



Patient Surveys: an advocacy tool

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Background and Rationale

EURORDIS MISSION

- 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 to fight against the impact of rare diseases on their lives.

**POSSIBLE DUE TO PARTICIPATION OF AND CONSULATION
WITH PATIENTS AND THE ORGANISATIONS THAT
REPRESENT THEM**

EURORDIS Contribution to the Adoption of...



- EU Regulation on Orphan Medicinal Products in 1999
- EU Regulation on Paediatric Drugs in 2006
- EU Regulation on Advanced Therapy Medicinal Products in 2007
- EU Commission Communication on Rare Diseases in 2008
- EU Council Recommendation on European Action for Rare Diseases in 2009
- EU Directive on Patients' Right to Cross-Border Healthcare in 2011
- Promotion and maintenance of rare diseases as:
 - EU Public Health Policy priority
 - EU Research Framework Programme priority

Participation activities at EURORDIS

- Patients and family members

- **Surveys**

- Deliberative debates
 - Online communities
 - Photos, videos, testimonies

- Patient organisations

- **Surveys**

- Workshops
 - Working groups
 - Policy consultations



Key concepts (1)

- **Participation** (in social science) refers to different mechanisms for the public to express opinions - and ideally exert influence - regarding political, economic, management or other social decisions.

Citizens as Partners OECD HANDBOOK ON INFORMATION, CONSULTATION AND PUBLIC PARTICIPATION IN POLICY-MAKING

- **Public participation** is the deliberative process by which interested or affected citizens, civil society organisations, and government actors are involved in policy-making before a political decision is taken

European Institute for Public Participation, *Public participation in Europe: an international perspective*, 2009, p. 10, viewed 8 April 2011, http://www.participationinstitute.org/wp-content/uploads/2009/06/pp_in_e_report_03_06.pdf

- **Evidence-based public health** is an integration of science-based interventions with community preferences for improving population health

Jacobs JA, Jones E, Gabella BA, Spring B, Brownson RC. Tools for Implementing an Evidence-Based Approach in Public Health Practice. *Prev Chronic Dis* 2012;9:110324

Key concepts (2)

- **Evidence-based policy** is a discourse or set of methods which informs the policy process through a rational, rigorous and systematic approach informed by available evidence.

