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Community research

EU Research policy: Framework Programmes Achievements and Perspectives

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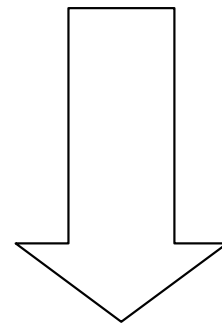
Health Directorate - Medical and Public Health Research Unit





EU historical support to RD research

Directorate General for Research =
European public research funding body



Framework Programme

- complementation of private sector-
 - funded research
 - European value added
- } **RARE DISEASES**





EU historical support to RD research

FP6: PRIORITY 1: Life Sciences, Genomics and Biotechnology for Health

- Four calls for proposals in Priority 1
- Success rate (RD in section 2.1.1): around 33 %
- 59 RD relevant projects selected for funding (including Priority 8, ERA-Net scheme, and Science and Society)
- Global budget for RD: around € 230 million
- <http://cordis.europa.eu/lifescihealth/major/rare-diseases-projects-1.htm>





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**Coordination of
RD research (SSA)**

**Cystic
fibrosis (CA)**

**Planning for early
clinical trials in
Wilson disease
(CA)**

**Genetic skin
disorders (CA)**

**Inborn errors of
metabolism (CA)**

**Neuromuscular
disorders (NoE)**

**Bone and
cartilage
(STREP)**

**Protein
folding
(STREP)**

**RD of plasma
membrane
transporters for
amino-acids
(STREP)**

**RD of
mitochondria
(IP)**

**Lysosomal
disorder:
α-mannosidosis
(STREP)**

**Nuclear
disorders
(2 STREP)**

**Prader-Willi
syndrome
(STREP)**

**Autoimmune
disorders
(3 STREP)**

**Major diseases,
CVD, DB, RD section**



Achievements

Success!

- Mobilisation of top researchers, tackling fragmentation, production of new knowledge
- Coordination of the field at EU level (e.g. OrphanPlatform, ERA-Net)
- Mobilisation of and dialogue with stakeholders (including patients)

...and room for **improvement**:

- clinical research
- focused topics
- emerging consortia/topics

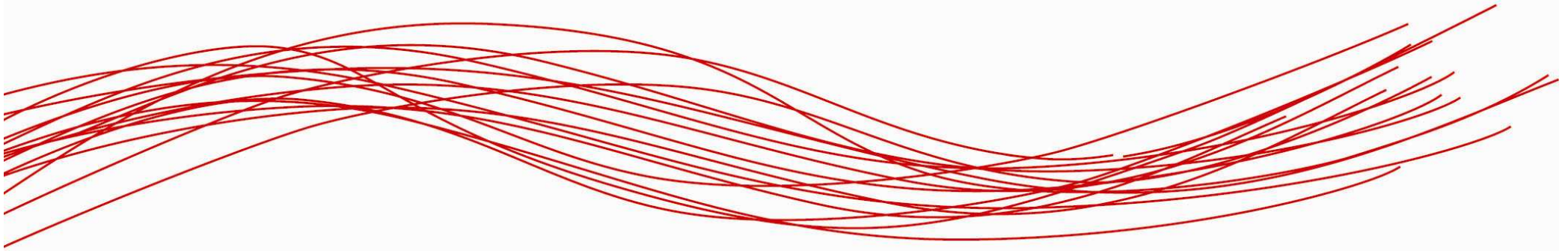




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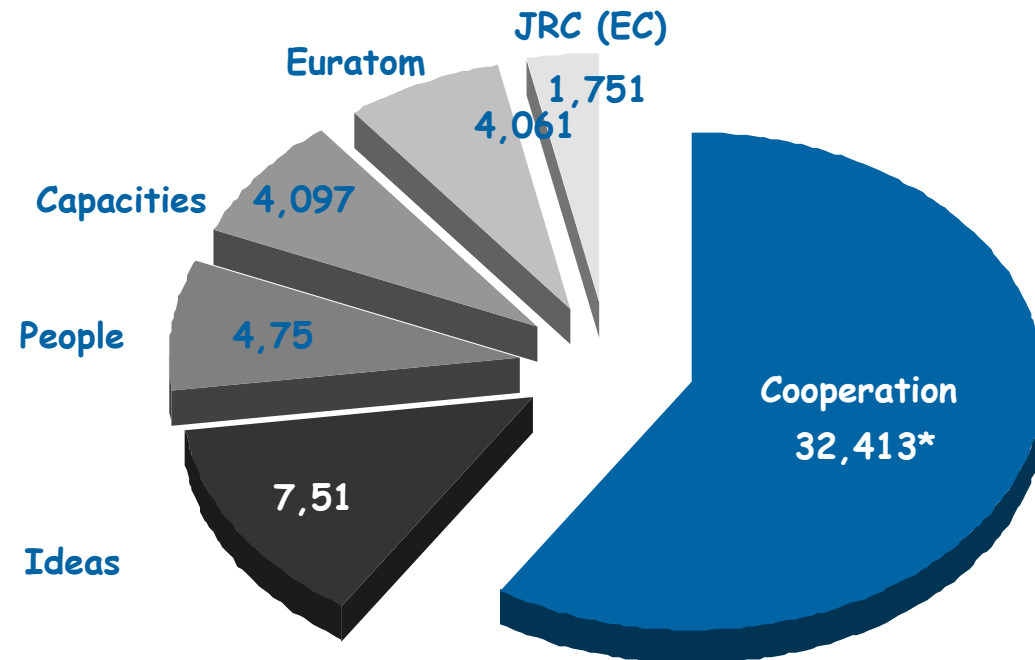
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Building a Europe of Knowledge Seventh Framework Programme 2007-2013



