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

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ERN GUARD-HEART



ERN GUARD-HEART BIMONTHLY NEWSLETTER

YEAR 2017 NUMBER 2

The Launch of ERN GUARD-HEART Website

1 June 2017 - Amsterdam, Netherlands

The European Commission has created the web space <http://guardheart.ern-net.eu/> for ERN GUARD-HEART. The website has been designed by the Project Management Office of ERN GUARD-HEART, and launched on 1 June 2017. It is a public-facing website for the Network to publish content. Currently, the website contains information about European Reference Networks in general, the aims and the structure of ERN GUARD-HEART, the target groups and the thematic areas of the Network, the participating healthcare providers (HCPs) and their representative members, and most important activities of ERN GUARD-HEART. The Newsletters of the Network are also published on the website. In the future, additional pages with more detailed information on the individual members from each specific HCP, educational materials for patients, and ERN-related meetings, publications, and interviews will also be published.

Members of ERN GUARD-HEART are invited to publish content on the website (including the names, positions, function, and photos of their members) by sending an email to the Project Management Office. Members can also request a personal e-mail (e.g., firstname.lastname@guardheart.ern-net.eu). These e-mails will be accessible via Microsoft Outlook or smartphones but also via webmail at <https://ern.awsapps.com/mail/>. ♥

Example pages of the ERN GUARD-HEART website



Network Agreement Form

20 June 2017 – Amsterdam, Netherlands

The European Commission has provided the European Reference Networks (ERNs) with a uniform draft Network Agreement. The Network Agreement formalises the relationship of the members within the ERNs. The Network Agreement is a private and voluntary agreement between partners (members of the Network) to set out common rules and obligations among themselves. The Agreement is solely meant to support working arrangements between members of the Network.

Although the Network Agreement does not involve the European Commission, and neither the Commission nor any person acting on Commission's behalf may be held responsible for the use which may be made of the information contained in the draft Network Agreement, it is important to realize that the EU legal texts are the only authentic legal reference.

The Network Agreement for the members of ERN GUARD-HEART has been signed by most of the HCP representatives, and will be sent to the rest of HCP representatives in the near future. Because the same form should be signed by all members, this process is expected to take some time. The Network Agreement should be signed by the representative members of the HCP or an official from the HCP, whatever is deemed appropriate. ♥



Official ERN Logo

15 June 2017 – Brussels, Belgium

The European Commission has created a unique graphic identifier ('logo') which ERN GUARD-HEART Members can use for the activities organized by the Network. The ERN logo constitutes the visual identity of the Network and its Members. It has been registered as a Trademark by the European Union (EU). EU is willing to grant the Network a licence to use it only under a certain set of terms and conditions, which are set out in a Licensing Agreement (agreement between the EU and Network Coordinator) and a Sublicensing Agreement (agreement between Network Coordinator and Members of the Network). The Coordinator and Members of the Network are only entitled to use the ERN logo after the Licensing and Sublicensing Agreements are signed. The Licensing Agreements has been signed by Arthur Wilde. The Sublicensing Agreements has been sent to all representative members of ERN GUARD-HEART by e-mail. Most of the HCPs have already signed the Sublicensing Agreements, and eight HCPs have to sign the Agreement yet.

There are two versions of the ERN logo. The basic version of the logo is for all ERNs. The extended version is a customized version of the logo for ERN GUARD-HEART and its Members. There are also two linguistic versions available for the name of each HCP (in the native language of the Member State in which the HCP is located and in English). After the Sublicensing Agreements are signed, all versions of the logo and the user guides for the logos will be sent to the representative members of the HCPs. ♥



Basic version of the ERN logo



Example of the extended version of the ERN logo



Second Meeting of the Thematic Area Group “Familial Electrical Diseases”

