



The EU Framework Programme for Research and Innovation

# HORIZON 2020

## **Horizon Europe overview Paediatric developments and prospects**



**EPTRI Stakeholders Roundtable  
Online Meeting  
9 July 2020**

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Research and  
Innovation

Commission proposal for  
**Horizon Europe**

THE NEXT EU RESEARCH & INNOVATION  
PROGRAMME (2021 – 2027)

#HorizonEU



# Horizon Europe - Investing to shape our future



# Horizon Europe

The ambitious EU research and innovation framework programme (2021-2027)



**to strengthen the EU's scientific and technological bases and the European Research Area (ERA)**



**to boost Europe's innovation capacity, competitiveness and jobs**



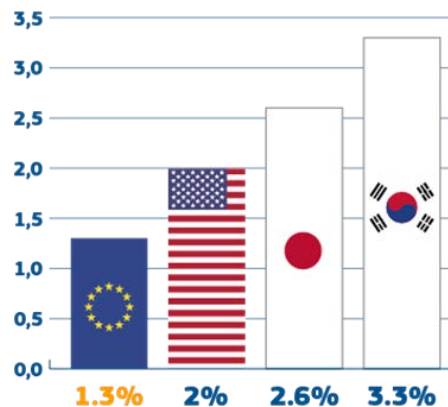
**to deliver on citizens' priorities and sustain our socio-economic model and values**

The Commission proposes a budget of € 100 billion for Horizon Europe.

# While benefiting from world-class research and strong industries...

Our knowledge and skills are our main resources.

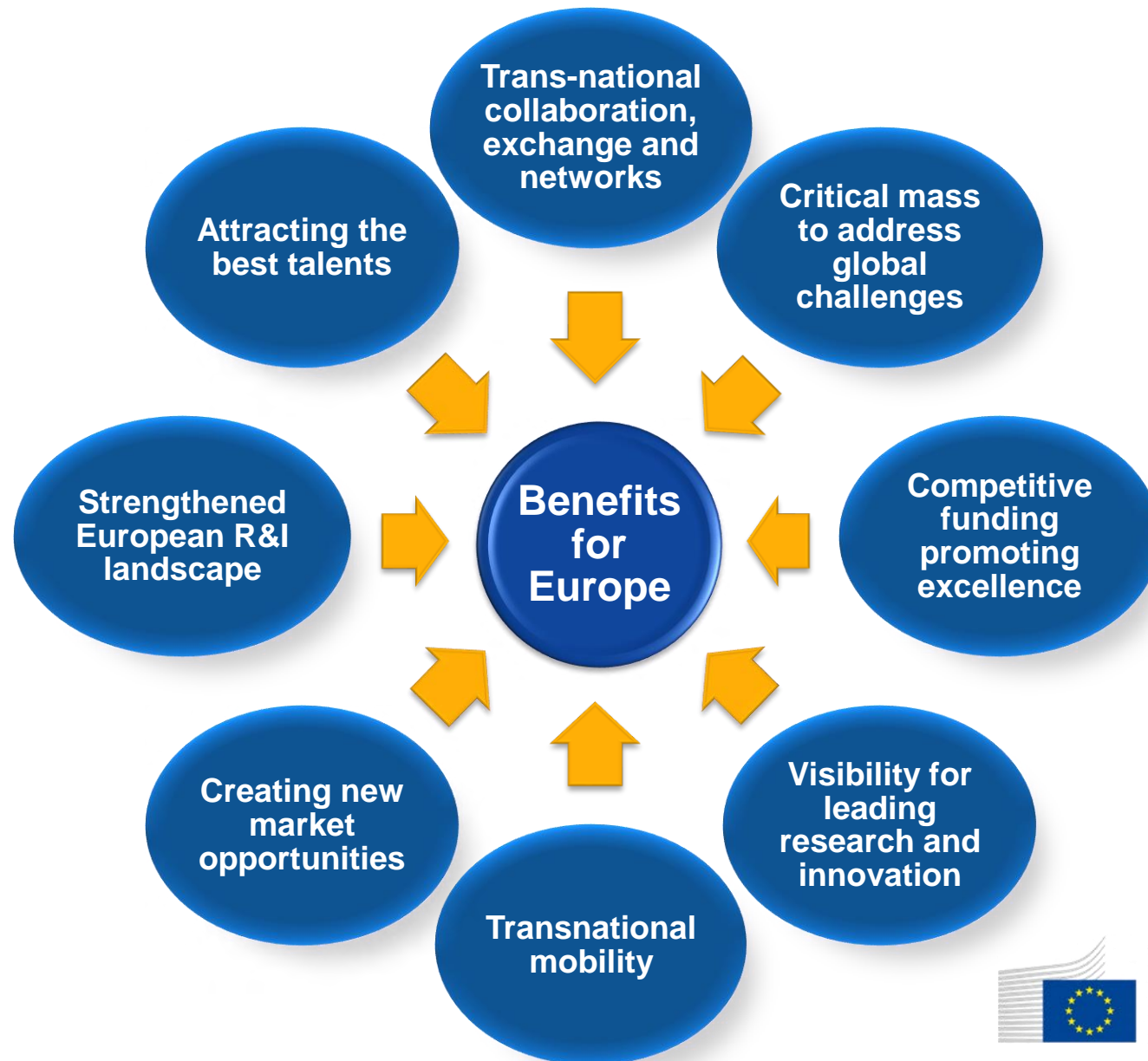
- **7%** of the world's population
- **20%** of global R&D
- **1/3** of all high-quality scientific publications



