

**Treatment Experiences and Preferences
of Patients with
Primary Immune Deficiency Diseases:
First National Survey**



IMMUNE DEFICIENCY FOUNDATION

The National Organization Devoted To Research And Education For The Primary Immune Deficiency Diseases.

**Prepared by:
Schulman, Ronca & Bucuvalas, Inc.**

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Table of Contents

Overview.....	1
Background: Immune Deficiency Diseases.....	2
First National Survey of Patients with Primary Immune Deficiency Diseases.....	2
Second National Survey of Patients with Primary Immune Deficiency Diseases.....	4
Treatment Experiences Survey of IGIV Users: 2002.....	4
Use of Intravenous Immunoglobulin.....	5
Characteristics of Patients Using Intravenous Immunoglobulin.....	6
Health Status and Treatment.....	7
IGIV Infusions.....	9
Satisfaction with Infusion Experience.....	11
Convenience.....	13
Efficacy of Treatment.....	13
Safety.....	15
Reactions and Side Effects.....	16
Availability.....	18
Product Experience.....	21
Product Differentiation.....	21
Current Product and Product Satisfaction.....	22
Choices in IGIV Products.....	23
Willingness to Switch.....	24
New IGIV Products.....	25
Cost and Coverage.....	26
Conclusions: Immune Deficient Patients using IGIV.....	28

Overview

The Immune Deficiency Foundation commissioned a national survey of the Treatment Experiences and Preferences of Patients with Primary Immune Deficiency Diseases. The survey was conducted in the fall and winter of 2002. A total of nearly 1,200 patients with primary immune deficiency diseases, who were currently being treated with intravenous immunoglobulin (IGIV) therapy, completed an eight-page, self-administered questionnaire as part of the survey. The survey was conducted by mail by Schulman, Ronca and Bucuvalas, Inc. (SRBI), an international research organization, under contract to the Immune Deficiency Foundation.

At the time that the current survey was commissioned, the only national sampling frame for patients with primary immune deficiency diseases was the participants from the First National Patient Survey conducted by IDF in 1996-1997. Nearly 3,000 persons with a diagnosis of a primary immune deficiency disease participated in the 1996 survey. The target population for the Survey of Treatment Experiences and Preferences was patients who were being treated with intravenous immunoglobulin (IGIV) for a primary immune deficiency disease. Approximately 2,000 of these participants in the First National Patient Survey reported being treated with IGIV for their condition.

A sampling frame of patients in 1996, unfortunately, would eliminate all patients diagnosed since 1996 from the current survey, as well as other diagnosed patients who were unknown to the Foundation at that time. To avoid this bias, the sample for the 2002 Treatment Experiences Survey was stratified into two subpopulations: patients from the 1996 survey, and new patients who were not included in the first survey. The new patient sample was identified by conducting a second national patient survey, nearly identical in content to the 1996 survey, among patients in the IDF database who were not participants in the earlier survey. A total of 6,000 potential new patients were sent a four-page mail questionnaire in the fall of 2002. More than 1,500 new patients with a diagnosis of primary immune deficiency disease completed and returned the new patient questionnaire in time to participate in the Treatment Experiences Survey.

The Treatment Experiences Survey was mailed to a sample of old patients from the 1996 patient survey and new patients from the 2002 patient survey. Among the old patient sample, the questionnaire was mailed to all patients, regardless of their IGIV use in 1996. Among the new patient sample, the questionnaire was only mailed to patients who were currently using IGIV therapy.

The sample design for the Treatment Experiences Survey called for an achieved sample of 1,000 completed interviews, relatively evenly divided between the two strata of new patients and old patients. This goal was surpassed in early January. By the end of February, a total of 535 interviews from the old patient sample and 651 interviews from the new patient sample were received by that time. The findings presented in this report are based on this sample of 1,186 patients.

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Background: Immune Deficiency Diseases

Primary immune deficiency 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 80 different primary immune deficiency diseases currently recognized by the World Health Organization.

Medical recognition of primary immune deficiency disease is only 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.

Although primary immune deficiency diseases are often described as rare disorders, the true population prevalence of these diseases, either individually or in the aggregate, is not well established. 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 immune deficiency 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. Hence, although these diseases are clinically described in the medical literature, there is no comprehensive portrait available of the patient with primary immune deficiency disease.

