1st IPOPI National Patient Organisations Survey

January 2011

Introduction

- The 1st IPOPI National Member Organisation (NMO) Survey was initiated (Feb-Aug 2010) to establish a mapping of the following criteria amongst IPOPI's NMO's:
  - Estimated Number of patients in each country
  - Number of known diagnosed PID cases
  - Patient demographics
  - Presence of national registries & potential collaborations with international registries
  - Provision of care, availability of treatment and funding of care in each country
  - National Medical Advisory Panels
  - Communication tools

Survey Background

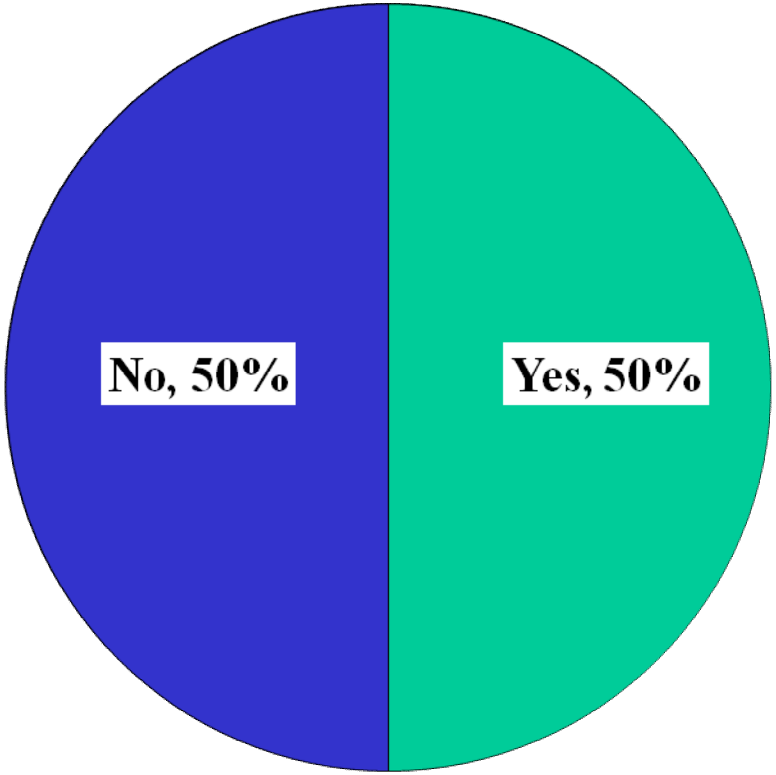
- IPOPI NMO Survey
  - Mixed mode, internet & paper
  - February – August 2010
  - NMOs allowed to resubmit updated data
  - Concerted effort to collect data from non-respondents
    - Included submissions of “off-line” surveys

- 33 NMO’s represented
  - Substantial completion of 32 surveys

Survey Participants

Countries Represented		
Argentina	Germany	Norway
Australia	Greece	Poland
Austria	Hungary	Russia
Belgium	Iceland	Serbia
Brasil	India	South Africa
Canada	Ireland	Spain
Colombia	Italy	Sweden
Cyprus	Japan	Switzerland
Denmark	Morocco	United Kingdom
Estonia	Netherlands	United States
France	New Zealand	Venezuela

Knowledge of Number of PID Cases in Country



The knowledge of diagnosed PID patients is uneven and varies from country to country. Half of IPOPI's national member organisations know how many patients have been diagnosed in their country

Number of NMO Patient Members

Range

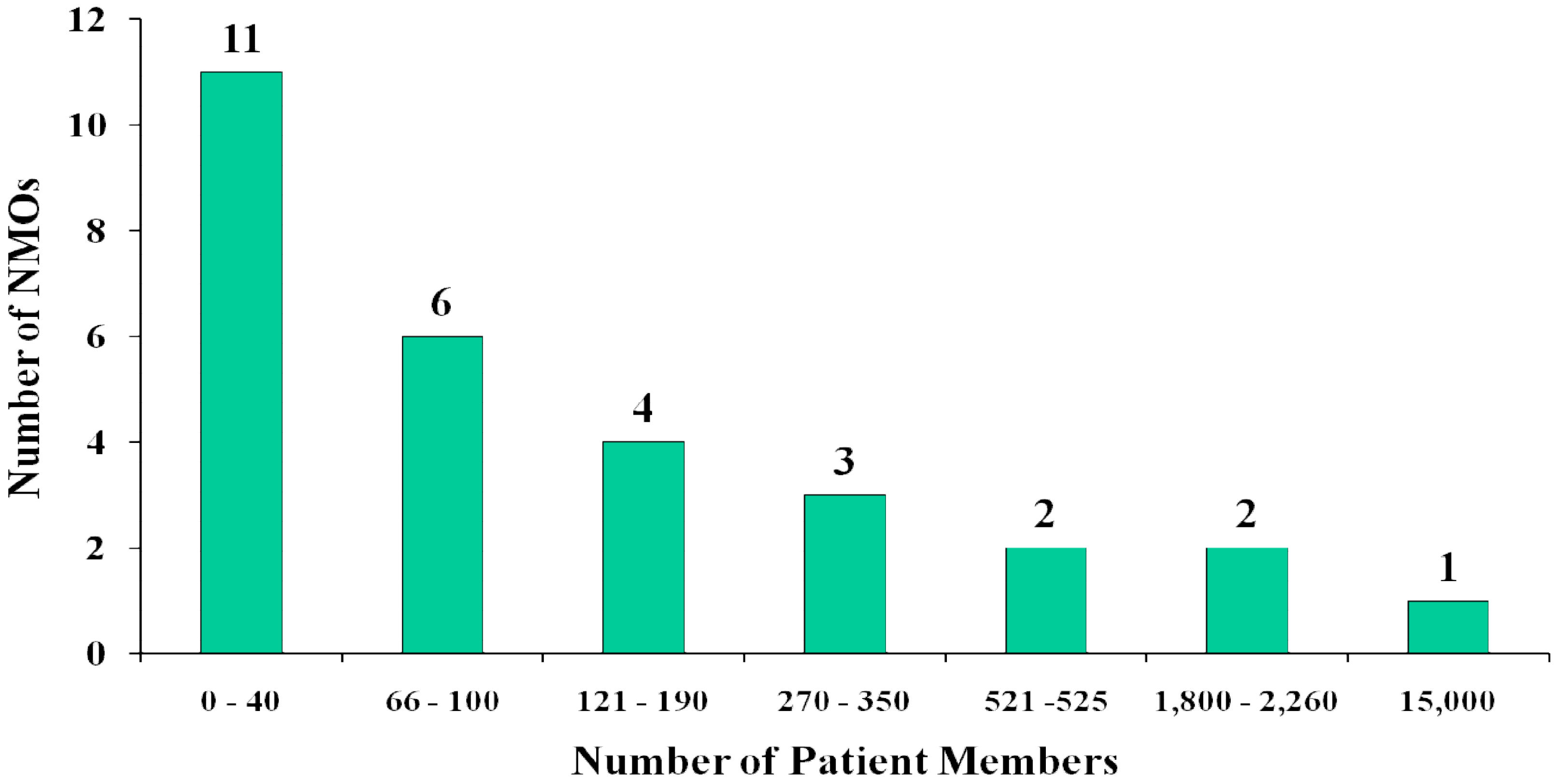
0 **15,000**

Mean = 770 **Median = 98**

Representing 22,332 Patients

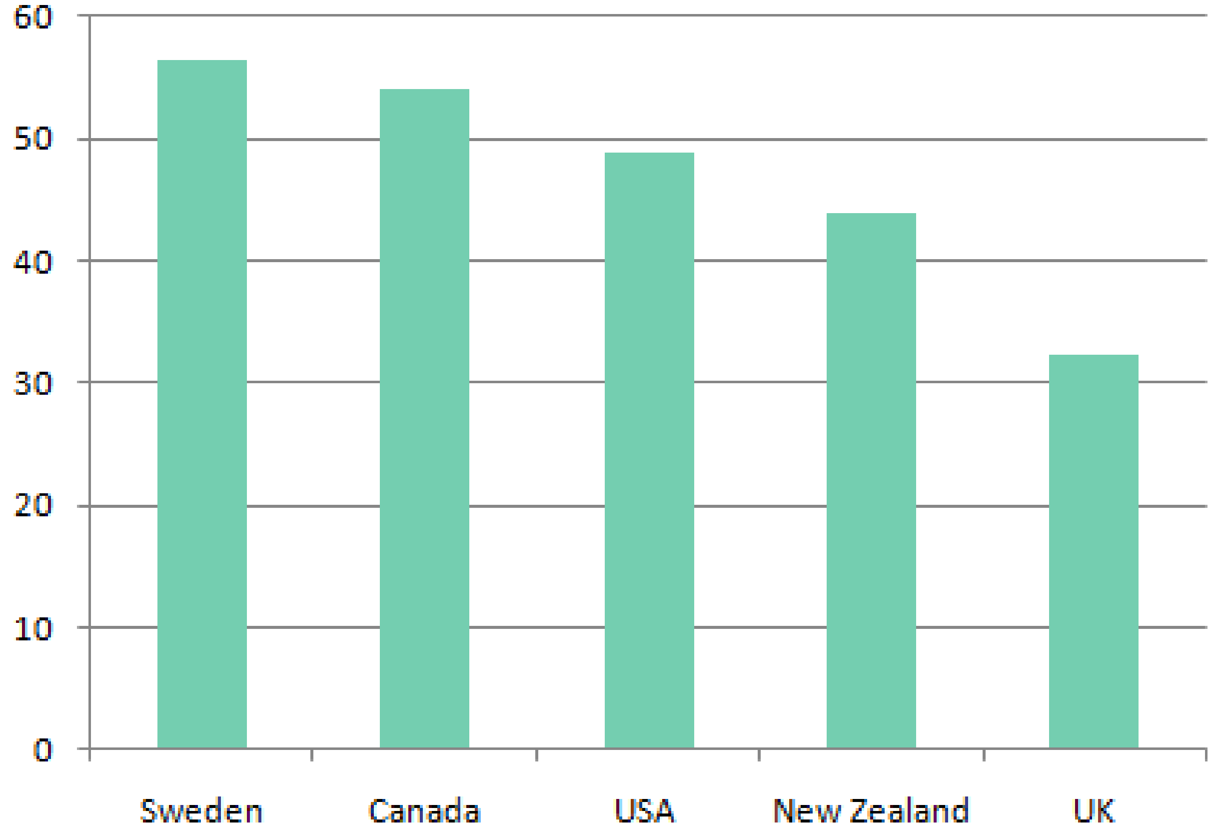
This is the range of the reported numbers of patients who are actually members of IPOPI's NMOs. The smallest NMO's do not yet have members either because they have just started or halted their activities.