Total FP7 budget: € 54,582 billion

EC Framework programme:
€ 50,521 billion



*of which Health € 6,1 billion





Ten Themes:

Health

Food, agriculture and fisheries, biotechnology

Information and communication technologies

Nanosciences, nanotechnologies, materials
and new production technologies

Energy

Environment (including climate change)

Transport (including aeronautics)

Socio-economic sciences and humanities

Space

Security



Rationale:

Objectives:

- Improving health of European citizens
 - Addressing global health issues including emerging epidemics
 - Increasing competitiveness of European health-related industries
- Sequencing of human genome and recent advances in post-genomics
⇒ to integrate vast amounts of data
 - Translational research
⇒ to bring knowledge into the clinic
 - Clinical research
⇒ international multi-centre trials
 - Reinforce health policy-driven research
⇒ comparison of national policies
 - Strong EU-based biomedical research
⇒ strengthen competitiveness of industries and SMEs



Health: 3 sub-themes

Sub-theme 1:

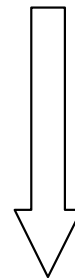
Biotechnology,
generic tools
and medical
technologies
for human health

Sub-theme 2:

Translating research
for human health

Sub-theme 3:

Optimising the
delivery of health
care to European
citizens



Translational research in major diseases:
cancer; cardiovascular disease;
diabetes/obesity; **rare diseases**; other
chronic diseases (e.g. rheumatic, respiratory,
musculo-skeletal diseases and arthritis)

Aim:

identify the future needs of the
rare diseases scientific community

in particular with respect to:

- **types of projects** (collaborative research projects, coordination/networking, new facilities, etc.)
- **size of projects** (limited and/or extended)
- **potential priorities in fields to be covered** (animal models, methods for clinical trials, specific condition/group of diseases, etc.)
- **specific topics?**



Results: recommendations to the EC

Types and size of projects

- collaborative research projects
- coordination/networking
- emerging teams



Results: recommendations to the EC

Priorities: open priorities:

- **infrastructures:** identification of genes and haplotyping, protein pathways, animal models, data management, biobanks
- **natural history** of diseases (throughout Europe)
- mendelian phenotypes of common diseases (including modifier genes)
- **physiopathology** and mechanism of (groups of) diseases (including disease course and modifier genes)
- **pre-clinical and early clinical studies** (including phase 1 and phase 2 clinical trials)
- **therapeutic interventions:** gene therapies, cell therapies, drugs, devices
- **social sciences** (social perception/daily experience/impact of early diagnosis/genetic counselling)

Rare diseases: focus and impact

Focus: The focus will be on Europe-wide studies of natural history, pathophysiology and on development of preventive, diagnostic and therapeutic interventions. This sector will include rare Mendelian phenotypes of common diseases.

Expected impact: This area should help identifying and mobilising the critical mass of expertise in order (i) to shed light on the course and/or mechanisms of rare diseases, or (ii) to test diagnostic, preventive and/or therapeutic approaches, to alleviate the negative impact of the disease on the quality of life of the patients and their families, as appropriate depending on the level of knowledge concerning the specific (group of) disease(s) under study.



Rare diseases: first call for proposals

HEALTH-2007-2.4.4-1: Natural course and pathophysiology of rare diseases.

Collaborative projects (small or medium-scale focused research projects).

HEALTH-2007-2.4.4-2: Research capacity-building in the field of rare diseases.

Coordination and support action (coordination or support action).



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Rare diseases: first call for proposals

Rare Diseases \neq necessary keyword to identify relevant topics in the work programme

Other parts of the Theme: other potential entry points e.g.

High throughput molecular diagnostics in individual patients for genetic diseases with heterogeneous clinical presentation (CP).





Conference on Rare Diseases Research

- "Rare Diseases Research: Building on Success"
- Brussels, 13 September 2007
- Aims:
 - increase visibility of RD research
 - RD community to express their needs
 - Sound basis for future calls for proposals





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Conclusion

Opportunities in FP7 to further strengthen research on rare diseases... which require the active participation of all stakeholders to make FP7 a success!





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THANK YOU
FOR YOUR ATTENTION!

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