EU Policy on Rare Diseases activities relevant for undiagnosed patients

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SCOPE and Context

European Reference Networks

- ✓ **Directive Patient's Rights to Cross border Healthcare (transposition deadline 25 October 2013)**
- ✓ **Cooperation between MS: Article 12 European Reference Networks**

Networks of healthcare providers designated as Centres of Expertise which aim at

Improving access to highly specialised healthcare

Patients/conditions requiring a particular concentration of expertise:

- **low prevalence / low incidence**
- **Complexity**
- **high cost of care of their disease or condition**

- ***Commission***

  - ✓ **Support MS in the development of ERN**
  - ✓ **Adopt Delegated & Implementing Acts**

- ***Member States***

  - ✓ **Connecting providers & Centres of Expertise at national level**
  - ✓ **Fostering participation in the ERN.**

- ***Voluntary*** *participation of providers**:***

  - ✓ **Fulfilling all required conditions and criteria**

Milestones and timeline for the implementation (ERN)

European Commission

2011 - 2015

Delegated Acts
(Art. 17)

Implementing acts
(Art. 16)
Committee

legislative process

Adoption of a list of criteria and conditions for the CR & ERN to fulfil
Art. 12.5

Exchange of information and expertise for ERN
Art. 12.4(c)

criteria for establishing and evaluating ERN
Art. 12.4(b)

Deployment Process Establishment of ERNs

Work on progress

European Commission

Looking at best practices: MS and Centres visits

Meetings with Stakeholders

Meetings with
- **EUCERD**
- **JA Against Cancer**
- **PARENT**

Cross-border Healthcare Expert Group

Advise

SANCO

Technical Brainstorming & workshops

Public Consultation

Reports and technical papers

Legislation

Draft Delegated Decision

Draft Implementing Decision

Commission Inter Services Consultation

Commission Inter Services Consultation

Vote in MS Cross border Committee

Adoption of Delegated Decision

Adoption of Implementing Decision

Structure of the elements for the delegated act

Objectives provided in Article 12 (2) → 8 objectives

Areas to be ensured as provided in Article 12 (4) (a) → 6 areas

Criteria suplemented by the delegated act. Mandate to the Commission (Article 12 (4) (a) and 12 (5) →

