



EURORDIS
Rare Diseases Europe

Role of Patient Groups in Research and their Priorities for the Future



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EURORDIS' Survey on Patient Organisations and Research

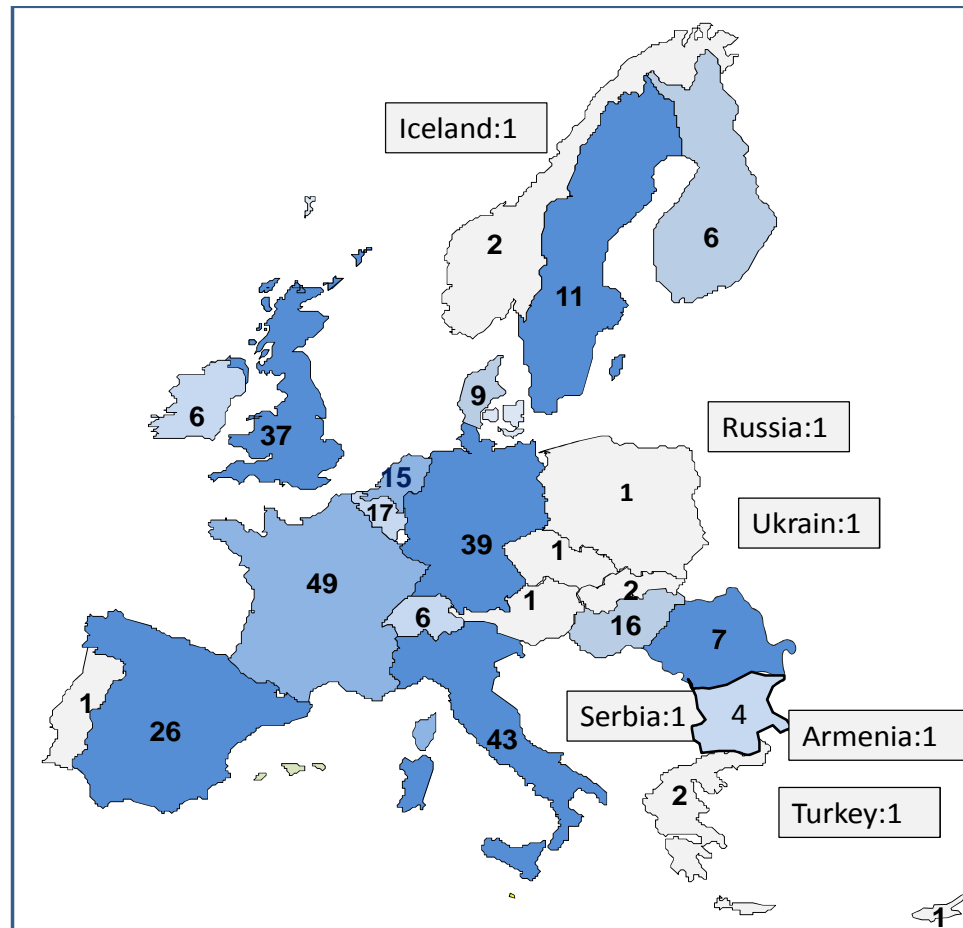
**In collaboration with the group of the
« Centre de sociologie de l'innovation »
(Ecole des Mines, Paris, France)**

Aim of the Survey

- 1. To measure POs' interest for research**
- 2. To evaluate POs' support to research: in what ways and to what extent.**
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- 4. To learn about POs' experience of collaboration with researchers**
- 5. To collect POs' opinion on Priorities and Obstacles for Rare Disease Research**

POs participating in the survey

309 responses (178 member + 131 non member POs)
= 40% response rate from 29 European countries



