



# Orphanet: Advancing collaboration on Rare Diseases in Europe

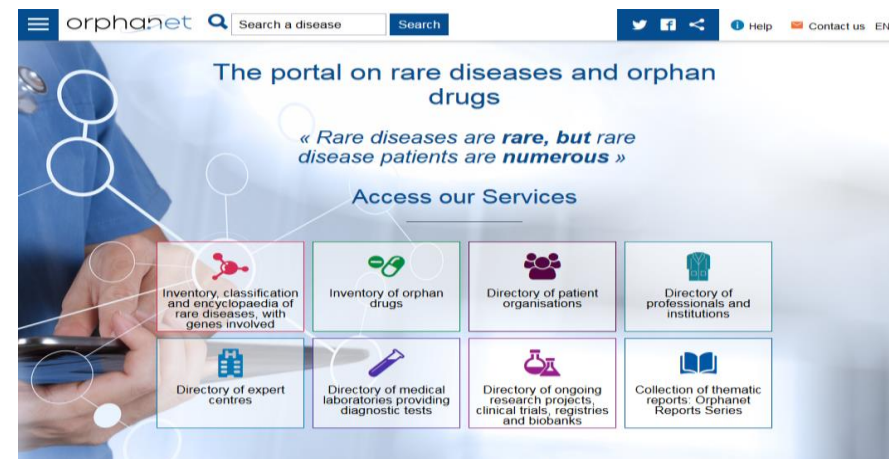
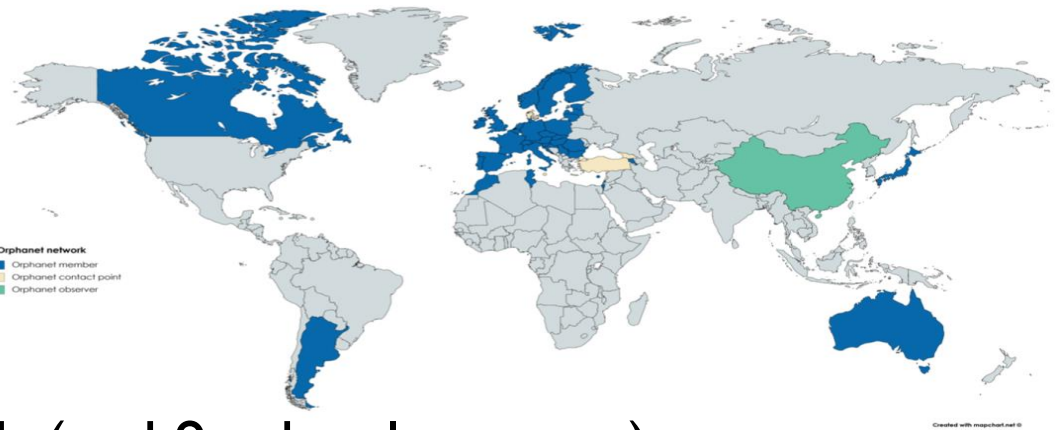
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Policy Advisor on European Affairs, Directorate General for Health

