ERA-NET – E-RARE

Dr. Ralph Schuster
PT-DLR: Project Management Agency
Health Research on behalf of
Federal Ministry of Education and Research
Bonn, Germany
PT-DLR: Structural profile

• Non-governmental, non-profit organisation specialised in management of health research funding

• Office located in Bonn: ~ 40 scientific & ~ 40 administrative staff

• Contracting authorities:
  - Federal Ministry of Education and Research (Germany´s health research program)
  - Federal Ministry of Health and Social Security

• funding volume: ~ 200 Mio € p.a.
German National Networks for Rare Diseases

Funding Initiative of the Federal Ministry for Education and Research (BMBF)
Goals of the call

- to support research on diagnosis and therapy of rare diseases
- to coordinate scattered capacities in research and health care into an integrated approach to create structures for an optimal flow of information, systematic research and a competent care of patients
- the networks should include the national top research groups, specialized clinical centres, specialized diagnostic laboratories and patient organizations for selected disease entities or groups of diseases
- the networks could include basic research, clinical research and health care research
- the networks should achieve a critical mass in coverage of patients in Germany
Corner stones of the programme

- out of 55 initial applications, 10 networks are funded

- Funding periods: 1. 2003 – 2006 (3 years)
  2. 2006 – 2008 (2 years)

- Budget:
  - 30 Mio € for 5 years
  - 15 Mio € in the first funding period
German National networks for rare diseases

<table>
<thead>
<tr>
<th>Coordinator</th>
<th>Disease entity</th>
<th>Central office</th>
</tr>
</thead>
<tbody>
<tr>
<td>Traupe</td>
<td>Ichthyosis</td>
<td>Münster</td>
</tr>
<tr>
<td>Bruckner-Tudermann</td>
<td>Epidermolysis bullosa</td>
<td>Freiburg</td>
</tr>
<tr>
<td>Krieg</td>
<td>Systemic Skleroderma</td>
<td>Köln</td>
</tr>
<tr>
<td>Zabel</td>
<td>Skeletal dysplasia</td>
<td>Mainz</td>
</tr>
<tr>
<td>Thyen</td>
<td>Disorders of somatosexual differentiation and intersexuality</td>
<td>Lübeck</td>
</tr>
<tr>
<td>Welte</td>
<td>Congenital Bone Marrow Failure Syndromes</td>
<td>Hannover</td>
</tr>
<tr>
<td>Wendel</td>
<td>Genetic Metabolic Diseases Detectable by Newborn Screening</td>
<td>Düsseldorf</td>
</tr>
<tr>
<td>Lochmüller</td>
<td>Muscular dystrophies</td>
<td>München</td>
</tr>
<tr>
<td>Schulz</td>
<td>Hereditary Movement Disorders</td>
<td>Göttingen</td>
</tr>
<tr>
<td>Gieselmann</td>
<td>Leukodystrophies</td>
<td>Bonn</td>
</tr>
</tbody>
</table>
## Types of projects within the networks

<table>
<thead>
<tr>
<th>Overall number of projects</th>
<th>91</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>built-up of infrastructure and communication</strong></td>
<td>24</td>
</tr>
<tr>
<td>network coordination</td>
<td>10</td>
</tr>
<tr>
<td>databases</td>
<td>13</td>
</tr>
<tr>
<td>tissue and DNA collections</td>
<td>6</td>
</tr>
<tr>
<td><strong>basic studies on the individual diseases</strong></td>
<td>55</td>
</tr>
<tr>
<td>identification of disease genes</td>
<td>17</td>
</tr>
<tr>
<td>genotype-phenotype correlations</td>
<td>16</td>
</tr>
<tr>
<td>biochemical and cell biological experiments on pathogenesis</td>
<td>22</td>
</tr>
<tr>
<td><strong>clinical studies</strong></td>
<td>25</td>
</tr>
<tr>
<td>documenting disease progression</td>
<td>16</td>
</tr>
<tr>
<td>testing diagnostic procedures</td>
<td>7</td>
</tr>
<tr>
<td>therapeutic studies</td>
<td>2</td>
</tr>
</tbody>
</table>
Other rare disease research

• Competence networks for pediatric oncology and for congenital heart defects
• National genome research network: Sarcoidosis, Neuroblastoma, x-chromosomal and mitochondrial diseases
• Immunodeficiencies and clinical trials for rare cancers
National programmes

- **GERMANY:** 10 national networks
  - 5 Mio. € / year for five years since 2003

- **FRANCE:** 59 networks, 27 research projects
  - 4.5 Mio. € / year for three years since 2002
  - GIS-Institut des maladies rares, Paris

- **SPAIN:** 13 networks
  - 6.6 Mio. € / year for three years since 2002
  - Instituto Carlos III, Madrid
ERA-NET Objectives

To step up the cooperation and coordination of national or regional research activities through:

• The **networking of programmes** (parts of programmes) which are strategically planned and nationally/regionally funded

• The **mutual opening** of national/regional research programmes

• Ambitious goals: Joint calls and joint programmes

• Exclusively for public authorities and implementing agencies
ERA-NET may include the following activities to foster coordination and cooperation between programmes:

- Systematic exchange of information & best practices
- Strategic activities
- Implementation of joint activities
- Transnational research activities
E-Rare : Specific Support Action

Objectives:

- Exchange of information (projects, funding mechanisms)
- Develop first strategic and joint activities
- Extend the consortium
- Develop common goals

Prepare a full ERA-Net application (Coordination action)
Partners of Coordination action E-Rare

- France, GIS, Institut de Maladies Rares, Director: Prof. Elisabeth Tournier-Lasserve, Coordination ERA-Net: Dr. Pascale Borensztein
- Germany, PT-DLR
- Spain, Insitut Carlos III
- Belgium, FNRS
- Israel, CSMOH
- Italy, ISS
- The Netherlands, ZonMw
- Turkey, TUBITAK
- Affiliated partners: Russia (MES RF) Region of Lombardia
Management structure

European Commission

Coordinator

Network Coordination Unit

Workpackage and Task leaders

Network Steering Committee

External Advisory Board

High Level Policy Makers
1- To increase the knowledge on programmes and research on rare diseases

- to identify targets for integration
- to develop strategic activities

→ Survey on partners’ programmes
→ Study of programmes in other countries
→ Programme maker information tool on ongoing research projects (collaboration with Orphanet)
2 - Strategic activities between programmes

Objectives:
• to develop research policy on national and European levels
• to shape future programmes
• to design national and transnational calls for research on rare diseases

→ Consultation of experts scientists and organisation of small thematic workshops to identify:
  - specific needs for research on RD
  - new innovative developments and technologies

→ Interaction with various stakeholders : EU, COMP/EMEA, patients organisations, industry, EU task force for rare diseases
3 - Joint activities

Objectives:

• to overcome fragmentation of research in the field of RD
• to promote multidisciplinary approach

→ Mutual opening of programmes for rotational positions to allow clinicians to get involved in basic research:
  - overview of support/training programmes
  - working plan for transnational opening

→ Mutual opening of platforms for collaboration
  - overview of existing infrastructures
  - propositions for organization of access
4 - Transnational research activities

Objectives:
• to develop tools for the opening and collaboration of national research programmes
• to provide information and guidelines to optimize the use of data management systems and biobanks within programmes

→ Funding of transnational research projects
  – Mutual opening of national calls for international cooperation
  – Synchronisation of national calls
  – Transnational calls