



**200 new therapies and means to diagnose most rare diseases by the year 2020**



**IRDIRC**

INTERNATIONAL  
**RARE DISEASES RESEARCH**  
CONSORTIUM

**Co-operation at international level  
to stimulate, better coordinate & maximise output  
of rare disease research efforts around the world**

# 34 committed members

## Europe

**E-RARE Consortium (EU)**

**European Commission (EU)**

**EURORDIS (EU)**

**French Association against Myopathies (FR)**

**French National Research Agency (FR)**

**German Federal Ministry of Education and research (DE)**

**Italian Higher Institute of Health Research (IT)**

**Italian Telethon Foundation (IT)**

**Lysogene (FR)**

**Netherlands Organisation for Health Research and Development**

**Prosensa (NL)**

**Spanish Carlos III Health Institute (ES)**

**UK National Institute for Health Research (UK)**



## Asia

**BGI (CN)**

**Korea National Institute of Health (KR)**

## Australia

**National Health and Medical Research Council**

**Western Australian Department of Health**

## North America

**Canadian Institutes for Health Research (CA)**

**FDA Orphan Products Grants Program (US)**

**Genome Canada (CA)**

**Genetic Alliance (US)**

**Mendelian Disorders Genome Centres (US)**

**National Centre for Translational Sciences (US)**

**National Cancer Institute (US)**

**National Institute of Neurological Disorders and Stroke (US)**

**National Institute of Arthritis and Musculoskeletal and Skin Diseases (US)**

**National Institute of Child Health and Human Development (US)**

**National Eye Institute (US)**

**NKT Therapeutics (US)**

**NORD (US)**

**Office of Rare Diseases (US)**

**PTC Therapeutics (US)**

**Sanford Research (US)**

**Shire (US)**

# International cooperation: Working on the big challenges

**NEW: Global Research  
Collaboration for Infectious  
Disease Preparedness**

**NEW: 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**

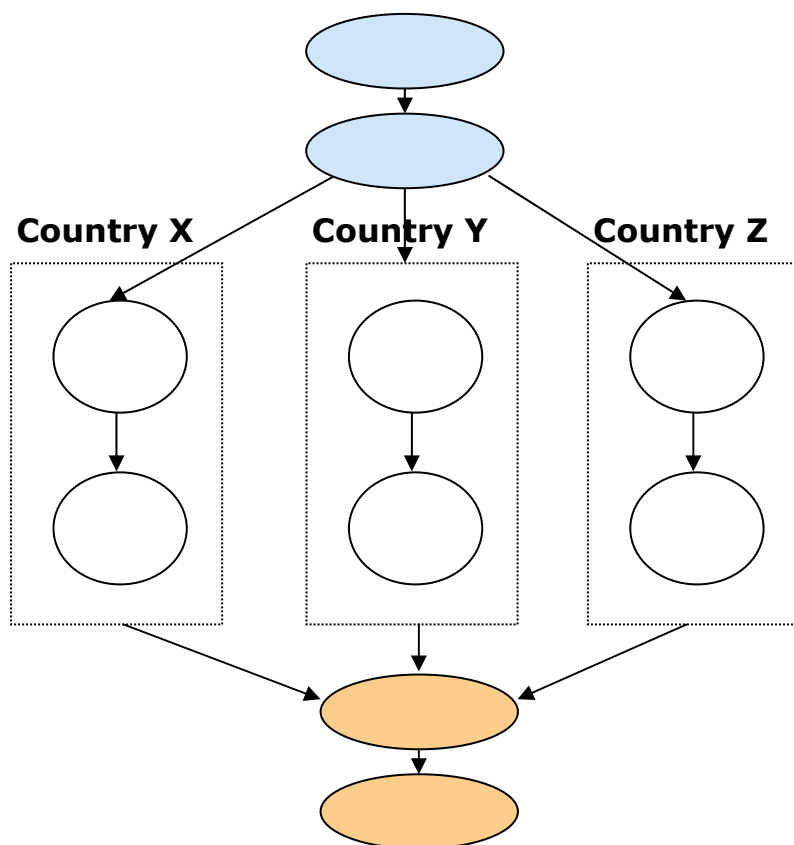
**International Human  
Epigenome Consortium**

**International Human  
Microbiome Consortium**





# 'Programme-level co-operation': how does it work?



Identify common strategic goals

Agree to share tasks (and costs)

Issuing of calls in the cooperating country(ies)

Independent evaluation, selection and funding of projects

Funded projects work closely together

Results, data and standards are being shared



# **Overview of EU research funding**

## **25 September 2013**

**HORIZON 2020**

A graphic for Horizon 2020 featuring a globe with a blue and white pattern, set against a background of blue light rays and a curved horizon line. The word "HORIZON 2020" is written in large, white, sans-serif capital letters across the center.

**Irene Norstedt**  
**Head of Personalised Medicine Unit**  
**Health Research Directorate**  
**DG Research & Innovation**



# R&I in Horizon 2020 (2014-2020)

**DISCLAIMER: Pending formal adoption by the EU of the Horizon 2020 legal texts, the content of these slides is not definitive and so should not be taken as such, nor used or referred to as such in any way or for any purpose**

# The European Union: 500 million people – 28 countries





# Concept



**all EU countries**



**pulling resources**



**into one R&I programme**

Research &  
Innovation





## Europe 2020 – Europe's growth strategy

- **Smart growth**

Better education, more research, greater use of communication technologies

- **Sustainable growth**

A resource - efficient, greener and more competitive economy

- **Inclusive growth**

More and better jobs, investment in skills and training, modernisation of the labour market and welfare systems, spreading the benefits of growth to all parts of the EU

