IDF 2013 Treatment Survey

- Mail-based, pencil & paper survey
  - Over 75 Main questions
- Survey in the field December 2013-March 2014
- IDF mail invitation to 4,000 individuals from IDF’s database identified as either a patient or a parent or a patient with a PI
- Second e-mail invitation sent January 27, 2014 to non-respondents
- Survey closed on Monday March 31, 2014
- 1,608 completed the survey
  - Traditional survey would place the expected survey sampling error at +/- 2.4% for the full sample at the 95% Confidence Level
- Data from patients that are currently using IVIG or SCIG will be presented on the following slides
Q1. Are you a patient with a primary immunodeficiency disease (PI) or parent/caregiver of a child in the household with PI? N=1,425
Base: Respondents that are currently receiving IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q5. What is the specific diagnosis of that person’s immunodeficiency disease? N=1,412
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
No Longer Using Ig Therapy

Q21b. Why is the patient no longer being treated with immunoglobulin? SELECT ALL THAT APPLY
Base: Patients who stopped using Ig therapy

- Immunoglobulin no longer prescribed by the doctor as medically necessary: 51% (IVIG Only), 42% (IVIG and SCIG), 67% (SCIG Only)
- Safety/side effects: 27% (IVIG Only), 42% (IVIG and SCIG), 0% (SCIG Only)
- Lack of insurance coverage/inadequate insurance: 24% (IVIG Only), 11% (IVIG and SCIG), 0% (SCIG Only)
- Too expensive (despite good insurance): 22% (IVIG Only), 21% (IVIG and SCIG), 33% (SCIG Only)

Source: IDF 2013 Treatment Survey
Q19a. Has the patient EVER been treated with intravenous immunoglobulin (IVIG), subcutaneous immunoglobulin (SCIG) therapy or intramuscular (IM) immunoglobulin therapy on a regular basis? SELECT ALL THAT APPLY

Base: Patients who currently use IVIG or SCIG
### Average Years on Ig Therapy by All Ig Therapies Ever Used

#### Currently on IVIG

<table>
<thead>
<tr>
<th>Therapy</th>
<th>N</th>
<th>Average Years</th>
<th>Median Years</th>
</tr>
</thead>
<tbody>
<tr>
<td>IVIG Only</td>
<td>619</td>
<td>12.5</td>
<td>10.0</td>
</tr>
<tr>
<td>IVIG and IM</td>
<td>30</td>
<td>31.9</td>
<td>33.0</td>
</tr>
<tr>
<td>IVIG, SCIG and IM</td>
<td>14</td>
<td>28.1</td>
<td>29.0</td>
</tr>
</tbody>
</table>

#### Currently on SCIG

<table>
<thead>
<tr>
<th>Therapy</th>
<th>N</th>
<th>Average Years</th>
<th>Median Years</th>
</tr>
</thead>
<tbody>
<tr>
<td>SCIG Only</td>
<td>87</td>
<td>4.5</td>
<td>3.0</td>
</tr>
<tr>
<td>IVIG and SCIG</td>
<td>417</td>
<td>10.2</td>
<td>9.0</td>
</tr>
<tr>
<td>SCIG and IM</td>
<td>2</td>
<td>12.0</td>
<td>12.0</td>
</tr>
<tr>
<td>IVIG, SCIG and IM</td>
<td>39</td>
<td>25.2</td>
<td>27.0</td>
</tr>
</tbody>
</table>

#### Currently on IM

<table>
<thead>
<tr>
<th>Therapy</th>
<th>N</th>
<th>Average Years</th>
<th>Median Years</th>
</tr>
</thead>
<tbody>
<tr>
<td>IM Only</td>
<td>1</td>
<td>1.0</td>
<td>1.0</td>
</tr>
<tr>
<td>IVIG and IM</td>
<td>1</td>
<td>11.0</td>
<td>11.0</td>
</tr>
<tr>
<td>SCIG and IM</td>
<td>1</td>
<td>1.0</td>
<td>1.0</td>
</tr>
</tbody>
</table>

Q19a. Has the patient EVER been treated with intravenous immunoglobulin (IVIG), subcutaneous immunoglobulin (SCIG) therapy or intramuscular (IM) immunoglobulin therapy on a regular basis? SELECT ALL THAT APPLY

Q20. How many years, in total, has he/she been treated for immunodeficiency (Ig therapy) on a regular basis?

**Base:** Patients who currently use IVIG, SCIG or IM

Source: IDF 2013 Treatment Survey
Q21a. Is he/she currently being treated with SCIG, IVIG or IM for his/her immunodeficiency disease? N=1,425
Base: Patients who currently use IVIG or SCIG
Q22. What year did you start SCIG? N=622
Base: Patients who currently use SCIG

Year Started Using SCIG Therapy

- 0.1% of patients started between 1980 to 1999.
- 41% of patients started between 2000 to 2009.
- 58% of patients started between 2010 to 2013.

