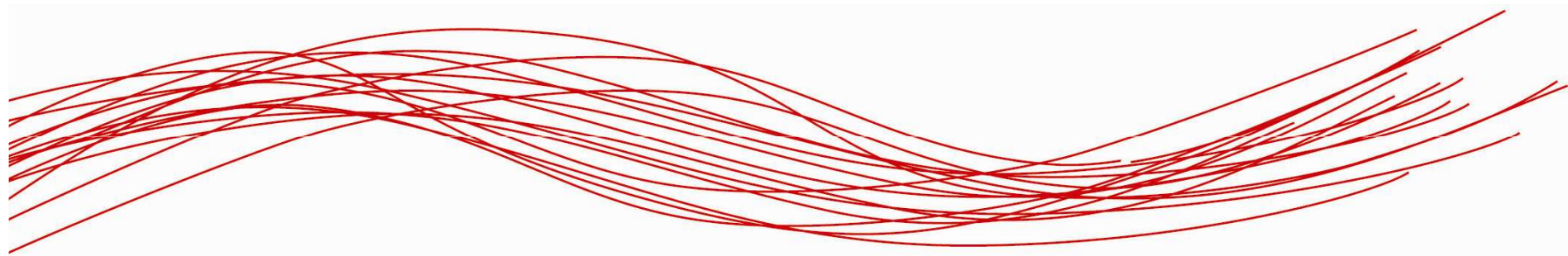


Rare diseases in the 7th EU Framework Programme for Research and Technological Development



Manuel Hallen, MD
Head of Unit
Medical & Public Health Research
DG Research
European Commission



The role of the EC in research

Funding agency

- Supporting research done by multi-national, multi-disciplinary teams in pre-defined areas
- Objective: to strengthen the S+T basis, to increase the competitiveness of industry and raise the international standing of Europe

Preparing + implementing policies

- European Research Area, 3% of GDP spent on R&D, European Patent, etc.

Setting up and supporting new bodies

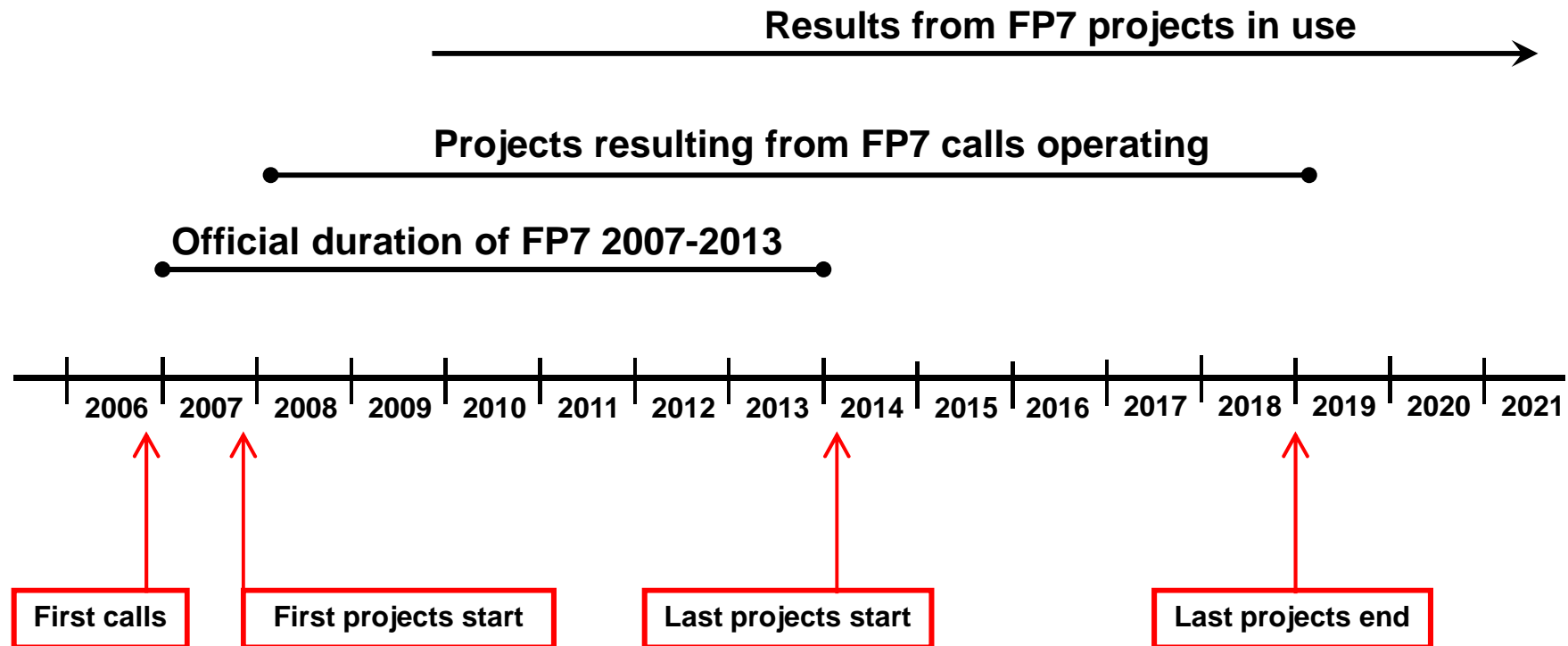
- EMA, ECDC, EDCTP, IMI ...