- **Good economic governance**

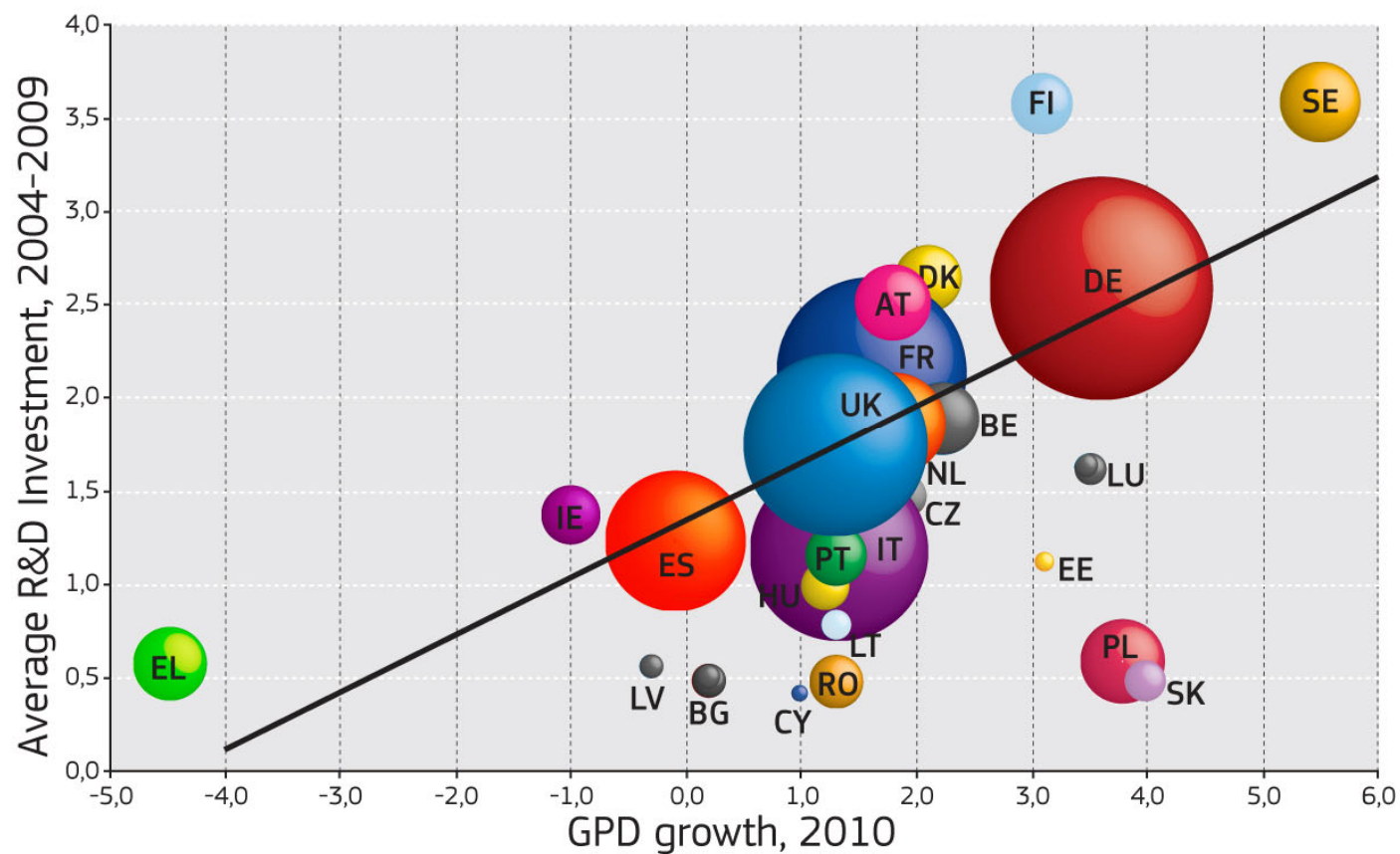
Better coordination of economic policy



## Europe 2020 - 5 EU headline targets

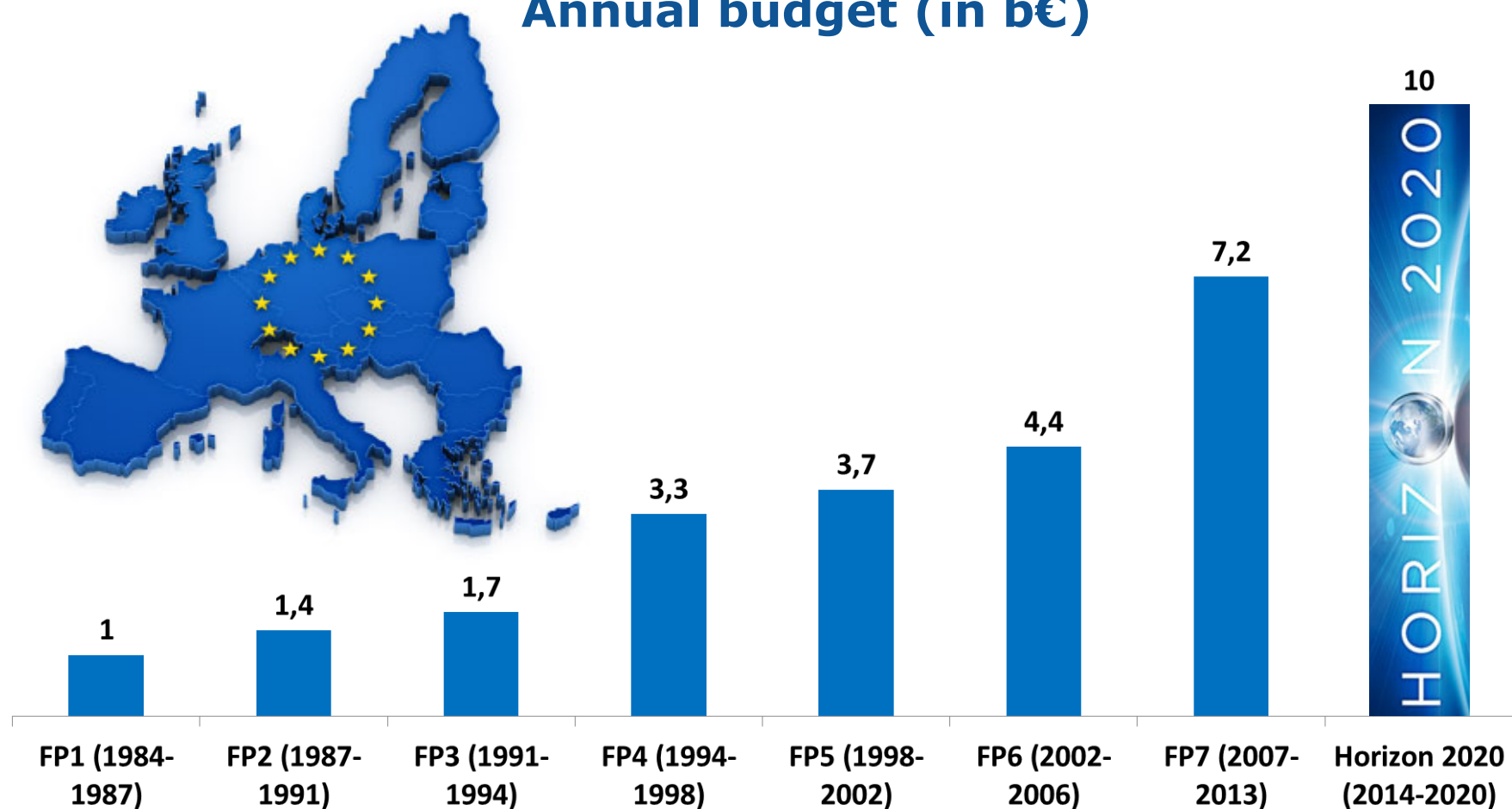
- **75% employment rate (% of population aged 20-64 years)**
- **3% investment in R&D (% of EU's GDP)**
- **"20/20/20" climate/energy targets**
- **School drop-out rates lower than 10% and at least 40% of the population aged 30-34 having completed tertiary education;**
- **Reducing those at risk of poverty or exclusion by at least 20 million**