Source: IDF 2013 Treatment Survey
Q23a. How difficult was it learning to administer SCIG? N=636
Base: Patients who currently use SCIG

Source: IDF 2013 Treatment Survey
Difficulty Administering SCIG

Q23a. How difficult is it to administer SCIG? N=634
Base: Patients who currently use SCIG

- Easy: 55%
- Not too difficult: 38%
- Somewhat difficult: 7%
- Very difficult: 0.5%

Source: IDF 2013 Treatment Survey
Q24. When was the last time a health professional, such as a nurse or doctor, observed and evaluated the patient’s SCIG infusion technique? N=629
Base: Patients who currently use SCIG

Source: IDF 2013 Treatment Survey
Q26. Prior to SCIG therapy did the patient receive IVIG therapy? N=636
Base: Patients who currently use SCIG

IVIG Prior to SCIG

- Yes: 79%
- No: 21%
Switched Products

Has the patient switched Ig products? N=1,273
Base: Patients who currently use IVIG, SCIG

- 36% Switched from IVIG to SCIG
- 8% Switched from SCIG to IVIG
- 10% Only Used SCIG
- 47% Only Used IVIG

Source: IDF 2013 Treatment Survey
Why Did the Patient Switch from IVIG to SCIG

Q27. Why did the patient switch from IVIG to SCIG? SELECT ALL THAT APPLY N=495
Base: Patients who received IVIG before using SCIG

- More Convenient: 56%
- Physician Recommended: 43%
- Reaction to IVIG: 41%
- Problems Finding Vein: 35%
- Problem Getting to Infusion Site: 13%
- Insurance Reasons: 9%
- Limited Mobility: 4%
- Patient is an Infant/Child: 4%
- Other: 22%

Source: IDF 2013 Treatment Survey
Q28. Compared to IVIG therapy how well do you feel SCIG therapy controls the patient’s PI? N=489
Base: Patients who received IVIG before using SCIG
Q29a. Has the patient ever been on SCIG therapy? N=762
Base: Patients who currently use IVIG or IM
Q29b. Did the patient experience any of the following infections during the 12 months prior to diagnosis? N=129
Base: Patients who currently use IVIG or IM that also used SCIG

Source: IDF 2013 Treatment Survey
Q30. On average, how often does he/she get their IVIG or SCIG therapy?

Base: Patients who currently use IVIG or SCIG

**IVIG**
- Every two weeks: 9%
- Every three weeks: 30%
- Every four weeks: 56%
- Every five weeks: 1%
- Every six weeks or more: 3%

**SCIG**
- Daily: 2%
- Three times per week: 3%
- Two times per week: 9%
- Every week: 81%
- Every two weeks: 4%
- Every three weeks: 0.2%
- Every four weeks: 1%

* p<.001

Source: IDF 2013 Treatment Survey
Q31. About how many grams of Ig per infusion does he/she normally receive?  
Base: Adult Patients who currently use IVIG or SCIG

**Source:** IDF 2013 Treatment Survey

**Grams of Ig per Infusion for Adult Patients**

<table>
<thead>
<tr>
<th>Gram Range</th>
<th>IVIG</th>
<th>SCIG</th>
</tr>
</thead>
<tbody>
<tr>
<td>1 to 10 grams</td>
<td>27%</td>
<td>58%</td>
</tr>
<tr>
<td>11 to 20 grams</td>
<td>30%</td>
<td>34%</td>
</tr>
<tr>
<td>21 to 30 grams</td>
<td>12%</td>
<td>1%</td>
</tr>
<tr>
<td>31 to 40 grams</td>
<td>4%</td>
<td>4%</td>
</tr>
<tr>
<td>More than 40 grams</td>
<td>4%</td>
<td>3%</td>
</tr>
</tbody>
</table>

**Mean**
- IVIG: 37.0 grams
- SCIG: 12.5 grams

**Median**
- IVIG: 35.0 grams
- SCIG: 10.0 grams

**N**
- IVIG: 612
- SCIG: 296
Q32. Who usually administers the therapy? Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Where is Ig Therapy Administered

Q33. Where does the patient usually receive his/her Ig therapy?  
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q34. How long does the therapy usually take?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Who Determines the Rate of Infusion

Q36. Who determines the rate of infusion? 
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q52. In general would you say the patient’s health is...
Base: Patients who currently use IVIG or SCIG
Other Chronic Conditions

Q10. During the 12 months prior to diagnosis, did he/she have ...? SELECT ALL THAT APPLY
Q53. During the PAST 12 MONTHS, has he/she had...
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q11. Did the patient experience any of the following infections during the 12 months prior to diagnosis?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Infections in the Past 12 Months
IVIG vs. SCIG

Q54. Did he/she experience the following infections during the PAST 12 MONTHS?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q54. Did the patient experience any of the following infections during the 12 months prior to diagnosis?
Base: Patients who currently use IVIG or SCIG and reported at least 1 infection

**Number of Infections in the Past 12 Months**

**IVIG vs. SCIG**

- Sinusitis: IVIG 7.3, SCIG 4.1
- Bronchitis: IVIG 2.4, SCIG 2.2
- Pneumonia: IVIG 1.6, SCIG 1.7
- Ear Infection: IVIG 3.6, SCIG 2.9
- Diarrhea: IVIG 31.0, SCIG 32.3
- Candida: IVIG 8.7, SCIG 7.1
- Skin Infection: IVIG 5.8, SCIG 14.9
- Eye Infection: IVIG 3.4, SCIG 2.4
- Abscess: IVIG 2.2, SCIG 2.1

Source: IDF 2013 Treatment Survey
Q12. During the 12 months prior to diagnosis, how much was he/she limited in work, play or normal physical activity as a result of his/her health?

