

**TREATMENT EXPERIENCES AND
PREFERENCES AMONG PATIENTS WITH
PRIMARY IMMUNODEFICIENCY DISEASES**

NATIONAL SURVEY OF PATIENTS: 2008



**The National Patient Organization Dedicated to Advocacy, Education and Research
for
Primary Immunodeficiency Diseases**

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Abt SRBI, Inc.**

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Overview

The Immune Deficiency Foundation (IDF) conducted its Second National Survey of the Treatment Experiences and Preferences of Patients with Primary immunodeficiency Diseases in 2008. A total of 2,500 patients with primary immunodeficiency diseases (PIDD), who had reported either intravenous immunoglobulin (IVIG) therapy, or subcutaneous immunoglobulin (SCIG) therapy, were randomly selected from the IDF database for this survey. These individuals were mailed an eight-page, self-administered questionnaire, which they were asked to complete and return to IDF. A second mailing was made to those who did not respond to the first mailing. A total of 1,088 respondents returned a completed questionnaire for a 43.5% response rate. After removing cases of respondents with non-primary immunodeficiency disease diagnoses, or those not currently using immunoglobulin therapies, the survey yielded a total of 1,030 patients with primary immunodeficiency diseases who were currently being treated with either IVIG or SCIG. These cases provide a relatively large national sample of persons with primary immunodeficiency diseases, who have been treated with immunoglobulin 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 treatment experiences.

Background: Immunodeficiency Diseases

Primary immunodeficiency diseases represent a class of disorders in which there is an intrinsic defect in the human immune systems (rather than immune disorders that are secondary to infection, chemotherapy, or some other external agent). In some cases, the body fails to produce any or enough antibodies to fight infection. In other cases, the cellular defenses against infection fail to work properly. There are more than 150 different primary immunodeficiency diseases currently recognized by the World Health Organization.

Medical recognition of primary immunodeficiency disease is a little more than fifty years old. Although these disorders may have existed in antiquity, it was not until the development of antibiotics that infections could be controlled long enough to recognize there was an underlying defect in the immune system. Also, the parallel development of gamma globulin in World War II provided a replacement therapy for the antibody deficiency forms of immune deficiency. Today, immunoglobulin replacement therapy (IG therapy) is available in intravenous (IVIG) and subcutaneous (SCIG) modes of administration.

The major health surveys conducted by the government in the United States, the National Health Interview Survey and the National Health and Nutrition Examination Survey, do not collect information on primary immunodeficiency diseases. No comprehensive population survey has ever been undertaken by the federal government to estimate the prevalence or the population characteristics of these diseases in the United States. The 2005 prevalence survey conducted by the Immune Deficiency Foundation

provided the first reliable estimate of the prevalence of diagnosed primary immunodeficiency diseases in the United States. However, a probability sample of 10,000 households yielded only 23 cases of primary immunodeficiency diseases, which is too small to yield reliable estimates of the characteristics of patients and their treatment experiences. Hence, although these diseases are clinically described in the medical literature, there is no current, comprehensive portrait available of the patient with primary immunodeficiency disease, their medical condition and their treatment.

Treatment Experiences Survey of IVIG Users: 2002

In 1997 IDF conducted its first treatment survey based on respondents who stated that they were IVIG users in IDF's first patient survey. In 2002, the Immune Deficiency Foundation issued its first report on the Treatment Experiences and Preferences among Patients with Primary immunodeficiency Diseases. Two national surveys of patients with primary immunodeficiency diseases conducted by IDF in 1996/97 and 2002 provided the sampling frame for a follow-up survey of 1,000 persons identified as currently using intravenous immunoglobulin (IVIG). The treatment survey was first mailed to the 2,589 unique cases with a name and address in a database that could be linked with the 1996 patient survey information. However, since the 1996 survey information was more than five years old, a very substantial portion of the sample was expected to be bad addresses. The original addresses were matched with the National Change of Address database to update these addresses where possible.

An eight-page questionnaire with a cover letter was mailed by SRBI to these persons on August 29, 2002. The cover letter included toll-free numbers for both IDF and SRBI. The package included a postage-paid business reply envelope for respondents to return the completed questionnaire. A postcard reminder was sent to all potential respondents one week later. The questionnaire for the "old patients" from the 1996 survey was sent to all, regardless of their reported IVIG use in 1996/97. Consequently, the questionnaire for this population was structured so that patients who were no longer using IVIG ended the interview.

A second sample of new patients that did not participate in the 1996 survey was drawn from the Second National Patient Survey. Only patients with primary immunodeficiency diseases who were currently using IVIG to treat their condition were included in this sample. Assuming a response rate of approximately fifty percent to the second survey, nearly 1,000 eligible participants from the 2002 survey were sent questionnaire for the treatment survey. The first mailing to the new patients was sent on November 9, 2002. A second mailing to non-respondents was sent on January 2, 2003. A total of 651 out 957 (68.0%) cases in the "new patient" sample completed and returned the treatment questionnaires by the end of the field period.

The 2002 Treatment Experiences report was based on the first 1,186 completed questionnaires from the "old patient" and "new patient" samples. This included 535 patients who currently use IVIG, who were first identified in the baseline 1996 survey. It also includes 651 patients currently using IVIG who were first identified in the 2002

survey. The findings from the 2002 survey have been widely used by patients, health providers, third party payers and manufacturers to understand treatment patterns and outcomes in this population.

Treatment Experiences Survey of IG Therapy Users: 2008

There have been a number of developments since 2002 that are likely to have affected the pattern of treatment for patients with primary immunodeficiency diseases. First, since the earlier treatment survey, subcutaneous immunoglobulin has emerged as a treatment option with a licensed product in the United States. Second, changes to Medicare have affected reimbursement for IVIG, which has had an impact on site of service and access to treatment for Medicare patients. Third, there is anecdotal evidence that reimbursement for IVIG has become more difficult from private third-party payers as well. Fourth, standards of care guidelines for the treatment of primary immunodeficiency disease have been developed and disseminated within the specialist community. Finally, changes in the size and demographics of the PIDD population may have affected the patterns of treatment in that community. Consequently, IDF conducted a Third Treatment Experiences Survey among patients with PIDD in 2008.

A sampling frame for the Third Treatment Survey was constructed from the IDF patient database. The Foundation constructed a listing of persons identified as users of IG therapy in previous IDF surveys or new patients that had been identified as IgG users in the database. Next, all non-patients, deceased patients and non-US addresses were removed from this listing, which was then matched with the National Change of Address database to update addresses where possible. Finally, 2,500 potential respondents were selected from this sampling frame through random case selection. The sample size for the survey was selected to ensure a minimum sample size of 1,000 completed interviews, equivalent to the achieved sample for the 2002 Treatment Survey.

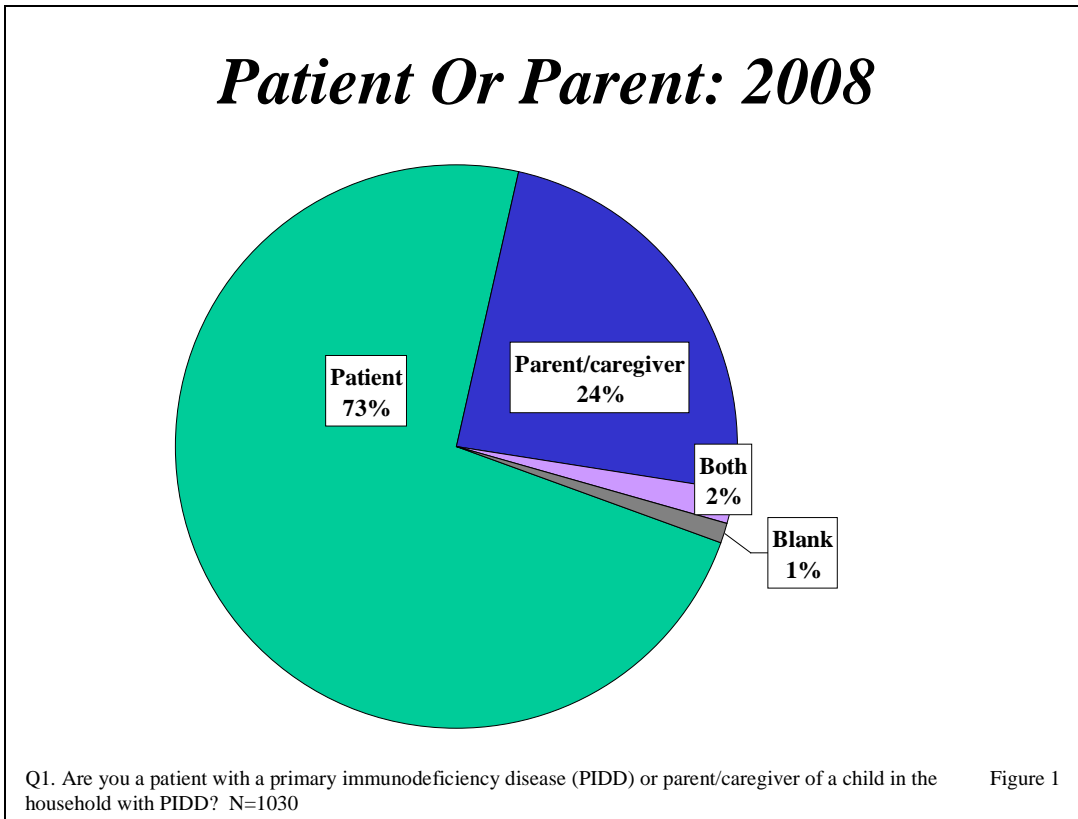
An eight-page questionnaire with a cover letter was initially mailed to the sample of 2,500 persons between July 9th and August 20, 2008. The package included a postage-paid business reply envelope for respondents to return the completed questionnaire. A second mailing to non-respondents began on a rolling basis on August 26, 2008. A total of 1,088 questionnaires were returned (43.5% response rate). From that total, 13 cases were eliminated for being neither a patient nor parent/caretaker of a person with PIDD, 7 cases were eliminated due to a diagnosis other than PIDD, and 38 cases were eliminated for having never used IG therapy. This resulted in a total of 1,030 cases in the sample, which form the basis for this report.

Characteristics of Patients with PIDD in the 2008 Treatment Survey

The population for the Treatment Survey was restricted to those patients with primary immunodeficiency diseases who have been treated with immunoglobulin therapy. Immunoglobulin is the medically recommended treatment for some, but not all primary immunodeficiency diseases. In the 1996 national survey of nearly 3,000 patients

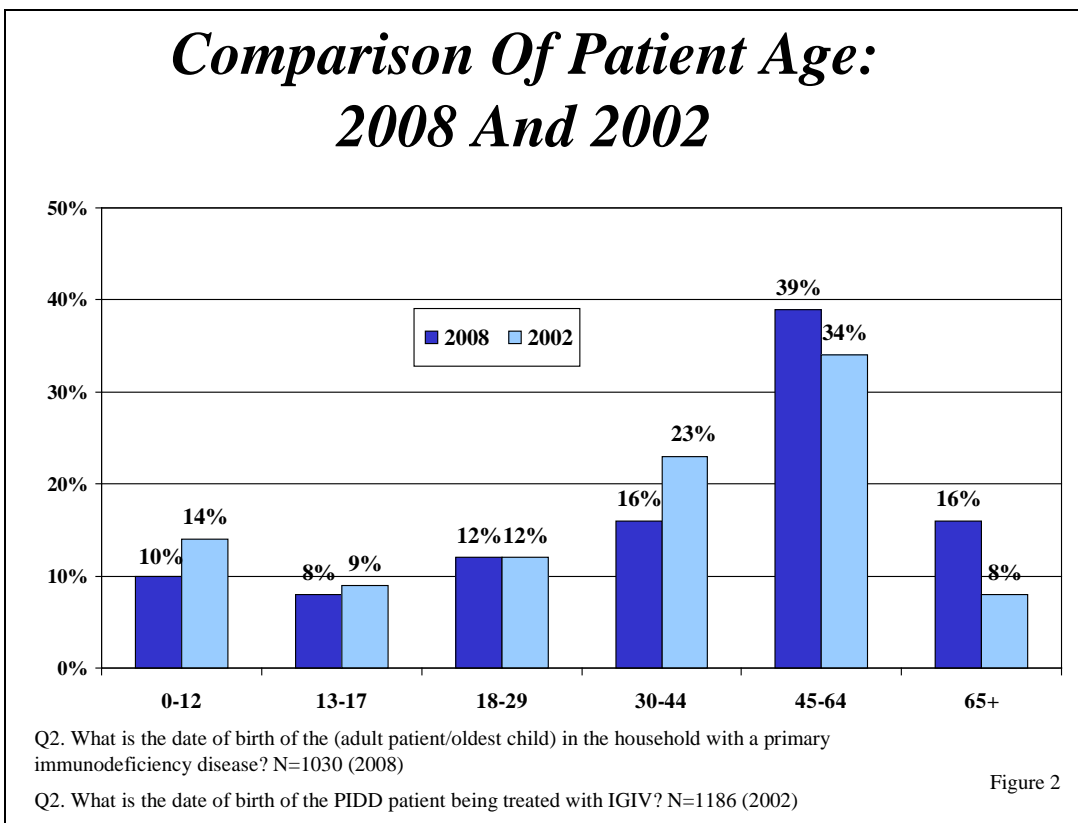
with primary immunodeficiency diseases, 70% reported that they had been treated with IVIG. In the Third National Patient Survey in 2007, 83% reported that they had ever been treated regularly with IVIG or SCIG.

Nearly three-quarters (73%) of respondents in the 2008 Treatment Survey were Patients with PIDD. Only 24% were either patients or caregivers of a patient with PIDD, and just 2% were both a patient and the caregiver/parent of a patient (Figure 1).



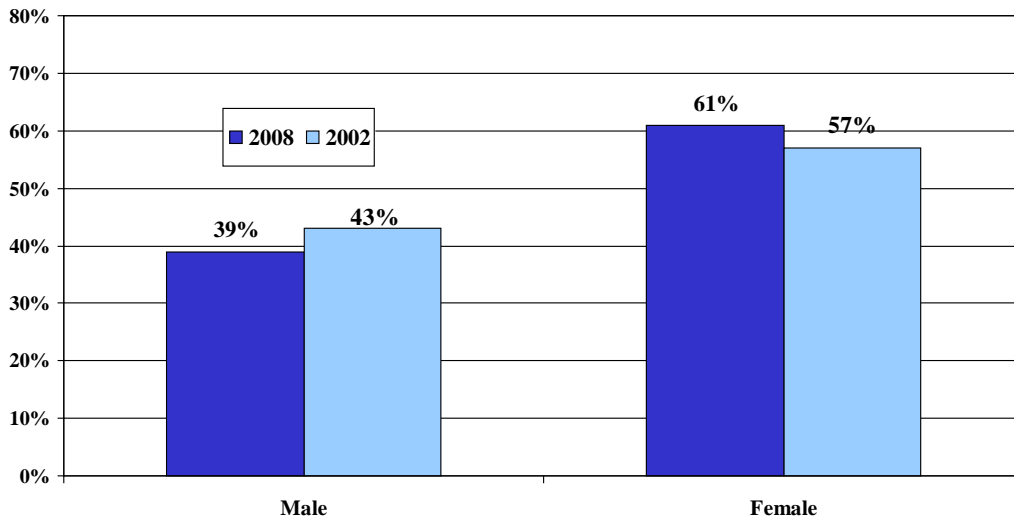
Similar to the findings of the 2002 report, primary immunodeficiency diseases are no longer a pediatric condition. Only one-in-ten patients (10%) were aged 12 or younger in 2008, compared to 14% in 2002. Eight percent were adolescents, aged thirteen to seventeen in 2008, compared to 9% in 2002. In total, only 18% of patients in the 2008 Treatment Survey were under the age of 18. Six years ago, in the 2002 study, 23% of patients were under 18 (Figure 2).

Twelve percent are young adults, aged 18 to 29, and 16% of the patient population is 30 to 44 years old. Nearly forty percent of patients (39%) are middle aged, 45 to 64 years old, and, sixteen percent (16%) of Patients with PIDD that have been treated with immunoglobulin therapy in 2008 are aged 65 or older. In 2002, only 8% of PIDD current users of IVIG were aged 65 and older (Figure 2).



In the 2008 treatment survey, 39% of respondents were male and 61% were female. There was a slightly higher percentage of males in the 2002 survey (43%) and a slightly lower percentage (57%) of females (Figure 3). There are, however, dramatic differences across age groups. Among respondents under 18, 71% were male, and just 29% were female. Among respondents over 18, the ratio of males to females drops to 33% male, 67% female.

