IPOPI PID Patient Needs & Outlooks Survey

A Report based on 300 patient questionnaires
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</table>
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)
- **Duration:** 30 minutes
- **Fieldwork:** 8 April – 17 October 2011
- **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.
  
  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?”)
  
- **Language:** Respondents were able to answer the survey in English, French, German, Italian, Spanish or Portuguese
- **Countries:** Shown in table on right

The research study was sponsored by Baxter and carried out by an independent market 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.

<table>
<thead>
<tr>
<th>Country</th>
<th>Groups Interviews</th>
</tr>
</thead>
<tbody>
<tr>
<td>UK</td>
<td>A, B</td>
</tr>
<tr>
<td>Sweden</td>
<td>A</td>
</tr>
<tr>
<td>Canada</td>
<td>C</td>
</tr>
<tr>
<td>France</td>
<td>A, B</td>
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<tr>
<td>Germany</td>
<td>A, B</td>
</tr>
<tr>
<td>Spain</td>
<td>A, B</td>
</tr>
<tr>
<td>Portugal</td>
<td>A</td>
</tr>
<tr>
<td>Argentina</td>
<td>C</td>
</tr>
<tr>
<td>Brazil</td>
<td>C</td>
</tr>
<tr>
<td>South Africa</td>
<td></td>
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<tr>
<td>Colombia</td>
<td>C</td>
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<tr>
<td>Italy</td>
<td>A, B</td>
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<tr>
<td>Switzerland</td>
<td>A</td>
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<tr>
<td>Belgium</td>
<td>A</td>
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<tr>
<td>New Zealand</td>
<td></td>
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<tr>
<td>Poland</td>
<td>A</td>
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<tr>
<td>Australia</td>
<td></td>
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<tr>
<td>Austria</td>
<td>A</td>
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<tr>
<td>Hungary</td>
<td>A</td>
</tr>
<tr>
<td>India</td>
<td></td>
</tr>
<tr>
<td>Netherlands</td>
<td>A</td>
</tr>
</tbody>
</table>

Groups: A (“Europe”), B (“EU5”), C (“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

<table>
<thead>
<tr>
<th>Region</th>
<th>Intravenous</th>
<th>Subcutaneous</th>
<th>Other</th>
</tr>
</thead>
<tbody>
<tr>
<td>All respondents</td>
<td>53%</td>
<td>45%</td>
<td></td>
</tr>
<tr>
<td>Europe</td>
<td>45%</td>
<td>53%</td>
<td></td>
</tr>
<tr>
<td>UK</td>
<td>68%</td>
<td>32%</td>
<td></td>
</tr>
<tr>
<td>Sweden</td>
<td>15%</td>
<td>82%</td>
<td>3%</td>
</tr>
<tr>
<td>France</td>
<td>32%</td>
<td>61%</td>
<td>6%</td>
</tr>
<tr>
<td>Germany</td>
<td>19%</td>
<td>77%</td>
<td>3%</td>
</tr>
<tr>
<td>Spain*</td>
<td>77%</td>
<td>23%</td>
<td></td>
</tr>
<tr>
<td>Portugal*</td>
<td>43%</td>
<td>57%</td>
<td></td>
</tr>
<tr>
<td>Americas</td>
<td>69%</td>
<td>28%</td>
<td>3%</td>
</tr>
<tr>
<td>Canada</td>
<td>45%</td>
<td>52%</td>
<td>3%</td>
</tr>
<tr>
<td>South America</td>
<td>89%</td>
<td>8%</td>
<td>3%</td>
</tr>
</tbody>
</table>

**Usage of intravenous administration varies by countries in Europe. Intravenous administration in the UK is significantly higher than Europe average.**

**Level of subcutaneous administration is significantly above average in Sweden and Germany.**

**In the Americas, overall intravenous administration used significantly more than in Europe, mainly driven by South American respondents.**

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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**

- **Specialist Immunology Doctor**: 42% Single MAIN person deciding, 51% Involved / Influential, 7% Not involved, 7% Don't know.
- **Caregiver**: 4% Single MAIN person deciding, 73% Involved / Influential, 23% Not involved, 7% Don't know.
- **The Patient**: 13% Single MAIN person deciding, 56% Involved / Influential, 29% Not involved, 7% Don't know.
- **Family / Partner**: 50% Single MAIN person deciding, 48% Involved / Influential, 48% Not involved, 4% Don't know.
- **Nurse**: 34% Single MAIN person deciding, 60% Involved / Influential, 5% Not involved, 5% Don't know.
- **Paediatric Doctor**: 17% Single MAIN person deciding, 75% Involved / Influential, 6% Not involved, 6% Don't know.
- **Pharmacist**: 4% Single MAIN person deciding, 92% Involved / Influential, 4% Not involved, 4% Don't know.