Diagnosed PID Cases
Grouped by Selected Ranges



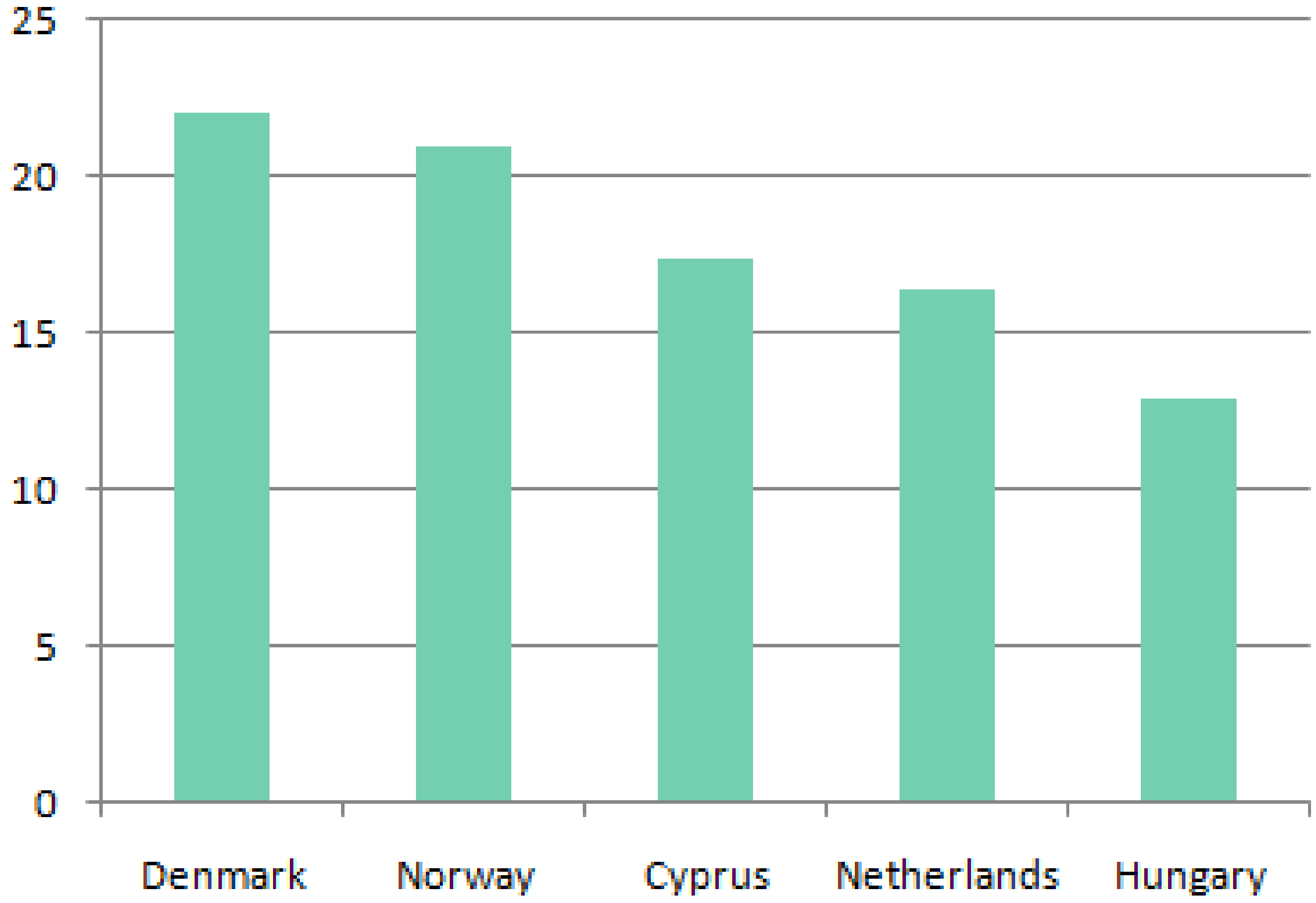
Q3a. How many patients with a PID diagnosis are members of your NMO in total? Base: Those responding (N =29)

Actual Reported Number of Patient Members per million population (>10 and < 30)

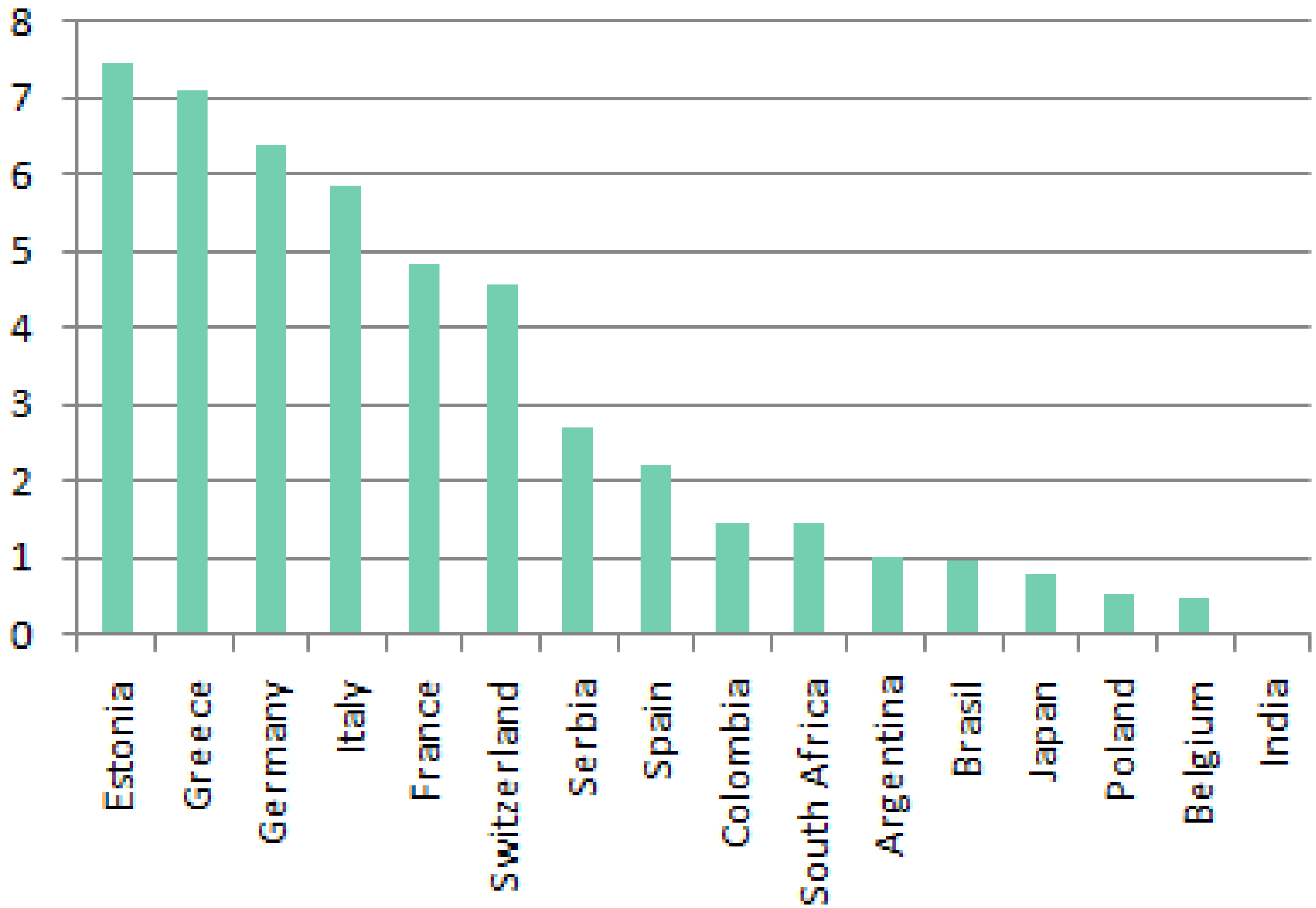


Countries with highest numbers of reported PID patients, members of their NMO's, per million population were Sweden, Canada, USA, NZ and UK.

Actual Reported Number of Patient Members per million population (>10 and < 30)



Actual Reported Number of Patient Members per million population (>10 and < 30)



