European Patient Advocacy Groups (ePAGs)

EURORDIS Membership Meeting, Edinburgh
26 May 2016
European Patient Advocacy Groups (ePAGs)

Part 1:
• What is the role of ePAGs? terms of reference, common Principles, common objectives
• How do patient representatives participate?
• Practical examples of patient representative participation in ERNs

Part 2:
• ePAG Vision - Open Panel Discussion with ePAG representatives
What is the Role of ePAGs? terms of reference, common principles, common objectives
Background

European Reference Networks (ERNs) created on founding principles of patient-centred care, patient advocate empowerment, patient engagement

ePAGs:

- **Forum** for dialogue, unity & solidarity to optimise involvement of patients
- **Representativeness** to engage into application and governance of rare diseases ERNs
- **Open** to members & non-member patient groups in EU
- **Aligned** with RD ERN scope
- **Composed** of elected ePAG representatives & member organisations
- **Democratically** established where there is an ERN application & progressively expanded (a work in progress; perfectible next years)
  - Terms of Reference
  - Call for Expression of Interest (16 March) & Elections’ of ePAG representatives (21 April – 4 May)
- **Processed launched in May 2015, Announcement in May 2016**
Background

ePAG Patient Representatives:

- Can become formal members of ERN boards (role & function agreed on with respective clinical leads)

- Ensure two-way, vertical flow of information between ERN and ePAG, reflecting patients viewpoint & supporting ERNs including governance & clinical & operational delivery,

EURORDIS supports ePAGs with:

- work processes in ERNs
- information exchange & capacity building across ePAGs and other relevant activities or
- RD-ERN external communication, common advocacy actions & operational links to Joint Action RD-Action and CEG-RD or CEG-CC
Common objectives of ePAGs

- Ensure care is **patient-centred & respects patients’ rights & choice**

- Ensure **transparency** in quality of care, safety standards, clinical outcomes & treatment options

- Ensure **ethical issues** for patients are addressed, balancing patient & clinical needs

- Contribute to the development of **patient information, policy, good practice, care pathways & guidelines**

- Advise on **planning, monitoring & evaluation of ERN initiatives**
Where have we come from?

A step-wise approach:

- **May 2012**
  - EURORDIS position paper on ERN

- **January 2013**
  - EUCERD Recommendations on ERN

- **2014**
  - EU Commission: delegated decision & implementing decision listing evaluation criteria for ERNs

- **May 2015**
  - Session dedicated to deliberating best ERN grouping model to ensure a home for RD patients at EMM

- **October 2014**
  - Council of European Federations & National Alliances, EURORDIS Board of Directors & EURORDIS Public Affairs Committee

- **April/May 2016**
  - ePAG Patient Representative Elections

- **2015 ongoing**
  - Development of RD groupings, from diagnosis through to treatment

Implemented by patient advocates:
## Where are we now?

### ePAG Patient Representative Elections

<table>
<thead>
<tr>
<th>Disease Grouping</th>
<th>ePAG</th>
<th>Total Responses</th>
<th>Elected patient reps</th>
<th>Elected reps (Non-members)</th>
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<td><strong>848</strong></td>
<td><strong>100</strong></td>
<td><strong>78</strong></td>
<td><strong>29%</strong></td>
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Where are we now?

- Closing **gaps** by involving more patient representatives & members to achieve fair representation of patients & diseases

**Overall feedback from ERNs positive e.g. Malformation ERN:**

- Patients involvement core activity of all ERN activities & governance structure i.e. involved in design & activities of all core workpackages (activities)

- Patients involved in ERN board & planning activities (setting-up governance & advising on evaluation of outcomes)

- Goal: fair representation of diseases & equitable representation across patient groups (ePAG currently spina bifida/hydrocephalus heavy)

**Balance representation e.g. endocrine ERN:**

- ePAG currently consists of 2 Dutch representatives (pituitary & adrenal disorders)

- Need to achieve more balanced & equitable representation across patient groups, countries & diseases

**Gap in representation cardiac ERN & gynaecological ERN:**

- No patient representatives at present

- Need to engage with membership & European patient communities to involve patient representatives
Where are we now?