**1.3%**  
EU business  
R&D  
investment

**...Europe can do better at transforming this into leadership in innovation and entrepreneurship**

# Added value through Horizon Europe:



# Horizon Europe – Political agreement

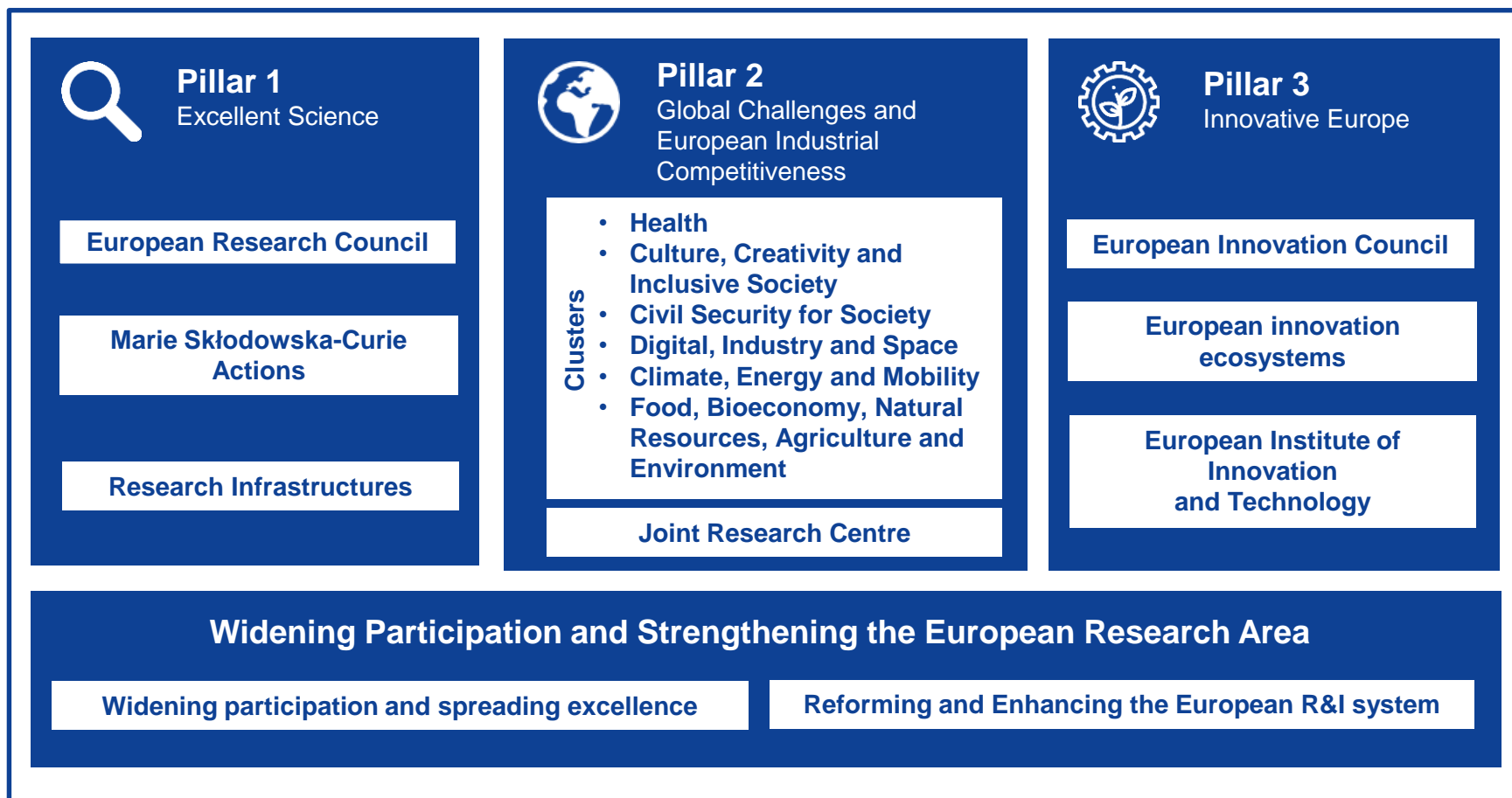
# European Parliament and Council reached a common understanding on Horizon Europe on 19 March 2019

- Budget, synergies and third country association still pending, depending on the overall MFF negotiations
- Commission has started preparations for the implementation of Horizon Europe

