



A vision for European biomedical and health research

Biomedical and health research: developing a vision for Europe

FEAM European Biomedical Policy Forum annual lecture

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Late spring 2018

- *Proposal for the EU's long term budget*
- Proposals for the EU programmes for the policy areas including for research and innovation (FP 9).



Main policy initiatives in the coming months beyond FP9

- *Digital transformation of health and care*
- *Improving Health Security in the EU – a one health approach to counteracting the threat from infectious diseases*
- *Council Recommendations on **Vaccination***
- *Review of EU activities on Personalised Medicine*

What challenges lie ahead ?

1. Unsustainable health and care costs



Increasing prevalence of chronic diseases and an ageing population

2. Role of environment on health



Lifestyle, pollutants, climate change

3. Increasing risks of infectious diseases



Resistance to antibiotics, (re)-emerging diseases

4. Health inequalities and access to health and care



Access to health promotion, prevention, treatment

5. Keeping a competitive European health and care industry



Keep Europe as the best innovator in healthcare area

- *Health care expenditure of EU countries is steadily increasing and now accounts for nearly 10% of GDP, as a result of increasing prevalence of chronic diseases due to unhealthy lifestyles and an ageing population.*
- *Avoidable environmental factors cause 1.4 million deaths yearly in the EU.*
- *Infections resistant to antimicrobials (AMR) cause 25'000 deaths yearly in the EU and 700 000 deaths globally.*
- *Life expectancy in some EU countries is six years lower than the average.*
- *EU healthcare industry accounts for 3% of GDP and 1.5 million employees.*

How to design a Research and Innovation programme that will have the impact we want on these challenges?



What do EU R&I offer? What is our basis?

A diversified palette of instruments for R&I -1-

- *More than 1000 **transnational collaborative projects** underway, with over 10 000 participants.*
 - *Increased European human capital on health research and its excellence. Networking of health R&I across Europe and beyond.*
- **A public-private partnership** (the world's largest in health): *IMI*
 - *European-wide unmet medical needs addressed*
- **Coordinating national funding programmes** : *EDCTP, JPND, JPAMR, HBM4EU*
 - *EU helps countries pooling their resources.*

What do EU R&I offer? What is our basis?

A diversified palette of instruments for R&I- 2

- *Some 10 **global consortia of health research funding agencies** aligning health R&I funding at international scale*
 - *Alignment of international health R&I efforts*
- *A **specific finance facility for loans** to innovators addressing infectious diseases*
 - *R&I investments leveraged, incentives to address crucial risky areas*
- **Horizon Prizes**
 - *Innovative solutions to targeted health challenges*

Some significant achievements already

- *A projected 56 000 high-level scientific articles in health*
- *500 000 new compound candidates for new drugs made them available to other researchers; and experts across Europe (IMI)*
- *A rapid response to help eradicating Ebola*

EU research investment leveraging other funds

Changing breast cancer practice

FP6, 2002-2006

Horizon 2020, 2014-2020

€ 7 million

Validation of the 70-gene signature

€ 4 million

2015: MammaPrint final clinical utility



HEALTHCARE PROFESSIONALS

PATIENTS

ABOUT



2002: 70-gene signature published

2007: FDA approves MammaPrint

2007

2011

MINDACT trial : Microarray In Node negative Disease may Avoid ChemoTherapy

MINDACT

Leveraged funds: € 43 million

MammaPrint reduced chemotherapy prescription post-surgery by 46%

Phase III CT 6693 women enrolled

The NEW ENGLAND
JOURNAL of MEDICINE

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70-Gene Signature as an Aid to Treatment Decisions in Early-Stage Breast Cancer

