

IPOPI PID Patient Needs & Outlooks Survey

A Report based on 300 patient questionnaires







PID Patient Needs & Outlooks Study

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Background & objectives



Background

- Primary immunodeficiencies (PID) are a group of diseases in which an individual lacks some of the components of the immune system: cells and/or proteins. This makes the individual more susceptible to infections
- The symptoms of PID can range from mild to severe
- PIDs are caused by hereditary or genetic defects in the immune system and WHO has identified over 150 specific types
- The International Patient Organisation for Primary Immunodeficiencies (IPOPI) is the association of national patient organisations and is dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide

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.

Research methodology



Mode:	Online (CAWI)	Country	Groups Inte	erviews
Duration:	30 minutes	UK	A. B	59
Fieldwork:	8 April – 17 October 2011	Sweden	A	34
Sample:	N=300: Patients & Care-givers of people with PID and treated	Canada	С	31
	with immunoglobulins. Sample sourced through national member	France	А, В	31
	organisations (NMOs) affiliated to the International Patient	Germany	А, В	31
	Organisation for Primary Immunodeficiencies (IPOPI). Sample	Spain	А, В	22
	was self-selecting amongst those invited by the NMOs.	Portugal	А	21
	0 0 ,	Argentina	С	15
	Questions were phrased differently so as to be appropriate for patients (e.g. "What is your current diagnosis?") and care-givers ("What is the diagnosis of the PID patient you care for?")	Brazil	С	13
		South Africa		10
		Colombia	С	9
Language:	Posnondants were able to answer the survey in	Italy	А, В	9
	English French Cormon Italian Spanish or Portuguese		А	4
	English, French, German, Italian, Spanish of Portuguese	Belgium	А	2
Countries:	Shown in table on right	New Zealand		2
		Poland	А	2
The research stud	y was sponsored by Baxter and carried out by an independent market	Australia		1

research company (BRYTER) in compliance with current codes of conduct and good practice.

Privacy and Data Protection: Bryter act in accordance with the ABPI, MRS and BHBIA codes of conduct regarding respondent anonymity and confidentiality: the aim of the market research survey was to gain views and was not promotional.

Bryter are fully compliant with the Data Protection Act, so that any information provided by respondents is treated in the strictest confidence: all results are pooled so answers are not attributed to any individuals.

Germany	А, Б	ЪТ
Spain	А, В	22
Portugal	A	21
Argentina	С	15
Brazil	С	13
South Africa		10
Colombia	С	9
Italy	А, В	9
Switzerland	A	4
Belgium	A	2
New Zealand		2
Poland	A	2
Australia		1
Austria	A	1
Hungary	A	1
India		1
Netherlands	А	1
		-
Groups: A ("Euro	ope"), B ("EU5"), C	

Gro ("Americas"), D ("South America")

I. Treatment

Background



Of the sample interviewed, 53% receive intravenous Ig infusions and 45% are infused subcutaneously. Usage of subcutaneous infusion is significantly higher in Europe than in the Americas



Route of infusion



D4. What route of infusion if used to administer your/the patient's current immunoglobulin therapy? Base: All Respondents (300), Europe (218), UK (59), Sweden (34), France (31), Germany (31), Spain (22), Portugal (21), Americas (68), Canada (31) 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) Understandably, the time between infusions is predetermined by the route of administration. The majority of intravenous patients (87%) have at least 3 weeks between infusions, while 99% subcutaneous patients wait a maximum of a week





D6. Typically, how long do you wait between your current immunoglobulin therapy infusions? Base: All Respondents (300), Intravenous (160), Subcutaneous (134)

Over 9 in 10 subcutaneous patients are infused at home, while most intravenous patients receive their treatment in a regional or local hospital (64%) or visit a specialist clinic (11%)





D9. Where does your/ the patient's current immunoglobulin therapy infusion usually take place? Base: Intravenous (160), Subcutaneous (134)

Base: Respondents who receive treatment at home or other place Subcutaneous (127), Intravenous (35)

For patients on intravenous administration it takes about an hour on average to get to the place of therapy. It takes 3 and a half hours to carry out the infusion part of the process. Overall time to complete treatment is over 6 hours.



IV patients: Time it takes to...



D11 How long does it typically take for ... Pre and Post infusion time (all the other time spent at the centre but not actually receiving product e.g. waiting to be checked in, loading the vials etc.)? Carry out the infusion part of the process? Base: Intravenous (160)

Overall time for IV treatment:



For subcutaneous patients it takes under 20 min on average to set up the infusion equipment and 1.5 hours to complete the infusion part. The average overall time it takes to complete one session is under 2 hours.



