



IPOPI Overview of Recent Achievements

7 June 2013

Johan Prevot
IPOPI Regional Patient Meeting
ASID Congress, Sun City
South Africa



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1. Introduction and History of IPOPI
2. Structure
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Mission Statement

- IPOPI is the Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide





History

- IPOPI came into being as a result of an initial meeting in Oxford, UK, in September 1990, when an interim organising committee was established
- Two years later in 1992, IPOPI was formally established. in Lugano, Switzerland.



THE GLOBAL ORGANIZATION WORKING
TO IMPROVE THE QUALITY OF LIFE FOR PEOPLE
WITH PRIMARY IMMUNODEFICIENCIES





IPOPI has 46 National Member Organisations (NMOs)

- **Argentina, Australia, Austria, Belarus, Belgium, Bosnia & Herzegovina (A), Brazil, Bulgaria (A), Canada, Chile, Colombia, Cyprus, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, India, Iran, Ireland, Italy, Japan, Mexico, Morocco, The Netherlands, New Zealand, Norway, Poland, Portugal , Romania, Russia, Serbia, Slovenia (A), South Africa, Spain, Sweden, Switzerland, Turkey, United Kingdom, United States of America (2), Uruguay, Venezuela**



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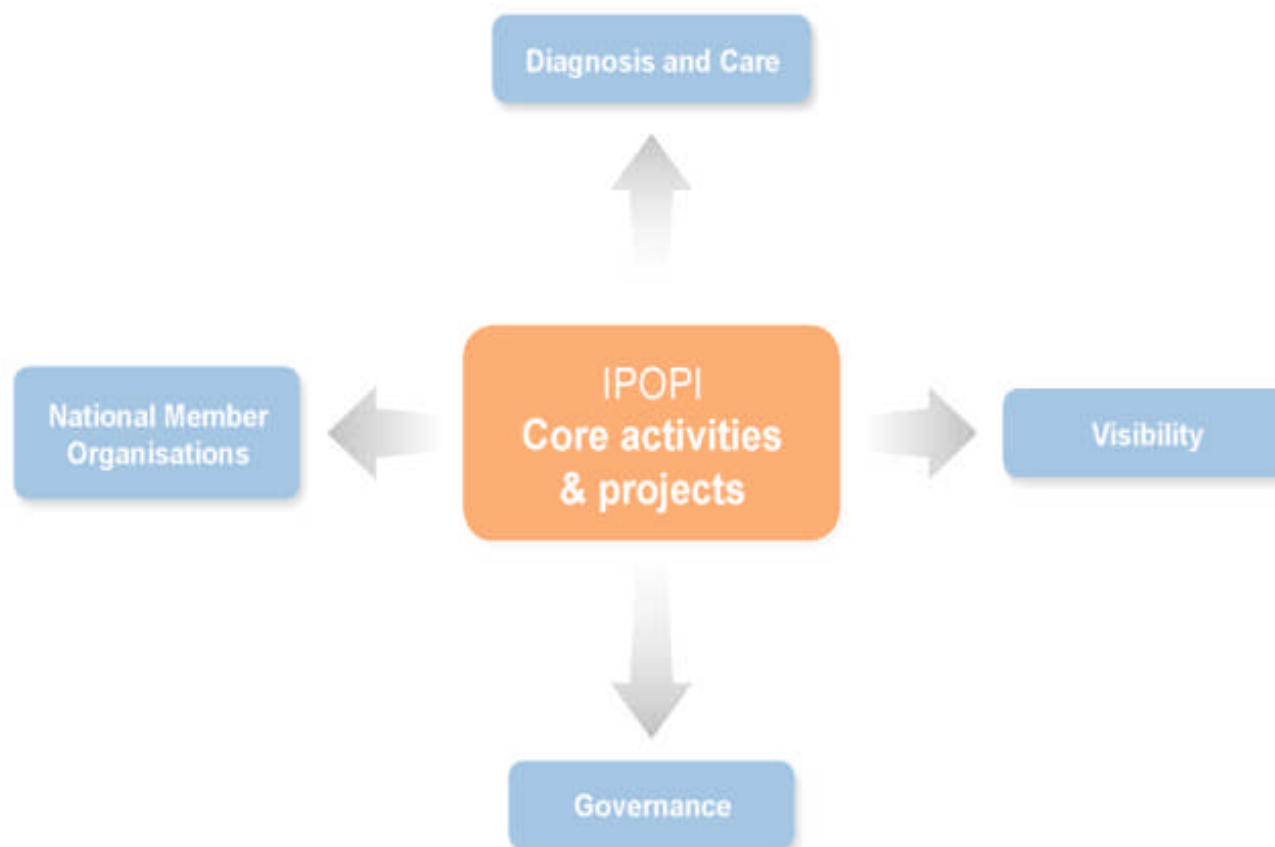


Staff

Johan Prévot	Executive Director
Magda Lourenço	Communications & NMO Programmes
Carla Morgado	Executive Assistant
Carol Tavener	Bookkeeping & Administration
Clare Glynn	Financial consultant
David Watters	Consultant – Projects

Administration office (UK) and Executive Office (Portugal)

Objectives





IPOPI implements these objectives by

- Being the global advocate of the PID patient community
 - in all relevant policy, legislative and regulatory matters
 - for improved access to early diagnosis and safe and effective treatment for PID patients
- Working in close collaboration
 - with its NMOs, other plasma protein user groups and relevant stakeholders
- Monitoring the international supply of immunoglobulins
- Assisting in the development of new NMOs
 - through twinning, advocacy and support programmes
- Organising key events
 - including a biennial conference in close partnership with the professional organisations for clinicians (ESID) and nurses (INGID)

EU Advocacy Campaigns

- European Parliament STOA Panel, 17 March 2004



European Primary Immunodeficiencies
Consensus Conference
19 – 20 June 2006
Paul-Ehrlich-Institut, Langen, Germany

European Primary Immunodeficiencies
Consensus Conference

Consensus Report and Recommendations

Recommendations on:

1. Awareness & Education
2. Screening & Diagnosis
3. Treatment & Management



Recommendations of the PID Expert group chaired by
Jorgo Chatzimarkakis MEP



Working Meeting and Lunch

**DISCUSSION AND ADOPTION - EXPERT
RECOMMENDATIONS FOR BETTER MANAGEMENT OF
PRIMARY IMMUNODEFICIENCY (PID)**

- PID Forums 2011 & 2012



**Recommendations,
Parliamentary Questions
and EP resolutions**



**Building political momentum
around PID issues**

- PID Forum Follow up campaigns
 - SCID newborn screening meeting, UK, 2011



- SCID Newborn screening resolution campaign
- Rare Diseases plans – French Policy Event