F. Cardoso, L.J. van't Veer, J. Bogaerts, L. Slaets, G. Viale, S. Delaloge, J.-Y. Pierga, E. Brain, S. Causeret, M. DeLorenzi, A.M. Glas, V. Goulinopoulos, T. Goulioti, S. Knox, E. Matos, B. Meulemans, P.A. Neijenhuis, U. Nitz, R. Passalacqua, P. Ravdin, I.T. Rubio, M. Saghatelyan, T.J. Smilde, C. Sotiriou, L. Stork, C. Strahle, G. Thomas, A.M. Thompson, J.M. van der Hoven, P. Vuylsteke, R. Bernards, K. Tryfonidis, E. Rutgers, and M. Piccart, for the MINDACT Investigators*

THE MINDACT TRIAL

PERSONALISED MEDICINE

tailor-made prevention, diagnosis and treatment
for individuals or groups of individuals

Enabling

HEALTHIER, MORE PRODUCTIVE LIVES



Significant EU investments in research on
personalised medicine to

TREAT PATIENTS WITH THE THERAPIES THAT WORK BEST FOR THEM

many common medicines are not effective
for many patients

CUT HEALTHCARE COSTS

as Europe's population ages
and chronic diseases become
more prevalent



DRIVE HEALTHCARE INNOVATION

Establish Europe as a global leader in
healthcare industry and innovation,
and create jobs and economic growth



AVOID ADVERSE REACTIONS TO MEDICINES

6% of acute hospital admissions are
due to serious adverse reactions to
medicines

An example of success story



*The **project ESPOIR** (FP7) developed a **new heart valve replacement methodology for children** that does not activate the immune system and regenerates tissues. It is already accepted and reimbursed in 6 countries.*

Examples of orphan therapy development funded through Horizon 2020



BATCure : New therapies for a group of rare lysosomal diseases, neuronal ceroid lipofuscinoses or Batten disease



MYOCURE : Innovative gene therapy platform to cure rare hereditary muscle disorders focusing on myotubular myopathy (MTM) and glycogen storage disorder (GSD) type II



UM Cure 2020 : Novel therapeutic approaches for treatment of uveal melanoma



SCIDNET : Developing genetic medicines for Severe Combined Immunodeficiency (SCID)

VISION-DMD : Advancing development of steroid-like orphan drug VBP15 as therapy for patients with Duchenne Muscular Dystrophy

VISION-DMD

More information available on Cordis Project Search:
http://cordis.europa.eu/projects/home_en.html

How can we have more impact ?

Key findings of the Interim Evaluation of Horizon 2020:

An attractive, simplified and well-performing programme, highly relevant for stakeholders and societal needs.

On track to deliver **value for money** and **to meet its knowledge-creation objectives**.

Strong **EU Added Value** through unique opportunities, competition & access to new knowledge.

There is **room for improvement**:

- ***The **strategic challenges and objectives** are not always clearly translated in specific calls and topics.***
- ***Low involvement of civil society (but improved over FP7). Need to **bring R&I closer to the public**.***
- ***Establish an impact-focused, mission-oriented approach***



- *“Lamy report” - Recommendation 5: Adopt a mission-oriented, impact-focused approach to address global challenges*

Action: Set research and innovation missions that address global challenges and mobilise researchers, innovators and other stakeholders to realise them.

Towards FP9



"We need to define missions that break down silos.

We have made progress in Horizon 2020 to focus resources in selected areas. But we still support too many different projects that disperse or fragment our funding.



We need to set our eyes on a specific target, and drive our scientific efforts towards reaching that target. And we need to be ambitious about it."



The **report** describes **key criteria to select missions** and important **factors for implementation** (released by Prof. Mariana Mazzucato on 22/2/2018).