Q55. During the PAST 12 MONTHS, how much has he/she been limited in work, play or normal physical activity as a result of his/her health?

Base: Patients who currently use IVIG or SCIG

Prior to Diagnosis: 32% No to slight limitation
In the Past 12 Months: 58% No to slight limitation
Hospitalized Overnight

Q13a. Was he/she hospitalized overnight or longer for any reason during the 12 months prior to diagnosis?
Q56a. Has he/she been hospitalized overnight or longer for any reason during the past 12 months?
Base: Patients who currently use IVIG or SCIG

Prior to Diagnosis
- Yes: 46%
- No: 54%
N=1,407

In the Past 12 Months
- Yes: 22%
- No: 78%
N=1,403

Source: IDF 2013 Treatment Survey
### Number of Hospitalizations and Operations

#### Q13b. How many TIMES was he/she hospitalized in the 12 months prior to diagnosis?
- **Base**: Patients who currently use IVIG or SCIG who experienced an overnight hospitalization

<table>
<thead>
<tr>
<th>Times Hospitalized Overnight</th>
<th>N=600</th>
<th>N=307</th>
</tr>
</thead>
<tbody>
<tr>
<td>3.0</td>
<td>2.0</td>
<td>1.0</td>
</tr>
</tbody>
</table>

#### Q13c. How many NIGHTS was he/she hospitalized in the 12 months prior to diagnosis?

<table>
<thead>
<tr>
<th>Nights Hospitalized</th>
<th>N=575</th>
<th>N=303</th>
</tr>
</thead>
<tbody>
<tr>
<td>11.6</td>
<td>6.0</td>
<td>4.0</td>
</tr>
</tbody>
</table>

#### Q13d. How many NIGHTS, if any, was he/she in an INTENSIVE CARE UNIT in the 12 months prior to diagnosis?

<table>
<thead>
<tr>
<th>Nights in Intensive Care Unit</th>
<th>N=128</th>
<th>N=39</th>
</tr>
</thead>
<tbody>
<tr>
<td>6.9</td>
<td>4.5</td>
<td>4.0</td>
</tr>
</tbody>
</table>

#### Q56b. How many TIMES was he/she hospitalized in the past 12 months?

<table>
<thead>
<tr>
<th>Inpatient Operations</th>
<th>N=244</th>
<th>N=155</th>
</tr>
</thead>
<tbody>
<tr>
<td>1.9</td>
<td>1.0</td>
<td>1.0</td>
</tr>
</tbody>
</table>

#### Q56c. How many NIGHTS was he/she hospitalized in the past 12 months?

<table>
<thead>
<tr>
<th>Outpatient Operations</th>
<th>N=407</th>
<th>N=288</th>
</tr>
</thead>
<tbody>
<tr>
<td>2.1</td>
<td>1.6</td>
<td>1.6</td>
</tr>
</tbody>
</table>

#### Q56d. How many NIGHTS, if any, was he/she in an INTENSIVE CARE UNIT in the past 12 months?

| Average | Median
<table>
<thead>
<tr>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>2.0</td>
<td>1.0</td>
</tr>
</tbody>
</table>

Source: IDF 2013 Treatment Survey
Used for More Than One Week…

Q15. In the 12 months prior to diagnosis, approximately how many days did he/she use…….
Q58. Approximately how many days in the past 12 months did he/she use……..
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q16. Not counting hospitalizations, about how many DOCTOR VISITS did the patient make during the 12 months prior to diagnosis?

Q59a. Not counting hospitalizations, about how many doctor visits did the patient make during the past 12 months?

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Missed Days from Work or School

Q17. Not counting hospitalizations, how many days was he/she too sick to work, go to school or perform usual activities in the 12 months prior to diagnosis?
Q60. Not counting hospitalizations, how many days was he/she too sick to work, go to school or perform usual activities in the past 12 months?
Base: Patients who currently use IVIG or SCIG

Note: Patients with PI and Parents/Caregivers who are too disabled to work or retired were excluded
Q18a. Which type of physician is responsible for the treatment and management of the patient’s PI? N=1,413
Base: Patients who currently use IVIG or SCIG
In the Past 12 Months: Number of Times Physician Was Seen
IVIG vs. SCIG

Q18b. How many times over the past 12 months has the patient seen this physician?
Base: Patients who currently use IVIG or SCIG

* p<.001

Source: IDF 2013 Treatment Survey
Antihistamine Usually Prescribed Before Ig Therapy Administered