SCID Newborn screening – IPOPI's actions

IPOPI's EU SCID NBS campaign started in 2011 and has been conducted in two phases:

Phase I – preliminary policy positioning of need for EU action on SCID NBS, which included the following milestones:

- PID Forum SCID New born Screening, 15 June 2011, EU Parliament, Chaired by Socialist MEP Glenis Willmott. First policy positioning of SCID at EU level
- Set of EU Recommendations agreed June 2011
- Willmott Parliamentary Question on SCID NBS 20 July 2011
- EU Commission response 30 August 2011 (encouraging but more data needed)
- SCID NBS Follow Up UK East Midlands Meeting with Glenis Willmott – BBC and media coverage . First positioning of SCID NBS in UK (agreement on actions in UK and continuation of EU campaign) Nov 2011
- PLUS Meeting with Commissioner Dalli, Oct 2011 – Need for EU action on SCID NBS discussed & promoted
- Meeting with Mrs Willmott and Commission Head of Unit S Schreck Jan 2012 – Need for EP Resolution or Oral Question to influence upcoming Commission policies on NBS identified



Phase II – advocacy campaign for Rare Diseases NBS Oral Question and/or resolution using SCID as case study, which included the following milestones:

- Technical / expert policy support to Glenis Willmott office started Q2 2012
- Call to Action launched at EU Parliament to gather wide r support and stir the debate on European guidelines for rare diseases NBS
- Cross-party political support obtained Q3 2013
- Meeting with Socialist ENVI Coordinator L McAvan Sep 2012. McAvan support obtained.
- Meetings with Christian Democrat and Liberal ENVI Coordinators Q4 2012. Support obtained.
- ENVI Coordinators Meeting Jan 2013 – request for more information
- ENVI Committee Meeting 24 January – Support obtained & decision to table Oral Question
- Oral Question tabled 8 February 2013 – Key achievement
- ENVI Meeting 25 April 2013 – EP supportive, EC cautious and referring to NBS as national matter
- Next steps: EU -Meeting with Commission and EUCERD Outreach and National: Policy Events

Key EU Dossiers

IPOPI active on following key EU dossiers :

- Council Recommendations on Rare Diseases – July 2009 & National Rare Diseases Plans – an opportunity for the PID community
- Pharma package: Patient Information, Counterfeit and Pharmacovigilance
- Cross-border healthcare directive
- European Commission Blood Directive & Reports on Voluntary Donations
- European Commission Committee of Experts on Rare Diseases to advise and assist in formulating all legislation that affects the treatment and care of rare diseases
- EMA Patients and Consumers Working Party (PCWP)

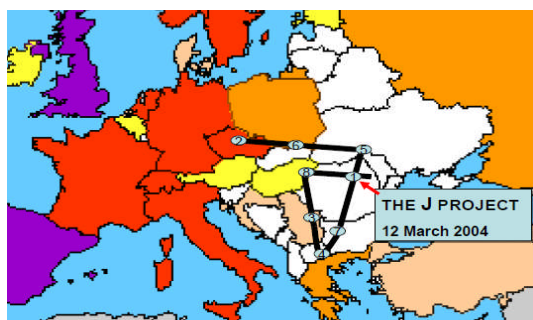
IPOPI in South America

- LASID/ IPOPI Meetings for Latin American PID patient groups, Cartagena & Mexico City



IPOPI in Eastern Europe

- Belgrade workshop 2012, First Russian PID patient meeting, Moscow, November 2011 & Eastern European Stakeholders Meeting Berlin Nov 2010



Specific aims of the J Project

1. To organize professional meetings on PID and related diseases in several ECE countries with low number of registered PID patients.
2. To discuss diagnostic and therapeutic practices and problems, and to define specific areas to be improved and to be supported by other European groups, institutions, companies, and foundations.
3. Updating national PID registries
4. Establishing PID professional working groups
5. Establishing PID Patients' groups

IPOPI in Africa

- IPOPI/ASID meetings 2010 & 2012, Casablanca & Hammamet.





IPOPI Biennial Meetings with



Group picture



Past Meetings:

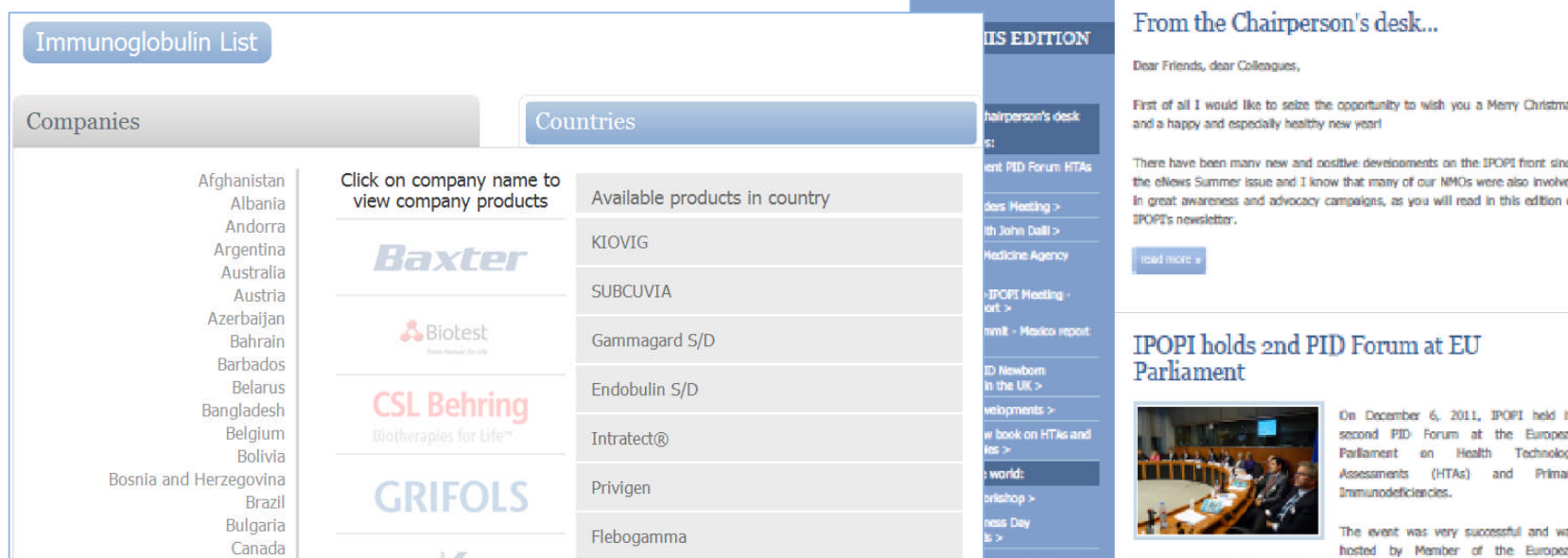
- 1992 Lugano, Switzerland
- 1994 Sitges, Spain
- 1996 Goteborg, Sweden
- 1998 Rhodos, Greece
- 2000 Geneva, Switzerland
- 2002 Weimar, Germany
- 2004 Versailles, France
- 2006 Budapest, Hungary
- 2008 Den Bosch, Holland
- 2010 Istanbul
- 2012 Florence