A (ERNs): 7 groups
B (Gen. CoEs): 5 groups
C (Spe. CoEs): 2 groups

Criteria and conditions for Networks

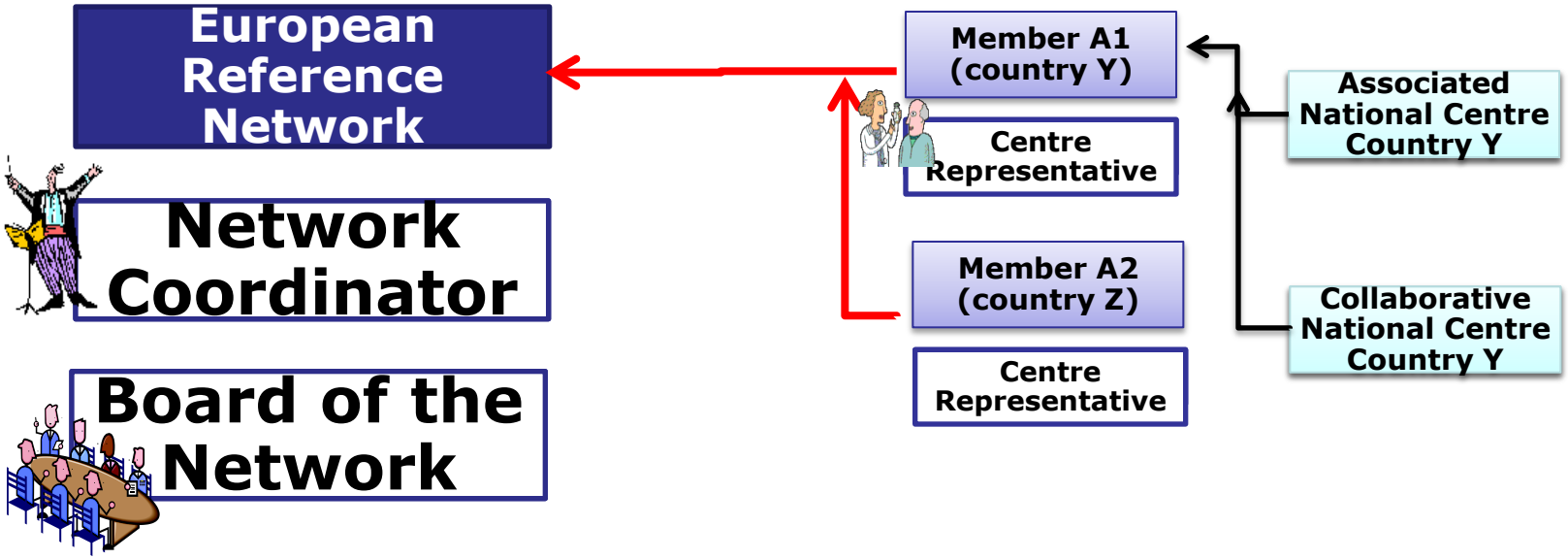
- ✓ *1. A.1.- have **knowledge and expertise to diagnose, follow-up and manage patients** with evidence of good outcomes*
- ✓ *1.A.2.- Follow a **multi-disciplinary approach***
- ✓ *1.A.3.- Offer a high level of expertise and have the capacity to **produce good practice guidelines** and to **implement outcome measures and quality control***
- ✓ *1.A.4.- Make a contribution to **research***
- ✓ *1.A.5.- Organise teaching and **training** activities*
- ✓ *1.A.6.- **Collaborate** closely with other centres of expertise and networks at national and international level*

Facilitate: cost-effective use of resources

Focusing on: highly specialised healthcare / treatment recognised by international medical science (safety, value and positive clinical outcomes)

Governance / coordination Networks

- ✓ ***Transparent and effective*** *coordination & governance (adequate structures and elements)*
- ✓ ***Networks can be flexible*** *& have different architectures and internal relations* ***but there are key organizational features.***



4. ***Collaboration / connection within and with other healthcare providers and Centers*** *(Associated and Collaborative National Centers, national hubs etc..)*

Healthcare Providers

General criteria for all Members in an ERN

- ✓ *shall have in common:*

  (a) the expertise they specialise in;

  (b) certain treatment(s) offered; or

  (c) disease (s) or health condition(s) they focus on.

- ✓ *shall contribute to the objectives and the conditions and criteria of the Networks:*

  (a) patients empowerment and centred care;

  (b) organisational, management and business continuity of the Centre of Expertise;

  (c) research and training capacity;

  (d) exchange of expertise, information systems and e-health tools; and

  (e) expertise, good practice, quality, patients safety and evaluation.

Healthcare Providers

Criteria for the Members specific to the scope of the Network

- ✓ *Shall demonstrate fulfilment with the specific criteria and conditions adapted to the concrete area of expertise, disease or condition addressed by the Network.*

(a) competence, experience and outcomes of care; and

(b) specific human structural and equipment resources and organisation.

ANNEXES

29 criteria

4 requisites

16 criteria

ANNEX *1*

Criteria and conditions to be fulfilled by the Networks

1. **Have knowledge and expertise to diagnose, fo evidence of good outcomes by:**
   a. Promoting good quality and safe care to and conditions by fostering adequate management of patients across the Networ
   b. Empowering and involving patients in safety of the care received.
2. **Follow a multi-disciplinary approach:**
   a. Identifying domains and best practices for
   b. Facilitating and promoting multidisciplina
3. **Offer a high level of expertise and have the guidelines and to implement outcome measure**
   a. Exchanging, gathering and disseminating within and outside the Network, in part therapeutic options and best practices rela treatments available for each particular dis
   b. Promoting and supporting the expertise increase the local, regional and national close as p to the patients;

