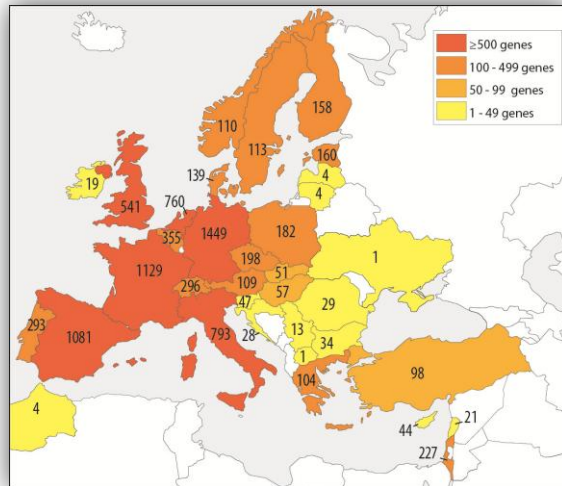




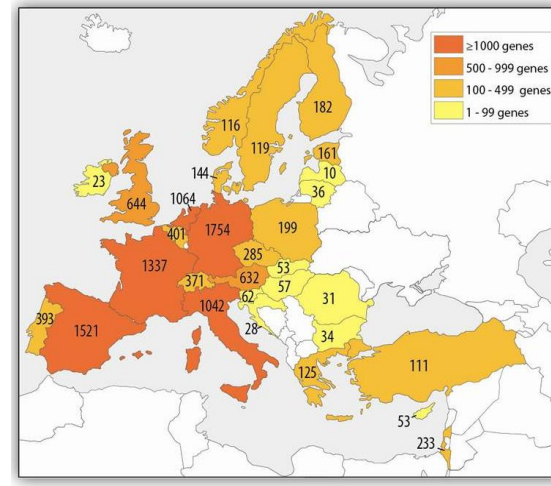
Current status of research in the field of rare diseases based on Orphanet data