Diversity of represented medical areas

**110 diseases from various clinical domains
(1.3 million patients specifically represented)**

**Multisyst
n=38**

**Neurology
n=30**

**Dermato
n=23**

**Musculosk
n=22**

**Ophthalmo
n=12**

**Metabolic
n=13**

**Neuromusc
n=27**

**Oncology
n=12**

**Cardiovasc
n=14**

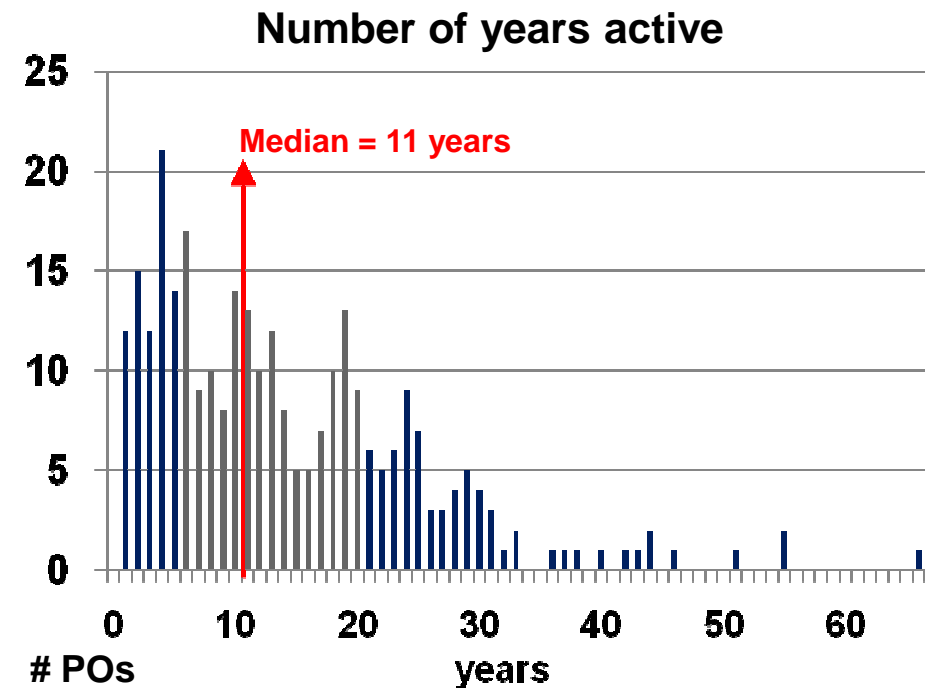
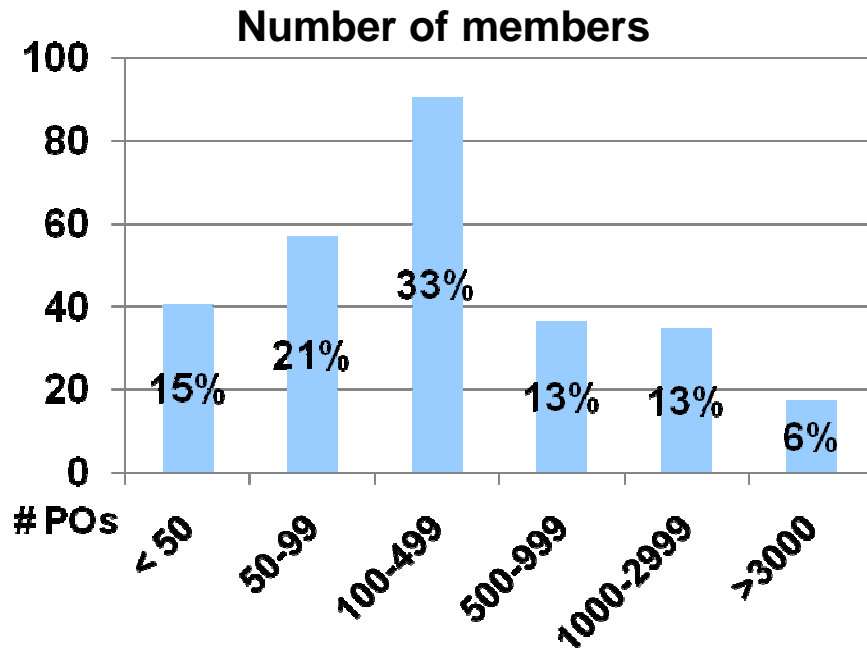
**Haemato
n=10**