# Investment in R&D is part of the solution to exit from the economic crises



# 30 years of EU funded R&I

Annual budget (in b€)







European  
Commission

# Horizon 2020

**Europe 2020 priorities**

*International cooperation*

*European Research Area*

**Shared objectives and principles**

**Common rules, toolkit of funding schemes**

## **Societal Challenges**

- Health, demographic change and wellbeing
- Food security, sustainable agriculture, marine and maritime research, and the bio-based economy
- Secure, clean and efficient energy
- Smart, green and integrated transport
- Climate action, resource efficiency and raw materials
- Inclusive, innovative and secure societies

## **Industrial Leadership**

- Leadership in enabling and industrial technologies (ICT, nano, materials, bio, manufacturing, space)
- Access to risk finance
- Innovation in SMEs

## **Excellent Science**

- European Research Council
- Future and Emerging Technologies
- Marie Skłodowska-Curie actions on skills, training and career development
- Research infrastructures

*Simplified access*

*Dissemination & knowledge transfer*

**Supporting the objectives:**

**European Institute for Innovation and Technology  
Joint Research Centre**



# Three priorities:

- 1. Excellent science**
- 2. Industrial leadership**
- 3. Societal challenges**



## **Priority 1. Excellent science**

### **What:**

- **World class science is the foundation of tomorrow's technologies, jobs and wellbeing**
- **Europe needs to develop, attract and retain research talent**
- **Researchers need access to the best infrastructures**



# Priority 1: Excellent science

## What:

### **European Research Council**

Frontier research by the best individual teams

### **Future and Emerging Technologies**

Collaborative research to open new fields of innovation

### **Marie Curie actions**

Opportunities for training and career development

### **Research infrastructures** (including e-infrastructure)

Ensuring access to world-class facilities





## **Priority 2. Industrial leadership**

### **Why:**

- **Strategic investments in key technologies (e.g. advanced manufacturing, micro-electronics) underpin innovation across existing and emerging sectors**
- **Europe needs to attract more private investment in research and innovation**
- **Europe needs more innovative SMEs to create growth and jobs**



## Priority 2 Industrial Leadership

### What:

**Leadership in enabling and industrial technologies** (ICT, nanotechnologies, materials, biotechnology, manufacturing, space)

**Access to risk finance**

Leveraging private finance and venture capital for research and innovation

**Innovation in SMEs**

Fostering all forms of innovation in all types of SMEs



## **Priority 3. Societal challenges**

### **Why:**

- **Concerns of citizens and society/EU policy objectives (climate, environment, energy, transport etc) cannot be achieved without innovation**
- **Breakthrough solutions come from multi-disciplinary collaborations, including social sciences & humanities**
- **Promising solutions need to be tested, demonstrated and scaled up**

## Priority 3 Societal challenges

### What:

Health, demographic change and wellbeing

Food security, sustainable agriculture, marine and maritime research & the bioeconomy

Secure, clean and efficient energy\*

Smart, green and integrated transport

Climate action, resource efficiency and raw materials

Inclusive, innovative and secure societies





# Horizon 2020 How?

- **Collaborative research projects require at least three legal entities from different EU Member States**
- **Activities requiring at least one legal entity**
  - European Research Council
  - Training & mobility action
  - SME instrument
  - Coordination & support actions
- **Calls for proposals open to organizations from countries outside Member States and Associated Countries**



