



# **EUCERD Recommendations on Rare Disease Patient Registration and Data Collection**

[Name, date and place of presentation)

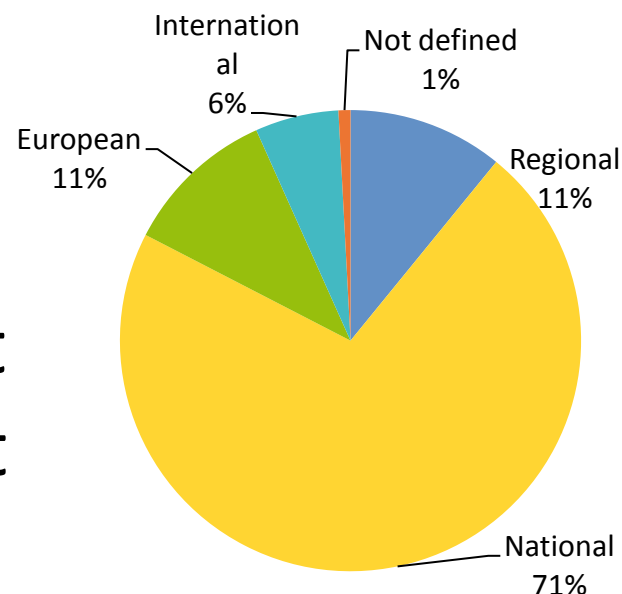
# RD PATIENT REGISTRIES/ THE CONTEXT

# Importance of registries and data collections in the field of rare diseases

- Key instruments to develop clinical research, to improve patient care and healthcare planning
- Only way to pool data to achieve sufficient sample size for research
- Vital to assess feasibility of clinical trials and support enrollment of patients
- Crucial for post-market authorisation surveillance of orphan drugs and surveillance of drugs used off-label

# State of the art of patient registries

- There are 588 disease registries in Europe
- Most concern diseases/groups of diseases with an innovative treatment on market or in development
- Most are academic, with a minority managed by Industry



# 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

# Evolving European and International context

- Opportune moment to ‘fix’ consensus on the topic considering:
  - Implementation phase of national plans/strategies for RD
  - Establishment of a European platform for rare disease registries at the Joint Research Centre in Ispra, Italy
  - Future work in the field of registries at the level of the IRDiRC (International Rare Diseases Research Consortium) concerning interoperability and pooling of data

# AIMS OF THE RECOMMENDATIONS

# Why a recommendation in this area

- Ideal moment to ‘fix’ the consensus reached to date amongst stakeholders, taking stock of the outcomes of previous work in the area
- To act as a solid basis for future discussions in this area
- To guide MS elaborating their national plans/strategies for rare diseases
- To advise the EC in their reflection concerning sustainability of registries



# Recommendations based on outputs of previous meetings and publications

**Recommendations have been based on the outputs of various multi-stakeholder meetings and previous publications, including:**

- Gliklich RE, Dreyer NA eds. *Registries for Evaluating Patient Outcomes: A User's Guide*. 2<sup>nd</sup> ed. (AHRQ, September 2010)
- RDTF: *Patient Registries in the Field of Rare Diseases*, Apr 2009, updated Jun 2011
- EMA/EUCERD: *Towards a Public-Private Partnership for Registries in the Field of Rare Diseases*, Workshop Report, London, 4 Oct 2011
- EUCERD Joint Action: *Workshop Report on Rare Disease Registration*, Luxembourg, 13<sup>th</sup> Nov 2012, and drafting group and breakout session discussions (29-30 January 2013)
- EJA- EPIRARE Joint Workshop on Registries (22-23 April 2013, Paris)
- EURORDIS/ CORD/ NORD : *Joint Declaration of 10 Key Principles for Rare Disease Patient Registries*, Nov 2012
- Joint EBE-EuropaBio Task Force on Rare Diseases and Orphan Medicines: *Position Paper for Rare Diseases and Orphan Drugs Registries and Databases*
- EPIRARE Rare Disease Registry Survey
- ENCePP E-Register of Studies Guide

