



# HORIZON EUROPE

## WHAT IS CHANGING IN R&I WHAT IS RELEVANT FOR RARE DISEASES

Council of European Federations  
8 November 2019

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# Horizon Europe

A EU funding  
programme for  
Research & Innovation

## Horizon Europe



### I. SINGLE MARKET, INNOVATION AND DIGITAL €187.4

- 1 Research and Innovation
- 2 European Strategic Investments
- 3 Single Market
- 4 Space



### II. COHESION AND VALUES €442.4

- 5 Regional Development and Cohesion
- 6 Economic and Monetary Union
- 7 Investing in People, Social Cohesion and Values



### III. NATURAL RESOURCES AND ENVIRONMENT €378.9

- 8 Agriculture and Maritime Policy
- 9 Environment and Climate Action



### IV. MIGRATION AND BORDER MANAGEMENT €34.9

- 10 Migration
- 11 Border Management



### V. SECURITY AND DEFENCE €27.5

- 12 Security
- 13 Defence
- 14 Crisis Response



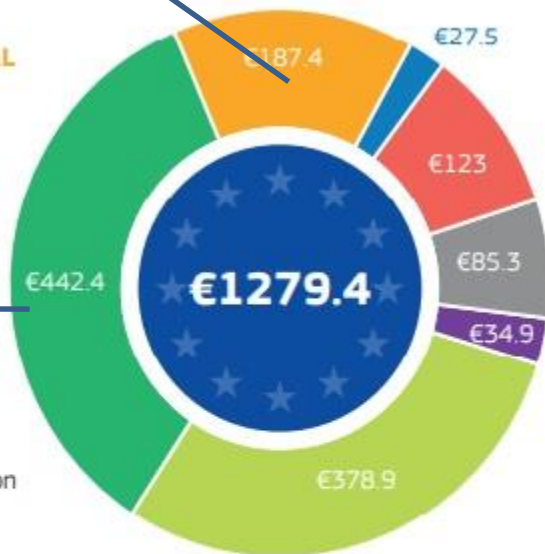
### VI. NEIGHBOURHOOD AND THE WORLD €123