# Horizon 2020 Partnerships

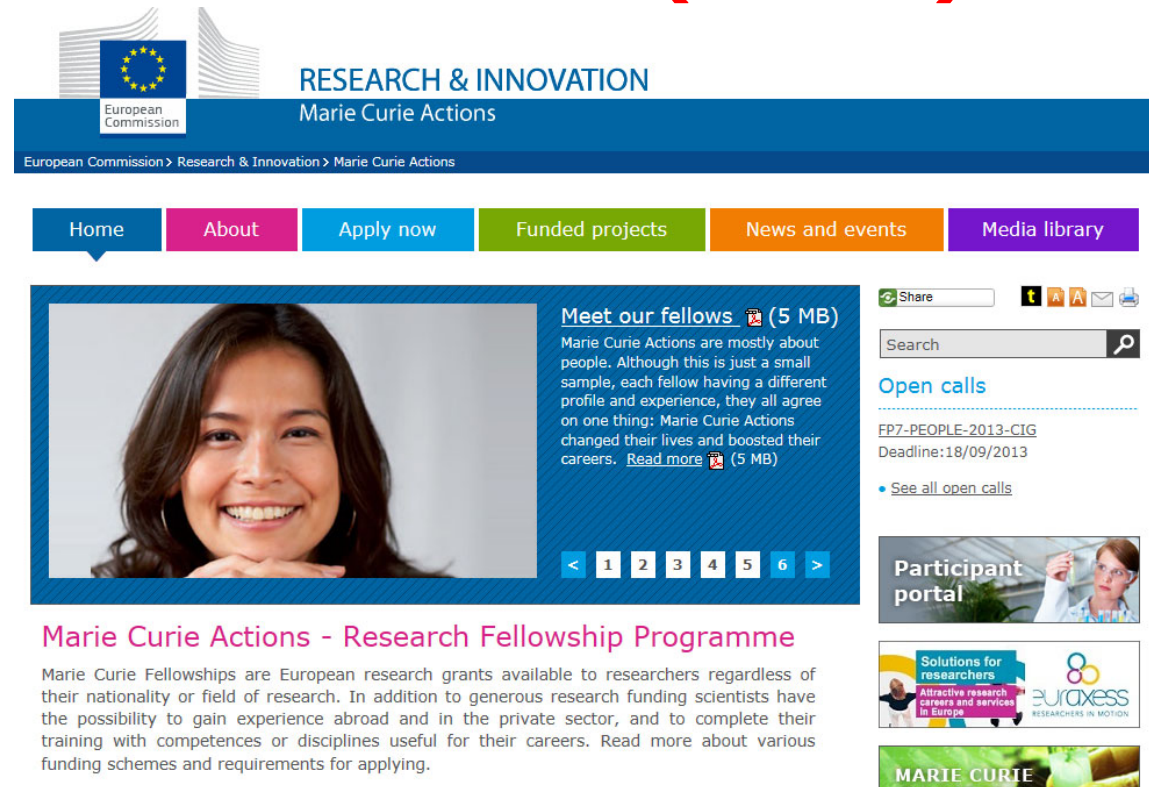
## Innovation Investment Package

**10 Public-public partnerships and Public-private  
partnerships presented -10 July 2013- proposed EU  
contribution 22 Billion Euro**

*Innovative Medicines Initiative 2, Clean Sky 2, Fuel Cells and Hydrogen 2, Bio-based Industries, Electronics (ECSEL), SESAR (European Air Traffic Management System), European and Developing Countries Clinical Trials Partnership (EDCTP2), The European Metrology Programme for Research and Innovation (EMPIR), Eurostars 2 (to stimulate growth and job creation by supporting innovative SMEs), Active and Assisted Living Research and Development Programme*



# Marie Skłodowska-Curie Actions (MSCA)



The screenshot shows the official website for Marie Curie Actions. At the top, there is a blue header with the European Commission logo and the text 'RESEARCH & INNOVATION Marie Curie Actions'. Below this is a navigation bar with links: Home, About, Apply now, Funded projects, News and events, and Media library. The main content area features a large image of a smiling woman, with the text 'Meet our fellows' (5 MB) and a description of the program. To the right of this image is a search bar and a section titled 'Open calls' with a link to 'FP7-PEOPLE-2013-CIG' and a deadline of 18/09/2013. Below the main content area, there is a section titled 'Marie Curie Actions - Research Fellowship Programme' with a paragraph describing the program. On the right side of the page, there are three smaller images: 'Participant portal', 'Solutions for researchers', and 'MARIE CURIE'.

European Commission

RESEARCH & INNOVATION  
Marie Curie Actions

European Commission > Research & Innovation > Marie Curie Actions

Home About Apply now Funded projects News and events Media library

Meet our fellows (5 MB)  
Marie Curie Actions are mostly about people. Although this is just a small sample, each fellow having a different profile and experience, they all agree on one thing: Marie Curie Actions changed their lives and boosted their careers. [Read more](#) (5 MB)

Share Search

Open calls  
FP7-PEOPLE-2013-CIG  
Deadline: 18/09/2013  
[See all open calls](#)

Participant portal

Solutions for researchers  
Attractive research careers and services in Europe  
europe  
RESEARCHERS IN MOTION

MARIE CURIE

Marie Curie Actions - Research Fellowship Programme

Marie Curie Fellowships are European research grants available to researchers regardless of their nationality or field of research. In addition to generous research funding scientists have the possibility to gain experience abroad and in the private sector, and to complete their training with competences or disciplines useful for their careers. Read more about various funding schemes and requirements for applying.

[www.ec.europa.eu/research/mariecurieactions](http://www.ec.europa.eu/research/mariecurieactions)





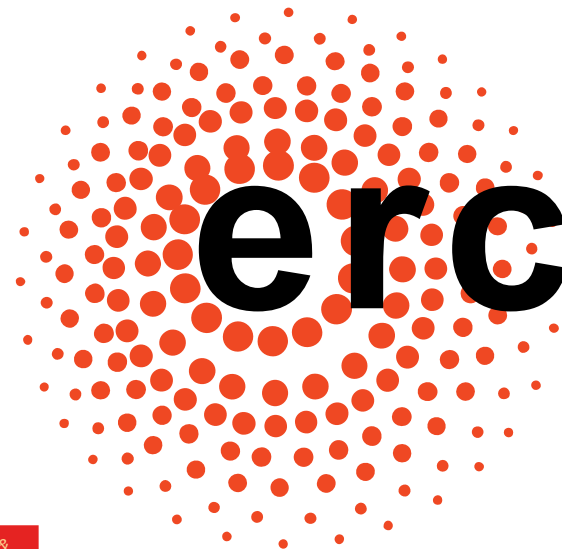
## Training Actions

Innovative Training Networks	<b>ITN</b>	Support for doctoral and early-stage training Training Networks, European Industrial Doctorates, European Joint Doctorates
Individual Fellowships	<b>IF</b>	Support for experienced researchers undertaking international and inter-sector mobility: European Fellowships and Global Fellowships  Dedicated support for career restart and reintegration
Research and Innovation Staff Exchange	<b>RISE</b>	International and inter-sector cooperation through the exchange of staff
Co-funding of programmes	<b>COFUND</b>	Co-funding of regional, national and international programmes: <ul style="list-style-type: none"><li>- doctoral programmes</li><li>- fellowship programmes</li></ul>



