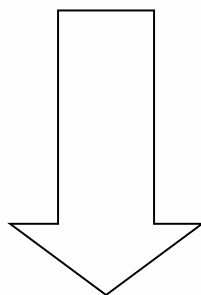


Rare diseases in the 7th EU Framework Programme for Research and Technological Development

**European Commission
Research DG
Berens, Catherine**

RD: history of support in FP

Directorate General for Research =
European public research funding body

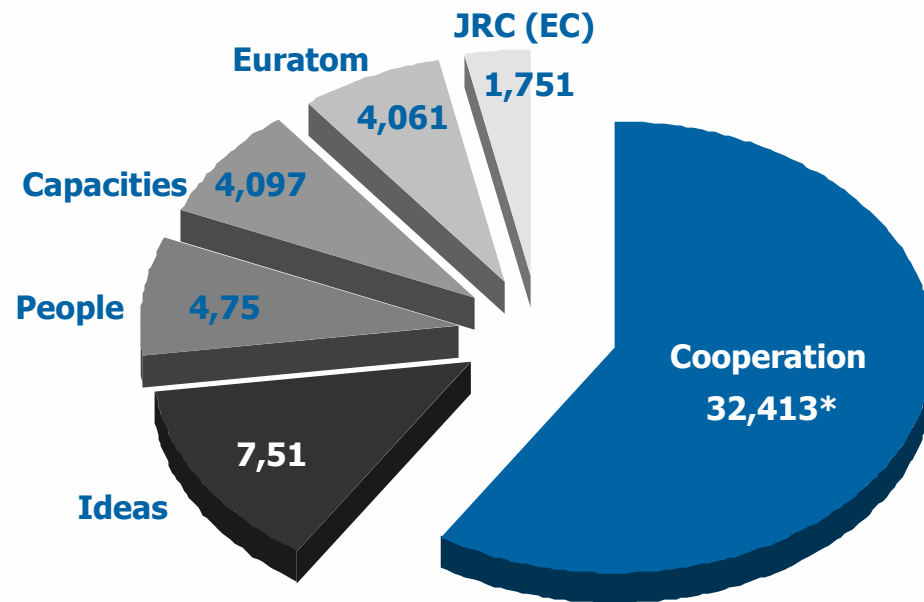


Framework Programme

- European value added
- Complementation of private sector-funded research

**RARE
DISEASES**

Total FP7 budget: € 54,582 billion



***of which Health € 6,1 billion**

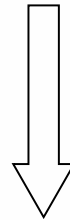
Health theme: 3 sub-themes

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)

- **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.

- **Health Theme:** 3 calls for proposals published
 - ➔ First call: 22/12/06
 - ➔ Second call: 19/06/07
 - ➔ Third call: 03/09/08
- **Rare diseases area:** open in 1st and 3rd calls

Rare diseases: 1st 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; max EC contribution € 3 million).

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

→ 11 projects (10 CP + 1 SA) supported for a global budget of € 30 million

- **Pemphigus** - From autoimmunity to disease
- Natural course, pathophysiology, models for early diagnosis, prevention and innovative treatment of **TNF Receptor Associated Periodic Syndrome** TRAPS with application for all hereditary recurrent fevers
- The Pathophysiology and Natural Course of Patients with **Primary Antibody Deficiencies** (PAD)
- European initiative to improve knowledge, treatment and survival of **haemophagocytic syndromes** in children
- Pathophysiology and natural course of **autoimmune adrenal failure** in Europe
- A European Consortium for **Lysosomal Disorders**

- European IPF Network: Natural course, Pathomechanisms and Novel Treatment Options in **Idiopathic Pulmonary Fibrosis**
- Pathophysiology of rare diseases due to **ciliary dysfunction**: nephronophthisis, Oral-facial-digital type 1 and Bardet-Biedl syndromes
- European Network for the Study of **Orphan Nephropathies**
- Investigation of the molecular pathogenesis and pathophysiology of **Disorders of Sex Development (DSD)**
- A European **Platform of Integrated Information Services for Researchers** in the Field of Rare Diseases and Orphan Drugs Supporting Team and Project Building

Rare diseases: 3rd call for proposals

HEALTH-2009-2.4.4-1: Rare neurological diseases.

Collaborative projects (small or medium-scale focused research projects; max EC contribution € 6 million).

HEALTH-2009-2.4.4-2: Preclinical development of substances with a clear potential as orphan drugs.

Collaborative projects (small or medium-scale focused research projects; max EC contribution € 3 million).

→ 7 projects (5 under 2.4.4-1 and 2 under 2.4.4-2) selected for funding for a global budget of € 34 million – negotiation ongoing

“New” EU Member States’ (“EU 10+2”) participation in area 2.4.4

● 1st call:

- ➔ 6,5 % of partners in submitted proposals
- ➔ 5 % of partners in proposals selected for funding

● 3rd call:

- ➔ 5 % of partners in submitted proposals
- ➔ 1 % of partners in proposals selected for funding

WHY so few?

Potential explanations

- **Lack of research activities in RD?**
- **Lack of interest in participating in your FP?**
- **Lack of information on the possibilities offered?**
- **Difficulties in networking?**

Potential solutions

- **Stimulate networking, both nationally and at European level** (ICRDOD etc.)
- **Stay tuned with FP7 activities** (Cordis website <http://cordis.europa.eu/fp7>)
- **Take full advantage of your National Contact Points** (e.g. 4 Bulgarian Health NCPs; http://cordis.europa.eu/fp7/ncp_en.html)
- **Suggestions on specific FP activities?**

Rare diseases: INDICATIVE 4th call for proposals NOT YET PUBLISHED (expected in July 2009)

- **Clinical development of substances with a clear potential as orphan drugs**
- **ERA-Net on rare diseases**

Rare diseases: future calls for proposals

- **Gaps in research portfolio** (bearing in mind EU value added and EU research potential)
- **Based on**
 - ➔ results from previous calls
 - ➔ contribution to EU policy objectives
 - ➔ priorities identified from discussions with
 - ◆ stakeholders (notably during workshops, conferences)
 - ◆ Health Theme Advisory Group (scientific community representatives, providing independent advice for the implementation of a Theme)
 - ◆ Health Theme Programme Committee (Member States representatives)

Conclusion

Opportunities in FP7 to further strengthen research on rare diseases...

which require the active participation of all stakeholders to make FP7 a success!

Thank you for your attention!

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