Range of Estimated PID Cases in Each Country

Range

40

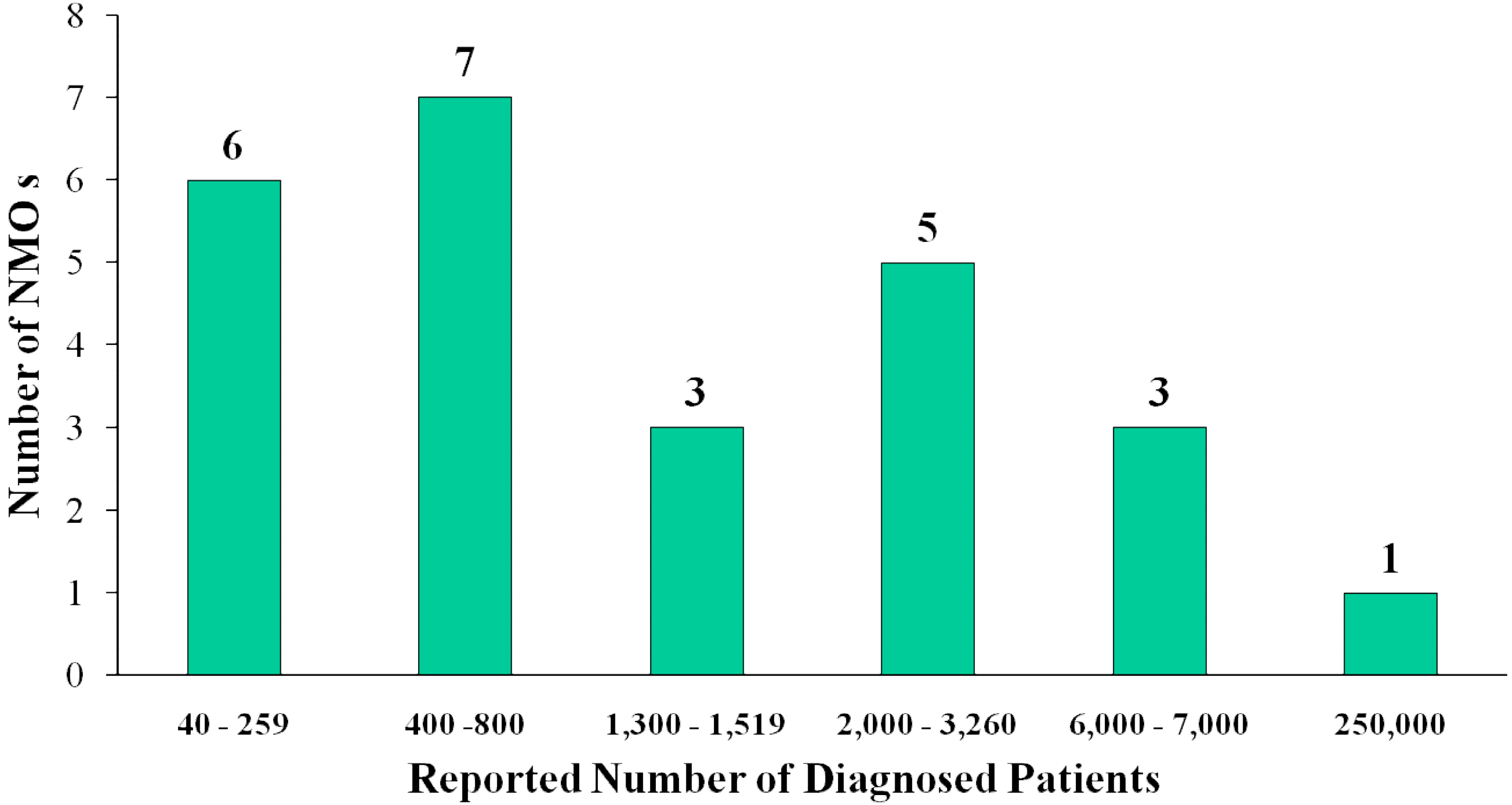
250,000

Mean = 11,635 **Median = 800**

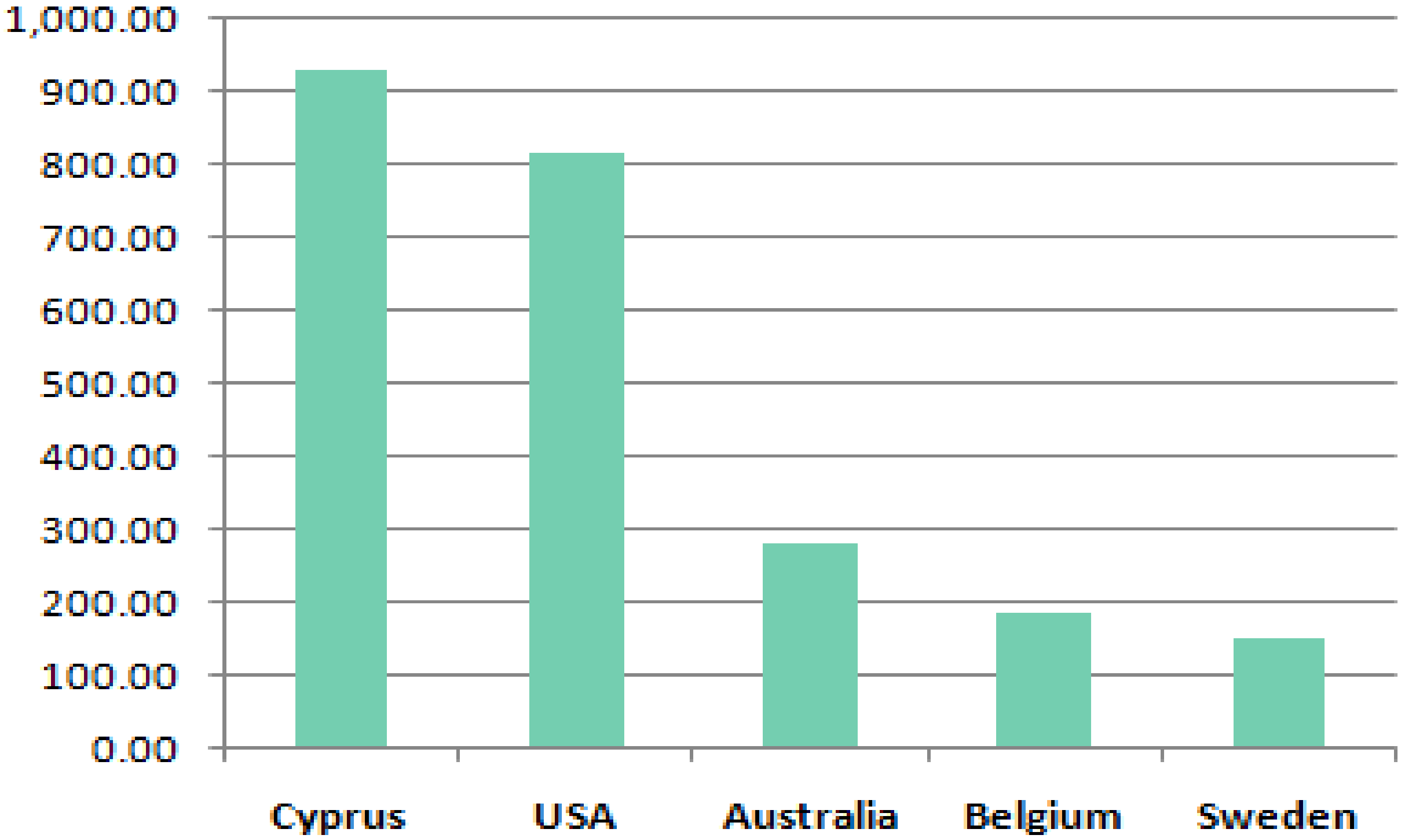
290,881 Total Reported/ Estimated Cases

IPOPI national member organisations provided the number of estimated of PID patients in their countries. It is important to note that in some cases these are actually counts of patients in a registry, in others they are estimations based on surveys ,or based on estimates of population prevalence gathered from peer reviewed journal publications and extrapolated to the national population.

Estimated PID Cases
Grouped by Selected Ranges

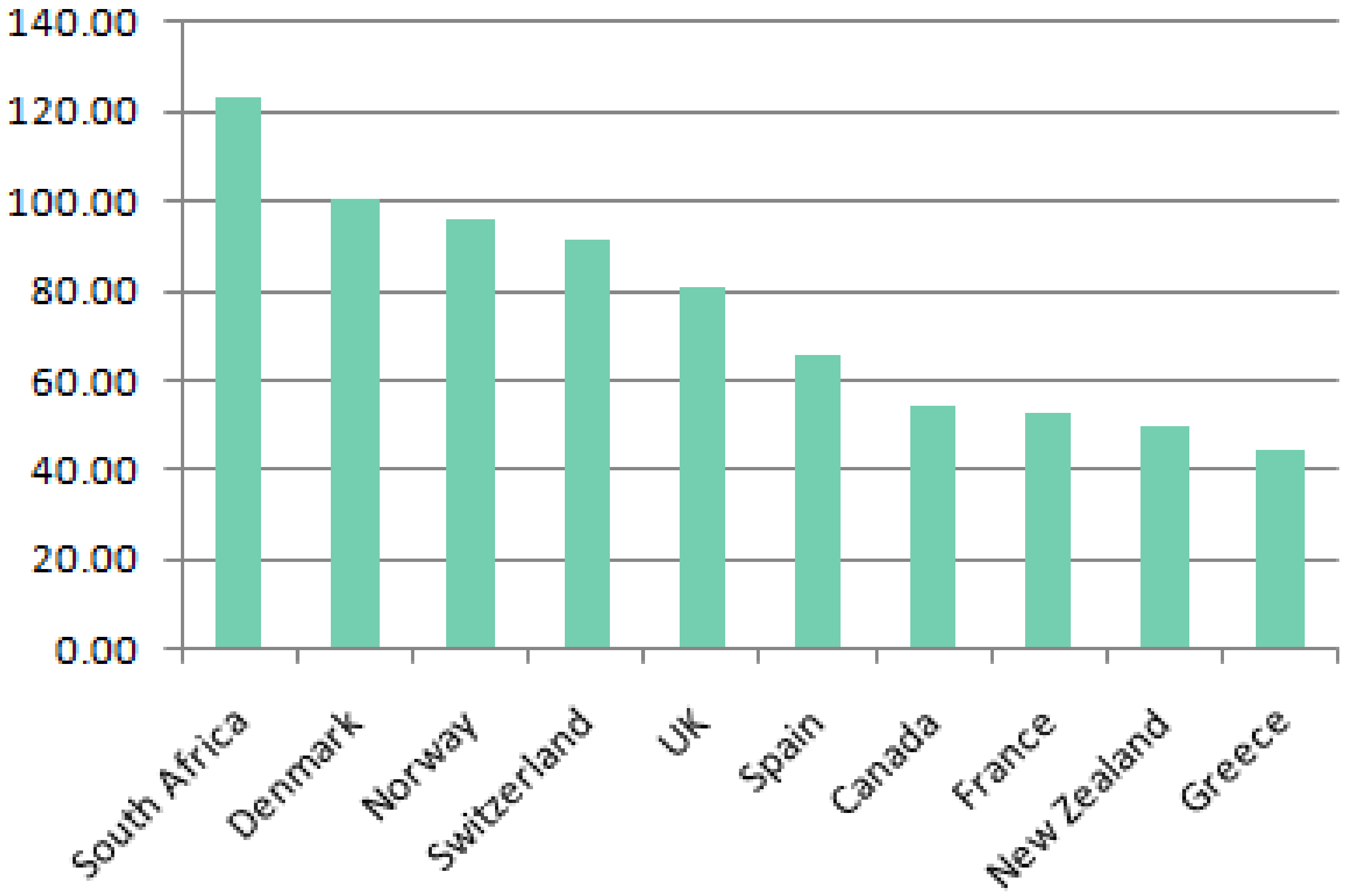


Estimated PID cases per million population (>100 and < 1000)

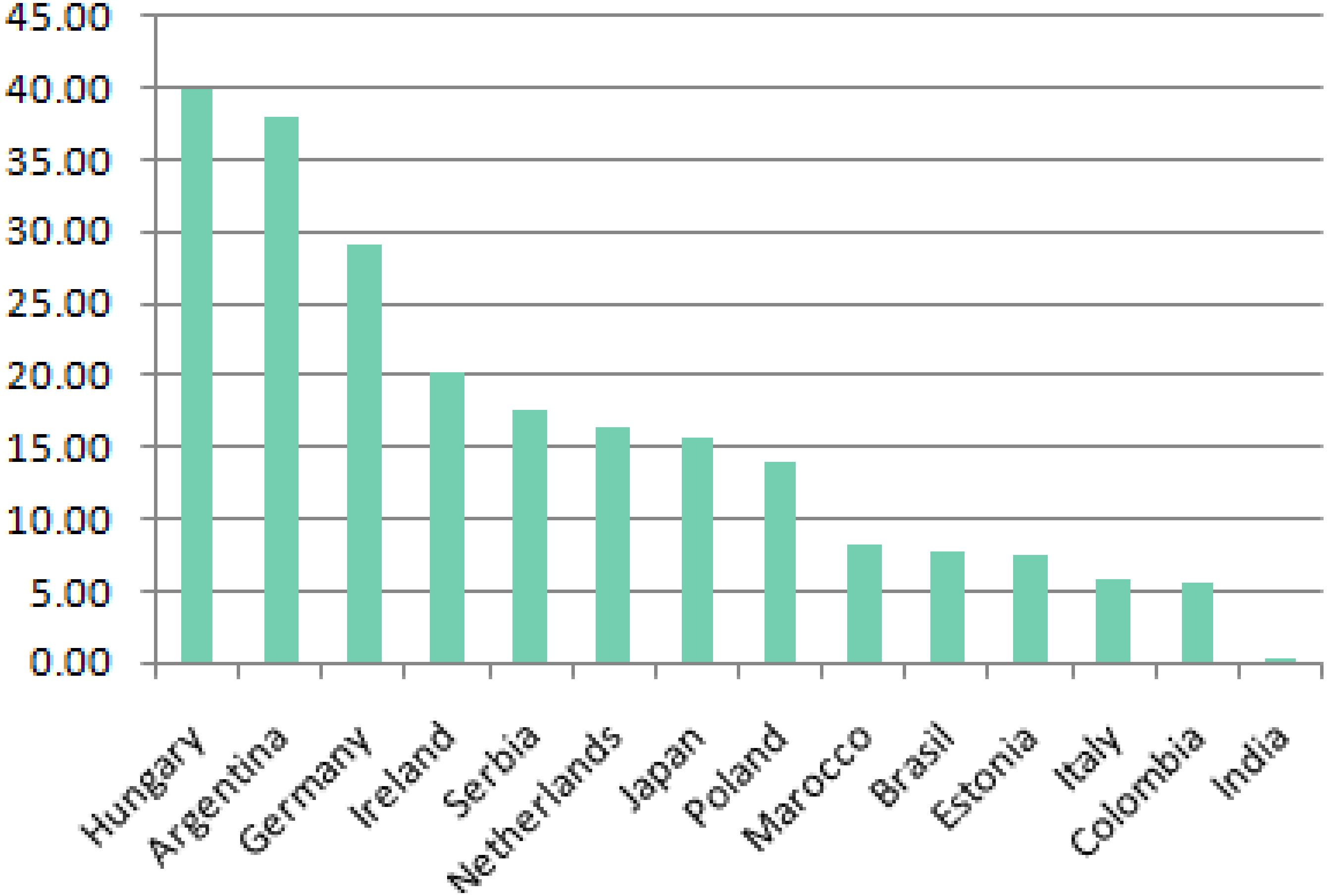


Countries with highest numbers of estimated PID patients per million population were Cyprus, USA, Australia, Belgium and Sweden

Estimated PID cases per million population (>40 and < 130)



Estimated PID cases per million population (< 40)