First National Survey of Patients with Primary Immune Deficiency Diseases

In 1996, the Immune Deficiency Foundation undertook the first national survey of the state of primary immune deficiency diseases in the United States. This survey had a number of objectives. First, the survey sought to provide an estimate of the general magnitude of primary immune deficiency in the American population, if not a precise estimate of population prevalence. Second, the survey sought to describe the general population characteristics of persons with these disorders. Third, the survey sought to describe the health of persons with primary immune deficiency diseases, both with and without treatment. Fourth, the survey sought to identify problems in access to treatment in this population. All of these goals are related to the primary objective of the Immune Deficiency Foundation: improving the diagnosis and treatment of persons with primary immune deficiency diseases. The survey was designed for IDF by Schulman, Ronca and Bucuvalas, Inc. (SRBI), a national public opinion research organization. SRBI analyzed the survey data and prepared the report for the Foundation.

The survey was designed within the constraints of primary immune deficiency diseases. In the absence of a rigorous set of symptom criteria that would uniquely define primary immune deficiency disease, the survey population must be restricted to the sub-population who already had a diagnosis of primary immune deficiency disease. Many persons with immune deficiency diseases may be relatively asymptomatic. Others may have chronic and/or unusual infections that are the hallmark of immune deficiency, but have not yet been diagnosed with the underlying disorder. This survey was restricted to the population who have been tested and diagnosed with a primary immune deficiency disease. No clinical confirmation of the diagnosis was incorporated into the study, so the survey is restricted to persons who report a physician diagnosis of a primary immune deficiency disease.

Since primary immune deficiency diseases are comparatively rare, and diagnosed cases of the condition will be rarer still, population screening to obtain a national sample of persons with these disorders was not feasible. However, it was possible to develop a relatively complete sampling frame for physicians who were most likely to treat these disorders. So, a multi-stage sampling strategy was developed to obtain a large, national sample of persons diagnosed with primary immune deficiency diseases.

The first stage in the sampling process was to construct a sampling frame of the specialists who were most likely to follow patients with primary immune deficiency diseases. This included the major medical associations and societies of specialties related to immune diseases (immunology and infectious diseases), chairmen of pediatric departments in medical centers, and previously identified treating physicians in IDF mailing lists and registries. The combined sampling frame included a total of 17,451 physicians.

The second stage in the sampling process was to conduct a systematic survey of this population to identify the sub-population who currently followed patients with primary immune deficiency diseases. The survey identified the most common of these disorders by name in order to reduce ambiguity about which disorders are primary immune deficiency diseases. A total of 1,502 physicians from the sampling frame reported that they were currently treating one or more patients with these disorders.

The third stage in the sampling process was to send patient questionnaires to these physicians for distribution to their patients with primary immune deficiency disease. In addition, this questionnaire was sent to all self-identified patients in the IDF database. A total of nearly 3,000 questionnaires were completed and returned by unique patients. This includes 1,289 adult patients, 1,190 parents or guardians of children with primary immune deficiency diseases, and 335 where the respondent did not identify themselves as the patient or caregiver.

The sampling frame used for the survey did not provide complete coverage of physicians treating patients with primary immune deficiency diseases, nor by extension the patients themselves. The multi-stage sampling process introduced opportunity for non-sampling bias, even among the truncated sampling frame. In the absence of any

denominator for the number of questionnaires distributed to eligible patients, we cannot estimate the response rate to the patient survey or adjust for non-response bias. Nonetheless, the sampling and field procedures produced a large, geographically diverse and relatively unclustered sample of persons with primary immune deficiency diseases in the United States.

Second National Survey of Patients with Primary Immune Deficiency Diseases

In the fall of 2002, the Immune Deficiency Foundation undertook the second national survey of the state of primary immune deficiency diseases in the United States. This survey was designed to supplement the 1996 survey. The IDF contact database provided the first stage in the construction of the sampling frame. Persons who were identified as being physicians, other health professionals, or other interested non-patients in the IDF database were eliminated from the sampling frame. Patients who had participated in the 1996 survey were also eliminated from the sampling frame for the second national survey. The approximately 6,000 cases remaining in the database after these two steps provided a sampling frame for the Second National Survey of Patients with Primary Immune Deficiency Diseases.

The first 1,000 cases from this frame were sent an advance letter in September 2002 inviting them to participate in the survey by Internet. They were provided a web address for the Internet survey and personal identification number to access the survey. However, less than ten percent of this sample contacted the survey website to begin the interview. Consequently, the remaining cases in the sample were mailed a four page self-administered questionnaire, along with a cover letter explaining the purposes of the survey, and a postage-paid return envelope. The first questionnaire mailing was conducted between 9/17 and 10/17/02. A second mailing to non-respondents was conducted on November 21, 2002.

