Clinical Trials and Personalised Medicine - Commission's view

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HORIZON 2020

ESF Workshop on Health Research Strategic Needs in Europ
Brussels, 13 March 2015

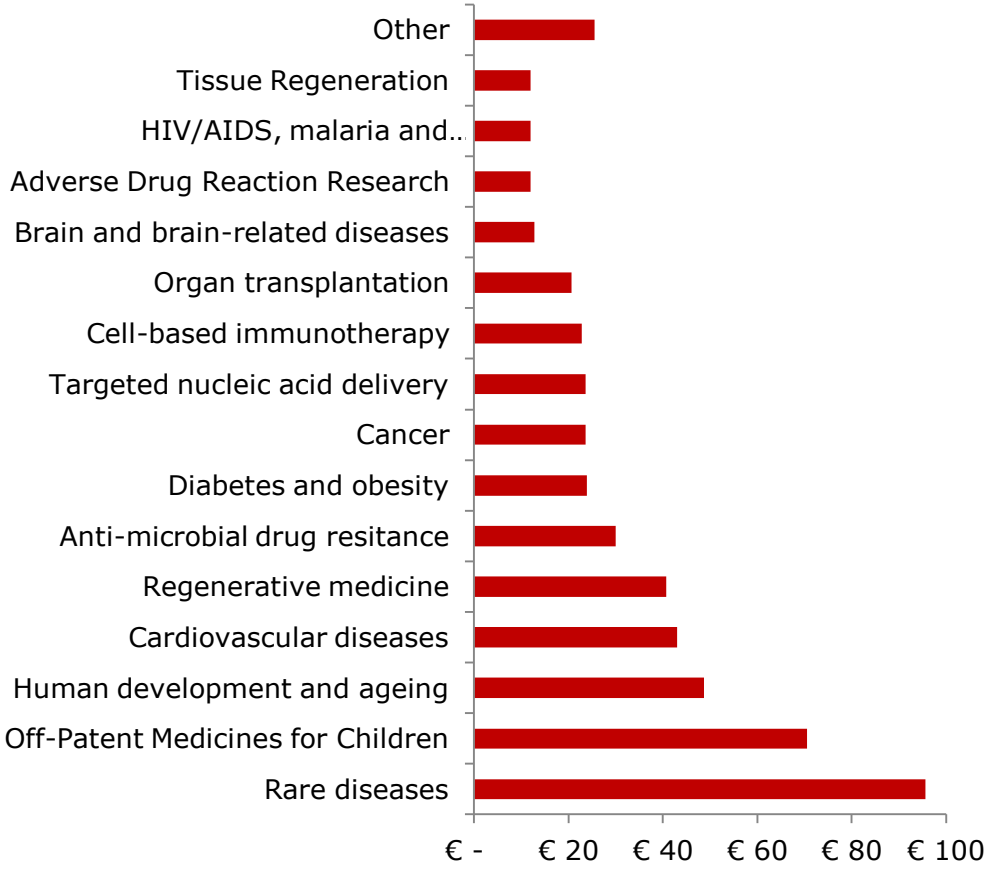
Clinical Trials in the age of 'personalised medicine'

- 'Molecular taxonomy' → Patient stratification → smaller (sub)groups
- Challenges to clinical trial design (adaptive?, enrichment?, Bayesian approaches?, well-conducted observational studies?)
- Close links to –omics/biobanks required
- **Advantages of European approach:**
  - larger recruitment base (for smaller subgroups)
  - ↑efficiency through common platforms
  - professionalisation

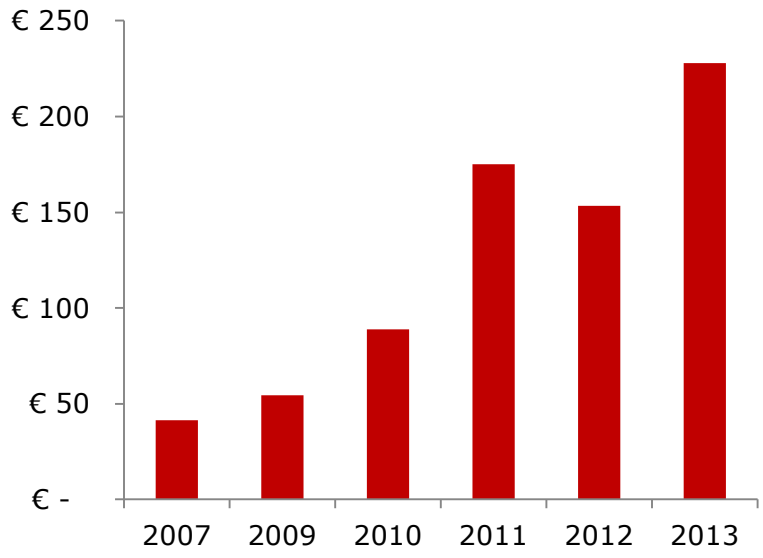
EU-funded Clinical Trials – from FP7 to Horizon 2020