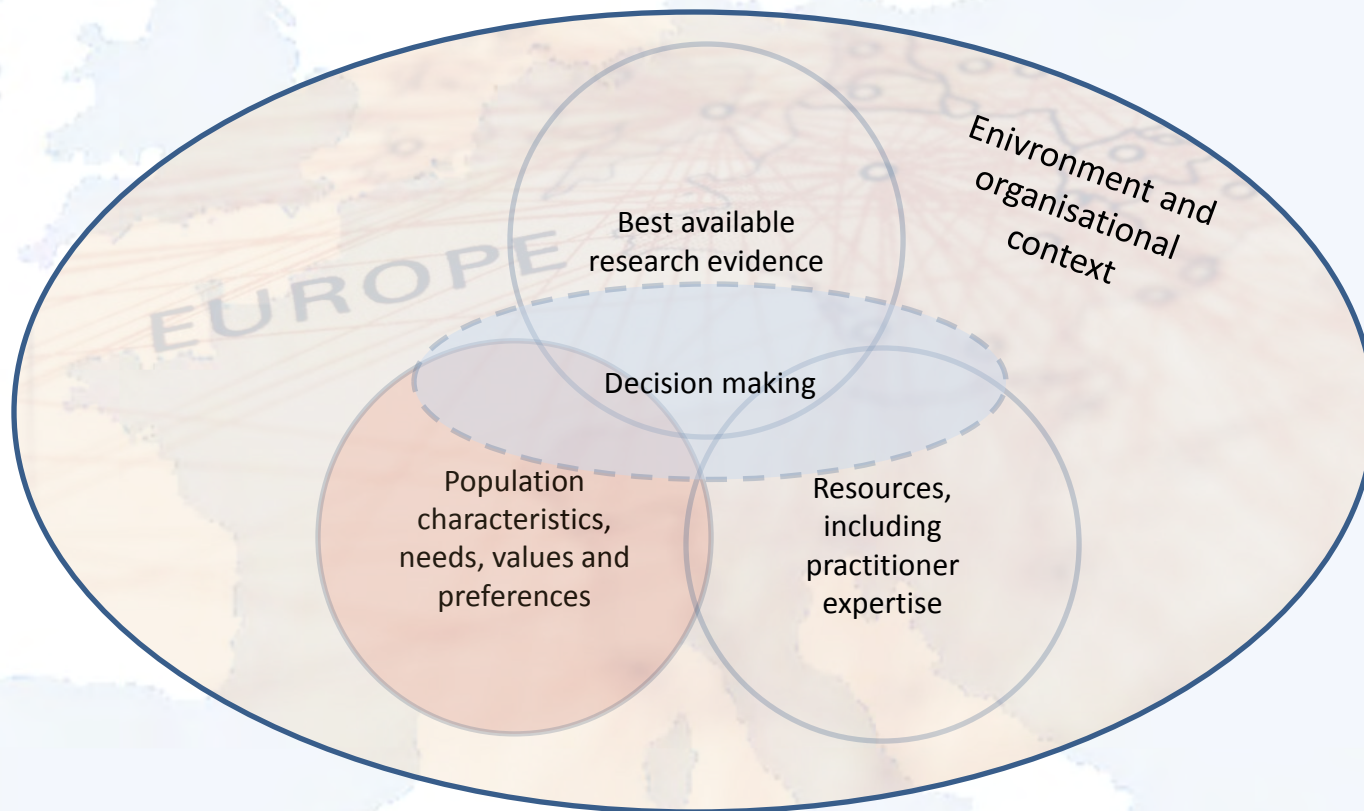
Evidence-Based Policymaking: What is it? How does it work? What relevance for developing countries? Sophie Sutcliffe and Julius Court, Overseas Development Institute, November 2005.

- **Stakeholder engagement** is the process by which an organisation involves people who may be affected by the decisions it makes or can influence the implementation of its decisions. They may support or oppose the decisions, be influential in the organization or within the community in which it operates, hold relevant official positions or be affected in the long term.

Lavallee DC, Wicks P, Alfonso Cristancho R, Mullins CD. Stakeholder engagement in patient-centered outcomes research: high-touch or high-tech? Expert Rev Pharmacoecon Outcomes Res. 2014 Mar 24.

All approaches lead to better policy and ultimately better outcomes

The Role of the Patient Perspective in Decision Making



Jacobs JA, et al. Tools for Implementing an Evidence-Based Approach in Public Health Practice. *Prev Chronic Dis* 2012;9:110324

