

## Report CEF Workshop

3 November 2016, Paris

### Update on ERN applications and endorsement of Centres of Expertise by Member States

Yann Le Cam gave an up-date on the ERN application process and the endorsement of Centres of Expertise by Member States.

The main messages of this session were that National Alliances should know the full members in their country when ERNs are launched and to prepare for the next round of full member HCP affiliation:

Further, Alliances could perform a gap analysis for each ERN in their country & monitor implementation of plans of successful HCPs and potentially support more HCPs wishing to join ERNs

To help ensure equal benefits of ERNs across Europe, Alliances could identify national hubs, affiliated & collaborative centres to create national networks for better organisation of care, improved care & diagnosis and promote the integration of research infrastructures (IRDIRC, RD Action, EURORDIS, H2020, EJP) and ERNs in the context of national strategies on RDs across Europe

Finally, National Alliances can play an important role in promoting the adoption of ERN evidence-base practice, guidelines, etc. in local treating hospitals and drive quality outcomes at local level

### EURORDIS & Update on ePAG activities and ePAG Leadership Training

Lenja Wiehe gave an up-date on current ePAG activities including the newly developed Leadership Programme for ePAG representatives.

In summary, through the ePAGs, EURORDIS ensures a **streamlined & comprehensive** patient representation in ERN governance structures. The recently launched matchmaker tool supports the achievement of a **fair** patient representation across diseases and the EU by encouraging patient organisations to join one or several ePAGs. **Support** for ePAG representatives is provided with information, online communities and communication support. Finally, via the Leadership Programme, EURORDIS **builds** the capacity of ePAG representatives to engage with & lead in ERN governance via Leadership Programme.



Co-funded by  
the Health Programme  
of the European Union

## Public consultation on the future of HTA cooperation in Europe

We're in an exciting phase: there is a momentum to launch a key proposal in Europe to create the **European Agency for Health Technology Assessment**.

Currently, all European countries have their own, different HTA practices; The European Network for HTA (EunetHTA) has existed for 10 years now. 79 agencies and NGOs collaborate to define and implement a sustainable EU cooperation on HTA, but the EU funding for this network ends in 2020. Until January 2017 a very important consultation from the European Commission, to prepare the future of EU cooperation in HTA, maybe with a new legislation and the creation of a EU agency for HTA. Is open to all interested parties.

All NAs are encouraged to participate in the consultation and to invite their members to do so. EURORDIS organizes 2 webinars to discuss the consultation and propose a common approach.

## Presentation & discussion of the position paper “International joint recommendations to address specific needs of undiagnosed rare disease patients”

EURORDIS, together with SWAN UK (the support group run by Genetic Alliance UK), the Wilhelm Foundation, Rare Voices Australia (RVA), the Canadian Organization for Rare Disorders (CORD), the Advocacy Service for Rare and Intractable Diseases' stakeholders in Japan (ASrid) and the National Organization for Rare Disorders (NORD) jointly submit a list of recommendations to address the specific needs of patients without a diagnosis urging all stakeholders to recognise undiagnosed patients as a specific population within the rare disease community. Undiagnosed rare disease patients require the availability of a complete health and social care pathway in advance of receiving a diagnosis. Such care should promote their chances of receiving an accurate diagnosis in as efficient and timely way as possible, while ensuring that, until a diagnosis is made, they nevertheless receive the best possible health and social care. These recommendations also highlight the importance of promoting ethical and responsible international data sharing to help inform a clinical diagnosis, accelerate research into novel conditions and provide insights into disease mechanisms. Furthermore, knowledge and information sharing among all stakeholders should be optimally coordinated and fostered so that patients can access appropriate resources in a timely and efficient manner.

Following its presentation at the joint CNA/CEF meeting, the recommendations have been presented at the Undiagnosed Diseases Network International Conference in Tokyo and are in the process of being adopted by the consortium.



Co-funded by  
the Health Programme  
of the European Union

## “Compassionate Use” Position Paper

Negotiating a CUP is one of the most important actions a patient organisation may conduct. They are a societal response to desperate situations, but inequities by disease and country prevails (100% of HIV products benefit from a CUP, France: 73% of OMPs that are authorised are available in average 36 months before the marketing authorization)

EURORDIS proposes one of the following options:

1. Promote the French ATU system, probably the most efficient compassionate use scheme so that every Member State adopts it;
2. or adopt an EU Regulation which would confer a greater role in the organisation of CUPs upon the EMA;
3. and/or apply the Directive on Patients’ Rights in Cross-Border Healthcare to include compassionate use as part of the care basket so that patients can benefit from these treatments wherever they live in the EU;
4. Generalise the Medicines Adaptive Pathways to Patients, where the EU regulator may authorise a medicine at an early stage and in a limited group of patients that are in high need for the product,
5. Amend the EMA guidelines for compassionate use, the role of the EMA could be reinforced with or without legal changes to the pharmaceutical legislation.

