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

Source: Orphanet data
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

Source: Orphanet data
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 (October 2015)

Countries included are: EU, IS, LI, NO
International rare diseases clinical trial landscape

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

Percentage of clinical trials by category:

- 80% Drug clinical trial
- 15% Protocol clinical trial
- 1% Gene therapy clinical trial
- 1% Cell therapy clinical trial

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

(October 2015)