- FP7: Clinical trials in more than 160 projects with >900 M € EU funding!
- Strong emphasis on 'investigator-initiated' CT
- Diversity in scope, methodology, nature of the intervention, disease area and target group:
- From *'Phase I trial of cell therapy of knee osteoarthritis'* to *'Cluster-randomised trial of isolation measures for the prevention of hospital-acquired MRSA'*

Clinical Trials – FP7



CT funded in FP7 by FP7 activity area



FP7 CT Budget Evolution

Clinical Trials – Horizon 2020

- Key element of health research supported in Horizon 2020: **no innovation without clinical trials!**
- Specific Programme (legal basis): "translation of research findings into the clinic, in particular through the conduct of clinical trials […] is essential"
- 2014/15 calls: Over €300 million of funding earmarked for clinical trials
- Emphasis on SMEs and on **investigator-driven clinical trials**

New EU Clinical Trials Regulation

- Responds to criticism of current EU Clinical Trials Directive:
  - Single submission portal for multinational trials
  - Joint assessment by member states' competent authorities
  - Tacit-approval deadlines
  - Reduced administrative burden (low-risk trials)
- Agreement between European Parliament and Council
- Adopted on 16 of April 2014, published in OJ on 27 May 2014
- Applicable two years after publication (+ transition period)

Challenges for multinational investigator-driven clinical trials

- **Insufficient professionalisation**
  - Planning/Protocol design: Time, Patient Recruitment, Finances, Contingency
  - Regulatory affairs
  - Quality assurance/quality control
- **Insufficient harmonisation/common approaches across Europe**
  - IT platforms, databases, links to biobanks
  - Monitoring, Quality assurance/control

Personalised medicine to address significant challenges…

…and benefit from opportunities

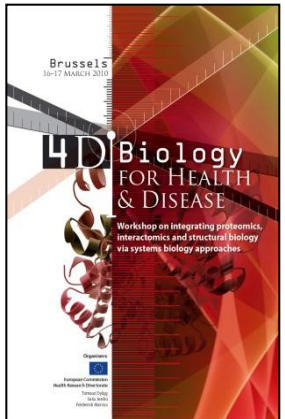
- **Burden of non-communicable diseases (loss of €35 trillion over next 20 years)**
- **Pressure on healthcare systems**
- **Gap between EU and global innovation leaders**
- **Challenges of drug development in Europe**

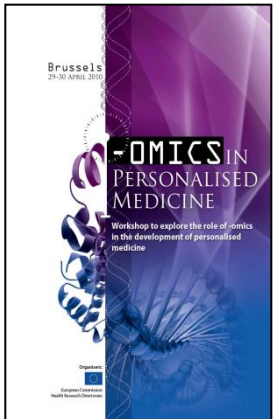
- *Better outcomes for patients and potential cost savings (as suggested by early studies of stratified approach*

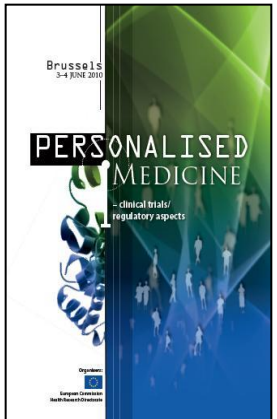
Europe can lead implementation of personalised medicine thanks to favourable conditions

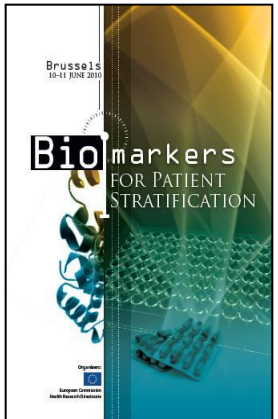
Personalised Medicine: preparing the ground