**Others
n=59**

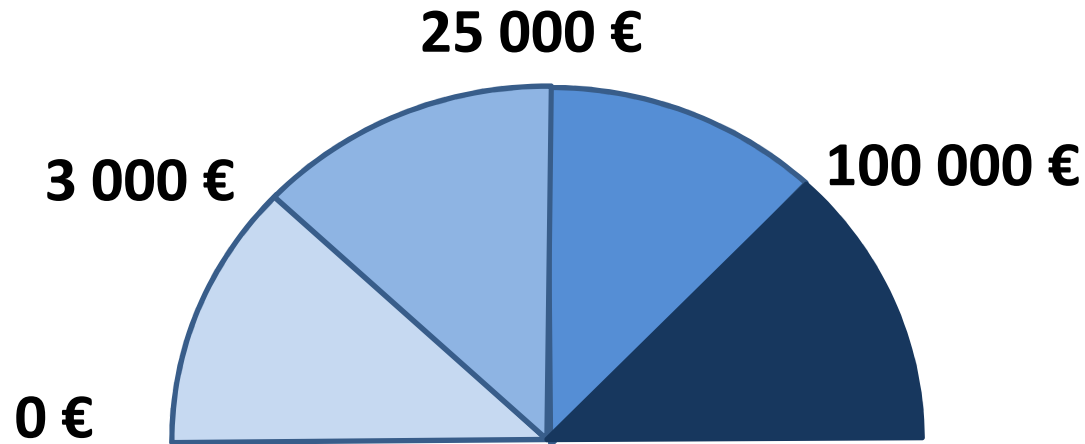
**Umbrella
n=15**

**15 Umbrella organisations from 10 countries
(20 million patients represented)**

Characteristics of participating POs



High diversity in POs' annual budgets



1/4 of POs have less than 3 000 €
10% less than 300 €,
6% no budget

1/4 of POs have more than 100 000 €
5% more than 1 200 000 €,

Main results: POs have a great interest in research

- **Overall a high commitment to research despite great differences across POs in terms of age, number of members and budget**
- **High response rate to the survey (40%)**
- **We observe the integration of external scientific competences within their governance structures: 56% of POs have a scientific board**
- **POs perform a continuous and multidisciplinary research monitoring and**
- **Have an acute perception of obstacles and priorities**

POs fund research!

Main results: POs' financial support to research

- **The percentage of POs that financially support research is linked to their total budgets**
- **Of the POs financially supporting research a total contribution of 13 M€ was provided over the last year (in addition the AFM* gives an average of 60 M€/year for research)**
- **The majority of POs financially support research using their own budget**
- **Financial support to research of some POs exceeds their own budget, which means they organise specific fundraisers for this purpose**

* French muscular dystrophy association

Financial support to research:

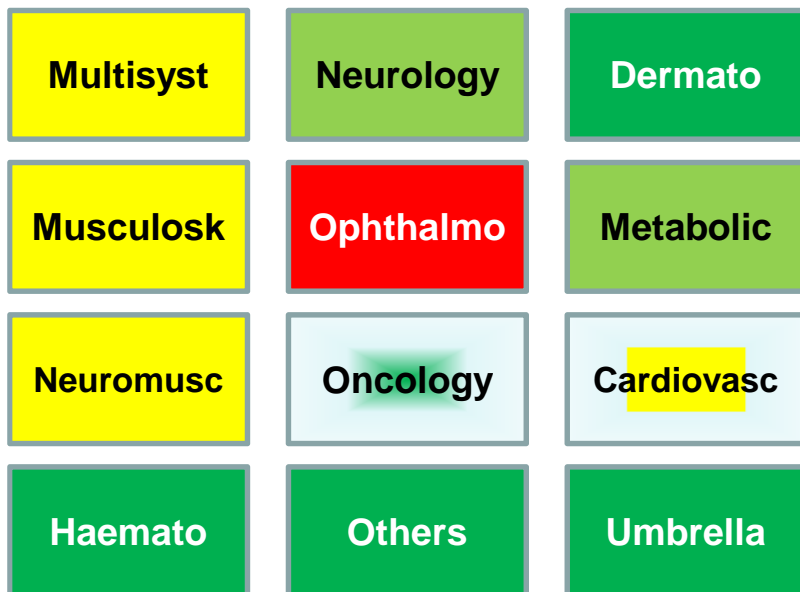
The percentage of POs that financially support research is linked to their total budget and their age

| POs' annual total budget | % of POs funding research |
|------------------------------------|---------------------------|
| Budget > 300.000 € | 81% |
| Budget between 30.000 to 300.000 € | 50% - 56% |
| Budget between 5000 to 30.000 € | 32% - 33% |
| Budget < 5000 € | 11% -14% |