20 June 2017 – Vienna, Austria

The second meeting of the experts in the Thematic Area Familial Electrical Diseases was held in Vienna, Austria, on 20 June 2017, during the EHRA EUROPACE – CARDIOSTIM Congress. This Thematic Group is led by **Dr. Carlo Napolitano** and **Dr. Silvia Priori**, from the Clinical Scientific Institutes Maugeri IRCCS in Pavia, Italy. The list of participants included **Carlo Napolitano**, **Arthur Wilde** (*AMC Amsterdam*), **Eric Schulze-Bahr** (*University Hospital Munster*), **Elijah Behr** (*St George’s Healthcare NHS Trust*), **Georgia Sarquella-Brugada** (*San Juan de Déu Barcelona’s Children Hospital*), **Philippe Chevalier** (*Louis Pradel Cardiovascular Hospital*), **Perry Elliott** (*Barts Health NHS Trust*), **Anita Hiipala** (*Helsinki University Hospital*), **Peter Schwartz** (*Instituto Auxologico Italiano IRCCS*), and **Rik Willems** and **Tomas Robyns** (*University Hospitals Leuven*).

The Coordinator of the Network, **Arthur Wilde**, opened the meeting and requested all participants to keep track of ERN-related events (i.e. publications, presentations, interviews, etc.) of their centre, and to send a list of these activities to the Project Management Office. Moreover, he emphasized the importance of widespread attention and dedication among all ERN GUARD-HEART members. After the opening, several important points were brought into the attention of the participants, including the Connecting Europe Facility TELECOM 2017 Call (see further and also ERN GUARD-HEART Newsletter of March 2017), the Network Agreement (see page 2 of this Newsletter), and the website of ERN GUARD-HEART.

At the meeting, ERN members agreed upon the need for specific patient information materials for the different diseases that are within the scope of ERN GUARD-HEART. Since most HCPs do have such materials in place, the Project Management Office has sent a request to the HCPs to collect the information materials (*and has received such materials from some of the HCPs*). In addition, Thematic Area Leaders were asked to formulate a minimum set of requirements for the patient materials (as to what sort of information the materials should contain).

Next, ERN members discussed the need for clinical guidelines for diseases that are within the

scope of ERN GUARD-HEART. They indicated that for most of the diseases guidelines may probably not be needed because the existing guidelines and expert consensus reports are from recent date or because the diseases are being dealt with by the European Society of Cardiology (ESC), European Heart Rhythm Association (EHRA), and/or the Heart Rhythm Society (HRS). However, an exception might be dilated cardiomyopathy.

ERN members made a list of current initiatives on **registries** or ongoing registries that could be part of ERN HEART-GUARD. For familial electrical diseases, current and/or ongoing registries include:

1. *CALM* genes: **Peter Schwartz** (*Instituto Auxologico Italiano IRCCS, Milan, ongoing with participation of many ERN members*)
2. CPVT international registry: **Arthur Wilde** (*AMC, Amsterdam, ongoing with participation of many ERN members*)
3. Brugada syndrome: proposed by **Pier Lambiase** (*Barts Health NHS Trust, London, UK*)*
4. Brugada syndrome GWAS: **Connie Bezzina** (*AMC, Amsterdam, ongoing with participation of many ERN members*)
5. Long QT syndrome GWAS: **Connie Bezzina** (*AMC, Amsterdam, ongoing with participation of many ERN members*)
6. Rare long QT syndrome types registry: **Georgia Sarquella-Brugada** (*San Juan de Déu - Barcelona’s Children Hospital*; 3 of 9 eligible grants will be funded; decision in July 2017).