DS5a. 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%).

Where do infusions take place?

- **Regional general hospital**: 3% (Subcutaneous) / 35% (Intravenous)
- **Local general hospital**: 1% (Subcutaneous) / 29% (Intravenous)
- **Specialist clinic**: 1% (Subcutaneous) / 11% (Intravenous)
- **Doctor's surgery/ health clinic**: 3% (Subcutaneous) / 14% (Intravenous)
- **At home**: 94% (Subcutaneous) / 14% (Intravenous)
- **Other**: 1% (Subcutaneous) / 7% (Intravenous)

In the UK, 40% of intravenous patients are infused at home, significantly more than intravenous patients on average.

D9b: Is the product delivered, or does it have to be collected? Base: Respondents who receive treatment at home or other place
- **Subcutaneous**: 42% (Delivered) / 20% (Collected from pharmacy)
- **Intravenous**: 35% (Delivered) / 11% (Collected from pharmacy) / 23% (Collected from hospital) / 69% (Delivered)

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

Get to where the immunoglobulin therapy takes place

- 19% take 1 - 15 minutes
- 31% take 16 - 30 minutes
- 20% take 31 - 60 minutes
- 22% take 61 - 180 minutes
- 7% take over 181 minutes

Average time: 1 hour 6 min

Pre and post infusion time (all the other time spent at the centre but not actually receiving product)

- 10% take less than 11 mins
- 9% take 11 - 15 mins
- 21% take 16 - 30 mins
- 21% take 31 - 60 mins
- 39% take over 60 mins

Average time: 1.5 hours

Carry out the infusion part of the process

- 3% take less than 31 mins
- 16% take 31 - 60 mins
- 24% take 61 - 120 mins
- 53% take over 121 mins

Average time: 3 hours 46 mins

Overall time for IV treatment: 6 hours 21 mins

D10 Approximately, how long does it typically take you to get to the ... where your current immunoglobulin therapy takes place?
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)
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...

**Setting up the infusion equipment (inc. preparation of the injection site)**

- Less than 6 minutes: 16%
- 6 - 10: 25%
- 11 - 15: 22%
- 16 - 30: 24%
- 31+: 13%
  
  Average time: 19 min

**Carry out the infusion part of the process**

- Less than 31 mins: 10%
- 31-60: 34%
- 61-120: 44%
- 121-180: 7%
- 181 mins+: 4%

  Average time: 1.5 hours

**Overall time for SubC treatment:**

- 1 hour 41 mins

D11 How long does it typically take for
.. Setting up the infusion equipment (to get everything done to the point of injection including preparation of the injection site)?
...Carry out the infusion part of the process?
Base: Subcutaneous (134)
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.

**Has this changed over time?**

Number of infusion sites used...

<table>
<thead>
<tr>
<th></th>
<th>Decreased</th>
<th>Not changed</th>
<th>Increased</th>
</tr>
</thead>
<tbody>
<tr>
<td>Intravenous</td>
<td>5%</td>
<td>78%</td>
<td>17%</td>
</tr>
<tr>
<td>Subcutaneous</td>
<td>9%</td>
<td>72%</td>
<td>19%</td>
</tr>
</tbody>
</table>

D12a How many infusion sites do you use at a time?  
D12b Has this changed over time?  
Base: Intravenous (160), Subcutaneous (134)
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)**

- **All occasions**: 26%
- **Most occasions**: 53%
- **Some occasions**: 13%
- **Few occasions**: 4%
- **Very few occasions**: 4%

**How many tries does it typically take to find a vein?**

- **2**: 51%
- **3**: 23%
- **4**: 17%
- **5+**: 9%

**Average**: 3

D14. Which of the following statements best described the ease of finding a vein when administering immunoglobulin therapy? 'I/my care giver can usually find a vein the first time.'

