2013 IDF National Immunoglobulin Treatment Survey

Immune Deficiency Foundation

About the Immune Deficiency Foundation and Primary Immunodeficiency Diseases

The Immune Deficiency Foundation (IDF), founded in 1980, is the national non-profit patient organization dedicated to improving the diagnosis, treatment and quality of life of persons with primary immunodeficiency disease (PI) through advocacy, education and research. There are approximately 250,000 people diagnosed with PI in the U.S. [1], thousands more go undetected.

Governed by a Board of Trustees – and supported by a Medical Advisory Committee comprised of some of the world's leading clinical immunologists, as well as hundreds of grassroots volunteers and a compassionate, professional staff – IDF has provided individuals and their families with vital knowledge by:

- Helping the patient and medical community gain a broader understanding of PI through education and outreach efforts;
- Promoting, participating, and funding research that has helped characterize PI and given patients and physicians substantially improved treatment options;
- Addressing patient needs through public policy programs by focusing on issues such as insurance reimbursement, patient confidentiality, ensuring the safety and availability of immunoglobulin therapy, and maintaining and enhancing patient access to treatment options.

Contributors to this Report

The 2013 IDF National Treatment Survey of Patients with Primary Immune Deficiency Diseases was designed and implemented by the staff at the Immune Deficiency Foundation. Principals include Christopher Scalchunes, MPA, Vice President of Research IDF; Mrs. Marcia Boyle, President & Founder IDF; and Tiffany Henderson, PhD, Survey Research Analyst IDF.

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www.primaryimmune.org

Table of Contents

Table of Figures

Background

Primary immunodeficiency diseases (PI) represent a group of more than 300 rare, chronic, genetic, diseases in which there is a defect in the human immune system. The human immune system is a network of many different, interrelated, processes and components that work together to provide defense against infection. To function properly, the immune system must detect and protect against a wide variety of pathogens-viruses, bacteria and fungi. The immune system also must be able to distinguish foreign pathogens from a body's own tissues, which need to be protected. When any component or part of this process is absent or does not function properly the result is a susceptibility to severe, persistent, unusual and recurrent infections[2]. Unfortunately, far too many patients are not diagnosed and treated for their PI prior to the onset of permanent functional impairments caused by these infections[3]. These impairments must be addressed and treated on their own accord in addition to the underlying primary immunodeficiency. There are various treatments for the different types of PI, including immunoglobulin (Ig) replacement therapy, bone marrow transplantation, gene therapy and gamma interferon. This survey and report focuses specifically on those individuals who receive Ig therapy for their PI.

Ig therapy is indicated for those persons who have a lack of and/or impaired antibody function. For these individuals, life-long, consistent Ig therapy is the best way to optimize their health, improve their quality of life and allow them to become productive members of society. Ig therapy is prepared from the plasma collected from a large number of individuals, usually between 10,000 - 50,000 who have been carefully screened to make sure they are healthy and do not harbor certain infectious diseases. The plasma is chemically and mechanically purified in a series of steps and results in the purification of a broad range of specific antibodies to many types of bacteria and viruses.

Ig replacement therapy only partly replaces what a body should be making, and it only provides temporary protection. Most antibodies are used up or "metabolized" by the body and for a person with PI, this means Ig therapy must be continuously replenished at regular intervals and is usually necessary for the patient's entire life. Depending on the route of Ig therapy administration, this may be done by giving small infusions under the skin (subcutaneous immunoglobulin or SCIG) weekly or as often as every one to three days, or by giving larger intravenous immunoglobulin (IVIG) once every three or four weeks.

This study examines in detail and focus, the patient experiences with Ig therapy. Data from this survey helps quantify the impact Ig therapy has on patient health outcomes, the issues and challenges patients may face while on Ig therapy and it helps further define the characteristics of patients who receive Ig therapy for their PI.

The 2013 Immune Deficiency Foundation (IDF) National Treatment Survey is the fourth such survey conducted by IDF. Others in this series were conducted in 1997, 2003 and in 2008. **Table 1** provides the sampling frame information for all four national treatment surveys.

Table 1. *IDF National Treatment Surveys of Patients with Primary Immunodeficiency Diseases*

Year	Sampling Frame	Total Sample	Completed Surveys
1997	▪ All self-identified patients and caregivers of patients in the IDF database identified as using Ig replacement therapy	2,815	908
2003	▪ Current users of Ig replacement therapy as identified in the 1996 and 2002 IDF National Patient Surveys[1]	2,589	1,186
2008	▪ Current users of Ig replacement therapy as identified in the 2007 IDF National Patient Survey ▪ Random selection of patients and caregivers of patients in the IDF database identified as using Ig replacement therapy.	2,500	1,030
2013	▪ Current users of Ig replacement therapy as identified in the 2012 IDF National Patient Survey ▪ Random selection of patients and caregivers of patients in the IDF database identified as using Ig replacement therapy.	4,000	1,608

Survey Methodology

IDF conducted its Third National Survey of the Treatment Experiences and Preferences of Patients with Primary Immunodeficiency Diseases in 2013. A total of 4,000 patients with primary immunodeficiency diseases (PI), who had reported either intravenous immunoglobulin (IVIG) therapy or subcutaneous immunoglobulin (SCIG) therapy, were selected from the IDF database for this survey. These individuals were mailed an 11-page, self-administered questionnaire, which they were asked to complete and return to IDF. Additionally, these individuals were each sent a SF-12v2 Quality of Life Questionnaire and an SF-10 (pediatric) quality of life questionnaire.

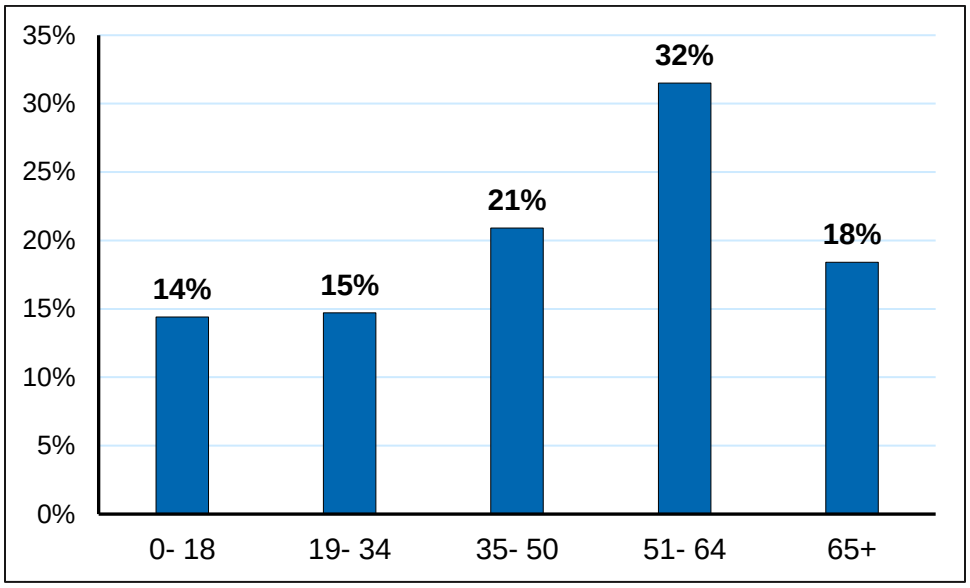
A second mailing was made to those who did not respond to the first mailing. A total of 1,608 respondents returned a completed questionnaire for a 40.2% response rate. After removing cases of respondents with non-primary immunodeficiency disease diagnoses, or those not currently using immunoglobulin (Ig) therapy, the survey yielded a total of 1,437 patients with PI who were currently being treated with either IVIG or SCIG. In addition, 1,277 SF-12v2 questionnaires were completed, as well as 299 SF-10 questionnaires. These cases provide a relatively large national sample of persons with PI, who have been treated with Ig therapy. Although this is not a probability sample from which we can make population estimates within known limits of sampling error, it provides the most representative sample currently available of patients with a rare disease from which we can examine immunoglobulin treatment experiences and patient reported health related quality of life.

IDF conducted National Patient Surveys in 1996, 2002, 2007 and 2012. These surveys are a series of general surveys of all diagnosis, not just those that receive Ig replacement therapy. The sampling frame for these surveys are those who are new to IDF-those who joined IDF since the last patient survey.

Characteristics of Persons with Primary Immunodeficiency

A large proportion of respondents (80%) identified as an adult with primary immunodeficiency diseases (PI). Only 18% were either parent/caregivers of a person with PI and just 3% were both a person with PI and the caregiver/parent of a person with PI. Accordingly, the vast majority of patients represented in the survey are over the age of 18 (**Chart 1**) with and overall average age of 46.1 years for the sample. Over two-thirds (67%) of those with PI in this survey are female.

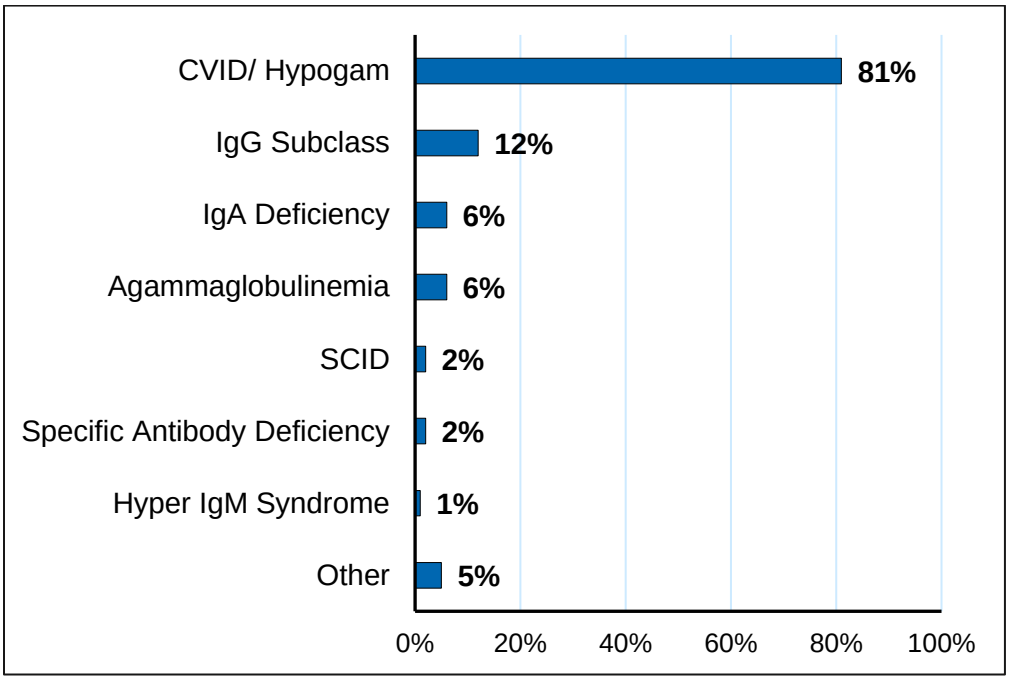
Chart 1. ***Age of Survey Respondents***



Q2. What is the date of birth of the adult patient/oldest child in the household with a primary immunodeficiency disease? Base: All respondents N = 1,437

Similar to the 2002 and 2008 IDF Treatment Surveys, the large majority of respondents to this survey reported their diagnoses as Common Variable Immune Deficiency (CVID) /Hypogammaglobulinemia (**Chart 2**).