A total of 1,587 completed short form questionnaires were returned by eligible respondents from the 5,922 cases in the sampling frame (26.8%). In addition, 49 cases were identified as deceased patients with primary immune deficiency diseases. Another 18 cases had misdiagnosed, transient or treated immune deficiency diseases. Another 56 cases reported that they were patients, but their condition was not a primary immune deficiency disease (e.g., auto-immune diseases).

Treatment Experiences Survey of IGIV Users: 2002

The two national surveys of patients with primary immune deficiency diseases provide the sampling frame for a follow-up survey of 1,000 persons identified as currently using intravenous immunoglobulin (IGIV). 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 IGIV use in 1996/7. Consequently, the questionnaire for this population was structured so that patients who were no longer using IGIV ended the interview at Question 10b at the bottom of the first page.

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 immune deficiency diseases who were currently using IGIV 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.

This report is based on the first 1,186 completed questionnaires from the “old patient” and “new patient” samples. This includes 535 patients who currently use IGIV, who were first identified in the baseline 1996 survey. It also includes 651 patients currently using IGIV who were first identified in the 2002 survey.

Use of Intravenous Immunoglobulin

Intravenous immunoglobulin is the medically recommended treatment for some, but not all primary immune deficiency diseases. In the 1996 national survey of nearly 3,000 patients with primary immune deficiency diseases, 70% reported that they had been treated with intravenous immunoglobulin. Among the sub-sample of 759 “old patients” from the 1996 survey who completed the 2002 survey, a virtually identical 71% reported that they are currently being treated with intravenous immunoglobulin (Figure 1). Among the national sample of 1587 “new patients” from the 2002 patient survey, a slightly smaller proportion (67%) reported that they were currently being treated with IGIV (Figure 2).

More than half of the patients with primary immune deficiency diseases who are not currently being treated with IGIV have never been treated with IGIV. However, 13% of immune deficient patients in both the old patient sample and the new patient sample, had been treated with IGIV in the past, but were not currently being treated with IGIV (Figures 1 and 2).

The reported diagnosis among discontinuing users among the “old patients” included a substantial number of persons with IgG subclass deficiency (16%) and IgA deficiency (12%). However, half (50%) had been diagnosed with common variable immune deficiency, while some others had been diagnosed with agammaglobulinemia (Figure 3). Hence, the majority of the discontinuing IGIV users had diagnoses that would normally recommend this therapy for treatment of their condition.

The discontinuing IGIV users were asked why they were no longer being treated with IGIV. Frequently, the discontinuing IGIV users reported that better health and/or normal to near normal immunoglobulin levels were the reasons that stopped the therapy. Others did so because their doctors wanted to see how they did without IGIV treatment. Some had been cured by bone marrow transplantation. Others were currently being treated with subcutaneous immunoglobulin, rather than IGIV, as part of clinical trials. Nonetheless, many patients diagnosed with primary immune deficiency disease reported that they had discontinued this treatment because of side effects or reactions to IGIV, fear of contracting disease through the product, costs or coverage of treatment, lack of product availability, inability to get a good vein and unwillingness to use a port (Figure 4). Nearly half of patients with primary immune deficiency diseases who had discontinued using IGIV had one of these “bad” reasons for not using IGIV.

Characteristics of Patients Using Intravenous Immunoglobulin

The target population for the Survey of Treatment Experiences and Preferences was patients with primary immune deficiency diseases who were currently being treated with intravenous immunoglobulin. Hence, the rest of this report will focus on the national sample of approximately 1,200 patients who were using IGIV in the fall and winter of 2002.

Among those who were currently using IGIV, the majority (60%) reported that their current diagnosis is common variable immunodeficiency (CVID). Another 8% reported a diagnosis of IgG subclass deficiency only. One out of six current IGIV users (17%) had a diagnosis of agammaglobulinemia. There were also small proportions of patients with SCID (4%), Hyper IgM syndrome (2%), or IgA deficiency (2%). The remaining IGIV users had another diagnosis (3%) or no specific diagnosis (2%) reported in the survey (Figure 5).

In 2002, primary immune deficiency diseases were no longer a pediatric condition. Only 5% of this sample of IGIV using immune deficient patients was six years of age or less. Nine percent were aged seven to twelve. Another 9% were adolescents, aged thirteen to seventeen. In total, less than a quarter of the patients were under 18 years of age. Twelve percent were young adults, aged 18 to 29. Nearly a quarter (23%) of the patient population was 30 to 44 years old. A third (34%) was middle aged, 45 to 64 years old. And, nearly one in ten (8%) of the IGIV using immune deficient patients were aged 65 or older (Figure 6).