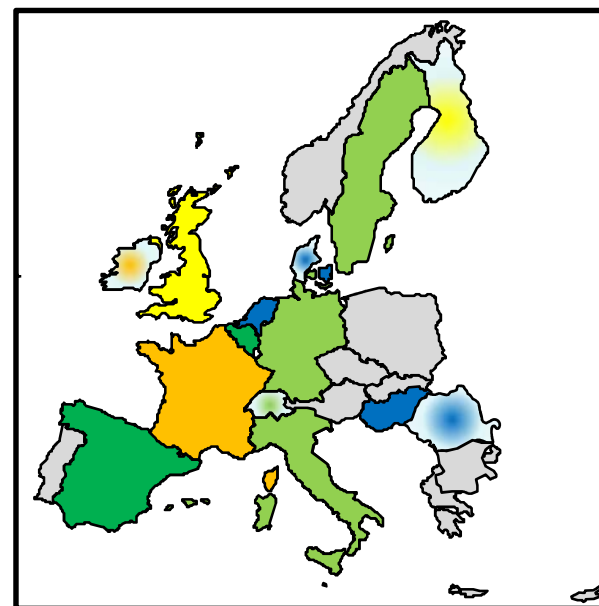
| Date of creation | % of POs funding research |
|---------------------|---------------------------|
| Before 1970 | 70% |
| Between 1970 - 1979 | 61% |
| Between 1980-1989 | 47% |
| Between 1990 -1999 | 35% |
| Between 2000-2009 | 30% |



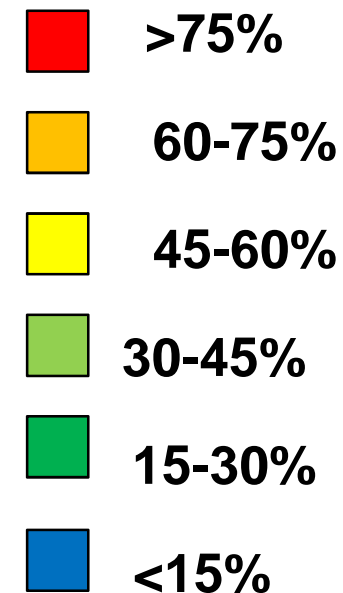
37% of POs financially support research



Distribution by disease type



Distribution by country



Main results: POs' financial support to research

- **POs prefer to provide financial support (or co-finance) for specific research projects**
- **Only a smaller number of POs supports research infrastructures or research on assistive technologies:**

POs seem to consider that these last areas do not fall in their field of action or under their responsibility.

Types of financial support

(37 % of POs who fund research)

77% Initiating and financing a specific research project

75% Co-financing the operating budget of a specific research project

54% Financing the acquisition of a specific research equipment (centrifuge, computer, etc)

47% Financing a fellowship for a young researcher

39% Co-financing meetings of researchers clinicians

30% Co-financing training of researchers / clinicians

What kind of research do patients fund?

81 % Basic research

57% Therapeutics

56% Diagnosis

54% Epidemiology / Natural history of the disease

46% Human and Social Science

24% Assistance technologies / Daily life

19% Research infrastructures

Patients support research in other ways too!

POs' non-financial support to research: a less visible but essential support

- A majority of POs support research mainly through actions aiming at creating links between patients, researchers and physicians.
- An important non-financial contribution is provided to clinical development through different activities
- Only a smaller number of POs is involved in campaigns for the collection of biological samples or participate in scientific committees in charge of defining research orientations.