ANNEX 2

(1) Criteria and conditions to be fulfilled by all healthcare providers

1. **Patient empowerment and patient-centred care:**
   a. Strategies in place to ensure patient-centred care, rights (e.g. right to informed consent; information concerning own health; medical records; privacy; complain and compensation), empowerment and participation (e.g. customer relationship management, patient education and active engagement strategies of patient and families throughout the institution);
   b. Clear and transparent information remedies and redress for both domesti
   c. Active measurement and feedback rep
   d. Personal data protection rules and a information in compliance with natio particular to Directive 95/46/EC and
   e. Transparency, including information quality and safety standards in place.
2. **Organisation, management and busines**
   a. Evidence of transparent and explicit orga including in particular the procedures re patients in their area of expertise;
   b. Transparency of the tariffs;
   c. Business continuity plan over a given tim

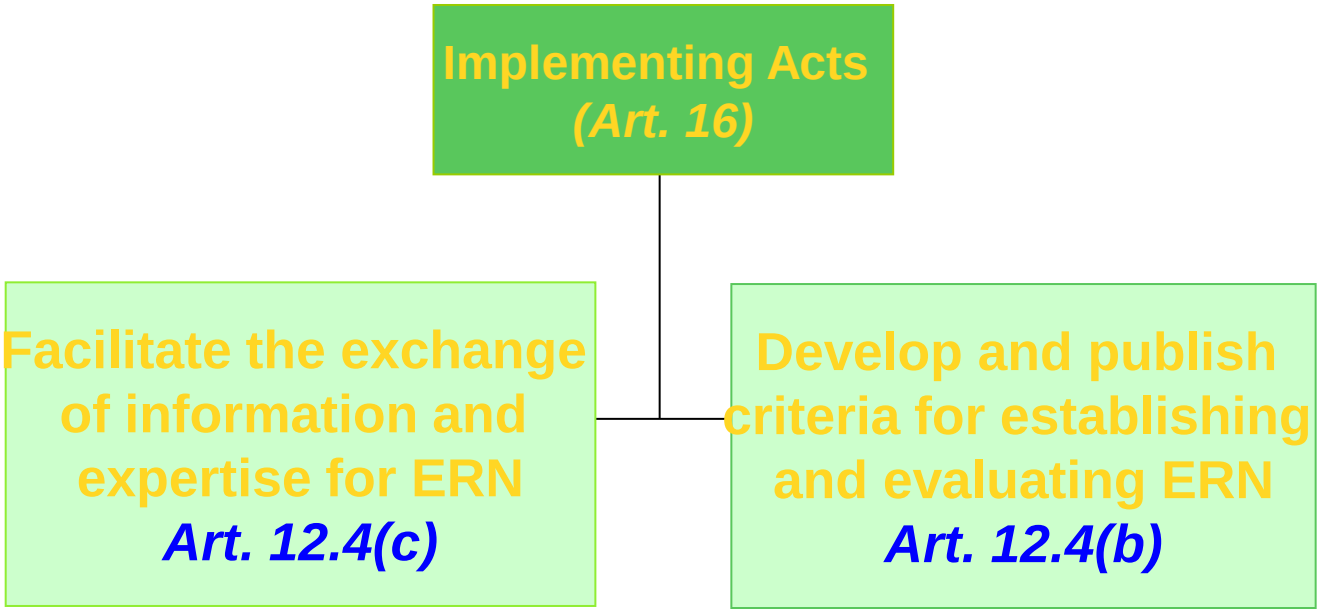
Annex 3

Other healthcare providers linked to the Networks: Associated and Collaborative National Centres

1. Associated National Centres shall have knowledge and expertise in the provision of care in a concrete area of healthcare addressing the specific disease or conditions covered by an existing Network, acting as reference or coordination centres at national or regional level.
2. Collaborative National Centres shall have knowledge, expertise and competence in areas related to the production and dissemination of knowledge, clinical tools and clinical guidelines, training, research or HTA and evaluation, related with the specific disease or conditions covered by an existing Network.
3. The Associated and Collaborative National Centres shall:
   a. be identified following a transparent and explicit procedure including information on the criteria used and the identification of the condition/ area / field of competence of the centre and proposed to be linked to a specific Network.
   b. state their willingness to collaborate with Networks and support its goals using the relevant tools (e.g. information system; operational and communication procedures; clinical guidelines and protocols; criteria for the referral / follow up of patients, training, research).

