

Tuesday 6 February 2024

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

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GUARD-Heart

Gateway to Uncommon And Rare Diseases of the Heart

ERN GUARD-HEART BIMONTHLY NEWSLETTER

YEAR 2024 NUMBER 1

New reimbursement rules – read about the ‘unit-costs’

Amsterdam, 04-02-2024

With the start of the new grant period 2023 – 2027 for ERNs, the European Commission has decided to implement the use of unit costs for travel, accommodation and subsistence costs. The travel costs have been determined on the basis of an analysis of market price for travel within the EU and between other countries and EU Member States. All distances to be measured using either the rail or flight calculator can be found at the website: https://ec.europa.eu/info/calculate-unit-costs-eligible-travel-costs_en

Also costs for hotels, and subsistence costs (renting locations, food, etc.) will be covered by unit costs. This has consequences for reimbursement for the exchange costs, patient meetings, board meetings, etc. Please contact the coordinating team if you have specific questions on how to count the reimbursement. ♥



ERN Grant Agreement signed!

Amsterdam, 01-02-2024

The grant application for the direct grant to ERNs, which provides support for the coordination, management and operational activities of the ERNs until 1 October 2027, has been revised, accepted, and signed just before Christmas. We are looking forward to proceed with all the activities of the ERN GUARD-Heart for the coming 4 years. ♥

ERN board meeting & parallel ePAG meeting in Paris (France) on 22 March 2024

Paris, 01-02-2024

The next hybrid board meeting of ERN GUARD-Heart will be held in Paris on Friday 22 March 2024. We collected your plans through a survey and are looking forward to meet 47 clinicians (of whom 16 online) and 10 e-PAGS. The location details for the meeting can be found in this newsletter. The detailed program will be send later this week by e-mail. ♥





Who are our ePAGs?

Amsterdam, 01-02-2024

One of the European patient advocates of ERN GUARD-Heart in the spotlight: *Carola Ossenkopp-Wetzig* from Fulda, Germany, tells her story and her active way to cope with it.

‘Sometimes life changes from one second to the next.’ It all started when, after a wonderful pregnancy, my gynecologist discovered that our son had a severe congenital heart defect a few weeks before my due date. The pediatric cardiologist suspected a problem with the pulmonary artery and the tricuspid valve. A shock that you have to digest at first. At the same time a blessing, because only few heart defects are even detected before birth.

My son Max was delivered by caesarean section and flown to the children's heart center a few days after birth (2009). Unbearable for me as a mother not being allowed to hold your child in your arms and leaving it to other people. My husband and I were with our son every day. We lived in the parents' apartment at the children's heart center. In our first 4 weeks we just waited for the pressure in the lungs to settle (he was panting like a little puppy) and for the pressure between the lungs and heart to adapt. After the examination by cardiac catheterization a few weeks later, the doctor welcomed us with the words: “I only have positive news for you”. We were happy. He told us that the pulmonary valve had opened on its own due to the changed pressure conditions. That was great news! But then he said that Max still needed surgery because his atrium was enlarged and that could lead to cardiac arrhythmias. The right heart valve would also need to be operated on, because it still wouldn't close properly. We were a bit shocked. That wasn't good news! But he assured me: “If we can operate on your child, that's good news. If there is no surgical option for him, then that's bad”. After this operation, Max would be virtually cured, he said. But open heart surgery scared us. And our worst fears should become true...The day of the operation came when Max was one month old and it was said that Max would be operated on for 2 hours. We had Max in surgery at 11:00 am. We tried to distract ourselves and went for a walk, but it was impossible to think of anything else.



We asked around 6:00 p.m. and they said Max was still in surgery. We were worried, but we never imagined what we would find out hours later. When my husband finally had news of our son at around 10 p.m., I again had the feeling to be the protagonist of a movie, a horror movie.

It took me years to talk about this day and weeks to come and yet I see the situation in front of me like it was on that summer day in August.

When my husband spoke to the doctor, I initially thought he had mixed up the baby. He talked about everything that had been done and it were all interventions that weren't even planned. My husband checked again for the child's name and looked at me in shock. Max's operation did not last 2 hours as planned, but 8 hours. The adjustment of the tricuspid valve had not worked properly as it was only tissue, so they had to reconstruct the valve completely. An operation that they normally do not perform on a baby of that age, but they didn't have a choice.

It was way too much for Max's body and he suffered from a cardiac arrest after surgery. He was in very critical conditions and stayed in it for the next 3 weeks. Max fought for survival and we had to talk about topics that young parents don't even think about, like a funeral. Doctors expected he could die any day. We couldn't sleep at night because we were always expecting to get a call from the hospital. I put earplugs in my ear because I was just so afraid of the noise of the ringtone.

But the call never came and after three weeks in intensive care, my son had won the battle. Whether his disabilities that came to notice when he became older come from that surgery or from his duplication 22q11 that genetic testing found out later? We will never know, but we are extremely thankful to the guardian angel that has saved our son.

-- See next page --



Who are our ePAGs?

Carola Ossenkop – Wetzig *(continued)*

The traumatic two month in hospital and the subsequent time at home made me want to share my experience with others and I founded a self- help group for children with congenital heart defects in Fulda in 2010. A lot of families have joined the group since then.