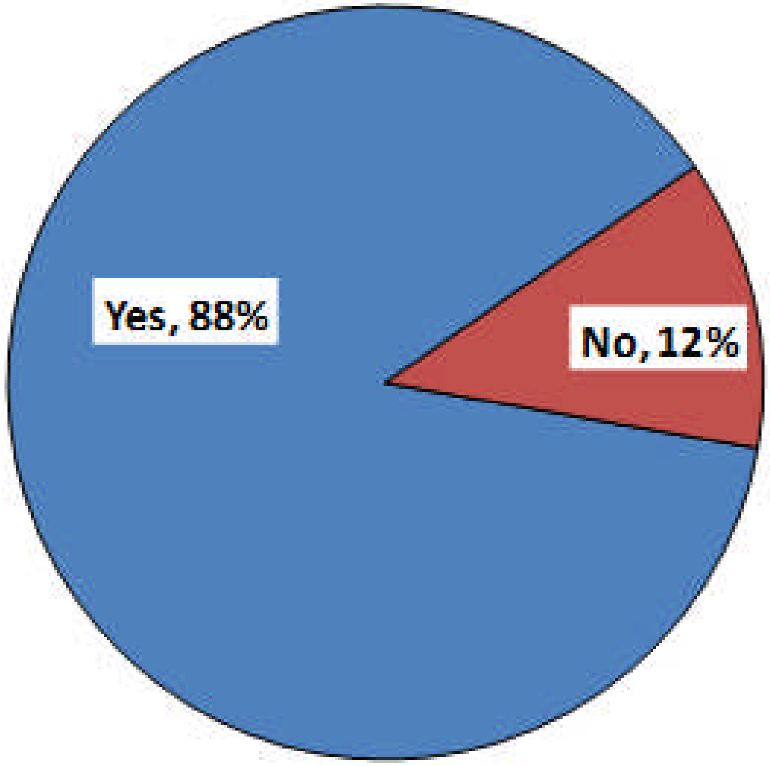
These are the countries with lowest numbers of estimated PID patients per million population.

Estimated PID cases per million population

- In conclusion, establishing prevalence rates remain very difficult as different calculation modes, different opinions and different diagnosis rates are used from country to country

→ IPOPI’s recommendation would be to define a worldwide prevalence rate for PID

Knowledge of Patient Demographics (Age)



The majority of IPOPI NMOs have a good knowledge of the proportion of adults and children within their membership

Demographics of Known Patients (Age)

Number Pediatric (0 – 17 years)

Range

2 – 6,000

Mean = 430
Median = 70

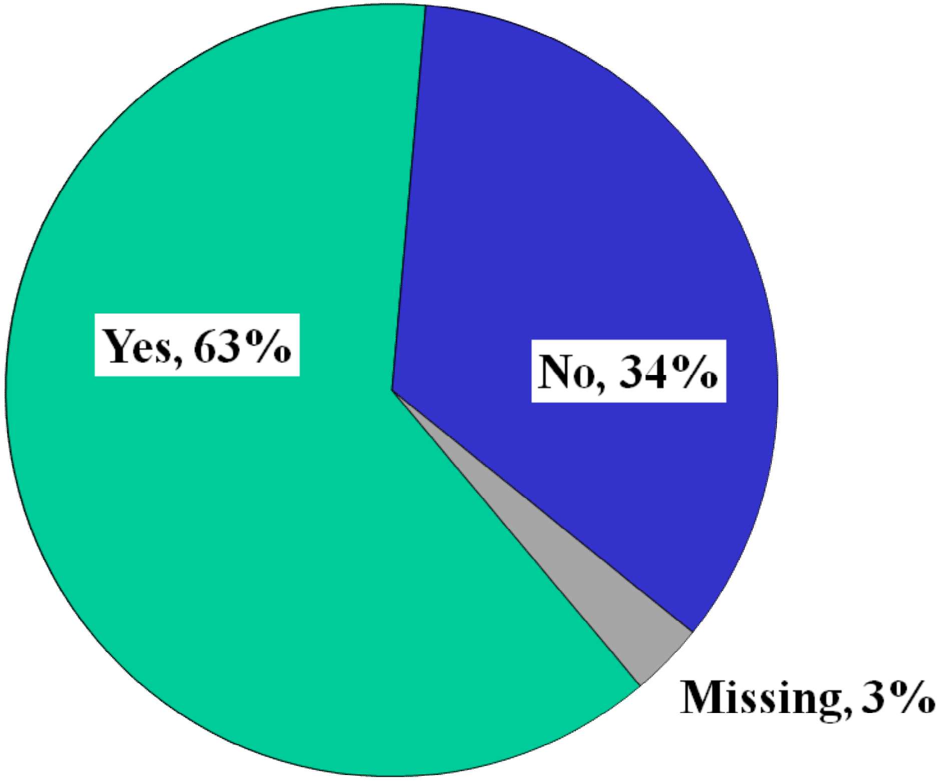
Number Adult (18+ years)

Range

0 – 9,000

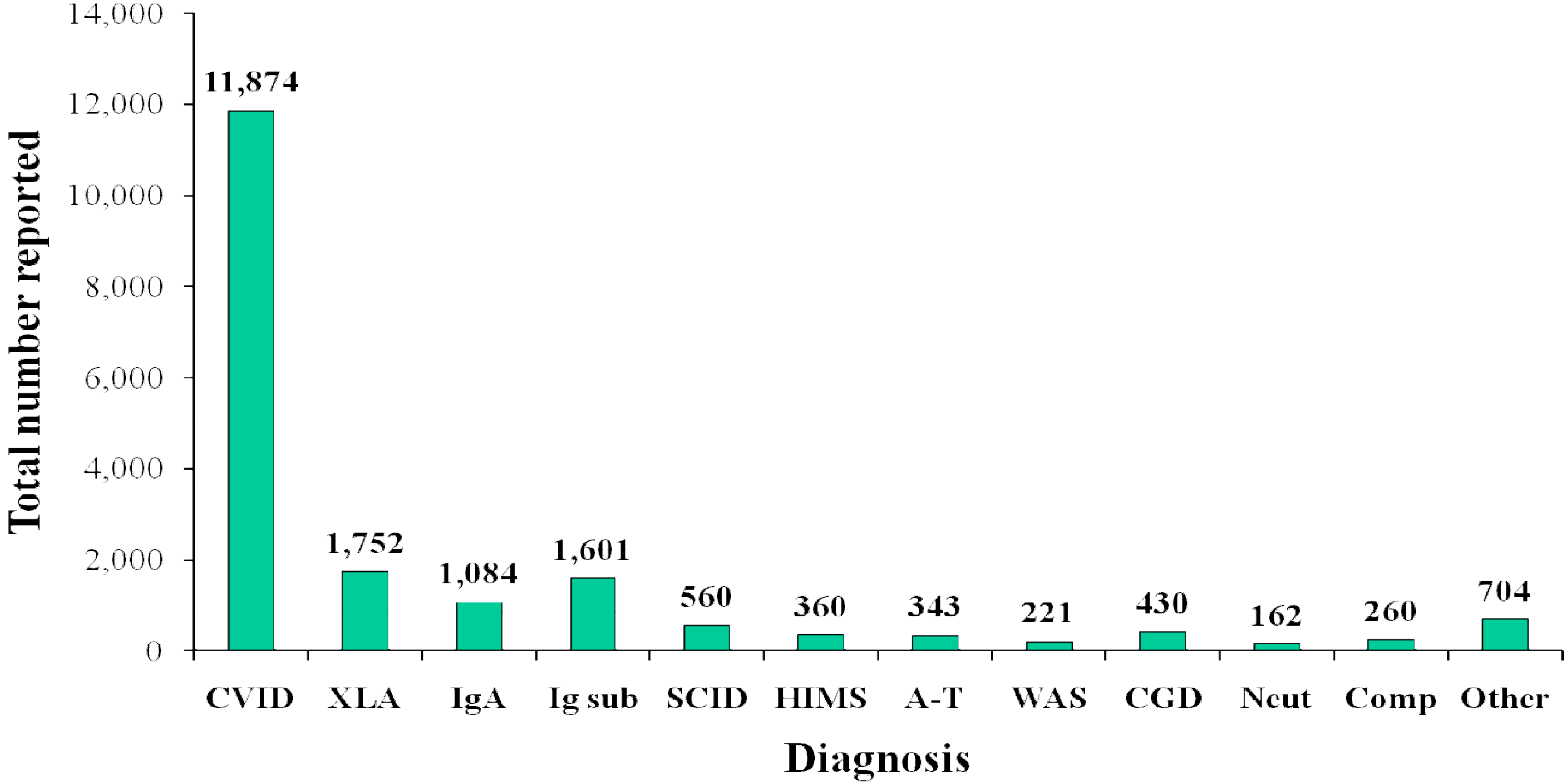
Mean = 661
Median = 60

Knowledge of Patient Demographics (Diagnosis)



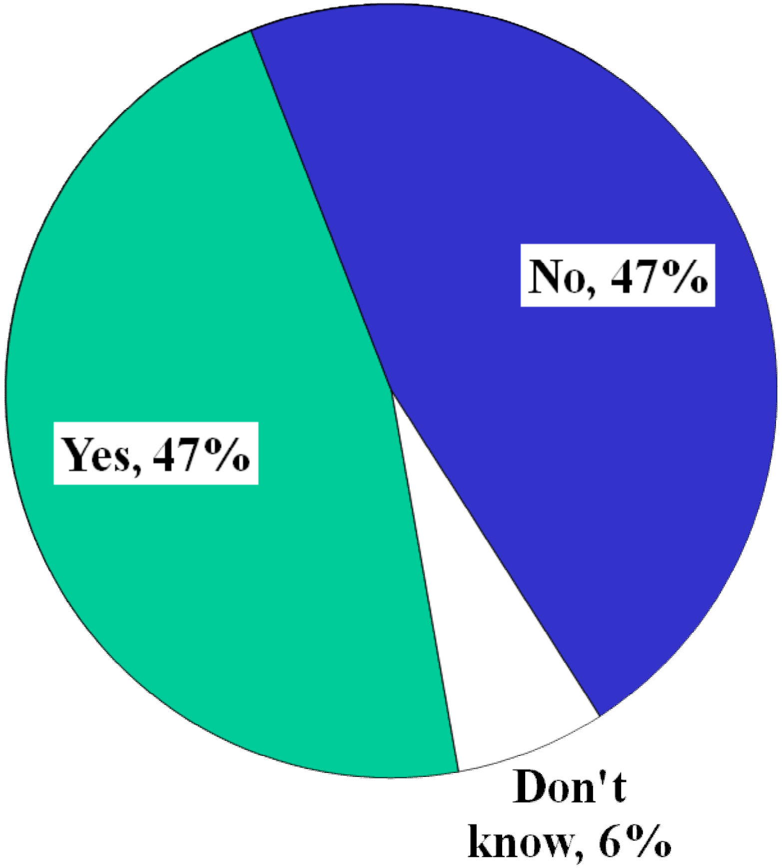
Two third of IPOPI NMOs were able to break down their membership numbers by diagnosis

Demographics of Known Patients (Diagnosis)



Common variable immunodeficiency (CVID) is the most frequent diagnosis reported by IPOPI's members. Importantly, other more severe forms, such as SCID where mortality rates are higher, are not always known or reported in these figures

National Registries



Approximately half of IPOPI members report having a national registry in their country

