The development of Expert European Networks on Rare Diseases

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PAAIR Network
Networks of experts as a solution

- Cost-effectiveness
- Quality standards, best practices
- Pool research resources
- Critical mass for clinical trials
- Education, training
- Data repositories
- Patients
Criteria for networks of expertise
Art.15 proposal directive cross-border health care

(i) capacities to diagnose, follow-up and manage patients with evidence of good outcomes so far as applicable;

(ii) capacity and activity to provide and maintain relevant services and maintain quality;

(iii) capacity to provide expert advice, diagnosis or confirmation of diagnosis, to produce and adhere to good practice guidelines and to implement outcome measures and quality control;

(iv) demonstrate a multi-disciplinary approach;

(v) provide high level of expertise and experience documented through publications, grants or honorific positions, teaching and training activities;

(vi) provide strong contribution to research;

(vii) be involved in epidemiological surveillance, such as registries;

(viii) have close links and collaboration with other expert centres and networks at national and international level and capacity to network;

(ix) have close links and collaboration with patients associations where such associations exist.
DG SANCO priorities on rare diseases
Pilot reference networks (centres of reference) for rare diseases
Call for proposals 2006

European Porphyria Network: providing better healthcare for patients and their families
Assistance Publique - Hôpitaux de Paris

Establishment of an European Network of Rare Bleeding Disorders
Università degli Studi di Milano

Patient Associations and Alpha1 International Registry
Stichting Alpha1 International Registry

A European Network of Centres of Reference for Dysmorphology
The University of Manchester

European Centres of Reference Network for Cystic Fibrosis
Klinikum der Johann Wolfgang Goethe-Universität
Pilot reference networks for rare diseases

Calls for proposals 2007

Langerhans cell histiocytosis and associated syndrome in EU
Assistance Publique Hôpitaux de Paris

Paediatric Neurological Diseases (NEUROPED)
European Network for Research on Alternating Hemiplegia

European network of paediatric Hodgkin’s lymphoma –
European-wide organisation of quality controlled treatment
University of Leipzig

European Network of Centres of Expertise For CF (Cystic Fibrosis), LAM
(Lymphangioliomyomatosis), and LTX (Lung Transplantation)
Klinikum der Johann Wolfgang Goethe-Universität

European network of expert centres for bipolar disorders
Fondation de Coopération Scientifique en Santé Mentale FondaMental
PAAIR: Patients’ Association and Alpha1 International Registry

- Identify and Map AIR centres (doctors) and patients’ groups
- Common standards of care
- Interaction between national patients’ organizations and national doctors organizations
- Epidemiology (registry)
- Impact of the network (satisfaction, Ph outcomes)

Lung doctors
17 EU countries + 7 nonEU

Patients’ groups
11 EU countries

Liver doctors
11 EU countries + Turkey
**PAAIR:**
Criteria for centres in the network

- Generate data-set of clinical items for AIR database
- Transfer data into web-based database
- Register number of doctor visits/year
- Take Health Status questionnaires
- Store plasma and blood
- Perform spirometry, CO diffusion
- Obtain EBC for biomarker detection in the lungs
- Measure liver function

**American Thoracic Society Documents**

*American Thoracic Society/European Respiratory Society Statement: Standards for the Diagnosis and Management of Individuals with Alpha-1 Antitrypsin Deficiency*

This Joint Statement of the American Thoracic Society and the European Respiratory Society was approved by the ATS Board of Directors, December 2002, and by the ERS Executive Committee, February 2003
Networks are different in organization and objectives

- Research/ diagnostic / clinical / public health
- Single disease / groups of diseases
- Hierarchy between centres of the network
- Patients’ involvement
- Data repository (databases, registries), information exchange
How were the Partners identified and the Network established?

- Established scientific/clinical collaboration
- Pre-existing networks
- Role of scientific societies
- Will in participating/commitment
- Self-sustainability
- Attention to geographical coverage
From Collaboration to Formalization

Networks based on spontaneous collaboration

Networks as formal structures

Outside the Network
- delivery of a working model
- external control
- increased “traffic”
- training/authority

Inside the Network
- governance, management
- financial sustainability
- quality and continuity
Doctors or Managers?

Increased time spent on medical issues

Increased scientific discussion/collaboration

Increased volume correspondence and meetings = more doctor-time spent on non-medical issues

Governance/coordination of the centres of the Network

Quality management

Intellectual property
Financial sustainability

- increased demand increases provision....
- more doctor time costs money.... €
- new projects cost money...
- databases, softwares...
- training of potential centres...

Long term financial sustainability is a problem
Questions?

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