

# EPIRARE: the outcomes of the professional's survey

***L. Vittozzi<sup>(1)</sup>, S. Gainotti<sup>(1)</sup>, E. Mollo<sup>(1)</sup>***

On behalf of the EPIRARE WP Leaders: *D. Taruscio<sup>(1)</sup> EPIRARE Project Coordinator, F. Bianchi<sup>(2)</sup>, A. Brand<sup>(3)</sup>, M. Ensini<sup>(4)</sup>, M. Posada<sup>(5)</sup>, R. Stefanov<sup>(6)</sup>*

*(1) Italian National Centre for Rare Diseases, Istituto Superiore di Sanità, Roma (Italy)*

*(2) Maastricht University, (The Netherlands)*

*(3) National Council of Research, Pisa (Italy)*

*(4) EURORDIS, Paris (France)*

*(5) Institute of Rare Diseases Research (IIER), Instituto de Salud Carlos III, Madrid (Spain)*

*(6) BAPES, Plovdiv (Bulgaria)*

# EPIRARE

**Co-funded by the EU Commission (DG SANCO)**

## **General objective**

- To build consensus and synergies to address regulatory, ethical and technical issues associated with the registration of RD patients and to elaborate possible policy scenarios.

## **Specific Objectives**

- To identify the **needs** of the EU registries and databases on RD
- To set up amendments to the draft **Data Protection Regulation**
- To agree on the Register and Platform **Scope, Governance and long-term sustainability**
- To agree on a **Common data set, purpose- and disease-specific** data collection and data **validation**
- To identify **tools and other facilities** supporting the operation of the platform users



