8th CEF Workshop

27-28 October 2015, Paris

Report

All presentations on: http://www.eurordis.org/content/council-european-rare-disease-federations-documents

27 October

Joint Meeting – CNA & CEF

ECRD 2016 Edinburgh – Get involved! Sharon Ashton

The overarching theme of ECRD 2016 Edinburgh, 26-28 May, is: Game Changers in Rare Diseases. Delivering 21st century healthcare to rare disease patients: Together we can change the future!

NAs and patient organisations in general can get involved in the ECRD via fellowships, by presenting a poster, by sharing a specific video or by helping to identify speakers.
Patient organisations can as well sign-up to participate in the research speed-networking session; share innovative strategies / approaches / services or projects during the open house / « soap box » session and disseminate promotional flyers.

The new RareConnect –Why & how to get involved for NAs and EFs? Denis Costello

RareConnect now offers 79 rare disease communities, with 269 volunteer moderators, representing 660 rare disease patient group partners, with 60,000 visitors/month. In addition to the 4 staff people in Barcelona, 4 more staff have been hired, in Zagreb and Belgrade, to significantly impact outreach and operational capability on a global and regional level. Currently, RareConnect exists in 6 languages (EN, FR, ES, IT, DE, PT) with 2 pass translation (Google Translate + Human Translation) Croatian, Serbian and Russian will be added shortly.
One of the new features of RareConnect are the featured discussion groups, which allow patients without a disease specific community to ask their questions and connect with others.

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National Alliances are invited to participate in Governance Committee, to co-promote RareConnect in their communication. In return, RareConnect will show their logo on it's homepage as an official partner and share information on a regular level. European Federations can create RareConnect communities for their disease, in addition to the above mentioned participation.
The following participants volunteered to be part of the Rareconnect Steering Committee:
CNA: Cor Oosterwijk, VSOP, Dorica Dan, RONARD, Anna Arellanesova, CAVO, Lene Jensen, RDD, Alain Fontaine, Alliance Maladies Rares.
CEF: Tobias Arndt, EDRIC, Anke Widenmann-Grolig, EAT.

Inclusion of in-kind contributions into financial reports, Patrice Regnier

EURORDIS has developed a set of principles / recommendations on how to include volunteer activity in financial reporting, based on its own experience. These principles were presented at the CNA/CEF meeting, a memo version will be sent out to the two groups and published on eurordis.org, to help patient organisations integrate the resource that volunteers represent into their financial reports.

European Year of Rare Diseases, Valentina Bottarelli

We have received information from within the European Commission through a communication with a member of DG Communications at the EC which suggested that Juncker's Commission are reluctant to designate European Years (they have not designated a year for 2016 for example) and implied that if they were to designate a year, it would be in line with the ten priorities of Juncker's Commission, which have a distinct economic focus. Nevertheless: all is not lost. The lack of designations could leave us with a blank canvas to launch the year as we wish, and perhaps look for a different sort of endorsement from the EC.
In any case, we still intend to go ahead with a European Year for Rare Diseases, even if it will be without official designation from the European Commission.
The European Brain Council was in a similar position and missed an official designation in 2014. Nonetheless they went ahead with an unofficial year this year, and in a recent meeting with EURORDIS described it as being a fantastic experience with the potential to create enormous impact.
Build a coalition. Engage all stakeholders and allow them to take ownership of the year themselves. Different partners could take leadership and sponsor different events and/or initiatives as long as overall coordination and global vision is provided by EURORDIS.
Streamline our events: from benchmarking activities it is clear that many of our activities could be communicated within the EYRD framework. Examples include conferences at the national and European level, surveys, exhibitions, workshops, media campaigns, such as a media relay between member state, via social media and local television and radio etc. In addition, the year allows a platform to launch or present other topics such as the foresight study.

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Policy opportunity: The 10 year anniversary of EU rare diseases actions and the 20 year anniversary of orphan medicines policy gives a good platform for further advocacy, at the time of setting the agenda for the following years.
The idea of an ***International Year for rare Diseases*** was proposed at the CNA/CEF however, our initial impression is that this is not in line with the key objectives of the EYRD2019 when it was first established.
2019 was chosen to coincide with the European anniversaries of the Regulation on Orphan Drugs and the Commission and Council recommendations on Rare Diseases in order to push for a further policy push. If it were to become international we would lose this hook with the European Institutions.
In addition we are not sure if we are ready for this. An international year would require a mature network. It also limits us from doing another year soon after (when perhaps RDI will be more developed).