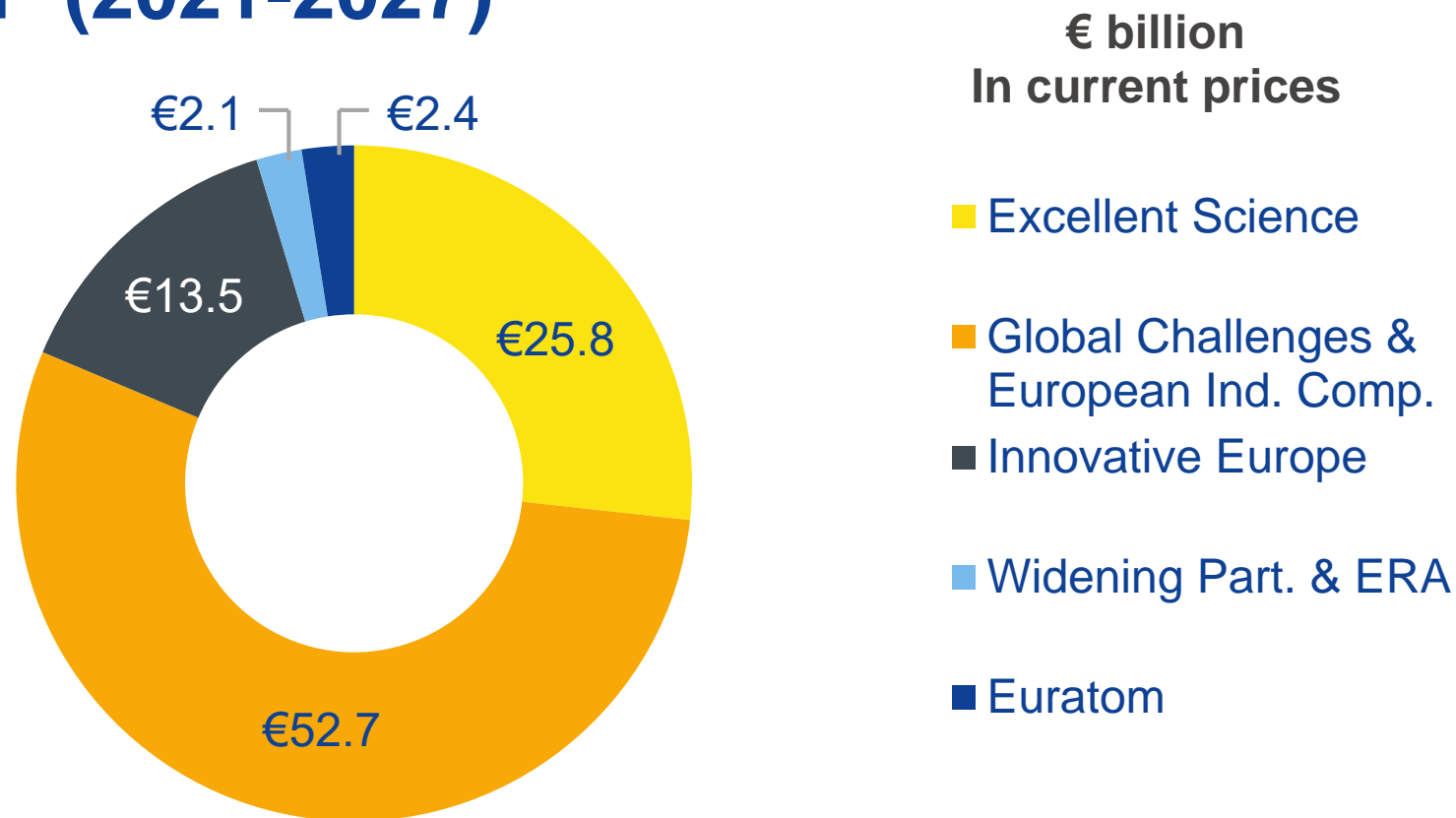




# Horizon Europe: Preliminary structure



# Commission proposal for budget: €100 billion\* (2021-2027)



\* This envelope includes EUR 3.5 billion allocated under the InvestEU Fund.

# Horizon Europe – Central elements

# Lessons Learned

## from Horizon 2020 Interim Evaluation

# Key Novelties

## in Horizon Europe



Support breakthrough innovation



European Innovation Council



Create more impact through mission-orientation and citizens' involvement



R&I Missions



Strengthen international cooperation



Extended association possibilities



Reinforce openness



Open science policy



Rationalise the funding landscape



New approach to Partnerships



Encourage participation



Spreading Excellence





# European Innovation Council

Support to innovations with breakthrough and disruptive nature and scale up potential that are too risky for private investors (**70% of the budget earmarked for SMEs**)

**European  
Innovation Council  
– a one-stop-shop**

Helping innovators create markets of the future, leverage private finance, scale up their companies, Innovation centric, risk taking & agile, pro-active management and follow up

Two complementary instruments bridging the gap from idea to investable project

**Pathfinder: grants**  
(from early technology  
to pre- commercial)

**Accelerator:**  
**grants only & blended finance**  
(from pre-commercial  
to market & scale-up)



# R&I Missions

## R&I Missions

Relating EU's research and innovation better to society and citizens' needs; with strong visibility and impact

A mission is a portfolio of actions across disciplines intended to achieve a **bold and inspirational and measurable goal** within a set timeframe, with **impact** for society and policy making as well as relevance for a significant part of the European population and wide range of European citizens.

Horizon Europe defines mission characteristics and elements of governance, and 5 missions areas.