Implementing Decision

The Implementing Act will establish methodology and procedures for the whole process.



ERNs – Implementing measures

Main elements and content

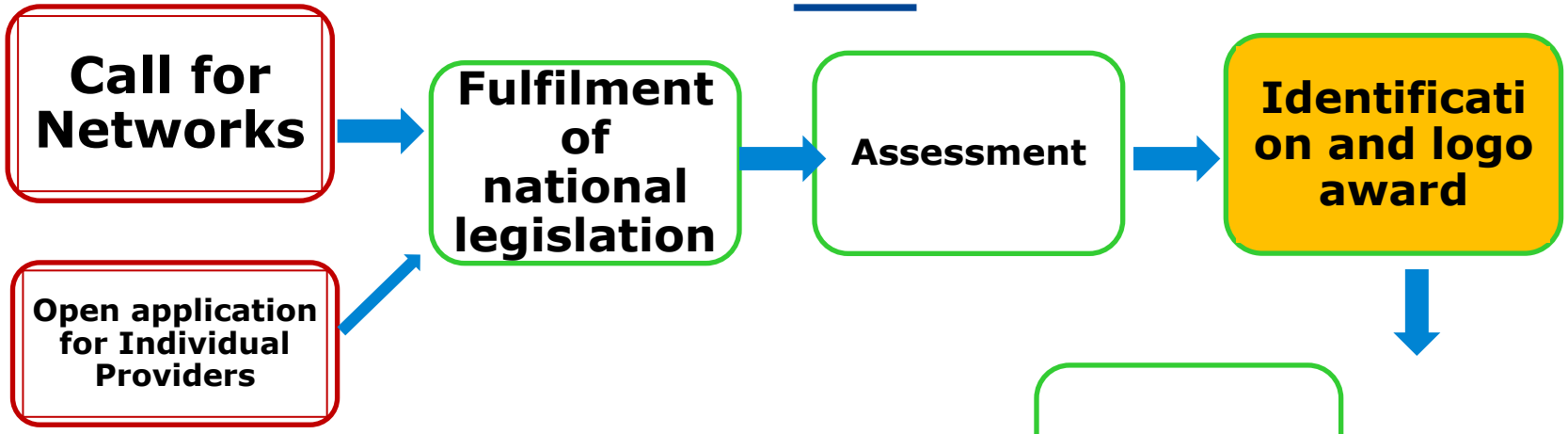
I.-Establishment

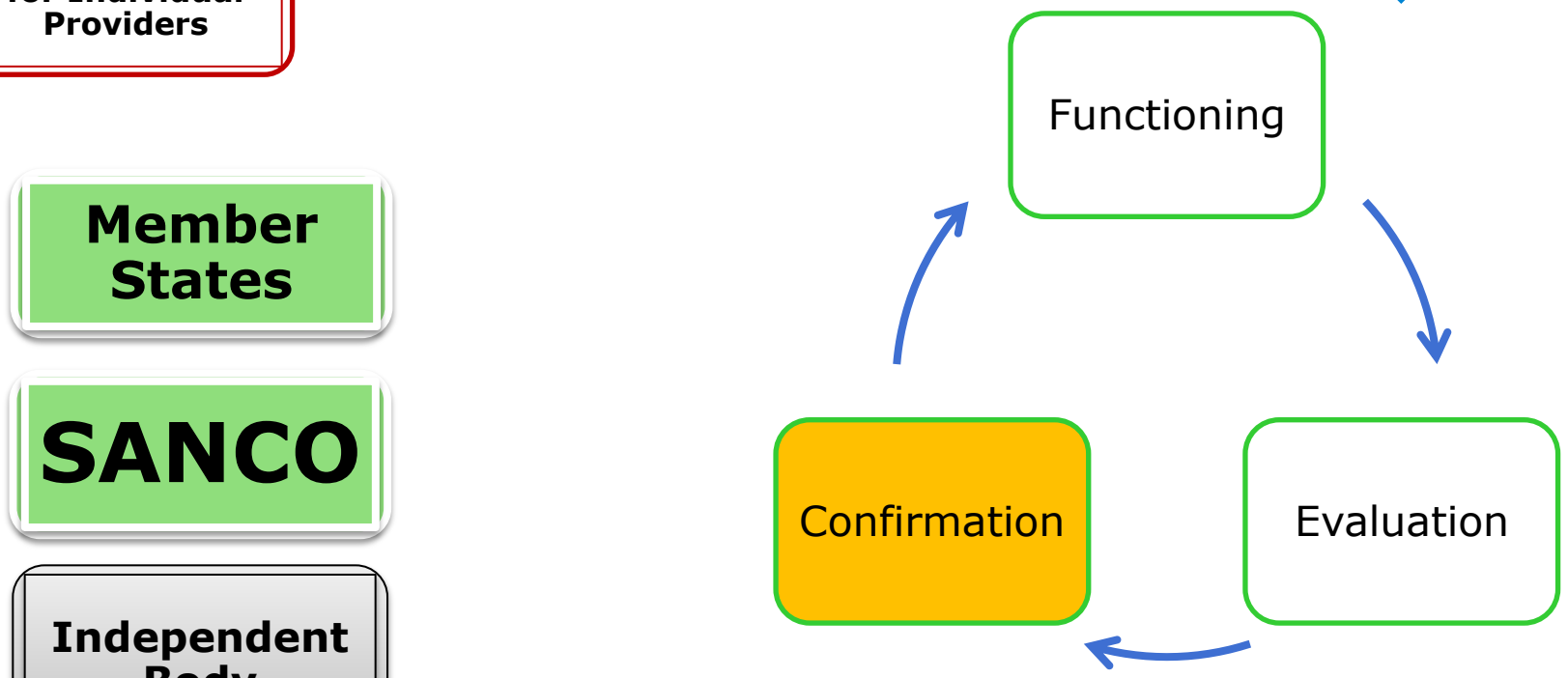
II Evaluation

III.- Assessment and evaluation body:

IV.-Exchange of information and expertise

ERN Circuit





Member States

SANCO

Independent Body

Implementing act

European Commission

Establishment of ERNs

Identification of the Assessment body

Assessment methodology, tools and process

Call for Networks (initial and when necessary)

Permanent open procedure for individual healthcare providers applying for membership of a ERNs'

Call for interest

Application procedures of ERNs and HCP

Assessment of ERNs projects and HCP

(according criteria delegated act)

Establishment of Networks and membership

Implementing act

European Commission

Evaluation of ERNs

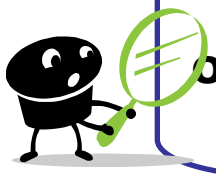
Identification of the evaluation body

Same as in establishment

Evaluation methodology, tools and process

Procedures, application & self-evaluation forms, timeline/recurrence

Submission of evaluation request by the Network



Evaluation: fulfilment of the objectives, the criteria and level of outcomes and performance

Every 5 years

Decision for the continuity of ERNS and Members

Characteristics of the Independent Body

***External and independent**: institutions or entities with a solid background*

- *Have **experience & knowledge** on healthcare systems;*
- *Have the **capacity and knowledge to develop methodology** (manual and procedures) based on the criteria established in the legal acts*
- *Have the **capacity to conduct and carry out assessment** (pre) and **evaluation** (post) of the Networks and healthcare providers;*