** Regarding the Brugada syndrome registry it must be checked whether such a registry is compatible with the UK registry or can be complimentary, for example by using the same web-based database.*

For familial cardiomyopathies, current and/or ongoing registries include:

1. Patients with a genetic variant in *PRAKG2* gene: proposed by **Philippe Charron** (*AP-HP Pitié Salpêtrière Hospital, Paris*)
2. Patients with a genetic variant in *LMNA* gene
3. Danon disease



Second Meeting of the Thematic Area Group Familial Electrical Diseases (continued)

Another important issue that was discussed during the 2nd Meeting of the Thematic Area Group on Familial Electrical Diseases was to design plans for future educational programs in cardiogenetics. In this regard, existing teaching courses on cardiogenetics provided by the European Society of Human Genetics (ESHG) were brought into attention. Members of the Network suggested the idea to initiate a platform to facilitate discussions on future educational programs between Network members and representatives of

the ESC Working Group on Myocardial and Pericardial Diseases, EHRA, ESHG, and the Association for European Cardiovascular Pathology (AECVP). A letter of invitation has been sent to ESC, and ESHG has also been approached.

At the end, the recently initiated collaboration between AMC Amsterdam (on behalf of ERN GUARD-HEART) and patient advocacy group “Hart4Onderzoek” (Heart4Research) was announced. A meeting to start a collaboration with EURORDIS is scheduled at the end of July 2017. All representative HCP members were asked to propose patient advocacy groups that are willing to be involved in this collaboration. ♥

Second Meeting of the Thematic Area Group “Special Electrophysiology Conditions in Children”



Georgia Sarquella-Brugada

The group of experts in the Thematic Area “Special Electrophysiology Conditions in Children” have organized their 2nd meeting on Wednesday, 19 July 2017 from 15:30-17:30 in Barcelona, Spain, during the 7th World Congress of Paediatric Cardiology and Cardiac Surgery. The location for the meeting will be arranged by **Dr. Georgia Sarquella-Brugada** (*San Juan de Déu-Barcelona’s Children Hospital*), who is leading this Thematic Area of ERN GUARD-HEART. The agenda of the meeting will include topics that were discussed during the Second Meeting of the Thematic Area Group “Familial Electrical Diseases”. ♥



ERN GUARD-HEART Board Meeting at the ESC Congress

As specified in the ERN grant application, Meetings of the Network Board will take place every six months with all members required to attend and discuss strategic and scientific issues. Following the kick-off meeting of ERN GUARD-HEART in Vilnius, Lithuania, in March 2017, the first next meeting of the Network Board will take place on **27 August 2017 (from 13:00 to 17:00)** at the San Juan De Déu - Barcelona’s Children Hospital (during the European Society of Cardiology Meeting). The exact location and agenda of the meeting will be announced soon on the website of ERN GUARD-HEART.





Second Meeting of the ERN Coordinators Group

21 June 2017 – Brussels, Belgium

Following the 1st meeting of the ERN Board of Coordinators on 26 April 2017, where there was the formalization of the Board of Coordinators and the discussion on the creation of Working Groups and an initial discussion on the governance was initiated. A second meeting was held on 21 June in Brussels. On behalf of ERN GUARD-HEART, **Arthur Wilde**, the Coordinator of the Network, participated at this meeting.

Governance of the ERN Board of Coordinators

A brief discussion has been conducted regarding the need of coordination, following an informal meeting that was held in Newcastle on 1 June 2017. The ERN Board of Coordinators agreed to identify a Chair and Vice-Chair of the ERN Coordinators Group. The role of the Chair and Vice-Chair would be to:

- liaise with all ERN Coordinators to gather input for definition of the agenda of the ERN Board of Coordinators
- collect feedback from Working Group Leaders to circulate updates and prepare summaries for the European Commission on progress of work
- chair the ERN Board of Coordinators meetings
- develop meeting reports and consensus documents
- represent the ERNs externally
- be the contact point for the European Commission with regards to the ERNs.

Both Chair and Vice-Chairs are elected among the 24 ERNs coordinators for a mandate of one year. The role is merely of coordination. No decisions can be taken without prior consultation and agreement with all ERN Coordinators. Decisions are taken by consensus. **Maurizio Scarpa**, Coordinator of MetabERN, was nominated unanimously to be the first Elected Chairman of the ERN Board of Coordinators. Maurizio Scarpa did accept the mandate and did act as Chair for the meeting. The mandate will last one year, starting June 21 2017. The Vice-Chair will be proposed shortly by the Chair and voted on by the Board.

