RARE DISEASES
A major unmet medical need
R&I Projects for Policy

The majority of rare diseases lack accurate diagnosis and efficient treatment.
It takes a very long time for new medicines to become available for patients with rare diseases.
Developing solutions requires sharing data and collaborating internationally and globally as patients are dispersed widely.

Improving the lives of rare disease patients requires cooperation between research and healthcare organisations.

27-36 million people in the EU have a rare disease. There are 5000 to 8000 rare diseases.

80% of rare diseases are genetic diseases.

Research for new and better solutions
The EU has invested in research on rare diseases.

€870 million
FP7 and Horizon 2020
2007-2015

164 projects have produced results on:
- gathering a critical mass of rare diseases cases and expertise for collaboration
- prevention, treatments and diagnostics for rare diseases
- tools and challenges relating to effective and ethical medical data sharing
- new approaches for clinical trials in small populations
- adapting implementation of regulatory requirements, especially for clinical trials on rare diseases
- supporting health technology assessment, standards and evidence-base for guiding public health policy
- developing legally and ethically robust agreements for collecting and exchanging health and genetic data
- collaborating globally through IRDiRC* to accelerate research on improving the lives of patients with rare diseases

*International Rare Diseases Research Consortium

Actions for policymakers

Support integration and networking among EU research, patient and healthcare organisations.
Adopt implementation of regulatory requirements, especially for clinical trials on rare diseases.
Support health technology assessment, standards and evidence-base for guiding public health policy.
Collaborate globally through IRDiRC* to accelerate research on improving the lives of patients with rare diseases.

MORE INFORMATION
Read the full R&I Projects for Policy report on Rare Diseases: http://euraxess.eu/N8460
Find all R&I Projects for Policy editions on: http://ec.europa.eu/research/p4p

Research and Innovation