Challenges: 
the Patients’ perspective

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EURORDIS
Facing the Challenges?

Or

Facing the Challenges!

Emilia - Achondroplasia (Photo contest winner 2011)
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II. Involvement of Patients
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   – Regulatory Processes
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   – Access

III. Conclusions

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EURORDIS

- Founded 1997
- 571 member patient organisations
- 52 countries (25 EU countries)
- 32 national alliances
- Over 800 patient groups represented
- Over 4,000 rare diseases represented
- 28 staff members (24.5 FTE)
- ≈ 100 volunteers
Mission statement

- To build a strong pan-European community of patient organisations and people living with rare diseases
- To be their voice at the European level
- and – directly or indirectly – to fight against the impact of rare diseases on their lives.
- EURORDIS is the voice of 30 million European patients
How we achieve these objectives – key fields of action

Advocacy

Information & Networking

Health Policy & Health Care Services

Research, Drugs & Therapies
Where are patients involved in research?

- Pre-clinical research
- Clinical research
- Regulatory processes
- Post-marketing and access
Patients’ involvement in basic research
EURORDIS’ Survey on Patient Organisations and Research

In collaboration with the group of the “Centre de sociologie de l'innovation” (Ecole des Mines, Paris, France)

1. To measure POs’ interest for research

2. To evaluate POs’ support to research: in what ways and to what extent.

3. To learn about POs’ experience of collaboration with researchers

4. To collect POs’ opinion on Priorities and Obstacles for Rare Disease Research
Diversity of medical areas represented

772 rare disease patient organisations received an invitation to fill out the online questionnaire, which was available in 6 languages (EN, FR, DE, IT, ES, HU)

Diseases represented include: Multisystem, Neurology, Dermatology, Musculoskeletal, Ophthalmology, Metabolic, Neuromuscular, Oncology, Cardiovascular, and Haematology,

Received 309 valid responses from members and non-members (40% response rate); representing 110 rare diseases and 1.3 million patients in 29 European countries.

http://www.eurordis.org/content/survey-patient-groups-research
<table>
<thead>
<tr>
<th>Percentage</th>
<th>Type of Financial Support</th>
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<tbody>
<tr>
<td>77%</td>
<td>Initiating and financing a specific research project</td>
</tr>
<tr>
<td>75%</td>
<td>Co-financing the operating budget of a specific research project</td>
</tr>
<tr>
<td>54%</td>
<td>Financing the acquisition of a specific research equipment (centrifuge, computer, etc)</td>
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<tr>
<td>47%</td>
<td>Financing a fellowship for a young researcher</td>
</tr>
<tr>
<td>39%</td>
<td>Co-financing meetings of researchers clinicians</td>
</tr>
<tr>
<td>30%</td>
<td>Co-financing training of researchers / clinicians</td>
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What kind of research do patients fund?

- 81% Basic research
- 57% Therapeutics
- 56% Diagnosis
- 54% Epidemiology / Natural history of the disease
- 46% Human and Social Science
- 24% Assistance technologies / Daily life
- 19% Research infrastructures

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# Non-financial support: from clinical trials to institutions

<table>
<thead>
<tr>
<th>Percentage</th>
<th>Description</th>
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<tbody>
<tr>
<td>76%</td>
<td>Actions aiming at creating links between patients, researchers and physicians</td>
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<tr>
<td>57%</td>
<td>Helping to identify patients to participate in clinical trials</td>
</tr>
<tr>
<td>49%</td>
<td>Providing information and counseling for potential participants in clinical trials</td>
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<tr>
<td>48%</td>
<td>Defining research projects by highlighting patients' needs and expectations</td>
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<tr>
<td>45%</td>
<td>Collaboration in clinical trials design</td>
</tr>
<tr>
<td>30%</td>
<td>Participation in scientific committees within institutions</td>
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<tr>
<td>28%</td>
<td>Launching campaigns for the collection of biological samples from patients</td>
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POs’ non-financial support to research: a less visible but essential support

- A majority of POs support research mainly through actions aiming at creating links between patients, researchers and physicians.

- An important non-financial contribution is provided to clinical development through different activities.