Specific missions will be programmed within the Global Challenges and European Industrial Competitiveness pillar (drawing on inputs from other pillars)

**Adaptation to climate  
change, including societal  
transformation**



**Mission  
areas**

**Healthy  
oceans,  
seas,  
coastal  
and inland  
waters**



**Cancer**



**Climate-neutral  
and smart cities**



**Soil health  
and food**



**European  
Commission**



# International Cooperation

## International Cooperation

Tackling together global societal challenges; access to the world's best talents, expertise and resources; enhanced supply and demand of innovative solutions

### Extended openness to association

- Third countries with good capacity in science, technology and innovation
- Taking into account objective of driving economic growth in Europe through innovation
- General opening for international participation
- Intensified targeted actions (flagship initiatives, joint calls, etc.)





# Open Science across the programme

## Open Science

Better dissemination and exploitation of R&I results and support to active engagement of society

**Mandatory Open Access to publications:** beneficiaries shall ensure that they or the authors retain sufficient intellectual property rights to comply with open access requirements

**Open Access to research data ensured:** in line with the principle "as open as possible, as closed as necessary"; Mandatory Data Management Plan for FAIR (Findable, Accessible, Interoperable, Re-usable) and Open Research Data

- Support to researcher skills and reward systems for open science
- Use of European Open Science Cloud

# Horizon Europe – In detail

## Pillar 1

# EXCELLENT SCIENCE:

reinforcing and extending the excellence of the Union's science base

### European Research Council

- Frontier research by the best researchers and their teams

**Commission proposal:**  
**€ 16.6 billion**

### Marie Skłodowska-Curie Actions

- Equipping researches with new knowledge and skills through mobility and training

**Commission proposal:**  
**€ 6.8 billion**

### Research Infrastructures

- Integrated and inter-connected world-class research infrastructures

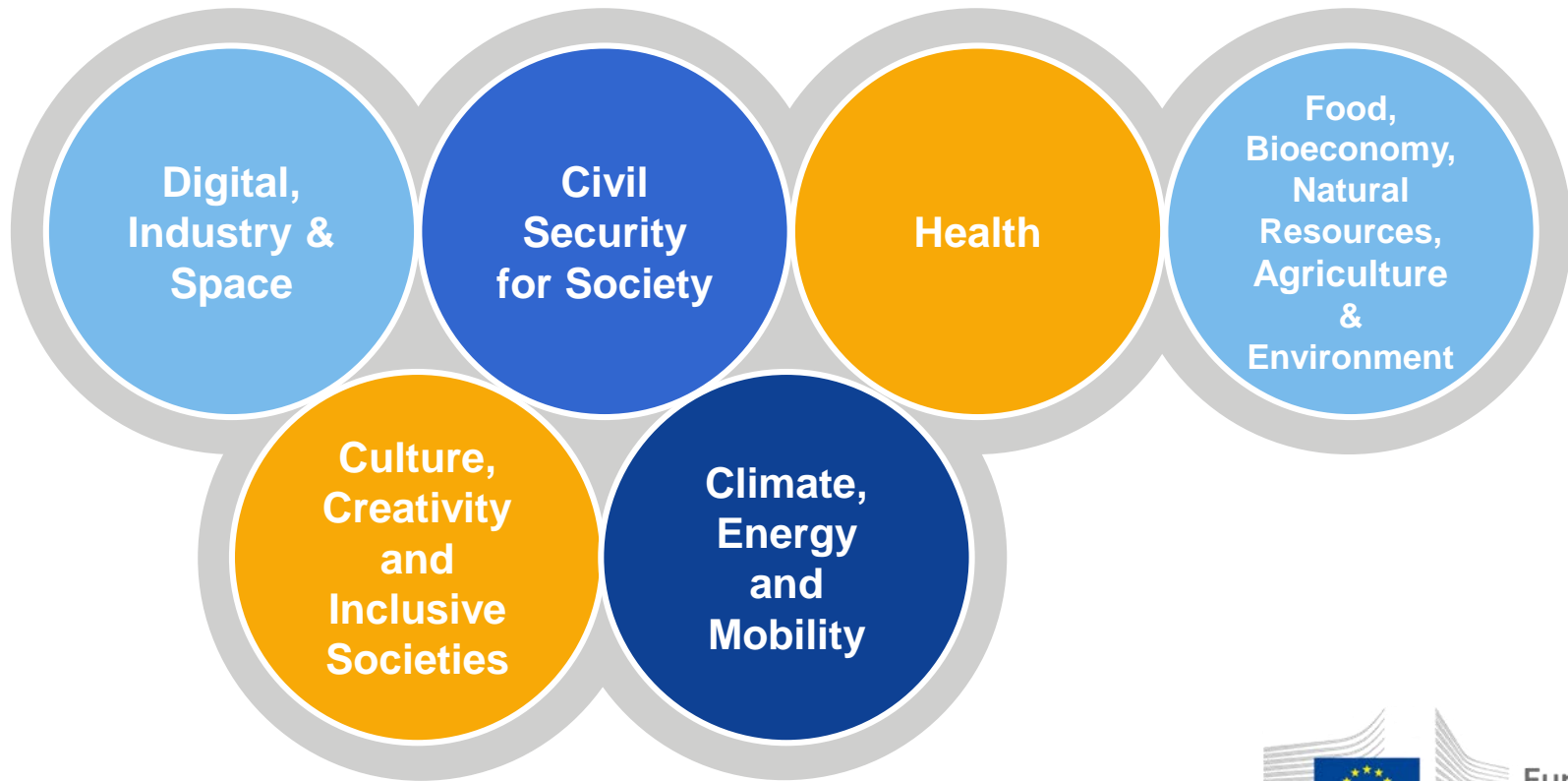
**Commission proposal:**  
**€ 2.4 billion**

