16-05-2019 ePAG face to face meeting

By Marjolein van Kessel

The Eurordis member meeting 2019 started with an ePAG face to face meeting. During the meeting participants explored a selection of good practices that ePAG patient advocates presented and discussed with their peers. As such, this meeting was an opportunity to learn from each other and build your network with patient advocates working in other European Reference Networks.

The presentation on patients journeys (last session) and the information given on registries (Orphanet and HPO) were very interesting.

Johan de Graaf, Patient involvement in the ENDO ERN Registry project (EuRReCa)

EuRReCa is a very powerful tool to maximize the opportunity for all patients, health care professionals and researchers to participate and use high quality, patient-centered registries for rare endocrine conditions covered within Endo-ERN. EuRRECa is closely linked to Endo-ERN but is open to all professionals providing endocrine care and who would like to participate.

When developing they organized a 'Patient, Parent and ethics working group'. Patient involvement was very important. Patients were recruited by clinicians. They marked checkpoints in four different registries. In the future the 'Patient, Parent and ethics working group' will have to give approval to register.

Data can be collected by patients or clinicians. Ideally it is a mixture. A lot of people are not aware of registrations. To raise awareness from patients and doctors people were asked to work for the registry. Registration wasn't only technical but also about quality of life.

Patients need to be involved at all stages esp. regarding governance. The core of a registry is a limited dataset. Data to be used should be coming from both patients as well as clinicians. It should be very clear wat the added value of a registry is.
Beware of the General Data Protection Regulation (GDPR) of registries.

Interfacing with registries to retrieve data for instance is not standardized (yet). Registries should be FAIR (Findablity, Accessibility, Interoperability and Reusability). The ownership of registries could not be clear.

Consent forms were developed which can be used by all countries in all hospitals.

To connect different registries you need to make a core data set, similarities in the registration. Not every registry was useful.

How many items do you need to register:
CPMS has over 800 items
ERN eye 16 items – 4 items were added for more complete registry
To have patients involved you need to add more items

Russel Wheeler, Rare eye Disease (RED) Ontology using Orphanet and HPO database

ERN-EYE recognized at an early stage that in order to facilitate collaboration across geographic and professional boundaries there was a clear need for a common language for use not only in research

and registries but in presentation of cases for virtual consultations. A workshop was convened to revise two of the main existing ontologies to ensure that they were fit for purpose.

The initial thinking was that, as this is a highly technical exercise, the involvement of patients would be a distraction and that non-scientists would be unable to engage in such high level debates.

We're all impatient patients. To become engaged read, search, be curious and study. To participate speak up and be present.

How do you identify diseases? Hospitals use different coding. Orphanet has a coding based on genetics, Human Phenotype Ontology a coding based on diagnosis. You need consent on the coding and the rare disease name in order to translate to other languages and to implement it in Clinical Patient Management System (CPMS). In this study there was a consensus paper signed.

Conclusion: For registry there are three important points:

1. We patients have a voice what should be in a registry
2. Long term data also brings in short term data. Ask people to stay in for long term data but publish short term data to motivate to be involved in registration
3. Train and communicate to show the benefit of registration and take away the fear of GDPR

Ilaria Galetti Patient involvement in identifying unmet needs on clinical patient guidelines

For each disease covered by ReConnet, an ePAG patient advocate has been appointed in ReCONNET. The diseases are divided in 3 disease groups with an ePAG patient advocate per group appointed as a ReCONNET Steering Committee (SC) member.

The SC decided to publish a narrative review of existing Clinical Practice Guidelines (CPG) to perform a state of the art of the existing Clinical Practice Guidelines (CPGs) per disease group. As a result of a proposal made by the ePAGs in ReConnet SC, the clinicians agreed, as part of this exercise, to identify the unmet patients' needs in each disease. It was then decided to have clinicians and patients drafting the papers per disease; the ePAG patient advocates contribution focused on collecting and writing the patients' unmet needs of each disease. Different ePAG patient advocates were involved in this exercise and their contribution as co-authors of the papers was fully acknowledged.

Patients had a positive impact on the published papers. Their unmet needs have been acknowledged by the healthcare professionals and will eventually result in new projects.

Clinicians and patients develop guidelines together. Usually when developing guidelines there is a chairman, a group of medical experts and one patient. To be part of this group you need to have some education about the process and understand the methods (like Delphi study). Patients work for free. Ilaria asked national patient support groups for anamneses. Patient representative in guideline were mentioned as co-author.

Guidelines were translated to understandable language. When simplifying the text patients were asked which words used were still too difficult. To simplify pictures can be used as well. Next step was to translate them to different languages. The translation of the guidelines was done by the European society for free. There was no money available.

They made 15 universal questions which will help patients when looking for information. To answer the questions they used 4 guidelines. An example of a question could be: 'do we have enough nurses for the quality of life?'

In this ERN study all European countries were involved, some of them even don't have a plan for rare diseases. The translation

All of this wouldn't have happened without Eurordis!

Ilaria Galetti, Publication of state of the art on clinical practice guidelines per disease

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Own notes: when developing guidelines there is a problem patients work for free and therefore taken less seriously. However patients were mentioned as co-authors.

Guidelines were translated to understandable language (simplified). As soon as a patient version was available she would ask patients which word were still too difficult. Pictures were added to the patient version.

Next was to translate the patient version of the guideline to different languages. She had all European countries involved, even those countries who do not have a plan for rare diseases yet. The language translations were done by the European community, however there was no money available.

Sue Routledge and Ammi Sundqvist, Developing Patient Journeys

The information presented in the table was summarized into a visual "Patient Journey" or "route map" to support user-friendly communication. At a face to face workshop with the ERN coordinating team, each ePAG patient advocate presented their Patient Journey, the life journey from first symptom, diagnosis through to care, treatment and follow up. This was presented to the wider ePAG group to support better understanding of each other's diagnosis. Between each presentation, the group were asked to highlight common needs that others had also experienced in their rare disease. The common needs for all the ERN ITHACA syndromes were captured through this workshop and summarized into one common Patient Journey. The next step was to identify recommendations for each of the common need identified and for the ERN to address both at a strategic and operational level how the Network will address these common needs. We are a small ePAG group who see each other at different conferences and we have tried to have small unofficial gatherings back-to-back to other meetings. One of the main concerns we had with this exercise was how to represent the journey of other diagnostic groups within ERN ITHACA. It was challenging to create a template that suited every disorder because some of us had struggled with getting a diagnosis, whereas others knew it from the beginning but had a problem of getting the right care. We brought up the concerns we had with Matt Bolz-Johnson and he created a template to describe a Patient Journey across the different diseases. To summarize our needs, we wrote down how we think our own journey in the healthcare system should be by filling in the template. Some of the ePAGs patient advocates already had a clear vision on how a multidisciplinary healthcare pathway should be, but they had never described it in writing.

Notes taken: they have developed patient journeys for patients (parents) to learn about the implications, because often the diagnosis of complications is often late. Spina bifida has about five different guidelines, which were all used together for the patient journey. It was useful to identify the needs to show to clinicians. It empowered patients with information. Sometimes the impact of information can be scary, so awareness is necessary – however a google search can give information that can be much worse!