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**EUROPEAN REFERENCE
NETWORKS
*for Rare Diseases***

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Cette présentation n'engage ni les autorités françaises, ni la Commission européenne

Background

- 2003: High Level Reflection Process on “**Patient Mobility**” & Healthcare developments at EU level
(European Commission / Health Ministers / Civil Society)
Official report of 08/12/03:
 - European Centres of Reference (“ECR”): provide healthcare services for **very specific conditions** requiring a **particular concentration of resources & high level of expertise**, contribute to medical training and research, information dissemination and evaluation
 - Recommendation: the Commission, in collaboration with the Member States, should carry out a mapping exercise relating to Centres of Expertise/Reference (within each Member State) and explore **how to foster networking and cooperation**, incl. the organisation, designation and development of these Centres
- 2004 (April): Commission Communication “*Follow-up of High Level Reflection Process*”
 - Confirmed the importance of working on ECR

High Level Group on Health Services and Medical Care (“HLG”)

- Established in July 2004 by DG SANCO (Chair: R. Madelin)
- Meetings of Senior Officials from Health Ministries (plenary sessions stopped end 2008)
- Establishment of 7 Working Groups (WG)
 - **ECR (2005: European Reference Networks / “ERN”)**
 - Patient Safety (2009: also Quality of Care)
 - Health Professionals (2009: “Green Paper”, 2010: Health Workforce for Health: follow-up Green Paper)
 - Information & E-Health (2006: work completed, c/o DG INFSO: “*E-Health 2010*” WG)
 - Health Technology Assessment (2005: work completed, c/o “EUNetHTA” DG SANCO project, then Joint Action)
 - Cross-border purchasing & provision (2008: work completed)
 - Health Systems Impact Assessment (2008: work completed)
- **HLG annual Reports presented to EU Council (“EPSSCO”)**
(EU Member States Health Ministers)

HLG WG on European Reference Networks (“HLG ERN”) (1)

- Chaired by France (FR Ministry of Health)
- DG SANCO provides secretarial support & strategic input
- Members: Senior Officials from the National Health Ministries
- DG SANCO / DG RTD / DG EMPL Officers (invited on *ad hoc* basis)
- Regular external input expertise: DG SANCO' RDTF / EURORDIS / ERN Pilot Project Leaders
- First meeting: September 2004 (last meeting: February 2010 but previous meeting: September 2008, next meeting...?)
- Adoption of 5 annual HLG Reports (2004 to 2008), incl. a section on **ERN (previously « ECR »: consensual evolution & change of the conceptual approach)**

Publication of HLG annual reports (2004/2005/2006) to be updated on Europa (2007/2008)...

“HLG ERN”

(2004)

(2)

- Agreed objective:

*“To work towards a common approach on the **organisation, designation and development of ECR which could then be implemented through pilot activities, taking into account ongoing activities at national level**”* (2004 HLG Report)

- Questionnaire (elaborated by HLG ERN) on the situation in the 25 Member States
 - result: very diverse situations...

“HLG ERN”

(3)

- **Rare Diseases for “experimental phase”**
- Overall aim: to develop a **general system of European Reference Networks** (could concern other very specific areas than Rare Diseases)
- 2005: Task Force on Rare Diseases (TFRD) was mandated by the HLG ERN to provide a technical and scientific input:
 - 2005: first TFRD Report on European Centres of Reference for RD (incl. mapping on national Centres of Reference and/or Expert Centres for Rare Diseases)
 - 2006: second TFRD Report on European Centres of Reference for Rare Diseases

Agreed general principles

(2005 HLG Report)

“ERN rather than ECR”

(i.e. expertise mobility rather than patient mobility)

- **EU networking of reference/expert centres (or teams) rather than EU isolated centres/physical structures of reference**
- Knowledge & Expertise (professionals, samples, information, etc.) should travel (virtually via e.g. telemedicine or physically) & cross borders rather than patients (still should be possible for patients to travel when really necessary)

NB: - No hierarchy of European Reference Networks over national (or regional) reference/expert centres & networks

- For ultra Rare Diseases and/or ultra rare conditions: no networking possible if/as expertise only exists in one or two States (« solution »: ECR incl. expertise & patient mobility...?)