- 15 External Action
- 16 Pre-Accession Assistance



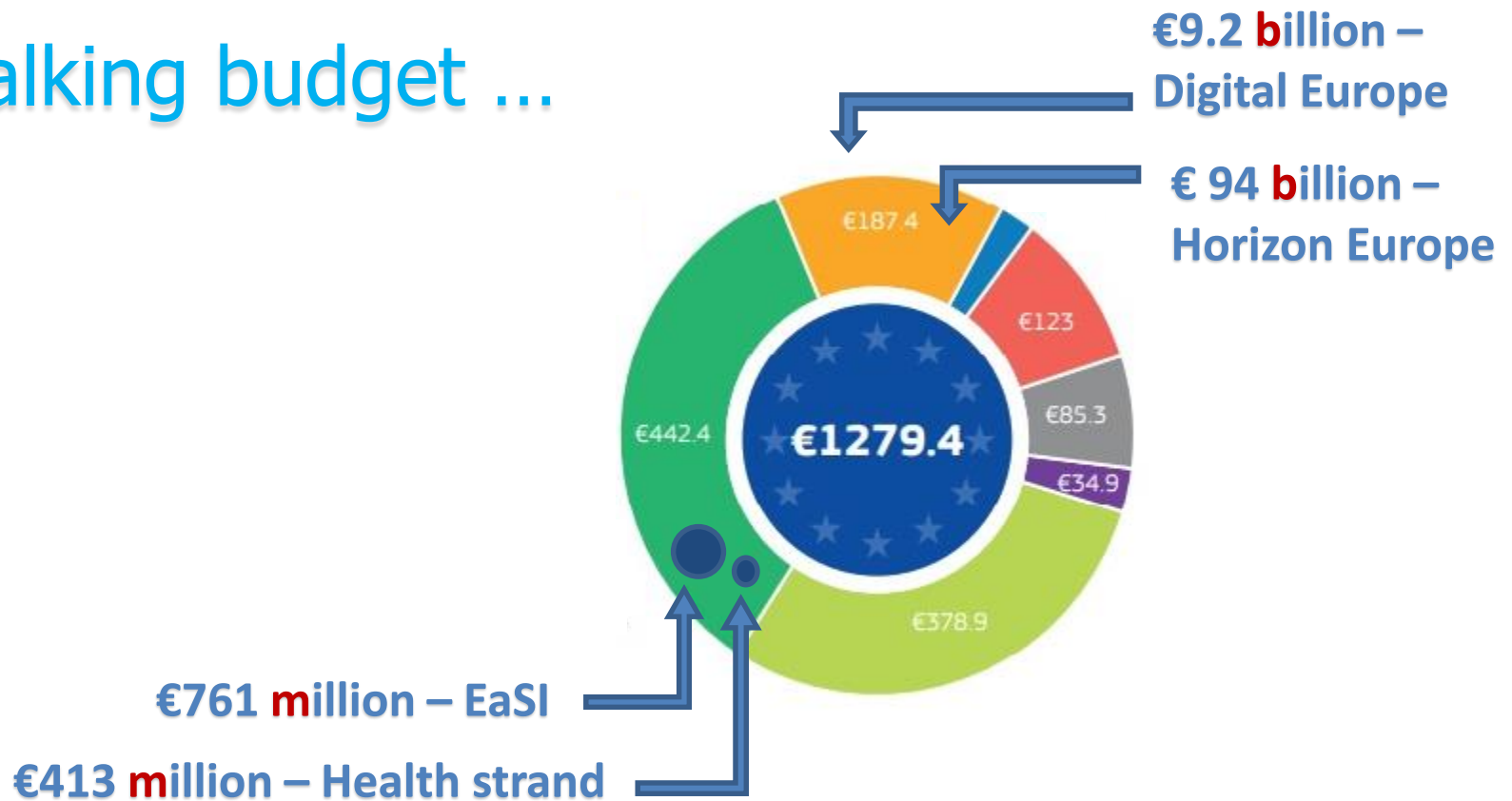
### VII. EUROPEAN PUBLIC ADMINISTRATION €85.3

- 17 European Public Administration



## Health Programme

# Talking budget ...



- Horizon Europe
  - ✓ real increase (from approx. 50 b in H2020 to 83 in HE)
  - ✓ also in Health (from 3.5 bEUR to 7.7 bEUR)
- Digital Europe
  - ✓ an important statement on where priorities are, regardless of the actual size of the programme (9.2 bEUR)

## Ongoing negotiations on budget

European Commission (EC)	European Parliament (EP)	Council (Member States)
94 billions	120 billions	Less than 94 billions?
Negotiations between the 3 Institutions on the entire EU budget 2021-2027, including other programmes and funding instruments		
The new Commissioner for Research Gabryiel promised to fight to maintain the EC proposal, likely to obtain EP support		

# 1) Zoom on Health in the future EU budget



## A STRONG HEALTH DIMENSION IN THE FUTURE EU BUDGET

Health-related resources will be part of the budget of several priority areas: social protection, research and innovation, the digitisation of society, cohesion and global responsibility. Health policies will get EU funding through various mechanisms, mainly:

**European Social Fund  
Plus (ESF+)**

**European Regional Development  
Fund (ERDF)**

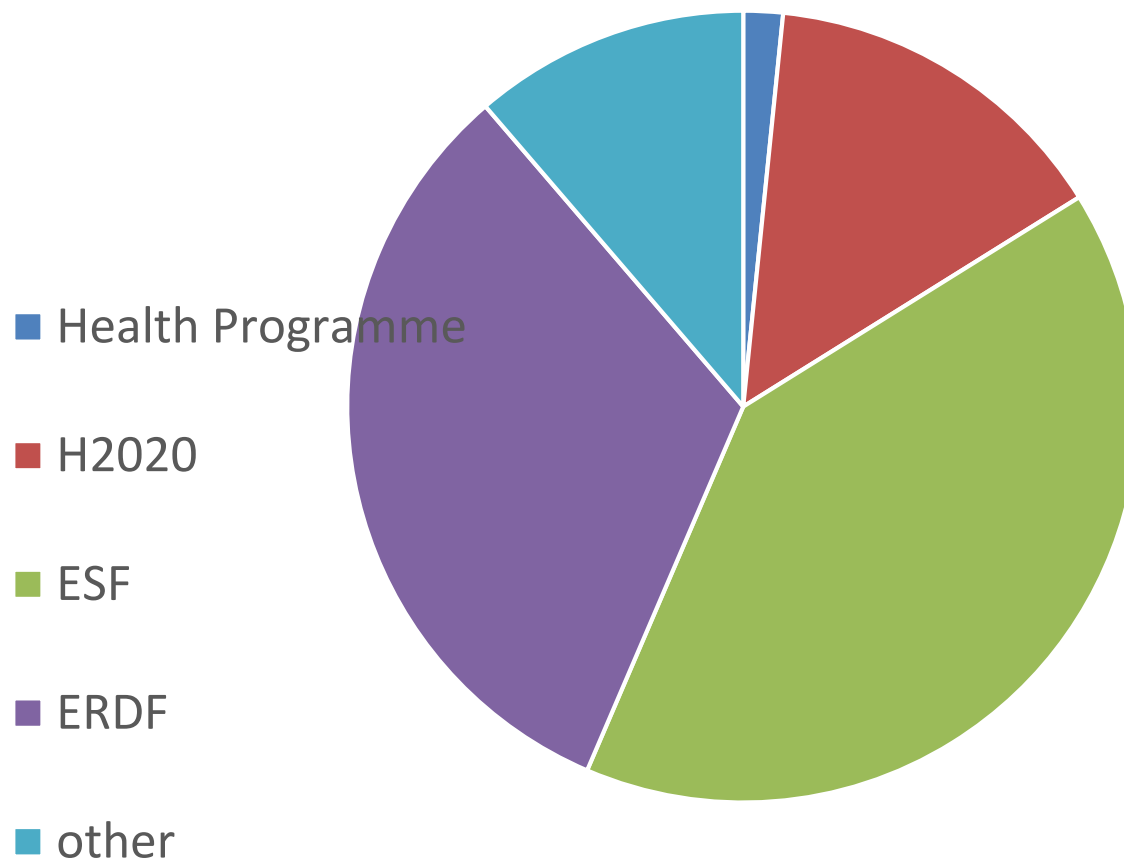
**Horizon Europe  
(EU research programme)**