Organization of ERN Working Groups

On the occasion of the 1st ERN Board of Coordinators meeting, the setup of 7 working groups was agreed upon. The organization of these working groups was discussed during the 2nd meeting of the ERN Board of Coordinators and the following was proposed:

1. IT and data sharing

Chair: **Ruth Landestein**, ERN PaedCan

2. Research

Chair: **Eduardo Lopez Granados**, ERN TRANSPLANT-CHILD

3. Knowledge generation

Chair: **Paolo Casali**, ERN EURACAN

4. Data Protection, ethics, informed consent

Chair: **Nicoline Hoogerbrugge**, ERN GENTURIS

5. Cross-border healthcare and business continuity

Chair: **Holm Grässner**, ERN-RND

6. Monitoring, Assessment, quality improvement

Chair: **Christopher Chapple**, ERN eUROGEN

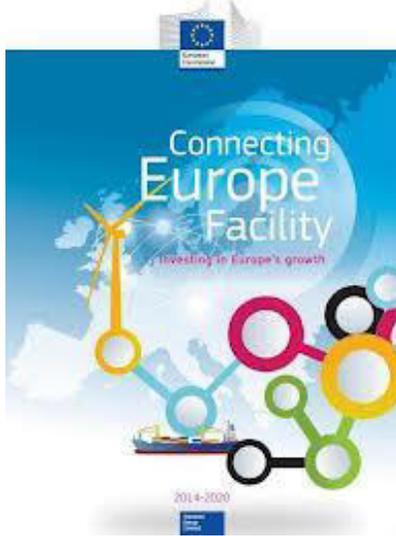
ERN GUARD-HEART will participate in the working groups Research and Cross-border healthcare and business continuity (Brexit). The working groups will identify a list of topics to discuss and prioritize. **Dr. Enrique Terol**, the Project Officer from the European Commission, will be regularly informed in order to optimize the impact of the activities of the working groups on the ERN management and planning. Each ERN can participate to a maximum of three working groups. Although the European Commission allows ERNs to select their best experts to participate in the working groups, the Coordinator of each ERN remains responsible.



Maurizio Scarpa
Coordinator of
MetabERN, the
ERN for Rare
Hereditary
Metabolic
Diseases



Second Meeting of the ERN Coordinators Group (continued)



Connecting Europe Facility TELECOM 2017 Calls for Proposals

On 6 May 2017, the Innovation and Networks Executive Agency (INEA) and European Commission launched the Connecting Europe Facility (CEF) Telecom call for 2017. The call makes funding available to stimulate and support the deployment of European Digital Service Infrastructures (DSIs). Within the eHealth area, the call makes €3 million available for facilitating the connection between members of ERNs by ensuring adequate and efficient use of the ERN core eHealth services (i.e., IT Collaborative Platform and the Clinical Patient Management System) by members of the ERNs. The maximum amount of funding for each proposal is €125.000,- for a period of one year. The funding may be used for the establishment of an IT technical helpdesk, training in ERN eHealth services, purchase of hardware and/or software necessary for the implementation of the ERN eHealth services in an ERN member HCP. Interested applicants can submit proposals at the latest by 21 September 2017.

The Coordinators found the INEA proposal a great opportunity to tune up the activity of ERNs on the Clinical Patient Management System (CPMS) and the IT platform. It was unanimously decided by to apply with similar proposals aimed at hiring personnel that is able to introduce and manage the new IT systems (CPMS, IT platform, and WebEx) to support implementation but also improvements and adaptations specific for each ERN. Thus a team of ERN-IT tool administrators will be created amongst ERNs able to optimize the application of the IT systems for the daily activity of the ERN.