The role of the EC in research

European Commission programmes bring down barriers

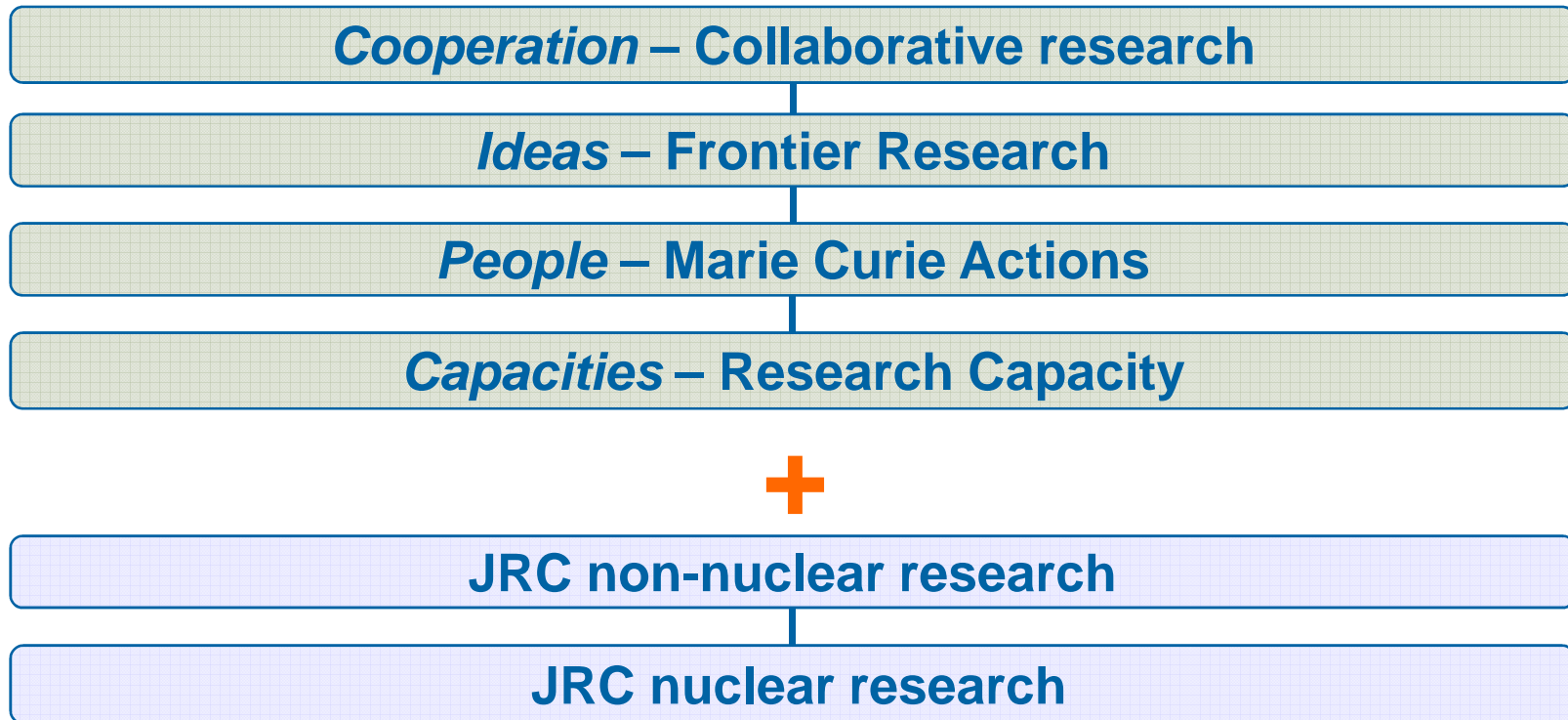
- between countries:
 - ➔ multinational consortia
 - ➔ researchers from any country in the world can participate
 - ➔ coordination among national funding programmes
- between different types of organizations: universities, research centres, SMEs, large companies, etc.
- between disciplines: increased focus on translational research
- and encourage mobility: Marie Curie fellowships available for researchers and for host institutes

