

Wednesday 20 September 2017



## GUARD-Heart

Gateway to Uncommon And Rare Diseases of the Heart



ERN GUARD-HEART BIMONTHLY NEWSLETTER

YEAR 2017 NUMBER 3

### Second Network Board Meeting

27 August 2017 - Barcelona, Spain

The 2<sup>nd</sup> meeting of the Board of ERN GUARD-Heart was held on Sunday 27 August 2017 at San Juan de Déu - Barcelona's Children Hospital, during the European Society of Cardiology congress. The meeting was participated by 22 of the 24 representatives of the participating centres of the ERN. The meeting was also attended by representatives of four patient advocacy groups across Europe (see page 2).



*Georgia Sarquella-Brugada & Arthur Wilde at the 2<sup>nd</sup> Board meeting. Georgia Sarquella-Brugada is the leader of the thematic area "Special Electrophysiology Conditions in Children". She hosted the 2<sup>nd</sup> Board meeting of the ERN.*

Arthur Wilde, the coordinator of the ERN, and Ahmad Amin, the project manager, gave a presentation about the current activities of the Network. Several issues were discussed among Network members including 2<sup>nd</sup> opinion service set up with e-tools provided by the EU, patient and family participation, research projects & patient registries, education strategy, definition of clinical outcome indicators, and the implementation of internal and external communication channels. The minutes of the Board meeting are available on the **IT Collaborative Platform**. To access the IT Platform, please follow the next steps:

1. Create an EU Login using the link:  
<https://webgate.ec.europa.eu/cas/>
2. Go to the link:  
<https://webgate.ec.europa.eu/ern/>  
and use your EU Login to enter the Platform.
3. Click on 'Access/request access to my network' ♥

### CALL FOR PARTICIPATION

#### CASQ2-related CPVT Gene Therapy



18 September 2017 - San Francisco, USA

Audentes Therapeutics is developing AT307, an adeno-associated virus (AAV)-mediated gene therapy for recessive CASQ2-related CPVT. AT307 is comprised of an AAV vector containing a functional copy of the CASQ2 gene, with the goal of delivering functional copies of the CASQ2 gene to cardiomyocytes, where it is expressed to restore the CASQ2 protein levels and reduce the risk of life-threatening arrhythmias. Results from a pre-clinical study in murine knock-in models were published by Dr. Silvia Priori's team in Pavia, Italy, and showed that after a single administration of AAV-CASQ2 gene therapy, CASQ2 protein levels were significantly recovered, protein interactions and cellular architectures were restored, and the number of arrhythmic episodes was significantly reduced. Histological analysis of cardiomyocytes at 12 months showed no structural abnormalities or inflammation, and no safety findings were reported in this study.

Audentes Therapeutic uses among others the ERN GUARD-Heart platform to select the centres with potential patients for this study.

For more information, please contact the project management office of ERN GUARD-Heart.



## Patient Advocacy Board

27 August 2017 – Barcelona, Spain

The 2<sup>nd</sup> Network Board Meeting was attended by Ms Lenja Wiehe, the representative of EURORDIS (a non-governmental alliance of patient advocacy groups that represent 761 rare disease patient organisations in 68 countries) and representatives of four European patients advocacy groups (ePAGs):

- “Association des Maladies héréditaires du Rythme Cardiaque” (AMRYC) - France (Sophie Pierre)
- “Cardiac Risk in the Young” (CRY) – UK (Michael Papadakis)
- “Hart4Onderzoek” (H4O), Netherlands (Patricia Vlasman and Dayenne Zwaagman)
- “Síndromes Arrítmicos relacionados con la Muerte Súbita” (SAMS) - Spain (Ester Costafreda)

After an introductory presentation by Ms Lenja Wiehe, ePAG members provided talks about their groups and activities, and expressed their devotion to establish the Patient Advocacy Board of ERN GUARD-Heart. The Patient Advocacy Board is responsible for bringing the voice of patients and families to the Network Board and to support ERN activities on patient engagement. During a parallel meeting after the 1<sup>st</sup> part of the Board meeting, ePAG representatives decided to delegate Ms Ester Costafreda (SAMS) and Ms Patricia Vlasman (H4O) to represent the Patient Advocacy Board in the future meetings of the Network Board. In addition, the Swedish Heart Children's Association (Hjärtebarn) was invited to participate in the Patient Advocacy Group. More information about the ePAGs is available on the [website](#) of ERN GUARD-Heart. ♥



Representatives of the European patients advocacy groups (ePAGs) AMRYC, H4O and SAMS after establishing the Patient Advocacy Board of ERN GUARD-Heart at the Network Board meeting in Barcelona, Spain.

