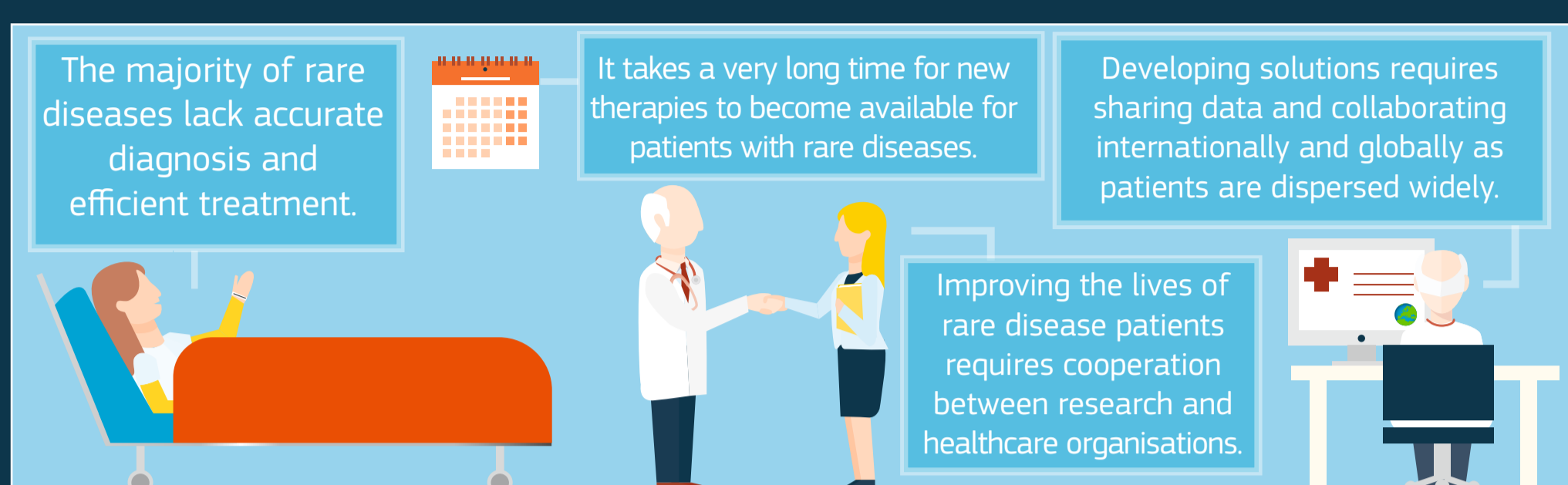


RARE DISEASES

A major unmet medical need

R&I Projects for Policy

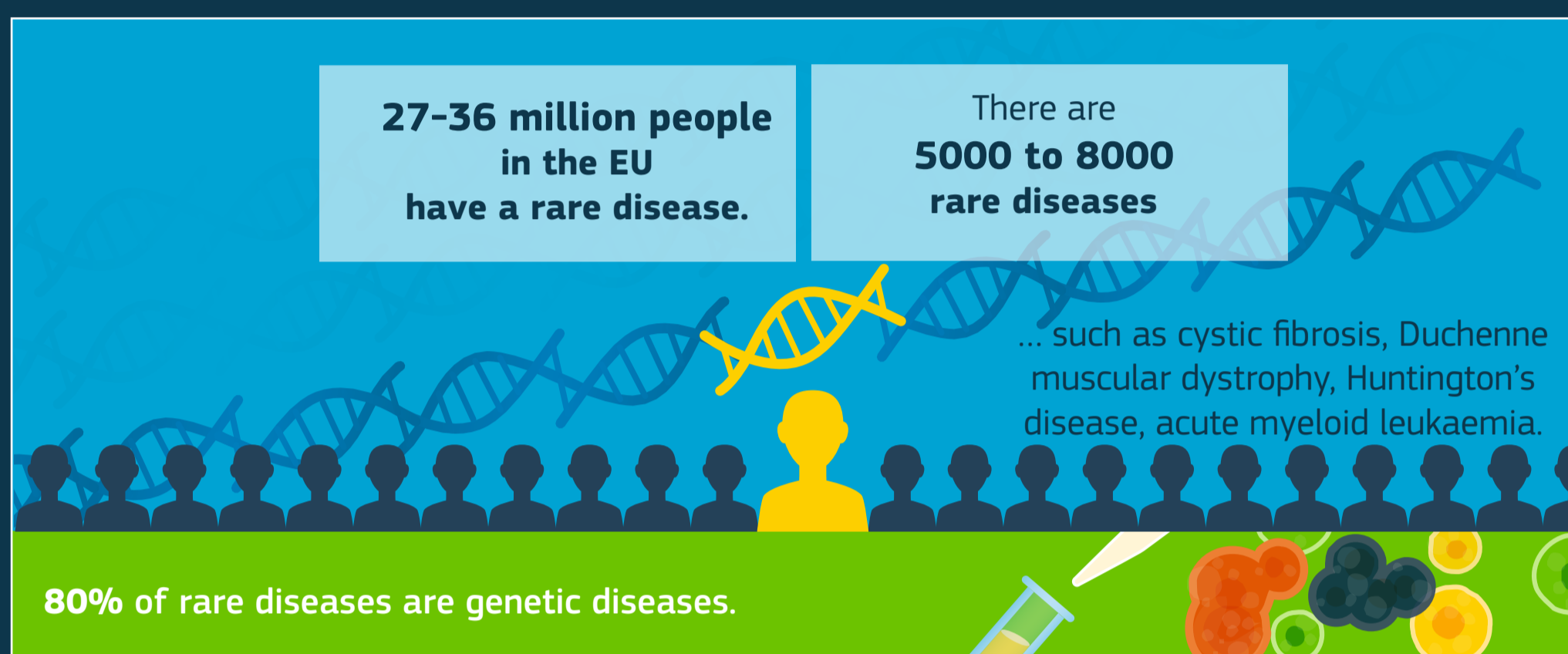


The majority of rare diseases lack accurate diagnosis and efficient treatment.