Competitive Call

European Reference Networks LOGO

- ***1 common logo for all*** *European Reference Networks and Centres*
- ***Registered*** *trademark*
- *Each designated Centre of Expertise will be* ***granted to use the logo*** *according to* ***fixed clear rules***
- ***Name of the Network and Centre*** *will be included* ***at one side of*** *the common logo (concrete font, colours, position etc.)*

Implementing act

Exchange of information and expertise

Conferences and experts meetings:

- *Forum for technical/scientific debate*
- *To share expertise and information on the establishment/evaluation ERNs*
- *To identify and discuss strategic, operational and common networking elements (communication tools, information systems, etc.)*
- *Participation of ERNs, MS representatives, EC officials, relevant stakeholders, or centres applying for membership of a network.*
- *Participation and advice of the independent assessment and evaluation body*

Pilot European Reference networks

Dyscerne: European Network of Centres of Reference for Dysmorphology (ended)

ECORN CF: European Centres of Reference Network for Cystic Fibrosis (ended)

PAAIR: Patient Associations and Alpha1 International Registry (PAAIR) (ended)

EPNET: European Porphyria Network - providing better healthcare for patients and their families (ended)

EN-RBD: Establishment of a European Network of Rare Bleeding Disorders (ended)

Paediatric Hodgkins Lymphoma Network: European-wide organisation of quality controlled treatment (on-going)

NEUROPED: European Network of Reference for Rare Paediatric Neurological Diseases (ended)

EURO HISTIO NET: A reference network for Langerhans cell histiocytosis and associated syndrome in EU (on-going)

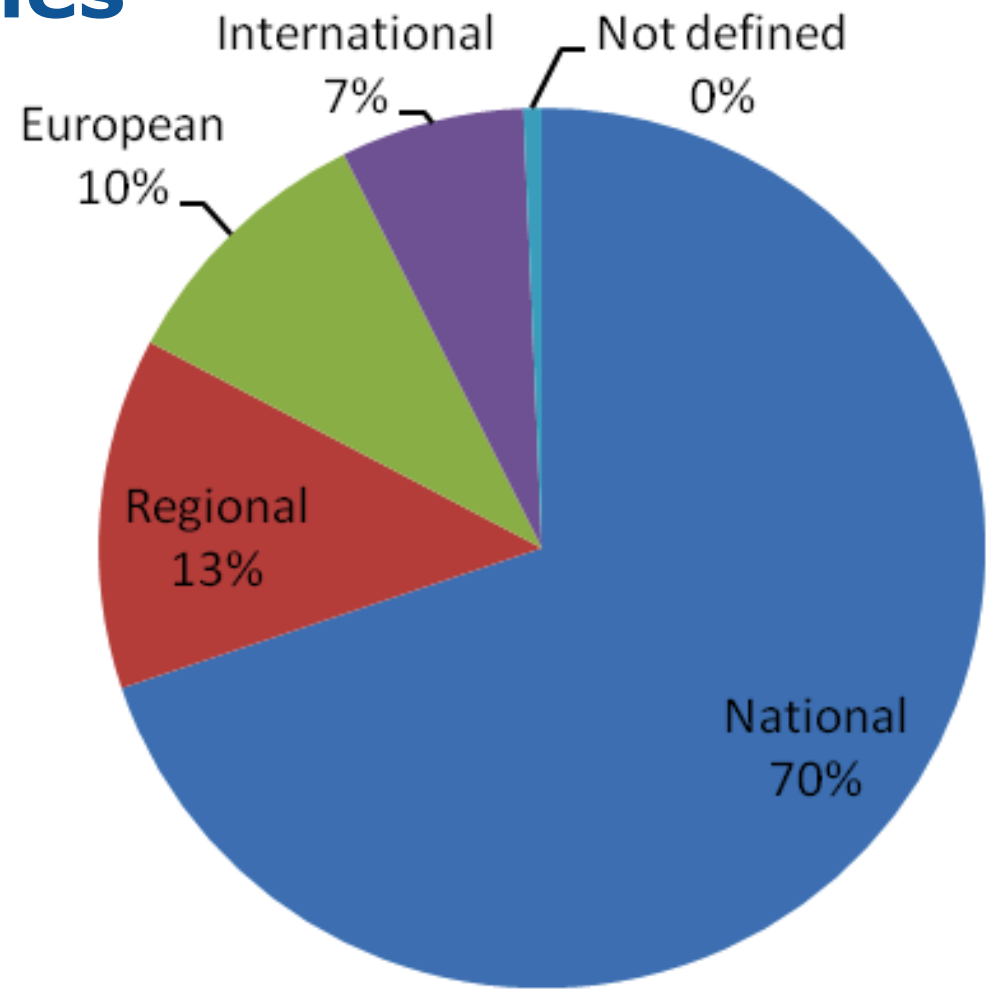
TAG: Improving Health Care and Social Support for Patients and Family affected by Severe Genodermatoses – TogetherAgainstGenodermatoses (on-going)