**Digital Europe Programme**

**InvestEU Fund**

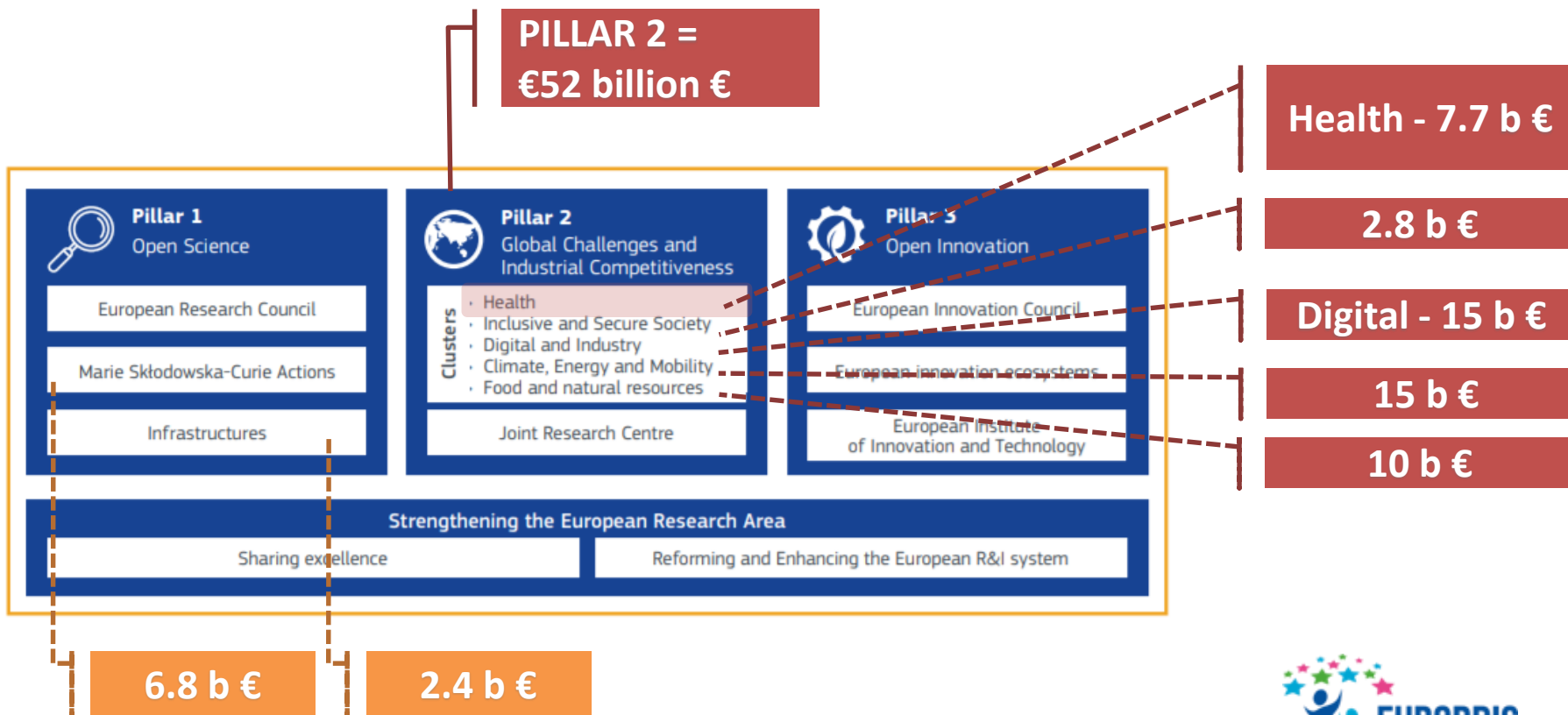
**Emergency Aid Rescue (rescEU)**

# Health expenditure 2014-2018 through EU funding programmes



# 1) Zoom on Research in future EU budget

- From Horizon 2020 to « **Horizon Europe** »
- Largely increased budget = **€94 billion**





# What's new in Horizon Europe



## WHAT'S NEW?



Support breakthrough innovation →



Create more impact through mission-orientation and citizens' involvement →



Reinforce openness →



Rationalise the funding landscape →



Reduce administrative burden →

European Innovation Council

EU-wide R&I missions

Open science policy

New approach to Partnerships

Simpler rules

### Eur. Innovation Council

- From lab to real world, start ups and scale of most innovative ideas

### R&I Missions

- a sort of "sub-programmes"
  - measurable results for a given impact
  - within a set timeframe
  - visible to citizens
- e.g. strategy for cancer or reducing plastic in oceans*

### Partnerships

= streamline the n° of partnerships that the EU co-funds with partners like industry, civil society and funding foundations

# What is new

## Partnerships

- “initiatives where the EU, together with private and/or public partners (such as industry, research organisations, bodies with a public service mission at local, regional, national or international level or civil society organisations including foundations), commit to jointly support the development and implementation of a programme of research and innovation activities, including those related to market, regulatory or policy uptake;”
- **Ongoing discussions and negotiations with Member States**
- **8 *partnership areas* agreed (trilogue) and 44 *candidate partnerships* at present, including (Council to decide):**

- **Rare Diseases** (follow-up to EJP Rare Diseases post 2023)
- **Innovative Health Initiative** (follow-up to IMI2)

Also:

- AI, data and robotics

# What is new

## Missions

- “a portfolio of actions intended to achieve a measurable goal within a set timeframe, and impact for science and technology and/or society and citizens that could not be achieved through individual actions”
- **5 Missions agreed so far (trilogue):**
  - **Climate change** - .C Hedegaard
  - **Soil** – C. Veerman
  - **Cancer** – H. zu Hausen
  - **Oceans, Seas, Inland Waters** – P. Lamy
  - **Carbon neutral and Smart Cities** - Gronkiewicz-Waltz
- **Mission boards (15 members each) and appointed leaders**
- **EC is trying to introduce a clause for future possible inclusion of new Missions**