Q37a. Is he/she given medication before Ig therapy, like an antihistamine, cortico-steroid or anti-inflammatory like Tylenol or Motrin, to make it go easier or faster?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Antihistamine Usually Prescribed Before Ig Therapy Administered
IVIG vs. SCIG

By Mouth

- **IVIG**: 79%
- **SCIG**: 100%

IV

- **IVIG**: 21%
- **SCIG**: 0%

* p<.001

Q37a. Is he/she given medication before Ig therapy, like an antihistamine, cortico-steroid or anti-inflammatory like Tylenol or Motrin, to make it go easier or faster? SELECT ALL THAT APPLY
Base: Patients who currently use IVIG or SCIG that are usually prescribed antihistamines

Source: IDF 2013 Treatment Survey
Q37a. Is he/she given medication before Ig therapy, like an antihistamine, cortico-steroid or anti-inflammatory like Tylenol or Motrin, to make it go easier or faster?

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Antihistamine Sometimes Prescribed Before Ig Therapy Administered
IVIG vs. SCIG

Q37a. Is he/she given medication before Ig therapy, like an antihistamine, cortico-steroid or anti-inflammatory like Tylenol or Motrin, to make it go easier or faster? SELECT ALL THAT APPLY
Base: Patients who currently use IVIG or SCIG that are sometimes prescribed antihistamines

* p<.001
Steroid Usually Prescribed Before Ig Therapy Administered

Q37a. Is he/she given medication before Ig therapy, like an antihistamine, cortico-steroid or anti-inflammatory like Tylenol or Motrin, to make it go easier or faster? Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Steroid Usually Prescribed Before Ig Therapy Administered: IVIG vs. SCIG

Q37a. Is he/she given medication before Ig therapy, like an antihistamine, cortico-steroid or anti-inflammatory like Tylenol or Motrin, to make it go easier or faster? SELECT ALL THAT APPLY.
Base: Patients who currently use IVIG or SCIG that are usually prescribed steroids.

* p<.001

Source: IDF 2013 Treatment Survey

By Mouth

- IVIG: 39%
- SCIG: 96%

IV

- IVIG: 61%
- SCIG: 4%
Q37a. Is he/she given medication before Ig therapy, like an antihistamine, cortico-steroid or anti-inflammatory like Tylenol or Motrin, to make it go easier or faster?

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Steroid Sometimes Prescribed Before Ig Therapy Administered
IVIG vs. SCIG

Q37a. Is he/she given medication before Ig therapy, like an antihistamine, cortico-steroid or anti-inflammatory like Tylenol or Motrin, to make it go easier or faster? SELECT ALL THAT APPLY
Base: Patients who currently use IVIG or SCIG that are sometimes prescribed steroids

* p<.01

Source: IDF 2013 Treatment Survey
Q38a. Does the patient (parent/caretaker) need to take off from school or work to get their Ig therapy?

Base: Patients who currently use IVIG or SCIG

* p<.001
Q38b. How many days in the past 12 months has the patient (parent/caretaker) needed to take off work or miss school to get their Ig therapy? N=89
Base: Patients who currently use IVIG or SCIG who missed days from school

Note: Patients with PI and Parents/Caregivers who are too disabled to work or retired were excluded

Mean: 10.1
Median: 10.0

- Less than 7 days: 17%
- 7 to 21 days: 12%
- 22 to 60 days: 66%
- None: 4%
Q38b. How many days in the past 12 months has the patient (parent/caretaker) needed to take off work or miss school to get their Ig therapy? N=196
Base: Patients who currently use IVIG or SCIG who missed days from school

Note: Patients with PI and Parents/Caregivers who are too disabled to work or retired were excluded
Q39. During the past 12 months, has he/she experienced any of the following during or after Ig therapy?

Base: Patients who currently use IVIG or SCIG

**IVIG**
- Yes, experienced side effects during, after or both: 87%
- No, did not experience ANY side effects at any time: 13%

N=780

**SCIG**
- Yes, experienced side effects during, after or both: 90%
- No, did not experience ANY side effects at any time: 10%