The majority of immune deficient patients being treated with IGIV in 2002 were adult patients (69%). Only three out of ten participants in the Treatment Experiences Survey (30%) were the parent or caregivers of children or incapacitated adults with a primary immune deficiency (Figure 7). Even among the “new patient” sample, 63% were adult patients, rather than parent/caregivers.

The gender distribution of persons with primary immune deficiency diseases in the original 1996 National Patient Survey mirrored the general population distribution of 48% males and 52% females. Among the national sample of IGIV users in 2002, however, 43% were male and 57% were female (Figure 8). The ratio of males to females was slightly lower in the new patient sample (42%/58%) than among the old patient sample (44%/56%) from the 1996 survey.

Health Status and Treatment

The majority of patients with primary immune deficiency disease, who were currently being treated with IGIV, described their current health status as excellent (6%), very good (18%), or good (35%). About a quarter (25%) described their health as only fair, compared to other persons of the same age. About one in seven said their health was poor (11%) or very poor (4%) compared to others of the same age (Figure 9).

The proportion of patients who rated their health as good or better fell from more than three-quarters (76%) of those under age seven to only 49% of those aged 45 to 64. More dramatically, the proportion of patients who described their health as excellent or very good dropped from 56% of those aged 6 or less, to 25-26% of those aged 7 to 17. It increased to 33% of those aged 18 to 29, before declining to 22% of those aged 30-44, and 16% of those aged 45 to 64. In general, the oldest patients with primary immune deficiency disease were in better health than all but the youngest patients (Figure 10).

The general health self-rating of the sample of patients with primary immune deficiency diseases can be compared to the general population of the United States from the National Health Interview Survey. The comparison shows a measurable deficit in health status between immune deficient patients and the general population in almost all age groups. However, the deficit was most pronounced in 30-44 year olds and 45-64 year olds. The reported health status was actually better among immune deficient patients aged 65 and older than in the general public (Figure 11).

Three out of four patients with primary immune deficiency diseases (76%), who were being treated with IGIV, reported no overnight hospitalizations in the past year. Most of the remainder (12%) reported only one hospitalization in the past 12 months. Nonetheless, 5% of patients reported two hospitalizations, and another 7% reported three or more hospitalizations in the past year (Figure 12).

There was significant variation in the average number of hospitalizations in the past year by specific diagnosis. The lowest average number of hospitalizations (.2) was reported by persons with agammaglobulinemia. The highest average number of

hospitalizations in the past year (1.1) was reported by persons with severe combined immune deficiency, followed by those with IgA deficiency (.9). Persons with common variable immune deficiency (.6), IgG subclass deficiency (.5) and Hyper IgM syndrome (.6) had about the same number of hospitalizations in the past year, on average. Persons with other diagnoses had 1.3 hospitalizations in the past year, on average. (Figure 13).

The type of doctor seen most often by the patient for his or her health care was split between primary care and specialists. More than two out of five patients reported that the doctor they see most often for their health care is in pediatrics (8%), family practice (17%) or internal medicine (16%). About the same proportion reported that the doctor they see most often for their health is a specialist in immunology (28%), hematology (5%), or other medical specialty (9%). About one in six patients (17%) reported seeing more than one kind of doctor most often for their health (Figure 14).

There were some important differences in overall health status of patients with primary immune deficiency diseases seen by doctors in different specialties. More than three out of four immune deficient patients who saw a pediatrician most often for their health (76%) described their health as excellent, very good or good. About two-thirds of immune deficient patients who see a family practice doctor (67%) or immunologist (64%) most often said their health is good or better. About six in ten of those who see an internist (59%) or hematologist (60%) most often said their health is good, very good or excellent. Only half of those who see some other specialist most often about their health (50%) said their health is good or better (Figure 15).

Most patients usually visited their primary care doctor in a private office (67%). About one in five patients (18%) usually see their doctor in a group practice or HMO setting. Less than one in ten (7%) usually see their doctor in a hospital outpatient or hospital clinic (Figure 16).

On average, IGIV users reported 4.2 immunologist visits in the past year, or about one every three months. Only one in five immune deficient patients (20%) reported no visits to see an immunologist in the past 12 months. By contrast, 15% reported twelve or more (i.e., once a month or more often) visits to an immunologist in the past year (Figure 17).

