3rd CEF Workshop
Paris 1 & 2 July 2010
Report

Why and how involve your patient organisation in EMA activities, Lise Murphy:

With her presentation, Lise strongly suggested patient organisation working at European level become eligible to work with the European Medicines Agency (EMA, former EMEA)

Lise pointed out that patients are the experts of their disease, and that it's important that patients are heard at EMA level. She explained that many patients were a bit afraid before going to one of the EMA's meetings first, but then realised that there was nothing to be afraid about and that their voice was heard. Only patients their parents and careers, can effectively speak about quality f life, or on how a treatment is taken for example.
However, some patient representatives were not pleased with having to sign a confidentiality agreement, which makes them unable to share information gained at the EMA with their own organisation.

Lise then explained the checklist used by the EMA to verify if a patient organisation can become eligible.
To participate in EMA activities, Not-for-profit organisations which are patient focused, and whereby patients and/or carers (the latter when patients are unable to represent themselves) represent the majority of members in governing bodies.

These could be either/or:

- *general umbrella organisations (e.g. representing either European specific disease organisations and/or national umbrella organisations);*
- *European disease-specific organisations (i.e. representing national organisations or individual patients on acute and/or chronic diseases).*

For an application, the form must be filled out, indicating the mission, activities, representation (list of member organisations), structure (explaining for example how your Board of Directors is elected), Accountability and transparency. The following documents must be attached:

- Statutes
- Latest detailed financial report

Transparency: need to say what you do on your website, as well as sources of funding. Important because conflict of interest can arise . (please look at the Code of Practice for guidance!)
You can see on the website of EMA the applications of other organisations.
Lise indicated as well that being eligible at the EMA means recognition at EU level, which is a big bonus.

Lise asked if it was ok to ask how the federations are getting on with their applications in three months´ time and also that the participants form groups of three who can stay in touch with each other and give support to each other

John Dart: indicated that Debra International became members, the only difficulty had been to explain their very small budget. However, if there is something EMA would like more information on, they come back to you.
Peter Finney: asked what to do in case of a drug that became licensed as an orphan drug and now is 20 times more expensive, who do we talk to?

Yann:invites participants to inform EURORDIS of situations such as this one at the very beginning, before the drug is approve. We can share the information with the CHMP, who gives marketing authorisation. The EMA does a scientific evaluation of a drug, but doesn't fix the price.

Road map Eurordis – European Federations and Action Plan

Yann Le Cam introduced Ms Karen Aiach, President of Sanfillippo Alliance, France, and as well consultant.
Karen Aiach is in charge of the Strategic Review of Eurordis, that was started 1.5 years ago. One of the results of the first part of this review was that the interaction between European Federations, National Alliances and EURORDIS is essential and needs to be enhanced.
Karen informed participants that she will contact some of them for an in depth interview to shape the collaboration between Eurordis and the European Federations
European Federations will be invited to fill out an online questionnaire, to define common priorities and goals in the next 5 years, discuss themes where collaboration is desired and the means to achieve it. Indicators need to be thought of as well, that will help to judge the success of the collaboration.
The final draft of the outcomes of this consultation will be presented at the next CEF meeting.
A list of volunteers for an in depth interview is established.

Rare Disease Day 2011, *Paloma Tejada*

Paloma Tejada presented the highlights of the third edition of the yearly Rare Disease Day (presentation can be uploaded here: http://www.rarediseaseday.org/article/rare-disease-day-2010-highlights.
In 2010, 46 countries , including 27 European countries, participated, making the day a truly international event.
While the overarching theme was "Rare Diseases, a public health priority", this year's focus was on "Bridging Patient and Research", reflected by the slogan *"Patients & Researchers: Partners for Life!*
Internet and social media once played an ever more important role in RDD. www.rarediseaseday.org got nearly 40.000 visits from 156 countries.
38 videos were shown on the RDD youtube channel and 269 pictures posted.
The Facebook group now has 10.238 fans and 778 followers on twitter.
The new tool "Friends of Rare Disease Day" was very successful, 189 people and/or companies signed up.
The Research Hall of Fame attracted nominations of 46 researchers from patient organizations all over the world.
Eurordis organized in collaboration with E-Rare, the European commission, Orphanet and Europlan, a workshop in Brussels on March 1st, bringing together RD stakeholders to discuss the future rare disease research agenda in Europe.
The Eurordis Survey "European RD Patient Groups in Research: current role and priorities for the future", was presented at this workshop.