SubC patients: How long does it take to...



Almost all intravenous patients use one infusion site at a time. For subcutaneous patients using two sites is the most common (53%), with a quarter of patients using 3 or more sites during an infusion session.



IPOPI

Amongst IV patients, 78% can find a vein on first try on most or all occasions. For those who don't find a vein for the first time, it takes an average of three attempts



Frequency of finding a vein on first attempt (IV patients)



vein for intravenous administration?

Base: All Respondents answering about IV therapy (160)

1 in 6 patients who receive subcutaneous therapy use a peristaltic/roller pump, with almost two third using a syringe driver to administer the infusion



Peristaltic pump or syringe driver (SubC only)



How the time is spent during the treatment very much depends on the type of administration (IV/SubC) and the place of therapy.As SubC therapy is primarily administered at home, SubC patients can spend time on more home activities





Time spent during treatment

D18: How do you/does the patient spend your/their time during your/their treatment? Base. Intravenous (160), Subcutaneous (134)

I. Treatment

Evaluation of current treatment / unmet needs



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) Subcutaneous patients like their treatment in all aspects of acceptability; time administration takes, being able to fit treatment into schedule, ability to self-administer and overall convenience



Acceptability of aspects of current lg treatment



D19b. Please indicate the extent to which you like or dislike the following aspects of your current immunoglobulin therapy ...

Significant difference between intravenous and subcutaneous therapy:

Base: Intravenous (160), Subcutaneous (134)

Subcutaneous therapy is perceived to perform better on a number of aspects relating to quality of life (convenience, allowing independence and personal freedom) in the survey sample



Evaluation of current therapy in terms of quality of life (mean scores)



D19c. And would you say your/the patient's current immunoglobulin therapy is...? Base: All Respondents (300) More subcutaneous patients experience side effects at the infusion site compared to IV patients. However, for the majority these side effects have low or medium impact on their life and they do not seek to change therapy.



To what extent is life impacted by current therapy – side effects at infusion site



More IV patients experience headaches as a side effect of the therapy. 1 in 10 IV patients discussed switching with their doctors because of headaches, but only 2% is moving to another treatment as a result.

To what extent is life impacted by current therapy – side effects at infusion site

Base: Intravenous (160), Subcutaneous (134)

1 in 3 (35%) intravenous patients consider it important to reduce the occurrence of headaches as a side effect of the intravenous Ig therapy

Importance of improving on aspects of treatment – Intravenous administration

D19e. How important to you would it be to improve on...? Base: Intravenous (160)

Pain and swelling at the infusion site are considered to be important areas to improve by subcutaneous patients. Headaches affect less patients, but for those affected it is an important area to improve

Importance of improving on aspects of treatment – Subcutaneous administration

D19e: How important to you would it be to improve on...?

Base: Subcutaneous (134)

II. Living with PID

84% of all patients with PID visit a specialist immunology doctor in relation to their PID.

Doctor visited in relation to PID

D23a. Which of the following specialist doctor(s) do you/ the patient recently visit relating to your/their PID? Base: All Respondents (300)

45% of subcutaneous patients delayed self-administering by three or more days in the last six months at least once, but 82% haven't skipped a dose. Among IV patients 9 in 10 haven't skipped a dose, significantly more than for SubC.

Delayed self-administering by 3 days or more Number of times in the last 6 months

Skipped a dose

Number of times in the last 6 months

D20b Thinking about your usage of immunoglobulin, how many times in the past six months do you estimate you have... Base: Intravenous (160), Subcutaneous (134)

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)

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)

More subcutaneous patients are optimistic about their lives over the next five years than intravenous patients

Outlook on life with PID over next five years

III. Future Treatment Needs

Ideal treatments and conjoint analysis

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)

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

				High	
	All Respondents	Current Intravenous	Current Subcutaneous	Average to high	
Convenience around scheduling	15%	13%	18%	Average	
Dosing frequency	19%	24%	14%	Low	
Where you take the treatment	22%	17%	29%	indicates whether a score of the subgroup is higher or lower compared to the score at total level.	
Number of needle sticks per treatment	20%	24%	15%		
Time to take each treatment	23%	23%	23%		

Colour coding

Utility shares show how appealing each level is, compared to other levels within the attribute. The larger the share of a level, the higher its appeal.

Conjoint analysis

Where you take the treatment

Number of needle sticks per treatment

Note that the utility share of levels of belonging to different attributes cannot be compared. Each block adds up to 100% (as they are shares).

