Research priorities and Networks

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EU Research Framework Programmes

The largest supra-national fund

- to fund unique international consortia, public-private partnerships and global cooperation
- involves leading players from Europe and across the globe
- to deliver excellent applied research and innovation
- to tackle key challenges which are too big for any one country alone
Connecting Researchers
FP 7 Health research
€6 billion EU investment

- 1,000 projects
- 11,000 teams
- 3,500 organisations
- 130 countries
Research and Innovation

Collaborative research
Real-life solutions based on joint problem-solving

Public-private partnerships
Investing together for next generation medicines and treatments

Global co-operation
One world, one team working to solve common health challenges

Public partnerships
Harnessing global expertise to fight poverty-related diseases
Examples of FP7 funded research contributing with research for better care

- Coordination actions
- Collaborative research projects
- ERA-nets (national and regional research funding bodies co-investment)
- Large scale international research consortia – programme level cooperation at international level

- Disease examples: rare diseases, traumatic brain injuries, cancer
EuroGentest

- Harmonisation, validation and standardisation in genetic testing
- Support professionals in achieving high quality in all aspects of genetic testing services
- Provide information on genetic testing to professionals and to the public
- Promote the implementation of novel technologies into current practice

Coordinator: Gert Matthijs, University of Leuven
RARE-Bestpractices

- create a platform to improve the management of rare disease patients
- promote communication on the management of rare diseases by disseminating trustworthy guidelines globally
- identify and prioritise rare diseases research needs
- facilitate timely, effective and efficient translation of research results into patient oriented strategy on the clinical as well as the public health level, in support to IRDiRC activities.

Coordinator: Domenica Taruscio, ISS, Italy
E-RARE 2 – coordinating European funders and beyond

- Yearly launching of joint calls on rare disease research
- 17 research agencies from 13 European and Associated countries
- Currently over 60 on-going projects
- Current investment of € 45,7 M (2007-2013) with EU investment of around € 2 M

Coordinator: Daria Julkowska, Inserm
200 therapies and means to diagnose most rare disease by 2020

www.irdirc.org
IRDiRC – basic principles

- Co-operation at international level to stimulate, better coordinate & maximise output of rare disease research efforts around the world
- Teams up public and private organisations investing in rare diseases research
- Research funders with relevant programmes >$10 million US over a 5-year period can join & work together
- Each organisation funds research its own way
- Funded projects adhere to a common framework
Committed members

**Europe**
- E-RARE 2 Consortium (EU)
- European Commission
- EURORDIS (EU)
- Academy of Finland
- French Muscular Dystrophy Association
- French National Research Agency
- French Foundation for Rare Diseases
- Children’s New Hospitals Management Group (GE)
- German Federal Ministry of Education and research
- Italian Higher Institute of Health Research
- Italian Telethon Foundation
- Lysogene (FR)
- Netherlands Organisation for Health Research and Development
- Prosensa (NL)
- Spanish Carlos III Health Institute
- UK National Institute for Health Research

**Australia**
- Western Australian Dept. of Health

**Asia**
- BGI (CN)
- Chinese Rare Disease Consortium
- Korea National Institute of Health

**North America**
- Canadian Institutes for Health Research
- Genome Canada
- FDA Orphan Products Grants Program
- Genetic Alliance (US)
- Genzyme (US)
- Isis Pharmaceuticals (US)
- Mendelian Disorders Genome Centres (US NIH)
- National Centre for Translational Sciences (US NIH)
- National Cancer Institute (US NIH)
- National Institute of Neurological Disorders and Stroke (US NIH)
- National Institute of Arthritis and Musculoskeletal and Skin Diseases (US NIH)
- National Institute of Child Health and Human Development (US NIH)
- National Eye Institute (US NIH)
- NKT Therapeutics (US)
- NORD (US)
- Office of Rare Diseases (US NIH)
- PTC Therapeutics (US)
- Sanford Research (US)
- Shire (US)
Going for global health research

- International Human Microbiome Consortium
- Global Alliance for Chronic Diseases
- International K.O. Mouse Consortium
- International Rare Disease Research Consortium
- International Cancer Genomics Consortium
- International Initiative for Traumatic Brain Injury Research
- International Human Epigenome Consortium
Global effort between EC, NINDS (US) and CIHR (CAN)