**Survey among current registries on  
“Activities and needs of RD registries  
in the EU”**

**Scope and Selected Results**

# The survey of registries

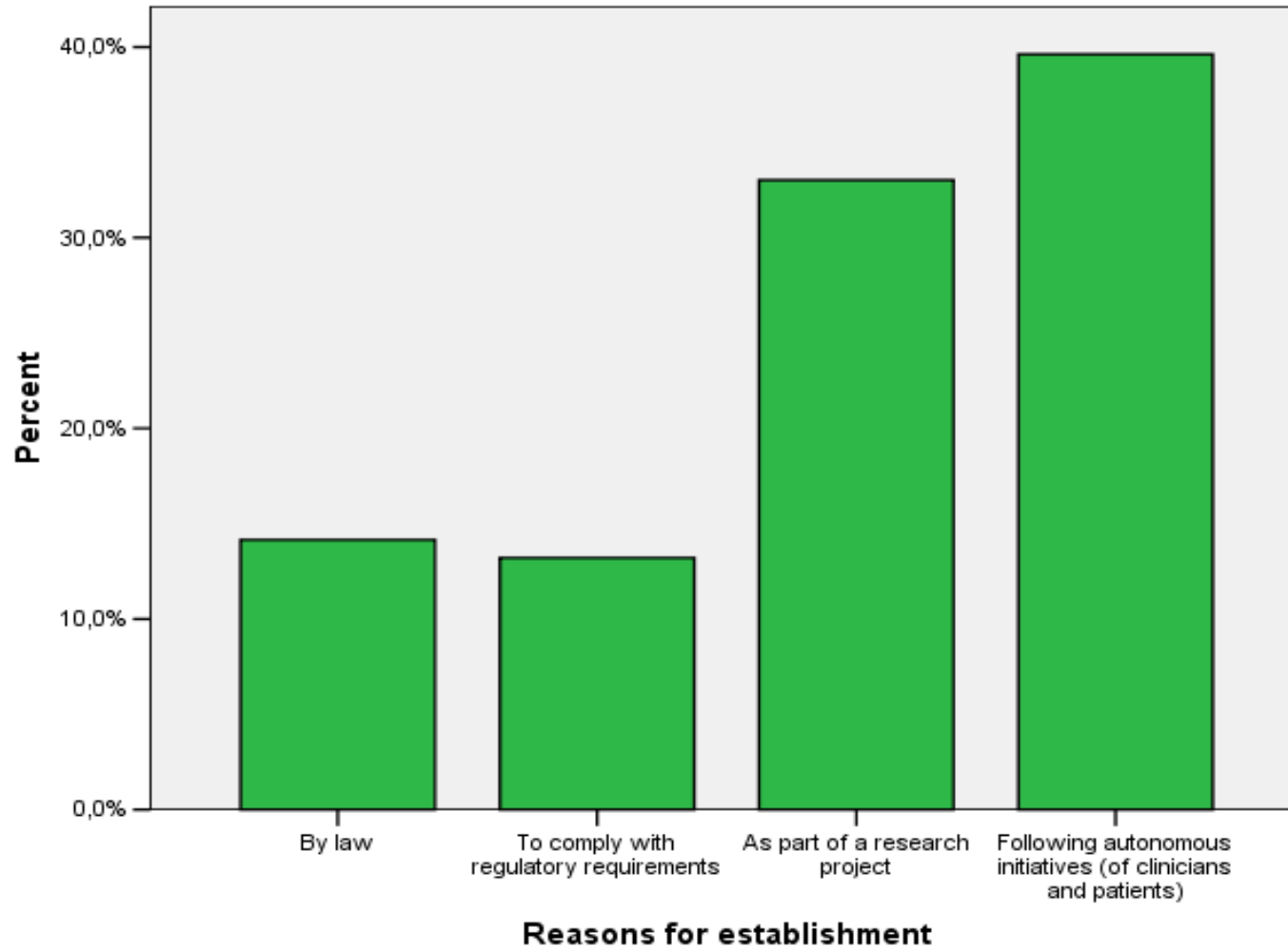
## The survey scope

- Aims and scope of existing RD registries
- Sources, data collected, methodology and quality issues
- Data protection and ethical issues
- Governance, communication and data sharing
- Financial sustainability
- Needs, expectations, opinions on the platform