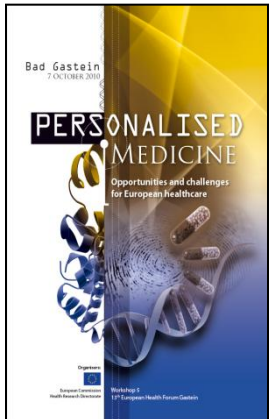
- **2010: Preparatory workshops (-omics, biomarkers, clinical trials/regulatory, uptake)**
- **2011: European Perspectives conference**
- **2013: Commission Staff Working Document on "use of '-omics' technologies in the development of personalised medicine"**
- **Identify key challenges to be addressed by research**











Identified key research challenges

Breaking barriers & speaking the same language

"cross-disciplinarity", capacity building, education & training

Translating knowledge to medical applications

disease taxonomy, biomarker validation, clinical trials

Generating knowledge & developing the right tools

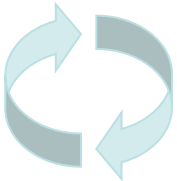
standards, clinical bioinformatics, adaptation of tools

Understanding the value & economic aspects

health care pilots, HTA, comparative effectiveness research, value chain

Framework for Personalised Medicine

R&D the basics	R&D stratifying tools	R&D test in human	Towards the market	Uptake in healthcare	In patients
"Omics" Technologies Data Samples Statistics	Biomarkers Identification Qualification Validation Data modelling tools Technical aspects & challenges	Clinical trials methodologies Patient - recruitment	Diagnostics & Therapies Approval processes Regulatory aspects	Pricing & Reimbursement Health economy HTA Novel models of healthcare organisation	Availability & usability in the clinic Patient perspective Equal treatment Social and legal issues Education and training



Prediction - Prevention – Treatment - Cure

European Commission

Over 1 billion EUR to top research

EU Health Research Programme:
Enabling personalised medicine 2007-2013

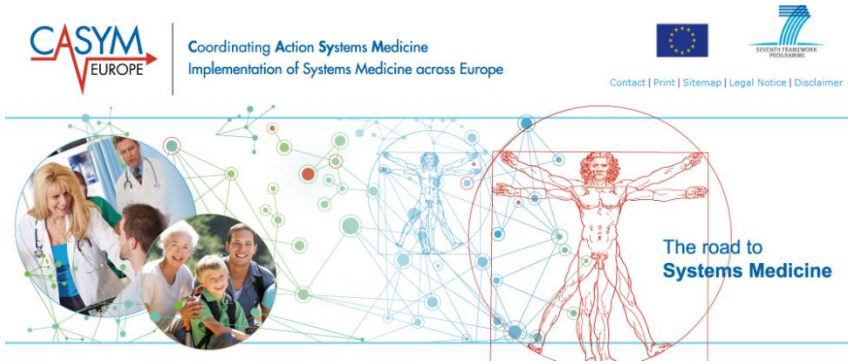
- Large scale data gathering and "-omics"
- Technology development
- Diagnostics
- Biomarkers
- Pre-clinical and clinical research
- Rare diseases: small patient populations
- Public health research
- IMI projects with pharma industry