Rare Barometer Programme Plan, Sandra Courbier

Following the EurordisCare Survey Programme which gathered the perspective of 12 000 patients, EURORDIS is now launching its new survey research programme : the Rare Barometer. This initiative aims to make the voice of rare disease patients stronger by improving the understanding of their needs and expectations and giving solid evidence about it. It will cover Europe at large (48 countries) and gather the perspectives of people living with a rare disease, their families and patient representatives. This project is dedicated to becoming an advocacy tool for EURORDIS and its members.

In order to carry out surveys on a regular basis and to give scientific consistency to the project, EURORDIS is creating a community of people living with rare diseases named Rare Barometer Voices. It will be made up of patients that are willing to take part in surveys on a regular basis. This panel will be owned solely by EURORDIS and will strictly adhere to the European data protection standards to ensure data confidentially. The project has been approved by the CNIL (French data protection authority).The questionnaire and the information given will be translated in 23 languages.

Patient organisations have several ways to participate in this initiative:

- Register to Rare Barometer Voices and take part in the upcoming surveys;
- Share the topics and analysis of studies and surveys that their organisation has previously carried out to help EURORDIS base the surveys on existing knowledge;
- Fully participate in this novel and innovative programme and take part in the design of the project, either the overall project or by survey.

A template email will be shared with member organisations to facilitate the dissemination of the registration link.

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Discussion of EURORDIS Membership Criteria for European Federations, Zoe Alahouzou

During the EURORDIS General Assembly in May 2015, a first discussion was had regarding the funding difficulties faced by patient organisations (mostly European Federations). Beyond having limited sources of funding some POs are also based in countries where in-kind contributions and volunteers cannot be monetised, which further exacerbates their situation. These difficulties hinder POs from fully adhering to the EURORDIS membership criteria, namely the point on financial independence [*Financial independence, particularly from the pharmaceutical industry (max. 50% of funding, from several companies)*] despite their longstanding and overall positive track record as patient organisations.

Final decisions as to EURORDIS membership have always been within the remit of the Board of Directors and criteria can be waived taking into account individual circumstances. However, the suggestion made during the General Assembly that European Federations may receive a waiver and have the information of their waiver made public on the EURORDIS website has not been deemed optimal since it may weaken their image and their position as a full member.

The discussion and vote that took place during the AGA in Madrid was productive and indicated that our criteria are accepted by a broad percentage of our membership (91 Yes, 8 No, 7 Abstentions) but there are actions however that can be taken to ease situation of European Federations. The actions proposed are as follows:

- European Federations are encouraged to include information regarding in-kind contributions in financial information: this is a legitimate source of funding that can be taken into account by EURORDIS even if this is not included in formal accounting. The information can be included as an annex in annual financial reports.
- The EURORDIS membership criteria will be updated to specify that the specific funding criteria can be waived based on the Board's individual assessment of track record of activity and reputation; independence and structure of funding

Rare Diseases International – Progress & Membership of International Federations, Paloma Tejada

Rare Diseases International (RDI) is the Global Alliance of People Living with Rare Diseases of all Nationalities across all Rare Diseases. It is an informal network that will eventually be registered and have a legal identity – a later decision by its members. It currently has 28 member organisations. Membership is open to umbrella organisations : National Alliances, representing several rare diseases in one country or International Federations, representing the same disease or family of diseases in several countries.

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RDI is needed to:

- To unite, expand and reinforce the RD movement of patient organisations and patient advocates
- To enable the emergence of RD as a public health & research priority internationally and to enable the emergence of a strong common voice on behalf of the +300 million people living with rare diseases (PLWRDs) around the world
- To influence international organisations (UN, WHO)
- To enable the local rare disease patient groups to act at national, regional, international levels and to interact with other areas in the field of RDs
- Most international initiatives are ad hoc and linked to a particular diseases. RDI is an opportunity to work globally and to create a global RD community through advocacy and exchange of experience.

RARE-Bestpractices project, Juliette Senecat

How can European Federations benefit from this project?

- Identify and access guidelines developped in different EU countries and languages.
- Share guidelines internationally with patients and other stakeholders.
- Access information about the quality of guidelines.
- Access training resources and methodological support.

The project has developed a RD guidelines' database: http://rbpguidelines.eu. The collection of guidelines has started for an initial list of 44 RD. The project is using the AGREE II tool to assess the quality of guidelines. Key issues to assess the quality of RD guidelines are rigour of development, external review by experts (these should absolutely include patients, carers and/or patient groups), editorial independence (limited because of the scarcity of experts), overall guideline assessment.

Next steps: how can CEF members be involved in the project?

- Contribute to the search, collection and appraisal of guidelines.
- Disseminate project tools.
- Attend project trainings: 3-4 December 2015, Rome ; 10-12 February 2016, Milan.