Number of genes tested in each country in Europe by year

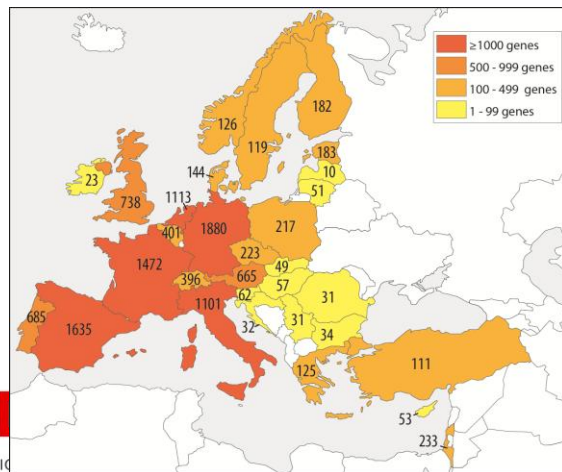
2011



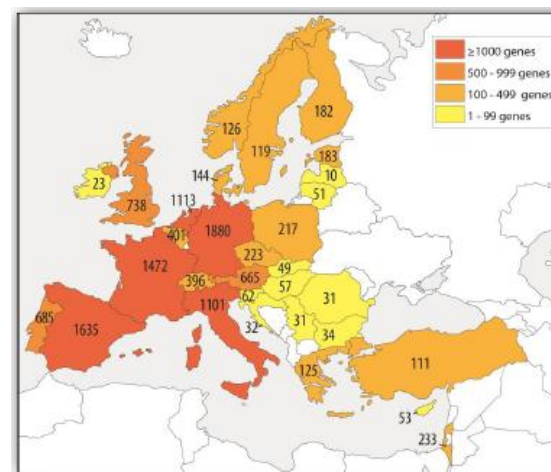
2012



2013

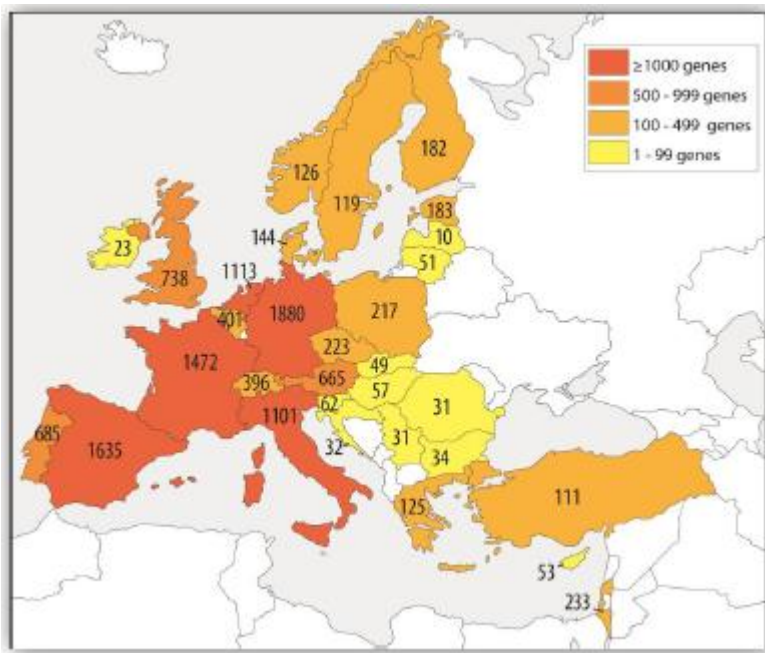


2014

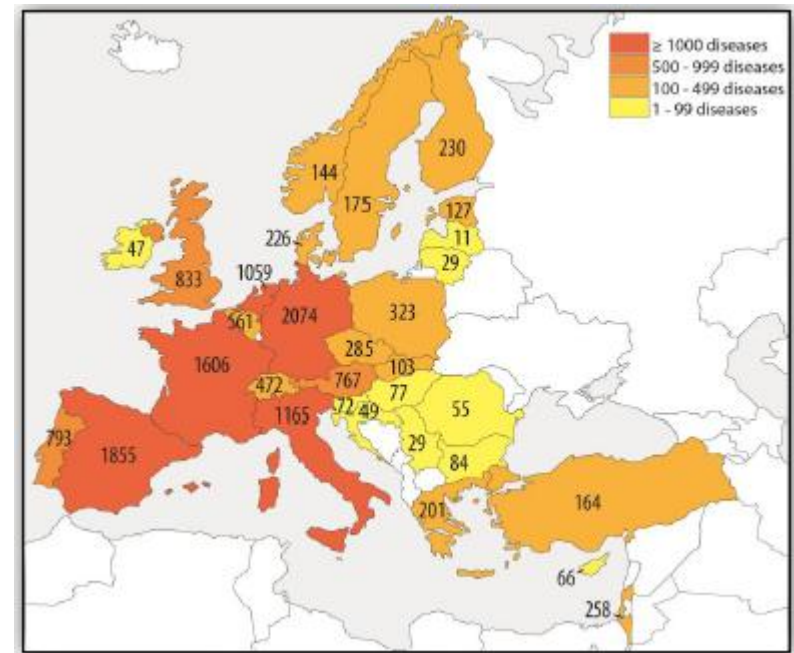


Possibility to diagnose Rare Diseases

Number of genes tested by country



Number of rare diseases tested by country



(January 2015)

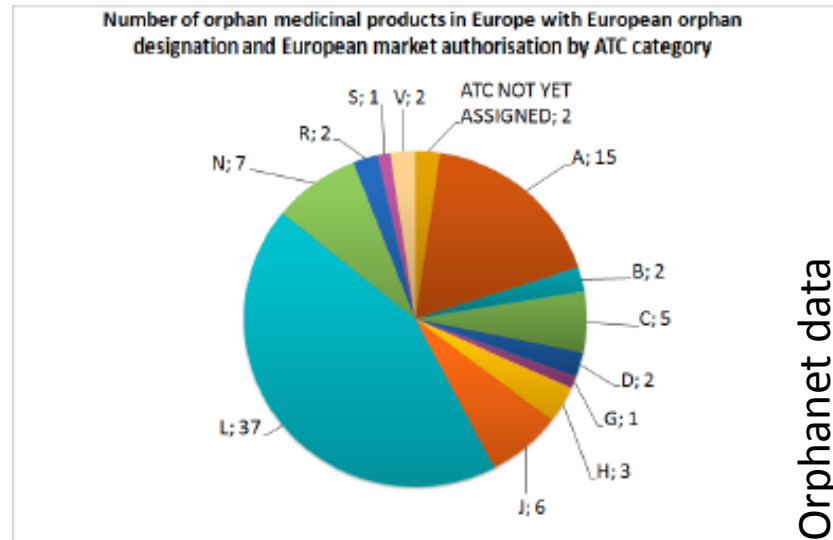
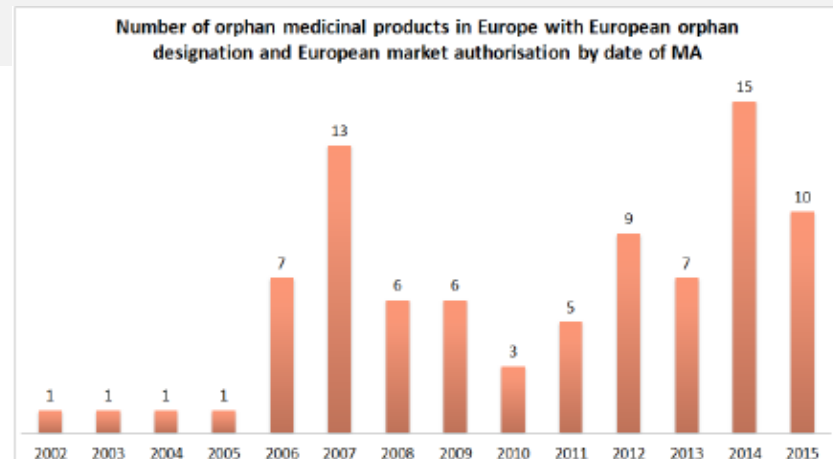
Source: Orphanet data