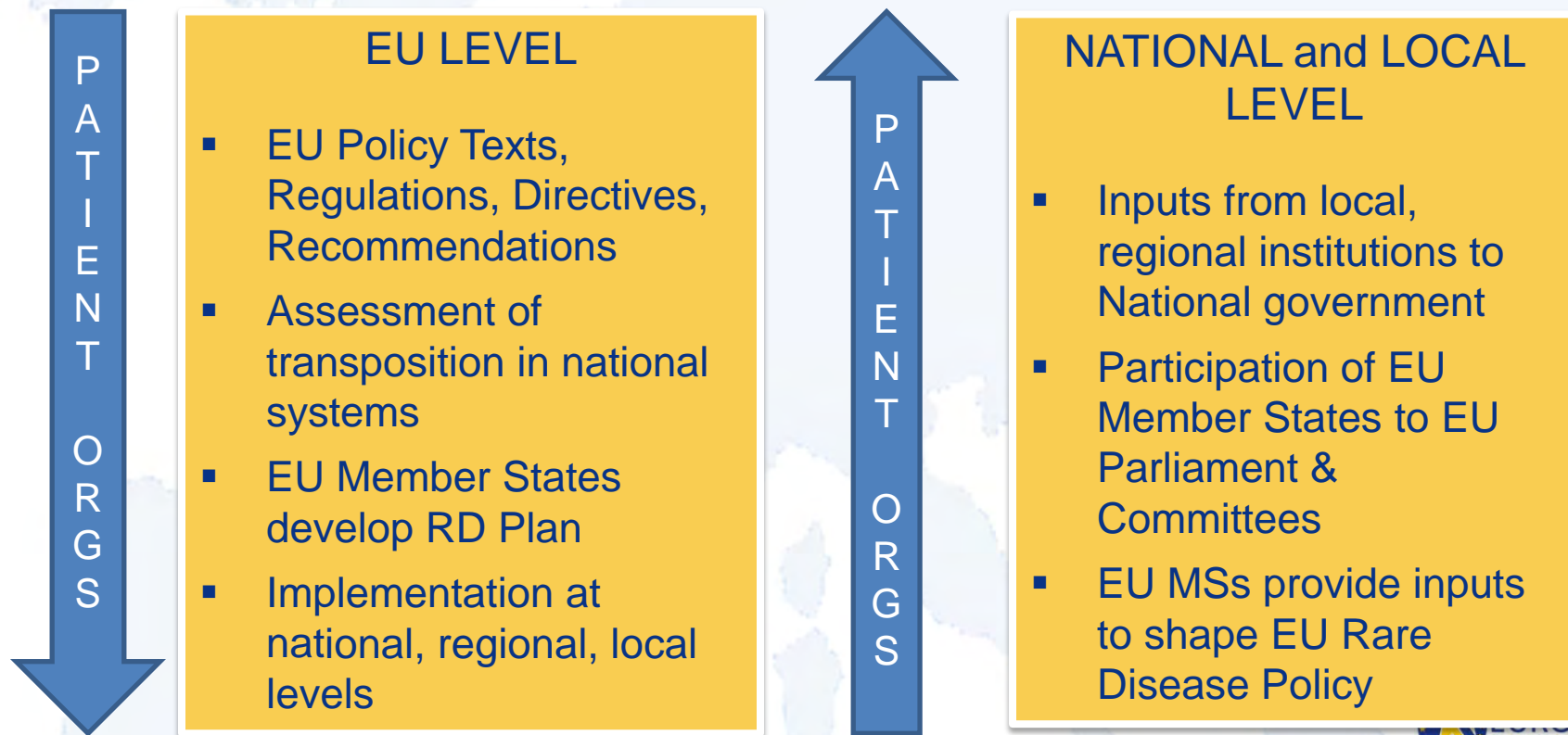
Rare Disease Patient Perspective

Out of necessity, rights, duty and solidarity, rare disease patients and their representatives have been and continue to be directly involved in decision making in health, research and social policy:

- To respond to an unmet need (Lack of attention and knowledge in the field)
- Because they are experts of their disease
- Because they have the most to gain
- Because they are the ultimate “end-users” of policy

Dynamics of Policy Development

The development of rare disease policies is the result of a dynamic communication between the European level, national and local levels



EURORDIS



Voice the RD patients'
needs at EU level

Organisations and Individuals

- Receive info, training
- provide inputs, respond to surveys, raise awareness

National Alliances

- Receive info, training
- Reach out to local patient groups
- Input into national plans for rare diseases
- Respond to surveys, raise awareness

European Federations/Networks of specific rare diseases

- Receive info, training
- Participate in EU research programmes
- Work on access to healthcare, respond to surveys, raise awareness

- **EURORDIS, National Alliances and RD patient organisations are active at all levels**

EURORDIS Policy at the European Level

- Representation at European Medicines Agency Committees:
 - COMP: Committee for Orphan Medicinal Products
 - PDCO: Paediatric Committee
 - CAT: Committee for Advanced Therapies
 - PCWP: Patients' and Consumers' Working Party
- Representation on the International Rare Disease Research Consortium (IRDiRC)
 - Executive Committee
 - Scientific Committees
 - Working Groups
- Representation at Commission Expert Group on Rare Diseases (formerly EUCERD)