## Pillar 2 - Clusters

# Global Challenges & European Industrial

**Competitiveness:** boosting key technologies and solutions underpinning EU policies & Sustainable Development Goals

**Commission proposal for budget: € 52.7 billion**





## Pillar 3

# INNOVATIVE EUROPE:

stimulating market-creating breakthroughs and ecosystems conducive to innovation

### European Innovation Council

- Support to innovations with breakthrough and market creating potential

**Commission proposal: € 10.5 billion, incl. up to € 500 million for ecosystems**

### European innovation ecosystems

- Connecting with regional and national innovation actors

### European Institute of Innovation and Technology (EIT)

- Bringing key actors (research, education and business) together around a common goal for nurturing innovation

**Commission proposal: € 3 billion**

# **Widening Participation and Strengthening the European Research Area:** optimising strengths & potential for a more innovative Europe

## **Widening Participation and Spreading Excellence, e.g.**

- Teaming & twinning
- ERA Chairs
- COST
- Support to NCPs
- Brain circulation and excellence initiatives
- “Hop-on“

**Common understanding: At least 3.3 % of Horizon Europe budget**

## **Reforming and enhancing the European R&I system**

- Scientific evidence & foresight
- Open Science
- Policy Support Facility
- Attractive researcher careers
- Citizen science, Responsible Research & Innovation
- Gender equality

# InvestEU for Research and Innovation (R&I)

**Stimulates more investment in research and innovation**, notably by the private sector; leverages and complements national/regional initiatives

**No market distortion:** intervention only to address financing gaps in the R&I delivery chain (notably due to high risk)

**Support through:**

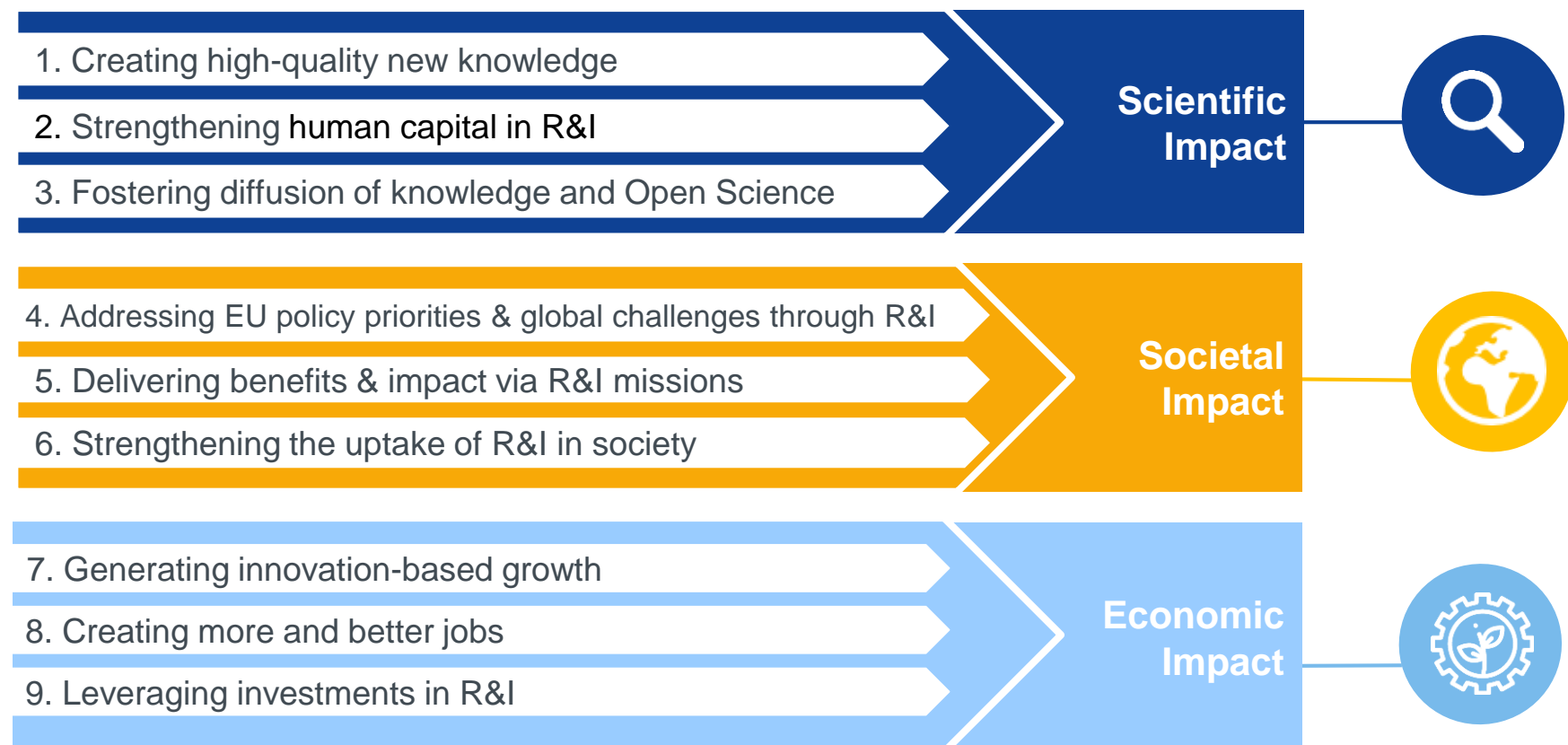
- **InvestEU Fund**
- **InvestEU Assistance**
- **InvestEU Portal**