# 3) Zoom on Digital in future EU budget

A brand new programme « **Digital Europe** »

**€9.2 billion**

## FIVE FOCUS AREAS UNDER DIGITAL EUROPE PROGRAMME:





**Horizon Europe**

**Health Cluster**

# R&I for Health in Horizon Europe

## (a) Cluster 'Health':

“Improving and protecting the health of citizens at all ages, by developing innovative solutions to prevent, diagnose, monitor, treat and cure diseases; mitigating health risks, protecting populations and promoting good health; making public health systems more cost-effective, equitable and sustainable; and supporting and enabling patients' participation and self-management.”

## Areas of intervention:

- Health throughout the life course;
- Environmental and social health determinants; **Non-communicable and rare diseases**;
- Infectious diseases;
- Tools, technologies and digital solutions for health and care;
- Health care systems

## Health in Horizon Europe - orientations

- Reference to the **EU Pillar of Social Rights** (right to timely access to affordable, preventive and curative health care that is safe and of good quality)
- Link to the EU's commitment to the **UN's Sustainable Development Goals** calling for universal health coverage for all and at all ages by 2030, leaving no one behind, and ending preventable deaths
- Address areas of unmet clinical needs such as for **example rare or hard to treat diseases** (cancers, such as paediatric and lung cancer)
- Will take full advantage of **genomic** and other **multiomics** frontier research, **personalised medicine** approaches, **digitalisation** in health and care “without jeopardising the right to privacy and data protection”
- **Synergies with the “Health Strand” in ESF+** (the future Health Programme): co-managed with DG SANTE

# Health in Horizon Europe – challenges

- Accessibility and affordability of health and care;
- Lack of effective health promotion and disease prevention;
- Rise of non-communicable diseases;
- Increased cases of cancer;
- Increasing costs for European health care system;
- Increasing pressure on the European health and care industry to remain competitive in and by developing health innovation vis-à-vis emerging global players.



# Health in Horizon Europe – Non Communicable and Rare Diseases

## Preidentified priority areas:

- Understanding the mechanisms underlying the development of non-communicable diseases
- Longitudinal population studies to support understanding health and disease parameters;
- Diagnostic tools and techniques for earlier and more accurate diagnosis and for timely patient-adapted treatment
- Prevention and screening programmes, in line with or going beyond WHO, UN and EU recommendations
- Integrated solutions for self-monitoring, health promotion, disease prevention, and management of chronic conditions and multi-morbidities;
- Treatments, cures or other therapeutic interventions;
- ***Areas of high unmet clinical need, such as rare diseases, incl paediatric cancers;***
- Assessment of comparative effectiveness of interventions and solutions, including based on Real World Data (RWD);
- ***Development of research and improvement of information, care and treatment, including personalised medicine, for rare diseases***