Chart 2. *Respondent Diagnoses*

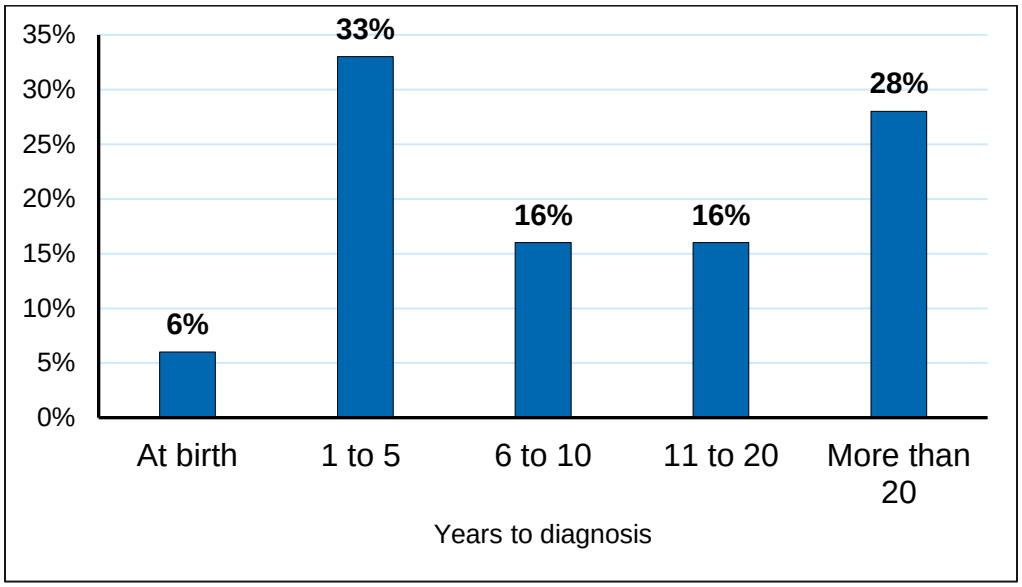


Q5. What is the specific diagnosis of that person's primary immunodeficiency disease? Base: All respondents N = 1,437

Compared to other human immune defects, CVID is a relatively frequent form of PI; this is why it is known as "common." The degree and type of deficiency can and does vary from patient to patient, hence the word "variable." CVID makes up the "single" largest group of PI diagnoses that require immunoglobulin (Ig) replacement therapy. In the majority of cases, a diagnosis of CVID does not occur until the third or fourth decades in life[5]. This being the case, it is not surprising that the average age of patients represented in this survey is 46 years.

Infections are often the hallmark of PI, and 95% of the patients in the survey reported having infections prior to diagnosis. Unfortunately, all too often these infections are missed opportunities for earlier diagnosis. The average time to diagnosis from this infection onset for all diagnoses from this sample is 15 years (median of 8 years) with almost 3 out of 10 (28%) who reported a diagnostic delay of more than 20 years (**Chart 3**). A more accurate depiction of the diagnostic delays experienced by those with PI, however, would be a look at time to diagnosis by specific diagnosis (**Table 2**).

Chart 3. ***Time to Diagnosis from Symptom Onset***



Q4. At what age was that person first diagnosed with a primary immunodeficiency disease? Q6b. At what age (in years) did these repeated, serious or unusual infections begin? Base: All respondents with only 1 PI diagnosis N = 1,243

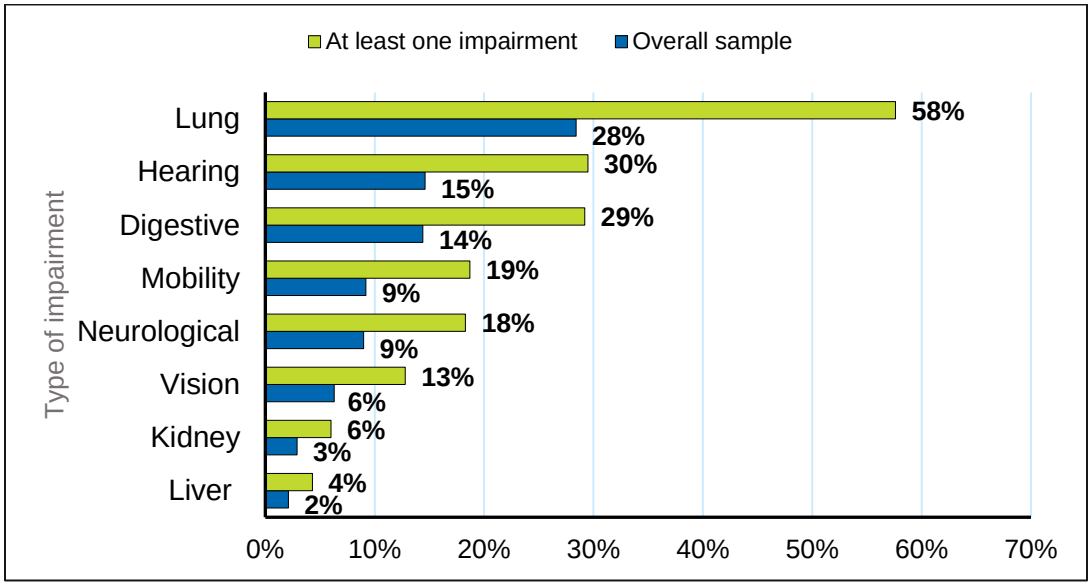
Table 2. ***Symptom Onset to Diagnosis by Diagnosis***

Diagnosis	Years to Diagnosis	
	Average	Median
Specific Antibody	20.3	10.5
CVID	15.8	9.0
IgG Subclass	10.9	8.0
Severe Combined (all)	6.9	0.5
Hyper IgM	2.8	2.0
Agammaglobulinemia	2.1	1.0
Wiskott-Aldrich	2.0	2.0
Other	3.5	2.0

Q4. At what age was that person first diagnosed with a primary immunodeficiency disease? Q5. What is the specific diagnosis of that person's primary immunodeficiency disease? Q6b. At what age (in years) did these repeated, serious or unusual infections begin?
Base: All respondents with only 1 PI diagnosis N = 1,243

Decades ago, these diseases were considered pediatric conditions. Today, we know this is not the case. Data from this survey indicates that the path to diagnosis for an individual with PI can sometimes take decades. These delays in diagnoses are unfortunate as almost half of the persons with PI (49%) reported at least one permanent functional impairment prior to their diagnoses. Most commonly, these occur due to infection[6]. Of those with at least one impairment, the most common cited permanent impairments include losses to the lungs, digestive system, hearing and mobility (**Chart 4**).

Chart 4. ***Permanent Impairments Prior to Diagnosis of Primary Immunodeficiency:***

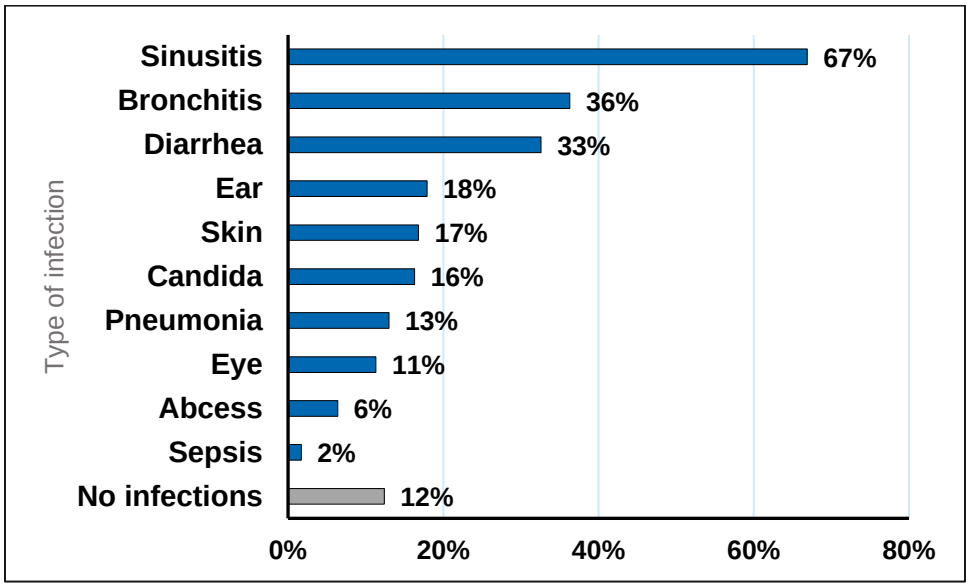


Q8. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of …? SELECT ALL THAT APPLY
Base: All respondents N = 1,394

For those with permanent impairments, 50% reported only one impairment; however, 24% reported two permanent impairments and 26% reported three or more permanent impairments prior to a diagnosis of PI. Unfortunately, these permanent impairments can have a profound, negative, impact on the quality of life for persons afflicted with these issues.

Despite a diagnosis and treatment for PI, 88% of the patients represented in this survey reported at least one type of infection during the most recent 12-month period (**Chart 5**).

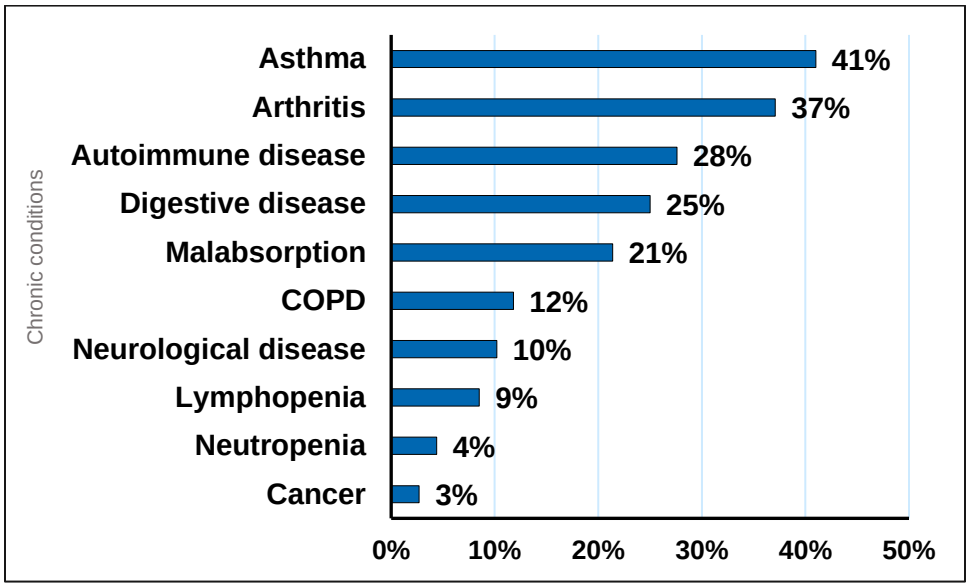
Chart 5. ***Infections Reported in the Past 12 Months***



Q54a-l. Did he/she experience the following infections during the past 12 months…? Base: All respondents N = 1,428

In addition to permanent impairments, 82% of individuals with PI in this survey suffer from a variety of other chronic conditions (**Chart 6**). Many of these conditions are related to their PI and are symptomatic of immune dysregulation[7]. Asthma or asthma-like symptoms, arthritis, digestive disease and other autoimmune conditions occur with great frequency in this community.