European Commission:
- *delegated decision & implementing decision
- patient empowerment & engagement *evaluation criteria
- EUCERD Addendum recommends patients representatives to be part of RD ERN governance and decision making structures

Need to incorporate the patient perspective in design, programming & evaluation of ERN initiatives

No legal obligation to place patient representatives in governance structure
What are the next steps?

• Building relationships with clinical leads & co-facilitating placement of ePAG patient representatives in ERN governance structure

• Creating links & sharing experiences between ePAGs & RD-ERNs

• Developing ePAG engagement roadmap & working procedures
  Identifying main themes & frameworks of ERN applications and areas for ePAG support

• Develop ePAG capacity building strategy & toolbox to build ePAG resources, structures, processes & evidence base

• Create a virtual forum for exchange & information sharing (RareConnect)
How do patient representatives participate?
Instruments of engagement

- **Teleconferences**
  - Work procedures

- **Workshops (EMM)**
  - Relationship & capacity building

- **RareConnect**
  - Discussions, resources, etc.

- **Webinars** (two-weekly)
  - Capacity-building

- **Online surveys**
  - Member consultations

- Behavior (13)
- Activity (20)
- Involvement (36)

- Genomics and the future of research into rare diseases
- Barriers and enablers of cross border

- Health promotion of work
- Recommendation education
Enhancing ePAG networks via RareConnect

ePAG online communities will:

• Connect ePAG representatives with each other in private groups

• Connect patient representatives & ePAG patient organisations with each other in closed groups (additional users can request permission to join)
ePAG communication via RareConnect

Members will have access to:

• group's description, updates & useful resources

• a space to ask questions & discuss

• a space to access all members, their profiles and their location within a global map
Practical examples of patient engagement in ERNs

Different approaches to engage patients as formal members in ERNs:

• ePAG representatives can be formal members of:
  - **ERN Boards** & management committees (scenario 1):
    - Scientific Advisory Board, Steering Committee and Management Committee
  - **Clinical Committees** (single disease or sub-group of diseases) supporting ERN Boards (scenario 2)
  - **Parent/Patient Advocacy Committee** (scenario 3)
Practical example 1: Rare Bone ePAG

ERN Architecture & Governance

Coordinator
Luca Sangiorgi - IOR

Steering Committee (max 12)
exerts from skeletal dysplasia network
exerts from metabolic disorders network
+ patients representatives
+ professional societies representatives

Management Committee
One representative of each HCP
+ patients representative

Scientific Advisory Board

Skeletal Dysplasia & Metabolic Bone Diseases

HCP: Healthcare Providers
Practical example 1: Rare Bone ePAG

ePAG: 32 member organisations

Engagement with wider European communities

Ines Alves

Joan Moitry

Rebecca Skarberg

writing group ‘excellence’ & ‘impact’

ERN Management Committee

ERN Steering Committee

ERN Scientific Advisory Board
Practical example 2: Rare Lung ePAG

**ERN LUNG**
Steering committee

**Advisory Board**
ELF, ERS, policy-makers, foundations, trade organisations, ethics specialists etc.

**Functional Committees**
To include independent patient representatives – identified via the advisory board application process or another application process. One or 2 patients on each committee or 5 patients to cover all committees.

**Patient Assembly**

- **ILD Core Network:** EURO-IPFF + LAM Europe 1 other patients rep. To include sarcoidosis, pulmonary alveolar proteinosis, connective tissue diseases
  - Fillipo Martone
  - Bernd Quadeer

- **PCD Core Network:** 2 patient rep.
  - Hilde de Keyser
  - Bernd Quadeer

- **CF Core Network:** CF Europe + 1 patient rep.
  - Luc Matthysse
  - Gergely Meszaros

- **PH Core Network:** PHA Europe + 1 patient rep.

- **NCFBE Core Network:** ELF NCFBE Patient Advisory Group 2 patient rep.

- **Alpha 1 Core Network:** Alpha Global 1 patients rep.