Non-financial support: from clinical trials to institutions

76% Actions aiming at creating links between patients, researchers and physicians

57% Helping to identify patients to participate in clinical trials

49% Providing information and counseling for potential participants in clinical trials

48% Defining research projects by highlighting patients' needs and expectations

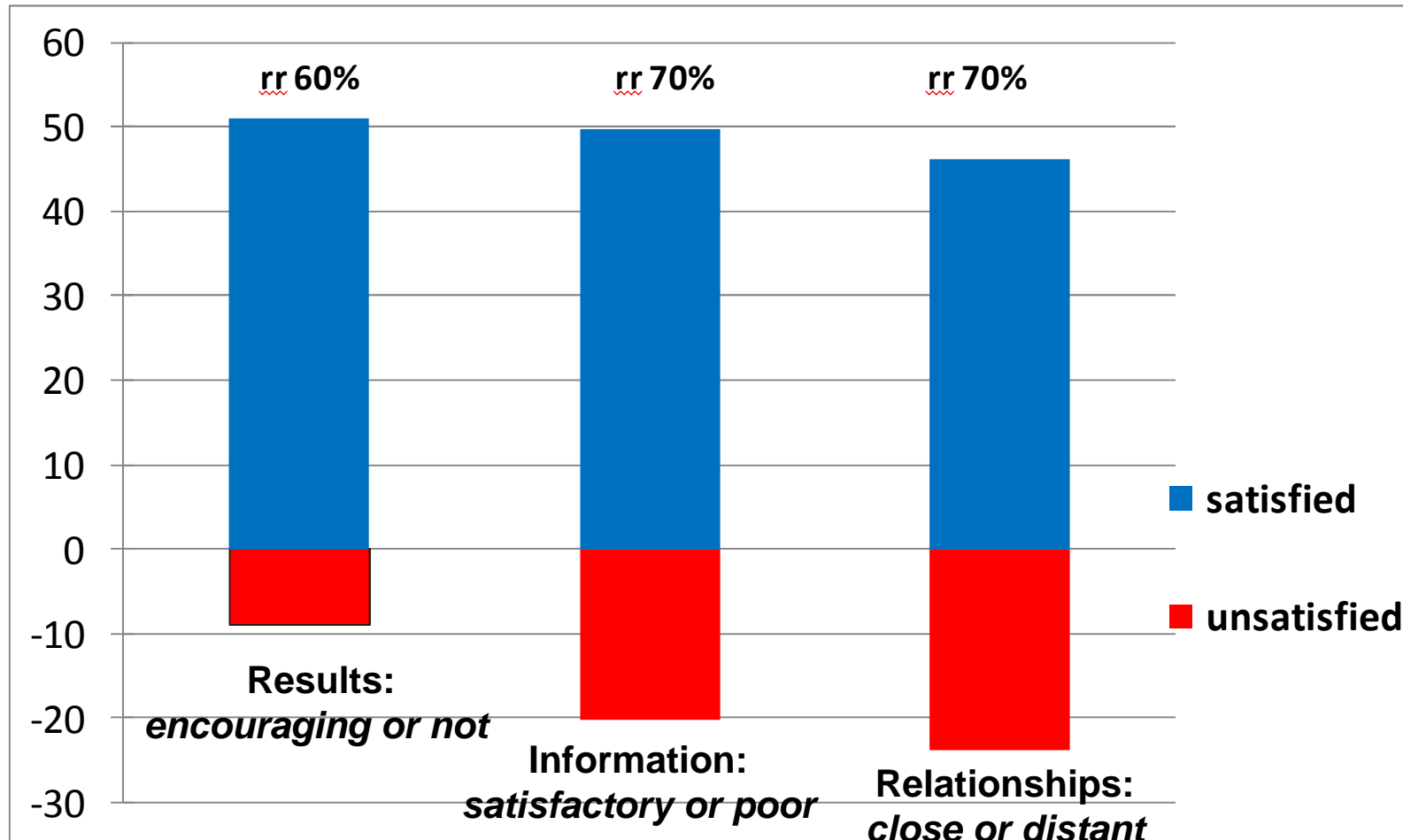
45% Collaboration in clinical trials design