Comparison Of Patient Gender: 2008 And 2002



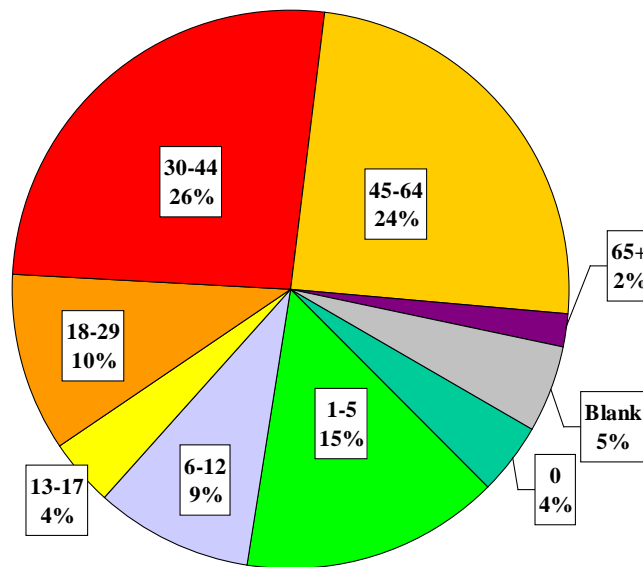
Q3. What is the gender of that person (adult patient/oldest child with PIDD)? N=1030 (2008)

Q7. What is the gender of that person? N=1,165 (2002)

Figure 3

One-third of respondents in the Treatment Survey (32%) had been diagnosed with a primary immunodeficiency disease before the age of 18. Four percent of respondents had been diagnosed before the age of 1, 15% were diagnosed between ages 1 and 5, 9% between ages 6 and 12, and 4% between ages 13 and 17. Another 10% were diagnosed as young adults, between the ages of 18 and 29. Just over one quarter (26%) were diagnosed between ages 30 and 44, and another 24% were diagnosed between ages 45 and 64. Only 2% of respondents in the Treatment Survey were diagnosed as having a primary immunodeficiency disease at age 65 or older (Figure 4).

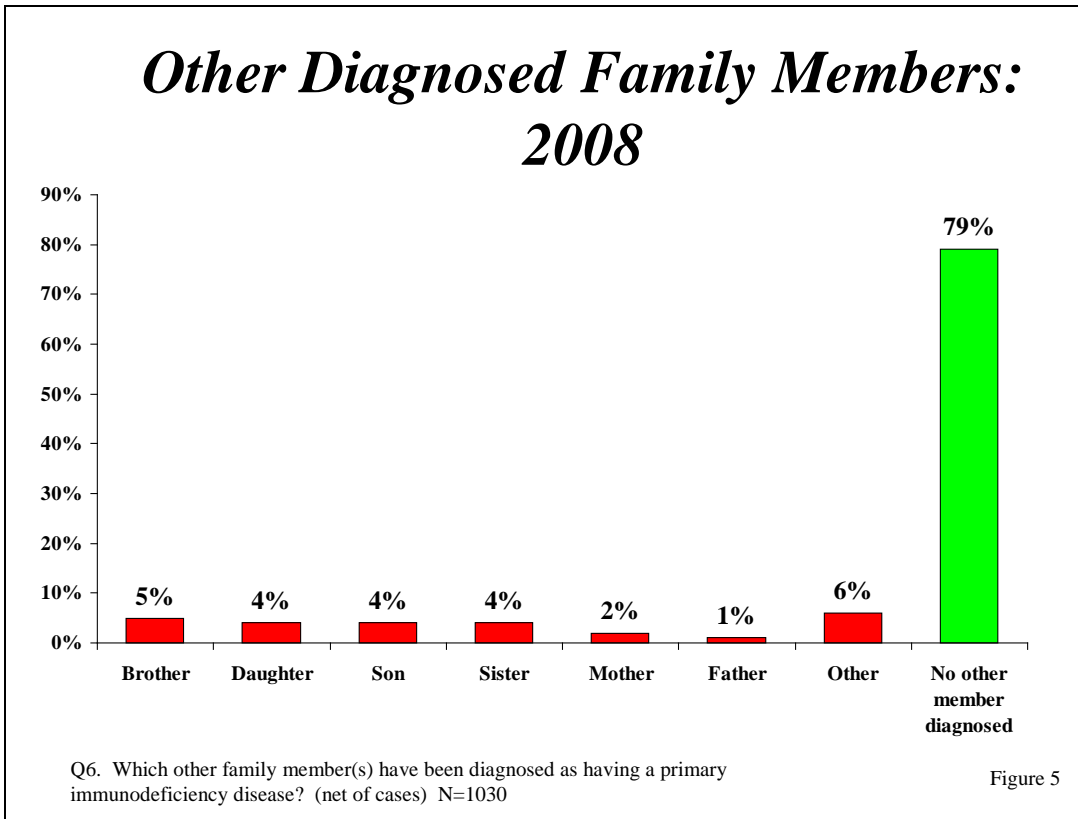
Patient Age At Diagnosis: 2008



Q4. At what age (in years) was that person first diagnosed with a primary immunodeficiency disease? N=1030

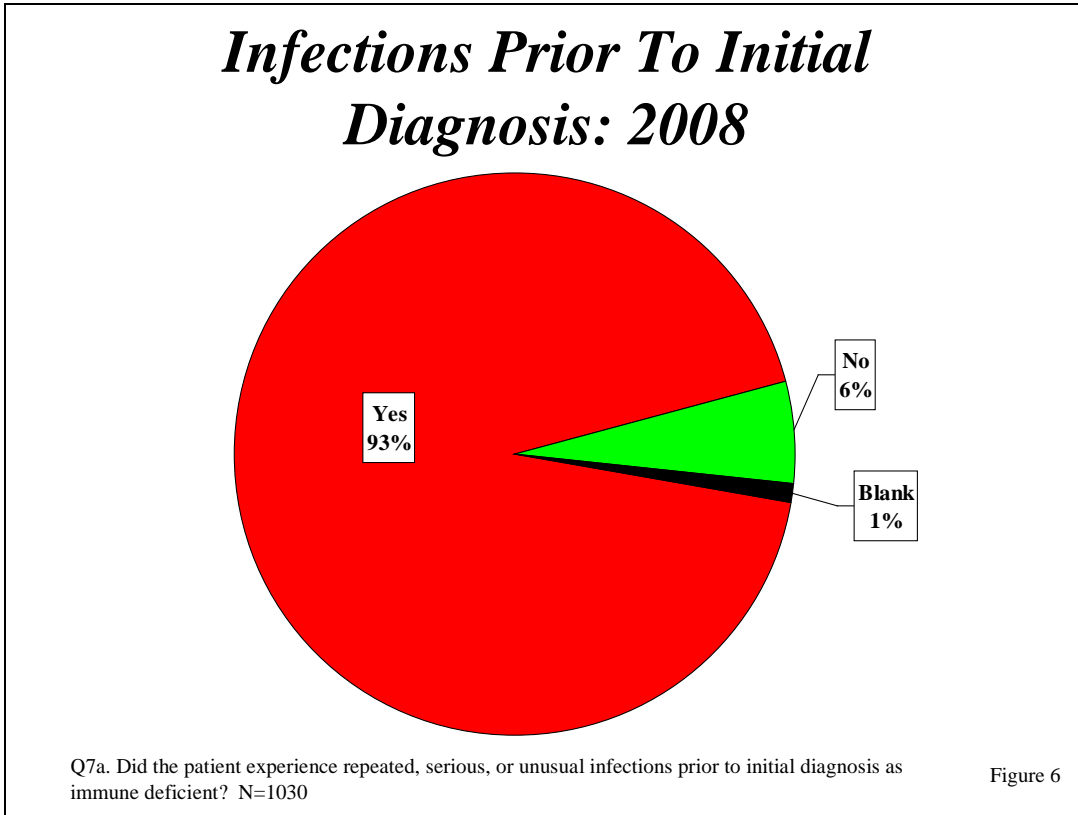
Figure 4

Most respondents (79%) had no family history of primary immunodeficiency disease (Figure 5). However, about one in five of these Patients with PIDD did have other family members with the disease, including 5% having a diagnosed brother, 4% having a diagnosed daughter, son or sister, 2% with a diagnosed mother and 1% with a diagnosed father. Another 6% had some other family member that had been diagnosed with a primary immunodeficiency disease.



Health Before Diagnosis

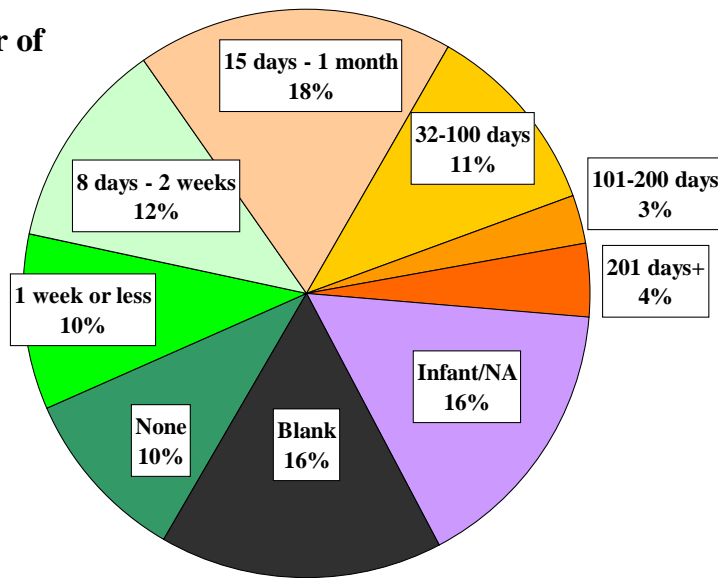
Although the vast majority of patients with PIDD have no family history of immune deficiency diseases, almost all experienced repeated, serious or unusual infections prior to diagnosis. More than nine in ten Patients with PIDD (93%) that have been treated with immunoglobulin therapy reported experiencing repeated, serious, or unusual infections prior to diagnosis (Figure 6). Only 6% reported no serious or repeated infections prior to diagnosis.



Only 10% of patients missed no days of work or school in the 12 months prior to diagnosis. Another 10% missed 1 week or less, 12% missed between 1 and 2 weeks, 18% missed between two weeks and 1 month, 11% missed between 32 and 100 days, and another 7% missed over 100 days (Figure 7). Excluding those who were too young to go to work or school (16%) and those who did not answer the question (16%), the survey found that Patients with PIDD were too sick to attend work or school or perform their usual activities an average of 36.8 days during the 12 months prior to diagnosis.

Days Too Sick To Attend Work/School In The 12 Months Prior To Diagnosis: 2008

Mean number of days 36.8

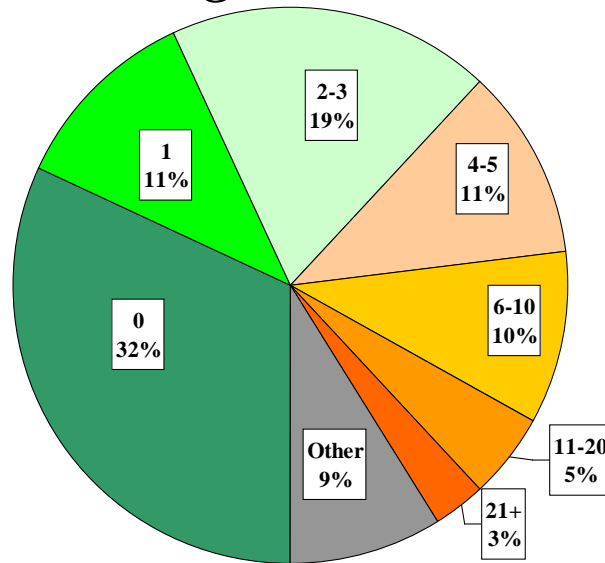


Q18. Not counting hospitalizations, how many days was he/she too sick to work, go to school or perform usual activities in the 12 months prior to diagnosis? N=1030

Figure 7

Only a little less than one-third of patients with PIDD (32%) reported that they were never hospitalized before diagnosis as immune deficient. Another 11% were hospitalized only once prior to diagnosis, while 19% were hospitalized 2-3 times. By contrast, 11% of patients with PIDD were hospitalized 4-5 times before diagnosis, and 10% were hospitalized 6-10 times. Another 5% reported 11-20 hospitalizations, and 3% reported over 20 hospitalizations before being diagnosed as immune deficient (Figure 8). The cost of undiagnosed primary immunodeficiency to patients, the health care system and society as a whole is staggering, just in terms of hospitalizations.

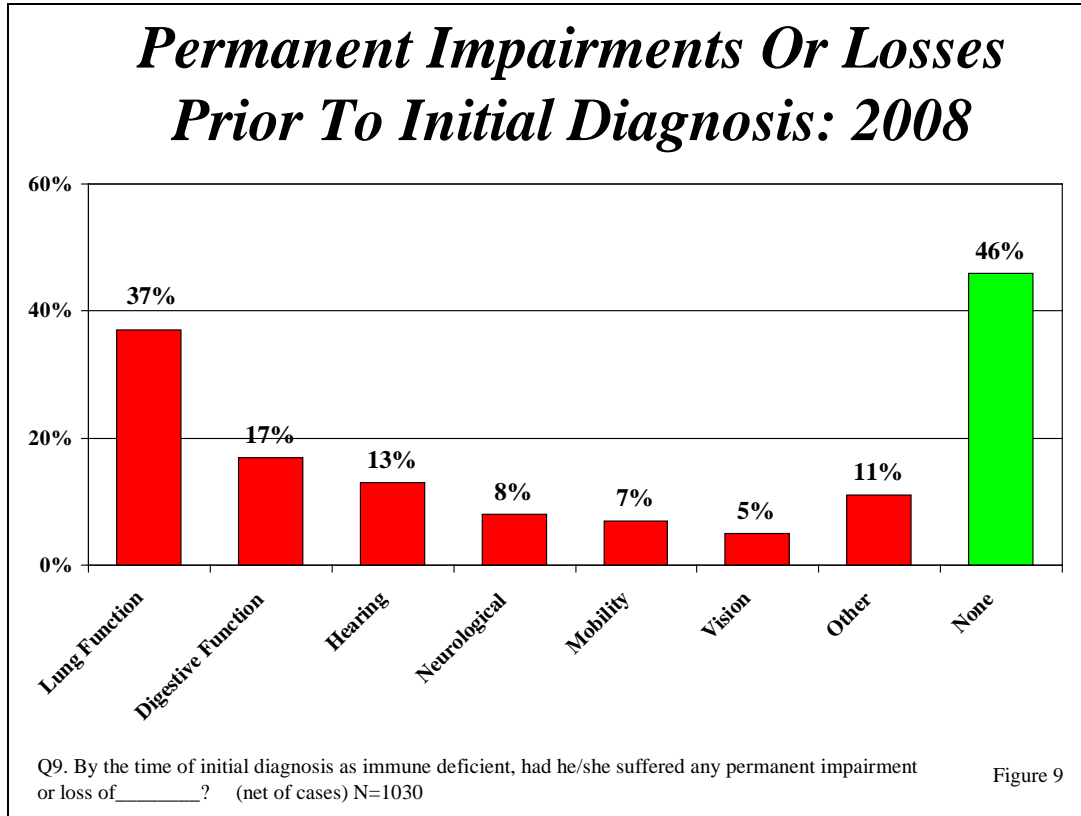
Times Hospitalized Prior To Diagnosis: 2008



Q8. How many times had he/she been hospitalized before diagnosis as immune deficient? N=1030