Upcoming Meetings:

- 2014 Prague
- 2016 Barcelona

Communications Tools

- IPOPI Website provides efficient online resource for PID patients and stakeholders
- New Social Media Tools  Join us on **facebook**  Follow us on **twitter**
- eNews – quarterly electronic newsletter
- IG Database: online directory of IG therapies available worldwide



Immunoglobulin List

Companies Countries

Click on company name to view company products

Available products in country
KIOVIG
SUBCUVIA
Gammagard S/D
Endobulin S/D
Intratect®
Privigen
Flebogamma

E-News
FALL/WINTER 2011
The e-newsletter of the International Patient Organisation for Primary Immunodeficiencies

From the Chairperson's desk...

Dear Friends, dear Colleagues,

First of all I would like to seize the opportunity to wish you a Merry Christmas and a happy and especially healthy new year!

There have been many new and positive developments on the IPOPI front since the eNews Summer Issue and I know that many of our NMOs were also involved in great awareness and advocacy campaigns, as you will read in this edition of IPOPI's newsletter.

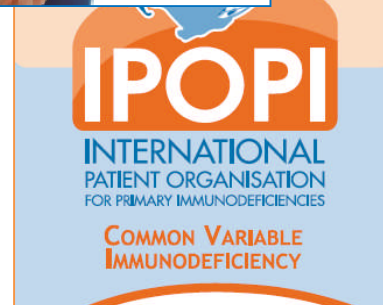
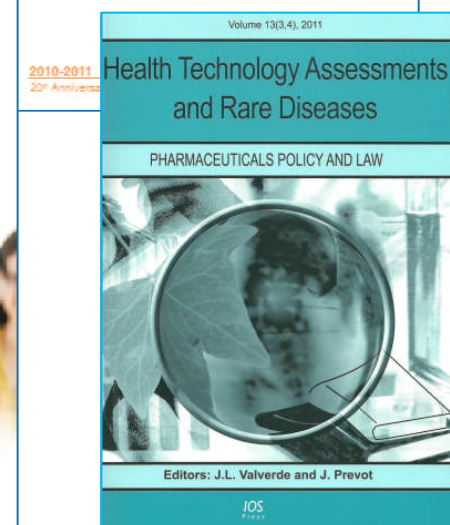
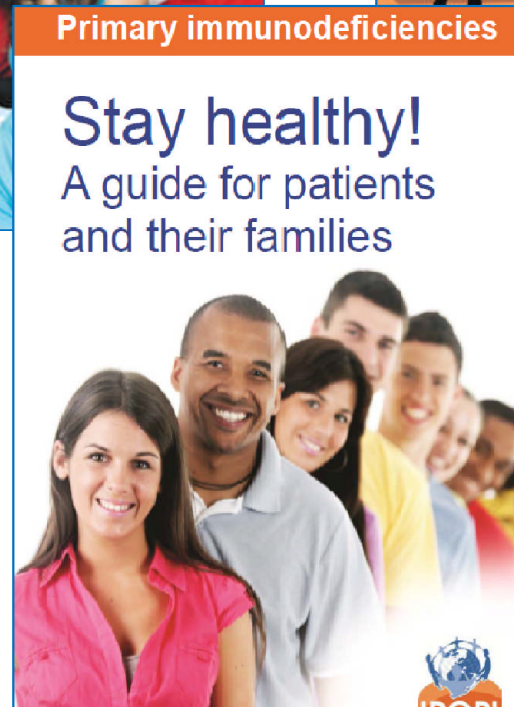
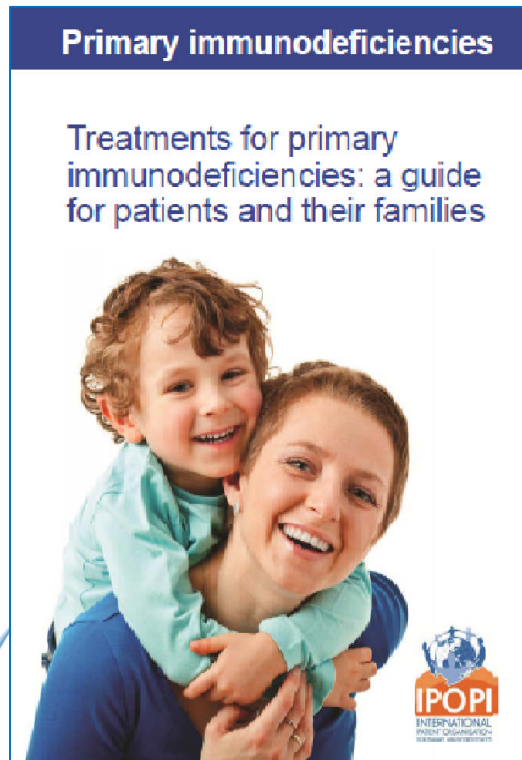
[Read more »](#)

IPOPI holds 2nd PID Forum at EU Parliament

On December 6, 2011, IPOPI held its second PID Forum at the European Parliament on Health Technology Assessments (HTAs) and Primary Immunodeficiencies.