IV. General Health

EQ-5d, SF12-v2, SF10

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EQ-5d– Weighted Summary Index

Patient health – EQ-5d (© 1990 EuroQol Group. EQ-5D™ is a trade mark of the EuroQol Group) NOTE: PATIENTS ONLY

Weighted Summary Index (using UK preference weights, TTO value set)

EQ-5d

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 100 Better health 90 80 SF-12 ® 70 Health Survey 60 **US norm** 46,5 50 46.0 45,2 50 44,3 44,2 42,4 41,7 41,1 40,7 37,9 40 30 20 10 Worse health 0 Role-Physical Bodily Pain (BP) General Health Physical Mental Physical Vitality (VT) Social Role-Emotional Mental Health Functioning (RP) Functioning (SF) (RE) Component Component (GH) (MH) Summary (PCS) Summary (MCS) (PF)

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)

Living with PID has a substantial impact on the physical wellbeing of the children. Psychosocially they are less affected, with their score being only slightly below the US norm.

100

90

G1: In general, would you say your child's health is... G2: During the past 4 weeks, has your child been limited in any of the following activities due to HEALTH problems? Bending, lifting or stooping / Doing things that take some energy such as riding a bike or skating G3: During the past 4 weeks, has your child been limited in the KIND of schoolwork or activities with friends he/she could do because of PHYSICAL health problems? G4: During the past 4 weeks, has your child been limited in the KIND of schoolwork or activities with friends he/she could do because of EMOTIONAL or BEHAVIOURAL problems? G5: During the past 4 weeks, how much bodily pain or discomfort has your child had? G6: During the past 4 weeks, how satisfied do you think your child has felt about his/her friendships? G7: During the past 4 weeks, how satisfied do you think your child behaved as if he/she was bothered or upset? G9: Compared to other children your child's age, in general would you say his/her behaviour is? Base: All Respondents who are carers of PID patients who are <20 (76)

SF-10 for

V. Information Sources

28% of people have used the IPOPI website to source information about PID over the past 12 months, with 69% of them finding it "very useful". 35% expect to use it in the next 12 months

% of users who

Sources of information regarding PID

D24a: Which of the following sources, if any, have you used to find out information about PID in the past 12 months, and which do you expect to use over the next coming months? Base: All Respondents (300) D24b: You indicated that you have used the following sources to find information on PID over the past 12 months. How useful were they? Base: Those who have used the source to find information about PID in the past 12 months

The most widely used NMO services used by members are therapy information and health / social support

NMO services provided and used by Members

17% of the patients are happy with the support provided by their national patient organisation as it currently is, but suggestions to improve support were made as shown below

What can the NMO do to better serve the needs of the PID community? (spontaneous answers)

H9 How could <NMO> better service the needs of the PID community in ...? MULTIPLE RESPONSE Base: All Respondents (300)

VI. Sample profile

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

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) Almost half (46%) of patients experienced a significant delay between their initial suspicion of PID and a formal diagnosis

Delay in official PID diagnosis

C2b: To the best of your knowledge, was there a delay between the initial suspicion of PID and its formal diagnosis? Base: All Respondents (300)

The age profile of intravenous respondents is in line with those on subcutaneous treatment for each age bracket, except for the 51-60 year-olds

Age at diagnosis

Q.C0a/Q.C1 How old are you? C2: How old were you when first diagnosed with PID? Base: All Respondents (300)

Summary of select findings – view of current treatments

IV and SubC treatments are roughly split 50/50 across the surveyed countries

Immunology Specialist physicians are the decision makers choosing administration route in 2 in 5 cases with patients and or caregivers being secondary influencers.

Impact of treatment

Sixty-six percent of patients (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. While 39% report no pain, 50% report moderate pain; 48% report problems with daily activities. Patients' mental well-being is affected too: 61% report no anxiety/depression, while 39% identify moderate or extreme anxiety/depression (averages were below population norms across physical and mental QoL elements)

One in five IV patients have tried SubC whilst 8 in 10 SubC patients have tried IV IV patients who had tried SubC were most likely to have stopped because of side effects (61%) and wanting longer between doses (32%). SubC patients who had previously tried IV most commonly stopped because of inconvenience of going to the centre (51%), wanting to treat at home (43%) or because it was recommended they change (48%). 33% said side effects

Side effects in IV and SC patients

95% of SubC experience swelling / bumps at infusion site but this causes a large impact for just 7%. 7% experience pain at infusion sites but to a large extent for just 5%. Headaches are most prominent for IV patients (51% experience) and these have a large impact on life for 11%

Summary of select findings – unmet needs and drivers of choice

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

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

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.