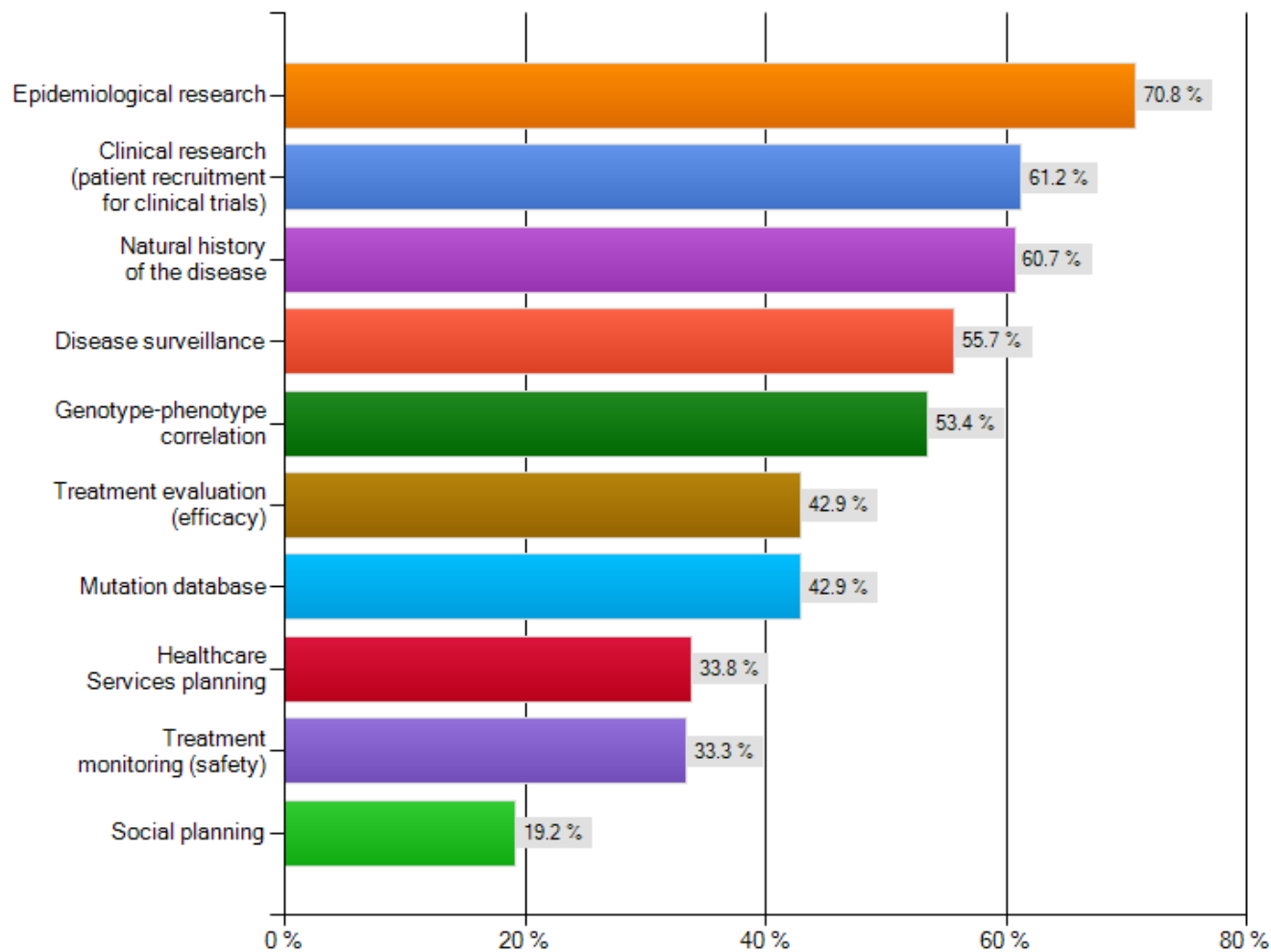
## Valid Respondents

- 220 registries

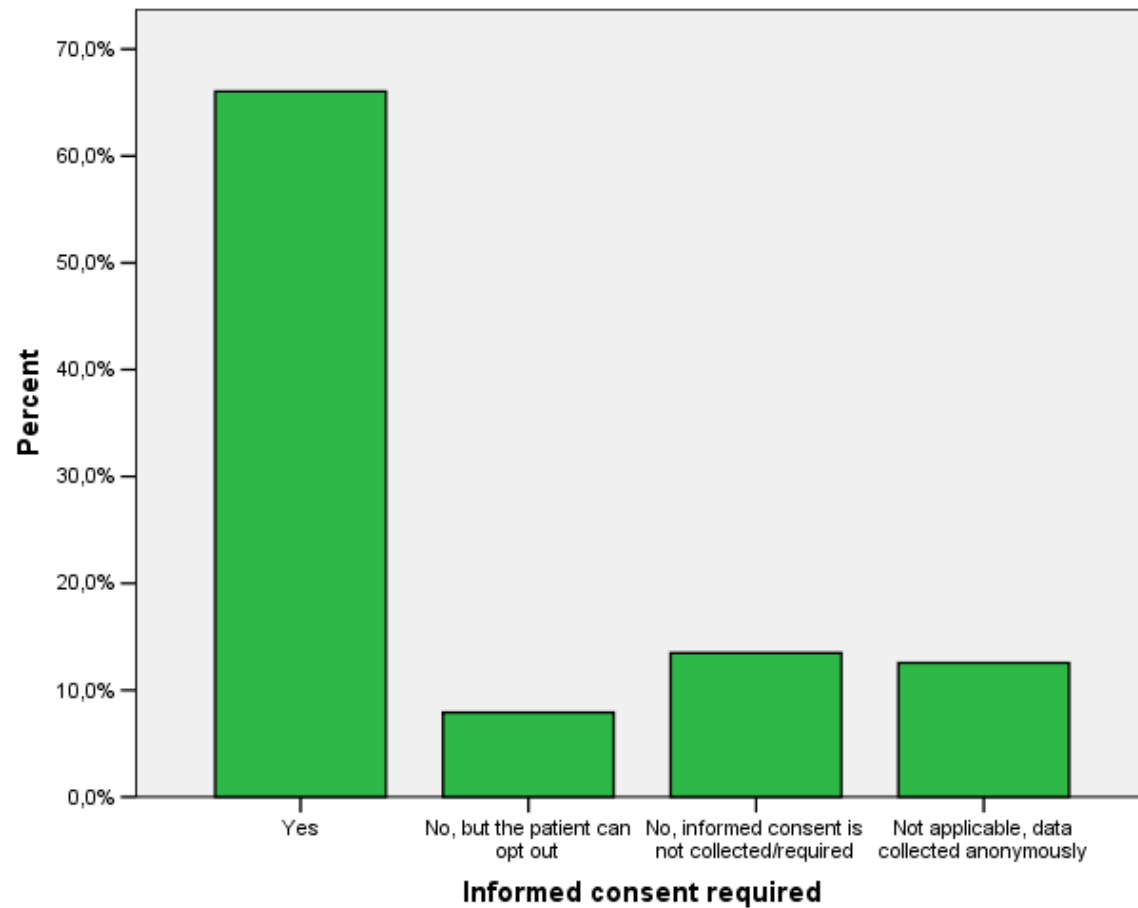
