European Policy in the field of Rare Disease to improve Patient Care and boost Research

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A case for action

RD are individually rare but patients are numerous as there are many RD.....

Patients with RD deserve the same attention as other patients
RD: a case for Health Care Policy
Emergence of the concept of RD

• **USA:**
  – 1983: Orphan drug regulation
  – 1993: Office of Rare Diseases

• **EU:**
  – 1985: Denmark – Rare Disorders Denmak
  – 1989: Sweden – Centres of reference and Agrenska
  – 1996: Orphanet
  – 1997: EURORDIS
  – 1998: Italy – Priority in Public Health
  – 2000: EU Regulation on **Orphan Medicinal Products**
EU Public Health Policy for Rare Diseases

• 1999-2007:
  • Rare diseases as one of the priorities in **Public Health**
  • Target: improving knowledge and facilitating access to **information**
    – Orphanet Europe established in 2000

• 2008-2013:
  • Exchange of information via existing **networks**
  • Strategies and mechanisms to encourage transnational **cooperation**: European Reference networks of Centres of Expertise
EU policy in Public Health

• **11 Nov 2008: EC Communication** « Rare Diseases: Europe’s challenge »

• **9 June 2009 « Council Recommendation** on an action in the field of rare diseases »
  – All countries should have a plan or a strategy by the end of 2013

• **European Union Committee of Experts in Rare Diseases:**
  – 51 representatives of Ministries of Health, of patients, of experts, of Industry representatives
National Plans in 2014
2014 EUCERD Report on the State of the Art of Rare Disease Activities in Europe

2013 REPORT ON THE STATE OF THE ART OF RARE DISEASE ACTIVITIES IN EUROPE OF THE EUROPEAN UNION COMMITTEE OF EXPERTS ON RARE DISEASES

PART I: OVERVIEW OF RARE DISEASE ACTIVITIES IN EUROPE

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www.eucerd.eu
RD: a case for Research Policy
EU policy development in Research

• 2000: RD as priority diseases for funding
• 2004: Common call for proposals among voluntary countries
• 2011: Establishment of the International Rare Diseases Research Consortium (IRDiRC)
• 2013: EC funding for a European Platform for registries in the field of rare diseases
• 2014: Recommendation to use Orphacodes to code rare diseases in addition to ICD10
Empowered Patient Organisations
EURORDIS
a single voice in Europe
2,392 disease-specific patient organisations
National alliance for rare disease patient organisations
Major achievements so far
National Centres of Expertise
Establishment of Centres of Expertise in each country

- EUCERD Recommendations on Quality Criteria for National Centres of Expertise
  - www.eucerd.eu
  - Process to identify them
  - Organisation of care
    - Quality of care
    - Good practice guidelines
    - Clinical research
    - Training/teaching
Experiences about CE to be shared

• How best to cover all RD patients’ needs at national level in terms of disease coverage?

• Countries with past experience in Europe ->
  • Expertise officially identified at national level and/or established centres specialised in some RD/groups of RD:
    • Denmark  (2 centres)
    • France    (131 centres)
    • Norway    (16 centres)
    • Spain     (62 centres)
    • UK        (50-60 centres)
    • Italy     (215 regional centres)
Better information to all citizens
Orphanet as a Joint Action between European Countries
A relational database which allows multiple sorts of queries in 7 languages

Dutch, English, French, German, Italian, Spanish, Portuguese
>25 000 accesses daily from over 200 countries
2/3 Professionals  1/3 Patients and relatives
The Orphanet consortium

Partners are public institutions representing their country. Partners dedicate resources to the Orphanet portal of rare diseases and orphan drugs. National Orphanet teams work according to the Orphanet Standard Operating Procedures.

www.eucerd.eu
Orphanews to inform the Community:
- 16,000 registered readers
- 20 issues a year
- Political and Scientific news
Conclusion

• Real impact of policies
• Dynamic at national level as well: adoption of plans or strategies
• Networking, Orphanet, registries and strong patient organisations are the key success factors
• All RD face the same problems
Thank you for your attention