A unique initiative on Rare  
Diseases and Rare Cancers

# Orphanet's ID

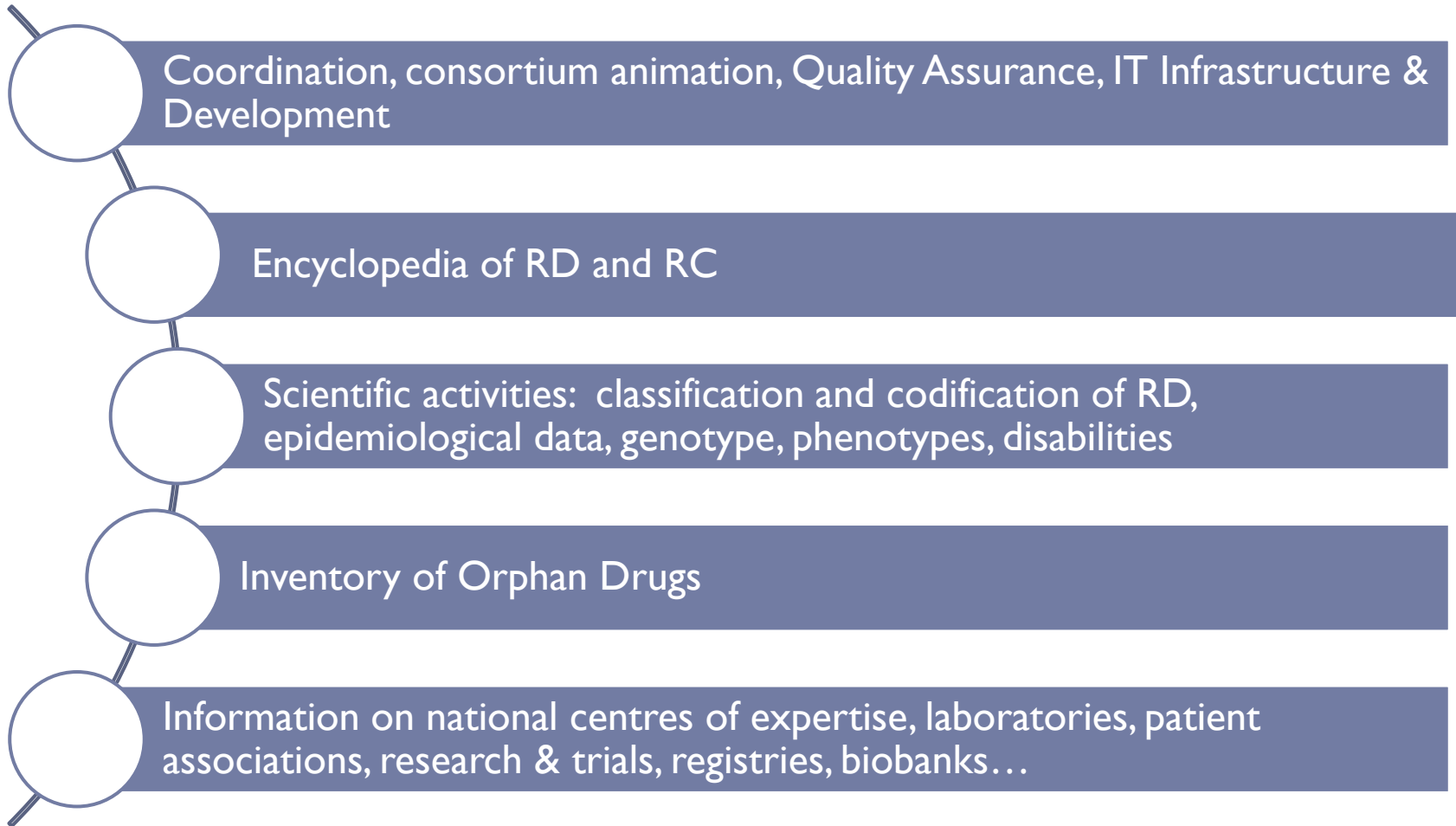
- ▶ **Date of birth:** 1997
- ▶ **Nationality:** European
- ▶ **Main language:** English (and 9 other languages)
- ▶ **Founders:** INSERM and Ministry of Health
- ▶ **Size:** 41 countries
- ▶ **Domains:** Rare Diseases, Rare Cancers
- ▶ **Missions:**
  - ▶ Improve knowledge on RD
  - ▶ Provide info on RD
  - ▶ Ensure access to info on RD
  - ▶ Develop a database of RD
  - ▶ Develop a nomenclature for RD

Orphanet network  
■ Orphanet member  
■ Orphanet contact point  
■ Orphanet observer