## What is ERC?

**The ERC supports excellence in frontier research through a bottom-up, individual-based, pan-European competition**





## ERC - 25 panels for all areas of science

**Physical  
Sciences  
&  
Engineering**

**10 panels**

**Mathematics**

**Fundamental constituents of matter**

**Condensed matter physics**

**Physical and analytical chemical sciences**

**Synthetic chemistry and materials**

**Computer science and informatics**

**Systems and communication engineering**

**Products and processes engineering**

**Universe sciences**

**Earth system science**

**Social Sciences & Humanities**

**6 panels**

**Individuals, institutions and markets**

**Institutions, values, beliefs and**

**behaviour**

**Environment, space and population**

**The Human Mind**

**Cultures and cultural production**

**The study of the human past**

**Life  
Sciences**

**9 panels**

**Molecular and structural biology and biochemistry**

**Genetics, genomics, bioinformatics and systems biology**

**Cellular and developmental biology**

**Physiology, pathophysiology and endocrinology**

**Neurosciences and neural disorders**

**Immunity and infection**

**Diagnostic tools, therapies and public health**

**Evolutionary, population and environmental biology**

**Applied life sciences and biotechnology**



# **EU-funded Health Research**

**Iiro Eerola**  
**Scientific Project Officer**  
**Unit for Personalised Medicine**  
**Directorate for Health Research**  
**DG Research & Innovation**  
**European Commission**





**~ 6,1 billion EUR for  
excellent health  
research & innovation**

# **The FP7 Health Programme**

**The largest multi-national fund**

- **for collaborative research, global consortia, and public-private co-operation**
- **to fund excellent applied health research and innovation**
- **to bring together leading players from Europe and across the globe**
- **to tackle key European and global health challenges**



## State of play

### Key figures

**6** b€ invested to date

**1,000** projects

**11,000** teams

**3,500** organisations

**130** countries

### First outcomes

(on 281 closed projects)

**200** patent applications

**9,000** publications

**3,3** average SJR\* publication

**30** spin-offs created



# EU health research delivers results for patients

- **Treat OA - better treatment & diagnosis of osteoarthritis**
- **EU contribution: €11.9 million**
- **Helped develop TIGENIX' lead product ChondroCelect© for cartilage regeneration in the knee**
- **This product is the first ATMP (Advanced Therapy Medicinal Product) approved in the EU.**

**[www.treatoa.eu](http://www.treatoa.eu)**



## **EU health research concentrates scarce resources**



- **Alpha Man project is developing a promising treatment for patients suffering from the rare disease, alpha-mannosidosis.**
- **Alpha mannosidosis has a prevalence of 1 in 500.000 people**
- **A biotechnologically derived human enzyme has successfully completed a phase 2a clinical trial**
- **Three projects from 2001 to 2013 have led to this stage, with a total EU contribution of ~€10 million**
- **European research has been crucial given the low case numbers per member state and the limited incentive for private investment**

## EU level health research co-ordinates

- ...objectives which can only be achieved at European / global level
- e.g. the Blueprint project – Europe's co-ordinated contribution to the International Human Epigenome Consortium
- Agreed goal of IHEC: generate 1000 epigenomes
- SMEs are embedded in the consortium, paving the way for the next phase of research, drug development.

**Duration:** October 2011 – April 2016

**Budget:** EUR 39.9 million (EU contribution EUR 30 million)

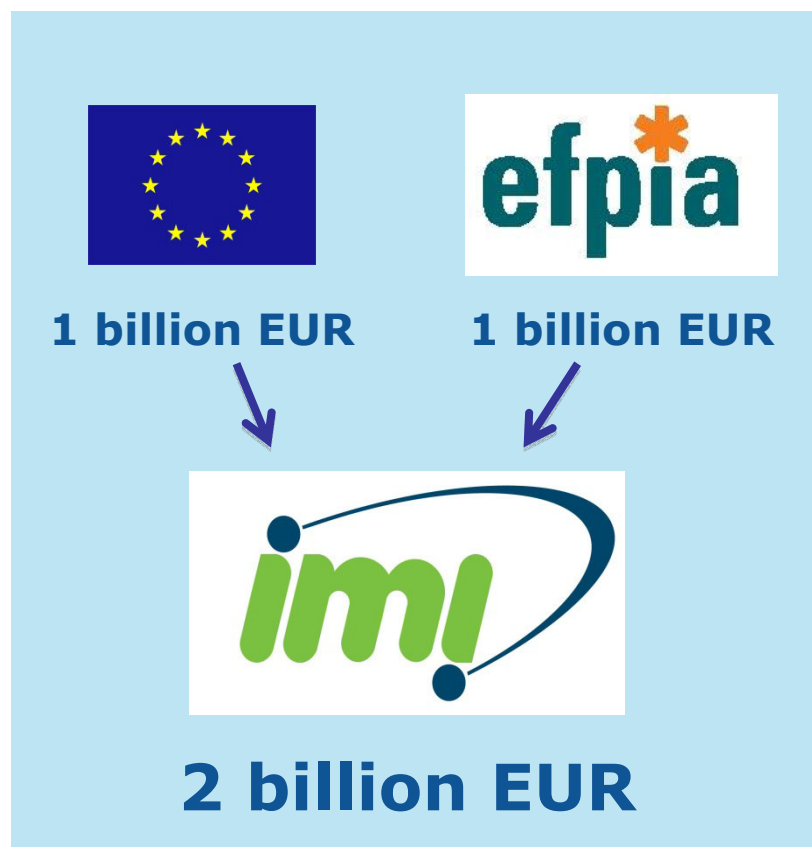
**Partners/countries**

BLUEPRINT involves 41 partners from nine EU Member States, Switzerland and Israel. A full list is available at [www.blueprint-epigenome.eu](http://www.blueprint-epigenome.eu)

**Project website**

[www.blueprint-epigenome.eu](http://www.blueprint-epigenome.eu)