A **Call for Feedback on missions** on the basis of this report is **open until 3 April 2018**

Criteria for selecting R&I missions

proposed by Prof Mazzucato



- **Bold, inspirational**, with wide **societal relevance**
- A **clear direction**: Targeted, measurable and time-bound
- **Ambitious but realistic** R&I actions
- **Cross-disciplinary, cross-sectoral and cross-actor** innovation
- Multiple **bottom-up solutions**

Key factors for implementing R&I missions at EU level



- **Engagement** of diverse national and regional stakeholders
- **Measurement of progress and impact** by goals and milestones
- A **portfolio of instruments** to foster bottom-up solutions
- **Flexibility, pro-active management and building in-house capabilities**
- **Public engagement**
 - Public participation in the selection process
 - Public inclusion in the implementation / Citizens as active participants in missions

Moving from broad specific missions

challenges to



“The '**granularity**' of European research and innovation missions sits between broad challenges and concrete projects. Missions set clear and ambitious objectives that can only be achieved by a portfolio of research and innovation projects and supportive measures.” (Mazzucato report)



Political Agenda
Setting and
Civic Engagement



Clear
Targeted
Missions



Portfolio of
projects and
bottom-up
experimentation



Illustrative example from Horizon 2020: tackling the global challenge of rare diseases

Challenges

- *6000-8000 diseases - more than 30 million patients in EU*
- *Huge unmet medical needs for patients across the globe*
- *Small patient populations for individual diseases*
- *Scarce and scattered research resources and expertise*



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➔ **Establishment of**  **IRDiRC** **in 2011**

INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

Over 50 international partners from 5 continents: public funders, private companies, foundations and patient organisations



"to stimulate, coordinate & maximise output of rare disease research efforts around the world"

www.IRDIRC.org

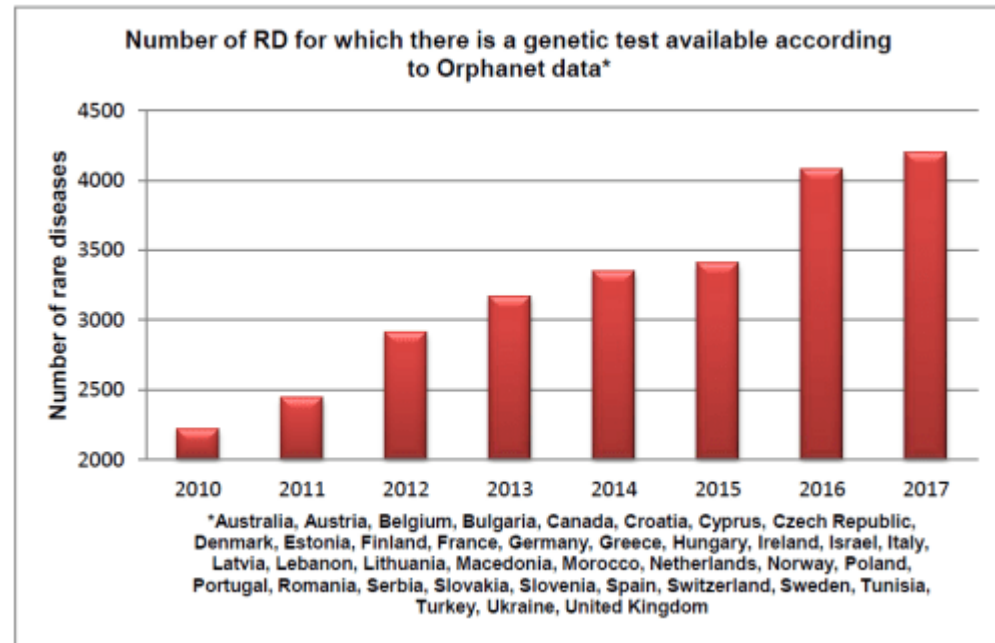
Goals by 2020:

- ▶ 200 new therapies for rare diseases
- ▶ Means to diagnose most rare diseases



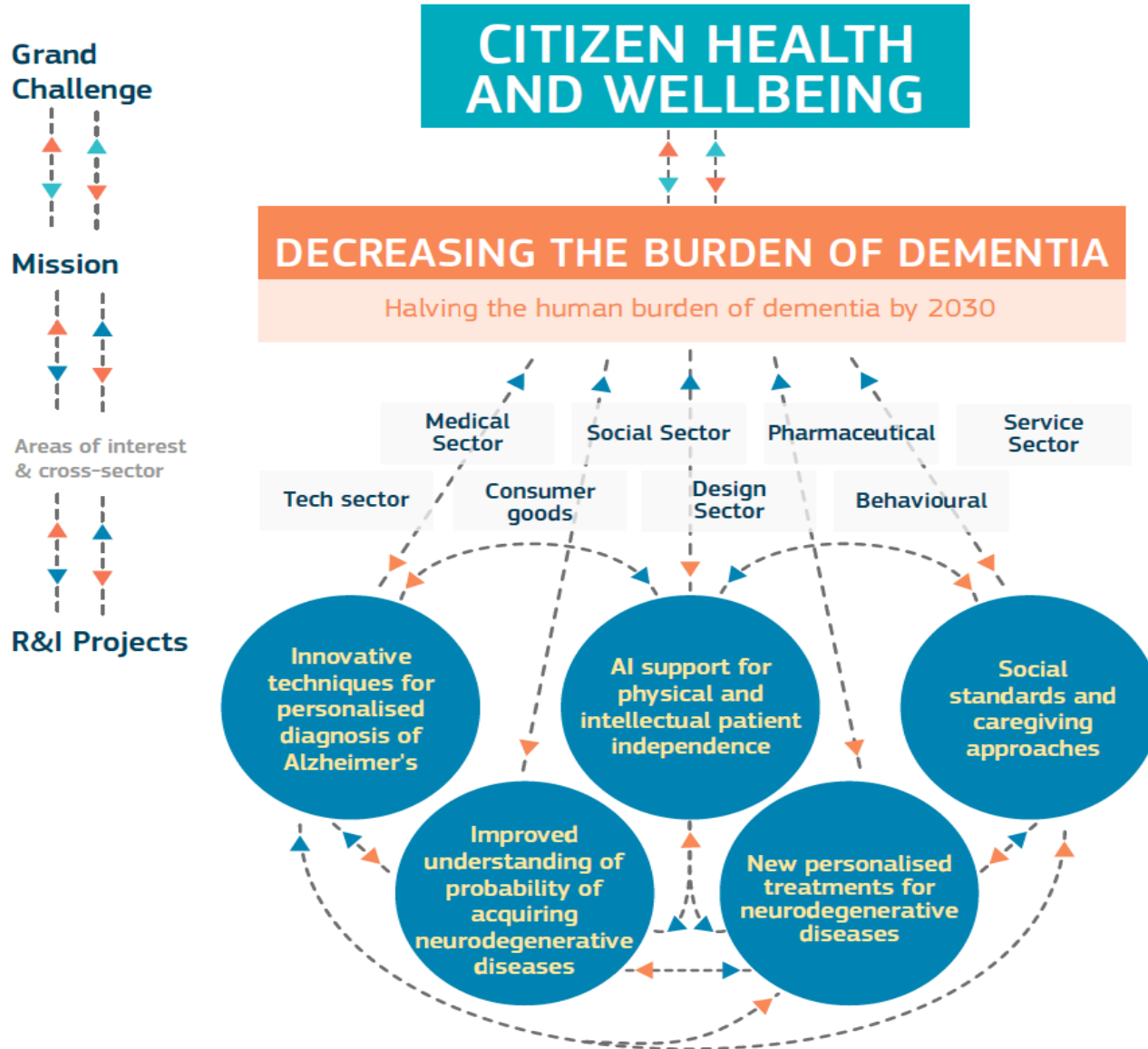
Goal achieved in 2016

Number of new orphan drugs, EMA and FDA cumulatively



Number of genetic tests available

Under FP9: An example of a possible mission





THANK YOU FOR YOUR ATTENTION

This presentation does not represent a formal position of the European Commission. Please refer to official documents



HORIZON 2020

Find out more:

<http://ec.europa.eu/programmes/horizon2020/>

<http://ec.europa.eu/research/health/index.cfm?pg=home>

Funding opportunities:

<http://ec.europa.eu/research/participants/portal/desktop/en/home.html>

Information about financial instruments:

<http://www.eib.org/products/blending/innovfin/>