Two years later, I founded the first sports group for children with congenital heart defect in the state of Hessen and was supported by our pediatric cardiologist. As I heard so many questions about nutrition during my work with the families, I initiated a brochure about nutrition for children with CHD with the help of scientists from my university that was published by the German association for congenital heart defects (BVHJ). It became one of the most demanded brochures of the association. The experiences in the self-help world have led me to the idea of writing my doctoral thesis. Through the patient's organization I got to know a professor that was active in the scene. We exchanged ideas and in 2015 I started my part-time Ph.D. project in the department of educational sciences and social work that I successfully finished in October 2023.

My doctoral thesis is about the role of self-help groups for families with children who have a congenital heart disease. Although it often is a life-changing experience for families, my survey with 827 affected families showed that only 12% seek outside help by joining self-help groups. The focus of my dissertation was to determine why families join self-help groups and what prevents them from doing so.

At the end of 2022 I became a member of the European Patient Advocacy Group (ePAG) for ERN GUARD- Heart, and recently became the co-chair for the Congenital group of the GUARD-Heart ePAGs. As a mom and as a researcher I would like to foster the collaboration between the clinicians, the patients and self-help groups. I would also like to advance research in the psychosocial field, also in form of participatory research, to give affected families a voice and the opportunity to determine needs-oriented research topics. ♥

National Patient Meetings

Amsterdam, 02-02-2024

The ERN has a yearly national budget for patient meetings. This budget depends on the number of member hospitals in each EU Member State. If you organize an onsite meeting, we can support you with the costs for the meeting room, catering (coffee/thee/lunch breaks), technical costs or travel costs (for patients). In January we started a survey to collect your plans for the first year of this grant. There were quite some plans announced and that is good news! We will contact the members who requested for support soon. Of course we will try to support everyone, but if this is not possible, we will take this information with us to next years plans and give other centers priority at that time (if necessary). ♥

ERN Exchange project

Amsterdam, 02-02-2024

The ERN Exchange Programme has been designed to spread knowledge across the ERN GUARD-Heart and to stimulate collaboration between healthcare professionals in different European Reference Networks (ERNs).

With funding from the ERN GUARD-Heart, each exchange package will provide funds for a 5-day exchange visit to an ERN healthcare provider (HCP) center – this can be an HCP within the ERN GUARD-Heart, or potentially an HCP within one of the other 23 ERNs, if relevant and appropriate.

Per HCP, one application (as a visitor) can be accepted per year. One package includes reimbursement of travel costs, hotel costs (based on the official unit costs) and a daily allowance (for local transport and food). https://www.eacea.ec.europa.eu/system/files/2023-09/unit-cost-decision-travel_en.pdf

Travel insurance is NOT included. We received some plans already, and some requests to help to find a hosting centre. However, it is still possible to send us your exchange plan request. The information and mobility agreement template, is on the ERN-website (<https://guardheart.ern-net.eu/exchange-programme-2023-2024/>). ♥



22 March 2024: ERN GUARD-Heart Board Meeting

Amsterdam, 01 February 2024

On Friday 22 March 2024, the ERN coordination team organizes the 15th (hybrid) ERN GUARD-Heart Board meeting in Paris and dinner on the evening before. Registration has been completed.

BOARD MEETING

Date: Friday 22 March 2024

Time: 8:30 – 15:30

Agenda: see separate attachment (to members/affiliated partners) later this week by e-mail.

Location: Sorbonne Universite – Faculte de Medecine – 91 Boulevard de l’Hopital 75013 Paris

Registration: If you did not register yet, but would like to receive a direct online link to this hybrid meeting, please send an e-mail request to Nynke (n.hofman@amsterdamumc.nl) **before** Wednesday 20 March.



*Sorbonne Universite, Faculte de medecine
91 Boulevard de l’Hopital 75013 Paris*

DINNER

Date: Thursday 21 March 2024

Time: 19:00

Location: ‘Le Train Bleu’, Gare de Lyon, Pl. Louis Armand hall 1, 75012 Paris

Registration: completed (personal confirmation e-mail will follow)



HOTEL: Villa Lutèce Port Royal

Date: Thursday 21 March 2024

Location: 52 rue Jenner, 75013 Paris

Registration: completed (personal confirmation e-mail will follow).





Rome, 31 January 2024

We are glad to inform you that Pedirhythm, the international biennial meeting focused on arrhythmia care for infants, children, and patients of all ages with congenital heart disease will take place in Rome on **September 19th-21st, 2024.**

Pedirhythm attracts physicians, as well as fellows in training, nurses, scientists, and industry representatives from across the globe. It has quickly developed into the premier stand-alone scientific meeting for pediatric and congenital electrophysiology. Pedirhythm is a great opportunity to learn and share advances in clinical and research electrophysiology for children and patients with congenital cardiac disease and a unique moment to meet the experts in this field. The faculty will come from all over the world including both senior figures in the subspecialty as well as promising young investigators. This 11th edition will be hosted by the Pediatric EP Complex Unit of the **Bambino Gesù Children's Hospital IRCCS** and we expect that this congress will be endorsed by the Association for European Paediatric and Congenital Cardiology, the Heart Rhythm Society, the Pediatric and Congenital EP Society, and other important national and international pediatric and EP society.