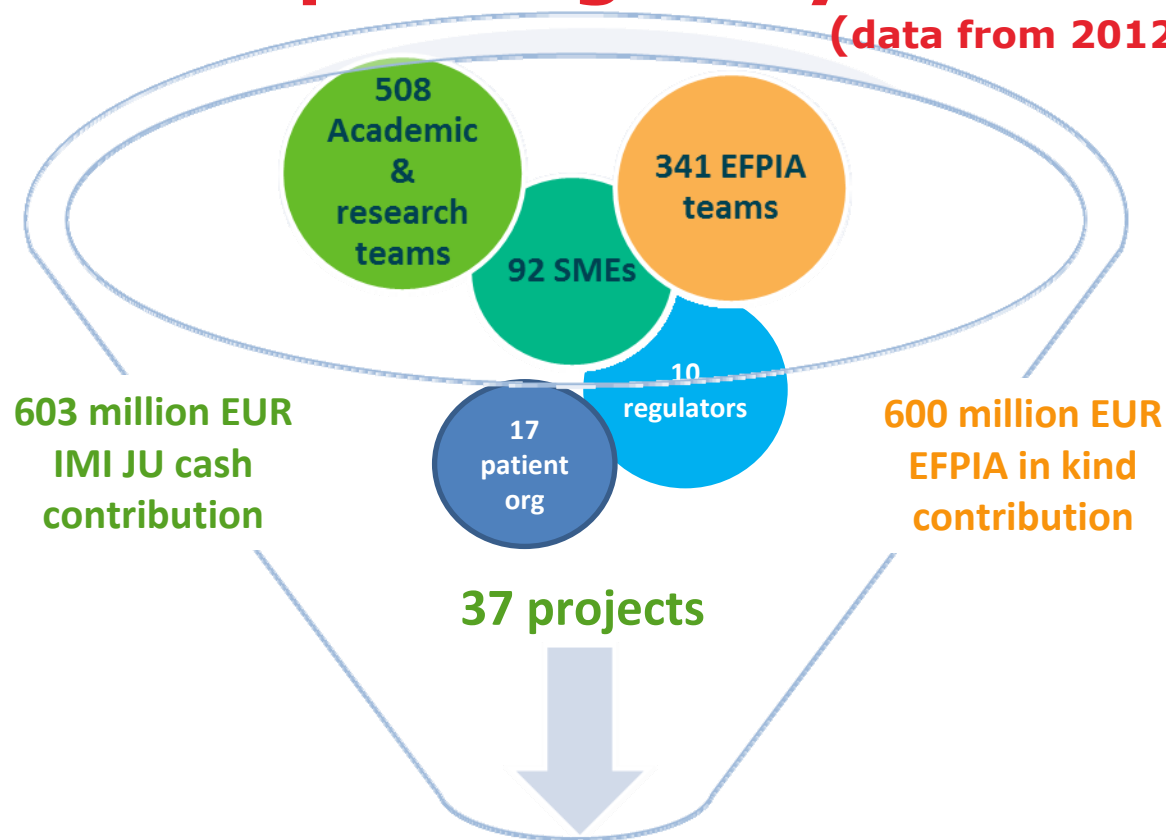
# IMI: Europe's highway to better medicines



- New type of public/private partnership
- 1:1 funding, joint decision making
- All EU funding goes to SMEs, academia, patient organisations and regulatory agencies
- Large pharmaceutical industry, represented by EFPIA, contributes in-kind

# Europe's highway to better medicines

(data from 2012 – currently 40 projects)



## Project example:



- 24 academic partners, 1 SME, 9 EFPIA partners
- 5 years, €38 million
- Started in April 2011



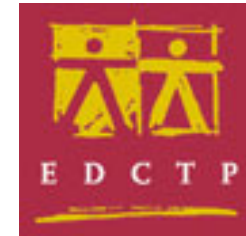


## In the pipeline: IMI2

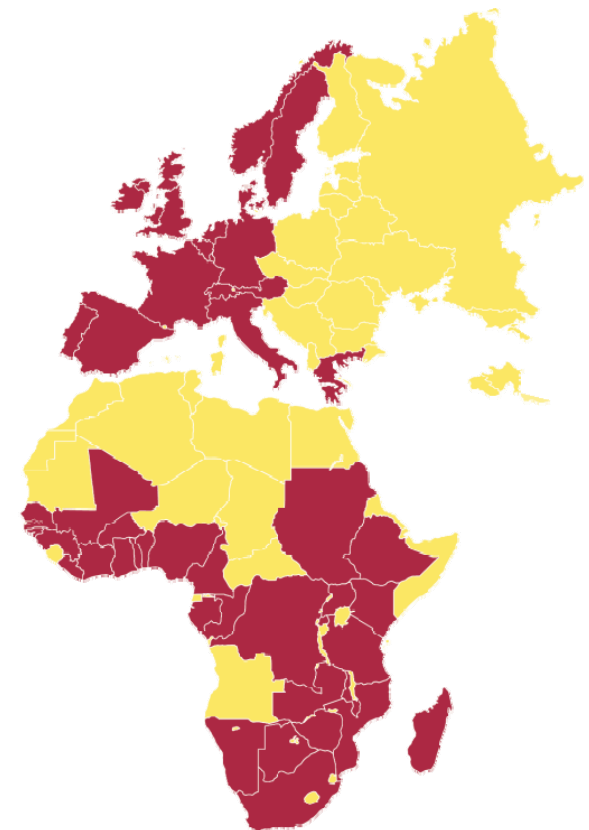
- **Commission proposal published in July**
- **Mission: create an even more powerful research and innovation machine – with broader objectives and a wider range of activities and partners**
- **Key objectives:**
  - **pave the way for the breakthrough vaccines, medicines and treatments which we will need in the near future**
  - **Enable top quality research and innovations with great public health benefits and commercial possibilities**



# European & Developing Countries Clinical Trials Partnership (EDCTP)



- A first in 'Joint Programming' between European national research programmes
- Test-bed to pioneer the coordination, joint implementation and integration of European national research programmes



