



E-Rare: Networking research programmes on rare diseases in Europe

**GIS-Institut des Maladies Rares (Paris, France)
E-Rare Network Coordination Unit**



Support for the coordination of activities

What is E-Rare?

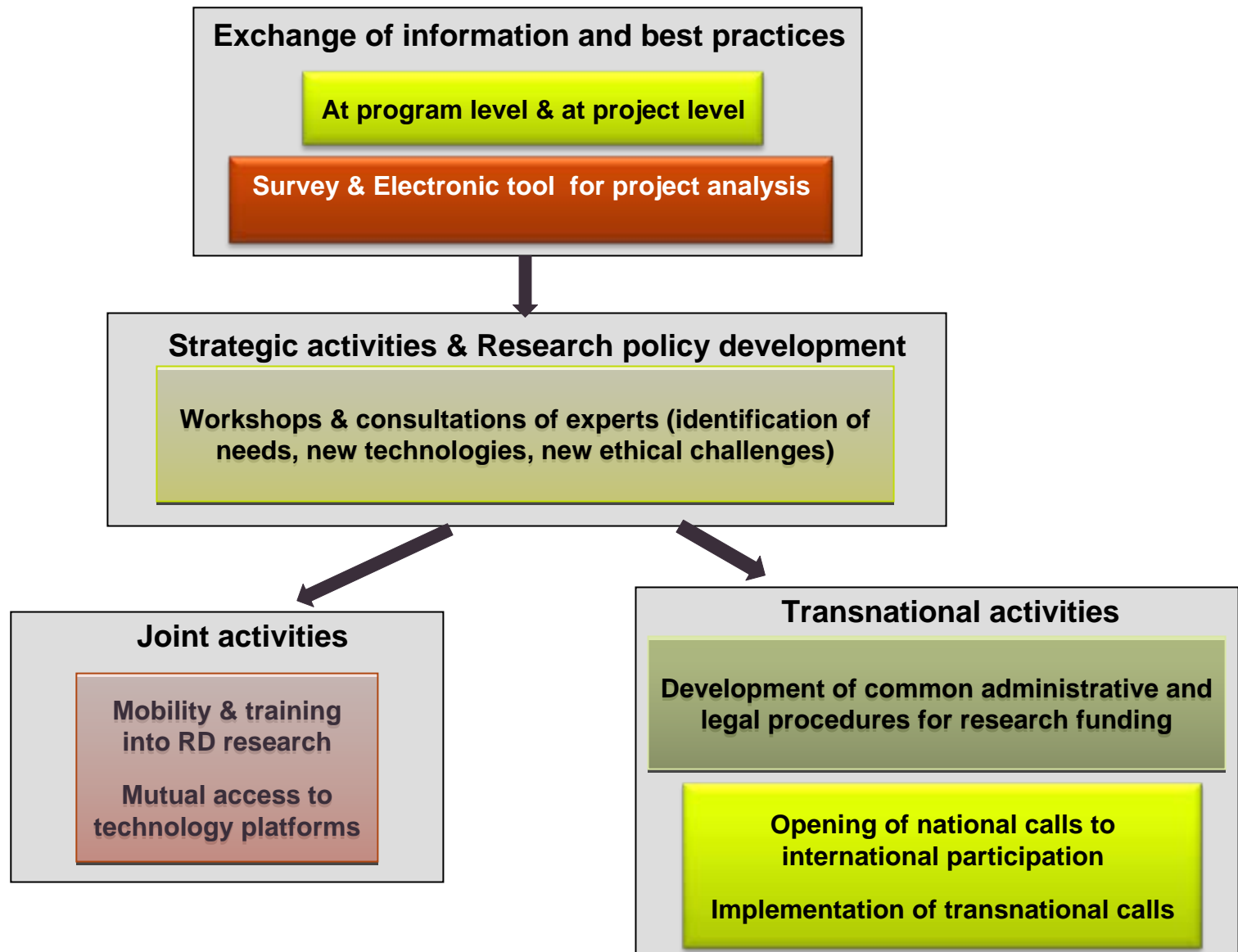
E-Rare: ERA-Net for research programmes on rare diseases