D14b. Approximately how many tries does it typically take to find a 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)**

- **Peristaltic/roller pump**: 14%
- **20ml driver**: 39%
- **30ml driver**: 2%
- **40ml driver**: 3%
- **50ml driver**: 13%
- **60ml driver**: 6%
- **Other size/Unsure**: 8%
- **Hand push/ don't use pump**: 7%
- **Don't know**: 8%

**Syringe driver**

- **63%**

**How many syringe drivers do you use each time to administer immunoglobulin?**

- **1 syringe driver**: 58%
- **2 syringe drivers**: 34%
- **3 syringe drivers**: 7%
- **4 syringe drivers**: 7%

Average: 1.5

Base: Subcutaneous therapy using a syringe driver (95)

**D16:** Do you use a peristaltic pump or a syringe driver?

**D17:** How many ...s' do you use each time you administer immunoglobulin?

Base: Subcutaneous (134)
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.

As most intravenous patients need to visit a hospital to get their treatment, a significantly higher share of them spends time with sleeping, talking to nurses and other patients.
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

<table>
<thead>
<tr>
<th>Waiting time at hospital/clinic (IV only)</th>
<th>Dissatisfied</th>
<th>Satisfied</th>
</tr>
</thead>
<tbody>
<tr>
<td>3% Complete dissatisfied</td>
<td>11% Quite dissatisfied</td>
<td>25% Neither/nor</td>
</tr>
<tr>
<td>14%</td>
<td>62%</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Time to get to therapy</th>
<th>Dissatisfied</th>
<th>Satisfied</th>
</tr>
</thead>
<tbody>
<tr>
<td>3% Complete dissatisfied</td>
<td>3% Quite dissatisfied</td>
<td>18% Neither/nor</td>
</tr>
<tr>
<td>6%</td>
<td>77%</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Time taken to set up infusion (SC only)</th>
<th>Dissatisfied</th>
<th>Satisfied</th>
</tr>
</thead>
<tbody>
<tr>
<td>1% Complete dissatisfied</td>
<td>16% Quite dissatisfied</td>
<td>39% Neither/nor</td>
</tr>
<tr>
<td>3%</td>
<td>80%</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Number of needles per month</th>
<th>Dissatisfied</th>
<th>Satisfied</th>
</tr>
</thead>
<tbody>
<tr>
<td>4% Complete dissatisfied</td>
<td>10% Quite dissatisfied</td>
<td>33% Neither/nor</td>
</tr>
<tr>
<td>14%</td>
<td>53%</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Frequency of sessions</th>
<th>Dissatisfied</th>
<th>Satisfied</th>
</tr>
</thead>
<tbody>
<tr>
<td>8% Complete dissatisfied</td>
<td>8% Quite dissatisfied</td>
<td>28% Neither/nor</td>
</tr>
<tr>
<td>10%</td>
<td>62%</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Device used to perform infusion</th>
<th>Dissatisfied</th>
<th>Satisfied</th>
</tr>
</thead>
<tbody>
<tr>
<td>5% Complete dissatisfied</td>
<td>11% Quite dissatisfied</td>
<td>42% Neither/nor</td>
</tr>
<tr>
<td>6%</td>
<td>83%</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Overall Satisfaction</th>
<th>Dissatisfied</th>
<th>Satisfied</th>
</tr>
</thead>
<tbody>
<tr>
<td>3% Complete dissatisfied</td>
<td>7% Quite dissatisfied</td>
<td>14% Neither/nor</td>
</tr>
<tr>
<td>10%</td>
<td>76%</td>
<td></td>
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</tbody>
</table>

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 Ig treatment

<table>
<thead>
<tr>
<th></th>
<th>Dislike very much</th>
<th>Dislike</th>
<th>No preference</th>
<th>Like</th>
<th>Like very much</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Time administration takes</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Intravenous</td>
<td>10%</td>
<td>26%</td>
<td>26%</td>
<td>27%</td>
<td>12%</td>
</tr>
<tr>
<td>Subcutaneous</td>
<td>18%</td>
<td>16%</td>
<td>39%</td>
<td>26%</td>
<td></td>
</tr>
<tr>
<td><strong>Being able to fit treatment into schedule</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Intravenous</td>
<td>6%</td>
<td>13%</td>
<td>23%</td>
<td>27%</td>
<td>31%</td>
</tr>
<tr>
<td>Subcutaneous</td>
<td>3%</td>
<td>6%</td>
<td>17%</td>
<td>73%</td>
<td></td>
</tr>
<tr>
<td><strong>Ability to self-administer</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Intravenous</td>
<td>18%</td>
<td>13%</td>
<td>39%</td>
<td>12%</td>
<td>19%</td>
</tr>
<tr>
<td>Subcutaneous</td>
<td>3%</td>
<td>6%</td>
<td>17%</td>
<td>74%</td>
<td></td>
</tr>
<tr>
<td><strong>The overall convenience</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Intravenous</td>
<td>4%</td>
<td>14%</td>
<td>22%</td>
<td>35%</td>
<td>25%</td>
</tr>
<tr>
<td>Subcutaneous</td>
<td>4%</td>
<td>7%</td>
<td>33%</td>
<td>55%</td>
<td></td>
</tr>
</tbody>
</table>