All information about the Programme, Registration and Abstract submission is available on the website: <https://pedirhythmxi.org/>.

Prof. Fabrizio Drago, Local Organizing Committee Chair

ECRD - Call for abstracts

This year, ECRD has a poster theme within the conference that is of specific relevance to ERNs: Access to Highly Specialised Care. Within this theme, ECRD is seeking examples of abstracts that are falling into the category of unmet needs/challenges faced by patients with ultra-rare disease or patients who need to undergo complex surgical interventions to access cross-border highly specialised services. As well as examples of EU cross-border arrangements to provide access to innovative therapies. In addition, the conference encourages submissions under 5 more themes related to rare diseases:

1. Research & Innovation
2. Mental Health & Wellbeing
3. Rare Disease National Plans
4. Access, Availability and Affordability of Treatments
5. Diagnosis & Screening

Posters can be submitted here: <https://www.rare-diseases.eu/posters/>

Poster abstracts are being accepted until 23 February 2024.





Monitoring data 2023

ATTENTION**Amsterdam, 05-02-2024**

In January 2024 the collection of data for the ERN monitoring has started again. All ERN members and affiliated partners have received instructions and requirements by e-mail. Please collect your data and send back your file before the deadline, which is 1 March 2024. ♥



Latest ERN GUARD-Heart Publications

1. Baruteau AE, Fraise A, Butera G, Bautista-Rodriguez C. The Transcatheter Closure of Patent Ductus Arteriosus in Extremely Low-Birth-Weight Infants: Technique and Results. *J Cardiovasc Dev Dis.* 2023 Nov 27;10(12):476. doi: 10.3390/jcdd10120476. PMID: 38132644; PMCID: PMC10744056.
2. van Pottelberghe S, Kupper N, Scheirlynck E, Amin AS, Wilde AAM, Hofman N, Callus E, Biller R, Nekkebroeck J, Van Dooren S, Hes FJ, van der Crabben SN. Are disease-specific patient-reported outcomes measures (PROMs) used in cardiogenetics? A systematic review. *Eur J Hum Genet.* 2023 Dec 14. doi: 10.1038/s41431-023-01510-w. Epub ahead of print. PMID: 38097768.
3. Wiethoff I, Sikking M, Evers S, Gabrio A, Henkens M, Michels M, Verdonschot J, Heymans S, Hiligsmann M. Quality of life and societal costs in patients with dilated cardiomyopathy. *Eur Heart J Qual Care Clin Outcomes.* 2023 Sep 14:qcad056. doi: 10.1093/ehjqcco/qcad056. Epub ahead of print. PMID: 37709575.
4. Iannotta M, d'Aiello FA, Van De Bruaene A, Caruso R, Conte G, Ferrero P, Bassareo PP, Pasqualin G, Chiarello C, Militaru C, Giamberti A, Bognoni L, Chessa M. Modern tools in congenital heart disease imaging and procedure planning: a European survey. *J Cardiovasc Med (Hagerstown).* 2024 Jan 1;25(1):76-87. doi: 10.2459/JCM.0000000000001569. Epub 2023 Dec 8. PMID: 38079284; PMCID: PMC10754484.
5. Thomson KL, Jiang C, Richardson E, Westphal DS, Burkard T, Wolf CM, Vatta M, Harrison SM, Ingles J, Bezzina CR, Kroncke BM, Vandenberg JI, Ng CA. Clinical interpretation of KCNH2 variants using a robust PS3/BS3 functional patch-clamp assay. *HGG Adv.* 2024 Jan 14;5(2):100270. doi: 10.1016/j.xhgg.2024.100270. Epub ahead of print. PMID: 38219013.
6. Carrick RT, De Marco C, Gasperetti A, Bosman LP, Gourraud JB, Trancuccio A, Mazzanti A, Murray B, Pendleton C, Tichnell C, Tandri H, Zeppenfeld K, Wilde AAM, Davies B, Seifer C, Roberts JD, Healey JS, MacIntyre C, Alqarawi W, Tadros R, Cutler MJ, Targetti M, Calò L, Vitali F, Bertini M, Compagnucci P, Casella M, Dello Russo A, Cappelletto C, De Luca A, Stolfo D, Duru F, Jensen HK, Svensson A, Dahlberg P, Hasselberg NE, Di Marco A, Jordà P, Arbelo E, Moreno Weidmann Z, Borowiec K, Delinière A, Biernacka EK, van Tintelen JP, Platonov PG, Olivotto I, Saguner AM, Haugaa KH, Cox M, Tondo C, Merlo M, Krahn AD, Te Riele ASJM, Wu KC, Calkins H, James CA, Cadrin-Tourigny J. Implantable cardioverter defibrillator use in arrhythmogenic right ventricular cardiomyopathy in North America and Europe. *Eur Heart J.* 2024 Jan 9:ehad799. doi: 10.1093/eurheartj/ehad799. Epub ahead of print. PMID: 38195003.

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