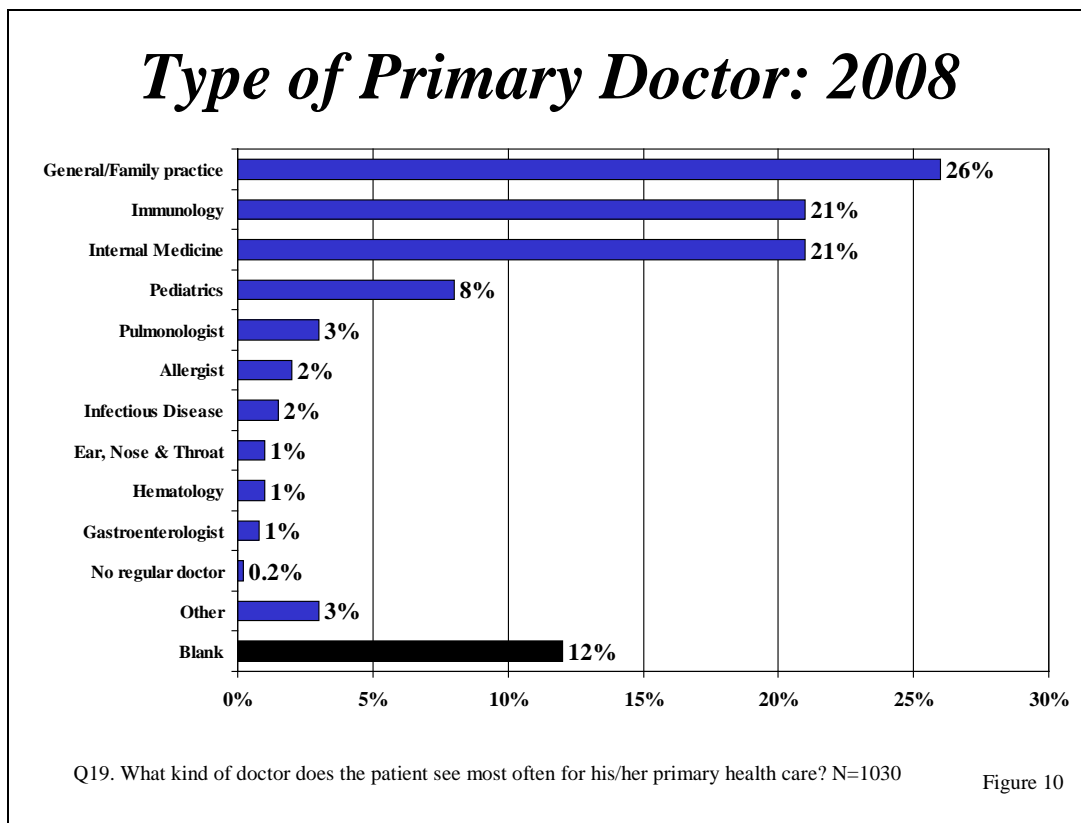
Figure 8

As a result of late diagnosis, and repeated and severe infections prior to diagnosis, over one half of patients with PIDD reported at least some permanent impairment before their diagnosis as immune deficient. Nearly two out of five patients with PIDD reported permanent impairment of lung function (37%) prior to diagnosis. Additionally, 17% had permanent impairment to digestive function, 13% hearing loss or impairment, 8% neurological impairment, 7% loss of mobility, and 5% had either vision loss or impairment. Another 11% had some other permanent impairment or loss prior to diagnosis as immune deficient (Figure 9).

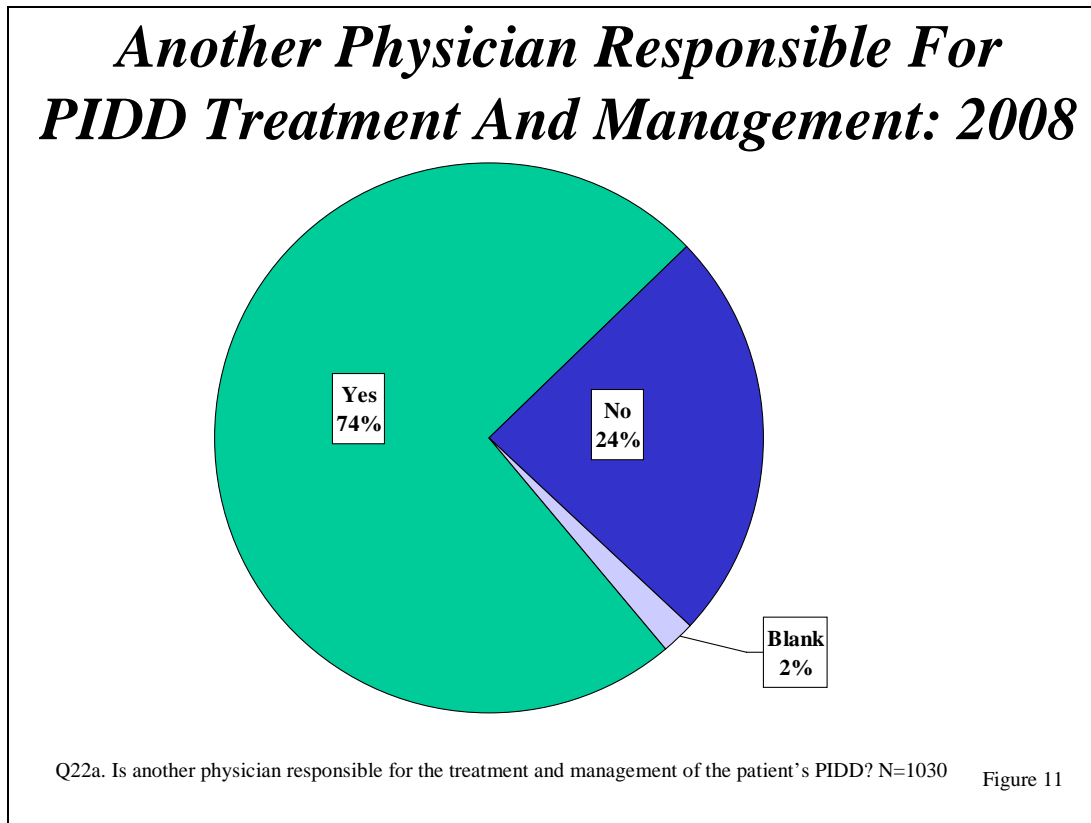


Current Sources of Care and Health Care Coverage

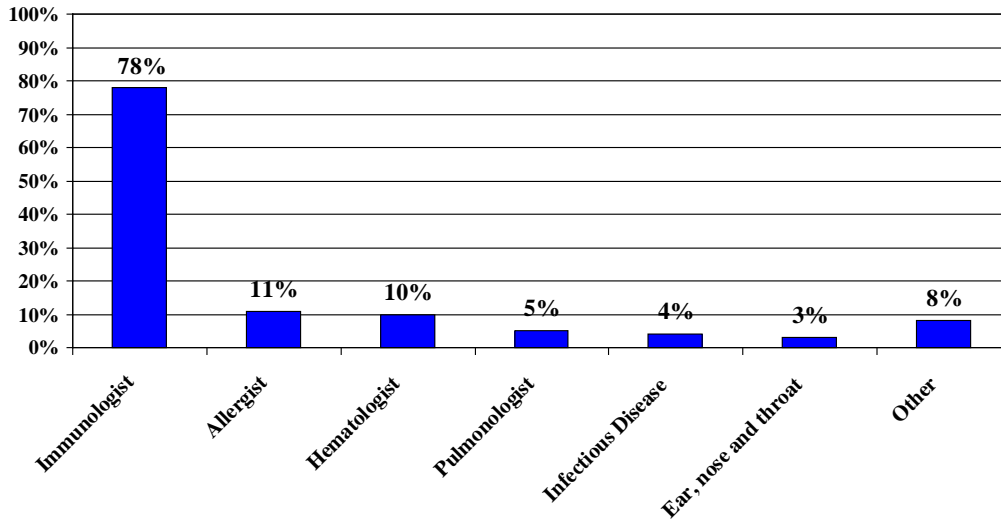
The majority of patients with primary immunodeficiency diseases who have been treated with immunoglobulin therapy see a primary care doctor most often for their health care, not a specialist. More than half of patients reported that the doctor they see most often for their health care is in family practice (26%), internal medicine (21%) or pediatrics (8%). However, one-in-five (21%) reported that they see an immunologist most often for their primary health care. Only a minority reported seeing other specialists most often for their health care, including 3% who see a pulmonologist as their primary doctor, 2% use an allergist, 1% each say they see an ear, nose and throat specialist (ENT) or hematologist, and 3% reported seeing an other type of doctor most often for their health care (Figure 10).



However, only a quarter of patients with PIDD (24%) said that they did not see another physician for the treatment and management of their immune deficiency condition. Three-quarters of these patients with PIDD (74%) reported that there was another physician responsible for the treatment and management of their PIDD (Figure 11). Among patients who had another doctor who was responsible for treatment and management of their primary immunodeficiency disease, over three-quarters (78%) said they saw an immunologist to treat their PIDD. In addition, 11% saw an allergist, 10% a hematologist, 5% a pulmonologist, 4% an infectious disease doctor, and 3% saw an ENT specialist. Another 8% had another type of doctor to treat their PIDD (Figure 12). Hence, while most patients with PIDD that have used immunoglobulin therapy rely on a primary care doctor as their main source of health care, the vast majority report also having a specialist responsible for the management and treatment of their PIDD condition.



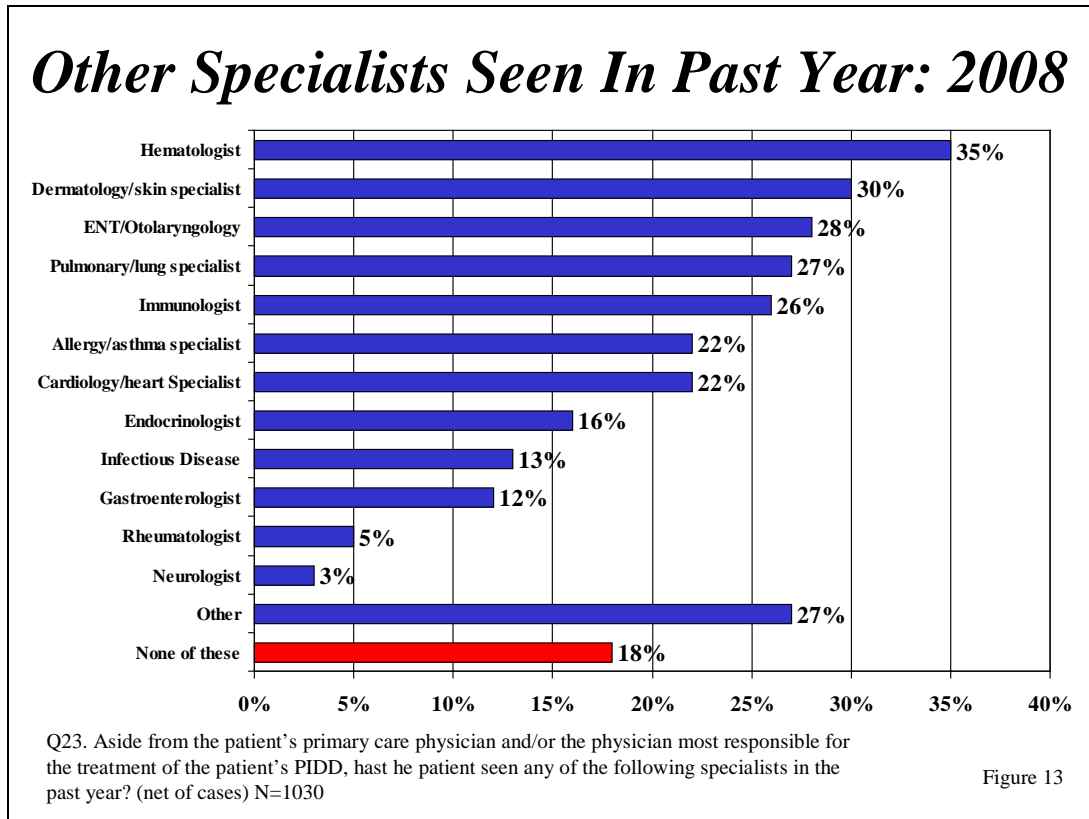
Type Of Doctor Responsible For PIDD Treatment And Management: 2008



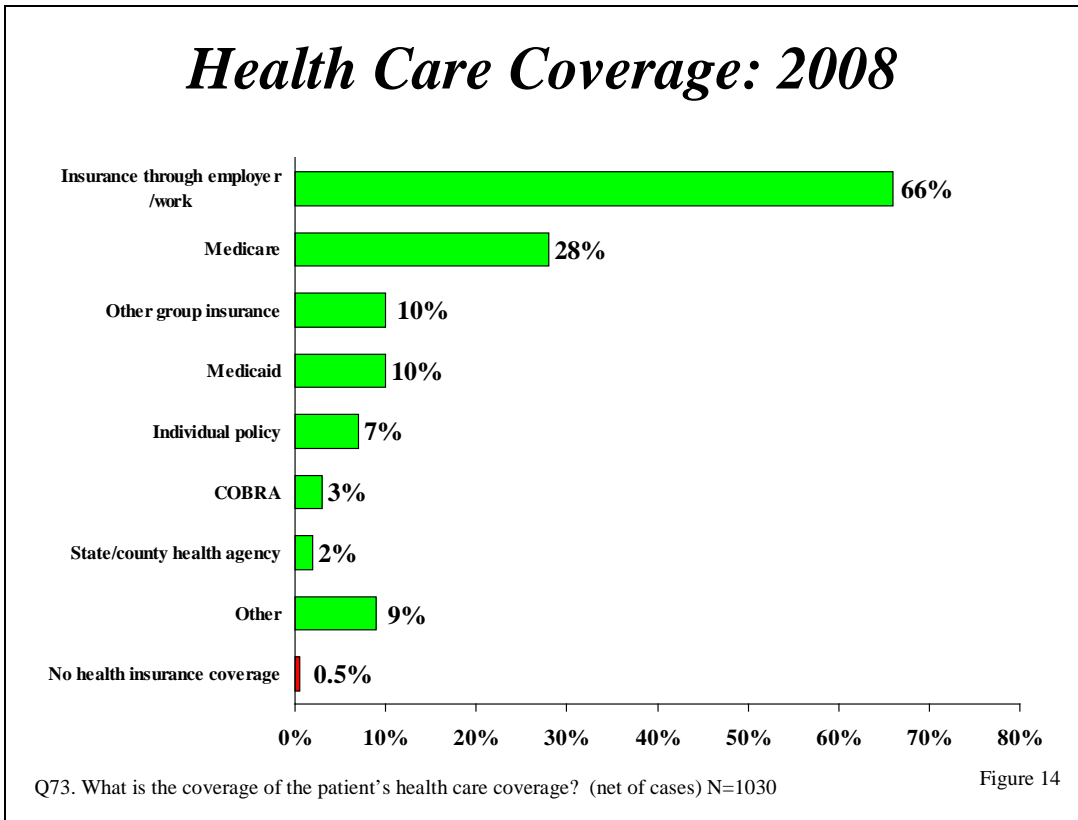
Q22b. Which type of physician is responsible for the treatment and management of the patient's PIDD?
BASE: Those who have a doctor other than the primary doctor to treat PIDD (net of cases) N=761

Figure 12

Moreover, in addition to their primary doctor and the doctor most responsible for the treatment of their immune deficiency disease, more than four out of five patients with PIDD (82%) saw at least one other specialist during the past year. Between one-quarter and one third of patients with PIDD saw a hematologist (35%), dermatologist (30%), ENT specialist (28%) pulmonologist (27%), or an immunologist (26%) other than their primary care doctor and the one responsible for the management of their condition. Twenty-two percent saw another allergist or cardiologist. One in seven patients with PIDD (16%) have seen an endocrinologist in the past year, while others report seeing an infectious disease specialist (13%), gastroenterologist (12%), rheumatologist (5%) or neurologist (3%) in the past year. Only 18% of patients saw none of these specialists unless they were their primary care doctor or the one responsible for the treatment of their immune deficiency disease (Figure 13).