Chart 6. ***Chronic Conditions Associated with Primary Immunodeficiency***



Q53. During the past 12 months, has he/she had: SELECT ALL THAT APPLY
Base: All respondents N = 1,428

The individuals represented in this survey exhibit the classical characteristics of diagnosed patients with primary antibody defects (PAD) [8]. Despite managed care and treatment, the challenges and issues that individuals with PI face in their daily lives remain. Life-long impairments, the threat of infections and other chronic conditions are for many, part of what it means to have a PI. Only through continued access to appropriate medical care, including immunoglobulin replacement therapy, can we hope to optimize the health of those with these diseases.

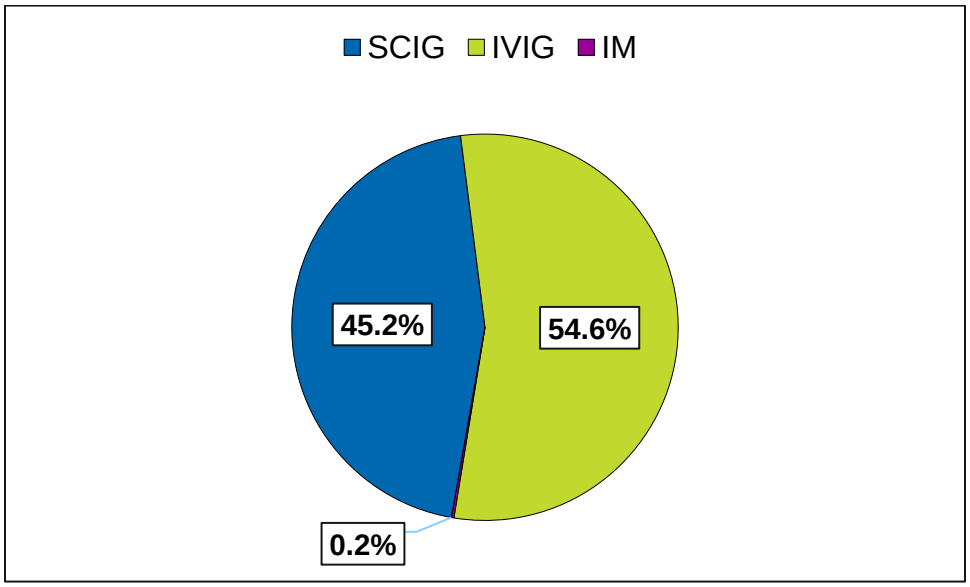
Immunoglobulin Replacement Therapy in PI

Immunoglobulin (Ig) replacement therapy represents a lifesaving intervention for many patients with primary immunodeficiency diseases (PI), specifically those with primary antibody deficiencies. In use for over 60 years, Ig therapy selection, dosing, route of therapy and frequency of dosing must be performed on an individual patient level. Ig therapy must be received on a consistent basis and is a life-long necessity needed to optimize patient health outcomes for those with PAD.

At the time of this survey there were 12 different FDA approved Ig therapy products, available through three different routes of use, intravenous (IVIG), subcutaneous (SCIG) and intramuscular (IM).

Since its introduction to the U.S. in 2006, subcutaneous Ig therapy for PI has continued to gain in use among those with PI. In the 2008 IDF National Treatment Survey, 25% of those on Ig therapy received it subcutaneously. In this survey, we found SCIG usage approaching 50% (**Chart 7**).

Chart 7. *Ig Therapy by Mode of Infusion*

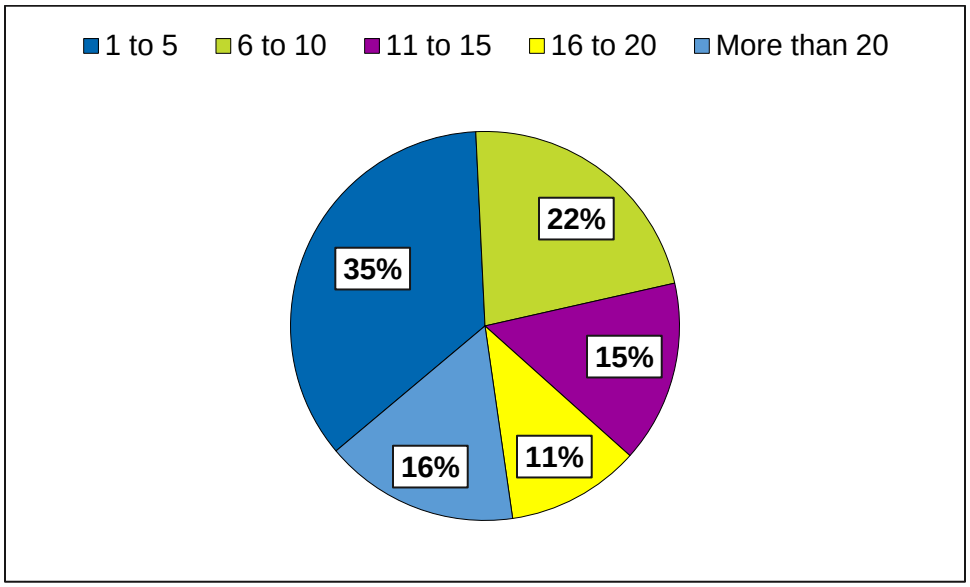


Q21a. Is he/she currently being treated with SCIG, IVIG or IM for his/her immunodeficiency disease? Base: All respondents N = 1,428

Those using SCIG in this survey tended to be a little younger than those using IVIG. SCIG users had an average age of 43.0 (median 47.0) compared to an average age of 48.3 (median 52.0) for those on IVIG.

Sixty-six percent of those currently on SCIG stated they had previously been on IVIG with 27% of the current SCIG users that stated they had only been on SCIG and never on IVIG. Alternatively, 12% of those currently on IVIG had at one time used SCIG but were now back to IVIG. Overall, the sample from this survey had on average, over a decades' worth of experience with Ig therapy (average 11.5 years, median 9.0 years). A substantial minority (16%) had more than 20 years (**Chart 8**).

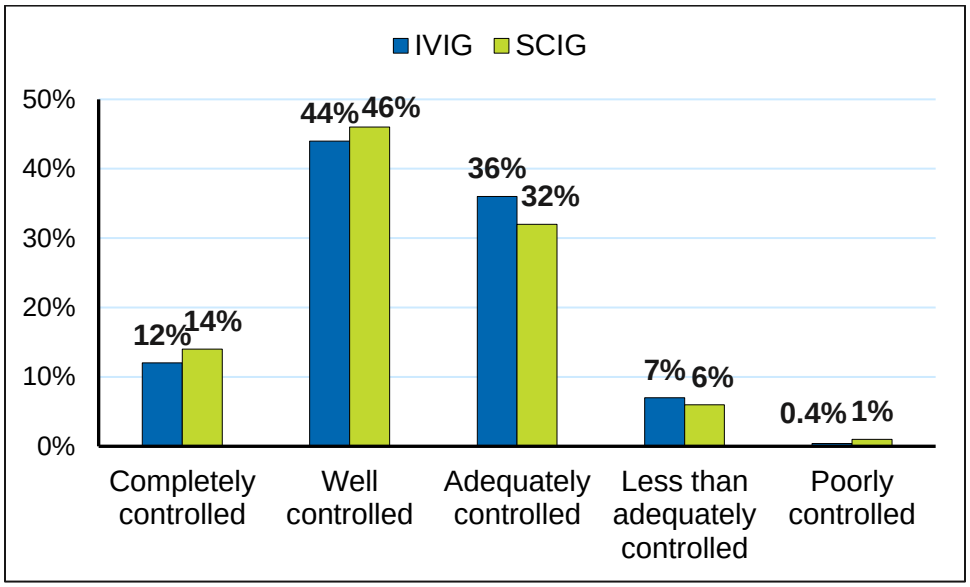
Chart 8. ***Years on Ig Therapy***



Q21a. Is he/she currently being treated with SCIG, IVIG or IM for his/her immunodeficiency disease? Q20. How many years in total has he/she been treated with IVIG, SCIG or IM? Base: All respondents N = 1,381

Most of those on IVIG received their therapy every three (30%) or four (56%) weeks. For those on SCIG the large majority received their IG therapy weekly (81%) or two times per week (9%). Although both IVIG and SCIG are equally effective at preventing serious infections (**Chart 9**), the patient experience with each of these modes of therapy is different enough that a side-by-side comparison of these experiences is useful (**Table 3**).

Chart 9. ***Ig therapy control of PI by mode of therapy***



Q42. How well does immunoglobulin control the patient's immunodeficiency?
Base: All respondents N = 1,381

Table 3. ***Comparison of Patient Experiences: IVIG & SCIG***

Attributes	IVIG	SCIG
Typical infusion schedule	Every 4 weeks	Every week
Average grams infused	37.0	12.5
Average time in minutes for infusion	240.0	126.0
Antihistamine use prior to therapy	54%	32%
Steroid use prior to therapy	21%	4%
Missed work/school for therapy	37%	5%

The patient experience with side effects either during or after the infusion also differs between those who use IVIG and those who use SCIG. We asked respondents if they experienced any side effects in the last 12 months from their Ig therapy, either during or after their infusion. Very few of those on with IVIG (13%) or SCIG (10%) reported no side effects from their Ig therapy over the most recent 12-month period. The most common reported side effects for IVIG tended to be fatigue and headaches. For SCIG the most common side effects were redness and swelling at the infusion site (**Charts 10 & 11**).