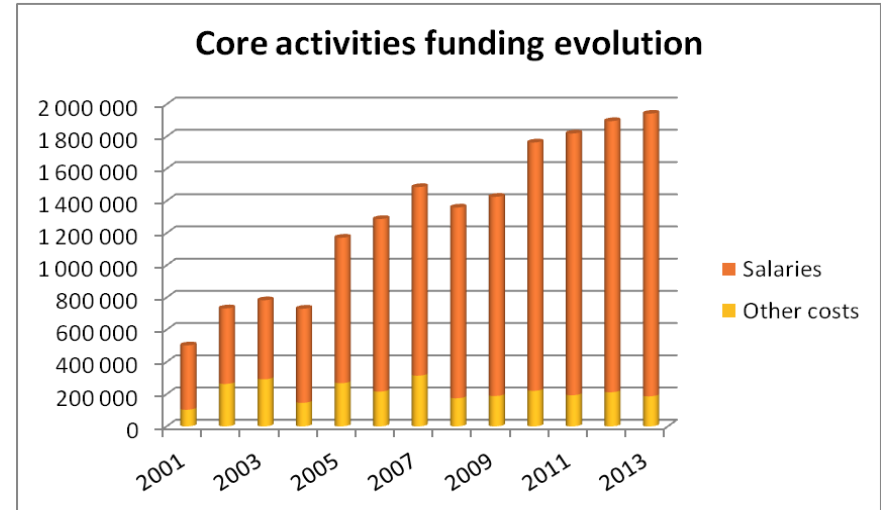
# Orphanet's activities in a nutshell

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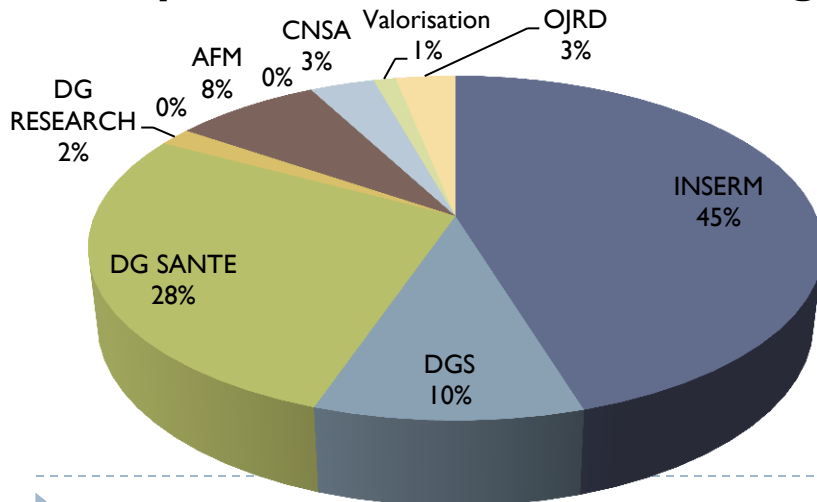


# Orphanet in figures

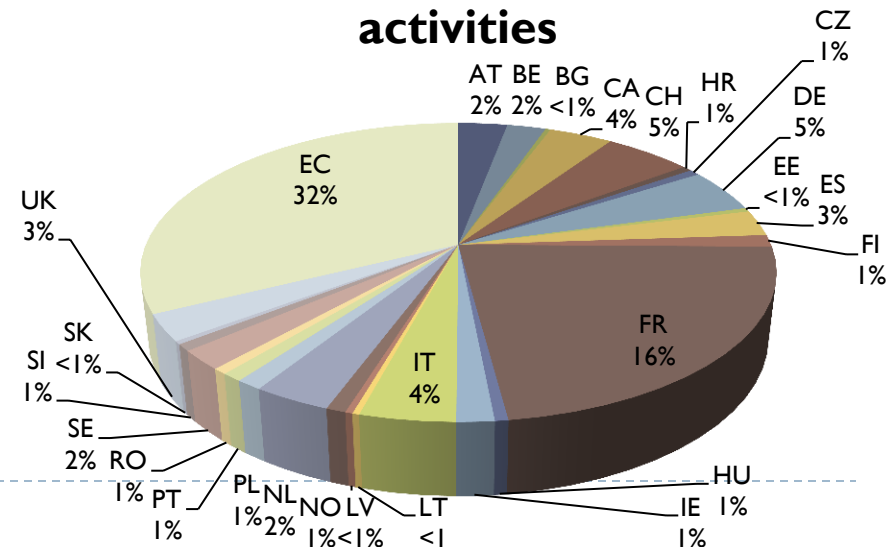
- ▶ Annual budget 3M€
- ▶ 39 staff
- ▶ 86% of fixed-term contracts
- ▶ Mixed-funding: FR, EU, MSs