Number of Patients in National Registries

Range

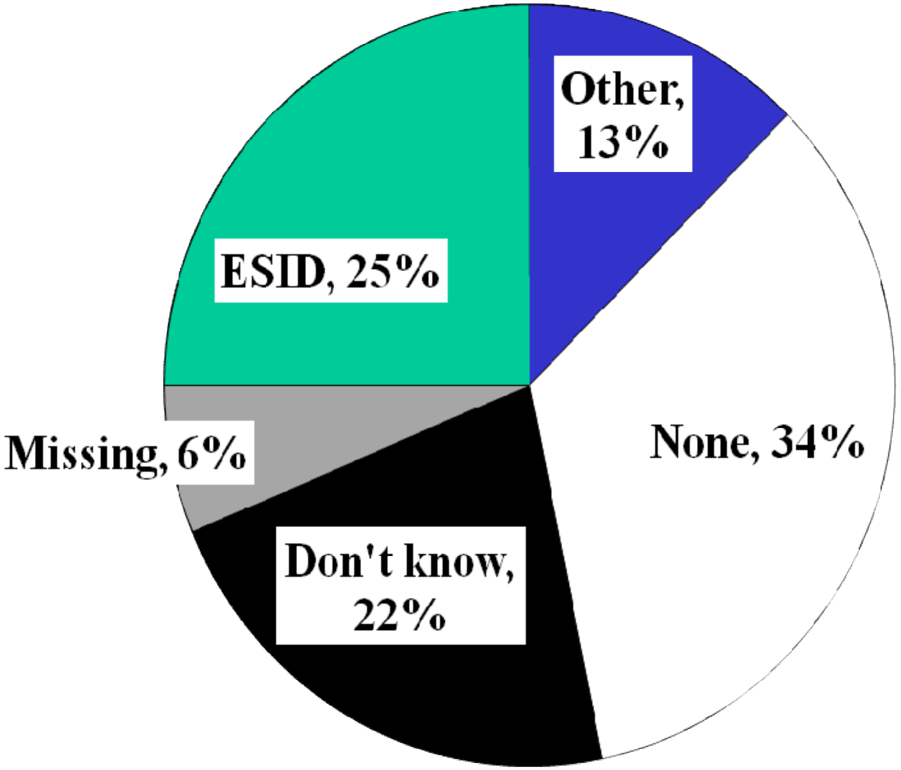
130 – **3,260**

Mean = 1,252 **Median = 1,288**

Representing 17,535 Patients

The range of actual patients registered in a national registry varies from country to country. In total 17,553 are registered in these registries.

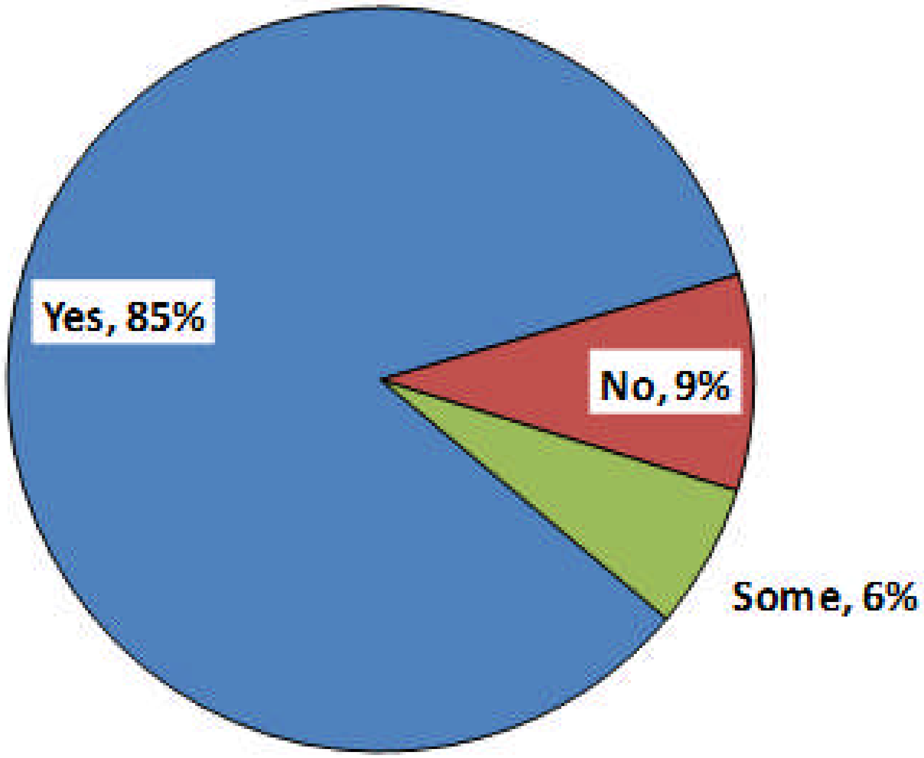
NMO Participation In International PID Registries



- Number of reported national registries participating in international registries such as ESID. Other international registries included ASCIA and LASID. This highlights that whilst international registry cooperation is happening much remains to be done.

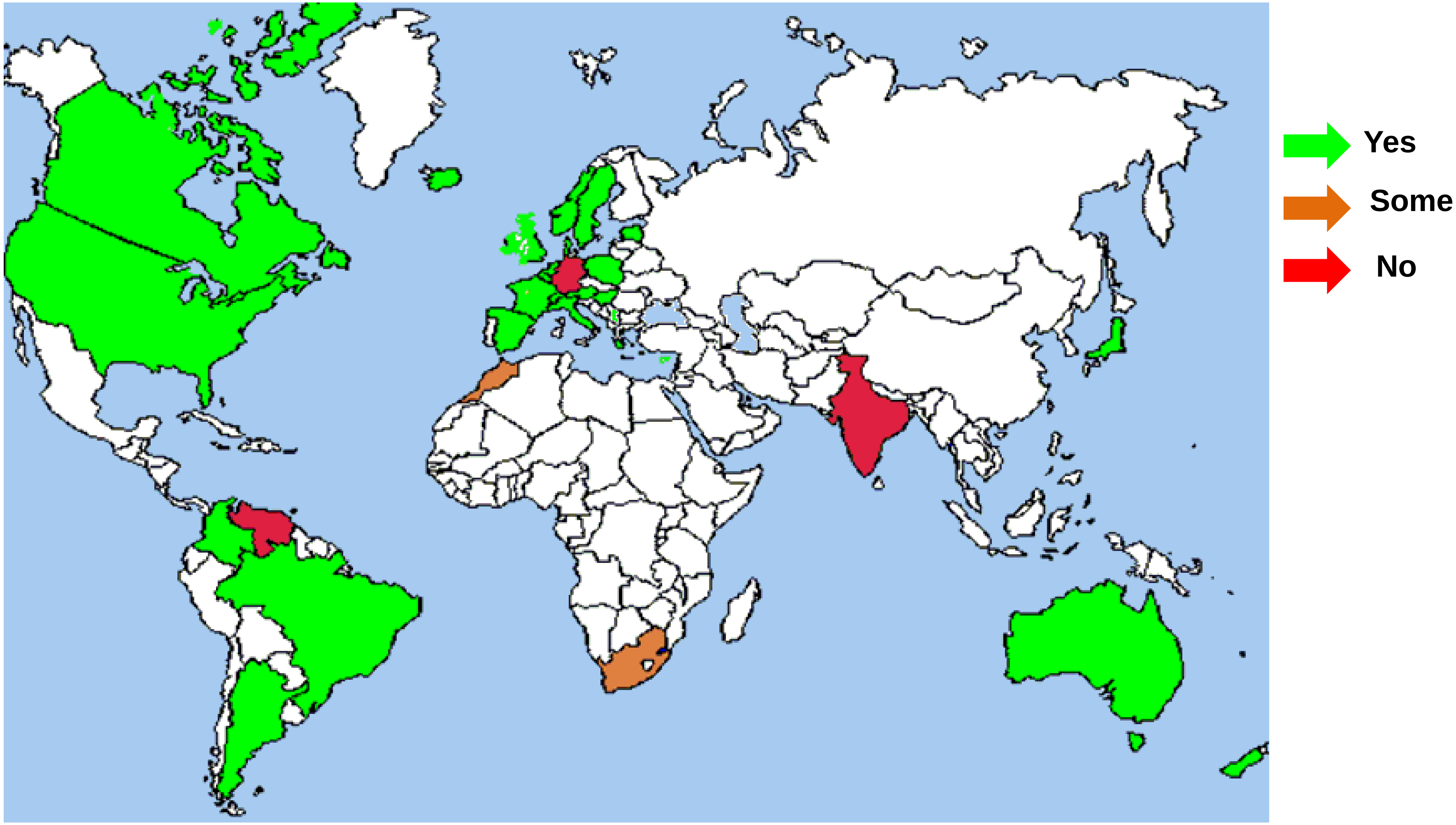
→ IPOPI encourages the harmonization and compatibility of registries to facilitate international cooperation

Provision of Care for Adult Patients With PID

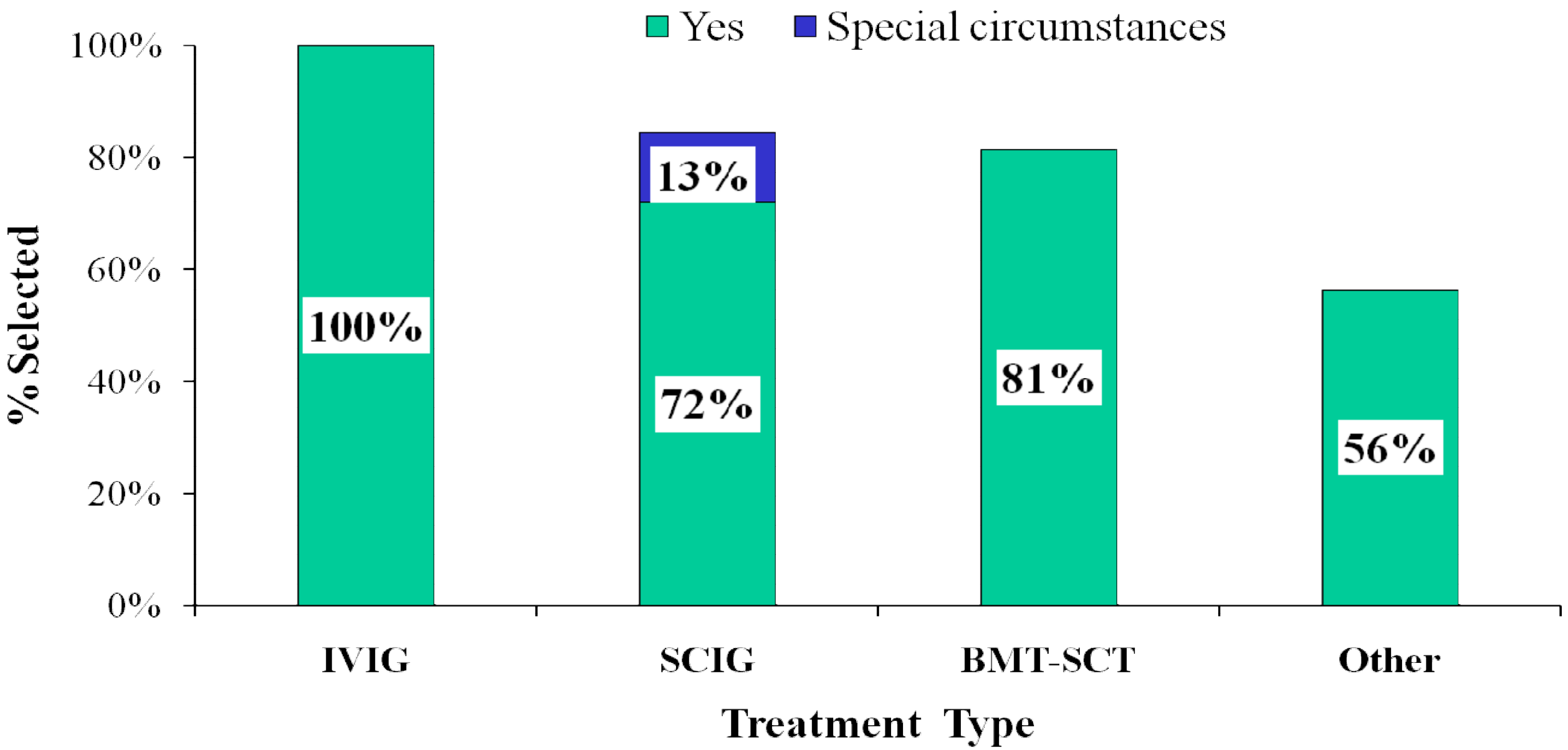


The majority of IPOPI's NMOs reported that adult care and treatment were available in their country. 9% reported this was not the case in their country. 6% reported that adult care was available but with limitations