30% Participation in scientific committees within institutions

28% Launching campaigns for the collection of biological samples from patients

**POs' experience when interacting with
the research community**

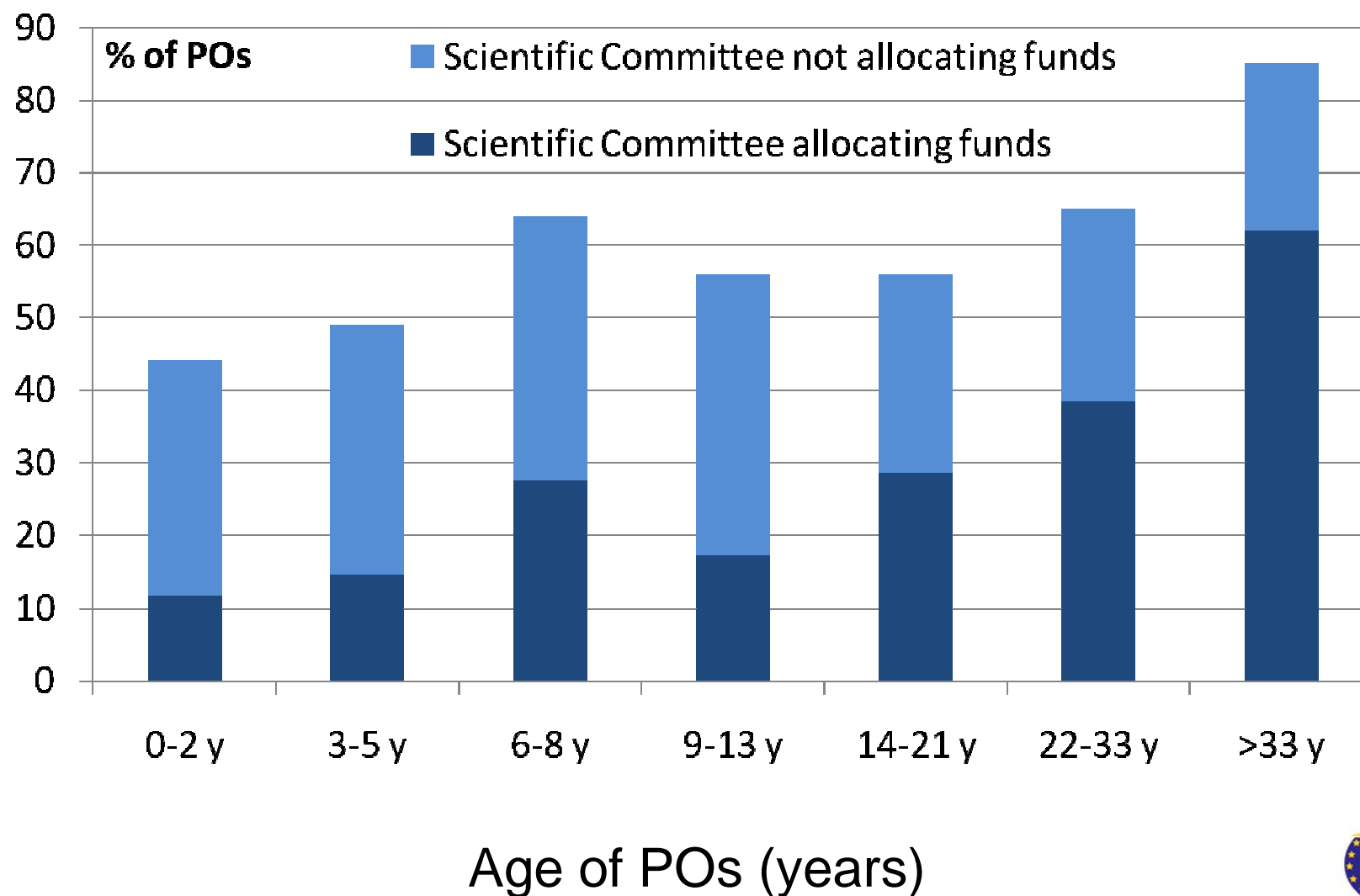
POs' experience of collaboration with researchers