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

- **Large Impact**
  - Experiencing side effect

- **Large impact on life/disclosed with doctor/Switching**
- **Large impact on life/Not discussed with doctor**
- **Low impact/ Not seeking to change**

### Swellings/bumps at infusion site
- **Intravenous**
  - Large impact: 3%
  - Medium impact: 8%
  - Low impact: 17%
  - Not experiencing: 72%

- **Subcutaneous**
  - Large impact: 4%
  - Medium impact: 30%
  - Low impact: 58%
  - Not experiencing: 5%

### Pain at infusion site
- **Intravenous**
  - Large impact: 2%
  - Medium impact: 11%
  - Low impact: 28%
  - Not experiencing: 56%

- **Subcutaneous**
  - Large impact: 3%
  - Medium impact: 28%
  - Low impact: 40%
  - Not experiencing: 25%

### Itching at infusion site
- **Intravenous**
  - Large impact: 44%
  - Medium impact: 16%
  - Low impact: 79%
  - Not experiencing: 2%

- **Subcutaneous**
  - Large impact: 4%
  - Medium impact: 15%
  - Low impact: 30%
  - Not experiencing: 46%

### Hardness at infusion site
- **Intravenous**
  - Large impact: 4%
  - Medium impact: 14%
  - Low impact: 77%
  - Not experiencing: 5%

- **Subcutaneous**
  - Large impact: 4%
  - Medium impact: 17%
  - Low impact: 31%
  - Not experiencing: 45%

D19d. Few treatments are perfect, and so considering the list of possible treatment-related side effects, please indicate how much your life is currently impacted by each, on your current medication

**Base:** Intravenous (160), Subcutaneous (134)
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

- **Intravenous**
  - Headaches: 8% (Large impact), 15% (Switching), 24% (Not discussed with doctor), 49% (Large impact and discussed with doctor).
  - Other side effects: 4% (Large impact), 9% (Switching), 14% (Not discussed with doctor), 55% (Large impact and discussed with doctor).

- **Subcutaneous**
  - Headaches: 3% (Large impact), 9% (Switching), 14% (Not discussed with doctor), 69% (Large impact and discussed with doctor).
  - Other side effects: 7% (Large impact), 5% (Switching), 6% (Not discussed with doctor), 67% (Large impact and discussed with doctor).

D19d. Few treatments are perfect, and so considering the list of possible treatment-related side effects, please indicate how much your life is currently impacted by each, on your current medication.

**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

<table>
<thead>
<tr>
<th>Aspect</th>
<th>Important (4-5)</th>
<th>Unimportant (0-1)</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pain at infusion site</td>
<td>23%</td>
<td>56%</td>
<td>51%</td>
</tr>
<tr>
<td>Headaches</td>
<td>34%</td>
<td>45%</td>
<td>79%</td>
</tr>
<tr>
<td>Swelling/bumps at infusion site</td>
<td>18%</td>
<td>63%</td>
<td>81%</td>
</tr>
<tr>
<td>Itching at infusion site</td>
<td>16%</td>
<td>66%</td>
<td>82%</td>
</tr>
</tbody>
</table>

- **Highly important (5)**: Pain at infusion site - 16%, Headaches - 27%, Swelling/bumps at infusion site - 11%, Itching at infusion site - 11%
- **Highly unimportant (0)**: Pain at infusion site - 46%, Headaches - 36%, Swelling/bumps at infusion site - 51%, Itching at infusion site - 59%