Provision of Care for Adult Patients With PID – Country Spilt

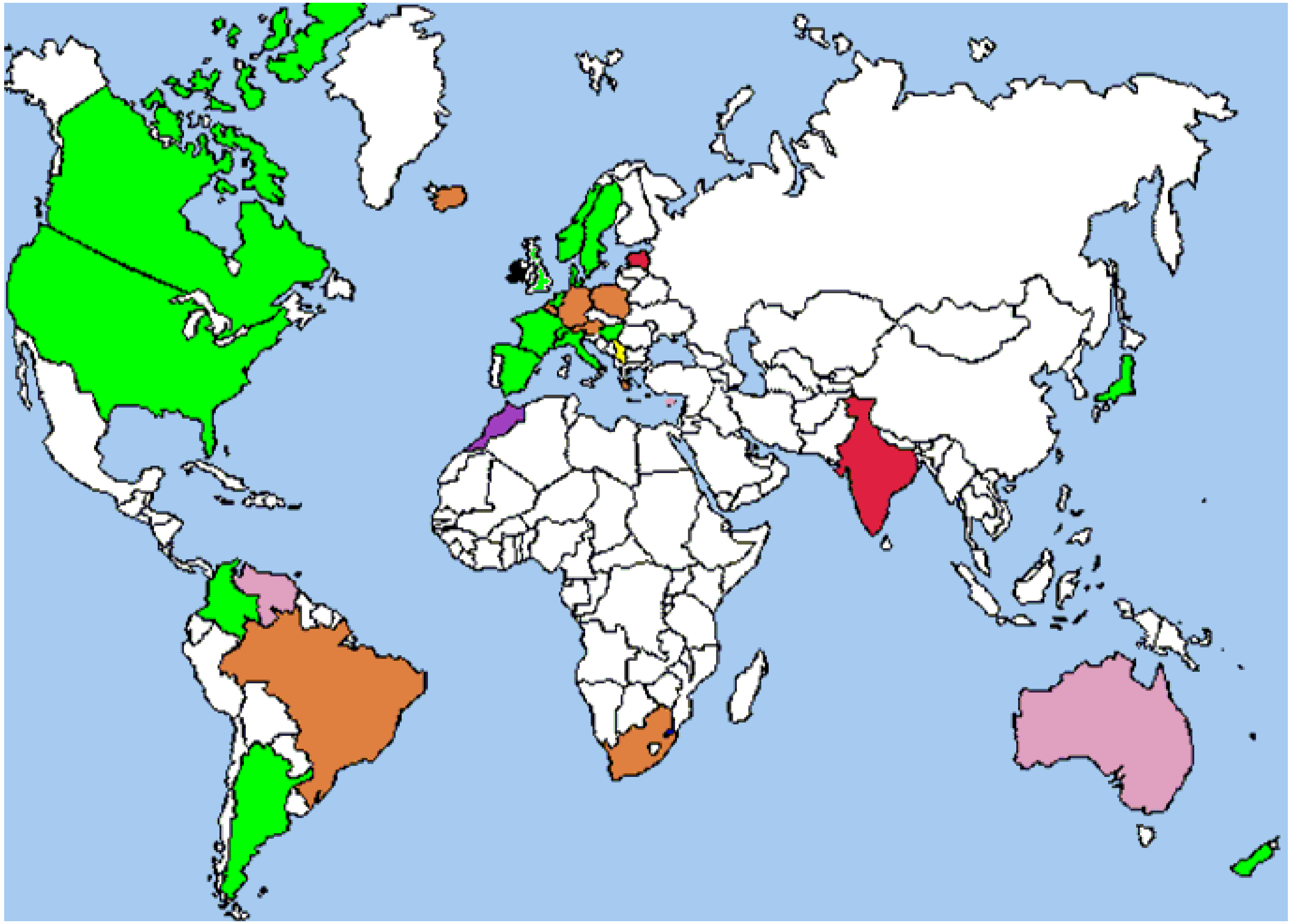


Availability of Treatment for Patients



IVIG is reported as the most widely available type of treatment in all surveyed countries. SCIG is reported to be available in 72% of countries In addition 13% of countries report having access to SCIG under special circumstances (ie clinical trials)

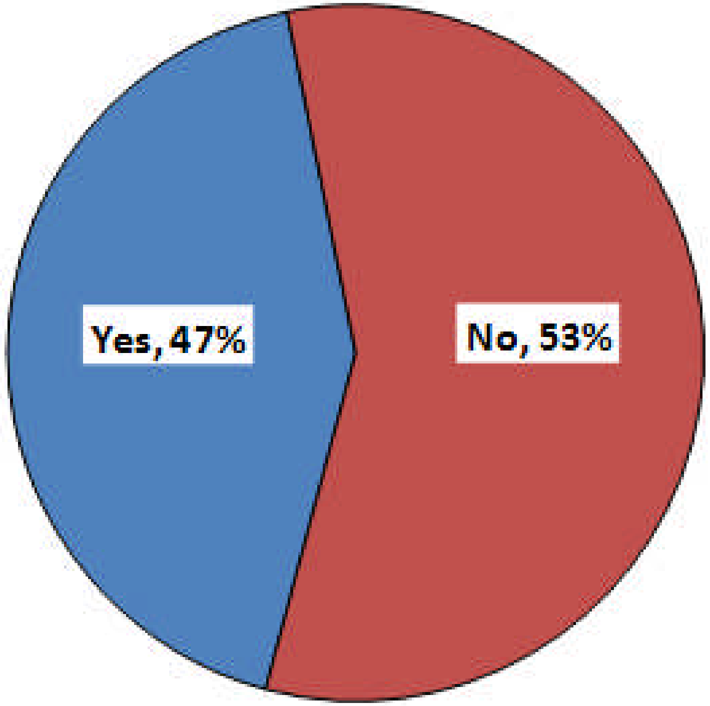
Availability of Treatment for Patients*



- 🟩 **IVIG /SCIG / BMT-SCT/ Other**
- 🟧 **IVIG /SCIG/ BMT-SCT**
- ▨ **IVIG /SCIG**
- 🟪 **IVIG /BMT-SCT/Other** (Morocco)
- 🟨 **IVIG /BMT-SCT** (Serbia)
- ⬛ **IVIG /SCIG/ Other** (Ireland)
- 🟥 **IVIG** (India, Estonia)

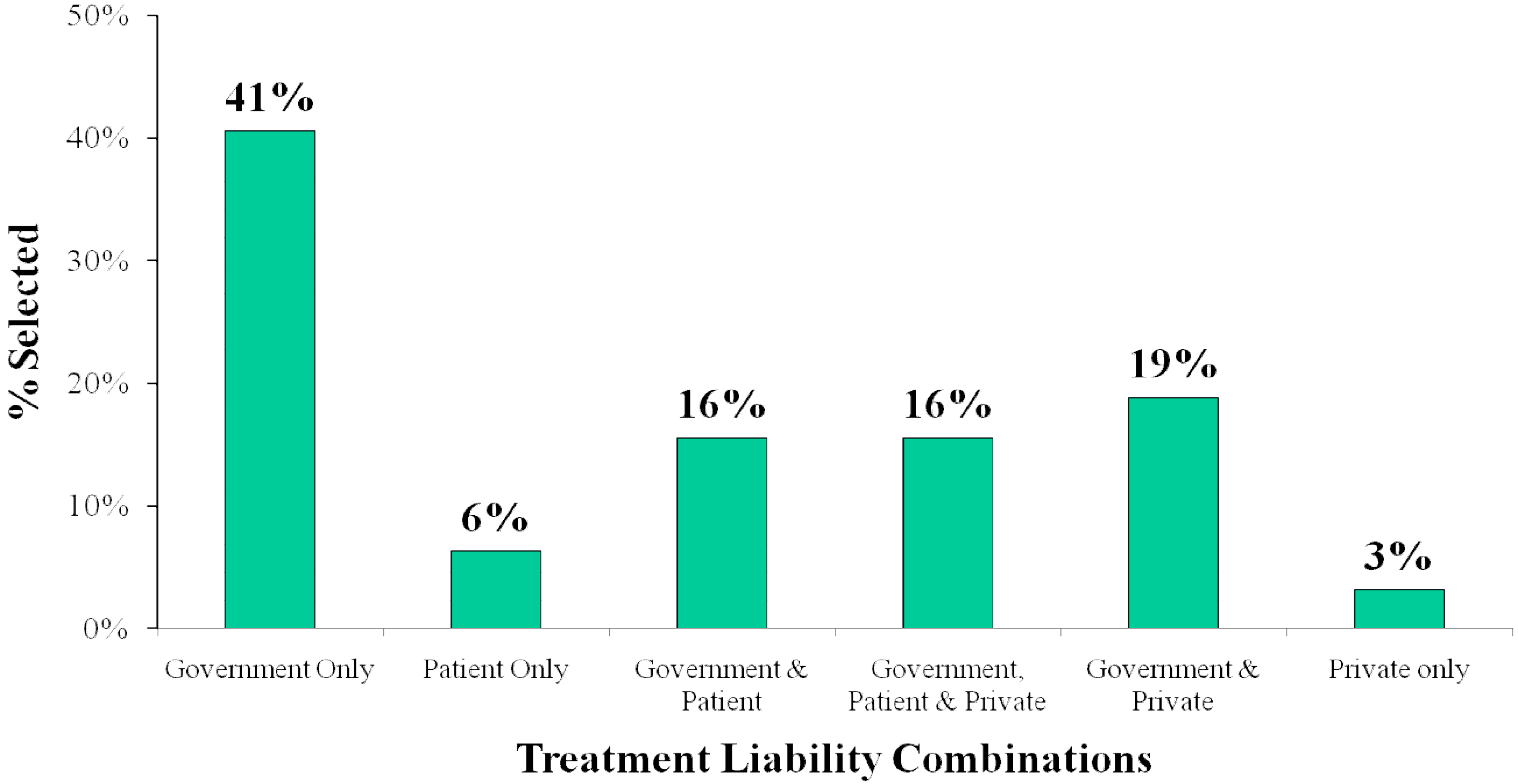
* Includes SCIG under special circumstances (i.e.: clinical trials)