Criteria to be fulfilled by ERN

(2005 HLG Report) (1)

- Appropriate capacities to diagnose, to follow-up and manage patients with evidence of good outcomes
- Capacity to provide expert advice, diagnosis or confirmation of diagnosis, to produce and adhere to good practice guidelines & to implement outcome measures and quality control
- Demonstration of a multi-disciplinary approach
- Sufficient activity and capacity to provide relevant services and maintain quality of the services provided

Criteria to be fulfilled by ERN

(2005 HLG Report) (2)

- Involvement in epidemiological surveillance, e.g. registries
- High level of expertise and experience documented through publications, grants or honorific positions, teaching and training activities
- Strong contribution to research
- Close links and collaboration with other concerned networks at EU and international levels
- Close links and collaboration with patients associations

NB: This list of criteria could be revised following the outputs coming from the pilot projects on ERN

Recommendation

(2005 HLG Report)

“ERN rather than ECR”

(i.e. expertise mobility rather than patient mobility)

“We recommend that Health Ministers endorse the above general principles, criteria and areas as a working concept for European Reference Networks.

On this basis, we invite the European Commission to test this approach of European Reference Networks in 2006 through one or more pilot projects”

Organisational & governance issues

(2006 HLG Report)

(1)

- Main focus of the work of HLG ERN WG in 2006
- ***“Options for a procedure for identification and development of ERN”***
- These options could be used in order to identify already existing EU potential ERN, to support their development but also in order to support establishing of new ERN
- Whatever proposed option is chosen, the necessary standards of transparency should be ensured throughout the whole process
- **Continuing compliance with criteria and evaluation of ERN** should be ensured

Organisational & governance issues

(2006 HLG Report)

(2)

- **Option 1 – Adaptation of existing EU mechanisms (such as Programme of Community action on Public Health)**
 - + does not require major structural changes
 - + relatively easy to execute in a short-term perspective
 - **very limited in terms of budget and time**
 - **does not guarantee long-term sustainability**
 - does not address related practical, financial and legal issues, which are specific for ERN

Organisational & governance issues

(2006 HLG Report)

(3)

- **Option 2 - New specific EU mechanism for ERN**
(incl. distribution of tasks between National Authorities and a Committee of Member States)
- **Option 3 – New specific “concentrated” procedure at EU level** (a Committee gathering Member States, the Commission, health professionals, patient representatives, etc.)

Both of them:

- + provide long-term sustainability
- + opportunity to address specific practical problems of ERN, including financial and legal issues
- require new specific instrument, so lengthy EU inter-institutional negotiations
- require specific allocation of resources from the Community budget

ERN Pilot Projects

(1)

■ Objective

- **To test feasibility** of the general principles, criteria, areas and processes agreed so far
 - **To see how this approach could be applied in practice** for a specific disease, group of diseases, group of Member States or other focal principles, **building where appropriate on existing teams, centres, expertise or networks**
 - To identify any specific (practical) problems for further consideration
- Supported by the Commission' Public Health Programme (2006 / 2007 / 2008 Call for proposals)
 - **Rare Diseases as priority & experimental area**

ERN Pilot Projects

(2)

- **The Work Plan 2006** for the implementation of the EU Public Health Programme introduced for **the first time as a priority in the area of Rare Diseases:**

“To develop European Networks of Centres of Reference for Rare Diseases” (“ENCR”...)

- According to this priority **10 projects have been selected for funding between 2006 and 2008**

ERN Pilot Project (call 2006)

(3)

- European Centres of Reference Network for **Cystic Fibrosis** ("*ECORN-CF*") (Project Leader: Klinikum der Johann Wolfgang Goethe-Universität / DE)
- European Network of Centres of Reference for **Dysmorphology** ("*DYSCERNE*") (Project Leader: University of Manchester / UK)
- Patient Associations and **Alpha1 International Registry** ("*PAAIR*") (Project Leader: Stichting Alpha1 International Registry / NL)
- European **Porphyria** Network: providing better healthcare for patients and their families ("*EPNET*") (Project Leader: Assistance Publique - Hôpitaux de Paris / FR)
- Establishment of a European Network of **Rare Bleeding Disorders** (Project Leader: Università degli Studi di Milano / IT): not really an ERN pilot project (i.e. epidemiology & database)