- **4**: Pain at infusion site - 12%, Headaches - 8%, Swelling/bumps at infusion site - 8%, Itching at infusion site - 9%
- **3**: Pain at infusion site - 9%, Headaches - 11%, Swelling/bumps at infusion site - 13%, Itching at infusion site - 8%
- **2**: Pain at infusion site - 9%, Headaches - 9%, Swelling/bumps at infusion site - 51%, Itching at infusion site - 59%
- **1**: Pain at infusion site - 16%, Headaches - 34%, Swelling/bumps at infusion site - 28%, Itching at infusion site - 21%

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**

<table>
<thead>
<tr>
<th>Aspect</th>
<th>Important (4-5)</th>
<th>Unimportant (0-1)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pain at infusion site</td>
<td>35%</td>
<td>35%</td>
</tr>
<tr>
<td>Headaches</td>
<td>25%</td>
<td>65%</td>
</tr>
<tr>
<td>Swelling/bumps at infusion site</td>
<td>31%</td>
<td>33%</td>
</tr>
<tr>
<td>Itching at infusion site</td>
<td>23%</td>
<td>51%</td>
</tr>
</tbody>
</table>

- Highly important (5): 16% Pain, 19% Headaches, 13% Swelling, 12% Itching
- Important (4): 19% Pain, 19% Headaches, 18% Swelling, 11% Itching
- Unimportant (3): 15% Pain, 16% Headaches, 19% Swelling, 16% Itching
- Highly unimportant (0): 19% Pain, 13% Headaches, 19% Swelling, 36% Itching

75% experience for Pain at infusion site, 31% experience for Headaches, 95% experience for Swelling/bumps at infusion site, 54% experience for Itching at infusion site.

**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.

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

In the UK and Canada a significantly higher share of patients (97% and 94% respectively) visit an immunology specialists in relation to PID.
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

<table>
<thead>
<tr>
<th>Number of times</th>
<th>Subcutaneous</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>55%</td>
</tr>
<tr>
<td>1</td>
<td>16%</td>
</tr>
<tr>
<td>2</td>
<td>11%</td>
</tr>
<tr>
<td>3</td>
<td>8%</td>
</tr>
<tr>
<td>4</td>
<td>1%</td>
</tr>
<tr>
<td>5</td>
<td>8%</td>
</tr>
<tr>
<td>3+</td>
<td>8%</td>
</tr>
</tbody>
</table>

Skipped a dose
Number of times in the last 6 months

<table>
<thead>
<tr>
<th>Number of times</th>
<th>Intravenous</th>
<th>Subcutaneous</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>92%</td>
<td>82%</td>
</tr>
<tr>
<td>1</td>
<td>5%</td>
<td>10%</td>
</tr>
<tr>
<td>2</td>
<td>3%</td>
<td>4%</td>
</tr>
<tr>
<td>3+</td>
<td>1%</td>
<td>4%</td>
</tr>
</tbody>
</table>

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

- **Regional general hospital**: Ave: 1.2
- **Local general hospital**: Ave: 1.0
- **Specialist clinic**: Ave: 1.3
- **Doctor’s surgery/health centre**: Ave: 3.6

There was no difference in the number of unscheduled visits based on the type of administration (IV vs. SubC).

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

- **Less than 11**: Intravenous 35%, Subcutaneous 31%
- **11 - 15**: Intravenous 8%, Subcutaneous 11%
- **16 - 30**: Intravenous 3%, Subcutaneous 9%
- **31 - 60**: Intravenous 5%, Subcutaneous 8%
- **61+**: Intravenous 13%, Subcutaneous 8%

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)