N=645
# Side Effects of Ig Therapy

<table>
<thead>
<tr>
<th>Side Effect</th>
<th>%</th>
<th>Side Effect</th>
<th>%</th>
</tr>
</thead>
<tbody>
<tr>
<td>Fatigue</td>
<td>71</td>
<td>Increase in blood pressure</td>
<td>17</td>
</tr>
<tr>
<td>Headache</td>
<td>69</td>
<td>Muscle spasms</td>
<td>13</td>
</tr>
<tr>
<td>Muscle aches</td>
<td>39</td>
<td>Abdominal pain</td>
<td>13</td>
</tr>
<tr>
<td>Chills</td>
<td>35</td>
<td>Drop in blood pressure</td>
<td>13</td>
</tr>
<tr>
<td>Weakness</td>
<td>29</td>
<td>Swelling at infusion site</td>
<td>12</td>
</tr>
<tr>
<td>Nausea</td>
<td>28</td>
<td>Wheezing</td>
<td>9</td>
</tr>
<tr>
<td>Migraine headache</td>
<td>28</td>
<td>Hives</td>
<td>8</td>
</tr>
<tr>
<td>Fever</td>
<td>25</td>
<td>Vomiting</td>
<td>8</td>
</tr>
<tr>
<td>Dizziness</td>
<td>21</td>
<td>Blood in tubing</td>
<td>3</td>
</tr>
<tr>
<td>Anxiety</td>
<td>19</td>
<td>Kidney problems</td>
<td>2</td>
</tr>
<tr>
<td>Diarrhea</td>
<td>19</td>
<td>Hepatitis</td>
<td>0.4</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Side Effect</th>
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<th>Side Effect</th>
<th>%</th>
</tr>
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<tbody>
<tr>
<td>Fatigue</td>
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<td>2</td>
</tr>
<tr>
<td>Diarrhea</td>
<td>19</td>
<td>Hepatitis</td>
<td>0.4</td>
</tr>
</tbody>
</table>

Q39. During the past 12 months, has he/she experienced any of the following during or after Ig therapy? 
Base: Patients who currently use IVIG or SCIG who experienced a side effect

Source: IDF 2013 Treatment Survey
Q40a. Has the patient ever had any of the following serious side-effects or reactions from their Ig?

Base: Patients who currently use IVIG or SCIG that experienced a serious side effect or reaction

Source: IDF 2013 Treatment Survey
Q40a. Has the patient ever had any of the following serious side-effects or reactions from their Ig?

PLEASE SELECT ALL THAT APPLY N=205

Base: Patients who currently use IVIG or SCIG that experienced a serious side effect or reaction

Source: IDF 2013 Treatment Survey
Last Time Serious Side Effects or Reactions Occurred

Q40b. When was the most recent time that he/she had a serious side effect or reaction from their Ig?
Base: Patients who currently use IVIG or SCIG who experienced a serious side effect or reaction

Source: IDF 2013 Treatment Survey
Q40c. Did the patient’s side effect or reaction cause him/her to … SELECT ALL THAT APPLY
Base: Patients who currently use IVIG or SCIG IM who experienced a serious side effect or reaction

Source: IDF 2013 Treatment Survey
Q40c. Did the patient’s side effect or reaction cause him/her to ... SELECT ALL THAT APPLY
Base: Patients who currently use IVIG or SCIG IM who experienced a serious side effect or reaction in the past 12 months

In the Past 12 Months
Serious Side Effects or Reactions Caused the Patient to...

- Slow down infusion rate: 62% IVIG, 55% SCIG
- Switch products: 24% IVIG, 35% SCIG
- Prefer a specific Ig product: 5% IVIG, 29% SCIG
- Only receive Ig in doctor’s office: 0% IVIG, 3% SCIG
- Report the event to the FDA: 6% IVIG, 10% SCIG
- Other: 25% IVIG, 32% SCIG
- None of these: 15% IVIG, 20% SCIG

Source: IDF 2013 Treatment Survey
Was Doctor Told the Patient had a Serious Side-Effect or Reaction?

Q40d. Was the doctor told about the patient’s serious side-effect or reaction?
Base: Patients who currently use IVIG or SCIG who experienced a serious side effect or reaction

Source: IDF 2013 Treatment Survey
In the Past 12 Months Was Doctor Told the Patient had a Serious Side-Effect or Reaction?

Q40d. Was the doctor told about the patient’s serious side-effect or reaction?
Base: Patients who currently use IVIG or SCIG who experienced a serious side effect or reaction in the past 12 months

Source: IDF 2013 Treatment Survey
What Did the Doctor Do?

- 55% of respondents said the doctor slowed the infusion rate.
- 37% switched products only.
- 36% gave medicine.
- 16% experienced nothing.
- 8% reduced amounts.
- 5% said it was normal.
- 1% changed from IVIG to SCIG only.
- 1% changed from SCIG to IVIG only.
- 1% switched products and changed from SCIG to IVIG.

Q40e. What did the doctor do? N=76
Base: Patients who currently use IVIG who experienced a serious side effect or reaction.

Source: IDF 2013 Treatment Survey
IVIG - In the Past 12 Months
What Did the Doctor Do?

- Switched products and Changed from SCIG to IVIG
- Changed from SCIG to IVIG ONLY
- Gave medicine
- Nothing
- Slowed infusion rate
- Reduced amounts
- Said it was normal
- Changed from SCIG to IVIG ONLY
- Switched products ONLY

Q40e. What did the doctor do? N=29
Base: Patients who currently use IVIG who experienced a serious side effect or reaction in the past 12 months

Source: IDF 2013 Treatment Survey
What Did the Doctor Do?

Q40e. What did the doctor do? N=102
Base: Patients who currently use SCIG who experienced a serious side effect or reaction
SCIG - In the Past 12 Months
What Did the Doctor Do?