The average number of times seen by an immunologist in the past year also varied by diagnosis. The lowest average number of immunologist visits (2.8) was reported by persons with IgA deficiency, followed by patients with agammaglobulinemia (3.0). The highest average number of immunologist visits in the past year (5.5) was reported by persons with IgG Subclass deficiency and those with severe combined immune deficiency (5.2). Persons with common variable immune deficiency (4.2) and Hyper IgM syndrome (4.1) saw an immunologist about the same number of times in the past year, on average (Figure 18).

Overall, IGIV using patients with primary immune deficiency diseases are satisfied with their primary doctor's management of their condition. More than three out

of five patients (62%) said that they are very satisfied with the primary doctor's management of their primary immune deficiency disease. Another 25% said that they are somewhat satisfied with their doctor's management of their disease. By contrast, only 7% said that they are neither satisfied nor dissatisfied. And, only six percent said that they are somewhat (4%) or very dissatisfied (2%) with their doctor's management of their primary immune deficiency disease (Figure 19).

IGIV Infusions

This sample of patients with primary immune deficiency disease had considerable experience with intravenous immunoglobulin therapy. These patients have taken IGIV on a regular basis for almost ten years (9.3) on average (Figure 20). The old patient sample, which by definition had been diagnosed with a primary immune deficiency disease in 1996, increased the average length of time on IGIV for the combined samples. Nonetheless, even among the new patient sample, the average number of years on IGIV was 7.0 years.

In a previous unpublished survey of nearly 1,000 IGIV users from the "old patient" population, we found that the time to diagnosis from symptom onset averaged 8.3 years. Hence, immune deficient patients for whom IGIV therapy was judged as therapeutic (i.e., they are currently being treated with the IGIV) have spent about an equal amount of time not being treated with IGIV (8.3 years) after symptoms but prior to diagnosis as the time spent being treated with IGIV since diagnosis (9.3 years). Moreover, the earlier survey data indicates no improvement in the time to diagnosis by decade of diagnosis. Further, the earlier survey data finds that time to diagnosis is correlated with the rate of permanent functional impairment prior to diagnosis among this population. It should be noted that while early diagnosis is critical to long term health outcomes in this population, this data suggests that early diagnosis could nearly double the demand for IGIV in the population with primary immune deficiency diseases.

The frequency of IGIV infusions varied considerably among persons with primary immune deficiency diseases. A majority (55%) reported that, on average, they get an infusion every four weeks. About a quarter (26%) reported that, on average, they get an infusion every three weeks. One in ten said they got their infusion every two weeks or more often (11%). A slightly smaller proportion said that they get their infusion every five weeks or less often (7%). (Figure 21)

Most patients reported that the average number of grams per infusion is 20-29 grams (26%) or 30-39 grams (23%). One in seven (14%) patients reported 10-19 grams per infusion. Only a handful (4%) reported infusions of less than ten grams. However, one in six patients (16%) reported infusion doses of more than 40 grams. One in ten patients or caregivers (11%) did not know the average number of grams of IGIV they used per infusion. Among those who did know how many grams they were receiving, the average number of grams per infusion was 28.3 (Figure 22).

The average weight of the IGIV using immune deficient patients varied with both age and gender. Overall, however, the average IGIV using patient weighs 146 pounds (Figure 23) or approximately 66 kilograms.

The average immune deficient patient on IGIV infused about 449 grams of IGIV per kilo of weight. There was some variability in the average dosing of IGIV by diagnosis. The largest doses (in grams per kilo) were found among patients with Hyper IgM (547), IgG Subclass deficiency (504) and severe combined immune deficiency (486). Somewhat lower doses were found among those with common variable immunodeficiency (445) and XLA (423). The lowest dose was found among patients with IgA deficiency (435). (Figure 24)

There was also some variation in the average dose amount by physician specialty. The highest doses of IGIV, on average, were found among those who saw an immunologist for their health (485 grams per kilo). Patients who were treated by a pediatrician also tended to have higher doses of IGIV (465) than the average. The lowest doses, on average, were found among patients seen most often by internists (415) or other specialists (413). (Figure 25)

The average grams per kilo infused by immune deficient patients also varied somewhat by health status. Indeed, the average amount of IGIV infused increased from those in excellent health (413), to those in very good health (429), to those in good health (450) to those in only fair health (469). The average amount infused by those in poor (458) and very poor health (453) was somewhat less than those in fair health, but more than those in excellent, very good or good health (Figure 26).

There was no clear relationship between the average amount of IGIV infused and the frequency of infusion. Those who infused every three weeks reported a larger amount of IGIV infused (29.8 grams), on average, than those infusing every 4 weeks (28.6 grams), every five weeks (22.8 grams) or every six weeks (26.5 grams). They did, however, infuse a larger amount than those who infuse every two weeks (25.0 grams), on average (Figure 27).