Timeframe for FP7 projects

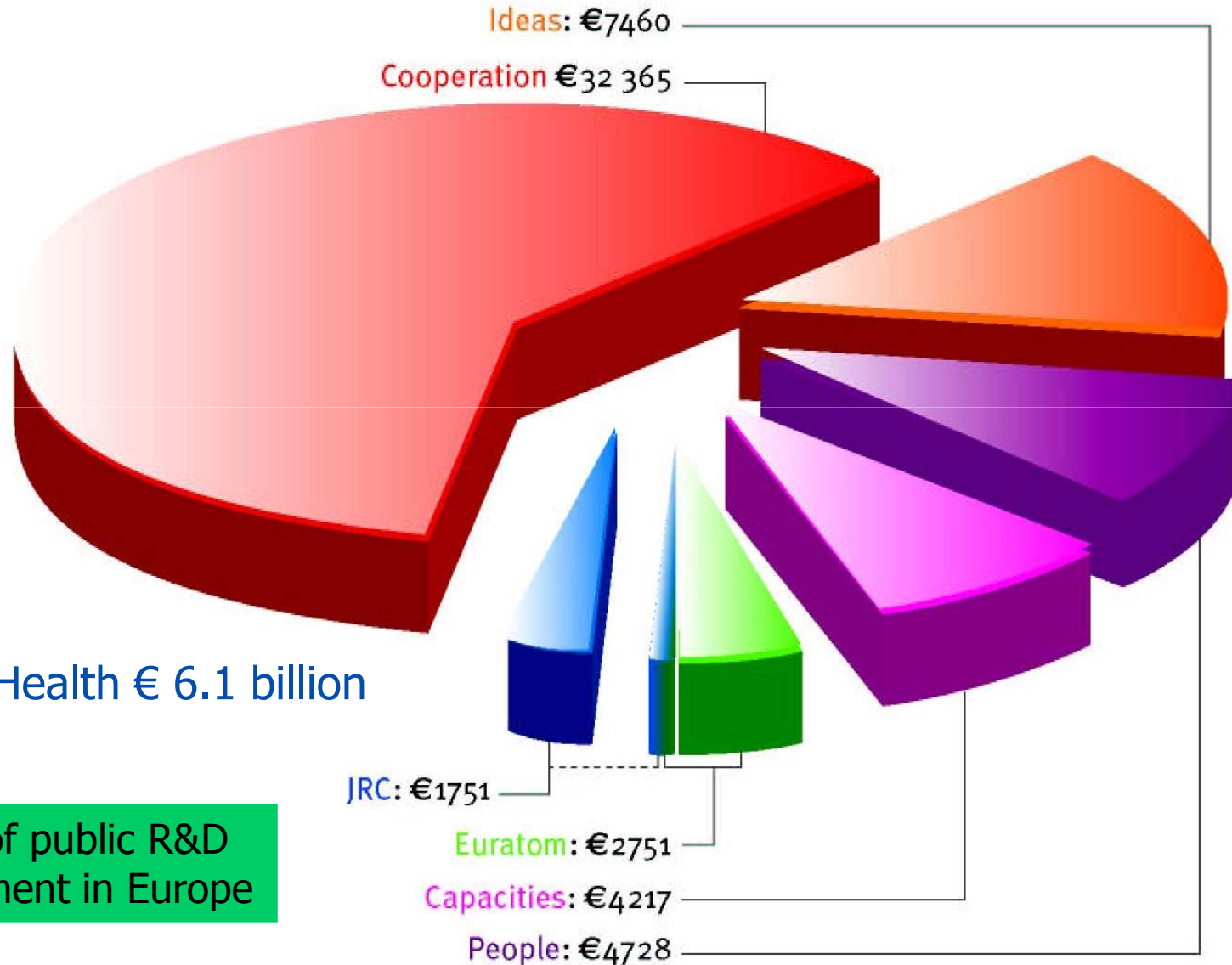




FP7 2007 –2013 Specific Programmes



FP7 budget (2007-2013): € 55 billion*



*of which Health € 6.1 billion

= ~6% of public R&D investment in Europe

Rationale for pan-European approaches

- Sequencing of human genome and recent advances in post-genomics ⇒ **vast amounts of data**
- Translational research (*translation of basic discoveries into clinical applications*) ⇒ **multidisciplinarity**
- Clinical research ⇒ **international multi-centre trials**
- Health policy-driven research ⇒ **comparison of national models & data**