The event was very successful and was hosted by Member of the European

Publications





IPOPI NMO Support Tools and Programmes

- Awareness and Advocacy Campaign Toolkits
- World PI Week NMO Support Programme
- Awareness campaign Workshops (National Scope)
- Advocacy and Media Training Intensive Workshop (Regional Scope)
- NMO Professionalization Programme (National scope)
- IPOPI NMO Support Grant Programme
- IPOPI World PI Week Support Grant Programme
- IPOPI Toolkits and Guidelines
- IPOPI NMO Website Support Tool



IPOPI PID Patient Needs & Outlooks Survey

A Report based on 300 patient questionnaires



Report Prepared by BRYTER 

Background & objectives

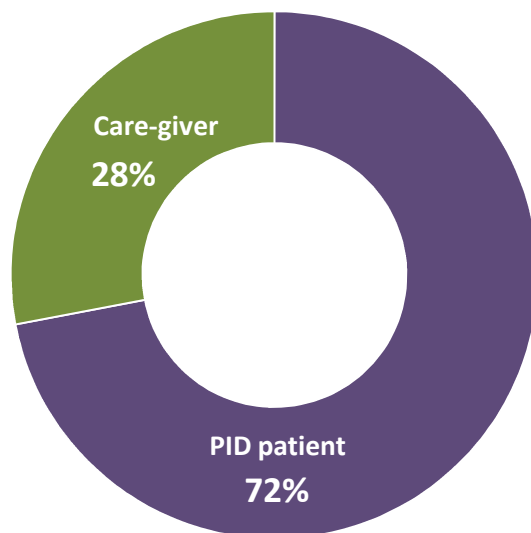
Research goals and objectives

- The study has been designed to provide detail on the current landscape, outlook and needs of patients in relation to their circumstances, outlooks and treatment needs with PID
- This study explores the patient experience of PID, covering aspects from treatment and unmet needs to the impact of PID on daily and social life.
- The conjoint section asked respondents to evaluate a number of treatment options in rotation to establish unmet needs.
- Sample:
- N=300: Patients & Care-givers of people with PID and treated with immunoglobulins. Sample sourced through national member organisations (NMOs) affiliated to the International Patient Organisation for Primary Immunodeficiencies (IPOPI). Sample was self-selecting amongst those invited by the NMOs.

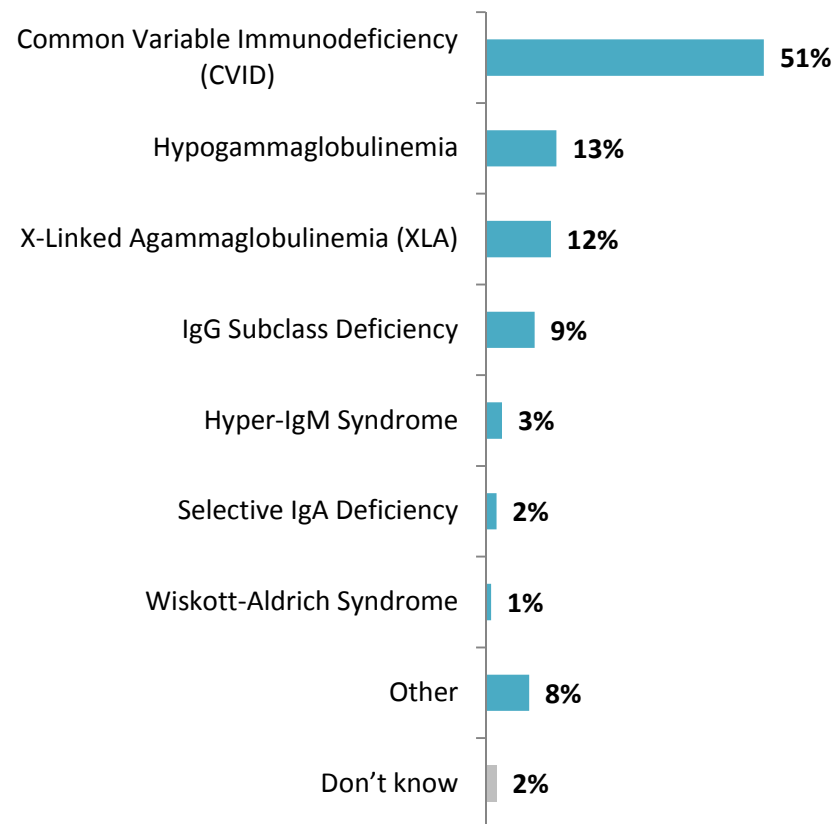
Country	Groups	Interviews
UK	A, B	59
Sweden	A	34
Canada	C	31
France	A, B	31
Germany	A, B	31
Spain	A, B	22
Portugal	A	21
Argentina	C	15
Brazil	C	13
South Africa		10
Colombia	C	9
Italy	A, B	9
Switzerland	A	4
Belgium	A	2
New Zealand		2
Poland	A	2
Australia		1
Austria	A	1
Hungary	A	1
India		1
Netherlands	A	1

7 in 10 survey respondents are patients with the remaining made up by care givers. CVID is the most widely represented diagnosis of PID.

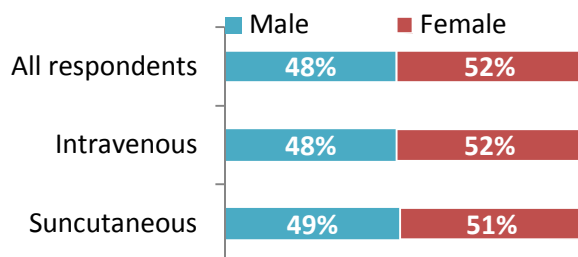
Role in relation to PID



Patient Diagnosis



Gender



P1: Which of the following best describes your role in relation to PID?

