Inclusion of rare diseases in scientific research

Rare Disease day policy event at the United Nations

21 February 2019

Irene Norstedt, Acting Director
Health Directorate
Directorate-General For Research and Innovation
European Commission
The EU funding of rare disease research

International collaboration

over 50 members: funders, industry, patients

 ✓ Launch in 2011
  • All patients to receive the diagnosis within one year
  • 1000 new therapies
  • Methodologies to assess impact of diagnoses and therapies on patients by 2027

Policies and guidelines to implement goals
New therapies and better diagnostics
H2020 collaborative projects, calls 2015 and 2017
• Research and innovation pipeline for rapid translation of research results into clinical applications and uptake in healthcare for the benefit of patients
• Research funders, universities, research institutes, research infrastructures, hospitals from ERNs and patient organisations from 35 countries (including 27 EU Member States, seven Associated Countries and Canada)
• Joint Transnational Calls for rare diseases research projects: JTC 2019 launched
• Virtual platform for rare diseases research data, information and tools
• Capacity building, training, facilitation of partnerships, validation of new methods for clinical trials

www.ejprarediseases.org
Commission proposal for
Horizon Europe
THE NEXT EU RESEARCH & INNOVATION PROGRAMME (2021 – 2027)
#HorizonEU
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 links 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
Health cluster: 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
Thank you

http://ec.europa.eu/programmes/horizon2020/
http://ec.europa.eu/research/health
http://ec.europa.eu/horizon-europe
More information on funded projects: https://cordis.europa.eu/projects/en
Calls for proposals:
http://ec.europa.eu/info/funding-tenders/opportunities/portal/screen/programmes/h2020