- **Meso Core Network:** 2 patient rep.
Practical example 2: Rare Lung ePAG

European Lung Foundation

Clinical lead of Rare Lung ERN

ePAG: 41 member organisations

Bernd Quadder
Gergely Meszaros
Luc Matthysen
Filippo Martone
Hilde de Keyser
Practical example 3: Paediatric Cancer ePAG

Paediatric Cancer ERN (PaedCan-ERN) Organizational Structure

- PaedCan-ERN Coordinator
- Board of the European Society for Paediatric Oncology (SIOPE)
- Hubs of Coordination (HoCs)
  - International Study coordinating site/Trial Umbrella organization site of president
  - NAPHOS nominated HoCs
  - Tumour entity specific sub-network HoCs
  - HoCs in countries with low health economy rate (LHEAR countries)

Advisory Board (Parent/Patients Advocacy Committee, Ethics Advisory Committee, European Clinical Research Council, Innovative Therapies for Children with Cancer, ENCCA TVA (Teenager and Young Adults with Cancer European Group Leaders))

The objective of this management structure within the consortium is to ensure the opportunity to take rapid and efficient decisions (i.e. avoid status-quo or a blocking situation) whenever necessary and simultaneously to allow each individual organization representation - in order to anticipate and avoid the occurrence of disagreements/conflicts.
Practical example 3: Paediatric Cancer ePAG

PaedCan-ERN HoC
Paediatric Oncology European Reference Network Hub of Coordination (HoC)

NaPO
National Parent Organisation

NatPaedOncC
National Paediatric Oncology Centre

LoPO
Local Parent Organisation
Practical example 3: Paediatric Cancer ePAG

Childhood Cancer International (CCI)/ePAG (9 member organisations)

PeadCan-ERN CCI/ePAG representatives:
- Anita Kienesberger/Stephanie Schremmer
- Luisa Basset
- Lejla Kameric

PaedCan-ERN Advisory Board

Member State Par. Org. National Contact Point capable to interact with PaedCan-ERN

Local Par. Org.
How has this been in reality?

1. Daniel Renault, Rare Renal ePAG
2. Anne-Sophie Lapointe, Rare Metabolic ePAG
3. Richard West, Rare Immunological & Auto-Inflammatory ePAG
ePAG rough guide or rough ride ... 1
Rare Renal Disease: one of the 21 ERN themes
Rare = Genetic in Kidney diseases
FEDERG = European Federation for Rare Renal Diseases

FEDERATION OF EUROPEAN ASSOCIATIONS OF PATIENTS AFFECTED BY GENETIC and/or RARE RENAL DISEASES.

Daniel Renault, Chair
Rare Renal ePAG

PROJECT: ERKNET

European Rare Kidney Disease Reference Network

Coordinator Pr. Franz Schaeffer Heidelberg Univ.
38 Health Care Providers – 14 Countries

(Belgium, Czech Republic, Finland, France, Germany, Hungary, Italy, Lithuania, Netherlands, Norway, Poland, Spain, Sweden, U.K)

Organized in 10 Sub-themes (type of pathologies, pediatric)

FEDERG actively involved since mid 2015
Our Hopes & Concerns

Our Vision: in Rare Renal Disease

Everyone in Europe benefits from the same good healthcare pathways (diagnosis, prognosis, treatment)
Rare Renal ePAG

Patients representatives roles

1.3.5. Governance: incorporate the patient’s voice (….possibly a seat in the board)

2.1.1. Clear pathways based on patients needs

2.1.5 Protocol /Transition childhood-adolescence-adult

Section 2 2.2.1 to 2.2.5 Empowering patients for safety and quality of care study

4.3.2. Ethical aspects of ERN

Section 5.1 Research in particular in 5.1.2. on collaborative research and 5.1.4 on information networks, shared registries, and databases
Expected key benefits? In three directions:
Solidarity, Complexity, Research

Patients with complex cases: better diagnosis, prognosis and treatment

All patients: improved research diagnosis, prognosis and treatment

All patients in Europe access to high quality Healthcare Pathways
Rare Renal ePAG

Other key patient perspectives in terms of foreseeable benefits

Key patient perspective

- **Strengthening** the network of patients throughout Europe

- **Stronger patients organisations** (National and Europe)