- Project funded by the European Union through FP6 Coordination action, **4 years** (start date: June 2006)
- Objective: coordinate existing national or regional research programmes on RD to:
 - Reduce fragmentation in research
 - Launch multinational joint calls and other joint initiatives
 - Eventually develop joint programmes on RD

E-Rare partners

- **France:** GIS-Institut des Maladies Rares (**coordinator**) & National Research Agency (ANR)
 - **Germany:** Project Management Agency of the German Aerospace Center (PT-DLR) & Federal Ministry for Education and Research (BMBWF)
 - **Spain:** Institute of Health Carlos III/(FIS & IIER)
 - **Belgium:** National Funds for Scientific Research (FNRS)
 - **Italy:** National Institute of Health (ISS)
 - **Israel:** Ministry of Health (CSO-MOH)
 - **Netherlands:** Organisation for Health Research and Development (ZonMw)
 - **Turkey:** Scientific and Technological Research Council (TUBITAK)
- 2 affiliated partners :** Russian Ministry of Science / Region of Lombardy (Italy)

E-Rare roadmap



Systematic exchange of information and best practices

- **Mutual knowledge:** inventory on the state of the art on activities and national programmes

Differences

- Funding in research in RD:
 1. programme within specific RD national plan (France);
 2. priority within a global health research programme (Germany);
 3. priority within a health national plan (Italy, Spain);
 4. Projects embedded within other health research-related programmes (Netherlands, Turkey, Israel.)
- National legal/administrative procedures

Similarities

- Research supported by Governmental Institutions mainly
 - Research priorities: Networking, research from the gene to treatment, health service and social research, epidemiology, diagnostics
 - Evaluation procedures
- **Analysis** of RD projects in Orphanet's database: development of an electronic tool for programme managers specific needs

Strategic activities

- **Setting priorities and recommendations** for RD research policy making

Consultation with leading scientists and clinicians (Rome, January 2007)

- Reinforcement of the major goals put forward by E-Rare project:
 1. to foster transnational networking among researchers and physicians;
 2. to access and share technological platforms
- Organization of meetings on transnational RD research projects:
 1. to obtain a feed-back on the added-values of transnational col.;
 2. to address unmet common needs of researchers in Europe

Workshop on Clinical trials and Natural History of RD (Madrid, April 2008)

- Legal & administrative constraints make difficult the funding of transnational clinical trials on RD
- Importance of including suitable methods to analyze the Natural History of a RD in research projects: should be taken into account when evaluating projects

Joint activities

- **Overcome the fragmentation of RD research in Europe & promote multidisciplinary approaches**

Mutual opening of training positions within partner programs, development of exchange programs:

- Survey of existing training /exchange programs in E-Rare countries: adaptation of those programs to foster exchange of researchers/ clinicians and training into RD

Increase and facilitate the access to technology platforms (across borders)

- Scrutiny of scarce, novel or highly specialized (specific know-how) platforms (high throughput gene dosage and molecule screening, animal models...):
 1. conditions for access;
 2. modus operandi;
 3. if restricted access, measures to facilitate opening

Transnational activities: E-Rare first joint call

- **Preparation:** administrative, legal & evaluation issues: Sept 2006-Jan 2007. Launch of the call: March 2007
- **6 organizing countries:** France, Germany, Israel, Italy, Spain & Turkey
- **Requirements:**
 - potential health impact
 - added-value of transnational collaboration
- **Topics:**
 - Cohorts/case series of patients/families and corresponding collections of biological material
 - Genetic and pathophysiological studies
 - Diagnostic and therapies
 - Social and human sciences (patient oriented), health services research and health economy