# Elaboration procedure

- Discussion of potential recommendations at 7th EUCERD meeting in Jan/Feb 2013
- Revision of recommendations and round of consultation in March 2013
- Finalisation of recommendations at EJA/EPIRARE workshop 22-23 April 2013
- Recommendations sent to EUCERD 4 weeks ahead of this meeting for adoption
- Adopted unanimously at 8th EUCERD meeting 5 June 2013

# CONTENT OF THE RECOMMENDATIONS



# Target audience

- Member States
- European Commission
- All stakeholders

# Scope and principal messages

- Importance of international operability of patient registries and data collections
- All sources of data to be considered as sources of information for RD registries and data collections
- Collected data to be utilised for public health and research purposes
- Patient registries and data collections to adhere to good practice guidelines in the field
- Importance of multi-stakeholder participation (design and governance)

# Scope and principal messages

- Existing and future patient registries and data collections to be flexible for future needs, i.e. adaptable to serve regulatory purposes, where required
- Patient registries and data collections to be sustainable for the foreseeable timespan of the registries' utility
- Public-private partnerships as long-term model for optimisation of resources, sustainability and co-creation of knowledge to be encouraged

# RECOMMENDATIONS IN DETAIL

# Interoperability

**1. RD patient registries and data collections need to be internationally interoperable as much as possible and the procedures to collect and exchange data need to be harmonised and consistent, to allow pooling of data when it is necessary to reach sufficient statistically significant numbers for clinical research and public health purposes.**



# Interoperability

- **1.1** They should use international standards and nomenclature to code the tentative or final RD diagnosis. Either the OMIM code or the Orpha codes are recommended alongside any other coding system in operation in the MS health systems, such as ICD and SNOMED-CT, with a view to establishing a common semantic approach.
- **1.2** There should be adoption of a minimum common data set across RD that registries should collect, in collaboration with global initiatives, to allow the establishment of national and/or European RD population registries, which have the potential to collect data on all RD patients.

# Interoperability

- **1.3** A minimum common data set should be defined, and supported with a semantic approach and Standard Operating Procedures. Interoperability (via means of mapping) of registry specific data sets towards this common data set should enable comparison across all RD and internationally.
- **1.4** For disease-specific registries, appropriate core data sets specific to the diseases or disease groups should be adopted. In the future, such disease-specific registries could fall under the remit of RD ERNs. Every effort should be made to incorporate current disease-specific registry initiatives where quality can be assured.

# Interoperability

- **1.5** To avoid duplication and to support Cross-Border Healthcare, the possible benefits of using a global or European RD patient identifier (possibly incorporating the current health identifier) should be investigated to provide a way to link information, samples and research data, and to ensure a quick and secure means of data sharing and protection.
- **1.6** For countries with regional organisation of healthcare, where multiple registries exist, overlap and duplication between the regional and national registries, should be avoided.

# Sources of Information

**2. All sources of data should be considered as sources of information for RD registries and data collections, to speed up the acquisition of knowledge and the development of clinical research.**

# Sources of Information

- **2.1** As with all registries, registries for RD should establish clear purposes and objectives of the data collection: the type of data collection should be suited to the need, and the data captured should be appropriate to the proposed use of the data, both in terms of scope and level of detail.
- **2.2** RD Centres of Expertise, where they exist, should contribute to a registry(ies). Other experts in the field should also contribute to the registry(ies).

# Sources of Information

- **2.3** (Electronic) health records from any sector of healthcare delivery are a valuable source for core data collection. Automatic data acquisition from these sources should be envisaged to ease the data collection process.
- **2.4** Collection of data on RD should be delineated in the National RD plan/strategy.
- **2.5** A system to allow the collection of data directly reported by patients should be included along with systems for data reported by clinicians.