National alliances and country organizers took advantage of RDDay to advocate at national level, organizing events with their MPs, policy makers and public authorities.. Local and national groups identified with the theme and put spotlight on research
General public events were organized across countries, and even the pope mentioned rare diseases in a speech that day.

The overarching theme of **RDD 2011** is still "RDD a public health priority" but a new focus is proposed: **Health inequalities.** This topic is in line with the EU Health strategy to reduce inequalities *"Solidarity in Health Initiative" which seeks to t*ackle gaps in health between and within MS, targeting vulnerable groups in particular. (see press release and EU document attached)
The advocacy objective at European level is to influence the third EU Public Health Programme
The European event will be organised with the following theme "RD as a priority in 3rd EU Public Health Programme" with the support of the DG SANCO
The date of this meeting will most certainly be the 22 February 2011.

Paloma invites all participants to send in examples of health inequalities in the field of RD. This can be in the form of patient testimonies or surveys/studies/ court rulings about health inequalities for your disease. Patient stories on video can be directly uploaded on to our Rare Disease Day Youtube channel by going to: http://www.rarediseaseday.org/yourstory

If you or someone from your federation or network would like to share his or her story with us orally or in writing please contact: paloma.tejada@eurordis.org or call her at +33 1 56 53 52 61

EURORDIS will send out a check list or form with the information we need for our documentation and website on Health Inequalities. We are counting on European Federations to provide us with the material necessary to feed the focus page on 'Health Inequalities' on the www.rarediseaseday.org website for the 2011 Campaign.

What can Federations do for RDDay 2011?

- **Download logo, banner, poster**
- **Organise an awareness-raising event or activity**
- **Contact your National Alliance**
- **Advertise www.rarediseaseday.org and the RDD logo as widely as possible**
- **Encourage your members and partners to join in the campaign**
- **Sign up to the RDD Facebook Group**
- **Upload a photo or a video on Youtube/rarediseasday Channel**
- **Come to the European event in Brussels**
- **Send us stories, surveys, studies and other examples of health inequalities for rare disease patients**

Eurordis support to European Federations, Anja Helm
(Terms & conditions and application form attached)

The programme is a pilot starting in 2010 with limited resources: 15 000 Euros. The programme is co-funded by the European Commission / Executive Agency Health & Consumers in the framework of the EURORDIS Operating Grant (60%) and by EURORDIS own resources (40%). Based, on evaluation as well as on available resources, it is EURORDIS intent to progressively grow this programme under the auspices of the Council of European Federations.

The aim of the programme is to promote patient empowerment through capacity building and European networking between patient organisation representatives in disease specific areas as well as to enhance the dialogue between these patient organisations representatives and clinicians or academic European leaders in their area.
The programme is designed to provide seed money and add-on money to enhance the own efforts of the European Federations, and not to provide significant or recurrent financial support.
CEF members as well as EURORDIS member organisations part of a fledgling network can apply for support, on a "first come first served" basis and within a maximum amount of 3000 €/year.

Collaboration with European Reference Networks of Centers of Expertise, *Anna Kole*

European Reference Networks (ERN) are the "networking of knowledge and expertise" through either physical or virtual transfer of expertise between Centres of Expertise (CoE) for a rare disease or groups of rare diseases
EURORDIS has played an active part in favor of the establishment of Coe and ERNs and the involvement of patient and patient organization in their activities, for example with the "Declaration of Common Principles on Centres of Expertise and European Reference Networks for Rare Diseases" or through the participation in the Commission Communication and Council Recommendation on Rare Diseases.

The Council Recommendations invite Member States to:

- Identify appropriate CoE throughout their national territory by the end of 2013
- Foster the participation of CoE in European Reference Networks
- Organise healthcare pathways for patients suffering from rare diseases
- Support the use of information and communication technologies such as telemedicine
- Include, in their plans or strategies, the necessary conditions for the diffusion and mobility of expertise and knowledge
- Encourage CoE to be based on a multidisciplinary approach to care

This and other corner stone documents are summarized in one of EURORDIS' advocacy tools, a fact sheet, supporting the implementation of key recommendations in the European Commission's Communication on Rare Diseases and Council Recommendation on Rare Diseases, and facilitating their transposition into national plans and strategies on rare diseases.

EURORDIS strongly advocates for the collaboration between CoE and patient representatives at the national level and the participation of patient groups in European Reference Networks at the European level. EURORDIS is proposing more specific guidelines for the relationship between PO's and Coe and PO's and ERN based on a common set of goals. Both these documents will be distributed electronically and we look forward to your feedback on using them in the "real world".