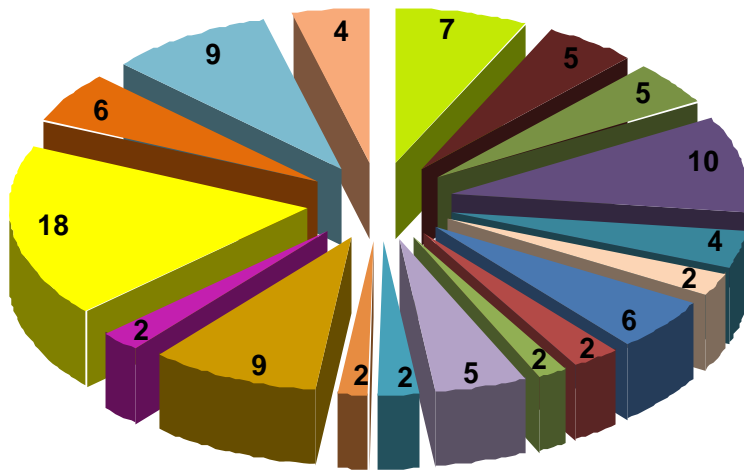
Facts about the E-Rare first transnational call

- **Budget:** over 9 million euros
- **Partners:** Mean of 5 partners/proposal (minimum required 2 of different countries)
- **Proposals:**

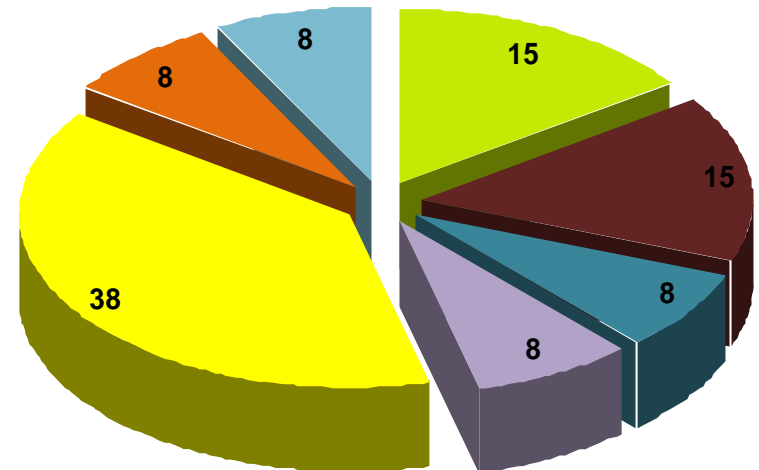
123 received; 18 thematic areas

13 funded; 7 thematic areas

% of projects per thematic area



- **Autoimmune and rare systemic diseases**
- Cardiovascular diseases
- **Dermatological diseases**
- Endocrinological diseases
- Immunodeficiency
- Lung diseases
- Metabolic diseases
- **Neurological diseases**
- Non malignant hematological diseases



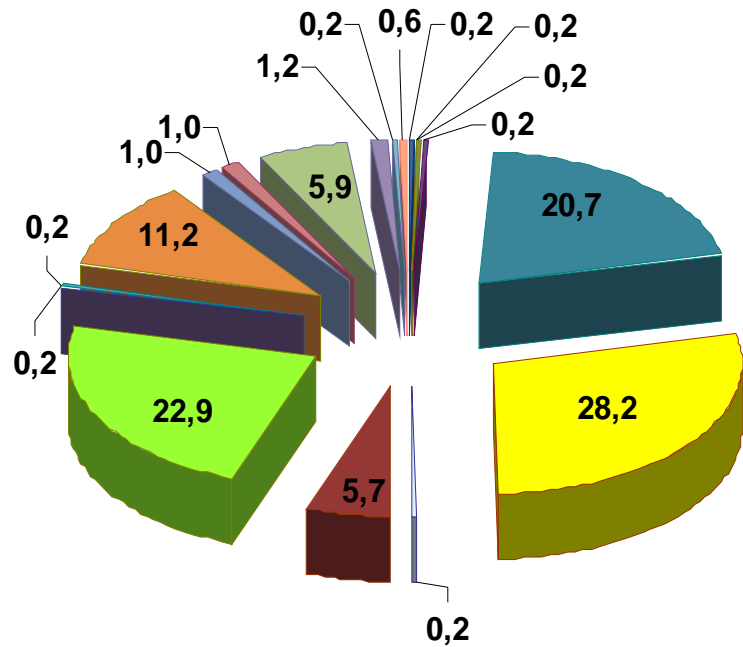
- **Bone diseases**
- Complex syndromes
- Embryonic development abnormalities
- Hepato Gastro Entero diseases
- Kidney diseases
- Lysosomal storage disorders
- Mitochondrial diseases
- Neuromuscular diseases
- Sense organs diseases