# Recommended good practices

## 3. Collected data should be utilised for public health and research purposes.

- **3.1** RD data collected should be used to support policy development at local, regional, national and international level.
- **3.2** RD data collected should, where possible, facilitate clinical and epidemiological research and the monitoring of care provision and therapeutic interventions, including off-label use of approved drugs and existing medications.
- **3.3** RD data collected should, where possible, be used to provide information for multi-centre and multi-national clinical trial feasibility studies .

# Recommended good practices

- **3.4** Pooling of data across data collections and other resources, including internationally, should be encouraged to reach a critical mass for data analysis. According to the governance/oversight criteria, data should be made accessible to groups with legitimate questions such as researchers and policy/decision makers.
- **3.5** Access and sharing of data should be defined to control how data is shared and published in the public domain and this should be facilitated through the national RD plan/strategy.



# Recommended good practices

**4. Patient registries and data collections should adhere to good practice guidelines in the field.**

**Specific to the current and future specificities of RD registries:**

- **4.1** Involvement of stakeholders such as patients, policymakers, researchers and clinicians (and industry, where appropriate) in the design, analysis and governance of registries is important to address the complexity and scarcity of knowledge on RD.

# Recommended good practices

- **4.2** Representatives of all stakeholders should be invited to provide best possible expert support through an advisory board or committee to ensure appropriate information flow and knowledge exchange into and from the registry, and they should define a sustainability and exit strategy for the registry. Where appropriate, representatives from industry should also provide input.
- **4.3** This multi-stakeholder model for registry governance should apply not only at a national level but also at the European level and/or pan-European Platform repository of RD registries.

# Recommended good practices

- **4.4** The process for consenting patients for participation in a RD registry should take into account the wider European and international context to ensure that patients are well informed of this dimension and the consent process is in line with the legal requirements at European and International level.
- **4.5** Patients already in a RD registry may be required to go through an additional consenting step to ensure compatibility with such systems.
- **4.6** RD registries should have a system to provide regular feedback to registered patients and their clinical teams, recognising their specific role in the success of registries in this field.

# Adaptability of Registries

**5. Existing and future patient registries and data collections should be adaptable to serve regulatory purposes, where required.**

- **5.1** For the monitoring of therapeutic interventions for RD, a strategy between industry, academia and regulators should be agreed to ensure that data collection is expanded as necessary, and in time embedded in disease-specific registries to serve, for example, the requirements for post-marketing surveillance, and to support development of new therapies. Data access needs to be compliant with agreed guidelines established by the registry.

# Adaptability of Registries

- **5.2** As quality assurance is crucial, it is a priority for existing RD registries to explore their capacity to adapt to collect data for regulatory purposes.
- **5.3** There should be an early dialogue on the type of registry required (and what data is required for regulatory purposes), and/or whether a registry exists for the condition targeted, with all stakeholders, in order to optimise the registration of patients and the generation of knowledge for RD for which a therapeutic intervention is being developed. Collection of data regarding off-label use of approved drugs and existing medications should be encouraged.

# Sustainability

## 6. Patient registries and data collections should be sustainable for the foreseeable timespan of the registries' utility.

- **6.1** Local, regional, national and European structures contributing to or overseeing data collection should all be supported financially to carry out this role in a sustainable way so that financial responsibility for registries is shared proportionately between stakeholders, MS and the EC and defined in the appropriate funding programmes.

# Sustainability

- **6.2** Public-private partnerships for RD registries should be considered where applicable as a long-term model for optimisation of resources, sustainability and co-creation of knowledge.
- **6.3** All registries and data collections should have in place an exit strategy in its work plan, including contingency planning for the data in the event that the registry is terminated. There should also be a procedure outlined for succession planning for registry continuation.

# Thank you for your attention!

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

[www.eucerd.eu](http://www.eucerd.eu)