters! We encourage all **ERN GUARD-Heart members** who contribute to or plan to contribute to ERN registries to take part in this important survey. The survey aims to identify challenges—especially around **legal and funding aspects**—and complement work done under [ERICA](#) and [JARDIN](#). It is designed for different roles within HCPs (clinicians, data managers, IT specialists).

Estimated time: 15–25 minutes

Deadline: **12 December 2025**

Take the Survey: [EU Survey – Survey](#)

High-Level Meeting on a European Research and Innovation Ecosystem for Rare Diseases

Brussels, 9-11 December 2025

The **High-Level Meeting (HLM RARE)** will take place on **9–11 December 2025** in **Brussels** and **online**, bringing together EU policymakers, researchers, patient advocates, healthcare providers, and industry leaders to strengthen the European research and innovation ecosystem for rare diseases.

Key objectives include:

- Advocating for the **EU’s adoption of a European Declaration on Rare and Complex Diseases**
- Building a **sustainable Research and Innovation Ecosystem**
- Enhancing collaboration among all stakeholders to improve access to innovative therapies

Location: Brussels & Online

Date: 9–11 December 2025

[Learn more and register](#) ❤️





European Rare Diseases
Research Alliance

ERDERA

Multi- Stakeholder Workshop: Prioritising Rare Diseases for ATMP Development

Heidelberg, September 2025

On 9–10 September 2025, ERDERA hosted a Multi-Stakeholder Workshop in Heidelberg to advance the prioritisation of rare diseases for Advanced Therapy Medicinal Products (ATMPs).

Key achievements:

- Finalised a three-domain prioritisation framework:
Unmet Medical Needs, Psychosocial & Societal Impact, Research & System Readiness
 - Refined criteria, scoring, and weighting in breakout groups
 - Tested the tool's usability and captured refinements for the ATMP platform decision tree
- On behalf of ERN GUARD-Heart, Andrea Greco (SJD, Barcelona) highlighted core needs in inherited cardiomyopathies and arrhythmia syndromes, stressing the importance of harmonised registries and endpoints for ATMP planning. Additionally, ERN GUARD-Heart ePAG representative Ruth Biller contributed the patient perspective. ❤️



[Read more on ERDERA](#)



2nd International Conference on Clinical Research Networks: Connected for impact

Heidelberg, 9-10 December 2025

ERDERA – the European Rare Diseases Research Alliance – will host the 2nd International Conference on Clinical Research Networks (CRNs) for Rare Diseases, co-organised with Rare Disease International and IRDiRC. [Read more and Register](#) ❤️

CONNECTED FOR IMPACT

Mobilising
the Global
Rare Disease
Clinical
Research
Ecosystem

2nd International Conference
on Clinical Research Networks
for Rare Diseases

9-10 December 2025

Heidelberg Germany

Hybrid format





Latest ERN GUARD-Heart Publications

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2. Garcia-Pavia P, Gonzalez-Lopez E, Anderson LJ, Cappelli F, Damy T, Fontana M, Gonzalez-Costello J, Jurcut R, Lairez O, van der Meer P, Merlo M, Perlini S, Bayes-Genis A. **Non-amyloid specific treatment for transthyretin cardiac amyloidosis: a clinical consensus statement of the ESC Heart Failure Association.** *Eur Heart J.* 2025 Oct 7:ehaf710. doi: 10.1093/eurheartj/ehaf710. Epub ahead of print. PMID: 41055898.
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ERN GUARD-Heart
Project
Management
Office



Arthur A.M. Wilde
Network Coordinator
Amsterdam UMC
Amsterdam, Netherlands
a.a.wilde@amsterdamumc.nl



Nynke Hofman
Network Manager
Amsterdam UMC
Amsterdam, Netherlands
n.hofman@amsterdamumc.nl



Mari Murel
Network Manager
Amsterdam UMC
Amsterdam, Netherlands
m.murel@amsterdamumc.nl



Ahmad S. Amin
Registry Manager
Amsterdam UMC
Amsterdam, Netherlands
a.s.amin@amsterdamumc.nl