# Horizon Europe

**RD research priorities  
(EURORDIS proposal)**

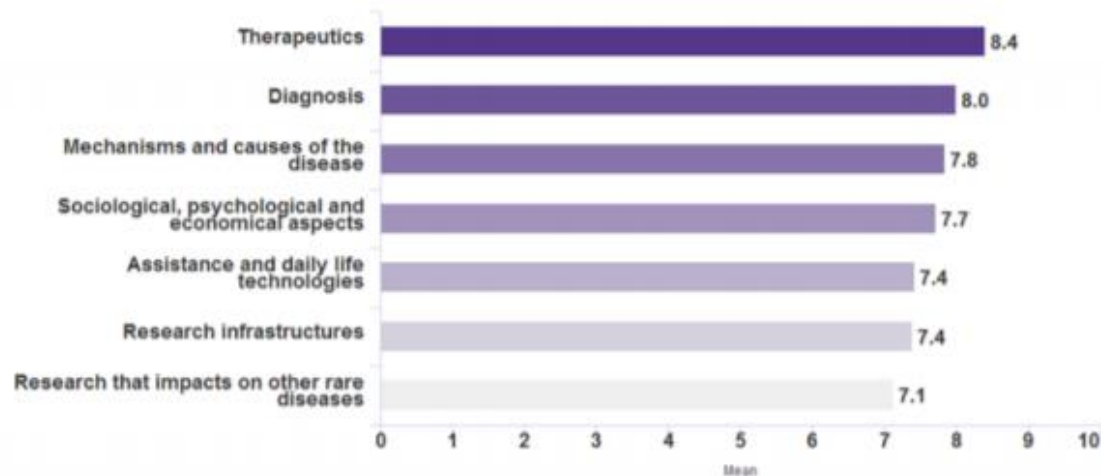
# What RD patients think

## 4.3 Rare disease patients' perspectives on rare disease research

### 4.3.1 Therapeutic research is the priority for rare disease patients

Graph. 7

According to you, on a scale from 1 to 10, what degree of priority should be given to the following research areas? (average grade)



The survey included a question on policy priorities within the rare disease research field that was only submitted to patients engaged in advocacy activities. The results show that all the research areas are important to patient representatives as they all obtained a grade above 7 out of 10. However, **therapeutic research is clearly identified as the number one priority** among the list. *Sociological, psychological and economical aspects*, with a grade of 7.7 out of 10 appear to be particularly valued from a patient point of view.



**EURORDIS**  
Rare Diseases Europe

**The Voice of Rare  
Disease Patients  
in Europe**

# EURORDIS'

*POSITION ON RARE DISEASE RESEARCH*



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## Horizon Europe Co-Design 2021-2024

- EC is preparing the Horizon Europe Strategic Plan 2021-2024
- In « Co-design with stakeholders »
  - Written consultation: Provide here further general input regarding the targeted impacts from Horizon Europe (5000 characters)
  - Draft Orientation following consultation
  - Research and Innovation Days 24-26 September 2019
  - Strategic Plan to be adopted end 2019 (tbc)

# EURORDIS proposal

Cross-cutting  
priorities

Delivering quality  
healthcare for rare  
diseases

Developing  
treatments for rare  
diseases

Improving diagnosis  
for people living  
with a rare disease

## Cross-cutting priorities

Continue to sustain the partnership on rare disease research (European Joint Programme on rare disease, EJP RD) beyond 2023

Improve the coordination between infrastructures that provide structural support to research activities in the field of rare diseases

Clinical Research Networks for Rare Disease

Scaling up trainings in European Joint Programme in Rare Disease

Establishing funding mechanisms that guarantee long-term sustainability of common EU research infrastructures or research projects of proven viability

Patient empowerment

Priorities linked to “Tackling diseases and reducing disease burden” and to “unlocking the full potential of new tools, technologies and digital solutions

## Delivering quality healthcare for rare diseases

Priorities linked to “Ensuring access to sustainable and high-quality health care in the EU

Artificial intelligence to support diagnosis and treatment of rare diseases

Addressing the health data challenge in the rare disease field

Clinical decision support tools for the digital age

Research in the clinical efficacy and financial effectiveness of virtual healthcare in ERNs to support NHS that lack expertise due to population size

Research in organisation of care and systems

Synergy between HTA and clinical guideline development as incentives for appropriate use of healthcare

Research into innovative supra-specialisation training in ERNs

Developing health professionals’ digital skills



Development of improved and larger newborn screening (NBS) programmes

Translating whole genome sequencing (WGS) from a research to diagnostic/clinical setting of integrating it as standard of care for suspected RD

## Improving diagnosis for people living with a rare disease

Genetic counselling

De novo mutations are increasingly being identified

# Developing treatments for rare diseases

Basic research to understand mechanisms underlying RDs and identify therapeutic targets

Selection and stratification and use of **PROMs** and **PREMs** adapted to RD to improve research and clinical outcomes of RD patients

Research on the “how to” of repurposing would be beneficial to the field and guide future research in that direction and to avoid failures

Natural, alternative, traditional and complementary (NATC) products may play an important role in disease primary/secondary prevention and/or treatment

Regulatory research on new disruptive technologies in clinical development

## Advanced therapies

- Continued collaboration on cell therapies
- Community engagement and patient-centred decision support interventions in gene and cell therapies
- Socio-economic assessment of advanced therapies and business/pricing model development
  - Ethical, legal and social aspects of use of advanced therapies in strategies of care to treat RDs