Perspectives for improvement for a confident collaboration

rr= response rate

Evolution toward a confident relationship



Age of POs (years)

Priorities and Obstacles

- Absolute priorities in public funding for rare disease research
- Identified obstacles
- Perspectives

Absolute priority for allocation of public funds

49% Therapeutics

40% Diagnosis

35% Basic research

26% Human and Social Science

24% Epidemiology / Natural history of the disease

21% Assistance technologies / Daily life

17% Research infrastructures



Domains of funding

Allocated by POs (POs funding the research= 37 %)

81% Basic research

57% Epidemiology

56% Therapeutics

54% Diagnosis

46% Human and Social Science

24% Assistance technologies / Daily life

19% Research infrastructures

Expected from public (Absolute priority)

49% Therapeutics

40% Diagnosis

35% Basic research

26% Human and Social Science

24% Epidemiology

21% Assistance technologies / Daily life

17% Research infrastructures

Do the following situations constitute obstacles to the progress of research?

68% Low number of clinicians specialised in your disease/s

67% Low number of researchers working on your disease/s

52% Lack of coordination between specialists

52% Difficulties in identifying patients

43% Lack of multidisciplinary research

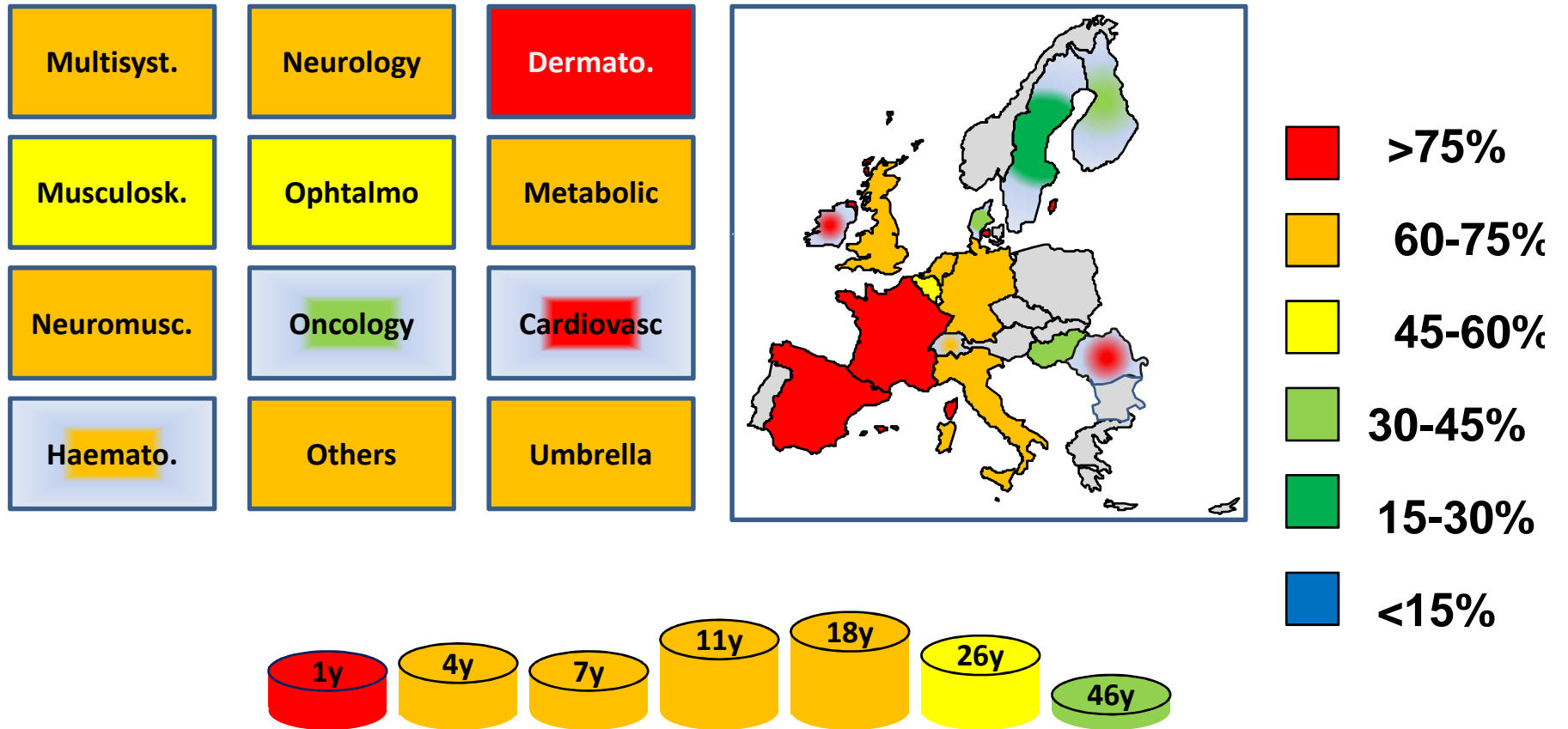
40% Lack of recognition of your disease

33% Lack of diagnostic tools

19% Wrong research focus

16% Absence of animal model

Obstacle 1: Low number of researchers working on your disease/s (overall: 67%)



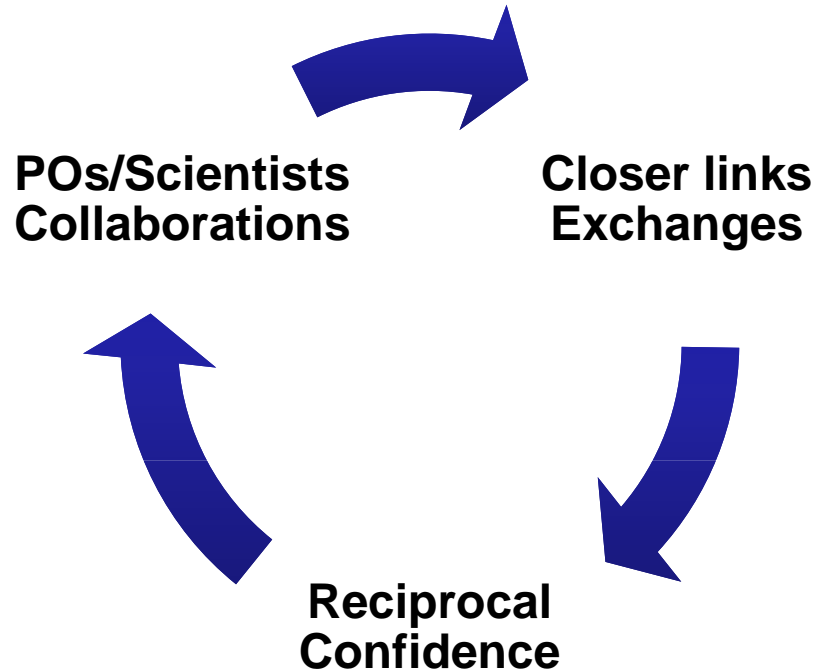
In summary

- **POs have a high commitment for research and are keen observers of all its areas** (Basic, Therapeutics, Social and Human Sciences, ...)
- **POs have a strong willingness for collaboration with researchers**
 - POs' efforts for linking Researchers/Clinicians/POs
 - Relations with researchers are quite good and seem to improve over the years.
- **POs play an important role as catalysts of research**
- **POs provide two types of support to research**
 - Financial and
 - Non-Financial: natural go-betweens for scientists from various fields from the more basic research to therapeutic applications, crucial support in clinical trials
- **But....POs have limited budgets**
 - Triggering role and momentarily “filling the gaps” by supporting the type of research that appears less attractive to the public or private sector

In summary

- **POs are fully aware that to understand the disease (basic research) and to cure the disease (therapeutic research) are two axes that must be supported concurrently as progress in one contributes to the other.**
- **The long history of several POs and their continuous commitment to basic research demonstrates that they understand that research is a slow and long-term process**
- **POs do not want to (and they cannot) replace public or private research institutions, but rather collaborate with them as fully recognised partners bringing important specific contributions.**

In summary



**POs are mature partners for
Research!**

THANK YOU !

EURORDIS

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