ERN Pilot Projects (call 2007)

(4)

- European Network of **Paediatric Hodgkin's Lymphoma** – European-wide organisation of quality controlled treatment
(Project Leader: University of Leipzig / DE)
- European Network of Reference for **Rare Paediatric Neurological Diseases** (“*NEUROPED*”)
(Project Leader: European Network for Research on Alternating Hemiplegia / AT)
- A Reference Network for **Langerhans Cell Histiocytosis** and associated syndrome in EU
(“*EURO-HISTIONET*”)
(Project Leader: Assistance Publique - Hôpitaux de Paris / FR)

ERN Pilot Projects (call 2008) (5)

- Improving Health Care and Social Support for Patients and Family affected by **Severe Genodermatoses** – Together Against Genodermatoses (“TAG”) (Project Leader: Fondation René Touraine / FR)
- European Reference Network of expert centres in **Rare Anaemias** (“ENERCA 3”) (Project Leader: Hospital Clínic de Barcelona / ES)

More details on these 10 DG SANCO Pilot Projects, see Europa:
http://ec.europa.eu/health/ph_threats/non_com/rare_8_en.htm#4

NB: - EURO-WILSON (c/o DG FP7/DG RTD, then c/o DG SANCO call 2010?)

What's next?

(1)

(Easy tasks...)

- **EU Committee of Experts on Rare Diseases (EUCERD), created by Commission Decision of 30 November 2009 should set up a WG on ERN for RD and now replace the HLG ERN WG** and take over its work developed since 2004 (incl. Follow-up of Pilot Projects)
 - > Attend Theme 5 - Session 25 of the Conference!
- **Evaluation Questionnaire established in 2007 by the HLG ERN WG** should be (re)sent to the 10 Pilot Projects (already sent in 2008 to the first 5 Projects selected in 2006, 4 out of 5 projects answered); answers should be analysed in an evaluation report; evaluation report should help to identify strong and weak points for further work

What's next?

(More difficult tasks...)

(2)

- Necessity of a real **legal basis** for ERN: c/o proposal for "the" Directive on "*the application of patients' rights in cross-border healthcare*" (Chapter 4 "*Cooperation on Healthcare*"- Article 13 "*ERN*"), when and if adopted (2010, 2011...?)

But real **concerns & worries about the "new Comitology"** in the framework of the (new) Lisbon Treaty

> Attend Theme 2 - Session 17 of the Conference!

- Necessity of (many) **more ERN financed & financial sustainability for the financed ERN**: dedicated budget line for Rare Diseases (incl. ERN) into the next Community Public Health & Research Programmes 2014-2019?

> "*Money, money, money, always money...*"

“If you had to remember only one slide relating to ERN”

- ERN represent an innovative EU coordinated disposal of cooperation between Member States' health systems, with high European added value, which helps **mutualising expertise & sharing knowledge for highly specialised and rare medical conditions, mainly and *by nature* Rare Diseases** as such, but also rare forms of cancer (incl. paediatric cancer) **or other rare & very specific and complex conditions**
- ERN concept is based on **expertise mobility** (via telemedicine or mobile teams) **rather than patient mobility** (only if really necessary and in a fair manner)
- ERN should provide **timely access to diagnosis and (global) care for all patients** (from all Member States) **with a medical condition requiring a particular concentration of resources and expertise**
- ERN should also **reduce costs** by optimising healthcare resources, contributing to shorter diagnosis delays and decrease waste due to misdiagnosis & prescription, etc.

More information

(check Europa...)

- **HLG on Health Services and Medical Care**, including HLG ERN WG (but do need to be updated...)
http://ec.europa.eu/health/ph_overview/co_operation/mobility/high_level_hsmc_en.htm
- **EU cooperation on Rare Diseases**
http://ec.europa.eu/health/ph_threats/non_com/rare_diseases_en.htm
- **ERN Pilot Projects on Rare Diseases**
http://ec.europa.eu/health/ph_threats/non_com/rare_8_en.htm#4



No aim can be
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