Q40e. What did the doctor do? N=18
Base: Patients who currently use SCIG who experienced a serious side effect or reaction

- Slowed infusion rate: 61%
- Switched products ONLY: 33%
- Gave medicine: 33%
- Changed from IVIG to SCIG ONLY: 22%
- Changed from SCIG to IVIG ONLY: 17%
- Said it was normal: 11%
- Reduced amounts: 11%
- Switched products and Changed from IVIG to SCIG: 6%
- Nothing: 6%

Source: IDF 2013 Treatment Survey
Patient had a Serious Side-Effect or Reaction When...

<table>
<thead>
<tr>
<th>Event</th>
<th>IVIG</th>
<th>SCIG</th>
</tr>
</thead>
<tbody>
<tr>
<td>Trying a new product for the first time</td>
<td>46%</td>
<td>46%</td>
</tr>
<tr>
<td>Using a product with no previous problems</td>
<td>19%</td>
<td>22%</td>
</tr>
<tr>
<td>Switched to a different product used before</td>
<td>16%</td>
<td>13%</td>
</tr>
<tr>
<td>None of these</td>
<td>36%</td>
<td>40%</td>
</tr>
</tbody>
</table>

Source: IDF 2013 Treatment Survey

Q40f. Has the patient ever had a serious side effect or reaction from Ig therapy when... SELECT ALL THAT APPLY
Base: Patients who currently use IVIG or SCIG who experienced a serious side effect or reaction
In the Past 12 Months
Patient had a Serious Side-Effect or Reaction When...

Q40f. Has the patient ever had a serious side effect or reaction from Ig therapy when...
SELECT ALL THAT APPLY

Base: Patients who currently use IVIG or SCIG who experienced a serious side effect or reaction in the past 12 months

Source: IDF 2013 Treatment Survey
Missed Work or School Due to Reaction from Ig Therapy

Q41a. Has the patient (parent/caretaker of a patient) ever missed school or work due to a reaction from their Ig therapy? N=1,381
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q41b. How many days in the past 12 months has the patient (parent/caretaker of a patient) missed school or work due to reactions from Ig therapy? N=127
Base: Patients who currently use IVIG or SCIG who missed days from school

Source: IDF 2013 Treatment Survey
Q41b. How many days in the past 12 months has the patient (parent/caretaker of a patient) missed school or work due to reactions from Ig therapy? N=193
Base: Patients who currently use IVIG or SCIG who missed days from work

Source: IDF 2013 Treatment Survey
How Well Does Immunoglobulin Control the Patient’s Immunodeficiency?  
IVIG vs. SCIG

IVIG: 57% Completely or well controlled  
SCIG: 61% Completely or well controlled

Q42. How well does immunoglobulin control the patient’s immunodeficiency?  
Base: Patients who currently use IVIG or SCIG
Does the Patient Experience Wear Off Effect?

Q43a. Does the patient experience periods of fatigue or low energy between Ig therapy treatments (wear off)?

Source: IDF 2013 Treatment Survey

Base: Patients who currently use IVIG or SCIG

* p<.001
How Long After the Infusion Does the Patient Experience the Wear Off Effect?

Q43b. How long after infusion does he/she feel this wear off?  
Base: Patients who currently use IVIG or SCIG who experience wear off effect

Source: IDF 2013 Treatment Survey
Wear Off Effect by Frequency of Ig Treatment

Q30. On average, how often does he/she get their IVIG or SCIG therapy? Q43b. How long after infusion does he/she feel this wear off?

Base: Patients who currently use IVIG or SCIG who experience wear off effect

Source: IDF 2013 Treatment Survey
The Wear Off Effect Leads to...

IVIG vs. SCIG

Q43c. Does the “wear off” result in any of the following? SELECT ALL THAT APPLY

Base: Current Ig therapy users who experienced wear off effect

Source: IDF 2013 Treatment Survey
Top 5 Wear Off Effects Side Effects by Frequency of IVIG Treatment

Q30. On average, how often does he/she get their IVIG or SCIG therapy?
Q43c. Does the “wear off” result in any of the following? SELECT ALL THAT APPLY
Base: Patients who currently use IVIG who experience wear off effect

Source: IDF 2013 Treatment Survey
**Top 5 Wear Off Effects Side Effects by Frequency of SCIG Treatment**

<table>
<thead>
<tr>
<th>Frequency</th>
<th>Decreased Performance in Usual Activities</th>
<th>Forgoing of a Pleasurable Activity</th>
<th>Forgoing Usual Activity</th>
<th>Infection</th>
<th>Need for Antibiotics</th>
</tr>
</thead>
<tbody>
<tr>
<td>Daily (N=1)</td>
<td>100%</td>
<td>100%</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Three times per week (N=8)</td>
<td>75%</td>
<td>63%</td>
<td>38%</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Two times per week (N=40)</td>
<td>83%</td>
<td>48% 45%</td>
<td>25% 28%</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Every week (N=281)</td>
<td>78%</td>
<td>52%</td>
<td>25% 48%</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Every two weeks (N=15)</td>
<td>87%</td>
<td>67% 67%</td>
<td>53% 47%</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Every three weeks (N=1)</td>
<td>100%</td>
<td>100%</td>
<td>100%</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Every four weeks (N=6)</td>
<td>67%</td>
<td>50% 50%</td>
<td>67% 67%</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Q30. On average, how often does he/she get their IVIG or SCIG therapy?**

**Q43c. Does the “wear off” result in any of the following? SELECT ALL THAT APPLY**

Source: IDF 2013 Treatment Survey
Q44. Does he/she tolerate any immunoglobulin products better than others, or are they all about the same?