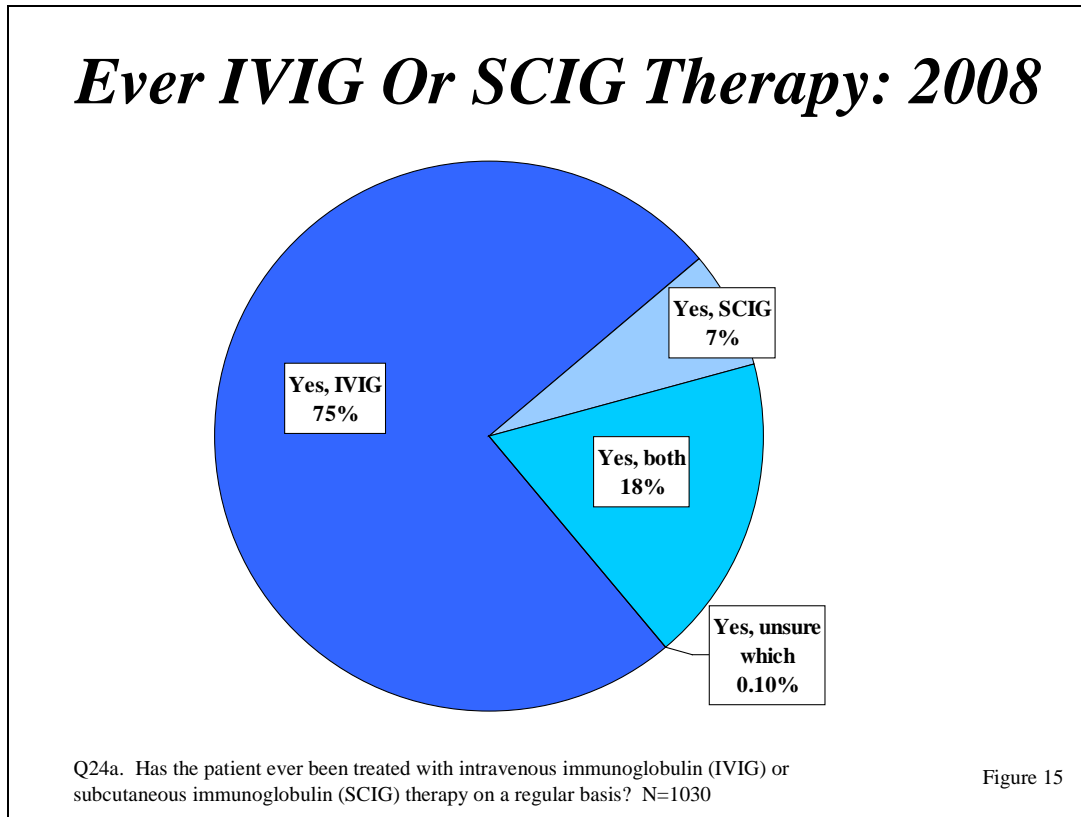


Almost all persons with primary immunodeficiency diseases being treated with IgG had some form of health insurance coverage. The majority (66%) had insurance through an employer group policy. Ten percent reported health care coverage through a non-employer group policy, while 7% had an individual policy. More than a quarter of the Patients with PIDD in this sample (28%) had Medicare coverage, while 10% had Medicaid coverage, and 2% were covered by a state or county health program. Three percent had health coverage through COBRA. Nine percent reported other forms of coverage, such as military or veterans. Less than one percent reported no health care coverage through any of these sources (Figure 14).

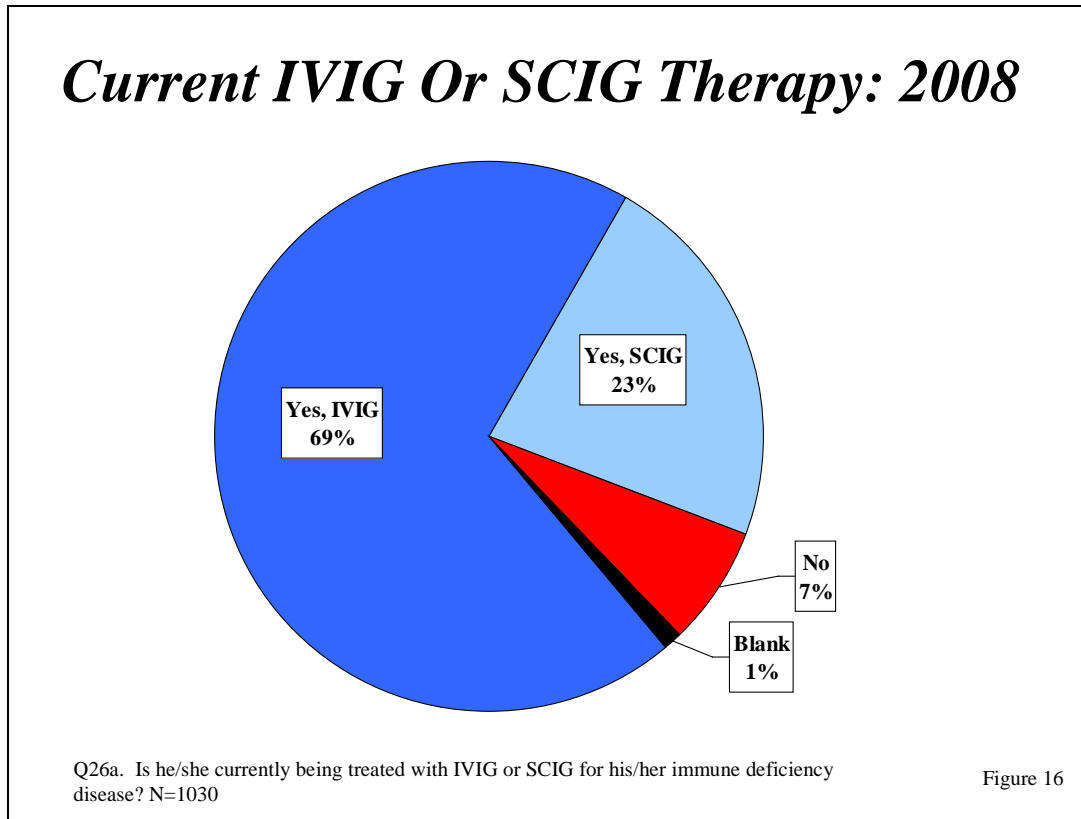


Treatment with IG Therapy

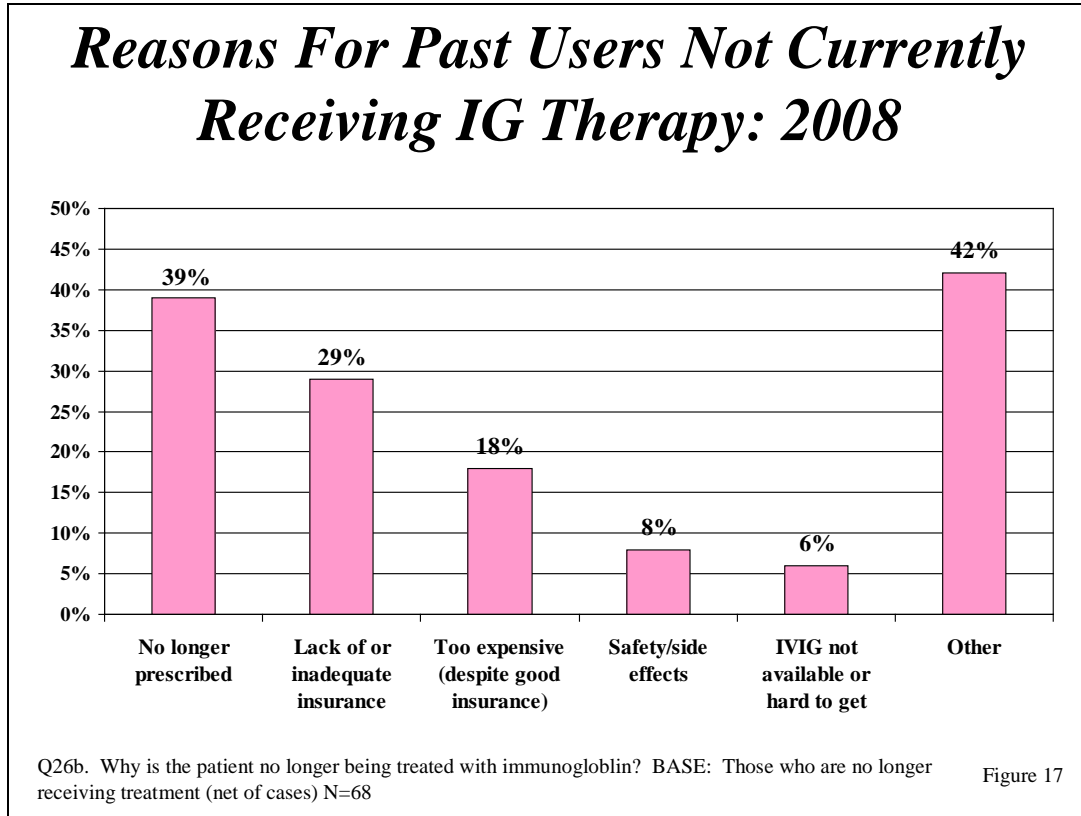
The Treatment Survey is restricted to patients with primary immunodeficiency diseases who have ever been treated with either IVIG (intravenous immunoglobulin) or SCIG (subcutaneous immunoglobulin). Almost all of these patients (93%) have been treated with IVIG, including those who have only been treated with IVIG (75%) and another 18% who have been treated with both IVIG and SCIG. A quarter of this sample has been treated with SCIG, including 7% who have only been treated with SCIG and another 18% who have been treated with both IVIG and SCIG (Figure 15). It should be noted that the proportion of SCIG users in the sample should not be used as a market estimate since the patients with PIDD in the IDF database are more likely to be exposed to SCIG as a treatment option either through the IDF or through the major medical centers from which many of the IDF connected patients are drawn.



Nearly seven in ten patients with PIDD (69%) in the treatment sample are currently using IVIG. Another 23% of the patients in the sample are currently using SCIG. Seven percent of respondents who have been treated in the past with either IVIG or SCIG are no longer using IG therapy (Figure 16). Once again, these proportions are more likely to reflect the nature of the patients with PIDD known to IDF, rather than true market share. However, the treatment survey provides very useful subsample sizes of IVIG users, SCIG users, and former IG therapy users for our analysis.

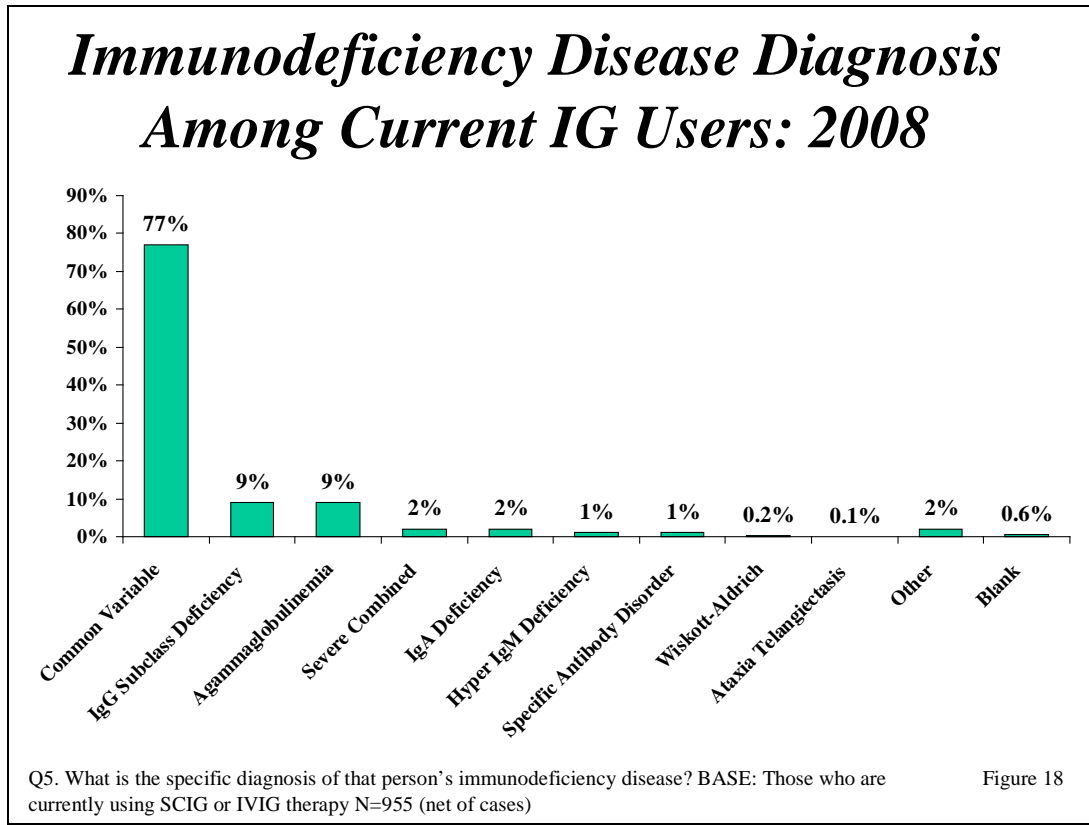


Among patients that are no longer being treated with IG therapy, nearly two in five (39%) report that IG therapy is no longer prescribed for them. Of greater concern, 29% of patients with PIDD who have discontinued immunoglobulin therapy had to stop due to a lack of insurance or inadequate insurance, and 18% claimed it was too expensive despite good insurance. For 8% of those who have stopped, safety issues or side effects were a concern, and for 6% availability was a problem. Forty-two percent of patients no longer using IG therapy reported other or additional issues as the reason why they have stopped (Figure 17).

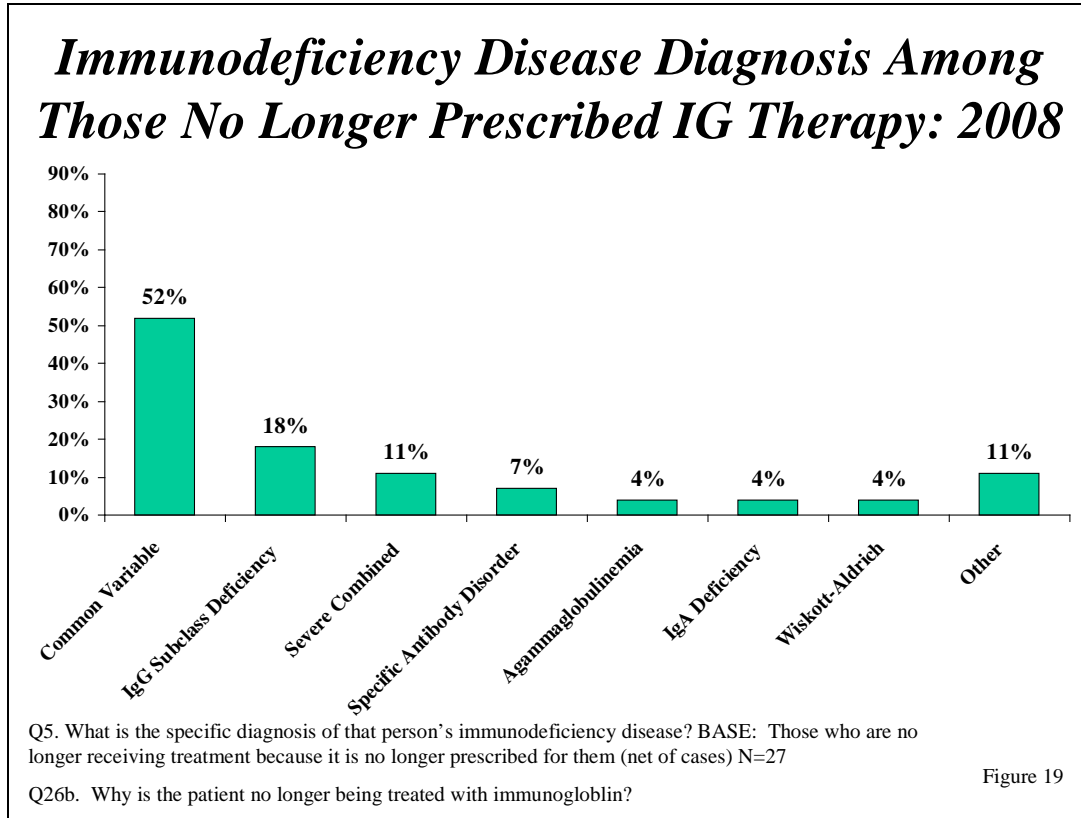


The appropriateness for IG therapy depends on the specific diagnosis of primary immunodeficiency disease. The current IDF Clinical Care Guidelines for Patients with Primary Immunodeficiency Diseases recommend treatment with immunoglobulin for Agammaglobulinemia, Common variable immunodeficiency (CVID), Hyper IgM, Wiskott-Aldrich, and Severe combined immunodeficiency (SCID). Specific antibody deficiency, which may be a part of IgG subclass deficiency, is also indicated, although IgG subclass deficiency alone is not.

Among this national sample of patients with primary immunodeficiency diseases who were currently using either as IVIG or SCIG, over three-quarters (77%) reported that their current diagnosis is CVID. Another 9% reported a diagnosis of Agammaglobulinemia. There were also small proportions of patients with SCID (2%), IgA deficiency (2%), Hyper IgM syndrome (1%) or Specific antibody disorder (1%). Only 9% of the current IG therapy users had a diagnosis of IgG subclass deficiency. The remaining IG therapy users (3%) had another diagnosis (Figure 18). Hence, about nine out of ten current IG therapy users among patients with primary immunodeficiency diseases in this sample had a diagnosis for which IG therapy is the current standard for care.