Even when controlling for the patients weight, there was no clear relationship between the average amount of the dose and the frequency of infusion. Those who infused every 2 weeks reported an average dose of only 386 grams per kilo. This increased to 491 grams per kilo for those infusing every three weeks on average. However, the average IGIV dosage dropped to 448 grams per kilo for those infusing every four weeks, and 366 for those infusing every 5 weeks, and then increased to 410 grams per kilo for those infusing every 6 weeks or less often (Figure 28).

About a quarter of current IGIV users (25%) reported that they had larger or more frequent doses of IGIV on a regular basis in the past (Figure 29). The most common reason that they were currently receiving less frequent infusions or smaller doses was better health. Others reported less frequent infusions because of larger doses, or smaller doses due to more frequent infusions. However, side effects, insurance, cost and

shortages were also reported as reasons for smaller doses or less frequent IGIV infusions (Figure 30).

Satisfaction with Infusion Experience

Two out of five (40%) IGIV users reported that they usually received their infusion at home. Three in ten (30%) IGIV users usually got their infusion at a hospital. Most of the remainder said that they usually got their infusion in a doctor's private office (12%) or an infusion suite (11%). (Figure 31)

There was relatively little difference in the average dose of IGIV by infusion site. Those who infused at home reported a somewhat lower dose (439), on average, but they were also more likely to be infusing every two weeks. There was little or no difference in the average dose infused in doctors' offices (461), hospital outpatient departments (452) and infusion suites (467). The highest doses of IGIV (480 grams per kilo) were infused in hospital clinics (Figure 32).

Most patients seemed to be satisfied with the convenience of the location of their infusion site. Two thirds (66%) said it was very convenient. Another 21% said that it was somewhat convenient. About one in seven IGIV users in this population said that their usual infusion site was somewhat (9%) or very (3%) inconvenient (Figure 33).

Satisfaction with convenience varied somewhat with infusion site. Almost all patients who infused at home (96%) were very satisfied with the convenience of their infusion site. By contrast, about half of patients who infused in a doctor's office (51%) or hospital outpatient department (48%) were very satisfied with the convenience of the location. Less than half of patients who usually got their IGIV in infusion suites (43%), hospital clinics (36%) and other places (38%) were very satisfied with the convenience of the location of their infusion site (Figure 34).

Most patients (69%) usually got their infusions during the normal workday. About one in ten (9%) got their infusions before 9 a.m. on weekdays. A similar proportion (12%) usually got their infusion after 5 p.m. on weekdays. Only 9% of IGIV using immune deficient patients usually got their infusions on the weekends (Figure 35).

Most patients also seemed to be satisfied with the convenience of the times that they got their infusions. Nearly two thirds (64%) said it was very convenient. Another 26% said that it was somewhat convenient. About one in ten IGIV users in this population said that their usual infusion site was somewhat (7%) or very (2%) inconvenient (Figure 36).

Six out of seven IGIV users (87%) reported that a nurse usually administers the infusions. Only one in ten users reported that the patient (5%) or another family member (4%) usually administered the IGIV. Doctors (2%) rarely administered the IGIV to patients with primary immune deficiency diseases (Figure 37).

Only 4% of IGIV using immune deficient patients reported that their infusions usually lasted one hour or less. About one in five patients (21%) reported that the infusion usually took from 1 to 2 hours. One in three (31%) said the infusion usually took 2 to 3 hours. However, two out of five patients said that infusion usually took 3-4 hours (20%), 4-5 hours (10%), or 5 hours or longer (11%). The average IGIV infusion time was 207 minutes, nearly 3 ½ hours (Figure 38).

Surprisingly, most patients seemed to be satisfied with the amount of time it takes to get their infusions. Only one third (33%) said they were very satisfied with their infusion time. But, another 37% said that they were somewhat satisfied. About one in seven IGIV users in this population (12%) said that they were neither satisfied nor dissatisfied with their infusion time. One in six patients said that they were somewhat (12%) or very (5%) dissatisfied with the time it took to get an infusion (Figure 39).

Although most patients were satisfied with the time it took to get an infusion, patient satisfaction was directly related with the amount of time it took them to get their infusions. Among those who were very satisfied with the time it took to get an infusion, the average infusion time was 163 minutes. Among those who were somewhat satisfied, the average infusion time increased to 207 minutes. The average infusion time was 230 minutes for those who were neither satisfied nor dissatisfied. The average infusion time increased to 260 minutes for those who were somewhat dissatisfied. Among those who were very dissatisfied with the time it took to get an infusion, the average infusion time was 322 minutes, more than five hours (Figure 40).