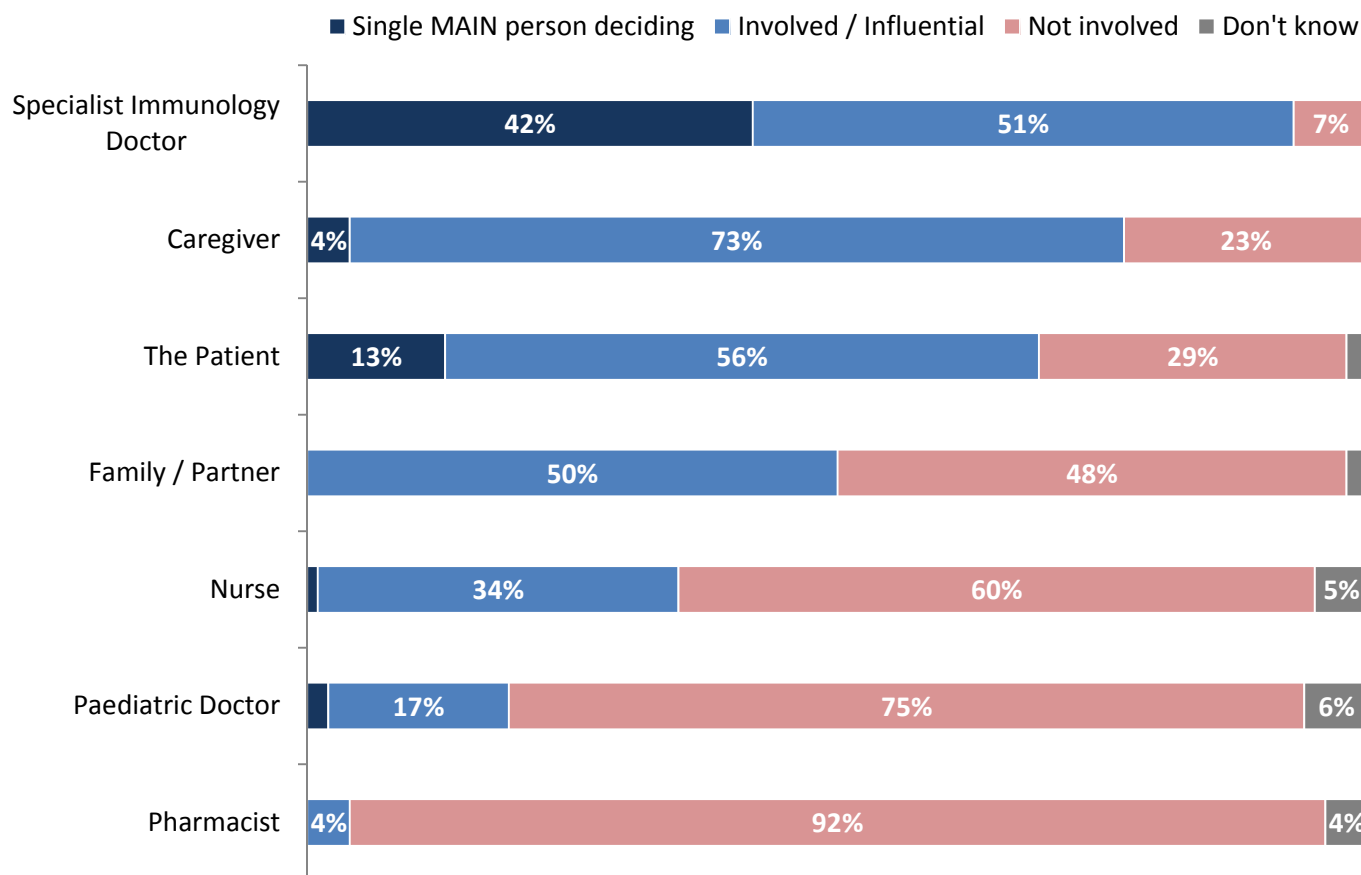
D1: And what is your specific diagnosis/ What is the diagnosis of the PID patient you care for?

Q.C0b/Q.C3: Are you ... Male or Female

Base: All Respondents (300)

For 42%, immunology specialists are the main decision maker regarding how Ig therapy is administered. However, around 70% of patients and 77% of caregivers were involved in the decision-making process

Decision makers for route of administration

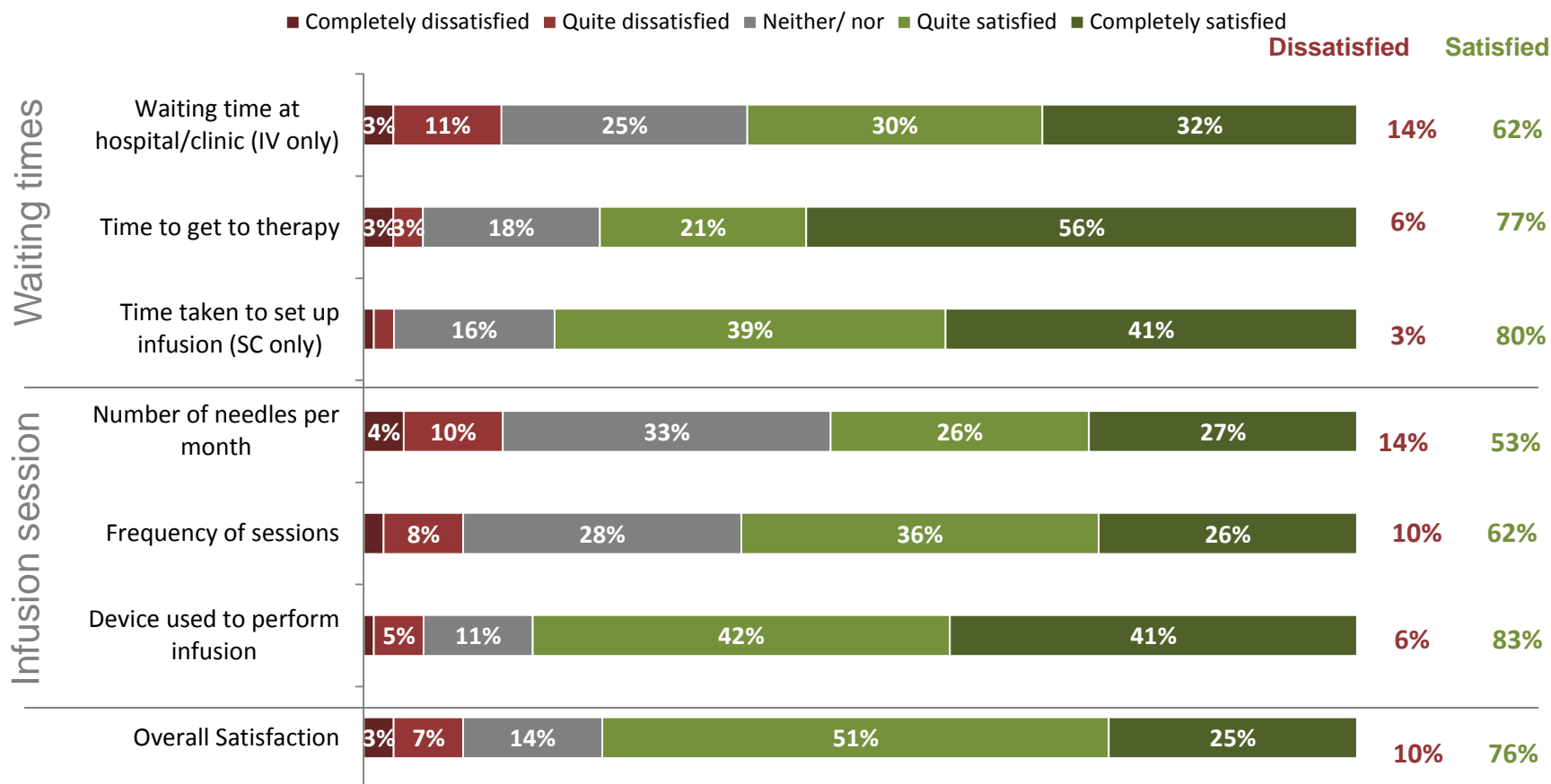


D5a. Thinking about the decisions and selection of the current therapy... Who was involved in choosing how the therapy is administered?