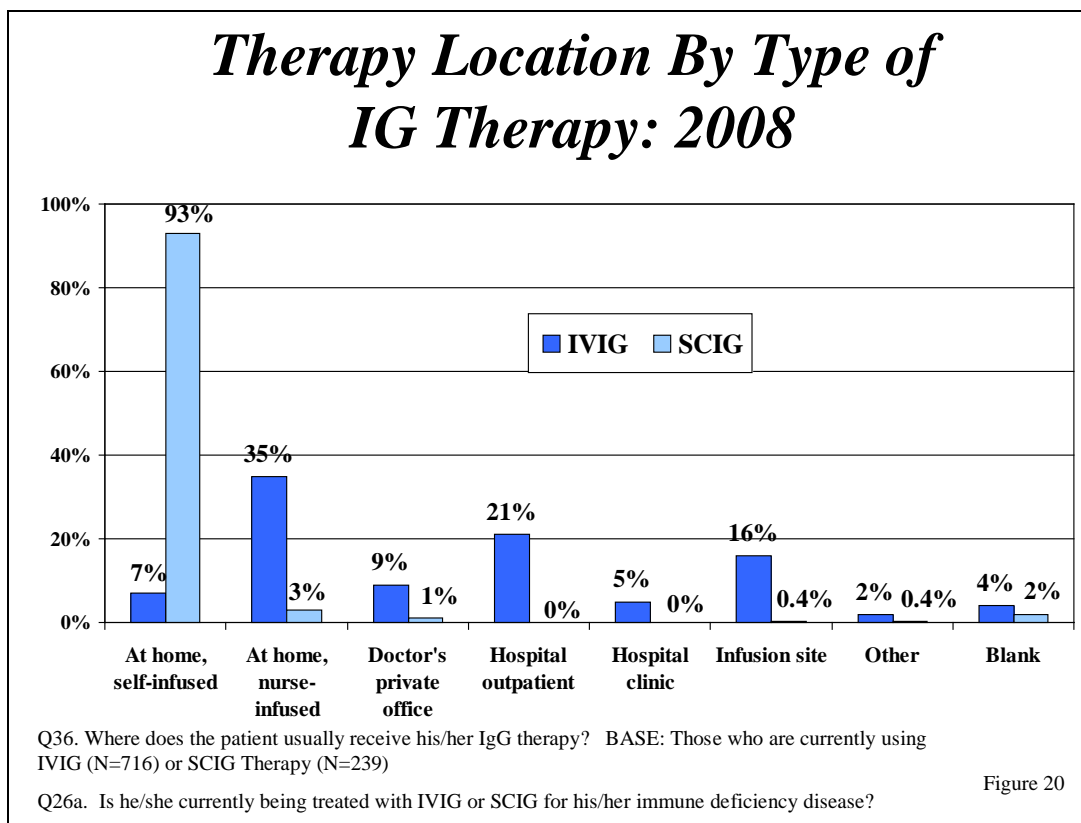


Despite the appropriateness of this treatment, over half of the respondents that had discontinued immunoglobulin therapy because it was no longer prescribed for them had a diagnosis of CVID (52%), while others were diagnosed with SCID, Agammaglobulinemia, IgA Deficiency, Specific antibody disorder, and Wiskott-Aldrich (Figure 19).



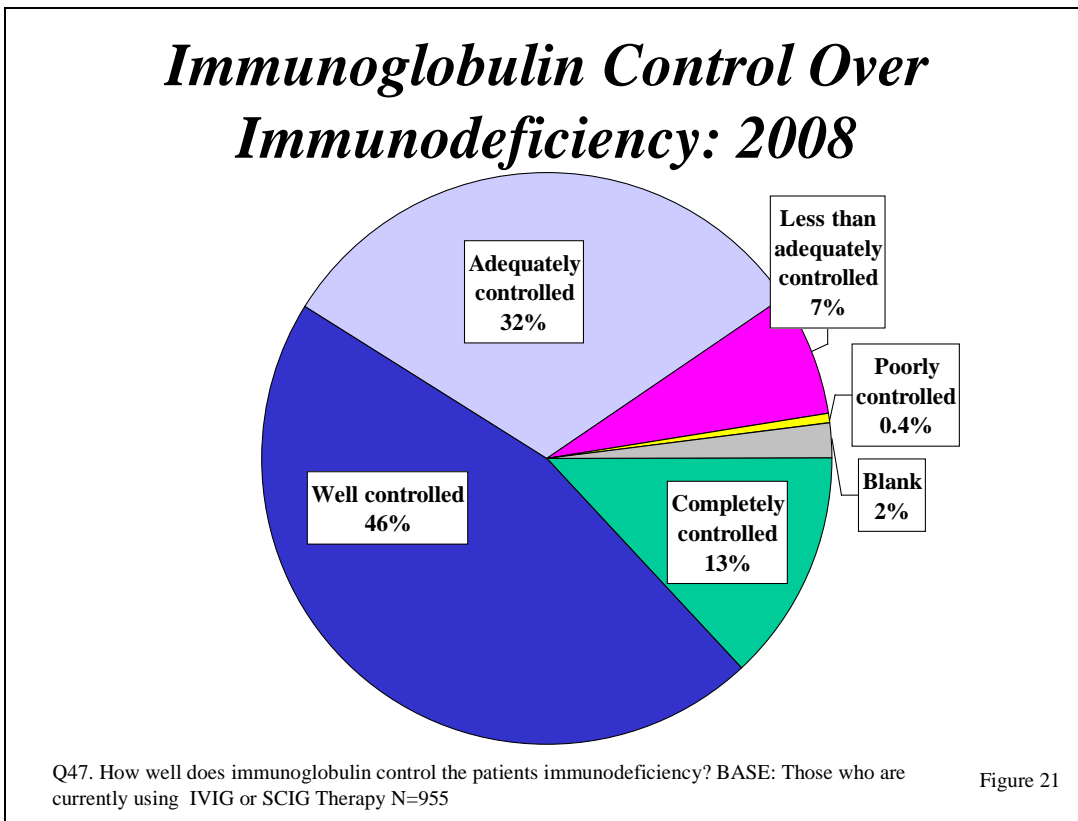
Two out of five (42%) IVIG users reported that they usually received their infusion at home. Of those, 7% were able to self-infuse, while the other 35% had a nurse perform the infusion. Twenty-six percent of IVIG users usually got their infusion at a hospital outpatient department (21%), or at a hospital clinic (5%). Most of the remainder said that they usually got their infusion in a doctor's private office (9%) or an infusion suite (16%). (Figure 20)

Nearly all SCIG users (93%) received their treatment at home, and were able to self-infuse. Of the remaining SCIG users, 3% also received their treatment at home, with a nurse performing the infusion, or at a doctor's private office (1%). (Figure 20)

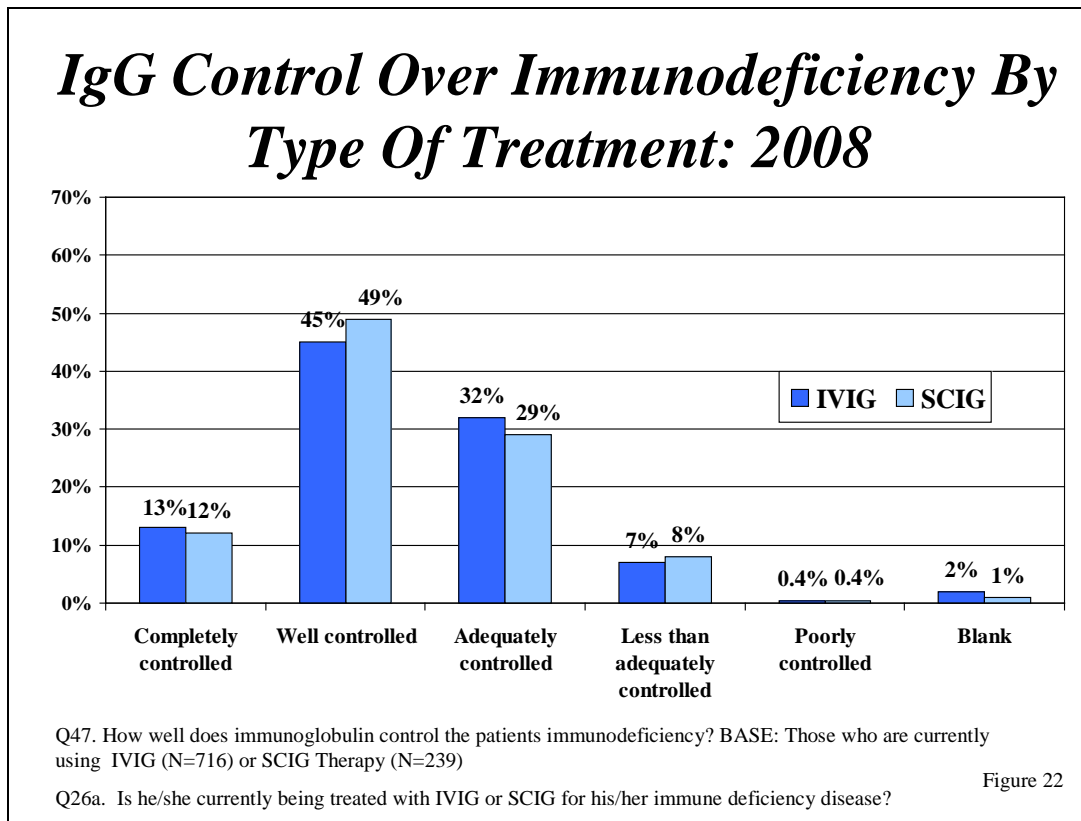


Product Effectiveness, Safety and Satisfaction

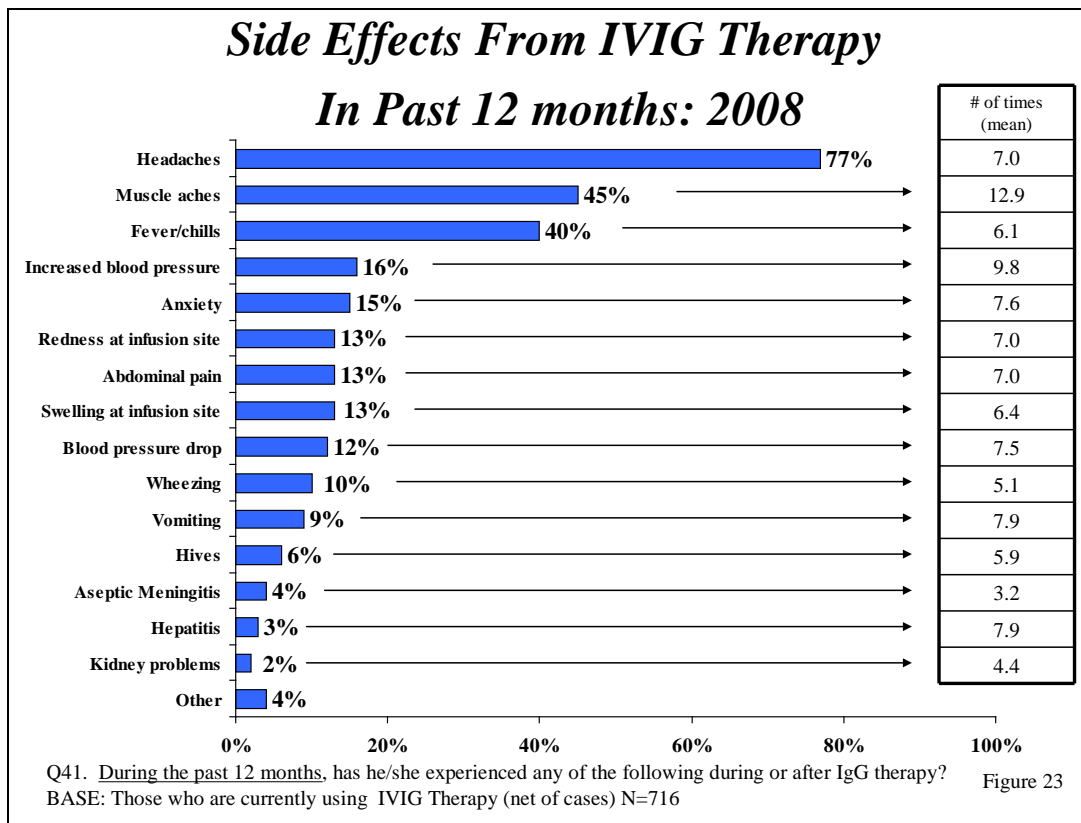
The vast majority of patients with PIDD on IG therapy are satisfied with their product’s effectiveness in controlling their immune deficiency. Nearly three out of five patients say that their immunodeficiency is either completely controlled (13%) or well controlled (46%) by their immunoglobulin. Another third of patients with PIDD (32%) feel their immunodeficiency is adequately controlled by IG therapy. Only 7% of patients with PIDD who are currently on IG therapy say that the therapy provides less than adequate control of their immunodeficiency, while less than 1% say it is poorly controlled (Figure 21).



There is no significant difference between users of IVIG and SCIG in perceived effectiveness of the product in controlling their immune deficiency. The survey finds that 13% of IVIG users and 12% of SCIG users say their treatment completely controls their PIDD; while another 45% of IVIG users and 49% of SCIG users say their immunodeficiency is well controlled by the treatment; and 32% of IVIG users and 29% of SCIG users feel their condition is adequately controlled by their treatment. By contrast, only seven percent of IVIG users and 8% of SCIG users feel that their immune deficiency is less than adequately controlled by their treatment, while almost none (0.04%), regardless of treatment type, think that their immunodeficiency is poorly controlled by their treatment (Figure 22).

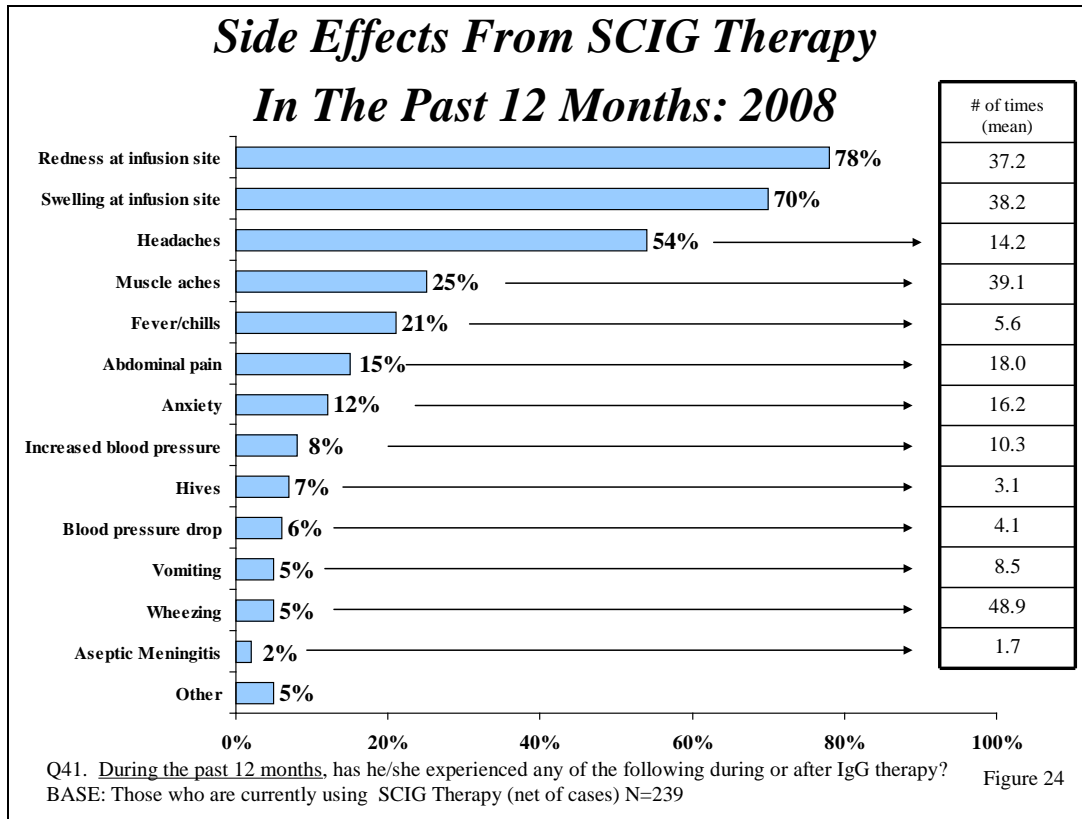


Many patients with PIDD have experienced side effects either during or after their IVIG or SCIG treatment. Among IVIG users, more than three quarters (77%) reported headaches during or after IG therapy. At least two out of five IVIG users reported experiencing muscle aches (45%) or fever/chills (40%) during or after IG therapy in the past 12 months. Less commonly, IVIG users also reported increased blood pressure (16%), anxiety (15%), redness at infusion site (13%), abdominal pain (13%), swelling at the infusion site (13%), blood pressure drops (12%) or wheezing (10%) during or after treatment in the past 12 months. A smaller proportion of IVIG users experienced vomiting (9%), hives (6%), aseptic meningitis (4%), hepatitis (3%) or kidney problems (2%) during or after their IVIG in the past 12 months (Figure 23).