Need for international coordination

- ▶ Rare Diseases tested by country
 - ↪ 353 diseases tested in at least 10 countries
 - ↪ 1267 diseases tested in at least 5 countries
 - ↪ 915 diseases tested in only 1 country

- ▶ Genes tested by country
 - ↪ 331 genes tested in at least 10 countries
 - ↪ 1197 genes tested in at least 5 countries
 - ↪ 458 genes tested in only 1 country

Medicinal products on the European market

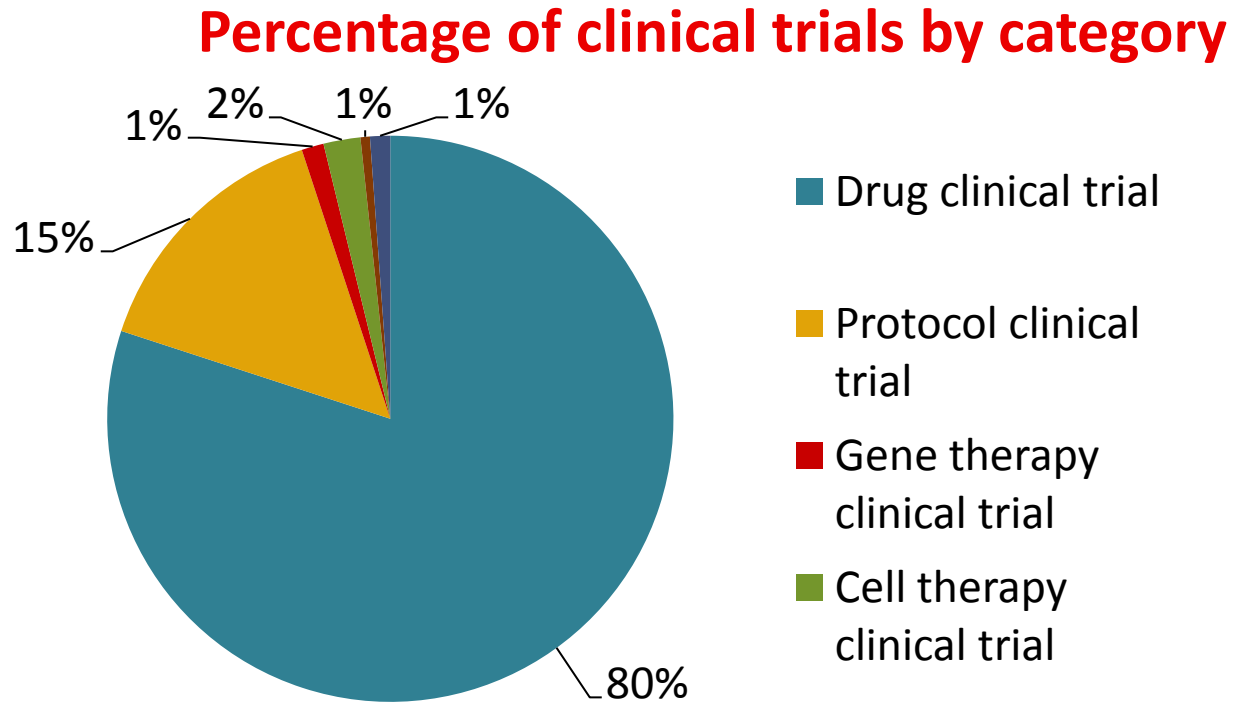


- 85 orphan medicinal products
- 115 medicinal product with an indication for a rare disease without orphan designation
- Totals 200 medicinal products for rare diseases

Source: Orphanet data

International rare diseases clinical trial landscape

- ▶ 2609 ongoing national or international clinical trials for 426 diseases in 31 countries



- Drug clinical trial
- Protocol clinical trial
- Gene therapy clinical trial
- Cell therapy clinical trial

Countries included are: EU, IL, CA, LB, TN, AU, TR, USA

(October 2015)