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Community Research

Support of the European Union to Research on Rare Diseases

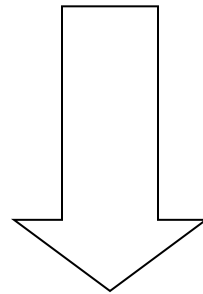
Dr Alain Vanvossel
European Commission
Research Directorate General
Health Directorate - Major Diseases Unit





Rare Diseases: is research needed?

Directorate General for Research =
European public research funding body



Framework Programme

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

**RARE
DISEASES**



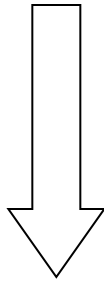


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Rare Diseases: is research needed?

General objectives
of FP - Health:



Specific
objectives of FP:

- improve the **prevention** and **management** of important causes of mortality and ill health in Europe,
- **pool** Europe's research **resources** for tackling (rare) diseases.

Topics mentioned in the **work programmes** of the calls for proposals





EU historical support to RD research

Previous programmes:

Promote cooperation, collaboration and knowledge building

FP5: Total 45 RD projects, € 62 million

- S** • **R&D:** Friedreich's Ataxia; Peroxisomal diseases; Myopathies; Congenital disorders of glycosylation; Juvenile systemic lupus erythematosus; Cystic fibrosis
- U**
- C**
- h** • **INFRA:** European network of DNA, cell and tissue banks on rare diseases
- a**
- S** • **Biotech:** Gene therapy for inherited diseases; Genechip technology for detection of molecularly heterogeneous genetic diseases



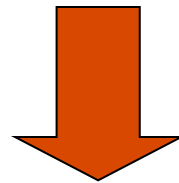


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RD research in FP6

FP6:
Develop scientific and technical excellence



Make a reality of the “European Research Area”, with a view to stepping up innovation in Europe, in conjunction with all the efforts made to this end at regional, national and European level.





RD research in FP6

INTEGRATING EUROPEAN RESEARCH								
THEMATIC PRIORITIES						SPECIFIC ACTIVITIES COVERING A WIDER FIELD OF RESEARCH		
1	2	3	4	5	6	7		
Life sciences, genomics and biotechnology for health	Information society technologies	Nanotechnologies and nanosciences, intelligent mat., new production processes	Aeronautics & space	Food quality and safety	Sustainable development, global change and ecosystems	Citizens & governance in a knowledge-based society	Supporting EU policies	Anticipating scientific and technological needs
							Horizontal research activities involving SMEs	
							Specific measures in support of international cooperation	
							Non-nuclear activities of the Joint Research Centre	

STRUCTURING THE EUROPEAN RESEARCH AREA (ERA)			
Research & Innovation	Human res., mobility	Research infrastruct.	Science and Society

STRENGTHENING THE FOUNDATIONS OF ERA	
Coordination of activities	Development of research/innovation policies



Objectives of Thematic Priority 1

(overall budget in FP6 : € 2255 Million)

Translate genome data into practical applications
to improve human health

How will this be achieved?

Necessity to reach a critical mass, management
simplification and integration of the research
capacities

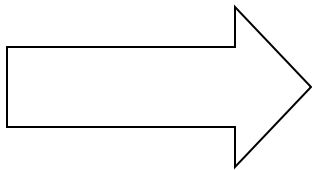
Introduction of new instruments:

Integrated Projects (IP)

and

Networks of Excellence (NoE)

To complement STREP/CA/SSA





PRIORITY 1: Life Sciences, Genomics and Biotechnology for Health

- Three calls for proposals already published
- 26 RD relevant projects selected for funding in the 2 first calls, total budget € 93 million
- Expected global budget for RD: up to € 150 million





“Rare Diseases” - calls overview

1st Call

RD of mitochondria
(*IP*)
RD of plasma
membrane transporters
for amino-acids
(*STREP*)
Network for early
clinical trials in Wilson
disease (*CA*)
Coordination of RD
research (*SSA*)
Rare hereditary
neurological disorders:
ataxias (*IP*)
Rare monogenic
neurological disorders
(*STREP*)

