

Council of European Rare Disease Federations (CEF)
2nd workshop, Brussels - 3 December 2009
Report

Representatives of 20 European Rare Disease Federations (EFs) met in Brussels for the second Workshop of the CEF (*participants list attached*)

Yann Le Cam (YLC) opened the meeting reminding participants of the outcomes of the first workshop, that took place in Athens in May 2009. The Terms of References of the CEF (*attached*) were adopted at that meeting.

The development of an action plan has been postponed. Karen Ayach, acting as consultant, will interview some representatives of European Federations to identify areas where common work is possible and define key elements for a Road Map. The Draft Road Map will be discussed with EURORDIS' Board of Directors (BoD) and CEF at large, before final adoption by the BoD. We are aiming at a Draft by June and a final adopt by Year End.

The development of an online community for the CEF has been postponed as well. EURORDIS will launch the first patient communities in December with 3 months delay. After testing the tools with patients, the CEF/ On Line Advocates Community could be launched in the second quarter of 2010.



YLC informs participants that they will increasingly be invited to endorse certain position papers, such as currently the CAVOD and participate in the development of future position papers. The next important document will be on "Why to do research on rare disease?". This paper, needed for advocacy for the FP8, will present political arguments in favor of rare disease research. We'll circulate the paper to you for your input.

The CEF shall be a platform where European federations can work together on specific common projects benefitting them all. Some of the projects will be presented in this workshop.

Some participants raise the question of the link of the CEF's work with national plans and strategies for rare diseases, in order to coordinate activities across Europe.

EURORDIS has so far considered national plans an issue involving the Council of National Alliances (CNA), in the framework of, notably, the *Europlan* project.

But YLC agrees that a link/overlap between the work of the CNA and the CEF on this topic could be beneficial. The ideal way of working together on this topic could be the future Online Advocates Community. However, a clear focus for the CEF is needed, not all themes can be treated by this Council, we need as much as possible to have each task force, committees

and council to work on their respective aims, completing each other's actions, so that we are more able to reach our common goals. From this discussion, the idea of merging the CNA and CEF On Line Community emerged as a way forward to share more information and experience across.

The EU Committee of Experts on Rare Diseases (CERD) will be created in February next year, where national and European policies concerning rare diseases are discussed. EURORDIS should have representatives in this committee and will report back to the CEF.



Rare Disease Day 2010

Presentation by Paloma Tejada (attached)

Theme: Rare Diseases a public health priority

Focus: Bridging patients and researchers

Main messages:

- Rare Disease Research is an important area in research
- Rare Disease Research needs to be better funded
- Patients and Researchers win by working together

European Event

A conference "Rare Disease Research in Europe – Bridging Patients & Researchers To Build the Future Agenda of Rare Disease Research in Europe" will take place in Brussels on 1st of March 2010. This conference, a EURORDIS initiative, is co-organised with E-Rare, in partnership with Orphanet and Europlan.

Presentations will be made on the E-Rare survey on researchers and policy makers priorities in RD research, RD Platform's study on determinants of RD research and the EURORDIS Survey "RD Patient Groups' Role & Priorities in Research".

The morning session will be devoted to discussing the needs, expectations, priorities and bottlenecks encountered by all relevant stakeholders (namely patients and researchers) in rare disease research. The panel discussion will revolve around the questions: What are the priorities for resource allocation at EU and national level? How can patients & scientists bridge the gap to boost RD research?

Taking into account the state of affairs described in the morning, the afternoon session will seek to answer the question: How to build a strong policy for RD research? Presentation from the EC DG Research, EUROPLAN Project, E-Rare and EURORDIS will address funding allocation for RD in FP7 and FP8, as well as the importance of coordination of national and European efforts and the importance of linking national initiatives with European initiatives such as European Reference Networks of Centres of expertise and European Research Networks.