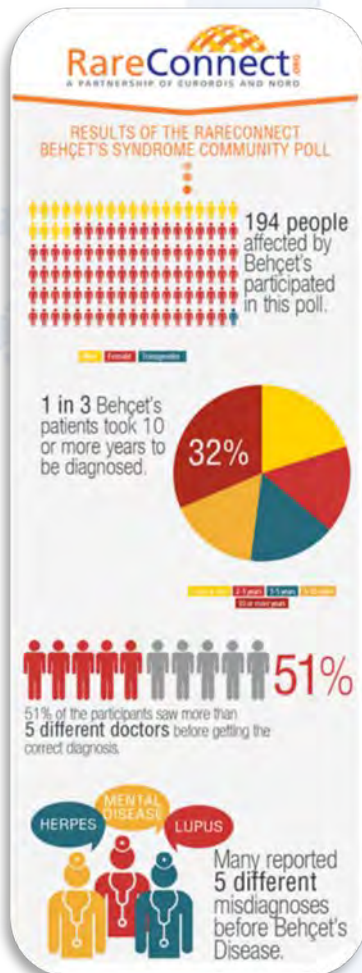


Policy at the National and Local Levels

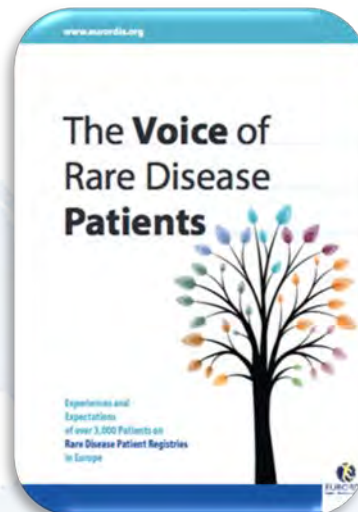
- Representation in National Plan Steering Committees
- Members of Advisory Boards of National Competence Authorities
- Members of Advisory Boards of Centres of Expertise and Social Services
- Raising awareness through Rare Disease Day and disease specific awareness raising campaigns