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Due to Tolerability Concerns, Patients...

Q45. As a result of concerns about product tolerability has he/she ever ... SELECT ALL THAT APPLY
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Due to Product Effectiveness, Patients...

Q46. As a result of product effectiveness has he/she ever ... SELECT ALL THAT APPLY
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
How Often Does the Patient Get Their Preferred Ig Product?

Q48. How often does he/she get the Ig product they prefer most?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Satisfaction with Current Ig Product
IVIG vs. SCIG

Q49. How satisfied is the patient with the Ig product currently being used?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q50. Who is primarily responsible for the selection of the Ig product that the patient uses?

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
How Much is the Patient Bothered by Convenience of Their Treatment

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
How Much is the Patient Bothered by Severe Side-Effects

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q51A. Thinking about the patient's experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
How Much is the Patient Bothered by Local Site Reactions

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
How Much is the Patient Bothered by Number of Needle Sticks

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.

Source: IDF 2013 Treatment Survey
Base: Patients who currently use IVIG or SCIG
How Much is the Patient Bothered by Time to Infuse

<table>
<thead>
<tr>
<th></th>
<th>Not Bothered at All</th>
<th>Bothered a Little Bit</th>
<th>Moderately Bothered</th>
<th>Bothered Quite a Bit</th>
<th>Extremely Bothered</th>
</tr>
</thead>
<tbody>
<tr>
<td>IVIG</td>
<td>43%</td>
<td>28%</td>
<td>17%</td>
<td>7%</td>
<td>5%</td>
</tr>
<tr>
<td>SCIG</td>
<td>42%</td>
<td>33%</td>
<td>16%</td>
<td>7%</td>
<td>2%</td>
</tr>
</tbody>
</table>

Source: IDF 2013 Treatment Survey

Base: Patients who currently use IVIG or SCIG

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.
How Much is the Patient Bothered by Number of Infusions Each Month

Source: IDF 2013 Treatment Survey

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.
Base: Patients who currently use IVIG or SCIG
How Much is the Patient Bothered by Cost of Infusions

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
How Much is the Patient Bothered by Interrupts Life

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.

Source: IDF 2013 Treatment Survey

Base: Patients who currently use IVIG or SCIG
How Much is the Patient Bothered by Operating Pump or Infusion Device

Source: IDF 2013 Treatment Survey

Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following.

Base: Patients who currently use IVIG or SCIG
Q51A. Thinking about the patient’s experience with their CURRENT Ig therapy, please tell us how much the patient is bothered, if at all, by each of the following. Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
How Much is the Patient Bothered by Ig Therapy Overall

Q51B. Overall, how bothered is the patient when they get Ig Therapy?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q61. In the past 12 months, how long has the patient taken antibiotics to prevent infections (prophylactically)? N=1,349
Base: Patients who currently use IVIG or SCIG
Prophylactic Antibiotic Therapy by Ig Therapy

Q42. How well does immunoglobulin control the patient’s immunodeficiency?
Q61. In the past 12 months, how long has the patient taken antibiotics to prevent infections (prophylactically)?

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q62. In the past 12 months, how long has the patient taken antibiotics for an active infection? N=1,365
Base: Patients who currently use IVIG or SCIG

- Less than 1 month: 46%
- 1 to 6 months: 34%
- Longer than 6 months: 13%
- Did not take any antibiotics: 7%

Source: IDF 2013 Treatment Survey
Q63. What kind of health insurance does the PATIENT currently have? SELECT ALL THAT APPLY

Base: Patients who currently use IVIG or SCIG
Q64. In the past three years, due to health insurance, has the patient ever had a problem in getting his/her regular infusion?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q65. When was the most recent time the patient had a problem getting his/her regular infusion due to health insurance? Base: Patients who currently use IVIG or SCIG
Q66. In the past 12 months, which of the following problems, if any, has the patient experienced due to health insurance?

Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q66. In the past 12 months, which of the following problems, if any, has the patient experienced due to health insurance? SELECT ALL THAT APPLY

Base: Patients who currently use IVIG or SCIG who experienced problems due to health insurance
Q67. How many times in the past 12 months has the patient experienced a problem getting his/her regular infusion?
Base: Patients who currently use IVIG or SCIG

Source: IDF 2013 Treatment Survey

IVIG Mean:  1.7
SCIG Mean:  1.9

N=220  N=187
Q1. Are you a patient with a primary immunodeficiency disease (PI) or parent/caregiver of a child in the household with PI? N=1,425
Base: Respondents that are currently receiving IVIG or SCIG

Source: IDF 2013 Treatment Survey
Q2. What is the date of birth of the (adult patient/oldest child) in the household with a primary immunodeficiency disease?
Base: Respondents that are currently receiving IVIG or SCIG
Q4. At what age (in years) was that person first diagnosed with a primary immunodeficiency disease?