2nd Call

Prader-Willi syndrome
(*STREP*)
Rare autoimmune
disorders (*STREP*)
Rare genetic skin
disorders (*CA*)
Disorders of inborn
errors of metabolism
(*CA*)

3rd Call

RD of nuclear
organisation (*STREP*)
Pharmacogenomics -
orphan medicinal products
(*STREP*)
Cystic fibrosis (*CA*)
Methodological challenges
of early clinical trials in
RD (*CA* or *SSA*)





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

e.g. 3rd call:

- Quality and safety standards for clinical gene transfer - *NoE*.
- Workshop on ethical issues raised by new diagnostic methods - *SSA*.

Rare diseases can serve as models for other diseases!





Rare Diseases (FG + Biotech)

DNA damage and repair mechanisms in health and disease (*IP*)

Functional genomics: study of peroxisomes in health and disease (*IP*)

Low abundance proteomics: cystic fibrosis model (*STREP*)

Genetic testing in Europe (*NoE*)

Molecular diagnostics in mitochondrial diseases (*STREP*)

Retroviral transgenesis in gene therapy of inherited diseases (*IP*)

Improving precision of nucleic acid-based therapy of cystic fibrosis (*STREP*)

Gene transfer for curative therapy of human skin disease (*STREP*)





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4th call for proposals
Emphasis on SMEs!

Indicative topics? (not published yet;
foreseen June 2005)

- protein folding
- rare diseases models
- bone/cartilage diseases
- neuromuscular diseases





Objectives of Priority 8

Underpin the formulation and implementation of
Community policies

One action line devoted to Public Health issues, including RD

EURO-POLICY-PID (*STREP*): Policy-oriented and
harmonising research activities in primary
immunodeficiency diseases (PIDs)

Objectives of Coordination of Activities

Encourage and support initiatives undertaken by several
countries, to develop synergy between their existing activities,
as well as to define and implement joint activities, in areas
such as health, including the RD area.

E-Rare (*SSA*): ERA-Net for research programmes
on RD





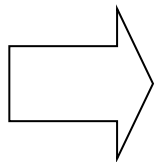
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Towards FP7

Communication: "Science and technology, the key to Europe's future - Guidelines for future European Union policy to support research"
http://europa.eu.int/comm/research/future/index_en.cfm

Open consultation on the future of
European research policy



Preparation of the proposal for the 7th
FRAMEWORK PROGRAMME (FP7)





Six major objectives:

1. **Creating European centres of excellence through collaboration between laboratories**
2. **Stimulating the creativity of basic research through competition between teams at European level**
3. **Launching European technological initiatives**
4. **Improving the coordination of national research programmes**
5. **Making Europe more attractive to the best researchers**
6. **Developing research infrastructures of European interest**





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Towards FP7

OTHER:

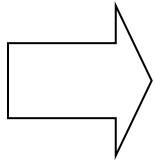
- Enhance european scientific presence in international setting
- Stimulate research for and by SME
- Encourage technology innovation





Research Themes for FP7?

Open consultation on thematic domains for future European support under FP7



Domains where support at European level

- is most needed
- will have the greatest impact

based on:

- European added value
- Contribution to EU policy objectives
- European research potential





Indicative roadmap for FP7

- (Decision on Financial Perspectives)
- **Commission proposal for FP: towards April 2005**
- **Commission proposals for Specific Programmes: towards September 2005**
- **Adoption of FP by European Council and European Parliament: foreseen June 2006**
- **Adoption of Specific Programmes by the European Council: foreseen September 2006**
- **Publication of first calls for proposals: November 2006**





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Towards FP7

Input

- Reflexion workshop April 2005 (DG RTD)
- Received position papers (e.g. EURORDIS)
- Conferences: ICORD, Lux. RD conference June 2005
- Your free input!





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**Cordis website for Priority 1:
<http://www.cordis.lu/lifescihealth/home.html>**

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