Only 15% of patients reported that they determined the rate of infusion. More often, patients reported that the doctor (43%) or a nurse (25%) determined the rate of infusion. The remaining IGIV users (16%) said that someone else determined the rate of infusion, such as a combination of doctor, nurse and patient (Figure 41).

Approximately half of all IGIV using patients with primary immune deficiency diseases reported the use of medication, such as antihistamines, corticosteroids or an anti-inflammatory, before getting an infusion. More than two out of five patients (45%) reported that they usually were given medication before infusion to make it go faster. Another 6% said that they were sometimes given such medication before infusion (Figure 42).

The use of medication before infusion was related to the average time it took to get an infusion. Those who usually got pre-infusion medication took an average of 246 minutes to infuse their IGIV. Those who sometimes got pre-infusion medication took an average of 200 minutes to infuse. By contrast, the average length of infusion was 171 minutes, just under three hours, for those who did not get medication before infusions (Figure 43).

Convenience

When asked in an open-ended fashion what was most important when they thought of convenience in an IGIV product, IGIV using patients gave a wide range of responses. Most commonly, they said that what was most important to them in IGIV product convenience was rate of infusion (18%), availability (17%), safety (18%), and side effects (13%). However, smaller proportions said that pre-mixed (6%), easily mixed (5%) or do-it-yourself (7%) products were most important to them when thinking of convenience. Location (5%) and delivery (3%) were also volunteered by some when thinking about convenience. Efficacy (9%), purity (6%) and cost (2%) were also volunteered by patients when asked to think about the meaning of convenience in an IGIV product (Figure 44).

In the choice between a product that came in solution and a product that came in a powder, IGIV using patients were seven times as likely to choose the solution (42%) than powder (6%). Nonetheless, half of the patients said that they had no preference between powder and solution (48%) or chose not to answer the question (4%). (Figure 45)

Among those who preferred IGIV in solution, convenience (60%) was the main reason that they preferred the form. At the same time, 25% of those who preferred IGIV in powder form also indicated that convenience was the main reason for their preference. Only 3% of those who preferred solution and 13% of those who preferred powder said that they preferred the form because of safety. Among those who preferred powder, 21% indicated their reason for preference was they had always used that form. Nineteen percent of those who preferred solution indicated their preference was due to always having used that form (Figure 46).

Most patients reported that they had to wait for more than a few minutes while the IGIV product was being prepared for infusion. Nearly a third (31%) said that they often had to wait more than a few minutes for product preparation. Another 27% said that they sometimes had to wait more than a few minutes. Only 40% of IGIV using immune deficient patients said that they never had to wait more than a few minutes for product preparation prior to infusion (Figure 47).

When asked about IGIV preparations that were stable at room temperature, which would not have to be kept in a refrigerator, most IGIV using patients saw this as a benefit. Indeed, a third (32%) considered this a major benefit. Another 28% saw this as a moderate benefit. Only 23% of IGIV using patients saw no benefit in a product that would not have to be refrigerated (Figure 48).

Efficacy of Treatment

Only one in seven immune deficient patients being treated with IGIV (12%) felt that the IGIV completely controlled their immune deficiency. But nearly half (45%) said

that they felt their immune deficiency was well, if not completely, controlled by the IGIV. Another third (34%) of patients said they felt their immune deficiency was adequately, if not well controlled, by their IGIV. Only 7% of IGIV using patients said their IGIV controlled their immune deficiency less than adequately (Figure 49).

When asked in an open-ended fashion what would indicate that IGIV was not effective in controlling their condition, most patients volunteered illness or infection. Most commonly, patients said that more frequent infections (40%) or more frequent illness (17%) would be the sign that their IGIV was not controlling their condition. By contrast, much smaller proportions of patients said that infections (10%) or sickness (12%), irrespective of frequency, would indicate a failure of control. Some patients also volunteered specific types of infections: sinus (12%), upper respiratory (6%) and pneumonia (7%) as indicative of ineffectiveness in IGIV. Fatigue was volunteered by 10% of patients as something that would indicate product ineffectiveness to them. Only a small proportion of patients said, top of mind, that what would indicate product ineffectiveness to them was hospitalization (3%) or low levels of immunoglobulins (2%). (Figure 50)

The patient's rating of the effectiveness of the IGIV in controlling their immune deficiency was closely related to their perception of their health status. Among those who felt that their IGIV completely controlled their immune deficiency, 87% rated their health as good or better. The self-rating of health as good or better fell to 74% of those who felt their immune deficiency was well controlled by IGIV, and to 42% of those who rated condition control as only adequate. Among those who felt their IGIV controlled their immune deficiency less than adequately or poorly, only 14% rated their health as good or better compared to other persons of the same age (Figure 51).