EURORDIS will present arguments to invest in RD research (draft paper it is currently being developed)

Survey:

About 300 patient organisations across Europe have participated in an On-line survey on:

- How patient organisations support RD research (financial and non financial)
- What are the priorities and bottlenecks in RD research?
 - Results will help to acknowledge the role of patient organisations in research
 - Results will help you advocate for more and better research for RD at the national level
 - Results will contribute to shaping the future research agenda of the European Union

The results of this survey will be announced at the European event March 1st and will help shape the future research agenda of the EU and National Plans.

How can federations participate in the RDDay 2010?

- Organise awareness raising events across Europe
- Announce their events on the “Europe” page of www.rarediseaseday.org
- Join the ‘Friends of RDDay’ list
- Attend the European event on March 1st
- Invite two key people to this event
- Nominate a researcher to the Hall of Fame
- Organise an event in a Science Museum
- Organise a Play Decide Session
- Contact MEP
- Send out press releases to their press contacts
- Inform their members
- Link to International Facebook Group

It’s important that federations inform their national member organisations, since there isn’t always a national alliance in each country.

A poster specifically designed for the European event and showing the logos of European Federations is discussed. However, most federations feel that such a poster isn’t necessary. The posters are rather for national organisations, therefore to be coordinated by national alliances.

Some federations would like to receive the outcomes of the survey for the disease they represent. Dr Faurisson, in charge of the analysis of the survey, will, on demand, share the results per disease, provided there is significant number of participating associations for the specific disease.

The global results of the survey will be available in January 2010.

EURORDIS will issue a fact sheet about RD research next year. We are currently drafting a position paper "Why research on rare diseases?". The draft will be presented at the European event of Rare Disease Day (March 1st) In the meantime, we'll try to obtain information from the European commission about which diseases and networks were supported by the FP6 and FP7.



Polka Play Decide

Presentation by Aiki Giannakopoulou & Anna Kole (attached)

Anna presented an update of the Play Decide element of the POLKA project. The six topics represented in Play Decide kits include:

- Neonatal screening
- Is there any upper limit of spending on a single patient?- the case of Orphan Drugs
- Cross-border healthcare
- Diagnosis, information to the patient, genetic counselling
- Stem cells
- Pre-implantation genetic diagnosis (PGD)

All kit materials will be translated into all European languages. European Federations are invited to participate in the checking and validation of the translations. The material will be available for download on the website <http://www.playdecide.eu> by the end of January 2010. Aiki presented a new interface for uploading the results of these games will also be available here by this date. This new results interface will allow patient organisations to identify themselves as participants of the POLKA project. This is important as patient organisations that organise the largest proportion of sessions in relation to their membership will be recognised with an award. The new results interface will also provide an "auto-generated" summary of the sessions that may be useful for reporting purposes.

Members of the POLKA project team are happy to come and visit federations to help organise sessions. POLKA project team is happy to:

- Introduce the project and Play Decide exercise to your federation staff and board
- To help organise sessions in conjunction with a previously planned membership meeting
- Help organise sessions as a separate event dedicated solely to Play Decide
- Help in any other way needed to reach our goal of 600-1000 sessions across Europe during the duration of the POLKA project.
- Help organise workshops for moderators

Based on the outcomes of the games, provided we obtain a critical mass, we can advocate based on the results, compare results between rare disease patients and the general population. Although it will be a long-term process, results of the play DECIDE games may contribute to position papers.

Support to European Federations

Presentation by Yann Le Cam (attached)

EURORDIS supports European Rare Disease Federations in a number of areas:

- Exchange of Information, Experience and other Networking Activities
- Access to Capacity Building and Empowerment
- Enabling Involvement in Addressing Common Issues in EU Policy
- Community Building of European Rare Disease Federations
- Raising Awareness

Concerning the financial support, 3 hypotheses are discussed.

- 1) Direct support from EURORDIS to EF (for the organization of certain meeting)

The first hypothesis has many drawbacks, one of which the matching fund of 40%, obligatory in any grant received by the EC, that EURORDIS cannot provide.