# Reason for establishment



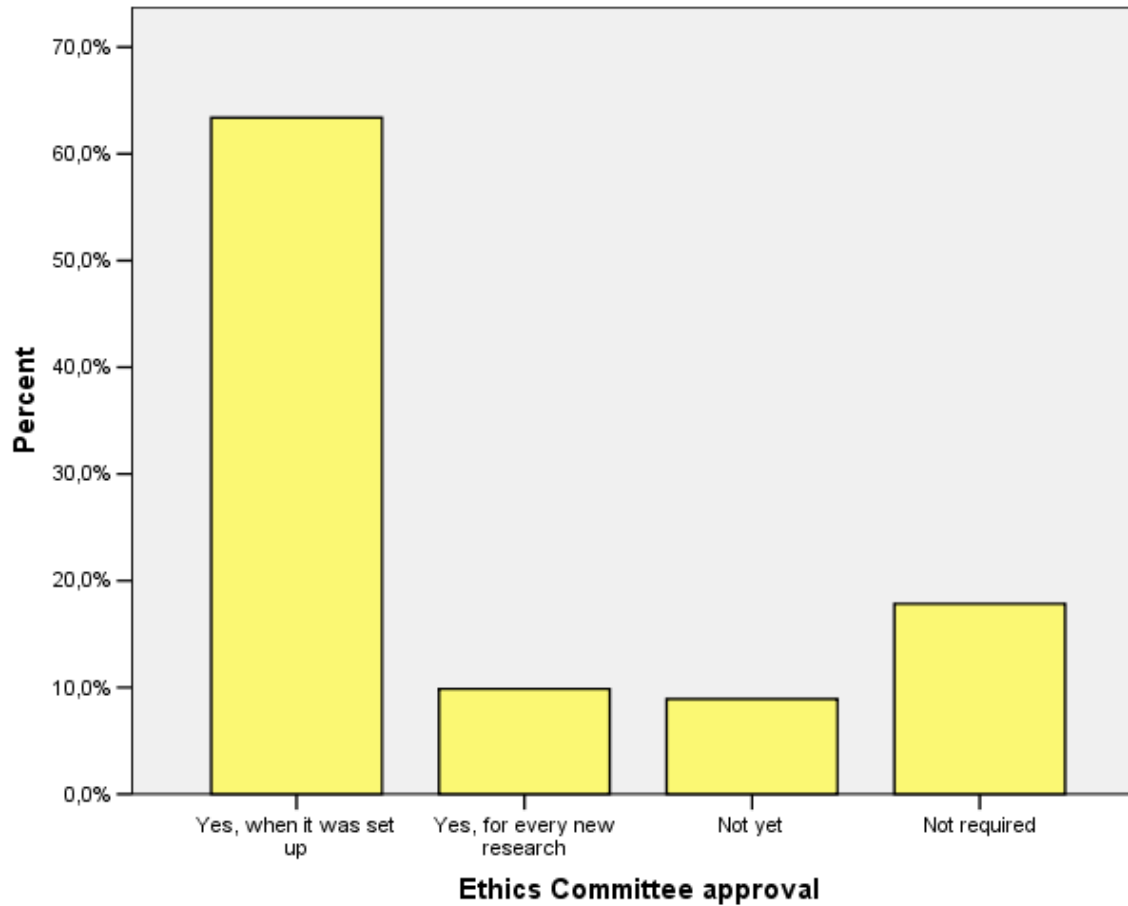
# Aims of the registry (select all that apply)