Base: All Respondents (300)

Overall, three quarters (74%) are satisfied with their treatment.
However, 1 in 5 (18%) are dissatisfied with the number of needles
to contend with each month

Satisfaction with aspects of current treatment



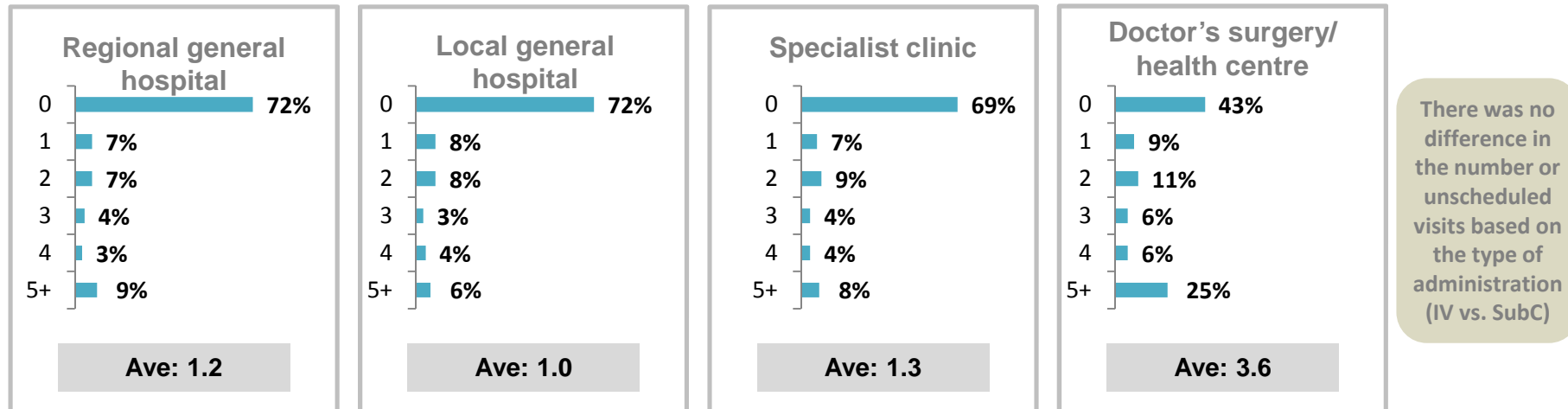
D19a: How satisfied are you with the following...

Base: All Respondents (300), SubC only (134), IV only (160)

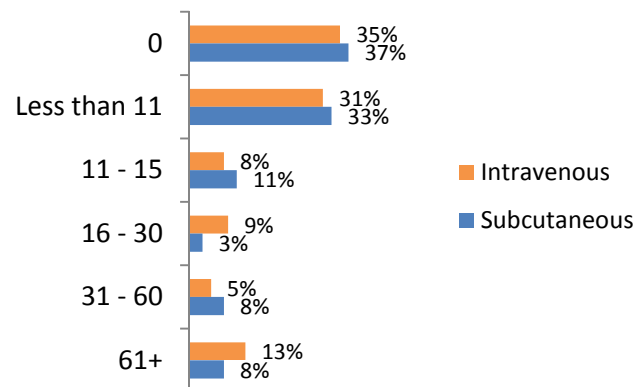
Sixty-six percent (using intravenous Ig) and 70% (using subcutaneous Ig) report missing 10 or fewer work/school days during the past 6 months. Of these, 35% (using intravenous Ig) and 37% (using subcutaneous Ig) missed 0 days



Unscheduled visits in relation to PID in last 12 months



Days missed at work/education due to ill health in last 6 months



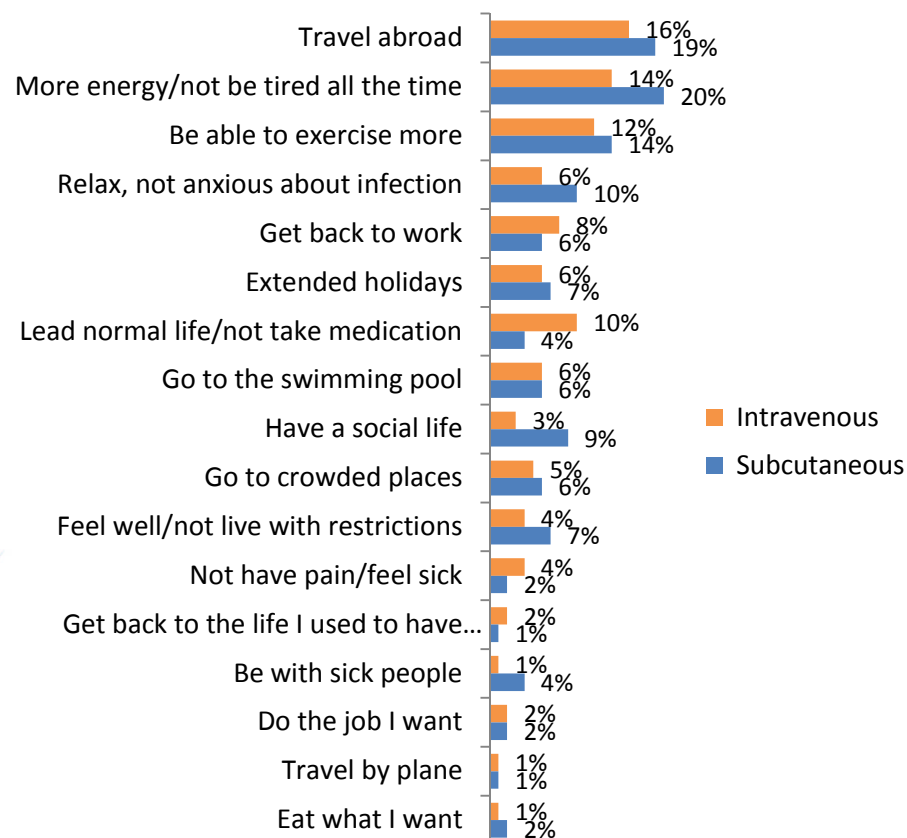
H4: In the last 12 months, how many unscheduled or emergency visits have you/the patient made to each of the following in relation to PID?
H5: And how many days have you/the patient missed at work/education due to ill health in the past six months?