- **Objective?** To improve clinical outcomes of TBI
- **How?** Through the discovery of causal relationships between treatments and clinically meaningful outcomes
  
  => Global effort for better standardisation and comparative effectiveness research for diagnosis and treatment of TBI

  ✓ Longitudinal observational studies

  ✓ Meta-analysis of individual patient data

- **Expected final deliverable?** Deliver evidence-based clinical guidelines

- **Implementation?** 2 EU projects, 2 US projects, 5 CAN projects
CONnective TIssue CAnsers NETwork in Adults and Children

Epidemiology, clinical registries

Largest sarcoma bb in the world

Various phase I/II-III IDCTs: treatments, care, predictive and prognostic markers

Discontinuation of imatinib in patients with advanced gastrointestinal stromal tumours after 3 years of treatment: an open-label multicentre randomised phase 3 trial

Lancet Oncol 2010; 11: 942-49

Risk of recurrence of gastrointestinal stromal tumour after surgery: an analysis of pooled population-based cohorts


Prognostic CINSARC sarcoma signature
AIMS

- European comprehensive strategy for clinical trial paediatric oncology
- Biology to guide innovative targeted therapy development
- Standardised and innovative methodology for clinical trial design and analysis
- Early evaluation and prioritisation of new anticancer drugs
- Risk adaptation via prognostic biomarkers
- Clinical epidemiology and prospective registries
- Survivorship
- Education and training

EXPECTED OUTCOME

- European-wide structure for paediatric oncology
- Central prioritisation and execution of biology-driven trials
- EU-wide implementation of trial outcome
- EU-wide monitoring of disease outcome and quality of life
Strengthen scientific and technological excellence in research and treatment of leukemias
Promote clinical trials
Prepare guidelines
Spread excellence

- 95 national leukemia study groups
- 102 interdisciplinary partner groups
- 1000 physicians and scientists
- Caring for ten thousands of patients

> 147 centers in 28 countries !!

Recommendations and Guidelines, implemented world-wide
Modelling and predicting sensitivity to targeted therapies in colorectal cancers

- **Determining resistance biomarkers**
  - Molecular profiling of colon cancer patient samples
  - Building and screening of predictive *in vitro* models
  - Screen for genes mediating resistance and sensitivity to targeted therapies in CRCs
  - Building of new algorithms to accelerate the design of rational therapies

- **Design stratified, biomarker-driven phase II trials**
  
  Start funding research project January 2011
  
  Start laboratory experiments May 2011
  
  Validation in animal experiments August 2011
  
  Publication in Nature March 2012
  
  First patient in trial at NKI November 2012
  
  First clinical responses April 2013
  
  CEA measurements
Research and Innovation

Collected intelligence networks

Improved R&D productivity of pharma industries
Innovative approaches for unmet public health needs

**IMI key figures**

*up to call 9*

- 650 Academic & research teams
- 409 EFPIA teams
- 120 SMEs
- 25 patient org.
- 17 regulators
- > 6000 researchers

Collective intelligence networks
What is Horizon 2020?

The EU’s 2014-20 programme for research & innovation

A core part of Europe 2020, Innovation Union & European Research Area

Three priorities:

1. Excellent science
2. Industrial leadership
3. Societal challenges
A new approach

Research and innovation: From bench to bedside

From vertical themes to horizontal challenges

Broad, less prescriptive, 2-year work programme

Stronger focus on end users
The direction

- Translate science into better health outcomes
- A competitive health and care sector
- New health and care models and tools
- Healthy and active ageing

Over €7 billion to health research
Research and Innovation Work Programme 2014/15

- €1.2 billion of EU funding for health research and innovation
- 34 'personalising health and care' topics
- 16 'co-ordination activities' topics
- Two 'inducement prizes'
34 personalising health and care topics

• Understanding health, ageing and disease

• Effective health promotion, disease prevention, preparedness and screening

• Improving diagnosis

• Innovative treatments and technologies

• Advancing active and healthy ageing

• Integrated, sustainable, citizen-centred care

• Improving health information, data exploitation and providing an evidence base for health policies and regulation
Participant Portal – your one-stop shop

http://ec.europa.eu/research/participants/portal
Thank you

www.ec.europa.eu/research/health
www.ec.europa.eu/research/horizon2020