It takes a very long time for new therapies 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**

... such as cystic fibrosis, Duchenne muscular dystrophy, Huntington's disease, acute myeloid leukaemia.

80% of rare diseases are genetic diseases.



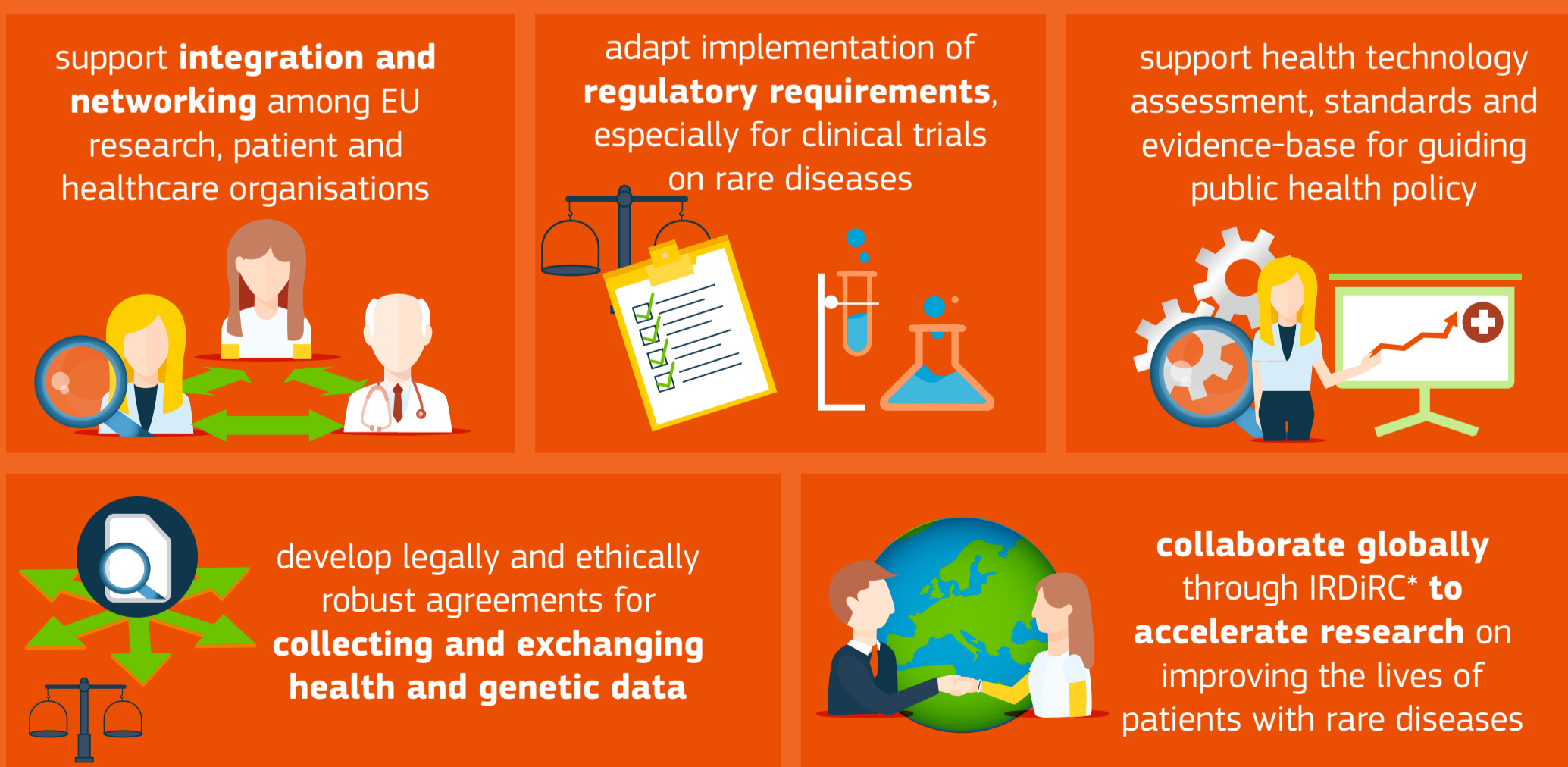
Research for new and better solutions
The EU has invested in research on rare diseases.

€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

Actions for policymakers



- support **integration and networking** among EU research, patient and healthcare organisations
- adapt implementation of **regulatory requirements**, especially for clinical trials on rare diseases
- support health technology assessment, standards and evidence-base for guiding public health policy
- develop legally and ethically robust agreements for **collecting and exchanging health and genetic data**
- collaborate globally** through IRDiRC* to **accelerate research** on improving the lives of patients with rare diseases

*International Rare Diseases Research Consortium

MORE INFORMATION

Read the full R&I Projects for Policy report on **Rare Diseases**:
<http://europa.eu/!Nu98YP>

See also:
<http://ec.europa.eu/research/health/rare>
<http://www.irdirc.org>

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