EURORDIS is often asked to participate in ERN and other European projects. However, EURORDIS cannot participate in each of them. Instead EURORDIS would like to encourage disease specific groups such as European Federations to participate instead. When it is helpful for patient groups to have EURORDIS' support in such projects EURORDIS asks that certain criteria be respected:such as receiving the complete project application (or description) at last 2 week before the deadline. Although EURORDIS cannot play an active

role but rather invites project leaders to involve the disease specific patient organizations or networks to join as work package leaders, EURORDIS staff members are available to play an advisory role.

Clinical Trials Charter, *Francois Faurisson, Rob Camp*

Patient organisations support research and want to be involved in the research paradigm. The best time for a patient organisation to become involved in a clinical trial is at the very beginning, with the protocol design, before changes become too complicated or institute. Ideally the sponsor and the patient representatives should meet while the trial is being designed, as there is a shared benefit for both parties.
However, often, collaboration does not work. Often, sponsors may not include patients because they think that patients would put at risk the confidentiality of the protocol (intellectual property rights, etc), or because they believe that the protocol is too complicated to understand for patients.
But if there is a relation of trust between the sponsor and the patient group, there should not be any fears regarding the confidentiality of the protocol. And most of the documents can be written or explained in such a way that patients can understand them.

With its Charter on Clinical Trials on Rare Diseases, EURORDIS wants to reach the following objectives:

- Provide clear grounds for the establishment of an in-depth collaboration between Patient Organisations (POs) and Sponsors
- Give patient organisations the opportunity to access the clinical development protocols for their diseases and to contribute to them
- A sponsor-patients interaction based on transparency via an ad-hoc Agreement of Understanding for each clinical trial

The Agreement of Understanding (AoU) is a non-confidential and non-legally-binding document aimed to guarantee transparency in the collaboration between patients and sponsors.

Why is it important that Patient Organisations work with the Sponsor?

- To make sure that priorities of patients are respected and patients protected
- To ensure that the trial includes as many patients as possible. Sponsors might exclude groups of patients to make it easier to recruit. For example, regarding gender: fewer women are usually involved in clinical trials and that could change by working with patient groups.

Eurordis can help networks of patients to sit down with a sponsor to talk about what type of collaboration both parties can agree upon for a specific trial.

So far, 4 sponsors have signed the Charter:

1. CSL Behring
2. Novartis Oncology
3. Swedish Orphan-Biovitrum
4. Takeda Pharmaceuticals Europe Ltd.

Rob Camp is currently working on the implementation of the Charter with a clinical trial for Tuberous Sclerosis (TS). The sponsor came to Eurordis after the trial was opened, which is not the ideal way of going forward but was a first chance to test the Charter.

The first problem encountered was that, from the patient side, as there is no European Federation for TS, it's hard to figure out who would sign the charter with the sponsor, although this may be a problem specific for big pharma only. Although now TS has now formed a non-legal coalition of approximately 10 groups who could sign it, the company is not sure that that will suffice for their legal department.

In the ideal situation patients should be involved from the beginning of the clinical development, rather than after a certain amount of patients had already been recruited.

In the TS situation, the patient organisations representatives educated the sponsor quite a bit about non-clinical aspects, for example that many children with TS suffer from a form of autism because of cancers in the brain, so there is a need for a lot of support for caregivers (parents, etc). The representatives reminded the Sponsor of many day-to-day quality of life issues that are part and parcel of TS and cannot be put to one side during the trial (number of clinic visits, subjective illness of the children and readiness for operations, etc).

The sponsor agreed to make sure that the medication is available to everyone until approval of the drug. They also agreed to facilitate the movement of patients to the nearest clinical trials site, even if that site is in another country. That issue brings up many other problems like language and the comfort level of dealing with a new doctor and medical team who are not your primary care team, etc.

Participants agree that the Charter is a good instrument, for patients and pharmaceutical companies or investigators when acting as sponsors. All parties need to be aware that good collaboration is needed and the trial will be better for everyone if the patients are involved early.

Preparation of next CEF works

All participants agree to hold the next CEF meeting in June or July 2011 in Paris, over a two day workshop. It's requested to have more time for exchange between federations.

The first day is the EF meeting, focusing on the Road Map, Rare Disease Day and exchange of information and best practices between federations.

The second day will be dedicated to a training session, possibly on the Clinical Trials.

In the meantime, EURORDIS will create a mailing list for European Federations to facilitate exchange of information.