Leverages an estimated **€ 200 bn** of investments into R&I; **market-based finance** for the exploitation and scale-up of European R&I

# Horizon Europe – Impact, flexibility, co-design



# Key impact pathways to track progress



# Strategic Plan for implementing Horizon Europe

The Strategic Plan (*new implementing act by the Commission*) will prepare the content in the work programmes and calls for proposals for the first four years

- Strategic orientation for R&I support, expected impacts
- Partnerships and missions
- Areas for international cooperation
- Issues such as:
  - Balance between research and innovation
  - Social Sciences and Humanities
  - Key Enabling Technologies and strategic value chains
  - Gender
  - Ethics and integrity
  - Dissemination and exploitation

# Strategic plan gives direction to the work programme



## Main Features

**Early involvement and extensive exchanges with Member States**

**Extensive exchanges with the European Parliament**

**Consultations with stakeholders and public at large**

# Health in Horizon Europe

# Rationale for EU funded health R&I

*Everyone has the right to timely access to affordable healthcare of good quality\**

## Newly emerging or persisting health challenges:

- Threats to citizens and public health
- Sustainability of social and health care systems
- Competitiveness of EU's health and care industry

**These challenges are complex, interlinked and global**

## The Health Cluster builds linkages between:

- Discovery, clinical, epidemiological, environmental and socio-economic research
- Academia – industry – healthcare providers – patients
- Expertise within the EU and beyond

\* EU Pillar of Social Rights, UN SDGs



# Facts and figures on health

- 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**.

# Six main 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

# What is new?

- **Digitalisation and personalisation** of health and care cut across all intervention areas
- **Health economics and health systems** are key for uptake of results and achieving impact
- **Patient-centered solutions and technologies** for health and care call for integrated approaches from medicines to medical devices (supported in Horizon 2020 under the pillar 'Leadership in Enabling and Industrial Technologies')

# Paediatric Research

## current figures and prospects

# CHILD HEALTH in FP7

**Funding efforts: 668 million euro, 141 projects**

<i>Off-patent paediatric drugs</i>	<i>21 projects, € 108M</i>
<i>Rare diseases</i>	<i>19 projects, € 90M</i>
<i>Brain and brain-related diseases</i>	<i>13 projects, € 79M</i>
<i>Diabetes and obesity</i>	<i>9 projects, € 38M</i>
<i>Cancer</i>	<i>4 projects, € 29M</i>
<i>Potentially new and re-emerging epidemics</i>	<i>2 projects, € 29M</i>
<i>Other chronic diseases</i>	<i>4 projects, € 24M</i>
<i>Neglected infectious diseases</i>	<i>2 projects, € 16M</i>
<i>HIV/AIDS, malaria and tuberculosis</i>	<i>5 projects, € 19M</i>
<i>Promoting healthy behaviour in children and adolescents</i>	<i>5 projects, € 13,5M</i>
<i>Adverse drug reaction research</i>	<i>4 projects, € 12M</i>
<i>Large scale data gathering</i>	<i>1 project, € 12M</i>
<i>Safety and efficacy of therapeutic vaccines</i>	<i>1 project, € 6M</i>
<i>Impact and cost-effectiveness of existing health programmes</i>	<i>2 projects, € 5,8M</i>
<i>Child and adolescent mental health</i>	<i>2 projects, € 5,6M</i>
<i>Identifying patients' needs in the clinical trial context</i>	<i>2 projects, € 1,4M</i>

*... and many other areas and projects*



# CHILD-related PROJECTS in HORIZON 2020

(July 2020, preliminary figures for all projects involving children)

Row Label ▼	Count of Project	Sum of Project Requested EU Contrib
☐ CNECT	37	138.606.439,64 €
☐ EASME	177	107.354.026,55 €
☐ ERCEA	238	393.018.292,98 €
☐ IMI2	11	271.281.166,71 €
☐ INEA	5	20.213.150,76 €
☐ REA	422	352.498.606,80 €
☐ RTD	81	512.450.410,05 €
Grand Total	971	1.795.422.093,49 €