Chart 10. ***Top 5 Side Effects of IVIG***

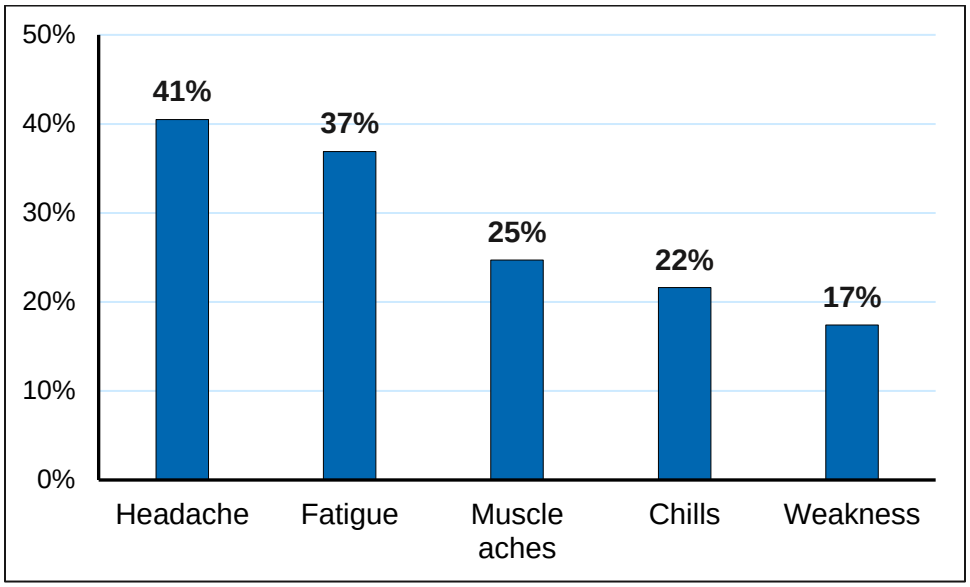
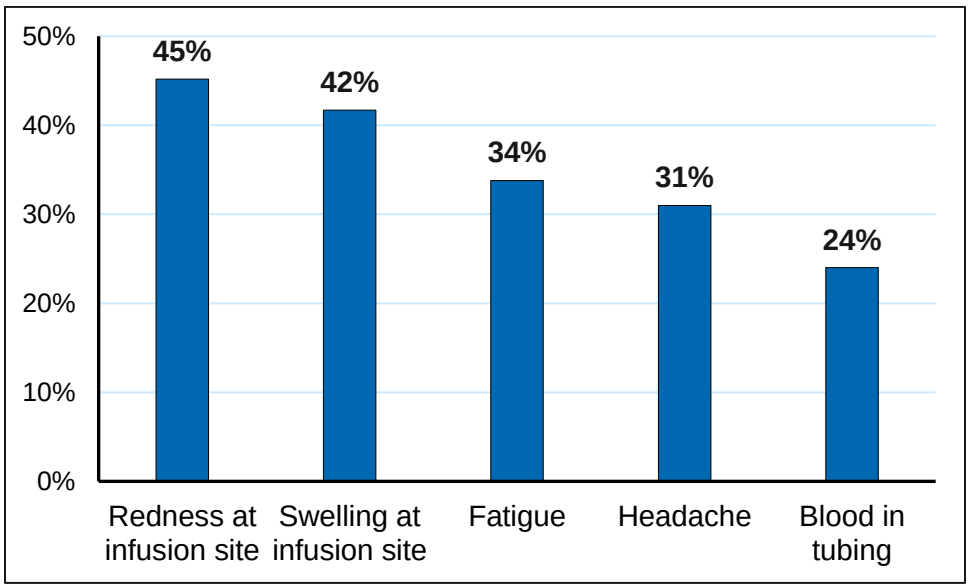


Chart 11. ***Top 5 Side Effects of SCIG***



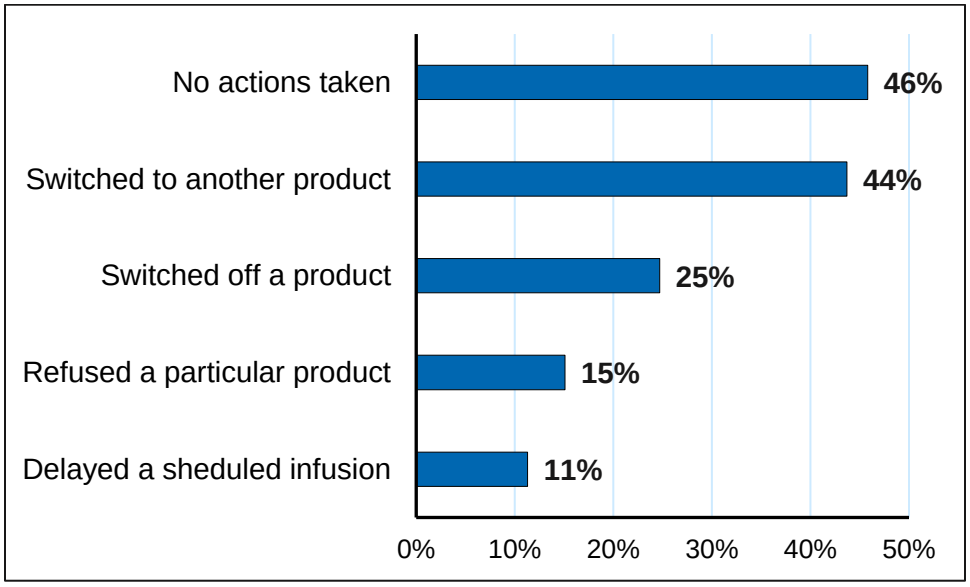
Q39. During the past 12 months, has he/she experienced any of the following during or after Ig therapy? Base: All respondents (IVIG N = 780) (SCIG N = 645)

As discussed earlier, at the time of this survey, there were 12 FDA approved Ig therapy products from 7 different manufacturers, available for PI. Each of these products has their own characteristics in content and in the manufacturing process. There are differences in product stabilizers, viscosity, protein make-up and composition. All of these differences mean these products are not generic equivalents. A substantial number of patients react to and tolerate different products, differently. According to our data, many patients take antihistamines and steroids prior to their Ig therapy to ease the infusion experience. In the instance of antihistamines 62% of those on IVIG and 40% of those on SCIG take an antihistamine to prior to their Ig therapy. Steroid use among those on IVIG is significantly higher when compared to those on SCIG, 39% to 7%.

Although 32% of the patients in this survey had only ever been on one Ig therapy product, 60% of the patients who have been on more than one product tolerate some Ig therapy products better than other products.

About half of those who have experienced tolerability issues with Ig products (54%) took steps in order to avoid those issues.

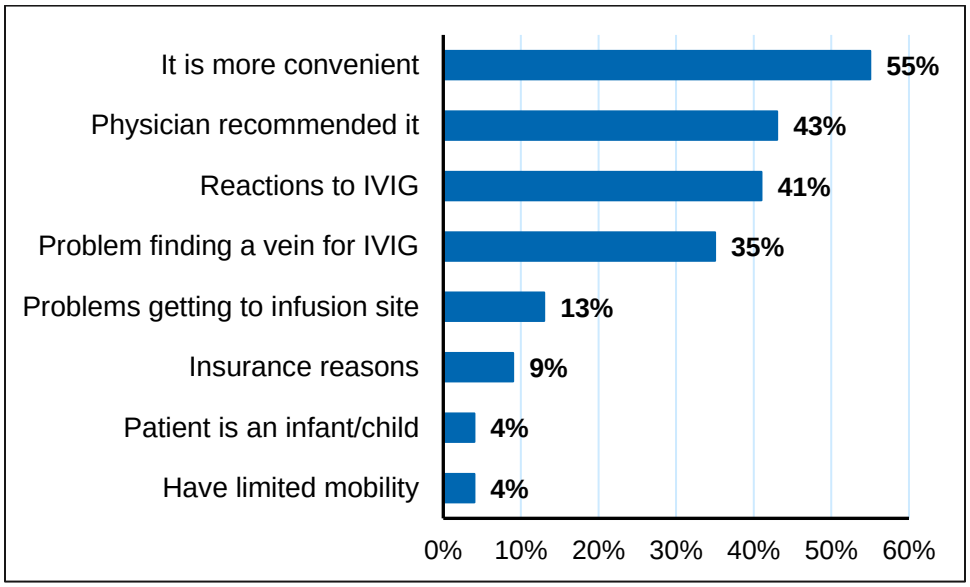
Chart 12. ***Patient Actions Based on Ig therapy Tolerability***



Q45. As a result of concerns about product tolerability has she/he ever? SELECT ALL THAT APPLY Base: Those who have tried more than one Ig therapy product and tolerate some products better than others, N= 572

Maintaining a patient on a well-tolerated Ig product is imperative. Compliance with regular, life-long Ig therapy is necessary to maximize patient health outcomes. There are times, however, when switching may lead to improved patient experience and outcomes. The advent of subcutaneous Ig therapy for PI in 2006 has changed the landscape dramatically. We asked respondents who were currently on SCIG but had switched to SCIG from IVIG why they switched (**Chart 13**). Patients that either suffered through difficult IVIG infusions or found self-infusion in the home more convenient now had an option that for many, improved the Ig therapy infusion experience.

Chart 13. ***Reasons for Switching from IVIG to SCIG***



Q27. Why did the patient switch from IVIG to SCIG? SELECT ALL THAT APPLY
Base: Prior IVIG users currently on SCIG, N= 509

Even though SCIG has improved the health and life for many with PI, this is not universally the case. We asked patients who were currently on IVIG if they had previously been on SCIG and if so, why they switched back to IVIG (**Table 4**). As seen below, side effects are often the main driver in patient satisfaction with their Ig therapy.

Table 4. ***Reasons for Switching from SCIG to IVIG***

Reason	Percent
Bad side effects	47%
Did no control PI as well	25%
Dosing was too frequent	23%
Self-administration too difficult	19%
Problem using needles	16%
Reminds patient of their disease	13%
Took too long to infuse	10%
Problems with the pump	3%
Insurance would not pay for SCIG	3%
SCIG cost too much	2%

Q29b. Why did the patient switch from SCIG to IVIGIG? SELECT ALL THAT APPLY Base: Prior SCIG users currently on IVIG, N= 134

It is apparent that regardless of mode of infusion for Ig therapy, a certain amount of "bother" is associated with receiving one's Ig therapy. We asked respondents how bothered they were with 10 different attributes of the Ig therapy infusion experience. Patients were able to respond to each attribute with a scaled response, ranging from "Not bothered at all" to "Extremely bothered." In order to facilitate a direct comparison between IVIG and SCIG we have created **Table 5** below. Please note two things. First, the data reported here are the proportion that reported, "Not bothered at all" for each of the attributes. This means that a higher percent indicates less bother. Second, we created a dichotomous variable- two-categories, "Not at all bothered" and "Bothered" (a little, moderately, quite a bit, extremely). This allowed us to look for significant differences in "bother" between the two modes of therapy. We used the Chi-square statistical procedure to test for significance. Anything with a p-value that is .05 or below is significant.

