

EPIRARE Common Data Elements

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EPIRARE

"Building Consensus and Synergies for the EU Registration of Rare Disease Patients"

Specific Objectives

- To identify the needs of the EU registries and databases on RD
- To identify key issues to prepare a legal basis
- To agree on the Register and Platform Scope,
 Governance and long-term sustainability
- To agree on a Common data set, disease-specific data collection and data validation
- To identify tools and other facilities supporting the operation of the platform users

The evidence base

Ad hoc funding, momentum and commitment (EPIRARE, CNMR)

- Survey of registries conditions and needs
- Survey of collected variables, definitions and formats
- Survey of patients expectations
- Consultation/survey of industry expectations
- Consultation/Information of national policy-makers
- Personal consultation with "payer" associations
- Review of policy actions and funding initiatives
- Liaison with US ORDR and RD-Connect (IRDiRC)
- Participation in EU initiatives and RD Committees



Main issues found in registries

Data quality control procedures
Coding and reference catalogues
Governance
Communication
Networking and data sharing

Needs of identified stakeholders' groups

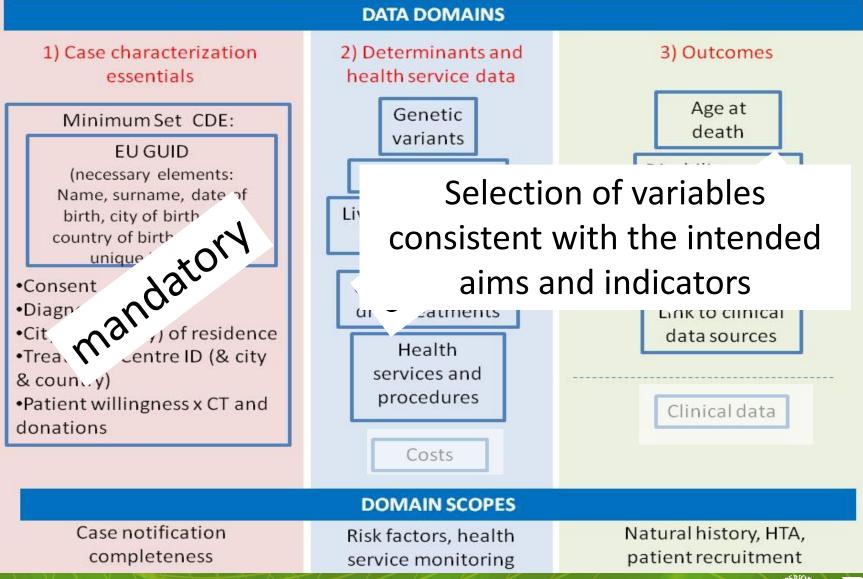
- Registry holders
- Patients
- National HA and Payers
- Pharmaceutical Industry
- •EMA and EC

=> EPIRARE Deliverable 5

Scope of the indicators and variables considered

- Epidemiological information
- Information for RD policy planning and monitoring;
- Monitoring RD-dedicated health services and integration in the NHS;
- Health technology assessment (appropriateness of OD and other treatments)
- OD and other treatment
- Patient recruitment
- Patient care benchmarking

The organization of the EPIRARE Database



International Summer School on Rare Disease and Orphan Drug Registries Rome (Italy),15-19 September 2014.

Domain 1) Case characterization essentials

- •EU Global Unique Identifier (EU GUID): name, surname, sex, date and place of birth, national ID
- Patient sex
- Patient date of birth
- Patient place (+Country) of birth
- Diagnosis
- Patient place (+Country) of residence
- ID Treatment Centre (+place+Country)
- Current and past participation in clinical trials
- Patient willingness to be contacted to participate in a clinical trial
- Patient willingness to be contacted about donating biomaterial
- Patient consent
- Patient contact preferences



Domain 2) Determinants and services

A sample selection of data

- other cases in the family
- healthy carriers in the family
- case parents are consanguineous
- Genetic features of the patient
- Current orphan drug treatment
- Hospitalizations (number)
- Transplantations (date and biomaterial transplanted)

Domain 3) Outcomes

- Patient vital status (and date of death)
- Education level
- Occupational status
- Patient HRQoL index score
- Comorbidity
- Remarkable or unusual symptoms

Recommended readings

Gliklich RE, Leavy MB, Levy D, Karl J, Campion DM, Taylor T. Registry of Patient Registries (RoPR) Policies and Procedures. Effective Health Care Program Research Report No. 41. 3rd edition (2014). effectivehealthcare.ahrq.gov/reports/final.cfm.

Manuel Posada de la Paz· Stephen C. Groft (Eds.) Rare Diseases Epidemiology. Adv. Exp. Med. Biol. (2013) 686, 475-491.

EPIRARE deliverables (www.epirare.eu)



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Thank you for your attention!

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