Patient Liability for PID Treatments



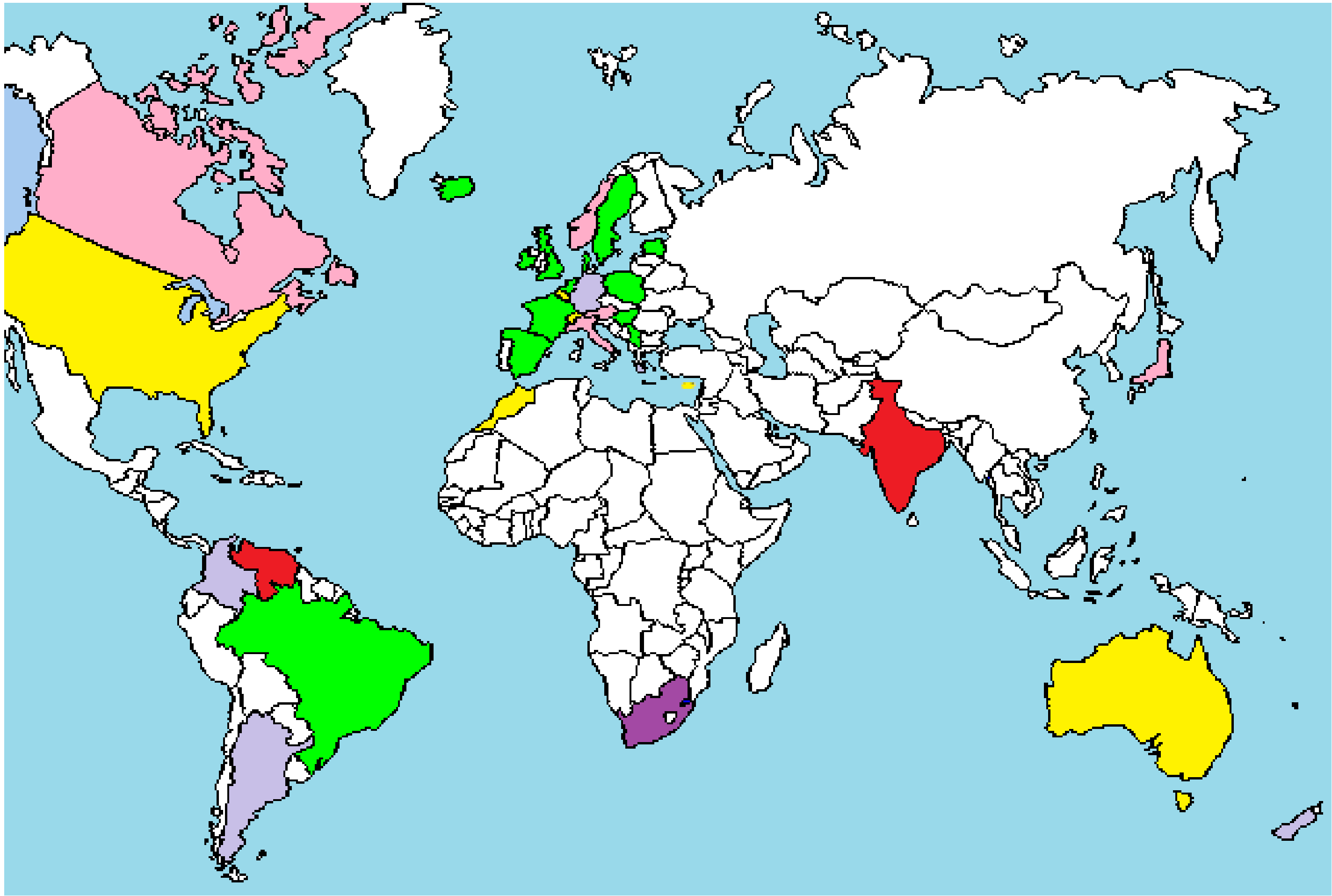
47% of IPOPI’s NMOs indicated that patients in their country do have to pay for some portion of their treatment. In some countries this includes paying for their IG therapies, in others only for a portion or for treatments such as antibiotics or for the day of hospitalization

How Patient Treatments are Paid



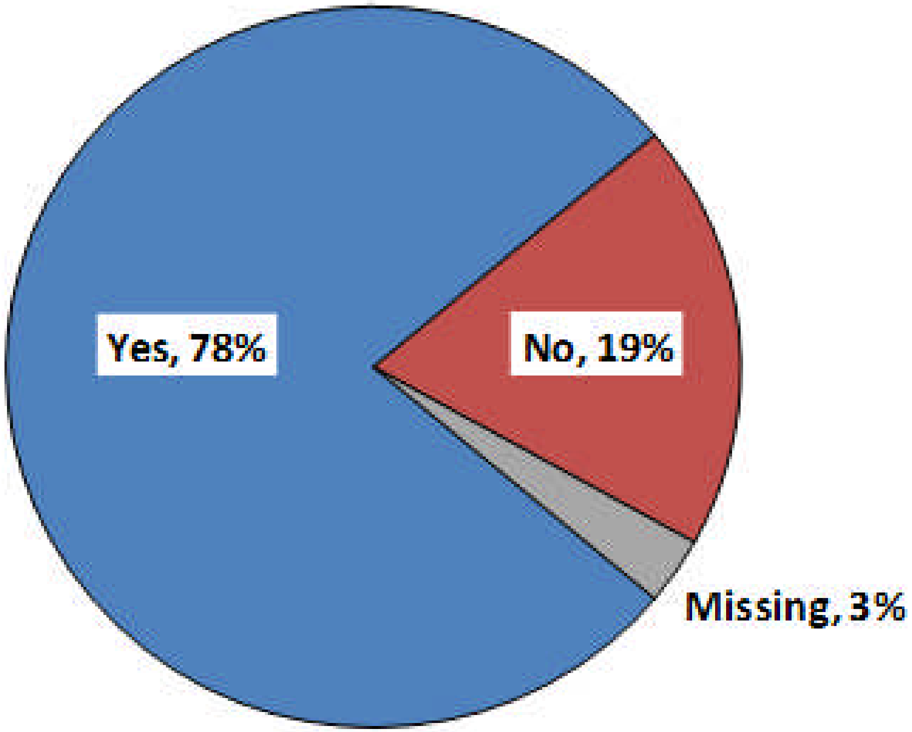
The most reported type of treatment coverage is through government health plans and private health plans. Still 6% of NMO's report that their patients have to cover the costs of treatment themselves

How Patient Treatments are Paid



- Government Health Plan
- Patient only
- Government & Patient Insurance
- Government & Private Insurance
- Private Insurance
- Government, Patient & Private Insurance

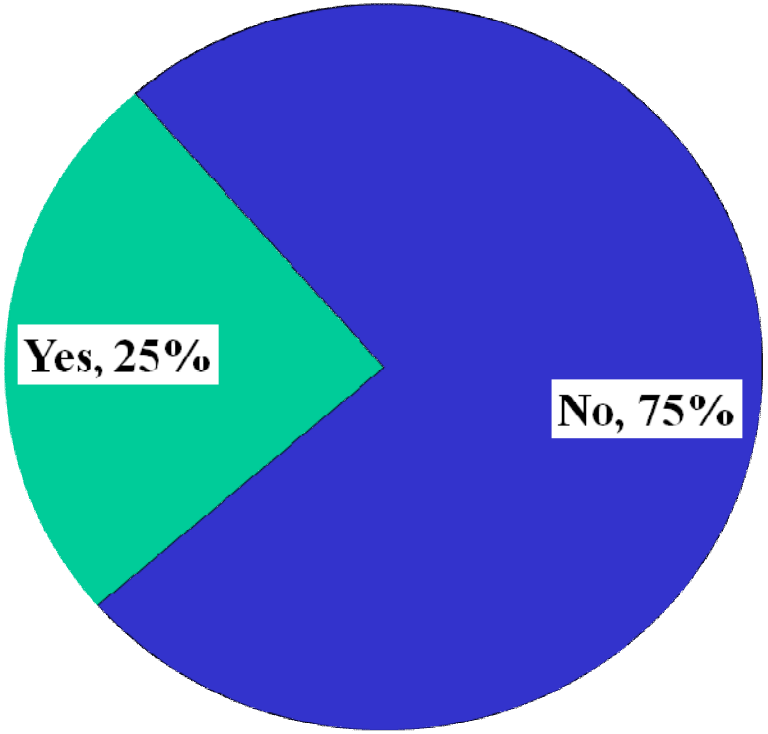
NMOs With Medical Advisory Committee



78% of IPOPI's NMOs have a Medical Advisory Panel

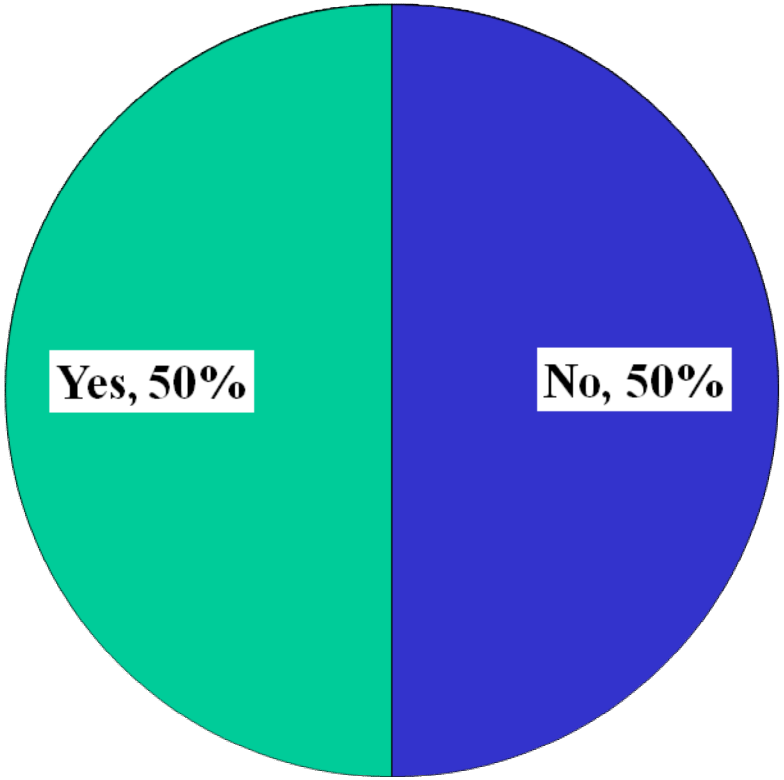
→ IPOPI strongly encourages all of its NMOs to establish a Medical Advisory Panel. The mission of the MAP is to provide guidance on all matters concerned with the medical care of people living with a PID.