Table 5. ***Comparison of Bother- IVIG vs. SCIG***

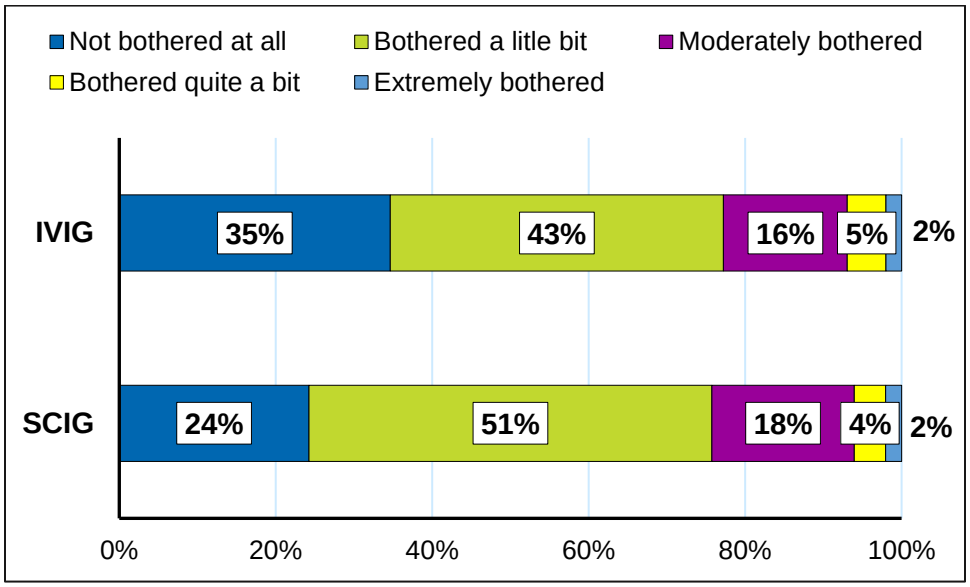
Attribute	SCIG		IVIG		p-value
	n	%	n	%	
Number of infusions/month	240	38	511	**67**	<.001
Number of needle sticks	220	35	402	**53**	<.001
Local site reactions	165	26	563	**74**	<.001
Severe side effects	491	**78**	541	70	0.002
Operating infusion pump/device	518	81	608	84	0.15
Minor side effects	220	35	277	36	0.614
Time to infuse	266	42	330	43	0.665
Convenience of treatment	370	59	446	58	0.828
Interrupts life	184	29	221	29	0.953
Cost of infusions	236	38	286	37	0.956

Q51a-j. 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: All those reporting current Ig therapy who were "Not bothered at all"; N = 1,402

Given SCIG typically is infused once per week with each infusion using 2-3 simultaneous needle sets, it is not surprising that those on IVIG report less bother with these attributes when compared to SCIG. Likewise, local site reactions such as swelling and itching at least to a certain degree are common with SCIG. Those on IVIG are however more likely to report at least some bother when it comes to severe side effects.

Data from this survey indicates that for the most part, Ig therapy infusions are well tolerated. In addition to 10 individual questions about "bother" with Ig therapy, respondents we asked a single question (**Chart 14**), "Overall, how bothered is the patient when they receive their Ig therapy?"

Chart 14. ***Overall Bother of Ig Therapy***



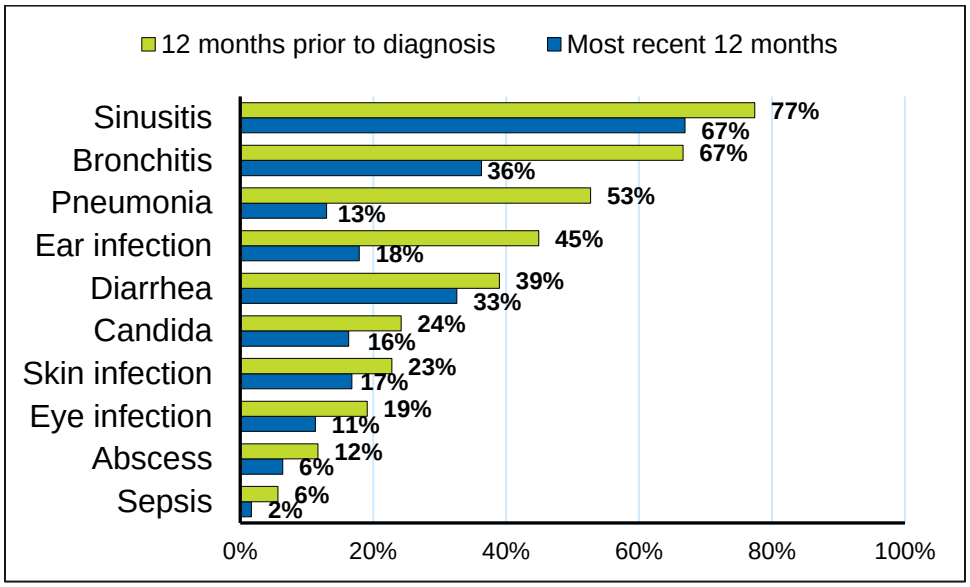
Q51b. Overall, how bothered is the patient when they get Ig therapy?
Base: Those using IVIG or SCIG, N = 1,425

Large majorities of persons on IVIG (78%) and SCIG (75%) reported little, to no bother, overall with their Ig therapy. However, this means that nearly one in four has a less than optimal experience with treatment. As discussed earlier, patients who do not tolerate their Ig therapy well, may take actions that may jeopardize their health. Opportunities exist to improve and tailor Ig therapy product and mode of infusion to individual patients.

Comparing Patient Reported Health Pre & Post Diagnosis

We asked survey participants two series of paired questions. The first set of questions asked respondents to tell us about their health in the 12 months prior to diagnosis with PI. The second set asked the same questions but the time reference was for the most recent 12 months. The purpose of these questions was to ascertain the impact of proper diagnosis and treatment with Ig therapy for those with PI. Across the board fewer incidences of any single infection were reported (**Chart 15**), with substantial decreases in the rate of pneumonia, bronchitis and ear infection.

Chart 15. ***Incidence of Infections Pre & Post Diagnosis***



Q11/Q54. Did he/she experience the following infections during the 12 months prior to diagnosis/During the past 12 months?
Base: Those using IVIG or SCIG, N = 1,384

Not only did patients experience a decline in the incidence of these infections, they also reported decreases in the frequency of most of these infections when they did occur (**Table 6**).

Table 6. ***Average Number of Reported Infections Pre & Post Diagnosis***

Infection Type	12 months prior to diagnosis	Most recent 12 months
Sinusitis	8.8	5.7
Bronchitis	2.3	2.3
Pneumonia	2.8	1.7
Ear infection	5.8	3.2
Diarrhea	41.0	31.5
Candida	8.8	7.8
Skin infection	6.5	10.0
Eye infection	3.7	3.0
Abscess	5.8	2.1
Sepsis	1.8	1.7

Q11/Q54. Did he/she experience the following infections during the 12 months prior to diagnosis/During the past 12 months? “How many times”
Base: Those using IVIG or SCIG, N = 1,384

A reduction in infections typically leads to a reduction in the need for antibiotics and antifungal medications. Many persons in this survey (51%), however, receive antibiotics prophylactically to help manage their PI.

We asked another series of paired questions that once again examined the patient experience in the 12 months prior to diagnoses and about the patient experience in the most recent 12 months. The questions in this series asked metrics about hospitalizations, including incidence, frequency and type as well as the number of missed days of school or work due to the patient's PI (**Table 7**).

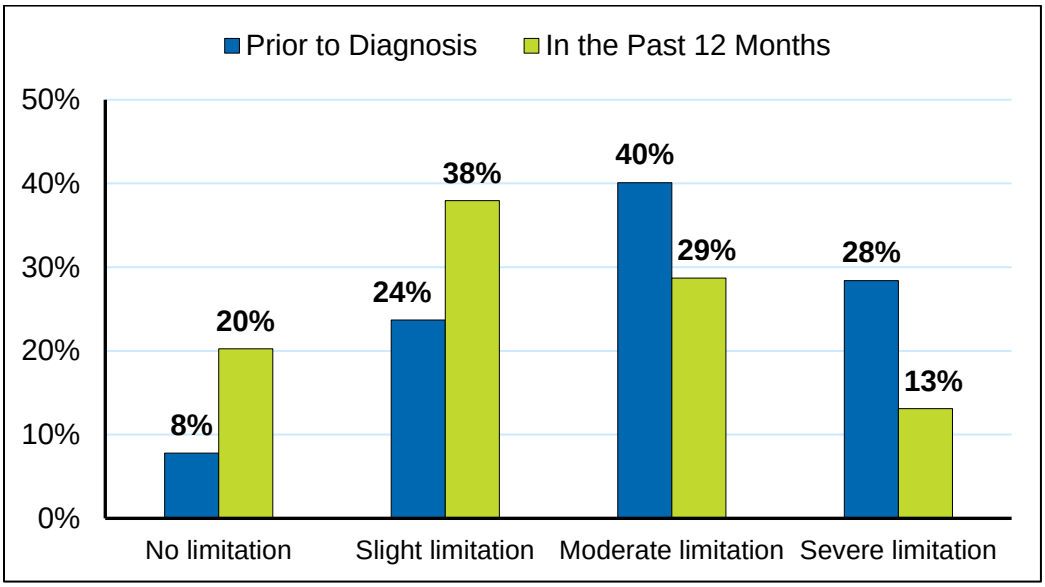
Table 7. ***Hospitalizations & Operations Pre & Post Diagnosis***

Metric	12 months prior to diagnosis	Most recent 12 months
Hospitalizations (any)	46%	23%
avg. number of times	2.97	1.99
avg. number of nights	11.6	7.8
Intensive Care Admissions (any)	10%	3%
avg. Number of nights in ICU	6.9	5.8
Operations (any)	46%	34%
avg. Inpatient operations	1.9	1.4
avg. Outpatient operation	2.1	1.6
Missed days work/school (any)	94%	90%
avg. Work days missed	29	16
avg. School days missed	32	14

Q13a-d./Q56a-d. Did he/she experience the following during the 12 months prior to diagnosis/During the past 12 months? "If so, how many times"
Base: Those using IVIG or SCIG, N = 1,384

When comparing hospitalizations in the year prior to diagnosis to the most recent year, not only were the reported instances of a hospitalizations cut in half (46% to 23%), but those who were hospitalized were hospitalized less frequently and stayed in the hospital for fewer days. Intensive care admissions were cut by a factor of three, while the reported average ICU stay also decreased by a full day. The incidence of any reported operations, either inpatient or outpatient, dropped from almost half down to one-third, while the number of operations needed dropped by about 25%. Adults reported missing an average of 13 fewer days of work and students on average reported missing 18 fewer days of school. These reductions are remarkable. Reduced hospitalizations, admissions and operations translates into reduced spending and less strain on the healthcare system. However, these are just metrics and do not answer questions about the potential impact these reductions may have on the lives of those with PI. In this survey, we asked two very basic measures of self-reported health. Self-reported activity limitations (**Chart 16**) and self-reported general health (**Chart 17**).