# IMI – advancing paediatric research

**New taxonomy**  
**Better understanding of disease**

**New endpoints,**  
**Clinical networks**  
**Innovative trials designs**  
**Modelling/simulation**

\* finished

\* not specific  
paediatric

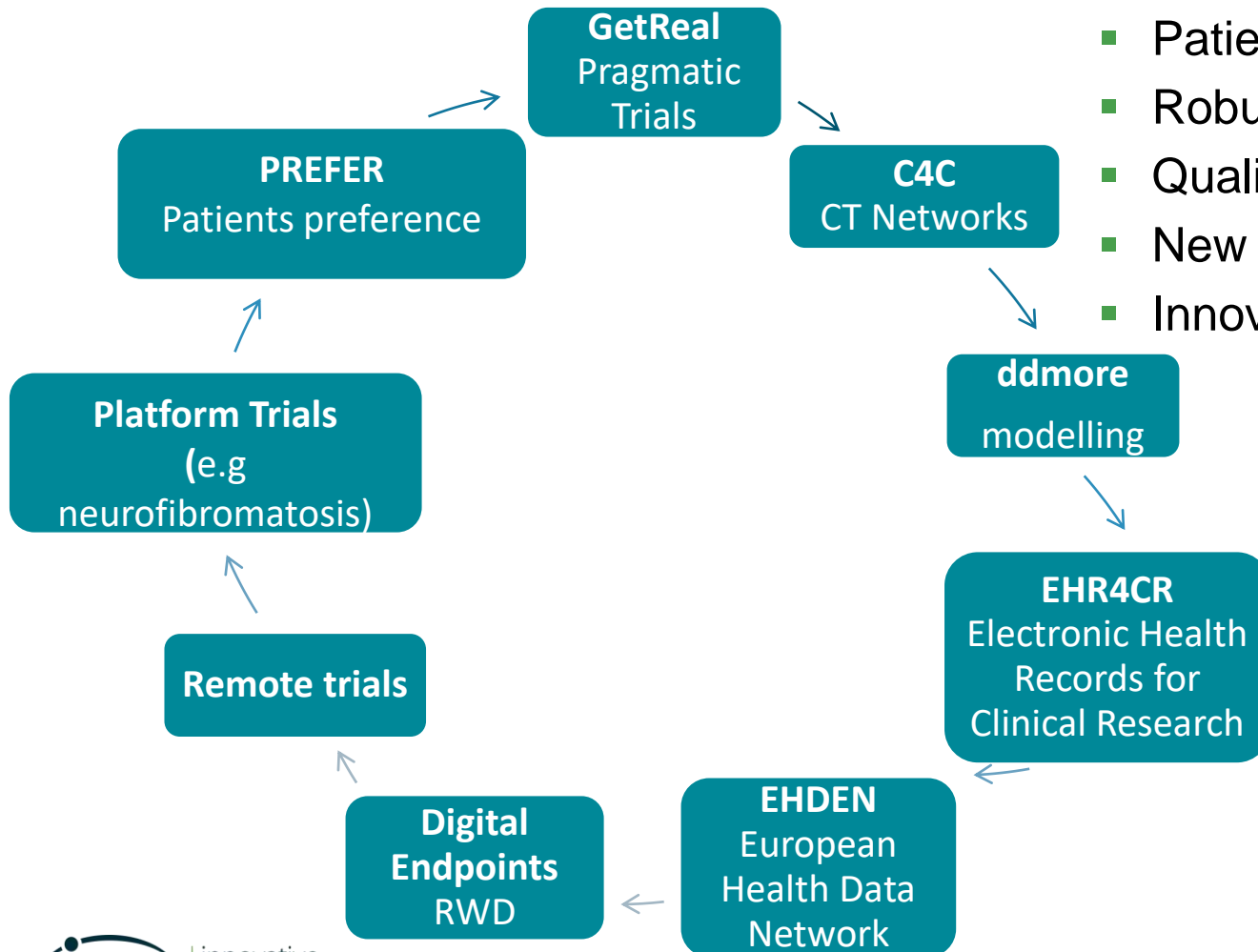


**New Targets**  
**New hits-to-lead**  
**candidates**  
**Data robustness**

**New models**  
**Novel biomarkers**  
**Predictive models for**  
**efficacy**  
**Novel assays**

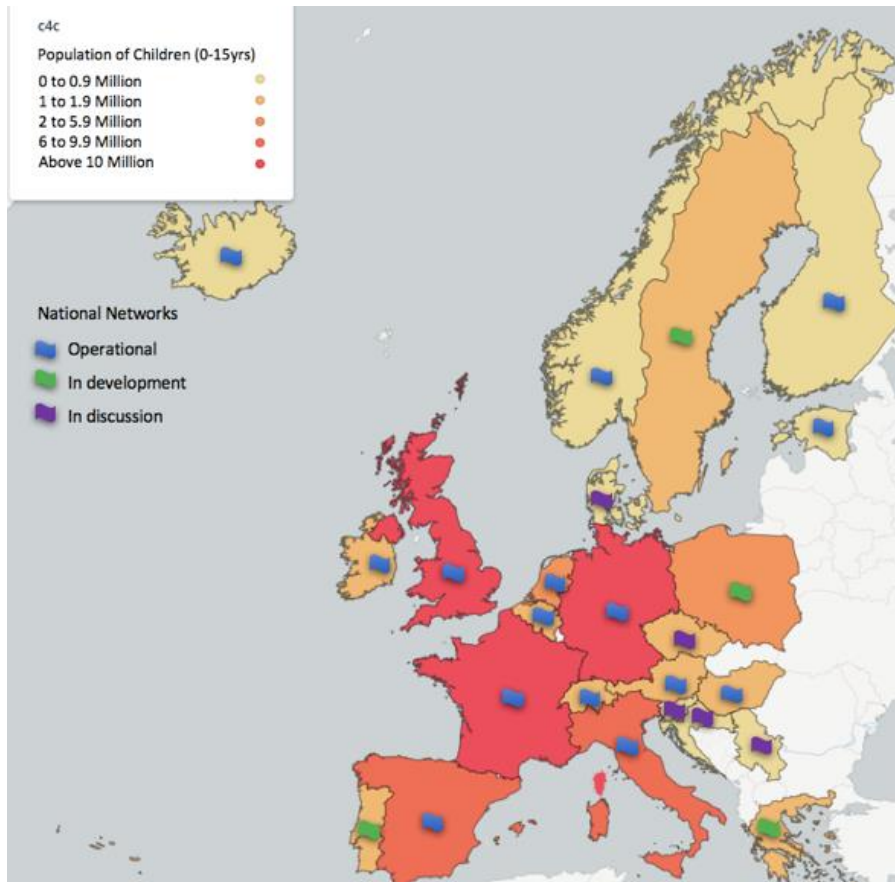
**Benefit risk**  
**methodologies**  
**New outcome measures**  
**Real World Evidence**  
**Patient engagement**