- Only a smaller number of POs is involved in campaigns for the collection of biological samples or participate in scientific committees in charge of defining research orientations.
Summary

• POs have a high commitment for research and are keen observers of all its areas (Basic, Therapeutics, Social and Human Sciences, ...)

• POs have a strong willingness for collaboration with researchers
  – POs’ efforts for linking Researchers/Clinicians/POs
  – Relations with researchers are quite good and seem to improve over the years.

• POs play an important role as catalysts of research

• POs provide two types of support to research
  ▪ Financial and
  ▪ Non-Financial: natural go-betweens for scientists from various fields from the more basic research to therapeutic applications, crucial support in clinical trials

• But….POs have limited budgets
  ▪ Triggering role and momentarily “filling the gaps” by supporting the type of research that appears less attractive to the public or private sector
Patients’ involvement in clinical research
Drug development process

Drug Discovery → Molecule Identification → Preclinical tests → Clinical Development

- Phase I
- Phase II
- Phase III

Phase IV
More than subjects in clinical trials, patients are actively involved in:

- Funding/initiating clinical trials
- Contributing to clinical trial protocols
  - Direct with sponsor
  - Via Medicines Agencies (national and centralised)
- European Clinical Research Infrastructure Network (ECRIN)
- EU Clinical Trials Register – design and ongoing consultation on results (equiv. to clinicaltrials.gov)
- Revision of Clinical Trials Directive
Patients’ involvement in regulatory processes
The European Medicines Agency (EMA) is a decentralised body of the European Union with headquarters in London.

Its main responsibility is the protection and promotion of public and animal health, through the evaluation and supervision of medicines for human and veterinary use.

Creation of EMA:

- to manage the centralised procedure
- to formulate scientific opinions

Sent to the European Commission

Commission Decision

(Pan European Marketing Authorisation)
Specific legislation – involving patients

- EU Regulation on Orphan Medicinal Products (Regulation (EC) No 141/2000)

- EU Regulation on Medicinal Products for Paediatric Use (Regulation (EC) No 1901/2006)

- EU Regulation on Advanced Therapy Medicinal Products in (Regulation (EC) No 1394/2007)

- EU Pharmacovigilance legislation (Regulation (EU) No 1235/2010)
EMA Scientific Committees where patients’ representatives are full members

Committee for Orphan Medicinal Products (COMP)
Paediatric Committee (PDCO)
Committee for Advanced Therapies (CAT)
Committee for Medicinal Products for Veterinary Use (CVMP)
Committee for Herbal Medicinal Products (HMCP)
Pharmacovigilance Risk Assessment Committee (PRAC)

Patients in Committees
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Patients involvement in the overall drug development process?
Patients in Orphan Drug regulatory process in EU

Sponsor
Submission for designation

Designation

Protocol Assistance

Submission for marketing authorisation

Sponsor
Marketing Authorisation

Information to patients and the public

European Medicines Agency (EMA)

Public Summary of Opinion (PSO) on Orphan Designation

Summary of European Public Assessment Report (EPAR)

Package Leaflet (PL) Summary of Product Characteristics (SmPC)

Drug Discovery
Molecule Identification
Preclinical tests

Clinical Development
Phase I
Phase II
Phase III
Phase IV

Industry (Sponsor) - EMA interactions during development

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Patients’ involvement in post-marketing and access
Patients’ involvement post-marketing

**Benefit/Risk assessment** – EMA Patients’ and Consumers’ working party (PCWP)

**Pharmacovigilance** – Pharmacovigilance Risk Assessment Committee (PRAC)

Direct reporting of **adverse events**

**Registries** – various projects including EPIRARE

**Health Technology Assessment** – various mechanisms including EUnetHTA Stakeholders’ Forum

**Access to medicinal products** – various initiatives
Despite the demonstrated added-value of patients’ involvement in all activities concerned with development of medicinal products and access...there is still a lack of acceptance in some cases.
Patients ARE already involved in many processes related to orphan medicinal products, which needs to be further encouraged:

- Research (pre-clinical) funding
- Design and funding of clinical trials
- Patient recruitment
- Regulatory processes in scientific committees (EU) and protocol assistance
- Contributions to HTA and discussion on value of a medicinal product
Conclusions

In this climate of **economic difficulties**, access to medicinal products becomes challenging and rare disease patients with high cost orphan medicinal products become the first targets.