### Orphanet core activities funding



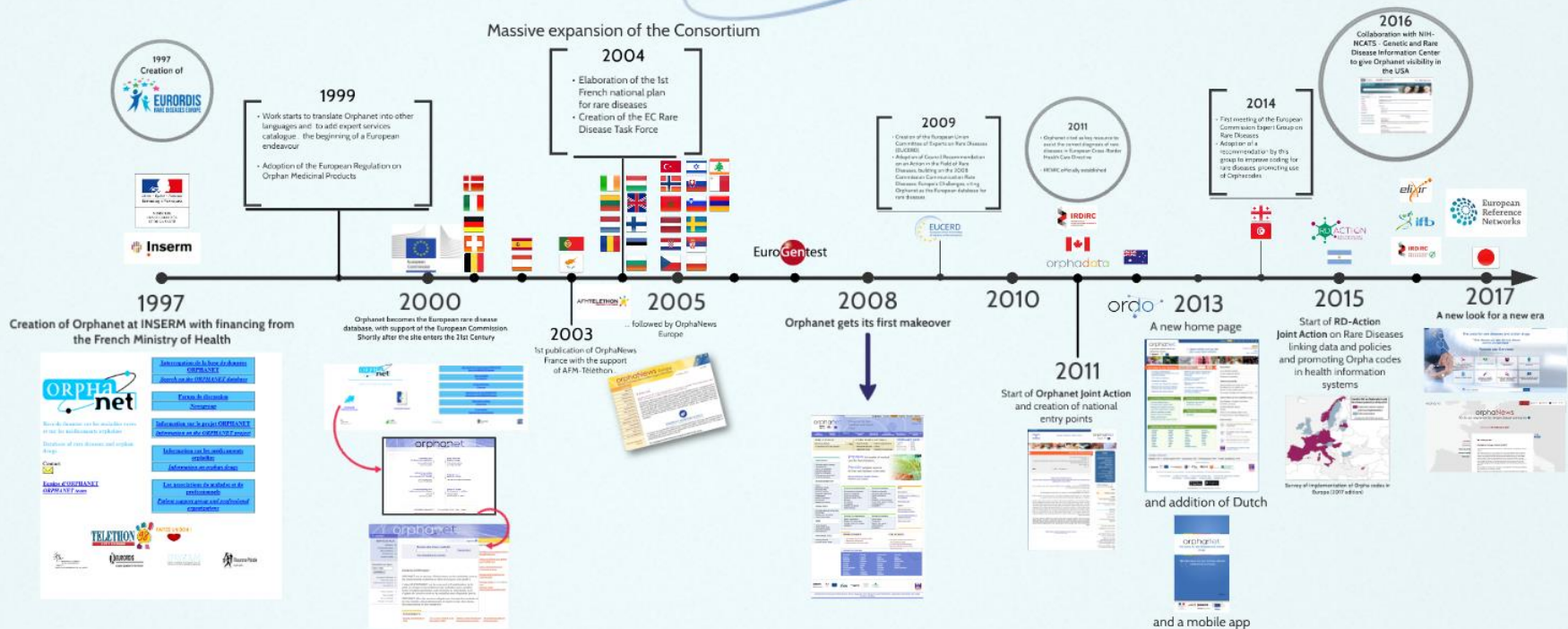
### Funding sources for national activities



Orphanet is born to be European  
and International

# 20 years of fruitful European and international cooperation

## 20 years Orphanet



# The European political context on RD

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- ▶ Commission's communication to European Parliament, Council, European Committee for regions and European Economic and Social Committee: Rare diseases, a common challenge for Europe (2008)  
Council Recommendation of 8 June 2009 on action for rare diseases
- ▶ Directive 2011/24/EU of 9 March 2011 on the application of patients' rights in cross-border healthcare – art 13
- ▶ Commission Expert Group on RDs : Recommendation on ways to improve codification for RDs in health information systems (2014)
- ▶ 24 European Reference Networks
  
- ▶ RARE DISEASES => STRONG ADDED VALUE FOR EUROPEAN COOPERATION (Research and healthcare)



# Orphanet's added value

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- ▶ Unique nomenclature on Rare Diseases and Rare Cancers – International reference for interoperability between countries, and between health and research.
- ▶ Comprehensive information on provision of healthcare for RDs (incl. Centres of expertise, experts, laboratories, orphan drugs, etc)
- ▶ Robust, accessible and high quality information (reports, newsletters, info leaflets)
- ▶ Complementarity with ERNs (knowledge generation, global visibility of ERNs activities...)
- ▶ Free of charge for users

Orphanet's future is starting now

# Main challenges

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- ▶ Continue providing free access to high quality and reliable information on RDs to patients and citizens
- ▶ Develop a sustainable cooperation model between
  - ▶ France
  - ▶ EU Member States
  - ▶ EU institutions
  - ▶ International partners
- ▶ Define a status for Orphanet at the EU level
- ▶ Secure sustainable funding