Chart 16. ***Reported Activity Limitations Pre & Post Diagnosis***

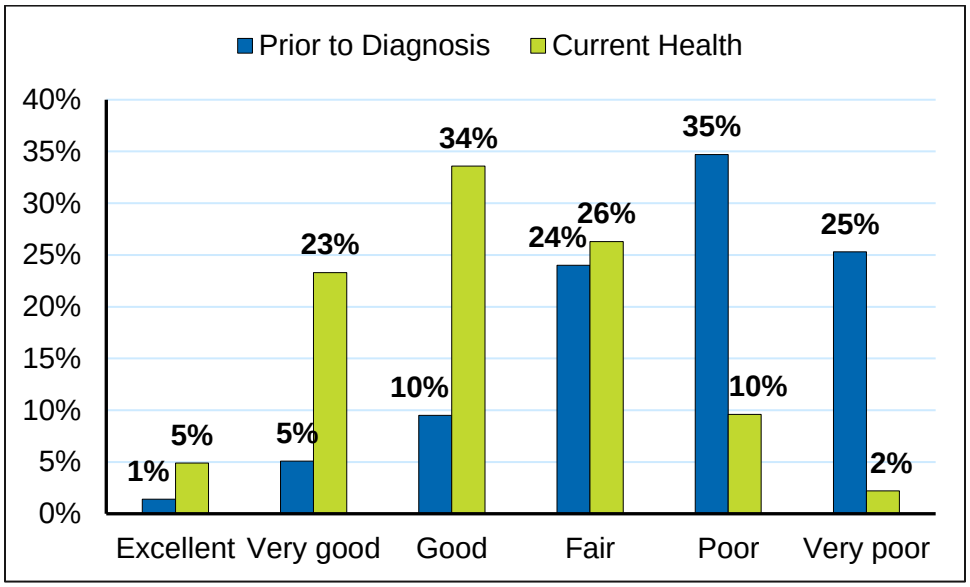


Q12./Q55. During the 12 months prior to diagnosis/During the past 12 months how much was he/she limited in work, play or normal physical activity as a result of his/her health? Base: Those using IVIG or SCIG, N = 1,395

Survey respondents were more likely to report no limitations in their physical activity in the most recent 12 months than in the 12 months prior to diagnosis. Conversely, respondents were less likely to report severe limitations due to their health in the most recent year when compared to the year prior to a diagnosis of PI.

We obtained perhaps the most significant data in this survey by asking a simple, paired set of questions examining patient health in the 12 months prior to diagnosis and the patient's current health (**Chart 17**).

Chart 17. ***Reported General Health Pre & Post Diagnosis***



Q9./Q52. Would you describe his/her heath in the 12 months prior to diagnosis as…/In general would you say the patient's health is..?
Base: Those using IVIG or SCIG, N = 1,389

Prior to diagnosis only 16% of the respondents reported patient health as "good or better." This stands in marked contrast to the patient reported current health in which we found 62% who stated their current health was "good or better."

The clinical effectiveness of Ig therapy for the treatment or primary antibody deficiencies is well documented[9,10,11]. Data from this survey, however, illustrates the patient perspective of the tremendous, positive impact, regular Ig therapy has on the health, well-being and quality of life for those with PI.

Conclusions

There is no doubt that immunoglobulin (Ig) replacement therapy has a significant and positive impact for people diagnosed with primary immunodeficiency diseases (PI). Once these patients begin receiving Ig therapy on a regular basis, their health status improves markedly, their activity limitations drop significantly and their quality of life improves dramatically. The cost of Ig therapy, which is medically indicated for improving the health of patients with PI who are antibody deficient, is expensive. We can, however, demonstrate significant cost savings that accompany treatment with Ig therapy. These savings in terms of hospital, healthcare and lost wages represents most of the costs of this therapy.

The key to a better prognosis for the patient is early diagnosis and treatment of their primary immunodeficiency. All too often patients are not diagnosed until well after the irreversible effects of repeated, crippling, infections have taken their toll. For many, this results in life-long impairments and disabilities that could have been avoided with the early intervention of Ig therapy. The additional cost for these permanently disabled individuals is much greater than any residual cost of the Ig therapy itself.

Ig therapy is a life-changing and lifesaving therapy for those with PI, and it creates healthier and more productive members of society. The early diagnosis of PI and subsequent treatment with Ig therapy is critical if patients with PI are to avoid many of the long-term debilitating conditions that place an increased burden not only on the patients but on the U.S. healthcare system as well.

Ig therapy products are not generic equivalents. As demonstrated with the data from this survey, many patients have varying degrees of tolerability with different Ig therapy products. Based on data from this survey, it is obvious there is room for improvement.

The goal for those with PI should be for “minimally disruptive medicine”—an approach to patient care that emphasizes individual preferences, takes account of comorbidity, and seeks to reduce the workload for patient and caregiver. Interventions that implement minimally disruptive medicine and attack the burden of treatment also need to provide coordinated, maximally supportive care that is person rather than disease centered.

Table 8. ***Selected Respondent Demographics***

Gender	
Male	33%
Female	67%
Patient age	
0 - 18 years	14%
19 - 34 years	15%
35 - 50 years	22%
51 - 64 years	31%
65+ years	18%
Race	
Asian/Pacific Islander	0.2%
Black/African-American	0.3%
Hispanic/Latino	1.6%
Native American	0.5%
White, non-Hispanic	95.6%
Two or more races	1.3%
Other	0.5%
Education Level	
Less than High School	5%
High School/GED	14%
1- 3 years college	27%
Undergraduate degree	28%
Graduate degree	27%
Household Income	
0 to $24,999	21%
$25,000 to $49,999	19%
$50,000 to $74,999	18%
$75,000 to $99,999	15%
$100,00 or more	27%

Endnotes

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Immune Deficiency Foundation

INSTRUCTIONS

IDF 2013 Treatment Survey

- **Adult patients:** Follow Instructions A
- **Adult patients with PI with a child with PI:** Follow Instructions B
- **Parent of a child with PI:** Follow Instructions C

FOR ADULT PATIENTS - INSTRUCTIONS A

1. Complete the IDF Treatment Survey.

2. Complete the survey *SF-12 Your Health and Well-Being.*

3. Place both the completed SF-12 and the completed IDF Treatment Survey into the return envelope provided for you and drop it in the mail. There is no need to place any postage on the return envelope. There is no need to return the uncompleted SF-10.

FOR ADULT PATIENTS WITH PI WITH A CHILD WITH PI - INSTRUCTIONS B

1. Complete the IDF Treatment Survey.

2. Complete the survey called *SF-12 Your Health and Well-Being.* IDF is only asking questions about YOUR health not of your child or children.

3. Please place both the completed SF-12 and the completed IDF Treatment Survey into the return envelope provided for you and drop it in the mail. There is no need to place any postage on the return envelope. There is no need to return the uncompleted SF-10.

FOR THE PARENT OF A CHILD WITH PI - INSTRUCTIONS C

1. Complete the IDF Treatment Survey.

2. Complete the survey called *SF-10 Health Survey for Children* **only** if your child is between the ages of 5 and 18. If your child is under the age of 5, you only need to complete and return the IDF Treatment Survey.

3. Place both the completed SF-10 and the completed IDF Treatment Survey into the return envelope provided for you and drop it in the mail. There is no need to place any postage on the return envelope. There is no need to return the uncompleted SF-12.

Please turn the page and begin the IDF 2013 Treatment Survey

Immune Deficiency Foundation

TREATMENT EXPERIENCES AND PREFERENCES AMONG PATIENTS WITH PRIMARY IMMUNODEFICIENCY: 2013

1. Are you a **patient** with a primary immunodeficiency disease (PI) or **parent/caregiver** of a child **in the household** with PI?

☐1 PI patient → **CONTINUE**
☐2 Parent/caregiver → **CONTINUE**
☐3 Both → **CONTINUE**
☐4 Neither → **PLEASE STOP**

IF YOU ARE A PATIENT WITH PI, PLEASE ANSWER SURVEY QUESTIONS ABOUT YOURSELF. IF YOU ARE NOT A PATIENT, PLEASE ANSWER SURVEY QUESTIONS ABOUT THE OLDEST CHILD WITH PI IN HOUSEHOLD.

2. What is the date of birth of the (adult patient/oldest child) in the household with a primary immunodeficiency disease?

_____ _____ _____
MONTH DAY YEAR

3. What is the gender of that person?

☐1 Male
☐2 Female

4. At what age (in years) was that person first diagnosed with a primary immunodeficiency disease?

_____ Age at diagnosis
☐00 Less than one year old

5. What is the specific diagnosis of that person's immunodeficiency disease?

☐1 Agammaglobulinemia (XLA)
☐2 Ataxia Telangiectasia
☐3 Common Variable Immunodeficiency (hypogammaglobulinemia)
☐4 Hyper IgM Syndrome
☐5 IgA Deficiency
☐6 IgG Subclass Deficiency
☐7 Severe Combined Immune Deficiency
☐8 Specific Antibody Deficiency
☐9 Wiskott-Aldrich Syndrome
☐10 Other (please specify)

_____

6a. Did the patient experience repeated, serious, or unusual infections prior to initial diagnosis as immune deficient?

☐1 Yes
☐2 No → **SKIP TO Q7**

6b. At what age (in years) did these repeated, serious, or unusual infections begin?

_____ Age of onset
☐00 Less than one year of age

7. How many times had he/she been hospitalized **before diagnosis** as immune deficient?

_____ Number of times
☐00 None

8. By the time of initial diagnosis as immune deficient, had he/she suffered any **permanent impairment or loss of** ...?
SELECT ALL THAT APPLY

☐1 Digestive function
☐2 Hearing
☐3 Kidney function
☐4 Liver function
☐5 Lung function
☐6 Mobility
☐7 Neurological function
☐8 Vision
☐9 Other (specify) _____
☐10 No permanent loss

THE NEXT QUESTIONS ARE ABOUT THE PATIENT'S HEALTH IN THE 12 MONTHS PRIOR TO BEING DIAGNOSED AS PRIMARY IMMUNODEFICIENT

9. Would you describe his/her health in the **12 months prior to diagnosis** as…

☐1 Excellent
☐2 Very good
☐3 Good
☐4 Fair
☐5 Poor
☐6 Very poor

10. During the **12 months prior to diagnosis**, did he/she have …

SELECT ALL THAT APPLY

- ☐1 Asthma
- ☐2 Arthritis
- ☐3 Autoimmune condition
- ☐4 Cancer/leukemia
- ☐5 COPD
- ☐6 Digestive disease
- ☐7 Hepatitis
- ☐8 Malabsorption/Diarrhea
- ☐9 Lymphopenia (low white count)
- ☐10 Neurological disease
- ☐11 Neutropenia
- ☐12 Other chronic condition (please specify)

_____

11. Did the patient experience any of the following infections during **the 12 months prior to diagnosis?**

Infection type	Yes	How many times
a. Abscess	☐1 →	_____
b. Bronchitis	☐1 →	_____
c. Candida (thrush)	☐1 →	_____
d. Diarrhea (repeated)	☐1 →	_____
e. Ear infection (repeated)	☐1 →	_____
f. Eye infection	☐1 →	_____
g. Pneumonia	☐1 →	_____
h. Sepsis (blood poisoning)	☐1 →	_____
i. Sinusitis	☐1 →	_____
j. Skin infection	☐1 →	_____
k. Other infections	☐1 →	_____
l. None of these	☐1	

12. 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.

- ☐1 No limitation
- ☐2 Slight limitation
- ☐3 Moderate limitation
- ☐4 Severe limitation

13a. Was he/she hospitalized overnight or longer for any reason during the **12 months prior to diagnosis**?

- ☐1 Yes
- ☐2 No → **SKIP TO Q14**

13b. How many TIMES was he/she hospitalized in the **12 months prior to diagnosis**?

_____ Times hospitalized

13c. How many NIGHTS was he/she hospitalized in the **12 months prior to diagnosis**?