Some answers to ensuring the continued success of development of orphan medicinal products:

- More collaboration between regulatory agencies
- Earlier dialogue with **all** stakeholders
- Continuous data collection
- Training of patients
Conclusion

Patients are…..

Facing the Challenges!
THANK YOU
Back-up slides
How to train patients’ representatives to participate in these activities?
For patient advocates in clinical trials and drug development

**FORMAT:** 4-day workshop in Barcelona, Spain

**FOCUS:** the role that patient representatives play during the regulatory process

To date **153** patients’ representatives representing more than **65** different rare diseases, **31** countries

**AIMS:**

- Educate, train and provide an environment for information exchange
- Bring together patient advocates for the first time and
- Enable patient representatives to interact with regulators, academics and industry partners
- Further the understanding of patient representatives of the process of drug development and clinical trials
Organisation of four-day programme

• Pre-Summer School preparation

• Four-day programme
  ▪ General organisation
    – Days 1 and 2 – Clinical trials and Drug development
    – Days 3 and 4 – EMA overview and committees
  ▪ Specific organisation
    – Mix of small group sessions and large group formal presentations
    – Problem-based learning model
    – Small group sessions of 10 individuals (maximum) to encourage interactions and exchanges
    – Case-based tutorials

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Other training initiatives – patients’ organisations

European Federation of Neurological Associations (EFNA) – HTA School

European Patients Forum (EPF)
http://www.eu-patient.eu/Initiatives-Policy/

European AIDS Treatment Group (EATG)
http://www.eatg.org/eatg/Capacity-Building/Trainings

And of course… EUPATI….
Launched in Feb ’12, runs for 5 years, 29 consortium members, PPP of EU Commission and EFPIA

will develop and disseminate objective, credible, correct and up-to-date public knowledge about medicines R&D

will build competencies & expert capacity among patients & public

will facilitate patient involvement in R&D to support academia, authorities, industry and ethics committees
1. Medicines development process from research to approval

2. Personalised and predictive medicine

3. Drug safety and risk/benefit assessment of medicines

4. Pharmaco-economics and health technology assessment

5. Design and objectives of clinical trials (& roles of stakeholders)

6. Patients roles & responsibilities in medicines development

...and NOT: develop indication- or therapy-specific information!
Reflecting European diversity: 7 languages

- 7 most frequently spoken languages:
  - English, French, German, Spanish, Polish, Russian, Italian

- Serving 12 European countries:
  - UK, Ireland, Malta, France, Luxemburg, the francophone Belgium, Germany, Austria, Switzerland, Spain, Italy and Poland, plus Russian-speaking population in CEE
Audiences: advocacy leaders and the public at large

EUPATI Certificate Training Programme

Patient Ambassadors in committees, HTA agencies, industry, regulatory bodies, academia etc
Patient Journalists raising awareness
Patient Trainers for patient communities and networks.

EUPATI Educational Toolbox

Educational tools for patient advocates (print, slide shows, eLearning, webinars, videos) for patient advocates

EUPATI Internet Library

Patients & lay public at large, e.g. on specific aspects of the development process of medicines for patients with low (health) literacy.

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• Leading pan-EU patient umbrella groups
• Strong impetus from key academic partners and research organisations
• Industry expertise in medicines R&D

• Advisory bodies & codes committed to ensure independence and good governance
  ▪ EMA, Swissmedic, MHRA, BfArM
  ▪ Key experts in bioethics, genetics, HTA, economics, evidence based med, patient advocacy

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More Information on
www.patientsacademy.eu

Web:
www.patientsacademy.eu

Twitter:
@eupatients

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Measurements of benefits of training

Results in numbers: Documents reviewed by trained volunteers or staff members for European Medicines Agency include:

- Public Summaries of Opinion (of Orphan Designation)
- European Public Assessment Report summaries
- Package Leaflets
- Protocol Assistance dossiers
- Scientific Advisory Groups

Scientific committees
- COMP
- CAT
- PDCO

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