# Scenario-based future

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## Scenario 1

- France

## Scenario 2

- #1
  - France
  - EU Member States
- #2
  - France
  - EU Institutions

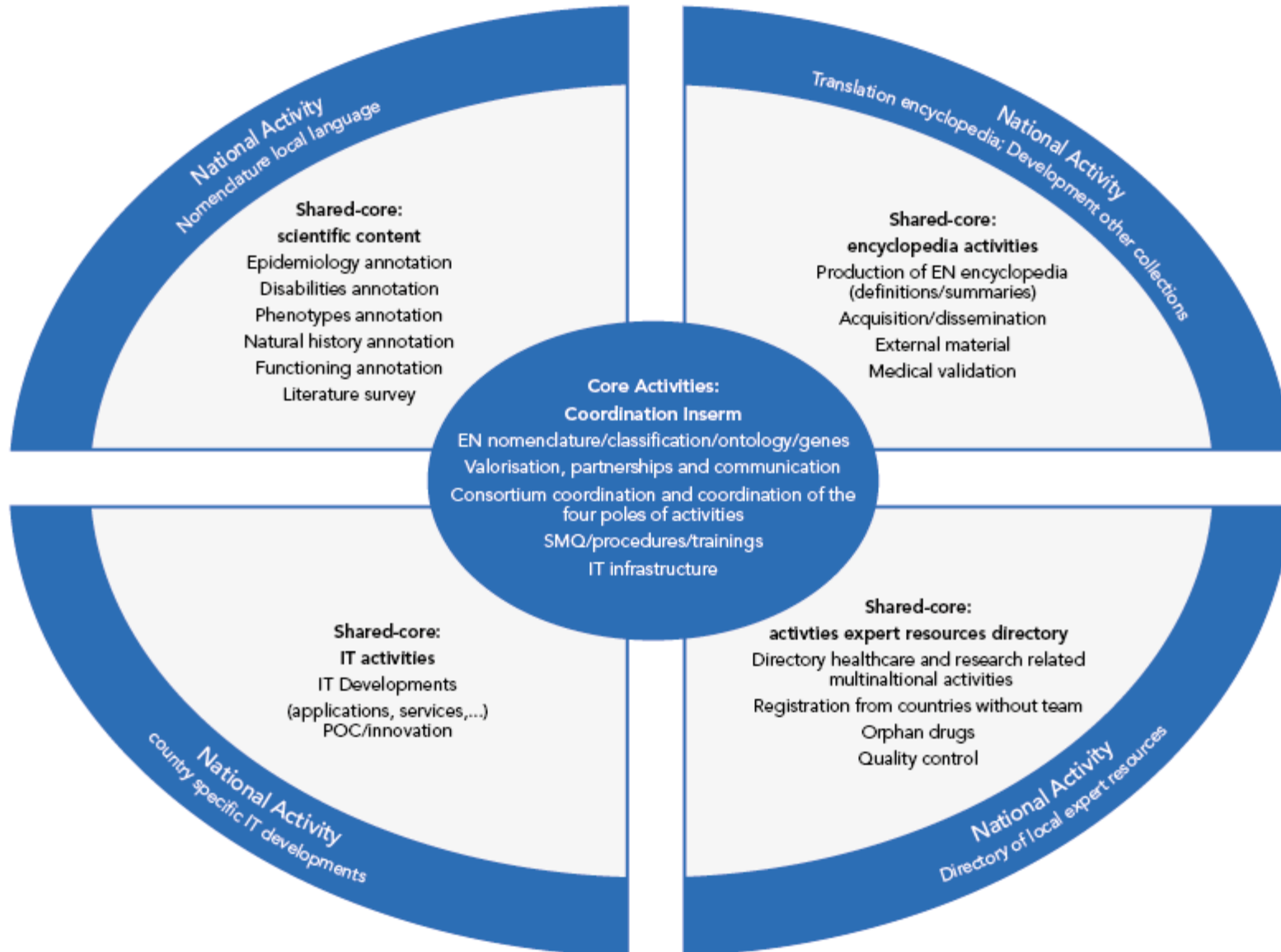
## Scenario 3

- France
- EU Member States
- EU Institutions

## Scenario 4

- France
- EU Member States
- EU Institutions
- International partners

# Possible cooperation model



# The scenario-based matrix

	<b>Actors</b>			
<b>activities</b>	<b>France</b>	<b>EU MS</b>	<b>EU institutions</b>	<b>International Partners</b>
<b>Core Activities</b>				
<i>Administrative Management</i>	X			
<i>Financial Management</i>	X			
<i>HR management</i>	X			
<i>Coordination</i>	X			
<i>Partnerships</i>	X	X		X
<i>IT database maintenance</i>	X		X	
<i>Core nomenclature and classification of RDs</i>	X	X	X	X
<b>Shared activities</b>	<b>TO BE DEFINED TOGETHER DEPENDING ON CHOSEN SCENARIO</b>			
<i>Scientific content</i>	X	X	X	X
<i>Encyclopedia</i>	X	X	X	X
<i>Expert resources</i>	X	X	X	X
<b>National Activities</b>				
<i>Classification of RDs, translation of encyclopedia in national language</i>	X	X		X
<i>Information on national provision and organization of care</i>	X	X		X



Conclusion

# Time for action

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- ▶ France to propose a scenario-based concept paper
- ▶ EU MS to decide how and where to get involved
- ▶ EU to define/identify sustainable funding options

=> Shape the European agenda on Rare Diseases and Rare  
Cancers