Q6B. At what age (in years) did these repeated, serious, or unusual infections begin? N=1,251

Mean: 15.0
Median: 8.0

Source: IDF 2013 Treatment Survey

Base: Patients that are currently receiving IVIG or SCIG
Prior to Diagnosis: Repeated, Unusual or Serious Infections

Q6a. Did the patient experience repeated, serious, or unusual infections prior to initial diagnosis as immune deficient? N=1,420
Base: Patients that are currently receiving IVIG or SCIG

Source: IDF 2013 Treatment Survey
Prior to Diagnosis: Age of Onset of Repeated, Unusual or Serious Infections
IVIG vs. SCIG

Q6B. At what age (in years) did these repeated, serious, or unusual infections begin?
Base: All patients who are currently taking IVIG or SCIG who experienced repeated, serious, or unusual infections prior to initial diagnosis

**IVIG**
- Mean: 18.5
- Median: 13.0
- N=684

**SCIG**
- Mean: 15.6
- Median: 7.0
- N=573

For IVIG:
- 57% before age 18
- 20% aged 19-34
- 17% aged 35-50
- 5% aged 51-64
- 1% 65 and older

For SCIG:
- 64% before age 18
- 18% aged 19-34
- 14% aged 35-50
- 4% aged 51-64
- 1% 65 and older
Take Aways
Type of Ig Therapy

- 55% of patients currently use IVIG and 45% of patients currently use SCIG.
- Current SCIG users are younger (mean age: 43 years old) than current IVIG users (mean age: 48 years old).
- Patients who currently use IVIG overwhelming (83%) report IVIG as the only Ig therapy they have ever used.
- Conversely, a little more than a quarter of current SCIG users (27%) report only using SCIG for their Ig therapy.
- SCIG users were more likely to have used IVIG and SCIG (66%). Only 12% of current IVIG users used both IVIG and SCIG.

Source: IDF 2013 Treatment Survey
Take Aways
No Longer Using Ig Therapy

- Patients that no longer use any form of Ig therapy were more likely to report their physician terminated their Ig therapy because it was no longer deemed medically necessary.

- This occurred more often with patients who used SCIG only versus patients that used IVIG only or both IVIG and SCIG.
Take Aways
Other Chronic Conditions

- Asthma (55%), Arthritis (50%), Autoimmune Condition (37%), Digestive Disease (34%), and Malabsorption/Diarrhea (29%) were among the top five chronic conditions reported by current Ig users in the past 12 months.

- Chronic conditions such as Arthritis, Autoimmune Condition, Neurological Disease and Cancer all increased - by more than 2 percentage points - post diagnosis for current Ig therapy users while all other chronic conditions decreased.

Source: IDF 2013 Treatment Survey
Take Aways
Infections

- The number of current Ig users reporting infections decreased post diagnosis.
  - For example:
    - Prior to Diagnosis
      - 67% of IVIG users had Bronchitis
      - 71% of SCIG users had Bronchitis
      - 55% of IVIG users had Pneumonia
      - 53% of SCIG users had Pneumonia
    - In the Past 12 months
      - 36% of IVIG users had Bronchitis
      - 38% of SCIG users had Bronchitis
      - 15% of IVIG users had Pneumonia
      - 11% of SCIG users had Pneumonia
  - In the past 12 months, current IVIG users reported more occurrences of Sinusitis, Ear Infections, Candida and Eye Infections than SCIG users. Current SCIG users reported more occurrences of Diarrhea and Skin Infections than IVIG users.

Source: IDF 2013 Treatment Survey
The top five side effects for current IVIG users were fatigue, headache, chills, weakness and nausea.

Most common side effects for current SCIG users were redness at infusion site, swelling at infusion site, fatigue, headache, and blood in tubing.

IVIG users were more likely to report not experiencing any side effects (13%) than SCIG users (10%).

Ig therapy users reported serious side effects such as aseptic meningitis, blurred vision and blood clots.
57% of current IVIG users reported their Ig therapy completely controls or well controls their PI vs. 61% of current SCIG users.

44% of current IVIG users always experience a wear off effect vs. 27% of current SCIG users
- 57% IVIG users report the wear effect lasts for 1 to 3 weeks vs. 90% of SCIG users who report less than 1 week.

Both IVIG (77%) and SCIG (77%) are equally satisfied with the Ig product they currently use.
54% of IVIG users have taken antibiotics along with their Ig therapy to help prevent infections vs. 48% of SCIG users.

When compared, IVIG users report better control of PI with Ig therapy alone (65% completely or well controlled) than IVIG and antibiotics (50%). Similar results were reported for SCIG users, 68% SCIG alone vs. 53% SCIG and antibiotics.