# Informed Consent

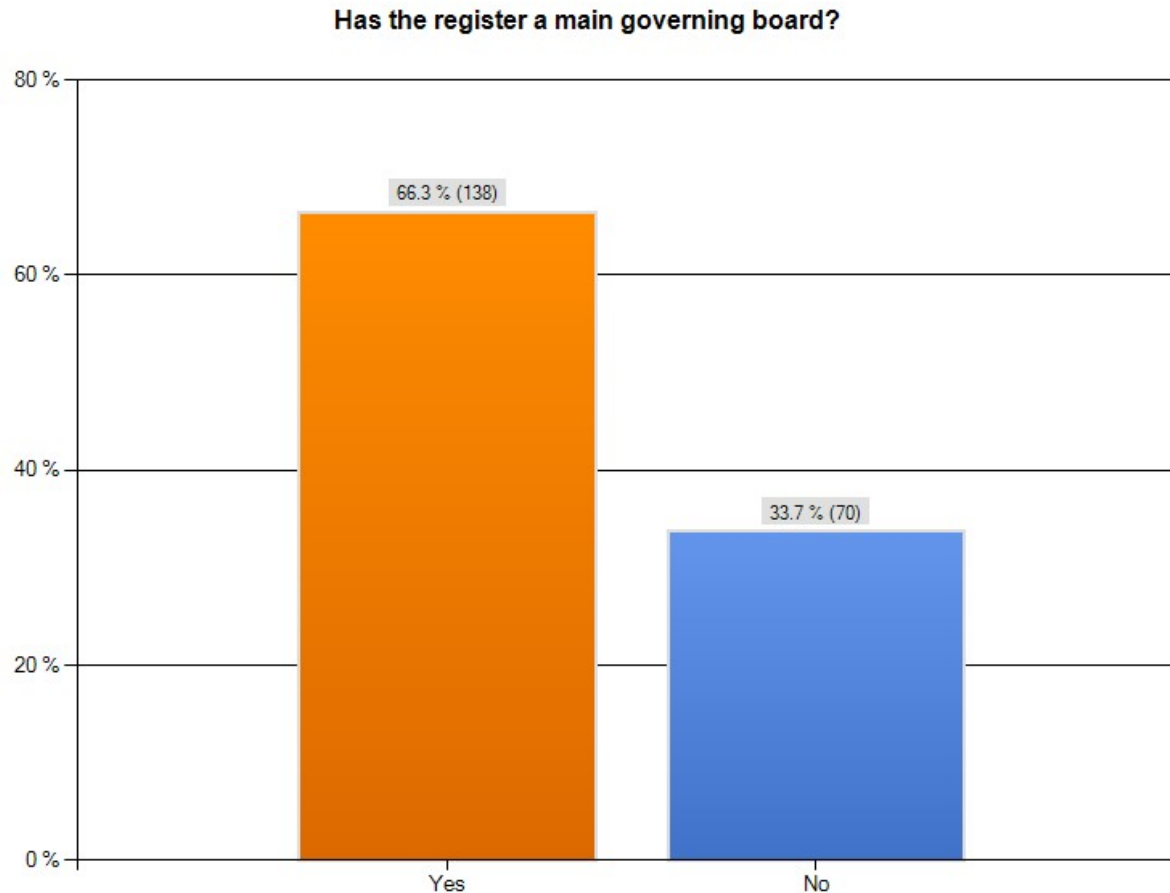


# Approval by a Research Ethics Committee

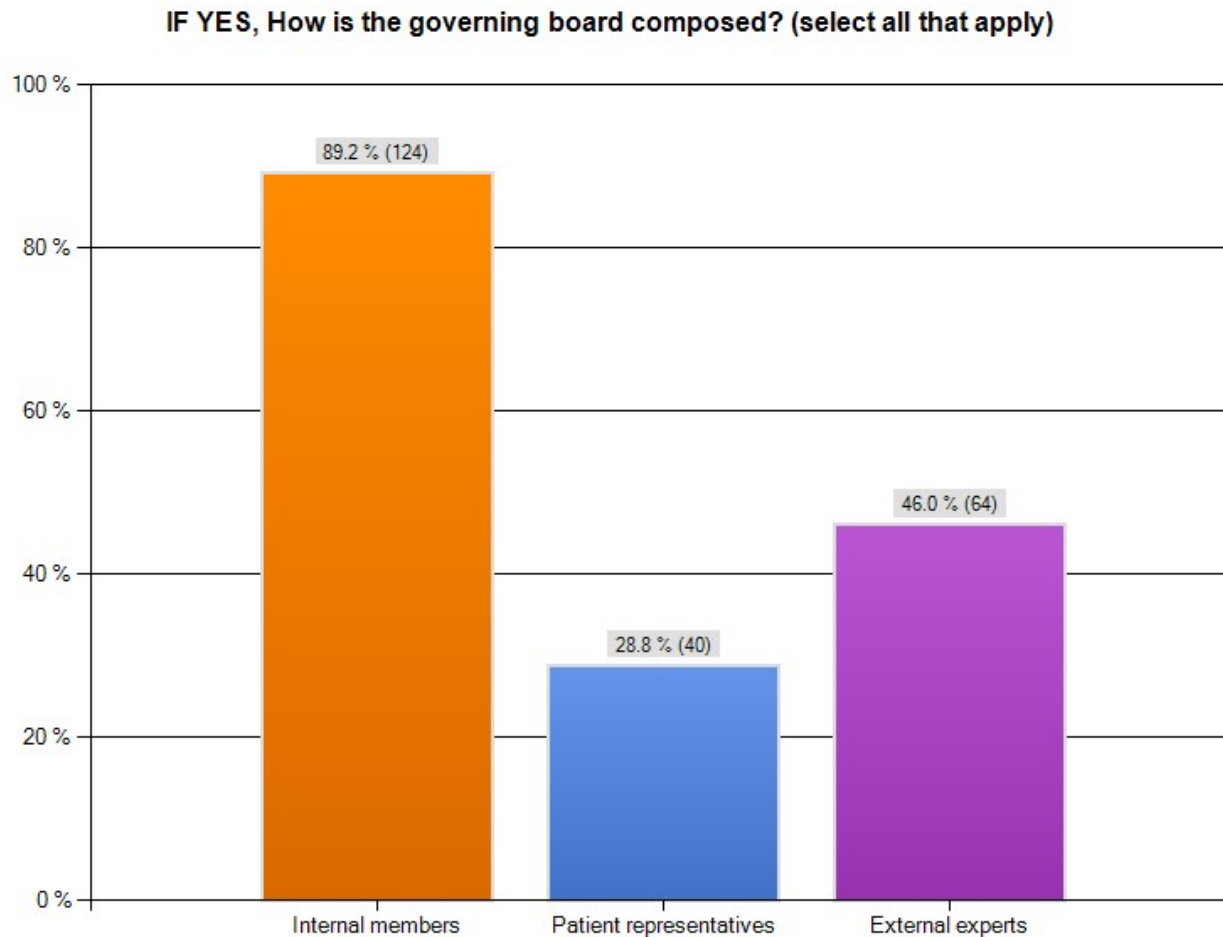




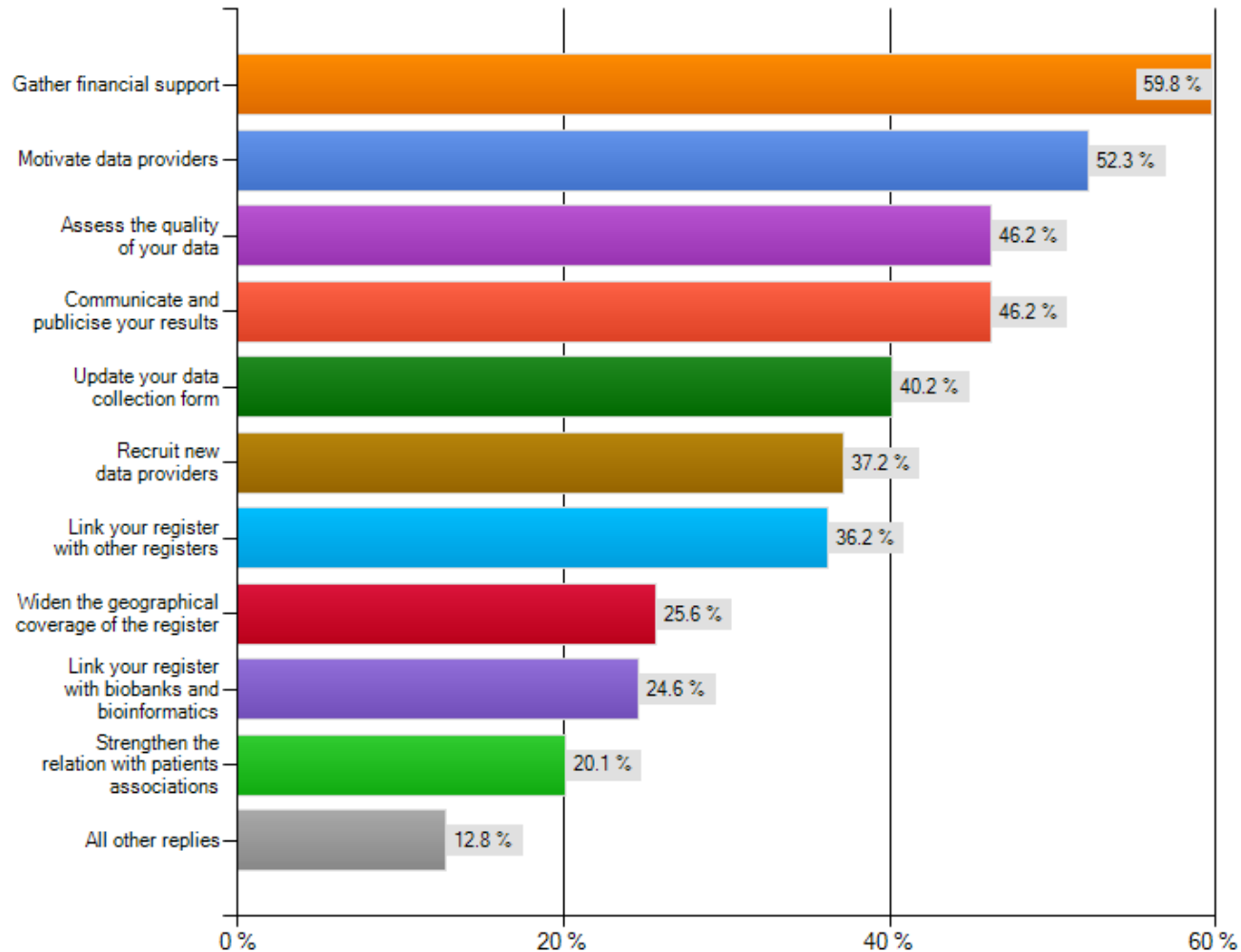
# Main Governing Board



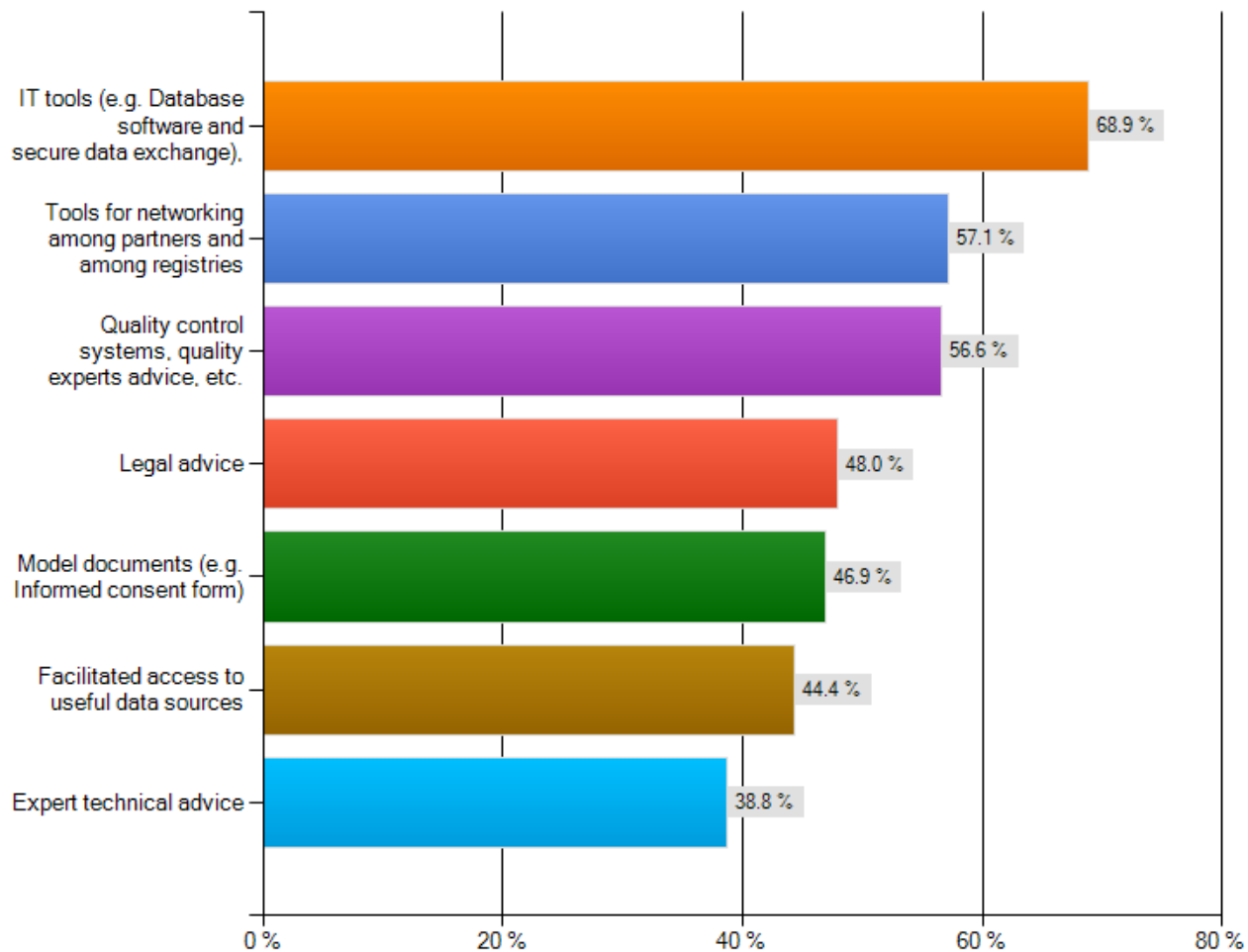
# Members of the Governing Board



# Registries main needs (select max 5 answers)



# Useful tools (select max 5 answers)



# The EPIRARE Platform model

## Functions:

- CDE data repository; metadata repository
- Provision of tools and resources of use to registries;
- Production of predefined information for institutions, patients and the public;
- Communication to the public, promotion of registration and networking.

## External Quality Assurance System (EQAS)

**The Governing Boards** include patients representatives and ensure ethical processing of data

## Multiple data sources

to increase accuracy, to reduce sample biases and to estimate underreporting

## Inclusive approach

- Participating registries and databases comply with data quality criteria.