Base: All Respondents (300)



PID patients would like to take part in 'everyday' activities: travelling / going abroad was mentioned by most (19%) of subcutaneous patients as the one thing they would like to be able to do, but don't feel they can because of PID

One thing patient would LIKE to be able to do but don't feel they can, because of PID (spontaneous mentions)



"Travel to countries very far away."

"Have enough energy for a full day and going out at night without needing a nap and early night!"

"Travel for over 2 weeks without the need to bring medical equipment or make advanced planning."

"Get back to life I used to have before I was diagnosed with PID."

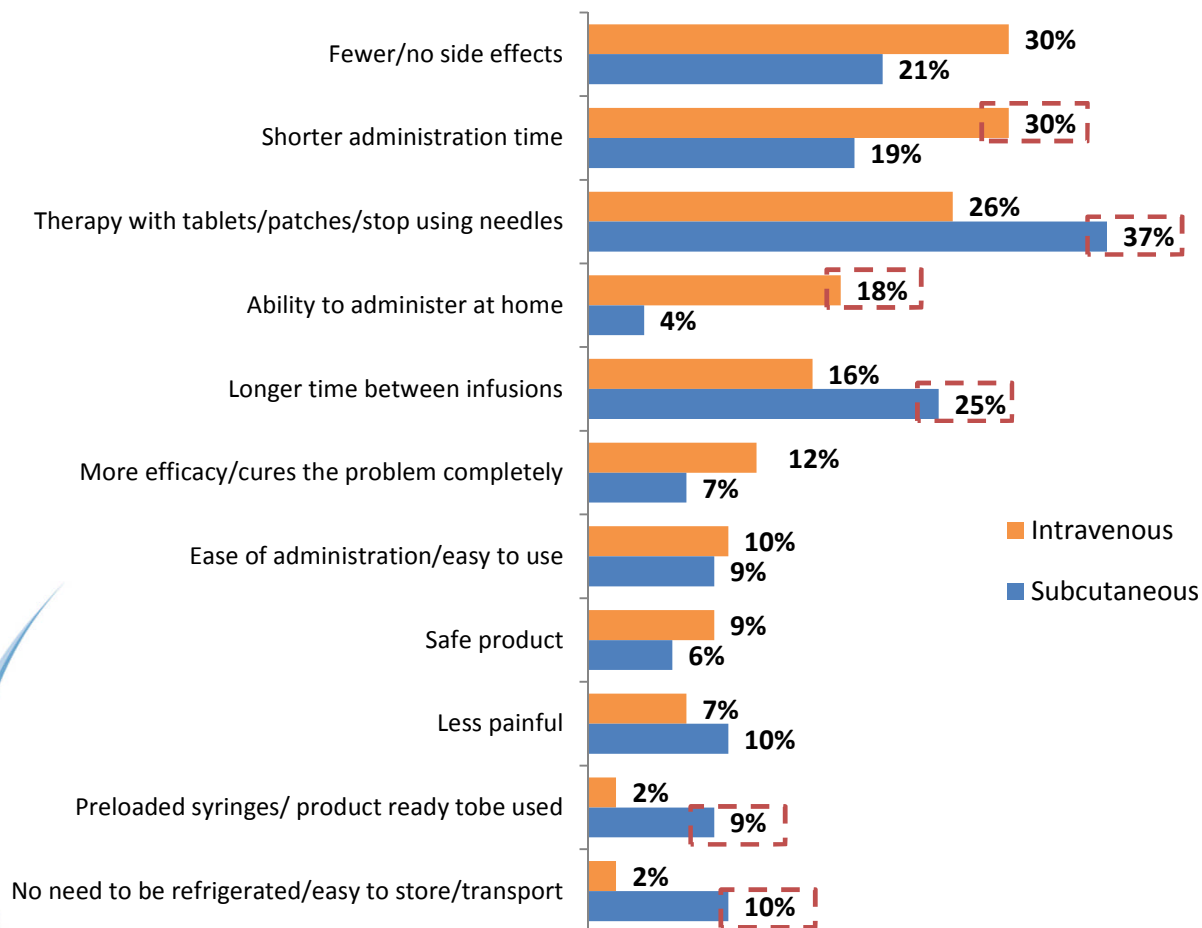
"I would like to meet other people even during times there's a lot of flu etc. Don't like trying to avoid crowded places etc. because the risk for infection."

H6: In your opinion, what is the one thing you would LIKE to be able to do but don't feel you can, because of PID? MULTIPLE RESPONSE

Base: All Respondents (300)

Intravenous and subcutaneous patients differ regarding the features they look for in an ideal product to treat PID.

Features of an ideal treatment for PID (spontaneous mentions)



Compared to subcutaneous patients, a significantly higher share of intravenous patients mentioned 'shorter administration time' and 'ability to administer at home' as a feature of an ideal product.

Among subcutaneous patients 'longer time between infusions' features more often as an attribute of an ideal product. 9% of them would like to use 'preloaded syringes'.

Significant difference between intravenous and subcutaneous therapy: [red dashed box]

D21: Thinking about future PID treatments imagine you were working with a medical design team what two features would you look for in the ideal product?

Base: All Respondents (300), Intravenous (160), Subcutaneous (134)



Amongst both intravenous and subcutaneous patients, the positive elements of their current treatment (e.g. intravenous – less frequent infusions needed) seem to have more weight in the decision about how therapy is administered

Importance of attributes by current route of administration (intravenous vs. subcutaneous)

Conjoint analysis

	All Respondents	Current Intravenous	Current Subcutaneous
Convenience around scheduling	15%	13%	18%
Dosing frequency	19%	24%	14%
Where you take the treatment	22%	17%	29%
Number of needle sticks per treatment	20%	24%	15%
Time to take each treatment	23%	23%	23%

Colour coding

High

Average to high

Average

Low

The colour coding indicates whether a score of the subgroup is higher or lower compared to the score at total level.