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Awareness Days –network building and communicating, Lara Chappell

Several European Federations spoke about their experience with their own awareness days. Many positive stories and successes.

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EURORDIS works traditionally with National Alliances on Rare Disease Day but would like to also investigate with Federations how they can be involved in Rare Disease Day. Of course they can download materials and build awareness for Rare Diseases and their own disease.

All the information can be found on rarediseaseday.org. With any questions write to rarediseaseday@eurordis.org.

INNOVCare project - innovation in social care provision for complex diseases, Raquel Castro

The new EU-funded INNOVCare project (2015-2018) addresses the social challenges faced by people living with a RD and gaps in the coordination between medical, social and support services in EU MS.

The project will develop and test an innovative care pathway that links health services with the social and support services that people with a RD and their families access on a daily, by using a resource centre for RD and regional case managers.

This pathway ensures the transfer of information/expertise between service providers; centralises the coordination of care in an effort to relieve the burden of care management for patients and families; and is expected to result in efficiency gains for national authorities.

Objectives of the project

- Assessment of social needs of people living with a RD and their families in Europe and analysis of existing care models in a selection of EU MS
- Implementation of a pilot of the innovative care pathway in Romania
- Evaluation of the socio-economic impact and cost-benefit analysis of the care model
- Analysis of opportunities to upscale the model to other MS and beyond RDs
- Exchange of good practices (via an European network of resource centres)

EURORDIS' role

- Representing people living with a RD and their families
- Communication, ensuring the link to the RD community and stakeholders (Work Package - WP3);
- Assessment of social needs of people living with a RD and analysis of care pathways in MS (WP4);
- Facilitation of the creation and animation of the European network of resource centres (WP5).

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CEF Training: European Reference Networks – Getting ready!, Matt Johnson

Overview of Assessment Process

The Council of European Federations capacity building workshop gave participants secured a detailed understanding of the high level overview of the Assessment Process, content of

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Assessment Manual and Toolbox and the proposed model for Operational Criteria that form the technical proposal that will be used in the assessment process for ERN applications in 2016. The workshop gave a more in-depth understanding of description of the EC ERN Assessment Manual and Toolbox; Understanding of the Operational Criteria, and; Overview of the decision points and guidelines.

The main discussion generated in the workshop was focused on the key decision points in the assessment process and strategic aspects in the preparation phase of ERN applications, specifically on the scope of an ERN application under the Commission Expert Group for Rare Diseases' Addendum on thematic groupings.

Participants gain an understanding of how network applications would be assess by the published decision guidelines, that required an overall compliance rate of ≥70% of the total maximum score and that each theme, at least 70% compliance against the maximum score.

There was discussion generated that explored the following aspects of the 1st ERN Call for applications:

- How an application would develop the scope and purpose of the network in terms of rare diseases under the thematic groupings
- An understanding of how the specific criteria for a HCP would need to fillfull to be part of a network application
- What the critical mandated steps are need to develop an application, eg: 10 HCP in 8 MS, all HCP need to be formally endorsed by their MS to take part in an application
- How Associate National and Collaborative Centres are identified at a Member State level
- The role of patient groups in the decision making structures of an ERN
- The timeline for developing and submitting an application and the assessment process

Engaging your organisation

The Council of European Federations workshop explored the impact of creating European Reference Networks under the Commission Expert Group for Rare Disease's groupings of rare diseases under over-arching thematic ERNs. Specifically, the workshop examined how structuring patient groups, through a democratic process, would optimize the empowerment of patient groups into the decision making structures of ERNs. The CEF members secured an understanding of the EC ERN Delegated Acts and the strategic recommendations of the CEGRD and their application of patient centred care and empowerment of patients in the newly formed ERNs. There was clear support from the workshop that the ambition to ensure '*every person with a rare disease has a home, a*

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pathway, under one ERN' as this maximized the inclusion of all people living with a rare disease and ensured equality for all. There was strong support for the formation of EURORDIS Patient Advocacy Groups. The CEF debated and named EPAGs in the workshop. The scope and function of EPAG was also explored and has become the basis for the terms of reference for EPAG, namely:

- One EPAG per each rare disease grouping, to empower patients in the decision making structures of ERNs

- EPAG to be inclusive for all patient organisations, being inclusive and supportive of even the rarest diseases

- Enabling structure patient engagement at a national, European and International level.

- To have a 360 degree view of the patient pathway, from public health, healthcare, research, social care, human and patient rights

The workshop identified and debated the challenge of aligning multi-system rare diseases to one thematic grouped ERN. That whilst one RD may provide the leadership of care, it was essential that ERNs cooperate between each other to meet the complex needs of these rare diseases.

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