EFs agree that they could provide the 40%. It is possible to use financing from pharmaceutical companies for these 40%.

EFs agree to focus support on meetings to develop ERN and/or on meetings in new & future MS, rather than on board meetings. However, a certain level of flexibility is needed.

- 2) Mini Operating Grants (Direct DG SANCO to EF)

The EC is not fond of this idea. Operating grants were created to support a minimum number of large federations acting in priority areas of the European health program (such as cancer or obesity), not for disease specific groups! In addition, the EC does not want to fragment resources nor add more work to their load.

- 3) Facilitate access to existing sources of funding at EU level

DG SANCO manages the EU Public Health Program, which issues the Annual Work Programme and created the Executive Agency for Health & Consumers (EAHC), in charge of managing the money, with the following financial instruments:

Projects are based on a call for projects, based on the annual work program of the DG SANCO.

Conference grants: cover expenses for a conference, on a disease or group of diseases, but including different areas of policy of health care (consensus conference)

Operating grants were created to support a minimum number of large federations acting in priority areas of the European health program

Joint actions: Projects of public institutions co funded by DG SANCO and Member States (For example Orphanet)

Call for tender: Detailed call for a specific purpose

In any case, the European Commission grants 60%, beneficiaries have to find 40% of co-funding (matching fund). The grants are for European projects, a conference therefore should take place in one of the MS, but for example the travel expenses for a speaker from out of Europe can be included.

YLC invites the EFs to have a look at the DG SanCo Work Program when it's available, end of December.

Each EF has to apply individually for a grant, so there might be competition between the federations.

EURORDIS proposes the organization of a capacity building workshop for European federations, where representatives of the DG SANCO/EAHC and consultants could teach patient representatives how to apply for conference and project grants, using case studies.



Template Work Package for European Federations within European Reference Networks (ERN)

Presentation by Anna Kole (attached)

Pilot European Reference Networks of Centres of Expertise were expected to involve patient organizations in their project, but in reality, they don't or they do it in a very limited capacity. EURORDIS proposes the creation of a standard work package to encourage not only systematic involvement of EFs within ERN, but also to obtain funding for EFs to allow them to conduct this work.

The CEF agrees with this proposal. The following implementation is proposed, to take place before the summer 2010.

- The draft will be circulated by e-mail to all EFs of the CEF (attached)
- EFs make their changes in tracking mode and return it to EURORDIS by April 2010.
- The final version is submitted to the EURORDIS Board of Directors for validation
- The documents is sent out to ERN for feedback
- The document is submitted to the DG SANCO, Tony Montserrat and Nick Fahy
- The final version is submitted to the EU Committee of Experts on Rare Diseases (CERD) for endorsement by MS

In the long term perspective, it's important to note that the proposal for the: Cross Border Healthcare directive, provides for the development of ERN to be facilitated by Member State This will allow more ERN to be created eventually as obviously developing them in the

framework of a pilot programmed as is done now is not sustainable or realistic as the Commission cannot possibly fund ERN for all rare diseases.

Next CEF Workshop

The CEF agrees that the next CEF workshop should include the capacity building workshop on funding possibilities from the EU.

Speakers should include representatives of DG SANCO, the EAHC and consultants specialized in DG SANCO grants.

Therefore, the next work shop will need to be 2 days long. Possible dates:

- 24 & 25 June 2010
- 1 & 2 July 2010

EURORDIS will cover the travel and accommodation of one representative per EF member of the CEF. A second representative can of course take part, but the costs have to be covered by the EF.

The meeting will take place either in Brussels or in Paris, with a clear preference for Paris.

Participants expressed high degree of satisfaction from this meeting considering it was worth the time and efforts. It is noteworthy to point out that the level of English across all participants was excellent, discussions proactive and lively and consensus easily built.