Of these side effects, muscle aches were felt the most often in the past 12 months (12.9 times on average), followed by increased blood pressure (9.8 times on average).

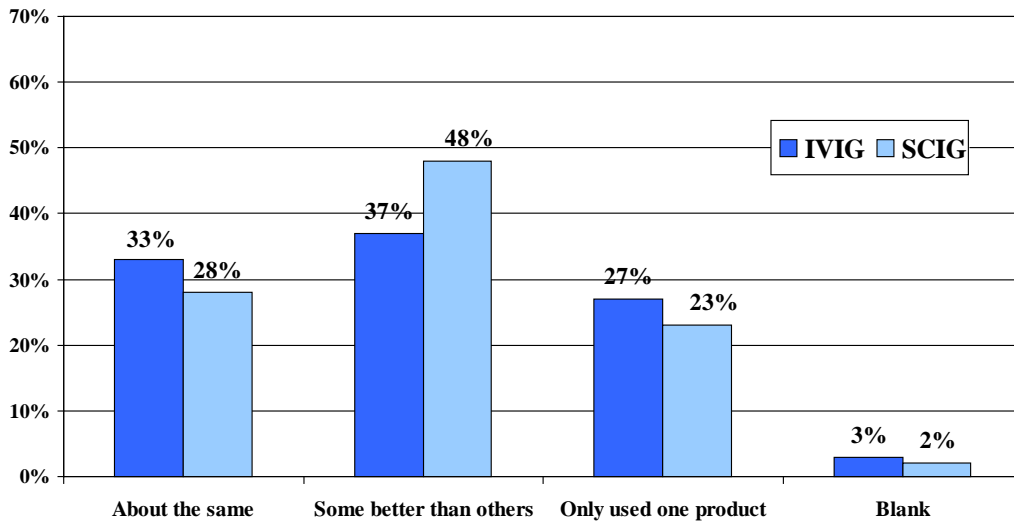
For SCIG users, the most commonly experienced symptoms during or after IG therapy in the past 12 months was redness (78%) or swelling (70%) at the infusion site. They were also the symptoms felt most often (37.2 and 38.2 times on average) during the past 12 months. In addition, 54% experienced headaches, 25% experienced muscle aches, and 21% experienced fever/chills during or after treatment in the past 12 months. SCIG users also reported abdominal pains (15%), anxiety (12%), increased blood pressure (8%), hives (7%), blood pressure drops (6%), vomiting (5%), wheezing (5%) and aseptic meningitis (2%) during or after treatment. (Figure 24)



Wheezing and muscle aches, though experienced by only a small percentage of SCIG users, were experienced frequently by those who had them. On average, muscle aches were experienced 39.1 times, and wheezing was experienced 48.9 times.

Just one-third of IVIG users (33%) and 28% of SCIG users report that they tolerate all immunoglobulin products similarly. Nearly half of SCIG users (48%) and 37% of IVIG users reported that they tolerate some immunoglobulin products better than others. Another 27% of IVIG users and 23% of SCIG users have only used one product so they could not evaluate their tolerance of different products (Figure 25).

Tolerance Of Immunoglobulin Products By Type Of Treatment: 2008



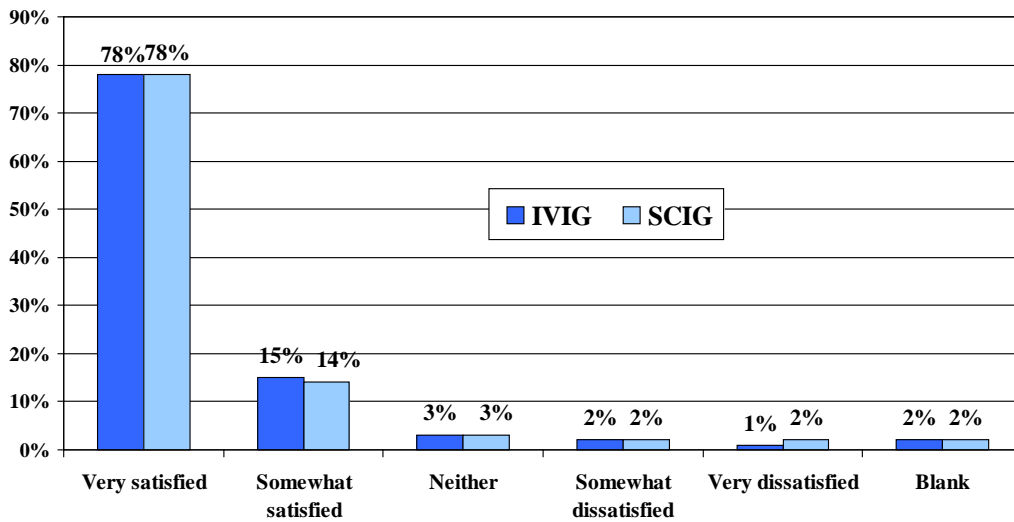
Q49. Does he/she tolerate any immunoglobulin products better than others, or are they all about the same? BASE: Those who are currently using IVIG (N=716) or SCIG therapy (N=239)

Q26a. Is he/she currently being treated with IVIG or SCIG for his/her immune deficiency disease?

Figure 25

Overall, nearly all patients with PIDD being treated with IG therapy are at least somewhat satisfied with their IG therapy product. Seventy-eight percent of respondents, regardless of treatment type, are very satisfied with their current IG therapy product. Another 15% of IVIG users and 14% of SCIG users are somewhat satisfied. Three percent of patients say that they are neither satisfied nor dissatisfied with the product that they are currently using. By comparison, only two percent of IVIG and SCIG users are somewhat dissatisfied with their current product, while just 1% of IVIG users and 2% of SCIG users are very dissatisfied with their current IG therapy product (Figure 26).

Satisfaction With Current IgG Product By Type Of Treatment: 2008



Q59. How satisfied is the patient with the IgG product currently being used? BASE: Those who are currently using IVIG (N=716) or SCIG therapy (N=239)

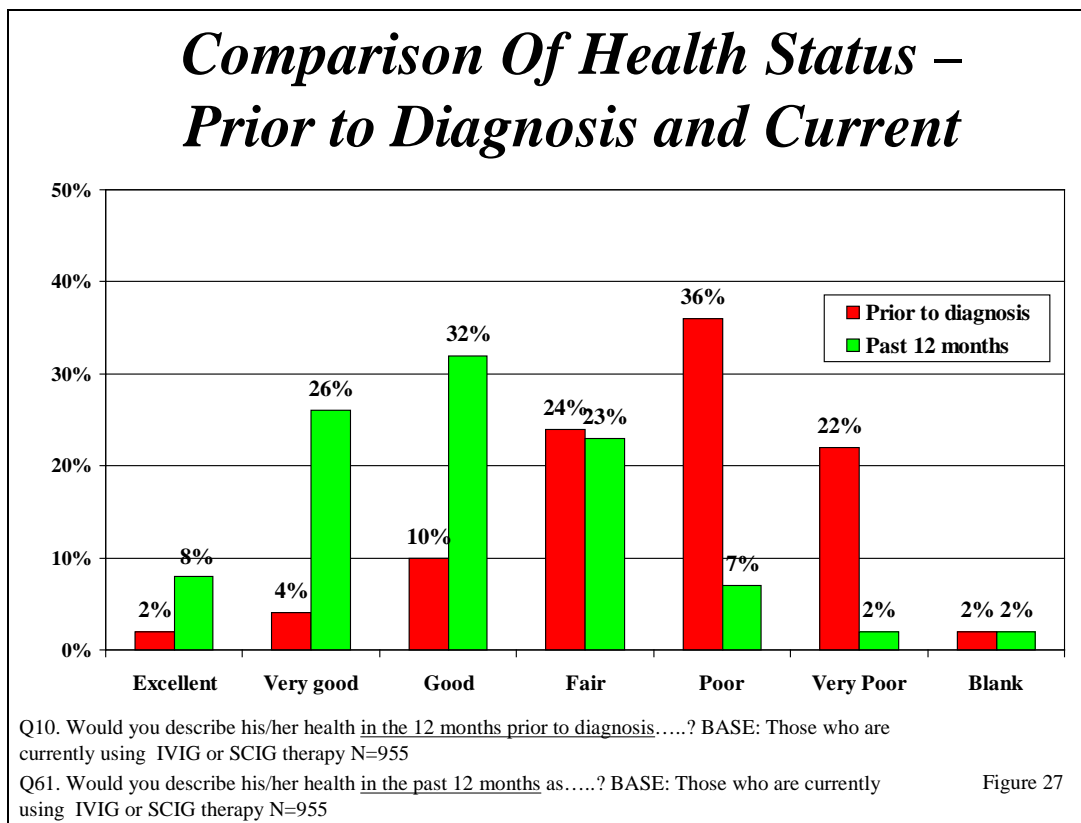
Q26a. Is he/she currently being treated with IVIG or SCIG for his/her immune deficiency disease?

Figure 26

Efficacy of Treatment

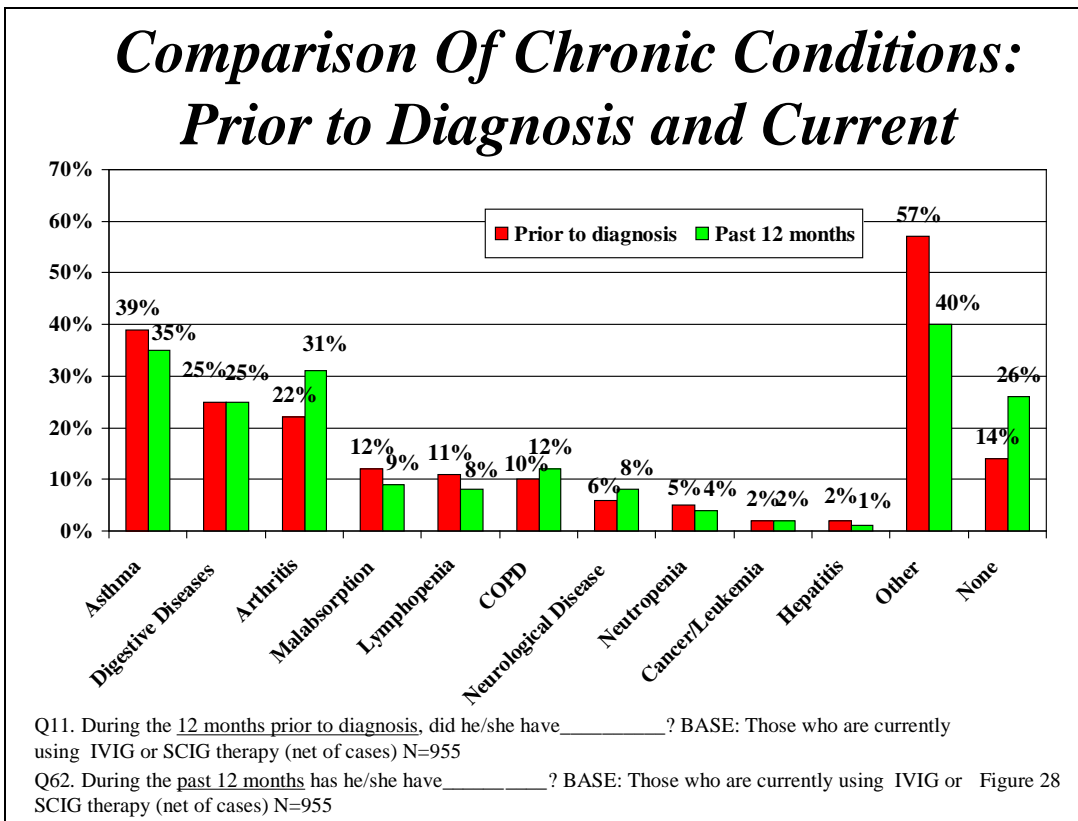
To best measure the efficacy of IG therapy among patients, a series of questions were asked to gauge the health of the patient in the twelve months prior to diagnosis and in the most recent twelve months. The comparison in health status provides the most compelling evidence of the efficacy of IG therapy to control PIDD, and demonstrates the improvement in the health and well-being of the patient since treatment has begun.

Prior to diagnosis, only one-in-seven patients with PIDD who currently use IVIG or SCIG (16%) described their health status as excellent (2%), very good (4%) or good (10%). Indeed, one-quarter (24%) would describe their health status prior to diagnosis as just fair. More than half of patients with PIDD described their health status as either poor (36%) or very poor (22%) in the 12 months prior to diagnosis (Figure 27).

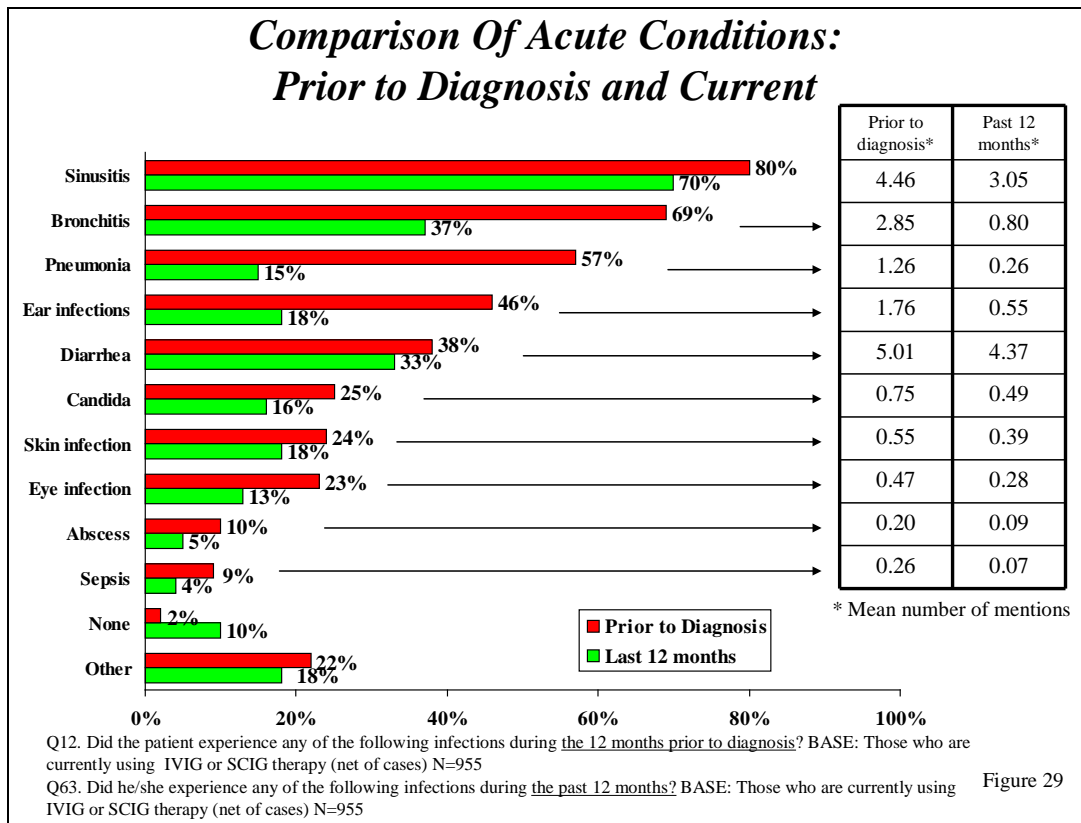


However, in the most recent twelve months, the same patients with PIDD are now describing their health status in a much more positive way. Two-thirds of patients with PIDD currently using IG therapy describe their health in the past 12 months as excellent (8%) very good (26%) or good (32%). Just 23% would describe their recent health status as fair. By contrast, less than one-in-ten current IgG users say their health status over the past twelve months has been poor (7%) or very poor (2%).