CARE NMD: Dissemination and Implementation of the Standards of Care for Duchene muscular Dystrophy in Europe (including Eastern countries) (on-going)

EUCERD recommendations

ERNs should have capacity to follow patients with unclear diagnosis and manage their care according to medical need

Registries



597 disease registries

European Platform on rare diseases registration

Annual work plan 2013

4.2.4.4. Support to rare diseases registries and networks in view of their sustainability

The aim of this action is to set up a sustainable platform to coordinate and maintain registries and networks on rare diseases. Registries and networks are key instruments in increasing knowledge of rare diseases and in developing clinical research. They are the only way to pool data in order to achieve a sufficient sample size for epidemiological research and/or clinical research. This action will build on activities and experiences developed through initiatives funded by the EU health programmes and research and innovation programmes.

[Project grant/Administrative agreement with the JRC] Indicative amount: EUR 2 000 000

Main objectives

- Support for new registries in view of interoperability with existing registries;

- Promote the interoperability of existing standalone registries;

Additional objectives

- Host activities of the surveillance networks EUROCAT (European Surveillance of Congenital Anomalies) and SCPE (Surveillance of Cerebral Palsy in Europe);

- Act as a hub providing access to all data collection in the field of RD;

- Provide IT tools to maintain already existing selected data collection (with high added value but lacking sustainability).

EU infrastructure on rare diseases

- Improve sustainability of the results achieved and develop future approaches
- Improve links between research, health professionals, patients, and health care decision makers
- Rare diseases as a model for common health problems
- Proper information is crucial for high quality research
- A more coherent and predictable approach to funding issues
- Overcome current fragmentation of data sources
  - Pulling together and inter-linking initiatives

ERIC - European Research Infrastructure Consortium (Council Regulation (EC) No 723/2009 of 25 June 2009)

- A legal instrument at EU level, to facilitate the joint establishment and operation of RI of European interest.
  - Directly applicable in the Member States
  - Legal personality recognized in all Member States
  - Qualifies as an international organization for the purposes of VAT (exemption under certain limits and conditions from VAT and excise duties) and Public Procurement Directives

Steps undertaken so far

- Drafting of a concept paper;
- Meeting with representatives of Member States and EU project coordinators of 17 June 2013;
  - Start of the "exploratory phase"
  - Establishment of a core group of interested countries;
- Presentation of the concept to the EU ERIC management committee of 21 June 2013;
- Drafting of an issues paper;

- 1st Meeting of the core group of 11-12 September 2013
  - Three ad hoc groups to prepare synoptic reports on
    - Areas of added value and possible inter-action with existing research infrastrustures of relevance for rare diseases;
    - Mapping of existing projects and initiatives of cross border dimension on rare diseases;
    - Relations with industry.
- First half of 2014:
  - Start of the "design phase" under Horizon 2020;
- 2015:
  - Results of the design study;

Thank you