



Landscape of Disease Registries in Europe and Challenges at Country level

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Patient registries in national and EU texts

- Development of registries and databases in the field of RD encouraged explicitly in:
 - ✓ **Council Recommendation on an Action in the Field of RD (2009/C 151/02) (8 June 2009)**
- Registries also a key element of **national plans/strategies** for rare diseases for epidemiological, basic/clinical research and public health purposes

What for ?

- Assess the size of the problem
- Support clinical decision-making
- Assess efficacy and safety
 - nb of patients, exposure, indication, adverse effects reports
- Assess cost-effectiveness
 - costs (direct and indirect), savings

Even better reasons to access patient data nowadays....

- Expensive treatments under scrutiny
 - Evidence required by payors
- Evolution toward adaptative licensing
 - Slow accumulation of data for small populations
 - Evidence as a process, even a long process
- Adaptative market entry
 - Real life data
 - Evidence of effectiveness for costly products

Key elements

- Comparative data across registries
 - need for quality standards
- Reaserchable data:
 - knowledge of what data is available, where, under which condition
 - Documentation of procedures
 - Data respectful of regulatory and legal obligations

An avalanche of Problems

- Registries are expensive
- Managing registries is boring
- But requires highly qualified staff
- Difficult to motivate reporting
- Few opportunities to publish for a heavy workload
- Extra burden for clinicians who have to report
- Many missing data, poor quality
- Data are under used
- When used are criticized.....

Common issues for RD registries

- Scarcity of cases and complexity of diseases impose a large geographical coverage, trans-national
- Resources are limited, funding is limited in time
- Waste of resources
 - in developing tools for each registry
 - In duplicating efforts
- Waste of expertise
 - Clinicians are not epidemiologists
- Waste of opportunities
 - Drug registries vs disease registries

Current status of RD Registries in Europe

Data from Orphanet

[http://www.orpha.net/orphacom/cahiers/docs/
GB/Registries.pdf](http://www.orpha.net/orphacom/cahiers/docs/GB/Registries.pdf)