## EDCTP-funded clinical trials 2003-2011

### HIV/AIDS 27 clinical trials

- **Drugs** 14
- **Vaccines** 9
- **Microbicides** 4

### Tuberculosis 18 clinical trials

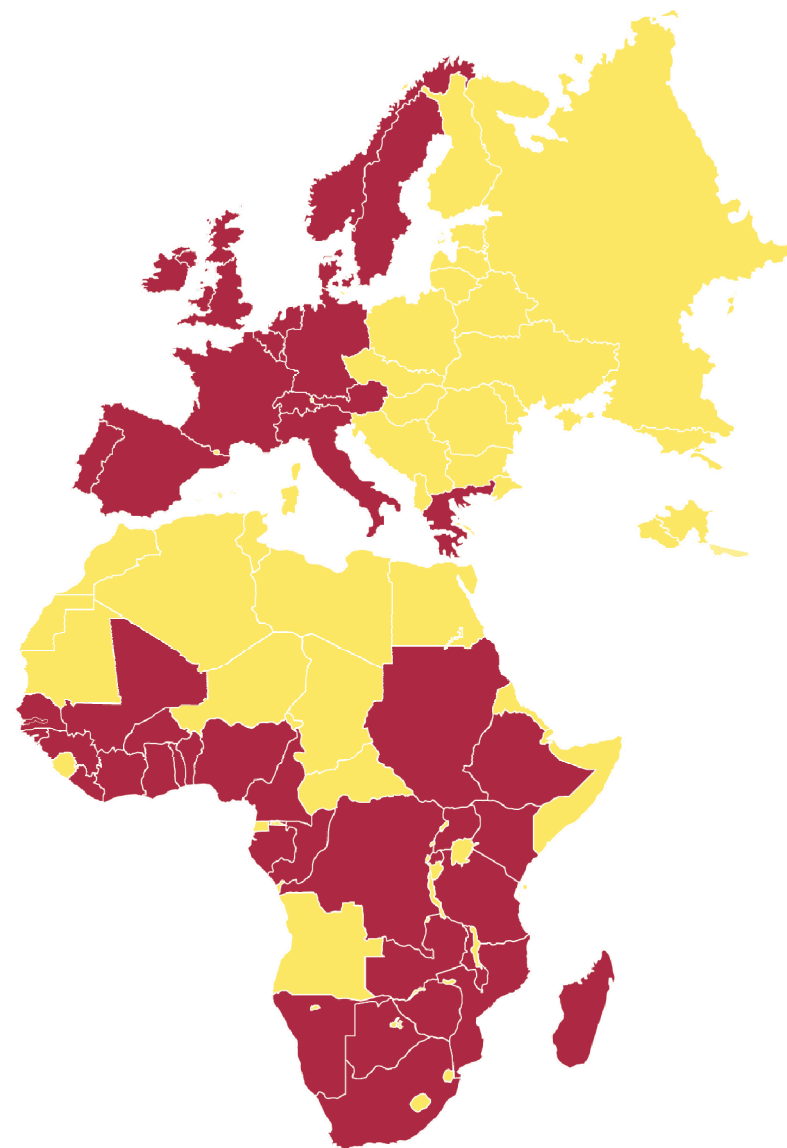
- **Drugs** 7
- **Vaccines** 7
- **Diagnostics** 4

### Malaria 12 clinical trials

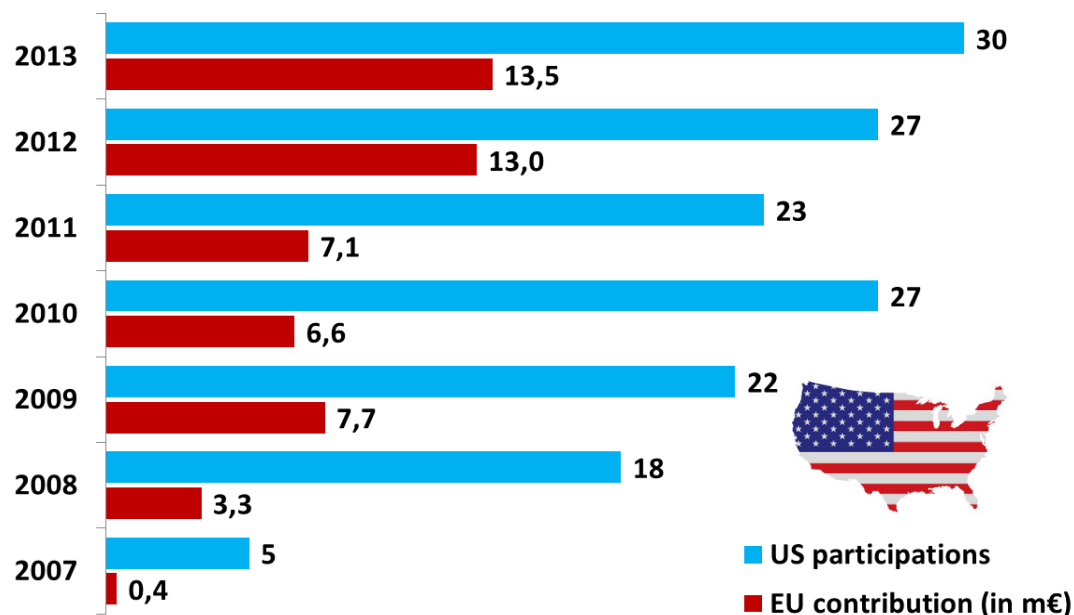
- **Drugs** 10
- **Vaccines** 2

## In the pipeline: EDCTP2

- **EDCTP2 launch in early 2014 expected with:**
  - a) Larger scope (including neglected infectious diseases, all clinical phases, diagnostics and delivery optimisation)
  - b) Bigger budget
  - c) Longer duration
- **16 European countries have committed to spend almost €600 million on EDCTP2**
- **EU will provide matching co-funding through its Horizon 2020 programme**