- A tailored quality assurance plan is proposed to non complying registries.

**Independence:** Institutional control and location in a public institution (JRC, Ispra)

# Proposed purposes of the Platform Information production

- Epidemiological information
- Information for RD policy planning and monitoring;
- Monitoring RD-dedicated health services and their integration in the NHS;
- Health technology assessment (appropriateness of OD and other treatments)
- OD and other treatment costs
- Patient recruitment and support to health research
- Patient care benchmarking

# EPIRARE-related initiatives



***Preliminary program and registration: [www.epirare.eu](http://www.epirare.eu)***

## **The International Summer School on Rare Disease and Orphan Drug Registries**

*Rome (ISS), September 16-20, 2013*



## **The Second International Workshop on Rare Disease and Orphan Drug Registries**

*Rome (ISS), October 21-22, 2013*

***First Announcement and CALL for  
ABSTRACTS***

# **Don't Stop Research on Rare Diseases!**

The new Draft Regulation on the General Data Protection, currently discussed in the EU Parliament, hinders the use of the small amount of RD data that are collected.

It will dramatically delay health research and the improvement of health care of RD patients!

This petition is supporting the amendments requested by EPIRARE, EURORDIS and many other organizations.

*Further explanations and model e-mails in 9 languages on the [www.epirare.eu](http://www.epirare.eu) website*

**Please sign the petition and spread the word;**

**the link is on [www.epirare.eu](http://www.epirare.eu)**

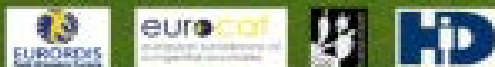
***As of 27 May we have reached 2406 signatures, but we aim at 10 000!***





# The EPIRARE Network

37 partners in 16 countries in 3 continents



*To join and be consulted on the EPIRARE documents, see instructions at: [www.epirare.eu](http://www.epirare.eu)*

**Would you like to join EPIRARE?**  
The EPIRARE project is open to new collaborations. If you want to join the network please send an email to [epirare@iss.it](mailto:epirare@iss.it)

[www.epirare.eu](http://www.epirare.eu)



## Background

The EU Council Recommendation on rare diseases (2009/872/CE) recommends support of registries and databases for epidemiological purposes. Its adoption is resulting in a burst of initiatives for the registration of rare disease patients calling for new systems for quality, governance and long term sustainability.



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contact:  
Domenica Taruscio

### ITALIAN NATIONAL CENTRE FOR RARE DISEASES

Italian National Institute of Health  
Viale Regina Elena, 299  
00161 Rome - Italy  
Tel. +39 06 4990 4016

[epirare@iss.it](mailto:epirare@iss.it)

[www.epirare.eu](http://www.epirare.eu)



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Thanks for your attention  
[www.epirare.eu](http://www.epirare.eu)

EURORDIS Membership Meeting, Dubrovnik, 31 May - 1 June 2013