E-Rare call: a transnational success

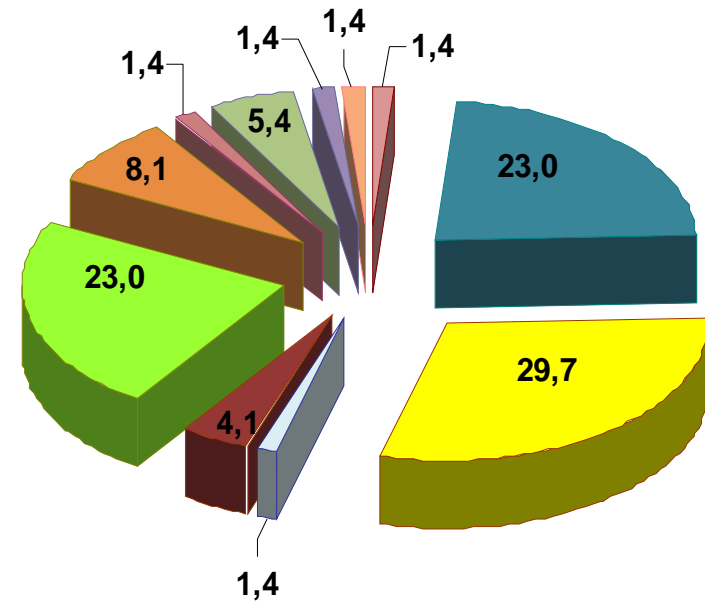
Received proposals

Funded proposals
(76 teams, 3-10 teams/proj.)

% of participant teams per country



- Austria
- China
- Denmark
- Finland
- **France**
- **Germany**
- Greece
- **Israel**
- **Italy**
- Japan
- Portugal
- **Spain**
- Switzerland
- The Netherlands
- **Turkey**
- United Kingdom
- Unites Arab Emirates
- USA



What's next?

- Success of 1st E-Rare call reflects expectations and needs of the RD research community
- Legal and administrative barriers among countries are still significant
- E-Rare will continue its efforts towards:
 - Facilitating access of RD researchers to the best technology platforms regardless their localization
 - Fostering multidisciplinary thorough training and exchange programs
 - Establishing joint programs for rare diseases research

New transnational call for proposals:

late 2008 – early 2009 !!!

www.e-rare.eu



ERA-NET
2006

Coordination action

rare opportunity
for co-operation

“ Coordination of scientific platforms and centres will open the way to networking and to the creation of a real European research programme on rare diseases. ”

The banner features a background of colorful puzzle pieces in shades of blue, green, and orange. A diagonal orange line runs across the image. On the left, the text 'ERA-NET' is written vertically in blue, with '2006' written vertically in white next to it. On the right, the text 'Coordination action' is enclosed in a thin orange border. Below this, the phrase 'rare opportunity for co-operation' is written in a large, white, serif font. At the bottom, a quote in a smaller, italicized font reads: '“ Coordination of scientific platforms and centres will open the way to networking and to the creation of a real European research programme on rare diseases. ”'. The background also includes a close-up photograph of a person's eye being examined with a medical instrument.