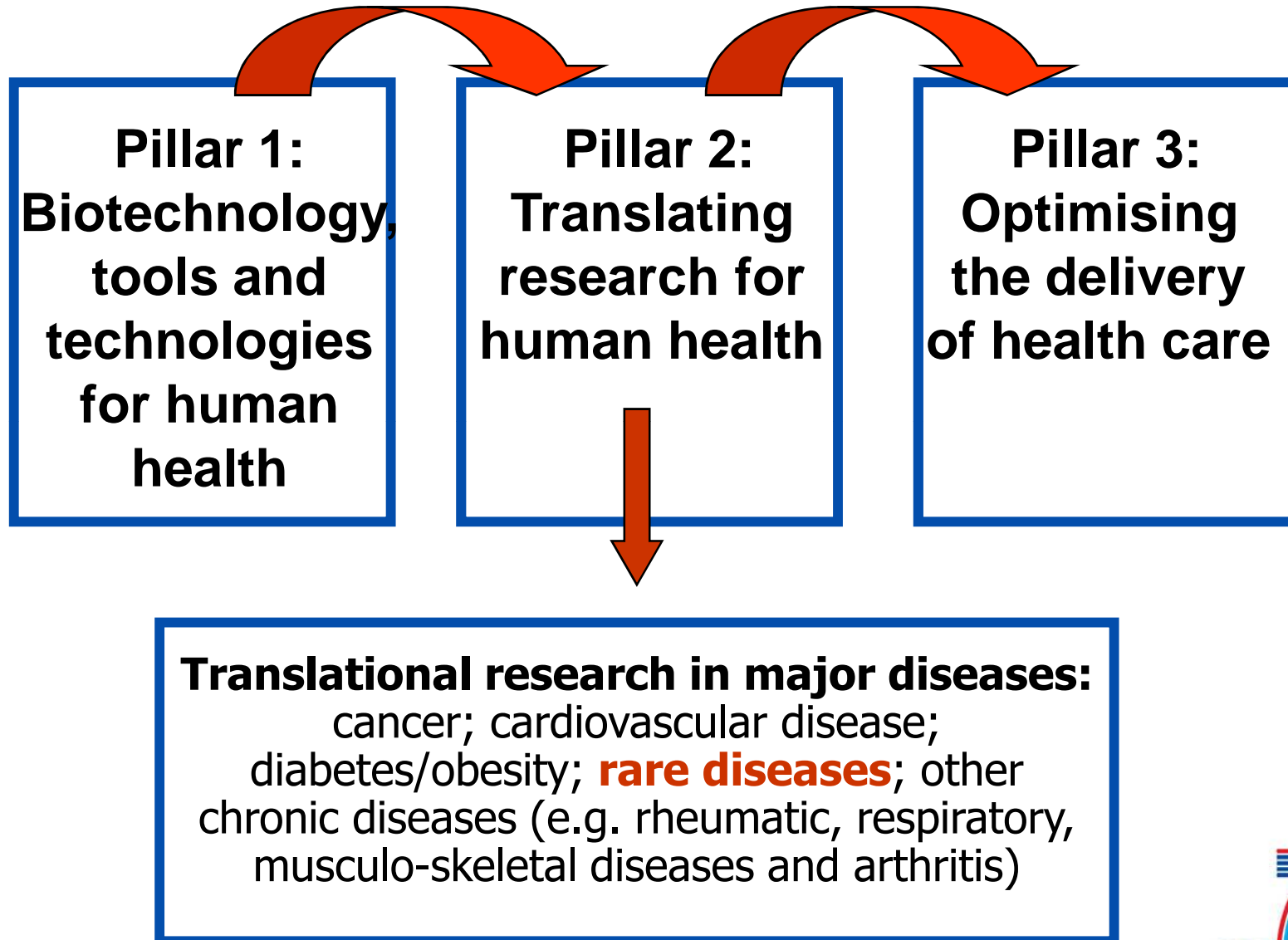
Patients!

Patients!