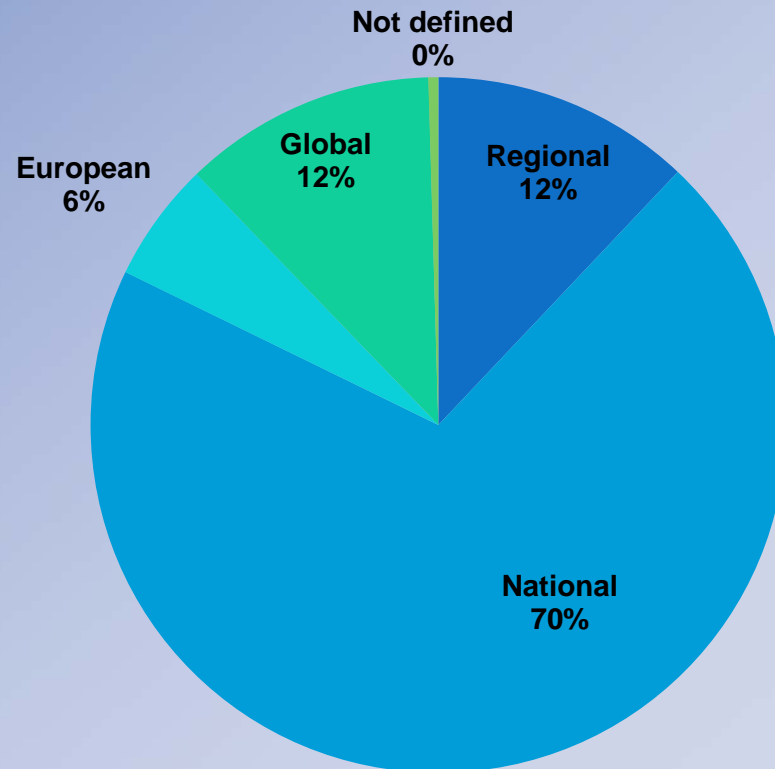


651 Registries in Europe

Number of disease registries per country

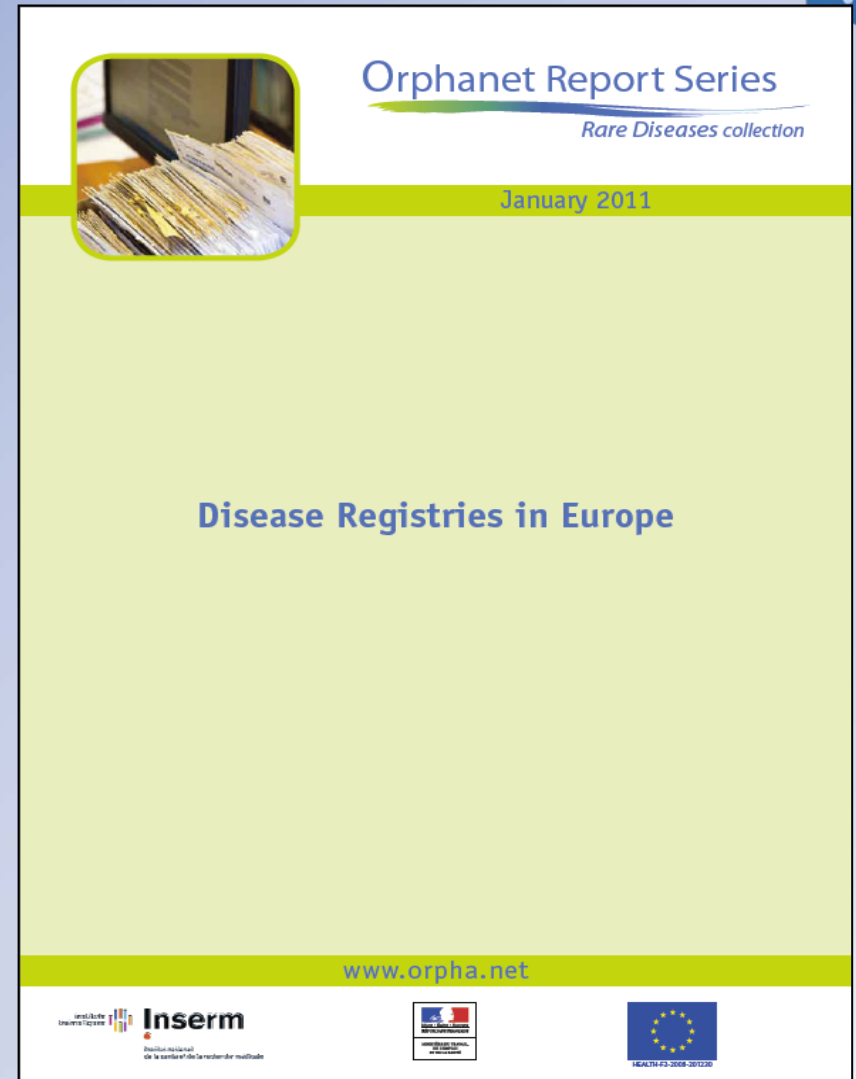
● France	130	● Poland	9
● Germany	115	● United States	10
● United Kingdom	70	● Switzerland	9
● Italy	70	● Bulgaria	11
● Spain	53	● Finland	8
● The Netherlands	26	● Norway	7
● Belgium	22	● Czech Republic	4
● Sweden	19	● Denmark	4
● Austria	18	● Serbia	4
● Portugal	16	● Turkey	4
● Ireland	11	● Hungary	3

Geographical coverage of rare disease registries registered in the Orphanet database