<table>
<thead>
<tr>
<th>Activity</th>
<th>Intravenous</th>
<th>Subcutaneous</th>
</tr>
</thead>
<tbody>
<tr>
<td>Travel abroad</td>
<td>16%</td>
<td>19%</td>
</tr>
<tr>
<td>More energy/not be tired all the time</td>
<td>14%</td>
<td>20%</td>
</tr>
<tr>
<td>Be able to exercise more</td>
<td>12%</td>
<td>14%</td>
</tr>
<tr>
<td>Relax, not anxious about infection</td>
<td>6%</td>
<td>10%</td>
</tr>
<tr>
<td>Get back to work</td>
<td>8%</td>
<td>6%</td>
</tr>
<tr>
<td>Extended holidays</td>
<td>6%</td>
<td>7%</td>
</tr>
<tr>
<td>Lead normal life/not take medication</td>
<td>4%</td>
<td>10%</td>
</tr>
<tr>
<td>Go to the swimming pool</td>
<td>6%</td>
<td>6%</td>
</tr>
<tr>
<td>Have a social life</td>
<td>3%</td>
<td>9%</td>
</tr>
<tr>
<td>Go to crowded places</td>
<td>5%</td>
<td>6%</td>
</tr>
<tr>
<td>Feel well/not live with restrictions</td>
<td>4%</td>
<td>7%</td>
</tr>
<tr>
<td>Not have pain/feel sick</td>
<td>4%</td>
<td>2%</td>
</tr>
<tr>
<td>Get back to the life I used to have...</td>
<td>2%</td>
<td>1%</td>
</tr>
<tr>
<td>Be with sick people</td>
<td>1%</td>
<td>4%</td>
</tr>
<tr>
<td>Do the job I want</td>
<td>2%</td>
<td>2%</td>
</tr>
<tr>
<td>Travel by plane</td>
<td>1%</td>
<td>1%</td>
</tr>
<tr>
<td>Eat what I want</td>
<td>1%</td>
<td>3%</td>
</tr>
</tbody>
</table>

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.

H7: Generally speaking, how optimistic is your outlook on life, living with PID in the next 5 years?
Base: All Respondents (300), Intravenous (160), Subcutaneous (134)
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)

- **Fewer/no side effects**: 30% (30% Intravenous, 21% Subcutaneous)
- **Shorter administration time**: 30% (30% Intravenous, 19% Subcutaneous)
- **Therapy with tablets/patches/stop using needles**: 26% (37% Intravenous, 18% Subcutaneous)
- **Ability to administer at home**: 18% (4% Intravenous, 18% Subcutaneous)
- **Longer time between infusions**: 25% (16% Intravenous, 25% Subcutaneous)
- **More efficacy/cures the problem completely**: 12% (7% Intravenous, 9% Subcutaneous)
- **Ease of administration/easy to use**: 10% (10% Intravenous, 9% Subcutaneous)
- **Safe product**: 9% (9% Intravenous, 9% Subcutaneous)
- **Less painful**: 10% (7% Intravenous, 10% Subcutaneous)
- **Preloaded syringes/ product ready to be used**: 9% (2% Intravenous, 9% Subcutaneous)
- **No need to be refrigerated/easy to store/transport**: 10% (2% Intravenous, 10% Subcutaneous)

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’.

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**

<table>
<thead>
<tr>
<th></th>
<th>All Respondents</th>
<th>Current Intravenous</th>
<th>Current Subcutaneous</th>
</tr>
</thead>
<tbody>
<tr>
<td>Convenience around scheduling</td>
<td>15%</td>
<td>13%</td>
<td>18%</td>
</tr>
<tr>
<td>Dosing frequency</td>
<td>19%</td>
<td>24%</td>
<td>14%</td>
</tr>
<tr>
<td>Where you take the treatment</td>
<td>22%</td>
<td>17%</td>
<td><strong>29%</strong></td>
</tr>
<tr>
<td>Number of needle sticks per treatment</td>
<td>20%</td>
<td>24%</td>
<td>15%</td>
</tr>
<tr>
<td>Time to take each treatment</td>
<td>23%</td>
<td>23%</td>
<td>23%</td>
</tr>
</tbody>
</table>

*Colour coding*

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)
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

Convenience around scheduling

- Self-administer: 65%
- Appointment with HC professional: 35%

Dosing frequency

- Once a month: 68%
- Twice a month: 18%
- Weekly: 15%

Where you take the treatment

- Home: 80%
- DRs office / surgery, hospital: 20%

Number of needle sticks per treatment

- 1: 63%
- 2: 27%
- 3: 10%

Time to take each treatment

- 2 hours: 70%
- 4 hours: 24%
- 6 hours: 6%

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).

Base: All Respondents (300)
IV. General Health

EQ-5d, SF12-v2, SF10

(© 1990 EuroQol Group. EQ-5D™ is a trade mark of the EuroQol Group)
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)

EQ5d: Please indicate which statements best describe your/the patient’s health today
Base: All Patients (216)
PID patients are below US norms across Physical and Mental elements

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

Scores for Total Sample

Better health

Worse health

US norm 50

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)
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.