NMOs With Chapters or Sub-Groups



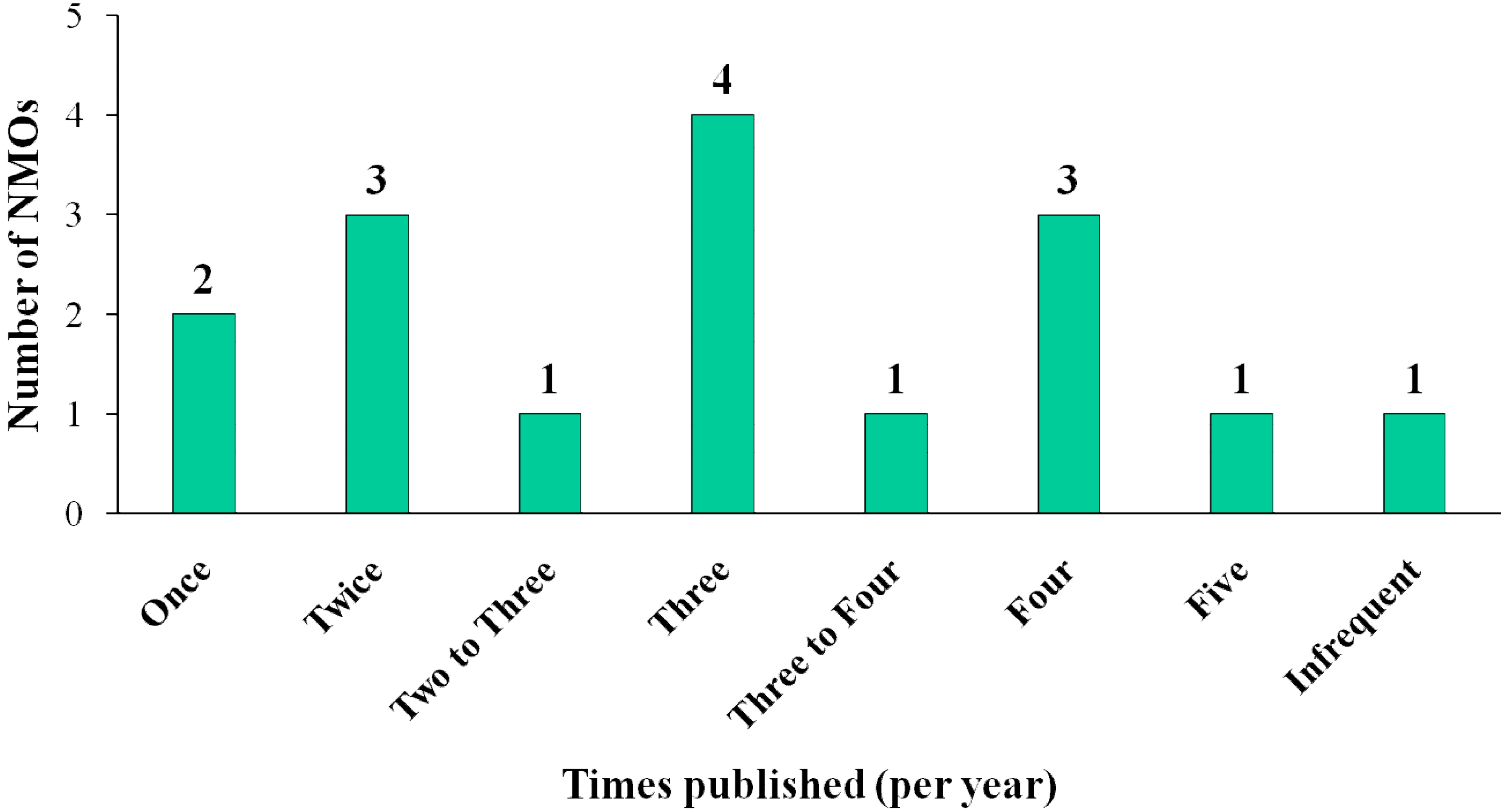
To ensure an efficient and consistent national strategy, IPOPI strongly encourages the presence of only one NMO per country. Should regional representation be absolutely needed chapters reporting to an overarching national organisation could be envisaged

NMO Printed Newsletter



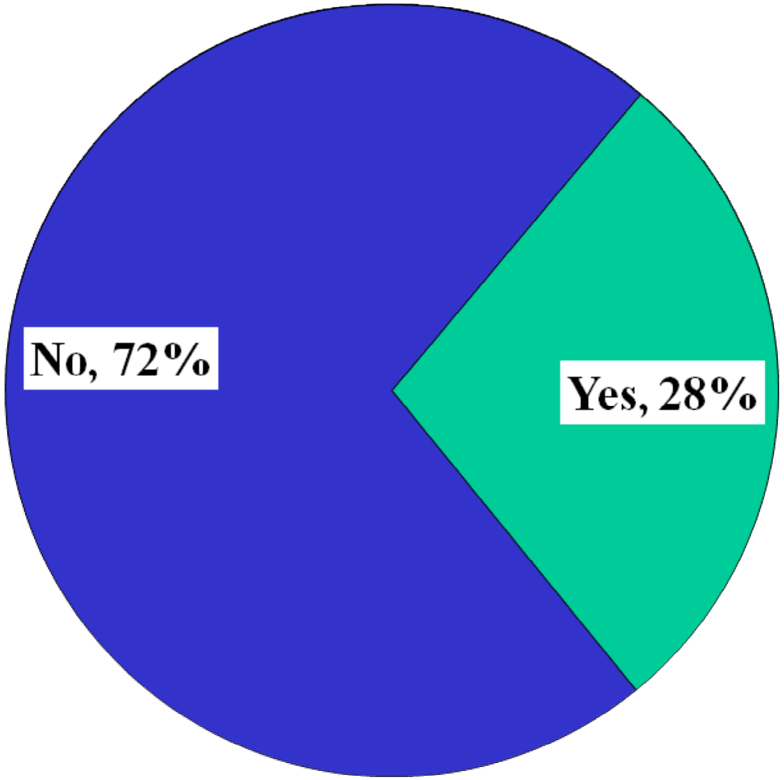
Half of IPOPI’s NMO’s report publishing a printed newsletter.

Frequency Print Newsletter is Published



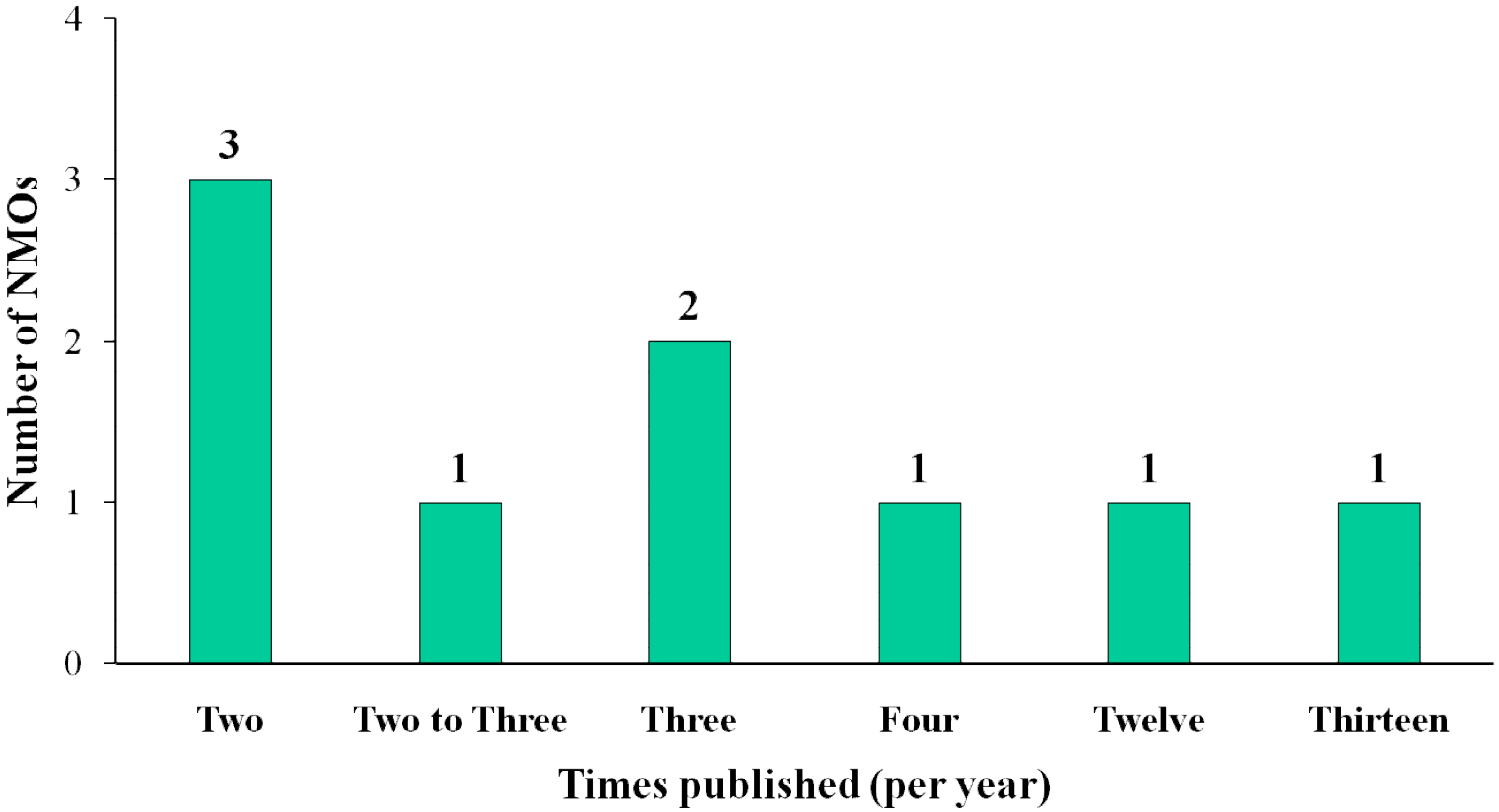
These newsletters are published between 1 to 5 times per year

NMO E-Newsletters



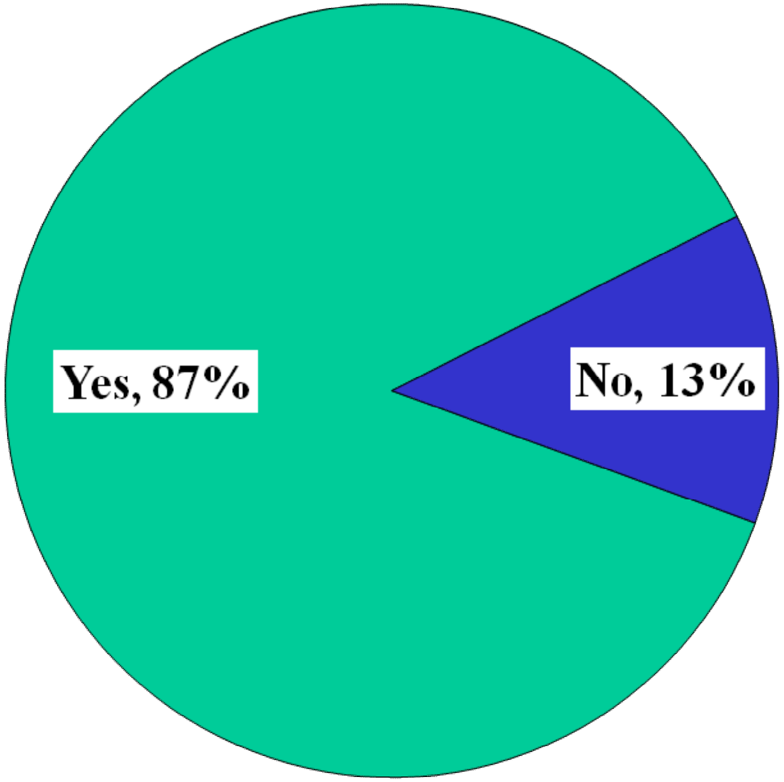
Only 28% of IPOPI’s NMOs report publishing an electronic newsletter

Frequency E-Newsletter is Published



These electronic newsletters are published between 1 to 13 times per year

NMO s With a Website



87% of IPOPI's NMO's report having an active website

Conclusions

Good to know....	...areas for improvement
Better estimate of how many patients and diagnosed patients per country	Gap between estimated number of patients and actual diagnosed cases
Demographics: good overall picture adult/paediatric patients and spilt per condition	Condition split figures to be improved (1/3 missing)
Presence of registries in 50% of countries	Presence of registries in 50% of countries Collaboration between national and international registries in 30% of cases
Overall good access to care for diagnoses patients	What about undiagnosed patients?
Overall good coverage through national health insurance plans	Still countries with no government coverage

Conclusions

Good to know....	...areas for improvement
Most NMO's have established a Medical Advisory Panel (MAP)	Minority without a MAP
Communication tools: Large majority of NMO's do have a website	Communication tools: Minority with no website 50% NMO's do not have a printed newsletter and 72% do not have an e-newsletter

Conclusions

- Encouraging participation from IPOPI NMO’s

- Good first overview on number of patients, demographics, provision of care, presence of registries and communication tools

- IPOPI intends to conduct more surveys in the future to build a database of key information. This will strengthen IPOPI’s advocacy efforts to promote access to care and early diagnosis on behalf of PID patients worldwide