One reason that most patients with PIDD describe their health as good or better in the past 12 months, compared to the 12 months prior to treatment, are a lower rate of chronic diseases. These patients reported a decrease in many chronic conditions, including a decrease in asthma (39% prior to diagnosis, 35% recently), malabsorption (12% prior, 9% recent), Lymphopenia (11% prior, 8% recent), Neutropenia (5% prior, 4% recent), and Hepatitis (2% prior, 1% recent). There is also a decrease in the rate of other chronic diseases not described in the survey (57% prior, 40% recently). By contrast, there has been no change in the rate of digestive diseases (25%) or Cancer/Leukemia (2%), while there has been a slight increase in some age-related conditions including arthritis (22% prior to diagnosis, 31% recently), COPD (10% prior, 12% recently), and neurological disease (6% prior, 8% recently). Overall, the percentage of patients with PIDD that reported no chronic conditions nearly doubled, from 14% in the 12 months before diagnosis to 26% in the past 12 months (Figure 28).

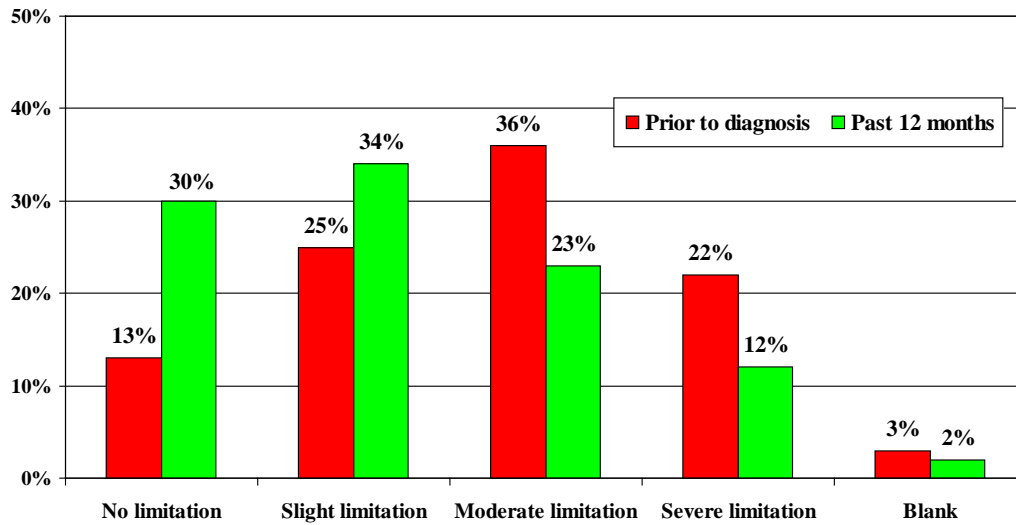


There is also a dramatic decrease in the rate of acute conditions before diagnosis compared to the past 12 months among patients with PIDD currently on IG therapy. IG therapy users are much less likely to suffer from pneumonia (57% prior to diagnosis, 15% recently), ear infections (46% prior, 18% recently), and bronchitis (69% prior, 37% recently). There is also a significant decrease in sinusitis (80% experienced prior to diagnosis, 70% in past 12 months). The proportion of patients who have experienced diarrhea, Candida, skin infections, eye infections, abscess and sepsis have also decreased, but not quite as dramatically. However, the average number of incidents of these infections has dropped even more dramatically. The proportion of patients with PIDD being treated with IG therapy that report no acute conditions increased from 2% prior to diagnosis to 10% in the past 12 months (Figure 29).



As a result of reduced chronic and acute conditions, current IgG patients have seen a significant decrease in their physical limitations between the time before they were diagnosed and the past twelve months. In the year prior to diagnosis, a majority of IgG users reported moderate (36%) or severe (22%) limitations in their work, play or normal physical activity as a result of their health. One-quarter (25%) reported only slight limitations, and only 13% reported no limitations. In the past year, however, IgG users are far less limited in their activities. Nearly two-thirds of current users reported no limitation (30%) or slight limitations (34%) in their lives. Just 23% reported moderate limitations, and only 12% reported severe limitations (Figure 30).

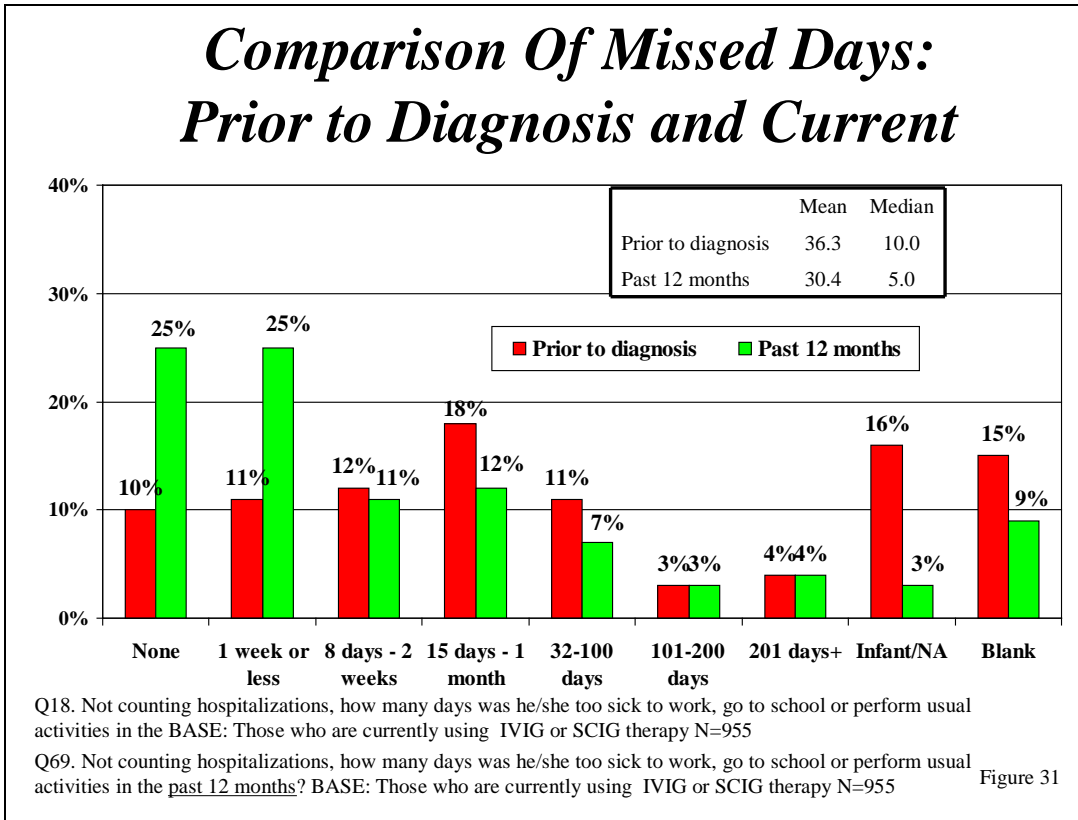
Comparison Of Limitations: Prior to Diagnosis and Current



Q13. 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? BASE: Those who are currently using IVIG or SCIG therapy N=955
 Q64. 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: Those who are currently using IVIG or SCIG therapy N=955

Figure 30

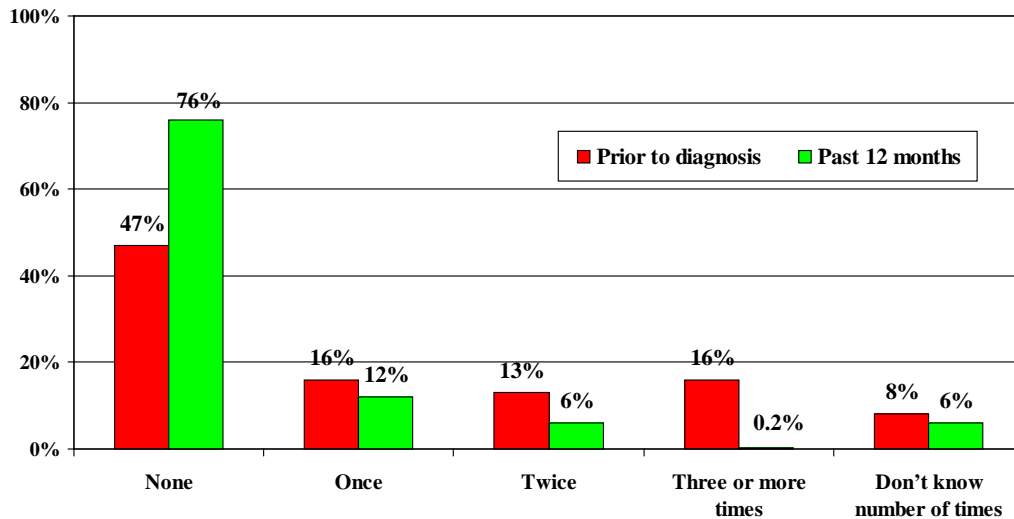
As a result of better health, there was a decline in the number of days the patient missed from school or work. The average number of days missed by a current IgG user in the twelve months prior to diagnosis was 36.3 days. In the past twelve months, that number dropped to 30.4 days. More dramatically, the median number of missed days from work or school as been cut in half from the 12 months prior to diagnosis (10.0) and the past 12 months (5.0) (Figure 31).



One half of respondents missed either no school or work (25%) or less than one week (25%) in the past 12 months. In the 12 months prior to diagnosis, only 10% did not miss any work or school, and 11% missed less than one week.

Current IG therapy users have also spent less time in the hospital in the past twelve months. A majority of IG users (53%) were hospitalized at least once in the twelve months prior to diagnosis. Sixteen percent were hospitalized once, 13% were hospitalized twice, 16% were hospitalized three or more times and an additional 8% couldn't recall the number of times they were hospitalized. Only 47% reported no hospitalizations (Figure 32). In the most recent twelve months, over three-quarters of IG users (76%) were not hospitalized at all.

Comparison Of Times Hospitalized: Prior to Diagnosis and Current



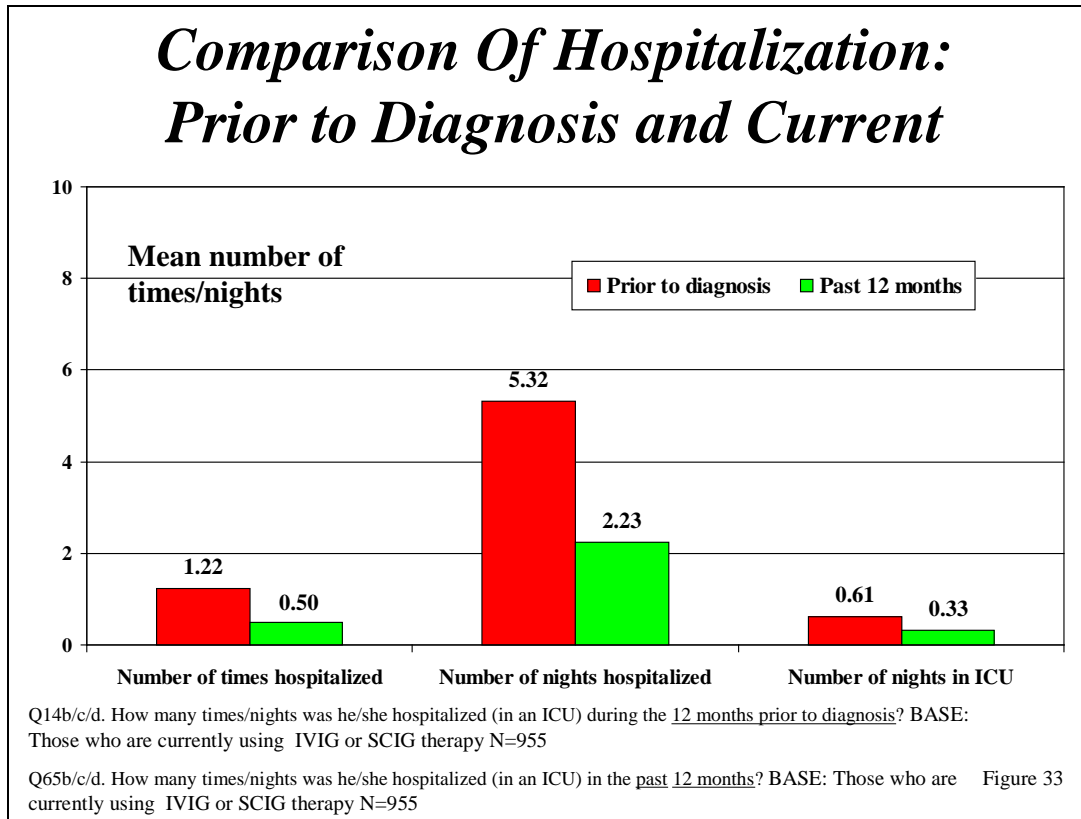
Q14b. How many times was he/she hospitalized in the 12 months prior to diagnosis? BASE: Those who are currently using IVIG or SCIG therapy N=955

Q65b. How many times was he/she hospitalized in the past 12 months? BASE: Those who are currently using IVIG or SCIG therapy N=955

Figure 32

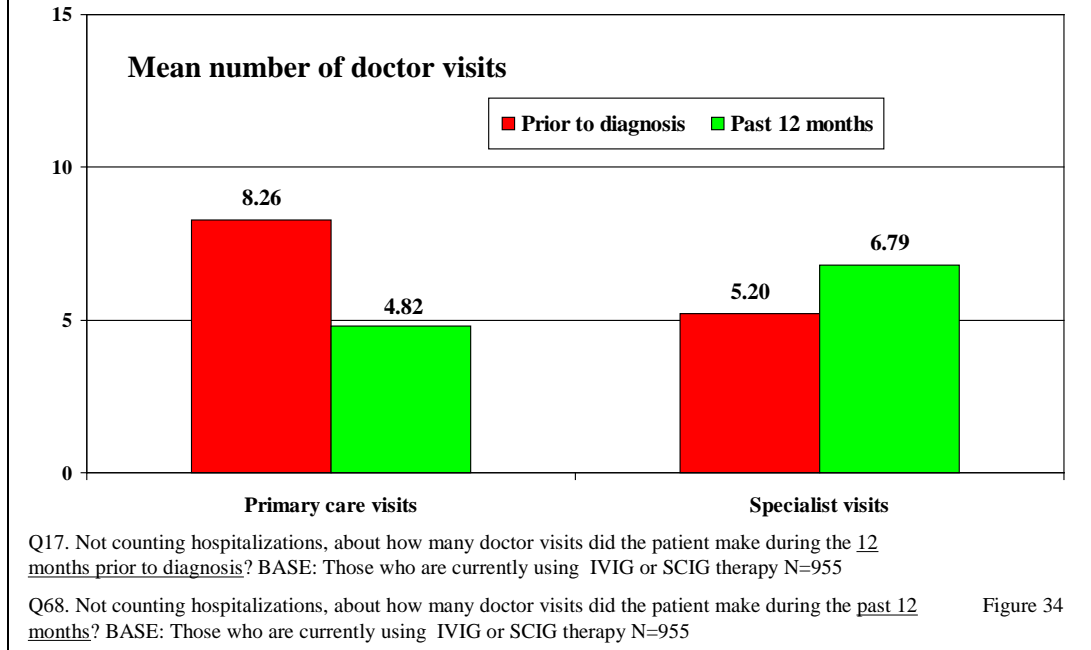
On average, patients on IG therapy were hospitalized 1.22 times in the twelve months prior to diagnosis. During the most recent year, these same patients were hospitalized an average of 0.50 times. In the 12 months prior to diagnosis, they spent an average of 5.32 nights in the hospital, compared to just 2.23 nights in the hospital in the past 12 months. In the year before diagnosis, these patients spent an average of 0.61 nights in the ICU compared to 0.33 nights in the ICU in the past 12 months (Figure 33). Hence, the improved health found in patients with PIDD after treatment with IG therapy has a significant impact on health care costs associated with hospitalization.