Orphanet Report Series: RD Registries in Europe

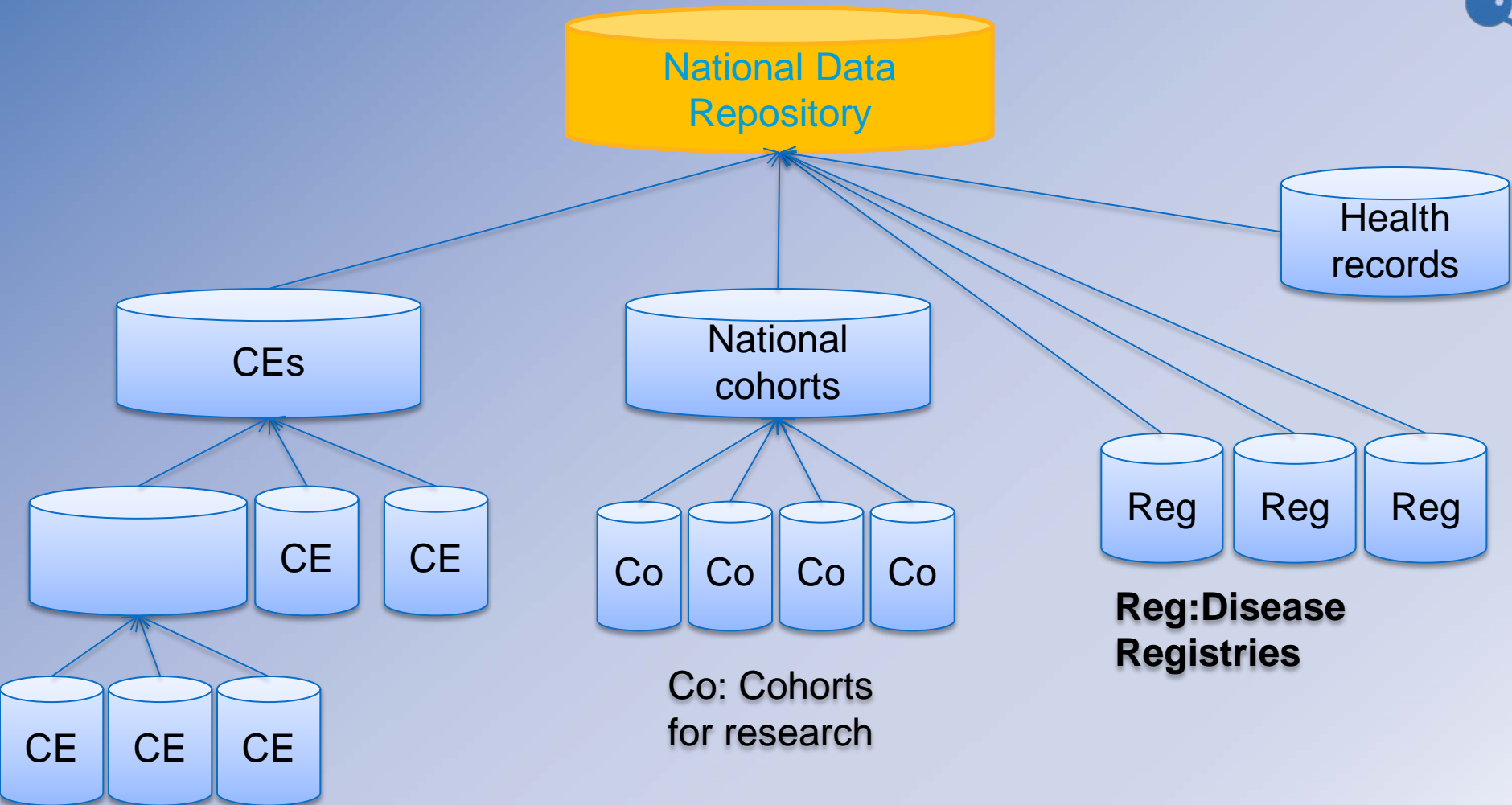
53,000 Downloads
in 2013



Too many duplications of efforts

- Foster the establishment of quality data repositories
 - To ease and speed up clinical research
 - To provide data to regulatory / reimbursement bodies
- Avoid duplication of efforts / waste of resources and expertise
- Provide unified sources of data for diseases where several products are available
- Take advantage of the technology to share data repositories

National Data Repository Model



National Data
Repository

Health
records

CEs

National
cohorts

Reg

Reg

Reg

CE

CE

Co

Co

Co

Co

CE

CE

CE

**Reg:Disease
Registries**

Co: Cohorts
for research

CE: Center of Expertise

Summary conclusions

- Apply a bottom-up approach when setting up and running registries
- Research opportunities are an important driving force
- Registries should be established as early as possible when a product is in development
- Registries should be conceived in partnership with Academic teams and Patient organisations
- Regulatory obligations for MA and post MA are an **opportunity** to establish public/private partnership
- Payors should be involved in the financial support to data collections

**EUCERD CORE RECOMMENDATIONS
ON RARE DISEASE PATIENT
REGISTRATION AND DATA COLLECTION**

**TO THE EUROPEAN COMMISSION,
MEMBER STATES
AND ALL STAKEHOLDERS**

Thank you for your attention!

The text of the recommendation can be found on the EUCERD's website

www.eucerd.eu