Base: All Respondents (300)

PID patients are below US norms across Physical and Mental elements

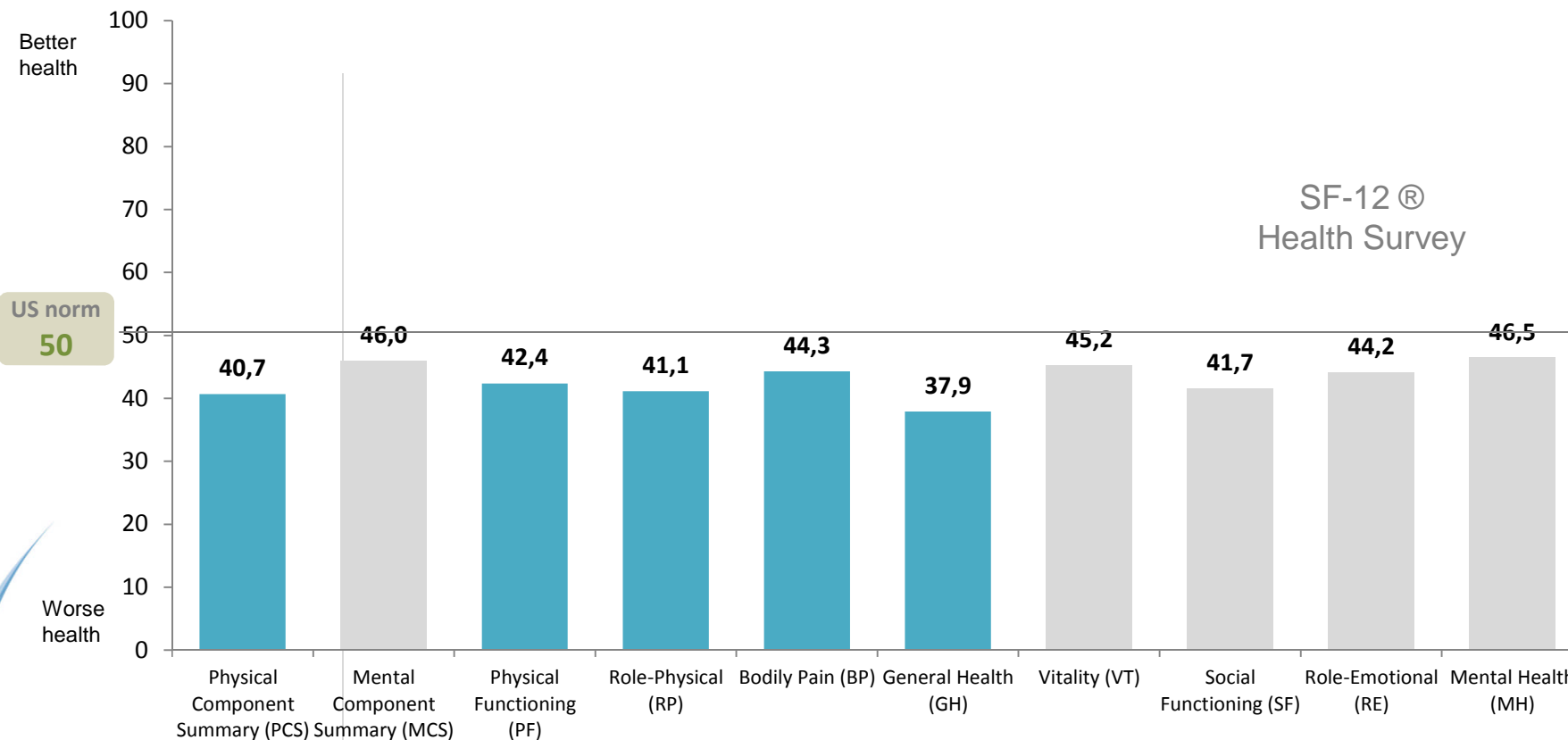


SF-12 Component Scores – Norm Based Scores (NBS)

SF-12v2® Health

Scores for Total Sample

Physical Health Scores Mental Health Scores



F1. In general, would you say your health is... F2. Does your health now limit you in these activities. F3: During the past 4 weeks, how much of the time have you had any of the following problems with your work/other regular daily activities as a result of your physical health? F4 During the past 4 weeks, how much of the time have you had any of the following problems with your work or other regular daily activities as a result of any emotional problems (such as feeling depressed or anxious)? F5: During the past 4 weeks, how much did pain interfere with your normal work (including both work outside the home and housework?) F6: How much time during the past 4 weeks ... F7 During the past 4 weeks, how much of the time has your physical health or emotional problems interfered with your social activities (like visiting friends, relatives, etc.)? Base: All Respondents who are PID patients (216)

Conclusions IPOPI Survey – Patient Needs and Outlooks



Main area where IV & SC patients would like treatments to be improved

34% of IV patients believe headaches are the most important area to improve. For SubC patients, pain at the infusion site and swelling at the infusion site are priorities for improvement though 19% say it is 'highly important' to focus on headaches too.

IV & SC patients views on ideal treatment

Spontaneously 30% of IV patients said they wanted a treatment with less side effects compared to 21% of SubC patients. SubC patients were most likely to say a therapy without needles (37%) and more time between infusions (25%). IV patients also mentioned a shorter admin time (30%) and the ability to administer at home (18%)

Preference analysis showed relatively level importance around attributes in choosing treatments

Drivers of choice were time to take each treatment (23%), site of treatment - at home vs. medical centre (22%), number of needle sticks (20%), dosing frequency (19%) and convenience on scheduling (15%).

A preference was shown for self-administration at home, infrequent, quick to administer dosing and few needle sticks

Half of IV respondents (52%) would prefer a SubC type therapy similar to those available. This would be preferable for 91% of existing SubC respondents.

- There is a range of projects and activities that IPOPI intends to roll out and implement in terms of
 - Access to appropriate care and treatment levels
 - Access to early diagnosis
 - Increased number of active and efficient national patient groups
 - Visibility for PID and political recognition of this group of important chronic disorders
 - Defending PID as a key indication for Igs which are life saving replacement therapies for which there is no alternative treatment
- Through IPOPI's international, regional and national core activities and ad-hoc projects....

1st IPIC



- First International Primary Immunodeficiencies Congress

Registration open!

7 & 8 November 2013, Estoril (Lisbon), Portugal





Many other projects....

- PID Forums
- Awareness and Advocacy Workshops
- Launch & professionalization of NMOs
in target geographies Latin America, Asia, Africa, Europe
- IPOPI survey – next projects QoL/cost benefit survey
- SCID newborn screening policy programme
- IPOPI National Parliamentary Programme
- New patient information publications & materials
- New communications tools including PID patients access to care
video & NMO support webinar programmes
- PLUS campaigns
- WPIW 2014
-and more!



Thank you to
IPOPI Core Sponsors



GOLD



SILVER

GRIFOLS





**Thank you for your
attention!**