_____ Nights hospitalized

13d. How many NIGHTS, if any, was he/she in an INTENSIVE CARE UNIT in the **12 months prior to diagnosis?**

_____ Nights in ICU

☐00 None

14. How many operations, if any, did he/she have in the **12 months prior to diagnosis**?

_____ Number inpatient

_____ Number outpatient

☐00 None

15. In the **12 months prior to diagnosis**, approximately how many days did he/she use..

_____ Antibiotics

_____ Other prescription drugs (not Ig)

_____ Respiratory therapy

_____ Oxygen

_____ Physical therapist

_____ Visiting nurse (not for Ig)

16. Not counting hospitalizations, about how many DOCTOR VISITS did the patient make during the **12 months prior to diagnosis**?

_____ Primary care visits

_____ Specialist visits

☐00 No doctor visits

17. 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**? **(parent/caregiver how many days missed due to patient illness)**

_____ Days missed work
_____ Days missed school
☐000 None
☐999 Infant/Not applicable

THIS SECTION IS ABOUT THE PATIENT'S TREATMENT EXPERIENCES

18a. Which type of physician is responsible for the treatment and management of the patient's PI?

☐1 Immunologist
☐2 Hematologist
☐3 Ear, nose & throat (ENT)
☐4 Allergist
☐5 Pulmonologist
☐6 Other (please specify)

_____

18b. How many times over the past 12 months has the patient seen this physician?

_____ Times

19a. 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

☐1 IVIG → **SKIP TO Q20**
☐2 SCIG → **SKIP TO Q20**
☐3 IM → **SKIP TO Q20**
☐4 No, neither → **CONTINUE**

19b. Is there any reason why the patient has never been treated with immunoglobulin replacement therapy?

☐1 Never prescribed by the doctor
☐2 Lack of insurance or inadequate insurance
☐3 Cost
☐4 Concerns about safety/side-effects
☐5 Fear of treatment
☐6 Other

IF NEVER USED SKIP TO Q71 ON PAGE 10

20. How many years, in total, has he/she been treated for immunodeficiency with IVIG, SCIG or IM (Ig therapy) on a regular basis?

_____ Years on immunoglobulin

21a. Is he/she currently being treated with SCIG, IVIG or IM for his/her immunodeficiency disease?

☐1 Yes, SCIG → **SKIP TO Q22**
☐2 Yes, IVIG → **SKIP TO Q29a**
☐3 Yes, IM → **SKIP TO Q29a**
☐4 No → **CONTINUE**

21b. Why is the patient no longer being treated with immunoglobulin?
SELECT ALL THAT APPLY

☐1 Immunoglobulin no longer prescribed by the doctor as medically necessary
☐2 Lack of insurance coverage/inadequate insurance
☐3 Too expensive (despite good insurance)
☐4 IVIG not available or hard to get
☐5 Safety/side effects
☐6 Other (PLEASE SPECIFY)

_____

IF NOT USING IVIG, SCIG OR IM SKIP TO Q71 ON PAGE 10

22. What year did you start SCIG?

_____ (please enter year)

23a. How difficult was it **learning** to administer SCIG?

☐1 Very difficult
☐2 Somewhat difficult
☐3 Not too difficult
☐4 Easy

23b. How difficult is it to **administer** SCIG?

☐1 Very difficult
☐2 Somewhat difficult
☐3 Not too difficult
☐4 Easy

24. When was the last time a health professional, such as a nurse or doctor, observed and evaluated the patient's SCIG infusion technique?

- ☐1 Less than six months
- ☐2 6-12 months
- ☐3 1 year or longer

25. What is the brand name of the SCIG pump the patient uses?

_____

26. Prior to SCIG therapy did the patient receive IVIG therapy?

- ☐1 Yes → **CONTINUE**
- ☐2 No → **SKIP TO Q30**

27. Why did the patient switch from IVIG to SCIG? **SELECT ALL THAT APPLY**

- ☐1 Insurance reasons
- ☐2 Physician recommended it
- ☐3 Problems finding a vein for IVIG
- ☐4 Patient is an infant/child
- ☐5 Problems getting to IVIG infusion site
- ☐6 Have limited mobility
- ☐7 It is more convenient
- ☐8 Reactions to IVIG
- ☐9 Other (specify below)

_____

28. Compared to IVIG therapy how well do you feel SCIG therapy controls the patient's PI?

- ☐1 Much better than IVIG
- ☐2 Better than IVIG
- ☐3 About the same as IVIG
- ☐4 Worse than IVIG
- ☐5 Much worse than IVIG

CURRENT SCIG USER SKIP TO Q30

29a. Has the patient ever been on SCIG therapy?

- ☐1 Yes
- ☐2 No → **SKIP TO Q30**

29b. Why is the patient no longer on SCIG therapy?

- ☐1 Had bad side-effects
- ☐2 Had a problem using needles
- ☐3 Had a problem with the pump
- ☐4 Too long to infuse
- ☐5 Cost too much
- ☐6 Did not control PI as well as IVIG
- ☐7 Dosing was too frequent
- ☐8 Self-administration was difficult
- ☐9 Insurance would not pay for SCIG
- ☐10 Reminds patient of their disease
- ☐11 Other _____

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

- ☐1 Daily
- ☐2 Three times per week
- ☐3 Two times per week
- ☐4 Every week
- ☐5 Every two weeks
- ☐6 Every three weeks
- ☐7 Every four weeks
- ☐8 Every five weeks
- ☐9 Every six weeks or more

31. About how many grams of Ig per infusion does he/she normally receive?

_____ Grams

_____ mL (if SCIG)

☐888 Not sure

32. Who usually administers the therapy?

- ☐1 Doctor
- ☐2 Nurse
- ☐3 Patient (self-infused)
- ☐4 Other family member
- ☐5 Other

33. Where does the patient usually receive his/her Ig therapy?

- ☐1 At home, self-infused
- ☐2 At home, nurse infused
- ☐3 Doctor's private office
- ☐4 Hospital outpatient
- ☐5 Hospital clinic
- ☐6 Infusion suite
- ☐7 Other (specify)

_____

34. How long does the therapy usually take?

_____ Hours (a)
_____ Minutes (b)

35. About how much does the patient weigh?

_____ Weight in pounds

36. Who determines the rate of infusion?

☐1 Patient/parent
☐2 Doctor
☐3 Nurse
☐4 Other (please specify)

_____

37a. 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

	By mouth	IV
a. Antihistamine, usually	☐1	☐2
b. Antihistamine, sometimes	☐1	☐2
c. Steroid, usually	☐1	☐2
d. Steroid, sometimes	☐1	☐2
e. Never any of these	☐1	

38a. Does the patient (parent/caretaker) need to take off from school or work to get their Ig therapy?

☐1 Yes
☐2 No → **SKIP TO Q39**

38b. 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?

_____ Days missed school

_____ Days missed work

39. **During the past 12 months**, has he/she experienced any of the following **during or after** Ig therapy?
SELECT ALL THAT APPLY

	During	After
a. Abdominal pain	☐1	☐2
b. Anxiety	☐1	☐2
c. Blood in tubing (SCIG)	☐1	☐2
d. Chills	☐1	☐2
e. Diarrhea	☐1	☐2
f. Dizziness	☐1	☐2
g. Fatigue	☐1	☐2
h. Fever	☐1	☐2
i. Headache	☐1	☐2
j. Hepatitis	☐1	☐2
k. Hives	☐1	☐2
l. Increase in blood pressure	☐1	☐2
m. Migraine headache	☐1	☐2
n. Muscle spasms	☐1	☐2
o. Nausea	☐1	☐2
p. Drop in blood pressure	☐1	☐2
q. Swelling at infusion site	☐1	☐2
r. Redness at infusion site	☐1	☐2
s. Muscle aches	☐1	☐2
t. Kidney problems	☐1	☐2
u. Vomiting	☐1	☐2
v. Weakness	☐1	☐2
w. Wheezing	☐1	☐2

40a. Has the patient ever had any of the following **serious side-effects or reactions** from their Ig therapy?
PLEASE SELECT ALL THAT APPLY

☐1 Aseptic meningitis
☐2 Blood clots
☐3 Blurred vision
☐4 Hemolytic anemia
☐5 Pulmonary embolism
☐6 Seizure
☐7 Stroke
☐8 NONE OF THESE → **SKIP TO Q41a**

40b. When was the most recent time that he/she had a **serious side effect or reaction** from their Ig?

- ☐1 0 to 6 months
- ☐2 7 to 12 months
- ☐3 1 to 2 years ago
- ☐4 3 to 4 years ago
- ☐5 5 years or more
- ☐6 Never → **SKIP TO Q41a**

40c. Did the patient's side effect or reaction cause him/her to …
SELECT ALL THAT APPLY

- ☐1 Slow down infusion rate
- ☐2 Switch products
- ☐3 Prefer a specific Ig product
- ☐4 Only receive Ig in doctor's office
- ☐5 Report the event to the FDA
- ☐6 Other (specify)

  _____
- ☐7 None of these

40d. Was the doctor told about the patient's serious side-effect or reaction?

- ☐1 Yes → **CONTINUE**
- ☐2 No → **SKIP TO Q40F**

40e. What did the doctor do?

- ☐1 Switched products
- ☐2 Reduced amounts
- ☐3 Slowed infusion rate
- ☐4 Gave medicine
- ☐5 Said it was normal
- ☐6 Changed from IVIG to SCIG
- ☐7 Changed from SCIG to IVIG
- ☐8 Nothing

40f. Has the patient ever had a serious side effect or reaction from Ig therapy when…
SELECT ALL THAT APPLY

- ☐1 Trying a new product for the first time
- ☐2 Switched to a different product used before
- ☐3 Using a product with no previous problems
- ☐4 None of these

41a. Has the patient (parent/caretaker of a patient) ever missed school or work **due to a reaction from** their Ig therapy?

- ☐1 Yes
- ☐2 No → **SKIP TO Q42**

41b. 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?

_____ Days missed school

_____ Days missed work

42. How well does immunoglobulin control the patient's immunodeficiency?

- ☐1 Completely controlled
- ☐2 Well controlled
- ☐3 Adequately controlled
- ☐4 Less than adequately controlled
- ☐5 Poorly controlled

43a. Does the patient experience periods of fatigue or low energy between Ig therapy treatments (wear off)?

- ☐1 Always → **CONTINUE**
- ☐2 Occasionally → **CONTINUE**
- ☐3 Never → **SKIP TO Q44**

43b. How long after infusion does he/she feel this wear off?

