Personalised Medicine and Rare Diseases

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HORIZON 2020

Personalised medicine: definition

Personalised medicine refers to a medical model using characterisation of individuals' phenotypes and genotypes (e.g. molecular profiling, medical imaging, lifestyle data) for tailoring the right therapeutic strategy for the right person at the right time, and/or to determine the predisposition to disease and/or to deliver timely and targeted prevention **- H2020 WP and Council Conclusions (2015/C 421/03)**

Examples of our PM policy activities and funding

European Commission

Personalised Medicine Conference 2016

View presentations, videos and read the conference report

ec.europa.eu/permed2016

Research and Innovation

Policy

Conference 2016

IC PerMed

Omics for prevention programmes

Piloting PM approaches

IMI projects

Diagnostics

Funding

International Consortium for Personalised Medicine (IC PerMed)

WHAT

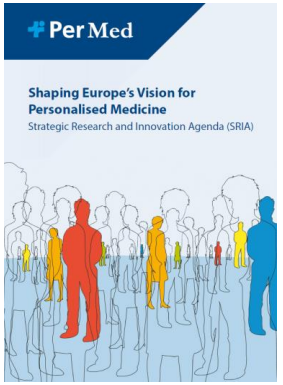
Collaboration of public & private research funders and policy makers

WHY

- **Establish Europe as a global leader in PM research**
- **Support the PM science base through a coordinated approach to research**
- **Provide evidence to demonstrate the benefit of PM to citizens and healthcare systems**
- **Pave the way for PM approaches for citizens**

HOW

Implementation of a roadmap based on PerMed Strategic Research Agenda (SRIA)



Implementing Strategic Research Agenda on Personalised Medicine (SC1-HCO-03–2017)

1 stage

- Based on FP7 funded PerMed project and continued work of IC PerMed
- ERA-NET Co-fund (for funding agencies)
- Co-funded call should implement key area of the PerMed Strategic Research Agenda
- Complementary with other funding programmes and activities at European and international levels

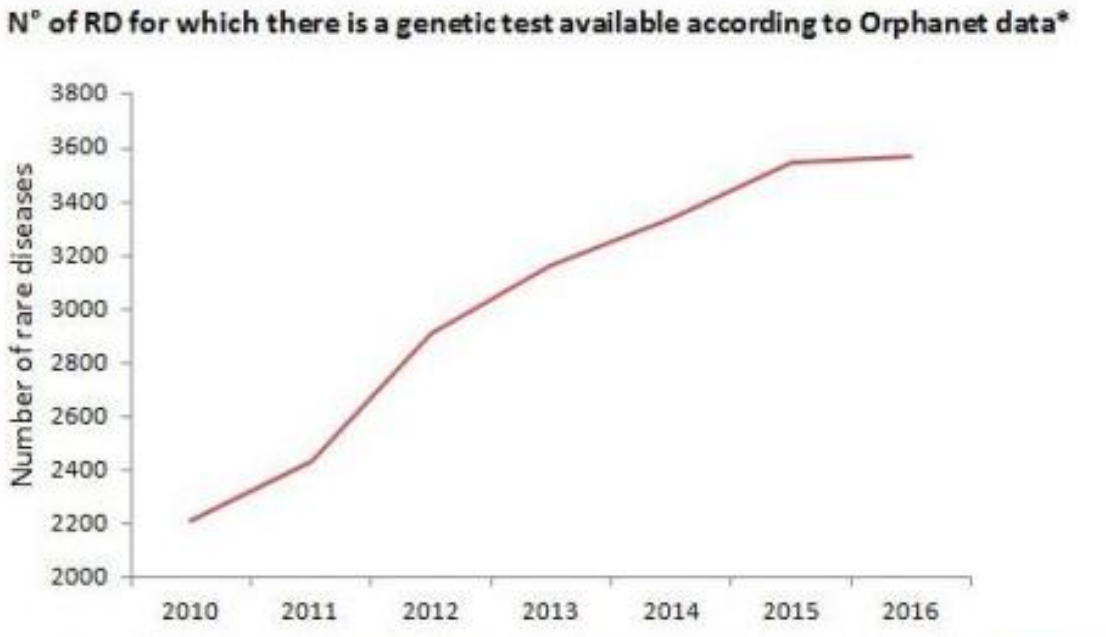
Project budget: ≈ 5 M EUR

Deadline : 11 April 2017

Information is indicative. Check topic texts against published Work Programme.

Rare diseases: a challenge too big to be mastered alone

- 6000-8000 diseases affecting 30 million people in EU
- ≈ 3600 diseases with diagnostic test
- 1329 designated orphan medicinal products
- 126 orphan medicines authorised



Source: IRDIRC based on Orphanet data – see www.irdirc.org

R&D collaboration is key

EU collaborative research funding

- Over 850 million Euro & more than 160 projects
- 29 clinical trials

E-RARE: research funders collaboration

- 25 partners from 17 EU countries and beyond
- 78 million Euro, 98 projects since 2006

www.erare.eu

IRDiRC: 200 new therapies and means to diagnose most rare diseases by 2020

- Over 40 members: funders, industry, patients
- Policies and guidelines to implement goals

IRDiRC
INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

More information:
www.irdirc.org

Research and Innovation

European Commission

Diagnostic characterisation of rare diseases (SC1-PM-03–2017)

1 stage

- Develop molecular diagnoses for a large number of undiagnosed rare diseases
- Molecular and/or functional characterisation may be part of the proposal to confirm diagnosis
- Common standards and terminologies for rare disease classification, appropriate bioinformatics tools and incentives to facilitate data sharing
- Existing resources to be used for depositing data
- Contribute to the objectives of and follow the guidelines and policies of IRDiRC

Project budget: ≈ 15 M EUR

Deadline : 11 April 2017

Information is indicative. Check topic texts against published Work Programme.

New therapies for rare diseases (SC1-PM-08–2017)

2 stage

- Focus on clinical trials on substances with orphan designation by EC
- Trial design to take into account recommendations from protocol assistance by EMA
- May include limited elements of late stage preclinical research
- Engage with patient organisations, MS health authorities and consider efficacy/potential clinical benefit, early indication on health economics
- Contribute to the objectives of and follow the guidelines and policies of IRDiRC

Information is indicative. Check topic texts against published Work Programme.

Project budget: ≈ 4 to 6 M EUR (overall 60 M EUR)

Deadlines Stage-1: 04 October 2016, Stage-2: 11 April 2017

Thank you

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http://ec.europa.eu/research/health

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