- **Better organised** through pathology chapters and committed to develop registries and biobanks, and better engaged into studies and trials.
Federg Mobilization high (low outside)

Patients = E-PAG

E-PAG elected representatives: D. Renault and Claudia Sproedt (Vice chair)

In each subtheme:
E-Pag elected Rep + Federg Patient (12 volunteers)
Role of an European organization
Assemble national associations (single entity)
Information, Management, Monitoring

The Gaps/challenges:
Unify associations & democratic representation
Building capacity/not loosing ground
Equitable coverage for less represented (countries, Ultra-Rare D)
Roles of National associations
Information to and from patients
Assessing pathways per condition
Monitoring of ERN achievements

The Gaps/challenges for FEDERG:
Associations in countries not covered

We are looking for patient’s groups in 20 countries
Thank you.
ePAG rough guide or rough ride ... 2
Rare Hereditary Metabolic ePAG

Implementation of an ERN: Ethical Issues
Anne-Sophie Lapointe
*Vaincre les Maladies Lysosomales*
Implementation of an ERN: Ethical Issues
Weaknesses

- Depend on a certain amount of maturity of the network
  History of previous collaborative networks, links with European learned societies etc.
- E-health tools
  Rarely available
- Access to treatment and diagnosis
  Inequity between countries
Strengths

✓ Multidisciplinary work
  Mutualisation, global vision, national & European level,
  + Sharing & dissemination of knowledge
    Clinical and Best practices guidelines
    + Shared registries service
      Natural history of the diseases
      + European Patient advocacy group
      + Patient Empowerment / Patient Involvement

= Creating quality outcomes and clinical excellence
  Improving outcomes for patients
Threats

- Complexity of rare diseases
  Lack of information, training, heterogeneity, undiagnosed diseases…

- Interoperability between IT system and sustainability:
  Needed to build and finance it
- Few treatments available
- Lack of relevant patient data in the scope of QoL, social consequences, burden of disease

Lack of indicators to prove the effectiveness of a treatment and not only the efficacy…..

- Transparency
  POs, Investigators, Sponsors
Opportunities

- Training in the scope of RD & Increasing awareness
  - Formation / Information
  - Patient centred approach
  - Relevant Data
- New therapies & New design for clinical trials for small population
  - Medicines Adaptive Pathways to Patients (MAPPs)
    - E-health tools
- ERN Platform, multidisciplinary work between ERNs
  - Research
  - H2020
Implementation of an ERN: Ethical Issues
ePAG rough guide or rough ride ... 3
Rare Immunological & Auto-Inflammatory ePAG

- European Reference Networks.
- Rare Immunological and Auto Inflammatory ERN (RITA)
- Immune Disease, Vasculitis Diseases and Auto Inflammatory Diseases – three pillars under one ERN.
1. Time frame too short to prepare when there were so many unknowns.

2. Forming groups was very difficult – especially if the symptoms of your disease could fit into other ERN’s and you are treated by various Clinicians.

3. No financial support, so busy Clinicians had to undertake their applications in addition to their already heavy workload. Massive burden of understanding the paperwork and completing it.

4. Patient groups appeared to have limited input to individual ERN’s – each ERN decided on their own constitution/guidelines/rules/protocol.
5. Member states were slow in designating Health Care Provider.

6. Restricted numbers to be admitted to the ERN’s in first call.

7. Patient elections to ePAG (Eurordis Patient Advisory Group) was rushed

8. RITA ePAG Patient Representatives, Diana Marinello (Associazione Italiana Sindrome e Malattia di Behcet, Italy), John Mills (Vasculitis UK), Peter Verhoeven (Vasculitis Stichting, Netherlands), Richard West, (Behcets International, UK) and Marianne Riviere (Association Française du Lupus et autres Maladies Auto-Immunes (AFL +), France).
Rare Immunological & Auto-Inflammatory ePAG

9. Good points – At least Patient Groups are involved – remains to be seen how and to what extent they have any real influence.

10. The ERN’s will mean better treatment and care for people with rare diseases and open up better prospects of research by the Clinicians and researchers working together.

11. It opens up the prospect of Registries for diseases.
Panel discussion
Thank you

Daniel - Sanfilippo syndrome