PID’s impact on the health status of children:
- Substantial impact: PHS/PSS < 47
- Some impact: PHS/PSS 47-50
- Little or no impact: PHS/PSS > 50

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 has felt about his/her life overall? G8: During the past 4 weeks, how much of the time 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)
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.

Sources of information regarding PID

- Specialist Immunology Doctor: 79% (83%)
- Nurse: 50% (73%)
- Family and friends: 22% (28%)
- Meetings / conferences / events: 44% (75%)
- Books, journals etc: 30% (51%)
- Newspapers or magazines: 23% (11%)
- Television or radio: 22% (10%)
- IPOPI website: 28% (69%)
- Internet blogs or forums: 34% (32%)
- Social networking sites (e.g. Facebook): 21% (14%)
- Pharmaceutical company websites: 23% (36%)
- Government/ local authority website: 23% (26%)
- NMO website: 51% (77%)
- Other NMO information: 74% (76%)
- Other IPOPI information: 47% (77%)
- None of the above: 5% (71%)

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.

H8: Which of the following does <patient organisation NMO name> provide you (the patient/the carer) in terms of support?

Base: All Respondents (300)
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)

- Happy with the current situation: 17%
- Have more and better information about PID: 12%
- Regional meetings for patients to meet each other: 9%
- Hospital personnel/Doctor more or better trained on PID: 9%
- More advertising: 8%
- Financial support: 7%
- Have more regional offices: 6%
- Better website: 5%
- Keeping patients up to date with everything that affects PID community: 5%
- Pressure official organisations to recognise the disease: 4%
- More support: 4%
- Don’t know: 18%

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**

- **PID patient**: 72%
- **Care-giver**: 28%

**Patient Diagnosis**

- **Common Variable Immunodeficiency (CVID)**: 51%
- **Hypogammaglobulinemia**: 13%
- **X-Linked Agammaglobulinemia (XLA)**: 12%
- **IgG Subclass Deficiency**: 9%
- **Hyper-IgM Syndrome**: 3%
- **Selective IgA Deficiency**: 2%
- **Wiskott-Aldrich Syndrome**: 1%
- **Other**: 8%
- **Don’t know**: 2%

**Gender**

- **All respondents**: Male 48%, Female 52%
- **Intravenous**: Male 48%, Female 52%
- **Suncutaneous**: Male 49%, Female 51%

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.

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.

<table>
<thead>
<tr>
<th>Age of patient</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>1-10 years old</td>
<td>15%</td>
</tr>
<tr>
<td>11-20 years old</td>
<td>17%</td>
</tr>
<tr>
<td>21-30 years old</td>
<td>10%</td>
</tr>
<tr>
<td>31-40 years old</td>
<td>16%</td>
</tr>
<tr>
<td>41-50 years old</td>
<td>14%</td>
</tr>
<tr>
<td>51-60 years old</td>
<td>14%</td>
</tr>
<tr>
<td>61+</td>
<td>12%</td>
</tr>
<tr>
<td>Prefer not to say</td>
<td>2%</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Age at diagnosis</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Less than 1 year</td>
<td>6%</td>
</tr>
<tr>
<td>1 - 10 years</td>
<td>32%</td>
</tr>
<tr>
<td>11 - 20 years</td>
<td>10%</td>
</tr>
<tr>
<td>21 - 30 years</td>
<td>13%</td>
</tr>
<tr>
<td>31 - 40 years</td>
<td>17%</td>
</tr>
<tr>
<td>41 - 50 years</td>
<td>9%</td>
</tr>
<tr>
<td>51 - 60 years</td>
<td>7%</td>
</tr>
<tr>
<td>61+</td>
<td>4%</td>
</tr>
<tr>
<td>Prefer not to say</td>
<td>1%</td>
</tr>
<tr>
<td>No answer</td>
<td>1%</td>
</tr>
</tbody>
</table>

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

<table>
<thead>
<tr>
<th>IV and SubC treatments are roughly split 50/50 across the surveyed countries</th>
</tr>
</thead>
<tbody>
<tr>
<td>140% 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)</td>
</tr>
</tbody>
</table>

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

<table>
<thead>
<tr>
<th>Impact of treatment</th>
</tr>
</thead>
<tbody>
<tr>
<td>One in five IV patients have tried SubC whilst 8 in 10 SubC patients have tried IV</td>
</tr>
<tr>
<td>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</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Side effects in IV and SC patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>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%</td>
</tr>
</tbody>
</table>
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

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.