Next Call to Join Existing ERNs

The call for new full Members to join existing ERNs will be announced in December, open in March 2018 and close in June 2018. There was a lot of discussion about the increased workload this could put on the ERNs.

It was also communicated that new Members could be defined as Affiliated Members, Associated Members, or National Coordination Hub Members. There was discussion about the Affiliated Partners as there was some confusion about this term. The Board of Member States (BoMS) has issued a paper in 2016 which explains this term ([paper](#)).

Only Member States can designate Affiliated Partners. However, it might be useful for individual ERNs to reflect on which Healthcare Providers from which Member States they wish to see join their ERN. The Affiliated Partner designation process (3 types: Associated Centres, Collaborative Centres, or Coordination Hubs) is a competence of the Member States and is not related at all with the next call for new Members.

The opinion about the opportunity to launch a new call for full membership has been opposed by several Coordinators, worried that this might be too early and complicating the initial organization of the ENRs at this stage. The same process will be followed, i.e., the Coordinating team will need to check that the HCP applicants meet required thresholds and submit the application forms and supporting documentation for them. This will be a very heavy workload, which some Coordinators expressed concern about not having sufficient resources to cope. However, other Coordinators welcomed this call as it gave them the chance to include HCPs that missed the first call. It has been decided to create a task force to liaise with the BoMS. This task force will be led by **Maurizio Scarpa**. Finally it was unanimously agreed that the European Commission and the BoMS should stick to the rules that were decided for the initial call. ♥



European Joint Programme for Rare Diseases

Research and Research Funding

15 June 2017 – Paris, France

Research on rare diseases is strongly supported by the European Union (EU) with close to 900 million Euros in more than 160 EU-funded projects. However, the EU finds the current landscape of research on rare diseases very fragmented and calls for stronger coordination of future research (and care) activities across Europe. Integrating rare disease research and care efforts is expected to maximize the potential of funded projects, tools and/or services that remain too fragmented, and to create a sustainable ecosystem for rare disease research allowing a virtuous circle between research, care and innovation.

In order to consolidate the transnational research on rare diseases through existing European platforms, and in the framework of the preparation of the Horizon 2020 calls for 2018-2020 (Societal Challenge 1, Health, Demographic change, and Well-being), the EU has launched a Consultative Group on the European Joint Programme (EJP) for Rare Diseases. An EJP is a Horizon 2020 co-fund action that is designed to support coordinated national research and innovation programmes.

The EU has provided ERNs the opportunity to participate in this Consultative Group to help refine and develop details of three initiatives (Pillars) of the EJP for Rare Diseases. The 3 Pillars are 1) research funding (to support rare diseases research by reinforcing and enlarging participation to transnational E-Rare calls and to develop new funding schemes), 2) organisation and sharing of information (to build a common virtual platform for coordinated access, repository and sharing of data and resources for research on rare diseases), and 3) education & capacity building (to share knowledge and know-how through training and support activities).

The work for the development of the EJP is divided among 4 Working Groups, one Working Group for Pillar 1, two for Pillar 2, and one for Pillar 3. Next, each Pillar of the EJP is organized into different Work Packages (WPs). ERN GUARD-HEART participates in Working Group 1 (Pillar 1, Research Funding), which has 4 WPs: WP 1) transnational calls; WP2) support of networking of new communities; WP3) Small scheme support/brokerage system; and WP4) monitoring of research funded activities.



ERNs are well-placed to contribute to the construction of the EJP for Rare Diseases to participate in the design of the programme of all 3 Pillars and help in specifying to end-user needs. ERNs will also be well-placed to develop implementation pilots for the information and data sharing platform. At the same time, ERN will benefit from the funding schemes, capacity building activities developed in the EJP for rare Diseases. And finally, there is mutual benefit to have a tight collaboration between the EJP and ERNs to foster rare disease research and care.