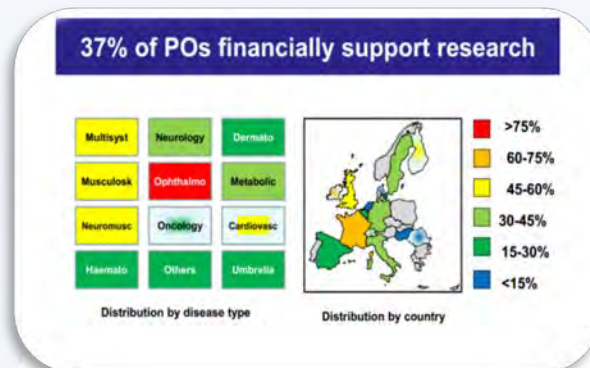
Patient Perspective examples



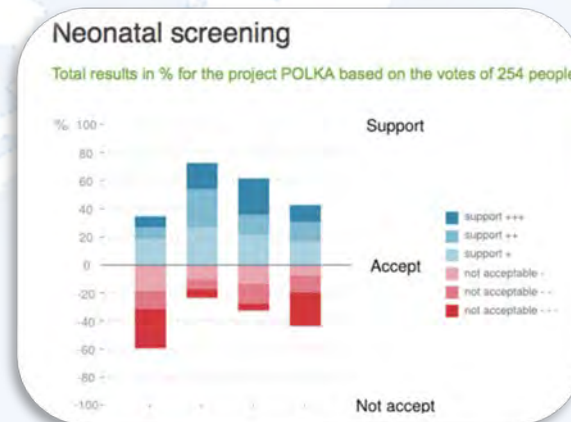
Disease-specific Surveys



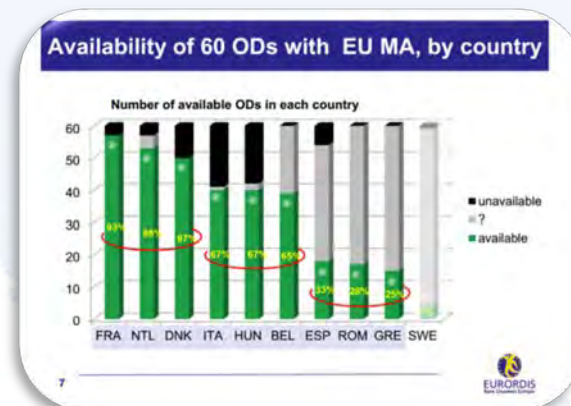
Diagnosis and Care/Patient Registries



Supporting Research

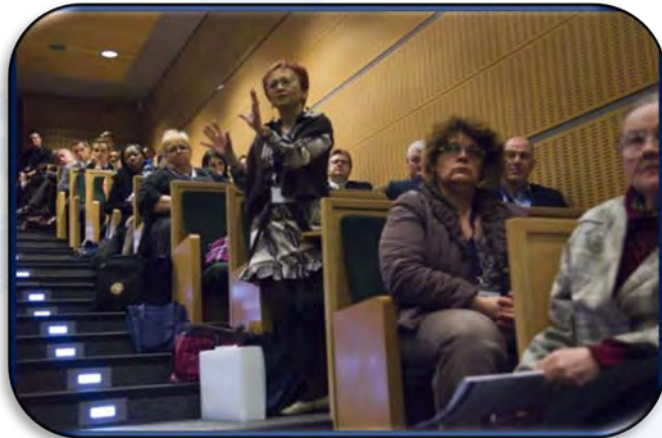


Deliberative Debates



Access to Orphan Drugs

Patient Perspective examples



Focus Groups



**Deliberative
Debates**



**Open-ended
questions**

EURORDISCARE 2 and 3: Diagnosis and Access to Care

- Delays in diagnosis, misdiagnosis, consequences
- Inadequate healthcare pathways and access to health and social care

→ Commission Communication/Council Recommendations

→ EUCERD Recommendations European Reference Networks/Centres of Expertise

→ European Joint Action - *Integration of Rare diseases into Social Policies and Services*

EURORDISCARE 4: Access to Orphan Drugs

- Equitable and timely access to approved orphan drugs for rare diseases patients remains an issue and is highly variable across member states
 - ➔ Recommendations on Pricing & Reimbursement of the EU High Level Pharmaceutical Forum
 - ➔ EUCERD Recommendations - Clinical Added-value of Orphan Medicinal Products Information Flow
 - ➔ Mechanism of coordinated access to orphan medicinal products as part of DG Enterprise and Industry Process on Corporate Responsibility in the field of Pharmaceuticals

POLKA: Play Decide

- Debates around:

- Preimplantation Genetic Diagnosis (PGD)
- Neonatal screening
- Orphan drugs
- Stem Cells
- Diagnosis, information to the patient, genetic counselling
- Cross border health care

➔ EU Directive on Patients' Right to Cross-Border Healthcare

➔ EUCERD Opinion on Newborn Screening in Europe

EPIRARE: Rare Disease Registries Survey

- Collective value of patient registries
- Gap between reality vs. patient expectations (healthcare and social planning vs. epidemiology and clinical research)
- Community support for European infrastructure
- Need for capacity building
 - ➔ Joint EURORDIS-NORD-CORD Statement
 - ➔ EUCERD Recommendations – Registries
 - ➔ JRC – DG SANCO Joint Agreement for a European Platform for Rare Disease Registries

New Paradigm

- Benefits and efficiencies associated with evidence-based programs or policies, including the patient engagement are being recognised.
- Requests for patient perspective are increasing
- Methods for information gathering and analysis are improving and more accessible

Benefits

- Through patient and patient organisation participation and engagement, EURORDIS can
 - More regularly generate evidence from the patient perspective
 - On a continued and professional basis
 - With shorter turn-around and quicker feedback
 - Creating a unified voice on a range of issues at the European level



Obstacles Capturing the Patient Perspective

- Disease related limitations in participation
- Difficulty in representativeness
- Proxy participation
- Complex topics, required parallel capacity building
- Quality analysis with limited resources, incentives, time and partnership
- Adequate return of results
- Increasing polarized debates
- Response fatigue
- Translations

LET US KNOW YOUR EXPERIENCE: ENGAGE!

- Have surveys been an effective survey tool?
- Are data collection methods well adapted?
- Are results adequately disseminated?
- What future topics are important to you?