_____ Days after infusion

43c. Does the "wear off" result in any of the following? **SELECT ALL THAT APPLY**

- ☐1 Infection
- ☐2 Need for antibiotics
- ☐3 Need for other medication
- ☐4 Missed school or work
- ☐5 Forgoing usual activity
- ☐6 Forgoing of a pleasurable activity
- ☐7 Decreased performance in usual activities

44. Does he/she tolerate any immunoglobulin products better than others, or are they all about the same?

- ☐1 All about the same
- ☐2 Some better than others
- ☐3 Have only been on one product

45. As a result of concerns about product tolerability has he/she ever …
SELECT ALL THAT APPLY

- ☐1 Refused a particular product
- ☐2 Switched off a product
- ☐3 Switched to another product
- ☐4 Delayed a scheduled infusion
- ☐5 None of these

46. As a result of product effectiveness has he/she ever…
SELECT ALL THAT APPLY

- ☐1 Refused a particular product
- ☐2 Switched off a product
- ☐3 Switched to another product
- ☐4 Delayed a scheduled infusion
- ☐5 None of these

47a. Which of the following Ig products has the patient ever used?		
47b. Which of the following Ig products do they **currently** use?		↓
Product	↓	↓
a. Bivigam (Biotest)	☐1	☐2
b. Carimune (CSL Behring)	☐1	☐2
c. Flebogamma (Grifols)	☐1	☐2
d. Gammagard Liquid (Baxter)	☐1	☐2
e. Gammagard S/D (Baxter)	☐1	☐2
f. Gammaked (Kedrion)	☐1	☐2
g. Gamunex-C (Grifols)	☐1	☐2
h. Gammaplex (Bio Products)	☐1	☐2
i. Hizentra (CSL Behring)	☐1	☐2
j. Octagam (Octapharma)	☐1	☐2
k. Privigen (CSL Behring)	☐1	☐2
l. Other	☐1	☐2

48. How often does he/she get the Ig product they prefer most?

- ☐1 Always
- ☐2 Most of the time
- ☐3 Some of the time
- ☐4 Only occasionally
- ☐5 Never
- ☐6 Only tried one product

49. How satisfied is the patient with the Ig product **currently** being used?

- ☐1 Very satisfied
- ☐2 Somewhat satisfied
- ☐3 Neither
- ☐4 Somewhat dissatisfied
- ☐5 Very dissatisfied

50. Who is primarily responsible for the selection of the Ig product that the patient uses?

- ☐1 Patient
- ☐2 Doctor
- ☐3 Medical plan/facility
- ☐4 Insurance provider
- ☐5 Other (please specify)

_____

51a. 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.

	Not bothered at all	Bothered a little bit	Moderately bothered	Bothered quite a bit	Extremely Bothered
	↓	↓	↓	↓	↓
a. Convenience of treatment	☐1	☐2	☐3	☐4	☐5
b. Severe side-effects	☐1	☐2	☐3	☐4	☐5
c. Minor side-effects	☐1	☐2	☐3	☐4	☐5
d. Local site reactions	☐1	☐2	☐3	☐4	☐5
e. Number of needle sticks	☐1	☐2	☐3	☐4	☐5
f. Time to infuse	☐1	☐2	☐3	☐4	☐5
g. Number of infusions each month	☐1	☐2	☐3	☐4	☐5
h. Cost of infusions	☐1	☐2	☐3	☐4	☐5
i. Interrupts life	☐1	☐2	☐3	☐4	☐5
j. Operating pump or infusion device	☐1	☐2	☐3	☐4	☐5
k. Other_____	☐1	☐2	☐3	☐4	☐5

51b. Overall, how bothered is the patient when they get Ig therapy?

- ☐1 Not bothered at all
- ☐2 Bothered a little bit
- ☐3 Moderately bothered
- ☐4 Bothered quite a bit
- ☐5 Extremely bothered

THE NEXT QUESTIONS ARE ABOUT THE PATIENT'S HEALTH IN THE PAST 12 MONTHS.

52. In general would you say the patient's health is:

- ☐1 Excellent
- ☐2 Very good
- ☐3 Good
- ☐4 Fair
- ☐5 Poor
- ☐6 Very poor

53. During the **past 12 months**, has he/she had:

- ☐1 Asthma
- ☐2 Arthritis
- ☐3 Autoimmune condition
- ☐4 Cancer/leukemia
- ☐5 COPD
- ☐6 Digestive disease
- ☐7 Hepatitis
- ☐8 Malabsorption/diarrhea
- ☐9 Lymphopenia (low white count)
- ☐10 Neurological disease
- ☐11 Neutropenia
- ☐12 Other chronic condition

54. Did he/she experience the following infections during the **past 12 months**?

Infection type	Yes	How many times
a. Abscess	☐1 →	_____
b. Bronchitis	☐1 →	_____
c. Candida (thrush)	☐1 →	_____
d. Diarrhea (repeated)	☐1 →	_____
e. Ear infection (repeated)	☐1 →	_____
f. Eye infection	☐1 →	_____
g. Pneumonia	☐1 →	_____
h. Sepsis (blood poisoning)	☐1 →	_____
i. Sinusitis	☐1 →	_____
j. Skin infection	☐1 →	_____
k. Other infections	☐1 →	_____
l. None of these	☐1	

55. 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?

- ☐1 No limitation
- ☐2 Slight limitation
- ☐3 Moderate limitation
- ☐4 Severe limitation

56a. Has he/she been hospitalized overnight or longer for any reason during the **past 12 months**?

- ☐1 Yes
- ☐2 No → **SKIP TO Q57**

56b. How many TIMES was he/she hospitalized in the **past 12 months**?

_____Times hospitalized

56c. How many NIGHTS was he/she hospitalized in the **past 12 months**?

_____ Nights hospitalized

56d. How many NIGHTS, if any, was he/she in an INTENSIVE CARE UNIT in the **past 12 months**?

_____ Nights in ICU

☐00 None

57. How many operations, if any, did he/she have in the past **12 months**?

_____ Number inpatient

_____ Number outpatient

☐00 None

58. Approximately how many days in the **past 12 months** did he/she use:

_____ Antibiotics

_____ Other prescription drugs (not Ig)

_____ Respiratory therapy

_____ Oxygen

_____ Physical therapist

_____ Visiting nurse (not for Ig)

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

_____ Primary care visits

_____ Specialist visits

☐00 No doctor visits

60. 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**? **(parent/caregiver how many days missed due to patient illness)**

_____ Days missed work

_____ Days missed school

☐000 None

☐999 Infant/Not applicable

61. In the **past 12 months**, how long has the patient taken antibiotics **to prevent infections (prophylactically)**?

☐1 Less than 1 month
☐2 1 to 6 months
☐3 Longer than 6 months
☐4 Did not take any antibiotics → **SKIP TO Q62**

(please list antibiotics below)

_____

_____

_____

62. In the **past 12 months**, how long has the patient taken antibiotics **for an active infection**?

☐1 Less than 1 month
☐2 1 to 6 months
☐3 Longer than 6 months
☐4 Did not take any antibiotics → **SKIP TO Q63**

(please list antibiotics below)

_____

_____

_____

63. What kind of health insurance does the **patient** currently have?
SELECT ALL THAT APPLY

☐1 Employer sponsored group plan
☐2 COBRA
☐3 Individual policy
☐4 Medicare A & B
☐5 Medicare Supplemental Plan
☐6 Medicare Advantage Plan
☐7 Medicare due to disability
☐8 Medicaid
☐9 SCHIP or other government policy
☐10 State Exchange/Marketplace
☐11 Federal Exchange/Marketplace
☐12 TRICARE
☐13 Veterans Policy
☐14 Other Insurance (specify)

_____

☐15 No health insurance coverage

64. In the past three years, **due to health insurance**, has the patient ever had a problem in getting his/her regular infusion?

☐1 Yes
☐2 No → **SKIP TO Q66**

65. When was the most recent time the patient had a problem getting his/her regular infusion **due to health insurance?**

☐1 Past month
☐2 Past six months
☐3 Past year
☐4 Two to three years ago

66. In **the past 12 months**, which of the following problems, if any, has the patient experienced **due to health insurance**?
SELECT ALL THAT APPLY

☐1 Site of care for infusion changed
☐2 Increased interval between infusion
☐3 Reduced dosage of infusion
☐4 Delayed infusions
☐5 Cancelled infusions
☐6 Switched to less tolerated product
☐7 Switched to less preferred product
☐8 Switched from IVIG to SCIG
☐9 Switched from SCIG to IVIG
☐10 No product available
☐11 Reimbursement problems
☐12 Treating physicians now out of network
☐13 Any other problem (specify)

_____

☐14 None

67. How many times in the **past 12 months** has the patient experienced a problem getting his/her regular infusion?

_____ times in past 12 months

☐888 None

68. As a result of health insurance policies, has the patient had any problems seeing healthcare specialists?

☐1 Yes
☐2 No

69. **Since December 2012**, what changes, if any, have you experienced with your **overall** health insurance costs?

☐1 I pay MORE for my health insurance
☐2 I pay LESS for my health insurance
☐3 I pay the SAME for my health insurance

70. Specifically thinking about your **personal costs** for Ig replacement therapy, **since December 2012** what changes, if any, have you experienced with paying for Ig replacement therapy?

☐1 It costs me MORE for Ig therapy
☐2 It costs me LESS for Ig therapy
☐3 It costs me the SAME for Ig therapy

71. As you may know, a health reform bill known as the Affordable Care Act (ACA) was signed into law in 2010. Given what you know about the ACA, which of the following statements most closely matches your view.

"Within the next 12 months I will be…"

☐1 **Voluntarily** enrolled in the Health Insurance Marketplace
☐2 **Forced** to enroll in the Health Insurance Marketplace
☐3 Insured through an employer
☐4 Insured through Medicare
☐5 Insured through Medicaid
☐6 Not sure how I will get my health insurance

THE LAST FEW QUESTIONS ARE TO HELP IDF LEARN MORE ABOUT WHO IS AFFECTED BY PRIMARY IMMUNODEFICIENCY DISEASES.

72. Which of the following categories would best describe the race or ethnicity of the patient?

☐1 American Indian/Alaskan native
☐2 Asian/Pacific Islander
☐3 Black/African-American
☐4 Hispanic or Latino
☐5 White, non-Hispanic
☐6 Two or more races
☐7 Other (Specify)

_____

PLEASE TURN PAGE

73. What is the current employment status of the **patient** (head of household if patient is a child)?

- ☐1 Employed full time
- ☐2 Employed part time
- ☐3 Unemployed, looking for work
- ☐4 Student
- ☐5 Homemaker
- ☐6 Disabled/too ill to work
- ☐7 Other

74. What is the last grade or year of school completed by the **patient** (head of household if patient is a child)?

- ☐1 8th grade or less
- ☐2 Some high school
- ☐3 High school grad/GED
- ☐4 1-3 years of college
- ☐5 4 year college grad
- ☐6 Graduate degree

75. What was the patient's (or household's) total income last year?

- ☐1 0 to $24,999
- ☐2 $25,000 to $49,999
- ☐3 $50,000 to $74,999
- ☐4 $75,000 to $99,000
- ☐5 $100,000 or more

PLEASE RETURN IN THE ENCLOSED ENVELOPE TO THE IMMUNE DEFICIENCY FOUNDATION

IF YOU HAVE ANY QUESTIONS ABOUT THIS SURVEY PLEASE CALL THE IMMUNE DEFICIENCY FOUNDATION AND ASK FOR THE DIRECTOR OF SURVEY RESEARCH 1.800.296.4433

Adult patients - Please complete the survey *SF-12 Your Health and Well Being.*

Adult patients with PI with a child with PI - Please complete the survey *SF-12 Your Health and Well Being.*

Parents of a child with PI - Please complete the survey *SF-10 Health Survey for Children* **only** if your child is between the ages of 5 and 18.