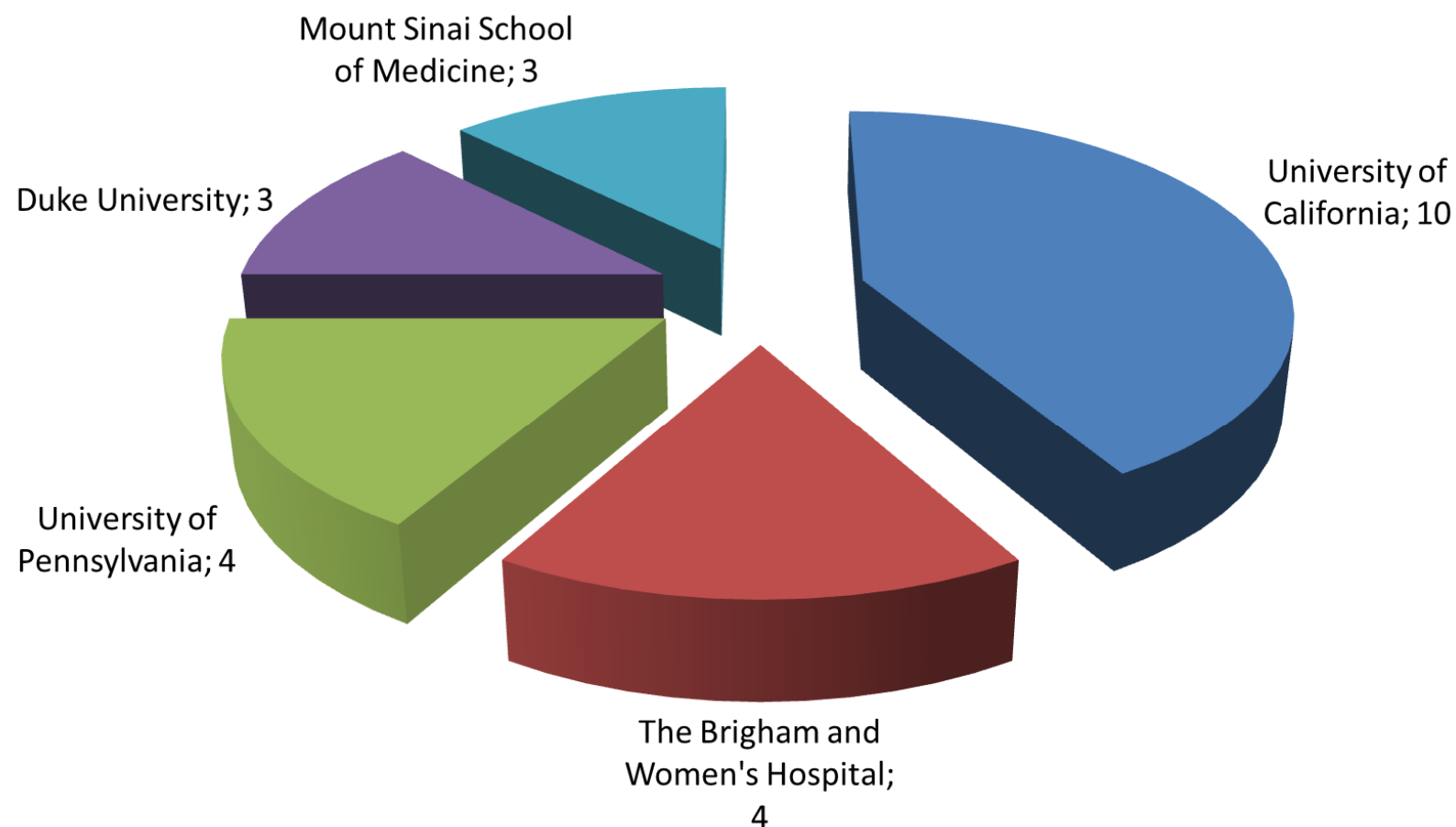
## US organisations in FP7 Health



- **152 US participations:**
  - 63 Universities
  - 39 Research organisations
  - 27 SMEs
  - 13 Foundations
  - 10 Other (International organisations, FDA etc.)

- **Receiving 51 million €**

## 5 main US participating organisations





**US participants in FP7**

## **BESTCILIA: Better Experimental Screening and Treatment for PCD**

- **Primary Ciliary Dyskinesia (PCD) is a rare, genetically heterogeneous disorder causing severe, chronic destructive airway disease with progressive loss of lung function**
- **The main goal of the project is to characterise the clinical course and improve the diagnosis and treatment of PCD patients**

**Participants: DE, CH, CY, DK, EL, NL, PL, UK, US (University of Miami)**

**EU contribution: € 2.9 million**

**[www.bestcilia.eu](http://www.bestcilia.eu)**







**US participants in FP7**

# **IDEA: Dissecting the Immunological Interplay between Poverty Related Diseases and Helminth infections: An African-European Research Initiative**

- **The primary objective of IDEA is to determine whether and how the presence of worm infections modulate immune responses specific to HIV, TB and Malaria, the clinical course of these diseases, and vaccination and vaccine-induced immune responses**

**Participants: 6 EU countries, 5 African countries and the US (Vaccine & Gene Therapy Institute Florida )**

**EU contribution € 10.3 million**

**[www.idearesearch.eu](http://www.idearesearch.eu)**





# Health R&I in Horizon 2020 (2014-2020)



**[DRAFT]**

# Health Challenge

**Understanding health, wellbeing and disease**

**Preventing disease**

**Treating and managing disease**

**Active ageing and self-management of health**

**Methods and data**

**Health care provision and integrated care**

Unravelling the complexity of health and disease

Towards personalised, safe and more effective health interventions

Advancing active and healthy ageing

Improving health information, data exploitation and providing an evidence base for health policies and regulation

Integrated, sustainable, citizen centred care



# How to participate in Horizon 2020



## The basics (based on health programme)

- **1b€/year** devoted to health research in Horizon 2020
- **One rule: Minimum 3 partners from 3 EU countries + anyone else from anywhere in the world**
- **Project duration: 3-5 years**
- **Call publication: planned December 2013**



## **Why should I go for EU funding?**

- 1. Build a strong network with top EU organisations and scientists**
- 2. Share complementary knowledge & experience**
- 3. Receive funding for world-class research**
- 4. Get a first-hand experience with EU businesses and healthcare markets**
- 5. Discover Europe: culture, art, food and wines 😊!**



## How to apply?

- 1. Read the call, identify a relevant topic**
- 2. Find EU partners**
- 3. Write and submit your proposal**
- 4. Get funded (~10% success rate)!**

**<http://ec.europa.eu/research/participants/portal>**





## Find a partner for you project

- **Get in touch with your national contact point:**  
<http://ec.europa.eu/research/participants/portal/page/nationalcontactpoint>
- **Fit for Health:**  
<http://www.fitforhealth.eu>
- **Health competence:**  
<http://www.healthcompetence.eu>
- **IMI partnering platform:**  
<http://www.imi-partnering.eu>

## We need your expertise

- Register as an expert
- ... or make sure that your profile is up to date

Join the database of independent experts for European research and innovation

The European Commission appoints independent experts to assist with research and innovation assignments including the evaluation of proposals, the review of projects and the monitoring of programmes or policies.



<http://ec.europa.eu/research/participants/portal/page/welcomeexpert>



# Thank you!

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**<http://ec.europa.eu/research/health>**