## How to use the INNOVCare survey results for your advocacy work?

**Sandra Courbier**

The aim of the presentation was to update CNA and CEF members about the first survey carried out through Rare Barometer Voices, detail possibilities offered by the project for their own advocacy work and encourage them to disseminate the link of the survey.

Rare Barometer Voices is a survey panel, gathering rare diseases patients who are willing to participate in EURORDIS surveys and studies. A reminder about the Rare Barometer Voices project was given, detailing the main features of the programme (surveys carried out in 23 languages, data protection rules of the project etc.). The number and repartition of registration so far (3850 have registered for Rare Barometer Voices, in 48 European countries) were also detailed.

The first survey carried out through this project was presented. This survey is about the impact of rare diseases on daily life and was launched in August 2016. The composition of the Topic Expert Committee, a committee composed of patient organisations, academic and corporate partners, all experts on the subject studied, was detailed. Processes put in place to design the survey were also presented, including patient organisations involvement in the project. Timing and organisation of the



Co-funded by  
the Health Programme  
of the European Union

This workshop has been funded with support from the European Union’s Health Programme and by the Association Française contre les Myopathies – Téléthon (AFM). This material only reflects the views of the author, and funders cannot be held responsible for any use which may be made of the information contained herein.

survey was presented in order to encourage member organisations to disseminate the survey before the final fieldwork deadline (mid-November 2016).

The questions of the surveys were presented in order for member organisations to determine how the questions could be of use for their advocacy work. Examples and ways of using the results coming from EURORDIS past experiences (12000 Voices of patients survey, inclusion of survey results in project application etc. ) were also shared and suggested in order to help member organisation to include the results of the surveys in their advocacy and communication work.

Communication tools available for patient organisations (email templates, translated Facebook posts etc. ) were also listed in order to help the dissemination work.

In the end, the questions and answers session was an opportunity to exchange on good practices about ways of disseminating the survey. News ideas such as the necessity to have paper questionnaire available to facilitate dissemination came up.

## **INNOVCare project: update on recent actions to promote holistic care for rare diseases**

The INNOVCare project presentation consisted of an update on the key actions developed during the first year of the project (10/2015-10/2016):

- [Survey](#) on the impact of rare diseases on daily life (via Rare Barometer);
- Design of the INNOVCare pilot - [case management](#) for RDs (Romania, intervention starting in January 2017);
- Creation of the European Network of Resource Centres for Rare Diseases (ongoing, launch in spring 2017);
- Organisation of [workshops](#) focused on integrated care for RDs (September 2016, Sweden; October 2016, Austria);
- Creation of [INNOVCare's Advisory Group](#) and involvement of policy makers in the activities of the project.

More information on the project available at [innovcare.eu](http://innovcare.eu).

## **What's next for the CEF? Discussion**

All representatives participating expressed their interest in continuing the work of the Council and meeting once a year. The meeting could be organized in other cities. EURORDIS should provide a few travel bursaries for CEF representatives who are not covered by the budget of their organisation. The CEF is pleased to share part of the workshop with the Council of National Alliances,



Co-funded by  
the Health Programme  
of the European Union

This workshop has been funded with support from the European Union's Health Programme and by the Association Française contre les Myopathies – Téléthon (AFM). This material only reflects the views of the author, and funders cannot be held responsible for any use which may be made of the information contained herein.

To enhance exchange and discussion, the creation of a CEF facebook page was proposed. This [closed group page](#) is now accessible to all CEF representatives.

## **Discussion of the RD Action concept paper on Prevention,**

The draft concept paper on Prevention was discussed by the group, as this paper when first sent to the rare disease community raised a lot of mainly ethical question and concerns.

EURORDIS staff has already sent a list of amendments to the drafting group and will integrate the comments made at the CEF meeting, to ensure that the paper reflects the patient's views and concerns. The CEF will receive a new draft shortly. In the meantime, background information documents will be posted on the CEF page on eurordis.org

## **Peer to peer: sharing of experience and knowledge**

The European Gaucher Alliance presented two projects: Go for Gaucher, an initiative for young people living with Gaucher disease, bringing the next generation forward and the International Gaucher Awareness Day.

Helma Gusseck presented a research project on Retinitis Pimentosa.

Several topics were discussed with the whole group:

- Going global: How to turn a European Federation into a Global one?
- -How to deal with a membership request from a patient organisation when there already is an organisation for the same disease in the country/network?

-----



Co-funded by  
the Health Programme  
of the European Union