## Cross-cutting action groups

18 September 2017 – Amsterdam, Netherlands

To facilitate cross-cutting actions, ERN GUARD-Heart, has defined 5 dedicated Cross-cutting Action Groups to conduct specific activities on areas that are relevant to all groups of rare and complex heart diseases. Current members of the Cross-Cutting Action Groups are:

### Action Group 1: Knowledge Management

- Ruxandra Jurcut (Bucharest, Romania)
- Anwar Baban (Rome, Italy)
- Sonia van Dooren (Brussels, Belgium)

### Action Group 2: Ethics and Policy

- Eloisa Arbustini (Pavia, Italy)
- Ahmad Amin (Amsterdam, Netherlands)

### Action Group 3: Research and Funding

- Peter Schwartz (Milan, Italy)
- Pablo Garcia Pavia (Madrid, Spain)
- Giuseppe Limongelli (Napels, Italy)
- Arthur Wilde (Amsterdam, Netherlands)

### Action Group 4: Teaching and E-learning

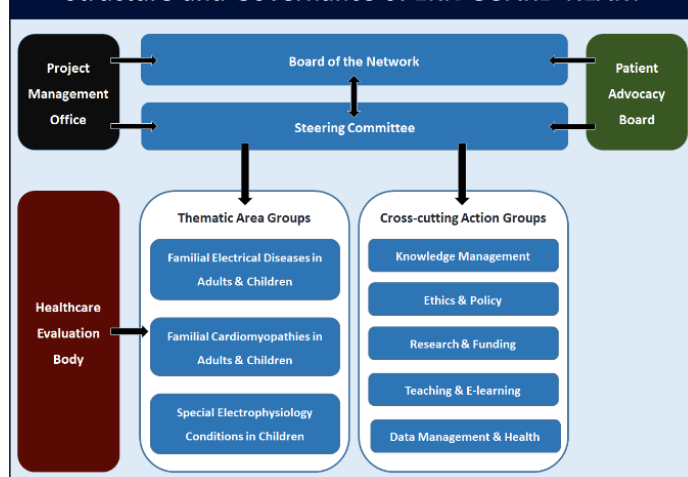
- Elijah Behr (London, UK)
- Jacob Tfelt-Hansen (Copenhagen, Denmark)
- Ruxandra Jurcut (Bucharest, Romania)
- Anwar Baban (Rome, Italy)
- Stellan Mörner (Umeå, Sweden)

### Action Group 5: Data management and E-health

- Sonia van Dooren (Brussels, Belgium)
- Ahmad Amin (Amsterdam, Netherlands)

ERN members willing to join the Groups can contact the project management office for information about the activities of the Groups. In the initial phase, a maximum of 5 participants per Action Group will be pursued. ♥

### Structure and Governance of ERN GUARD-HEART





## ERN GUARD-Heart Affiliation

15 September 2017 – Amsterdam, Netherlands

Official membership of ERN GUARD-Heart can be stated in scientific articles as an affiliation using the following text: *Member of the European Reference Network for rare, low prevalence and complex diseases of the heart - ERN GUARD-Heart*. Preferable as an additional affiliation and when that is not possible in the acknowledgements.

Official membership of ERN GUARD-Heart can be stated in scientific or educational presentations and press releases with the official logo of the ERN, the logo of the Member Healthcare Provider (HCP) or by following text: *Member of the European Reference Network for rare, low prevalence and complex diseases of the heart - ERN GUARD-Heart*. ♥

## Deliverables on IT Platform

30 August 2017 – Amsterdam, Netherlands

Eight deliverables of ERN GUARD-Heart Project have been submitted timely to the European Commission (EC). These deliverables were all approved by the EC. The approved deliverables are rendered available on the IT Collaborative Platform.

**It is of utmost importance that all ERN members gain access to the IT Platform, as the numbers of access requests and documents views will be used as a key performance indicator by the European Commission to assess ERN GUARD-Heart.** ♥

## Endorsement policy of the ERN

15 September 2017 – Amsterdam, Netherlands

Live events, e.g., meetings, congresses, and courses with physical or virtual attendance of participants, can be endorsed by ERN GUARD-Heart only if they comply with the following set of rules:

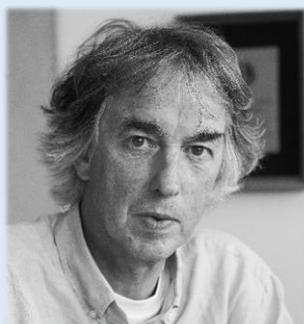
- at least one official Member of ERN GUARD-Heart has organized or contributed to the organization of the live event (official Members of the ERN are healthcare providers approved as Member of the Network by the Board of Member States);
- no Member of ERN GUARD-Heart should receive any financial or non-financial support for the specific purpose of endorsing the live event;
- the event must not promote single products/devices;
- learning objectives and expected outcomes of the live event must be clearly defined;
- the programme of the event must be in line with the best practice tools implemented by the ERN GUARD-Heart, i.e., :
  - guidelines set by the ESC;
  - expert consensus recommendations of the Heart Rhythm Society (HRS), European Heart Rhythm Association (EHRA), Asia Pacific Heart Rhythm Society (APHRS), and Association for European Paediatric and Congenital Cardiology (AEPC);
  - international task force consensus statements.

An template of the endorsement letter is available on the IT Collaborative Platform. **See instructions on page 1 of this Newsletter to access the IT Platform.** ♥

## 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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