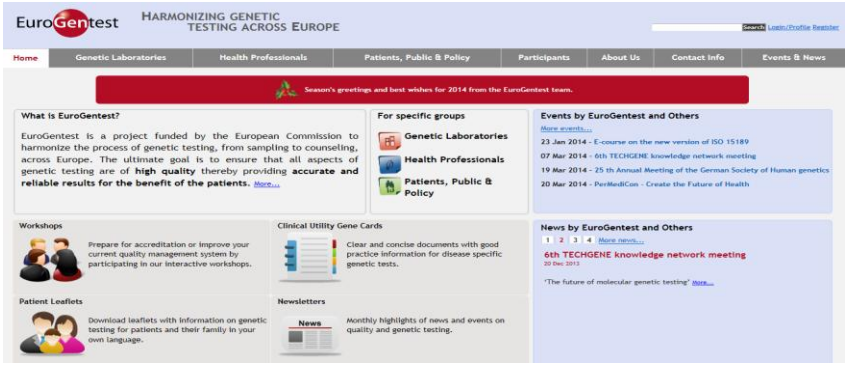
SEVENTH FRAMEWORK PROGRAMME

Research & Innovation

European Commission

Supporting policy development

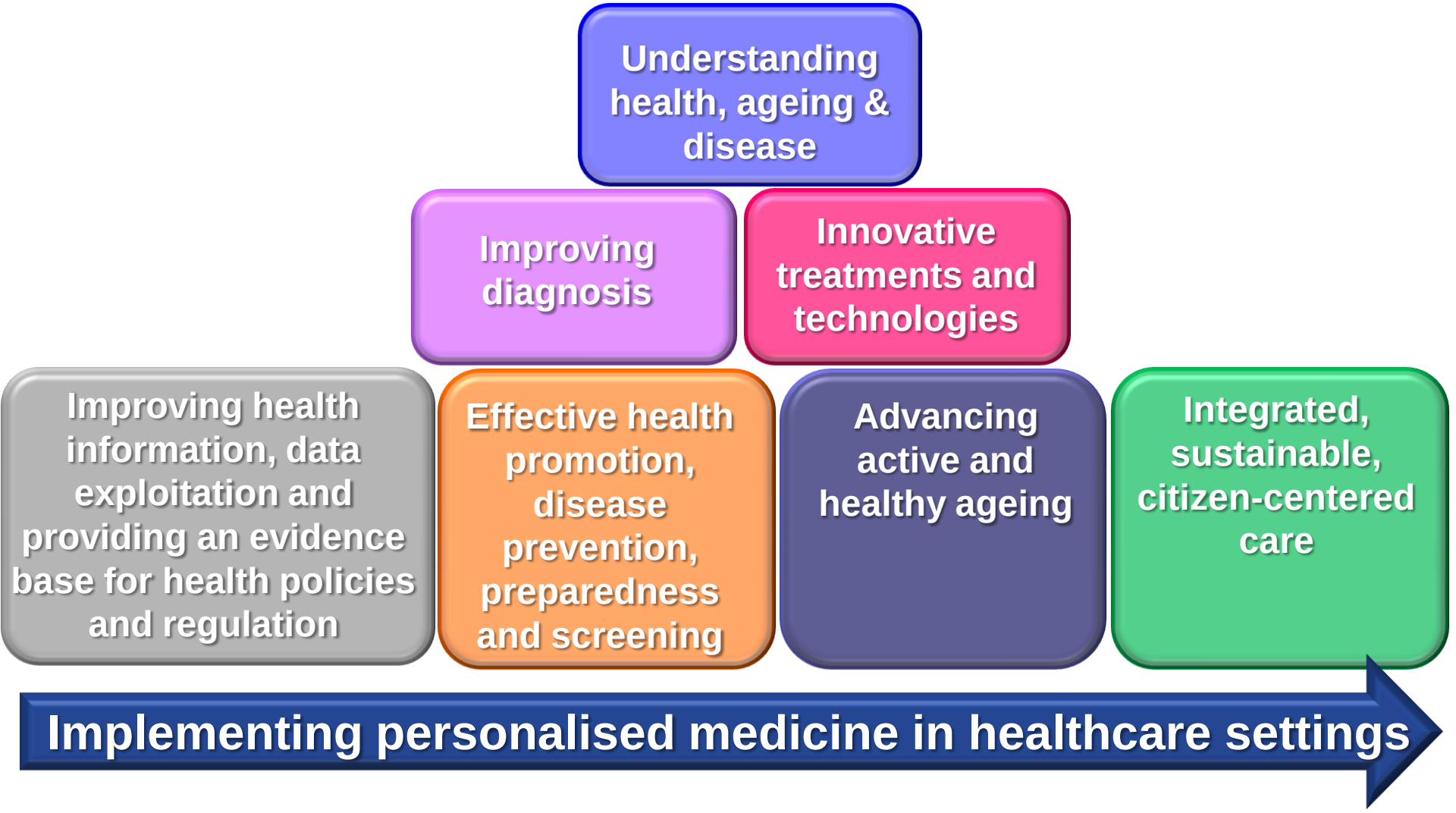




SEVENTH FRAMEWORK PROGRAMME

Research & Innovation

Focus areas of 2014-2015 Work Programme



THANK YOU!