# IMI Programme approach: improving clinical trials in children



- Patient centric approach
- Robust CT infrastructure
- Quality and connected data
- New sources of data
- Innovative approaches

# Connect4children - collaborative network for European clinical trials for children



- 6 years project (1<sup>st</sup> May 2018 start)
- Budget €140,5M
- 10 EFPIA companies
- 30 academics (=4 linked third parties)
- 1 SME
- 2 large patient advocacy groups

Beneficiaries connected to:

- 18 paediatric national networks
- 8 EU multinational specialty networks
- 3 global research networks
- ~ 200 large children's hospitals
- European reference networks

## c4c - objective

To generate a sustainable infrastructure that optimises the delivery of clinical trials in children through:

- a single point of contact for all sponsors, sites and investigators;
- efficient implementation of trials adopting consistent approaches, aligned quality standards and coordination of sites at national and international level;
- collaboration with specialist networks;
- high-quality input to study design and preparation through rigorous strategic and operational feasibility assessment;
- the promotion of innovative methodologies.



c4c is focused on enhanced study delivery, through efficient implementation and high-quality design.



# ITCC-P4 - paediatric preclinical proof of concept platform

- 5 years project (1<sup>st</sup> January 2017 start)
- Budget €16,5M
- 25 partners



2 SMEs  
6 EFPIA partners  
13 academics + 4 third parties

- Overall aim: to address the significant preclinical gap in identifying promising molecules to fight paediatric cancer



<https://www.itccp4.eu/>

# ITCC-P4 - objective

- establish a representative collection of patient-derived in vitro and in vivo models (PDX and genetic mouse models) (~400) of the most common paediatric solid high-risk entities (incl. models from relapses)
- molecularly characterise the models + matching primary tumour samples and germline controls with state-of-the-art molecular diagnostic tools
- enable EU regulatory filings through development of comprehensive preclinical data packages necessary to move drugs into clinical trials
- prioritise paediatric drug development using existing collections of molecular data for systematic target reports, followed by in vivo drug testing in faithful disease models including pharmacokinetics and pharmacodynamics where necessary (e.g., brain penetration)
- identify suitable biomarkers for future clinical stratification of patients

# PAEDIATRIC REGULATION

## Evaluation 2007-2017

- stimulated the development and availability of medicines for children
- redirected private and public investment in previously neglected areas through incentives, obligations and rewards
- increased the number of clinical trials in which children are recruited
- reduced the 'off-label' use of medicines for adults in children
- favored the creation of a 'paediatric research environment' in Europe
- the development of new medicines for children remains mainly driven by adults' needs and it does not necessarily address the highest therapeutic needs of children
- the extension of the Supplementary Protection Certificate 'SPC' partially fulfilled its role, but was not convincing in fostering drug development where the adult formulation is not attractive
- other rewards provided by the Regulation have rarely been used
- any future solution should aim at a balance between fostering innovation and ensuring the availability of and access to medicines for children.



# Get prepared - free of charge services to assist you

- **Call text, guide for applicants, FAQ: Participant portal**  
<http://ec.europa.eu/research/participants/portal/desktop/en/home.html>
- **Practical information & assistance: National Contact Points**  
[http://ec.europa.eu/research/participants/portal/desktop/en/support/national\\_contact\\_points.html](http://ec.europa.eu/research/participants/portal/desktop/en/support/national_contact_points.html)
- **Find partners, training, brokerage: Fit for Health 2.0**  
<http://www.fitforhealth.eu/>
- **Find out what projects are or have been funded: EU Health projects database**  
[http://cordis.europa.eu/projects/home\\_en.html](http://cordis.europa.eu/projects/home_en.html)
- **IP questions and advices: IPR helpdesk**  
<https://www.iprhelphdesk.eu/>
- **SME regulatory one stop shop: EMA SME office**  
[http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general\\_content\\_000059.jsp](http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000059.jsp)
- **Become evaluator! Select the best projects: Register as Expert**  
<http://ec.europa.eu/research/participants/portal/desktop/en/experts/index.html>
- **Loan guarantee, equity: Find out financial support available in your country**  
<http://europa.eu/youreurope/business/funding-grants/access-to-finance/>



# Thank you!

## #HorizonEU #EUmissions

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