Comparison Of Hospitalization: Prior to Diagnosis and Current

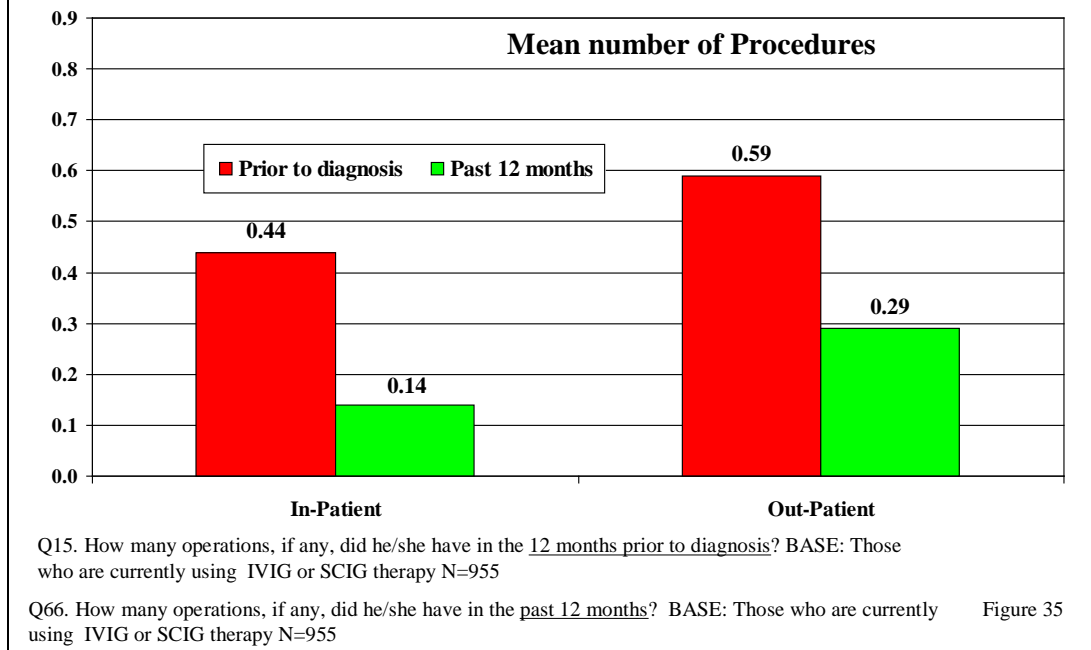


There was also a decrease in the number of doctor’s visits and surgical procedures for IgG users. Prior to diagnosis, IG users had an average of 8.26 visits to their primary care physician and 5.20 visits to specialists. In the past 12 months, IG users made only 4.82 visits to their primary care physician and 6.79 visits to specialists (Figure 34). Furthermore, the number of operations that IG users had to have also decreased with IG therapy use. Prior to diagnosis, IG users underwent an average of 0.44 in-patient and 0.59 out-patient procedures. In the past 12 months, those averages decreased to 0.14 in-patient procedures and 0.29 out-patient procedures (Figure 35).

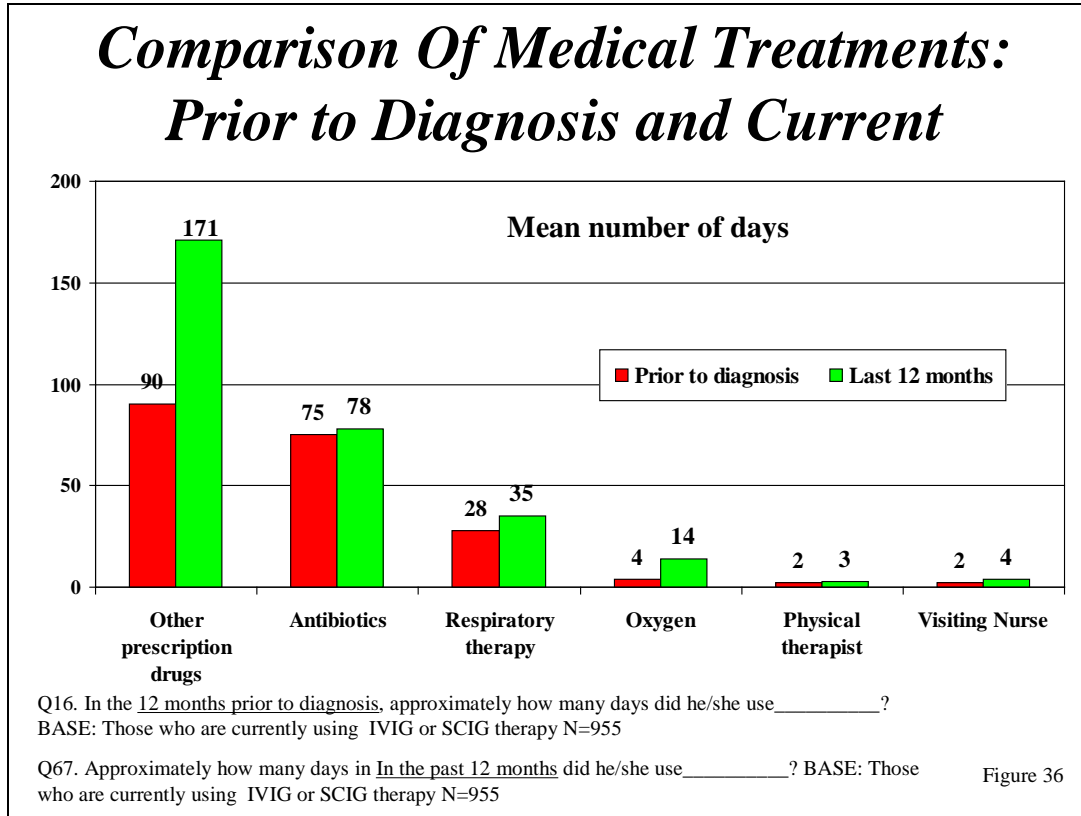
Comparison Of Doctor Visits: Prior to Diagnosis and Current



Comparison Of Surgical Procedures: Prior to Diagnosis and Current



Unfortunately, many IG users still require additional medical treatments, and some of these treatments are at a higher rate than prior to diagnosis. The use of prescription drugs among IG users has increased from an average of 90 days per year in the year prior to diagnosis to 171 days in the past 12 months. Antibiotic use increased from an average of 75 days to 78 days per year, respiratory therapy use increased from an average of 28 days to 35 days per year and oxygen use increased from an average of 4 days to 14 days in the recent twelve months. There is also an increase in the average use of a physical therapist (2 days prior, 3 days recently) and visiting nurses (2 days prior, 4 days recently). (Figure 36)

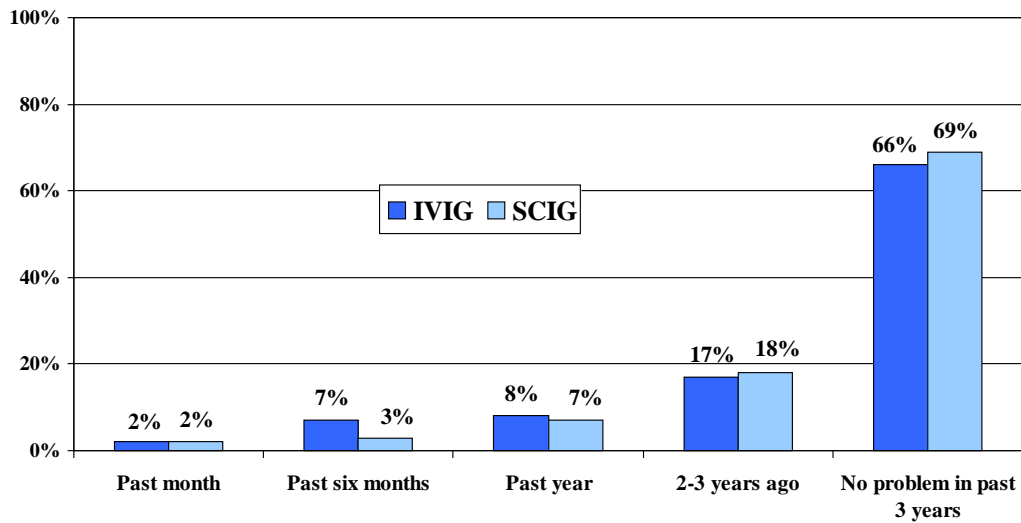


It should be noted that many of these treatments (e.g., respiratory therapy, physical therapists, and oxygen) represent treatments for functional disabilities incurred as a result of infections prior to treatment.

Availability

Few current users reported that they had difficulties in getting their regular IG therapy in the past few years. Two-thirds (66% of IVIG users and 69% of SCIG users) have not had a problem getting their regular therapy in the past three years. Among the remaining users, only 2% of IVIG and SCIG users reported a problem in the past month, 7% of IVIG users and 3% of SCIG users in the past 6 months, 8% of IVIG users and 7% of SCIG users in the past year. However, 17% of IVIG and 18% of SCIG patients reported having difficulty in getting IG therapy 2-3 years ago (Figure 37).

Most Recent Problem Getting Regular Infusion By Type Of Treatment: 2008

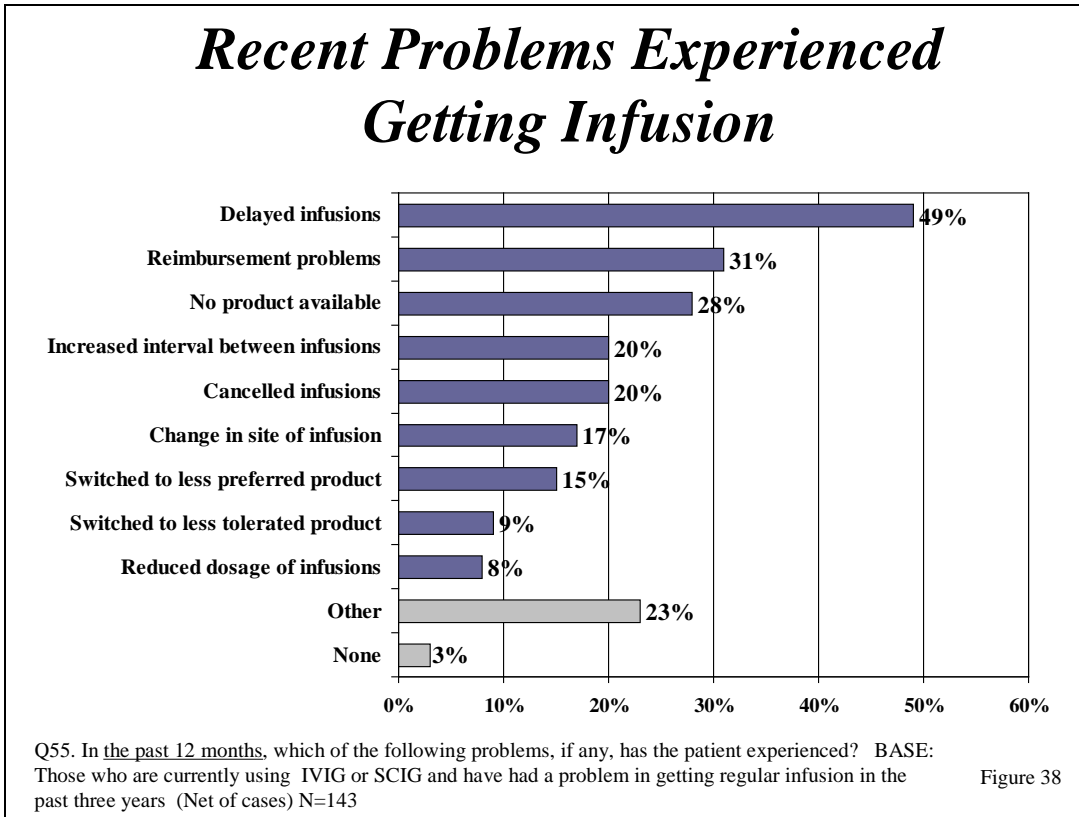


Q54. When was the most recent time the patient had a problem getting his/her regular infusion? BASE: Those who are currently using IVIG (N=716) or SCIG (N=293)

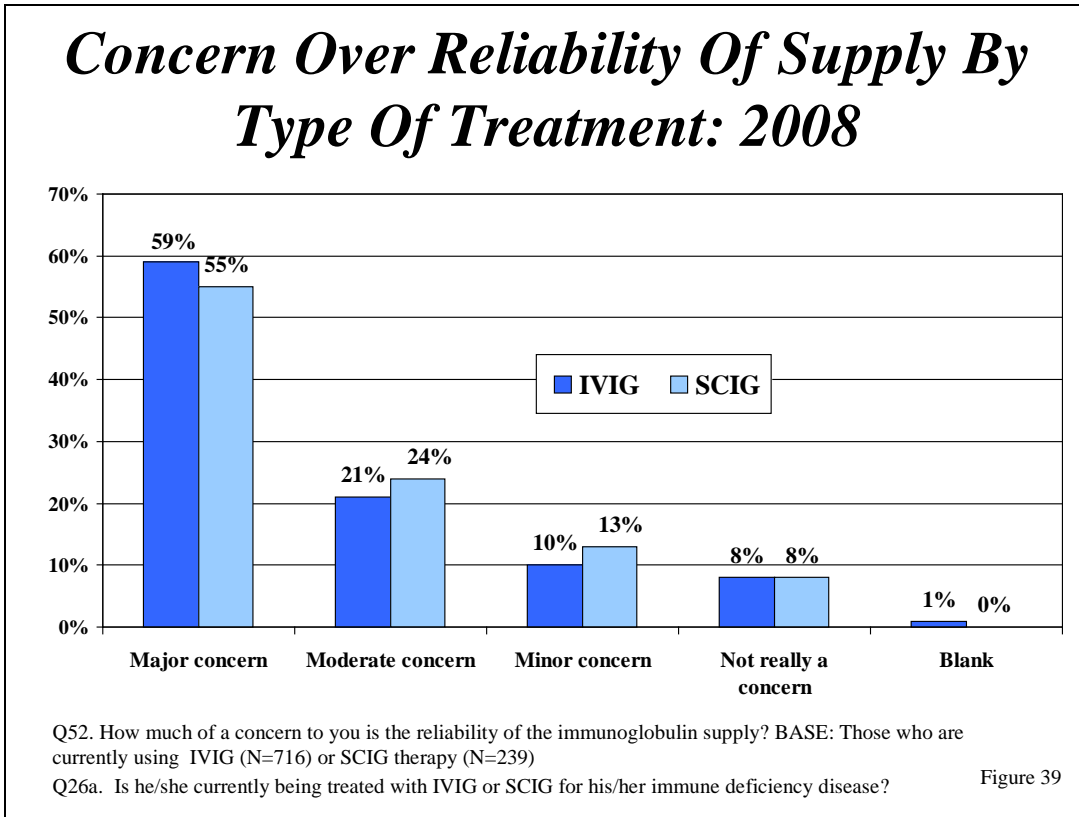
Figure 37

Q26a. Is he/she currently being treated with IVIG or SCIG for his/her immune deficiency disease?

Among respondents that had experienced a problem getting their regular IG therapy in the past three years, nearly half (49%) reported delayed infusions as the problem. Another 31% had problems with reimbursements, 28% had no product available, 20% had to deal with increased intervals between IG therapies, or cancelled therapies. In addition, there were problems of change in infusion site (17%), a switch to a less preferred product (15%) or less tolerated product (9%), or a reduced dosage of infusions (Figure 38).



Although relatively few patients experienced current difficulties with the supply of IG therapy, the majority of IG therapy users remain concerned about the reliability of the IG supply. Indeed, 59% of immune deficient patients using IVIG and 55% using SCIG said reliability of IG supply is a major concern for them. Another 21% of IVIG and 24% of SCIG users said that reliability of supply is a moderate concern. Only 10% of IVIG and 13% of SCIG patients said that reliability of the IG therapy supply is only a minor concern, while 8% said that it is not a concern for them (Figure 39).



Since the patients with primary immunodeficiency diseases have experienced dramatic improvements in their health since treatment with IG therapy, there is widespread satisfaction with the treatment. Not surprisingly, patients are concerned with the continued availability of these products and their access to them.

Conclusions

The results of this survey continue to support the tremendous efficacy of immunoglobulin replacement therapy for patients with primary immunodeficiency diseases who are antibody deficient. 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 differences in health, activity limitation, and infections before diagnosis and after the initiation of replacement therapy are considerable. Immunoglobulin replacement therapy is life saving.

Regrettably, there continues to be a long lag time between symptom onset of PIDD and the diagnosis and initiation of IG therapy in this patient population. As a result, many patients suffer debilitating, permanent functional impairments. Despite the effectiveness of IG therapy in dramatically improving patients' ability to avoid repeated, unusual and severe infections, IG therapy cannot reverse the permanent organ damage done by such infections prior to diagnosis and the initiation of IG therapy.

Given the effectiveness of IG therapy, it's imperative that patients receive proper diagnosis and have unimpeded access to this life-sustaining therapy. The early diagnosis of PIDD, and subsequent treatment with IG therapy is critical if antibody deficient patients with PIDD 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. health care system as well.