## HTA

- Alignment of HTA use at different levels
- Importance of additional patient data collection for HTA
- Specific attention to advanced innovative MD and other advanced technologies
  - Transferability of cos-effectiveness data

A European Fund to co-fund the post-marketing evidence generation as research activity

# Horizon Europe Co-Design 2021-2024

New consultation open **UNTIL 17 NOVEMBER!**

European Commission > Strategy > Digital Single Market > Consultations >

Digital Single Market

CONSULTATION | 31 October 2019 to 17 November 2019

**Share your views on the revised version of  
Orientations towards the first Strategic Plan  
for Horizon Europe**

<https://ec.europa.eu/digital-single-market/en/news/share-your-views-revised-version-orientations-towards-first-strategic-plan-horizon-europe>

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# Thank you for your attention

## FOR MORE INFO:

- [EU budget for health policies](#)
- [Press release - EU budget: A new Social Fund, Globalisation Adjustment Fund and Justice, Rights and Values Fund](#)
- [ESF+ legal text and factsheet](#)
- [Health factsheet](#)
- [Horizon Europe Factsheet](#)
- [Digital Europe Factsheet](#)
- [CEF Factsheet](#)

BACK UP SLIDES

# 3) Zoom on Digital in future EU budget

## **CEF « Connecting Europe Facility» Digital**      **€3 billion**

- Funds Trans European Networks, i.e. projects with cross-border impact that roll out ***European-wide interoperable systems and services***
- Projects with **high added-value at European scale** & leverage further investment from other sources
- Physical infrastructure necessary to enable the deployment of digital services
- Support key projects aligned with the 2016 Gigabit Society Strategy and essential for the success of the Digital Single Market, providing ***Gigabit connectivity for sociodrivens, such as hospitals, medical centres, schools and research centres***

## xxxxxEC Orientation Paper

1. *Protective Europe*, concerning security and defence policy (European Council: protecting citizens and freedoms);
2. *Competitive Europe*, which focusses on research and innovation, new technologies, digital capacities and industrial policy (European Council: developing a strong and vibrant economic base);
3. ***Fair Europe***, which centres on **social inclusion, health, rule of law and fundamental rights** (European Council: building a climate-neutral, green, fair and social Europe);
4. *Sustainable Europe*, regarding sustainable development, climate change, circular economy and energy (European Council: building a climate-neutral, green, fair and social Europe);
5. *Influential Europe*, dealing with international cooperation, **promoting European values and standards** (European Council: promoting European interests and values on the global stage).

## xxxxxxOrientation Paper – Impacts in Health Cluster (Fair Europe):

- **Healthy citizens in a rapidly changing society:** citizens stay healthier throughout the life course due to improved health promotion and disease prevention, and supported by healthier behaviours and lifestyles;
- **Healthy and health-promoting living and working environments:**
- **Effective health services to tackle diseases and reduce the burden of diseases:** patients can rely on effective health services to tackle their diseases, as well as to reduce the burden of diseases on them, their families and communities;
- **Improved access to innovative, sustainable and high-quality health care:** health systems are able to provide timely access to affordable health care services of high-quality to everybody while being environmentally and fiscally sustainable;
- **Unlocking the full potential of new tools, technologies and digital solutions for a healthy society:** new tools, technologies and digital solutions provide significant gains in health outcomes, address unmet medical needs and inform regulatory standards and requirements;
- **A sustainable and globally competitive health-related industry in the EU:** health industries, including SMEs, increase their productivity and sustainability in developing health innovation due to the potential of data-enabled research and development, the related convergence of pharmaceutical, digital and medical technologies, and the prospect of the digital transformation of health and care supported by data-driven manufacturing of tailor-made products and the delivery of personalised services



# Where we stand

## Orientation Paper of EC for MS

### Cluster Health

### 5 challenges

#### Challenge 1 – Stay healthy in a rapidly changing society

- 1.a - Safe pregnancy, birth, health childhood and families;
- 1.b - Healthy, independent and active living throughout the life course.

#### Challenge 3 – Tackling diseases and reducing disease burden

- 3.c – Unmet health needs of rare disease patients