Rationale for pan-European approaches

- **Strong EU-based biomedical and health research**
 - ▶ to strengthen the **competitiveness of large industries** (pharma) **as well as SMEs** (healthcare biotech & medical technology)
 - ▶ trans-national cooperation is essential to **face worldwide competition**
 - ▶ to develop new **norms & standards** for establishing the legislative framework of new medical technologies (e.g. regenerative medicine), essential for industry

Rare Diseases (RD) in FP7



- **Towards FP7: April 2005 EC workshop**

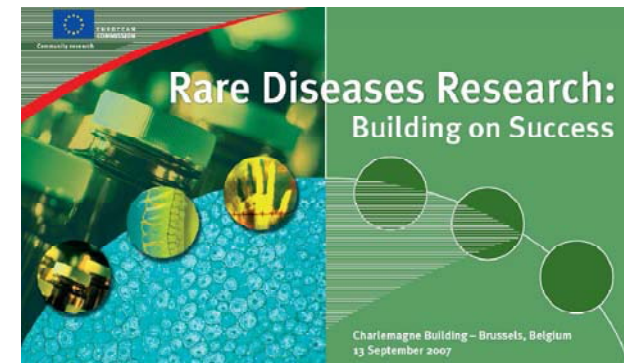
Aim:

- ▶ identify the future needs of the rare diseases scientific community (types/size of projects, potential priorities in fields to be covered, specific topics...)

- **Conference “Rare Diseases Research: Building on Success” - Brussels, September 2007**

Aim:

- ▶ increase visibility of RD research
- ▶ RD community to express their needs
- ▶ Sound basis for future calls for proposals



Focus of Rare Diseases Research in FP7

- Europe-wide studies of their **natural history, pathophysiology**
- Development of **preventative, diagnostic and therapeutic interventions.**
- This sector includes rare Mendelian phenotypes of common diseases.

Expected Impact of Rare Diseases Research in FP7

- This area should help identifying and mobilising the critical mass of expertise in order to:
 - ▶ shed light on the course and/or mechanisms of rare diseases, or
 - ▶ test preventative, diagnostic 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: 1st call for proposals

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

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

→ 11 projects selected for funding for a global budget of € 30 million

Project examples

Natural course and pathophysiology of rare diseases

- **Pemphigus** - From autoimmunity to disease
- 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**
- A European Consortium for **Lysosomal Disorders**
- European IPF Network: natural course, patho-mechanisms and novel treatment options in **Idiopathic Pulmonary Fibrosis**
- European Network for the Study of **Orphan Nephropathies**

Project selected

Research capacity-building in the field of rare diseases

- 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.

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

→ 9 projects selected for funding for a global budget of € 45 million

Project examples

Rare neurological diseases

- Identifying and validating pre-clinical biomarkers for diagnostics and therapeutics of **Neuromuscular Disorders**
- **Myasthenias**, a group of immune mediated neurological diseases: from aetiology to therapy
- Fighting **blindness of Usher syndrome**: diagnosis, pathogenesis and retinal treatment
- Therapeutic challenge in **Leukodystrophies**: Translational and ethical research towards clinical trials
- European Project on **Mendelian forms of Parkinson's Disease**

Project examples

Preclinical development of substances with a clear potential as orphan drugs

- Preclinical study of recombinant human anti-C5 for the treatment of **atypical haemolytic uraemic syndrome**
- Rod-derived **Cone Viability Factor**

Rare diseases: 4th call for proposals

- HEALTH.2010.2.4.4-1: **Clinical development of substances with a clear potential as orphan drugs.** Collaborative projects (small or medium-scale focused research projects; max EC contribution € 6 million).
 - HEALTH.2010.2.4.4-2: **ERA-Net on rare diseases.** Coordinating action (max EC contribution € 2 million).
- **Evaluation January/February 2010**
Start of negotiations May/June 2010



EC money

≠

Easy money

Rare diseases: FP7 future calls for proposals

- Gaps in research portfolio
- Based on
 - ▶ results from previous calls
 - ▶ contribution to EU policy objectives
 - ▶ priorities discussed with
 - ◆ Health Theme Advisory Group (scientific community representatives, providing independent advice for the implementation of a Theme)
 - ◆ Health Theme Programme Committee (Member States representatives)



FP8 ? Research on rare diseases ?

- FP7 will last until end 2013, last projects end in 2018
- Collaborative research on rare diseases?
 - ▶ European value added
 - ▶ Contribution to EU policy objectives
 - ▶ EU research potential: stakeholders' consultation
- Rare diseases community needs to speak with one voice

Information



- EU research:
<http://ec.europa.eu/research>
- Seventh Framework Programme:
<http://ec.europa.eu/research/fp7>
- Information on research programmes and projects: <http://cordis.europa.eu/>

- **research[★]eu**
the magazine of the european research area

<http://ec.europa.eu/research/research-eu/>

- Information requests:
<http://ec.europa.eu/research/enquiries/>



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THANK YOU FOR YOUR ATTENTION

Questions?