The kick-off meeting of the working groups took place on 15 June 2017 in Paris, France. On behalf of ERN GUARD-HEART, **Dr. Estelle Gandjbakhch** (AP-HP Pitié Salpêtrière Hospital, Paris), participated at this meeting in the working group for Pillar 1 (research funding). During this meeting, a time frame for the development of the EJP and different activities of the different working groups has been planned. ♥



Workshops for ERN-Related Activities

ERN-related workshops are organized by RD-ACTION, an EU Health Programme funded Joint Action that promotes implementation of the recommendations on policy, information and data for rare diseases. RD-ACTION is supported by the European Commission to rare diseases through its Directorate General for Health. A key objective of the RD-ACTION Policy is to provide support to rare disease community in conceptualising, implementing and evolving robust ERNs capable of meeting the needs and expectations of people living and working with conditions requiring a specific concentration of expertise.



Recent ERN-Related Workshops

1-2 June 2017 – Newcastle, UK

Indicators and Outcome Measures to demonstrate the impact and added value of ERNs

Context: ERNs should identify relevant Quality and Outcome Indicators across their Networks. Alongside, Clinical Performance (Impact) Indicators common to the work of all ERNs should be collected. The selection of such key Indicators and how they are captured will be of crucial importance to the future success of ERNs. During this workshop, a number of key questions were addressed, including how ERNs can demonstrate their added value, what can be measured (in terms of Indicators), and how ERNs embed a culture of knowledge generation through the implementation of outcome measures that are centred upon learning through complex case studies? Representatives of the ERNs, European Commission, EURORDIS (an alliance of rare disease patient organisations across Europe), RD-ACTION, ICHOM (International Consortium for Health Outcomes Measurement), Board of Member States, and UK Department of Health worked together to create a list of common indicators for all the ERNs to establish a continuous monitoring and assessment system to allow a closer follow up of the activities that are performed by the ERNs. ERNGUARD-HEART was represented at this workshop by Ahmad S. Amin, AMC, Amsterdam, the Netherlands.

Future ERN-Related Workshops in 2017

To participate at RD-ACTION workshops please contact the Project Management Office of ERNGUARD-HEART.

27 September 2017 – Barcelona, Spain

Exploring structured collaboration between ERNs and Biopharmaceutical Industry to support increased access to orphan therapies

Context: ERNs have the potential to make a meaningful contribution to improve access to rare disease therapies by stimulating the development of new therapies, supporting trials, enabling the collection of post-marketing data and real-world evidence, and streamlining the process by which existing therapies reach patients who need them. ERNs also possess the potential to favour the generation of less expensive, more reliable, quality EU-wide data. This workshop will focus on questions of data collection, i.e., how to create a system of information within ERNs which is sufficient and adequate for clinicians and the Industry to comply with their regulatory obligations, and on issues around working effectively with the Industry to optimise opportunities while avoiding any conflict of interest.

6-7 December 2017 – Rome, Italy

How can ERNs generate, appraise and utilise clinical practice guidelines to enhance the impact and deployment of consensus guidelines in national health systems

Context: The aim of this workshop is to create a harmonized 'dictionary' of terminology for ERNs to use for guideline generation and implementation, to confirm the role ERNs should play in the generation, appraisal, use and dissemination of guidelines, what methods should be used by ERNs when elaborating guidelines for rare diseases, how to promote good practices in relation to generating patient-friendly guidelines, how ERNs can utilize existing tools and resources, how often guidelines should be revised and updated, how ERNs may evaluate the impact of guidelines (e.g., in the healthcare system), and what does the 'status' of ERN best practice guidelines hold within individual Member States, i.e. how will national health systems utilise these outputs? ♥



Project Management Office

The Project Management Office of ERN GUARD-HEART coordinates the activities of the Network and the communication between Network members, European Commission, RD-ACTION, other ERNs and stakeholders. It also supports the Network in administrative tasks, organisation of meetings, designing and updating of the Network website, and creation and dissemination of the Newsletter.

For information and suggestions, and to provide content for the website and future issues of this Newsletter, please contact:



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