A similar, though less dramatic difference, was seen in the past year prevalence of hospitalization by perceived IGIV control of their immune deficiency. Nearly nine out of ten patients who felt that their IGIV completely controlled their immune deficiency (87%) reported no overnight hospitalization in the past 12 months. This was equivalent to the past year prevalence of hospitalization in the general public. However, the proportion of patients with no hospitalization in the past 12 months fell progressively to 79% among those whose condition was well controlled, 73% of those whose condition was adequately controlled, to 60% of those whose condition was less than adequately or poorly controlled by their IGIV (Figure 52).

Finally, there was a progressive decline in patient satisfaction with the doctor's management of their condition and how well the patient felt that IGIV controlled their immune deficiency. Nearly four out five patients who said the IGIV completely controlled their condition (78%) were very satisfied with the doctor's management of their condition. However, the proportion of patients who were very satisfied with their doctor's management of their condition declined to 69% among the well controlled, 53% of the adequately controlled, and 37% of those who felt that their condition was less than adequately controlled by IGIV (Figure 53).

Most immune deficient patients who use IGIV reported that they could feel when the effects of the infusion were wearing off. More than two in five IGIV users (42%) said that they could usually feel when the effects of the infusion are wearing off. Another 26% said that they could sometimes feel when the effects are wearing off. Only 30% of IGIV using immune deficient patients said that they could never feel the infusion effects wearing off (Figure 54).

The proportion of patients who felt the effects of their IGIV wearing off varied by frequency of infusion. The proportion who usually felt the effects wearing off was highest among those who usually infused every two weeks (50%) or every three weeks (57%). The proportion usually feeling the effects wearing off declined to 36% among those infusing every four weeks and 23% among those infusing every five weeks (Figure 55).

Among those who could feel the infusion effects wearing off, the average number of days post-infusion when the patient felt the IGIV effect wearing off was closely related to the frequency of infusion. Those who usually infused every two weeks felt the effects wearing off at 11 days post-infusion, on average. Those who infused every three weeks felt the effects wearing off at 16 days post-infusion. Those who infused every four weeks felt the effects wearing off after 21 days. Those who infused every five weeks felt the effects wearing off after 29 days. And, those who infused every six weeks or longer, felt the effects of the infusion wearing off after 37 days, on average (Figure 56).

Concerns about product effectiveness have caused a small proportion of patients to switch off a specific product (6%) or switch to another product (16%). Effectiveness concerns have also caused some patients to refuse a product (7%) or delay an infusion (5%). However, nearly three quarters of IGIV using immune deficient patients (72%) had done none of these things because of concerns of product effectiveness (Figure 57).

Safety

The safety of the IGIV products was a concern for most immune deficient patients who infuse these products. Nearly half of immune deficient IGIV users (46%) said that product safety was a major concern for them. Most of the rest (29%) said that product safety was a moderate concern for them. Only 15% of immune deficient patients who use IGIV said that product safety was only a minor concern to them. Fewer still (9%) said that IGIV product safety was not a concern for them (Figure 58).

When asked in an open-ended fashion about safety in IGIV, most immune deficient patients volunteered specific viral diseases that can be transmitted by blood. When thinking about IGIV safety, hepatitis (30%) and HIV/AIDS (24%) were the diseases most often volunteered by IGIV using patients. One in twenty patients volunteered CJD (5%) or West Nile Disease (6%) when thinking about IGIV safety. Risk of disease, generally, was volunteered by 27% of IGIV users when thinking about product safety. By contrast, only 15% said that they thought about contamination, as

contrast to disease, when thinking about IGIV safety. About one in eight IGIV users (13%) volunteered quality control measures when thinking, top of mind, about product safety (Figure 59).

Most IGIV users who are immune deficient recognize that there is some risk of disease from an infusion of IGIV. But in most cases, this is perceived as a low risk. Despite the fact that there is no evidence that it has ever happened to an immune deficient patient in the United States, about the same proportion of users felt there is a moderate to high risk of getting HIV (13%), CJD (14%), and Parvovirus (12%) from an infusion of IGIV. However, significantly more patients thought there is a moderate to high risk of getting hepatitis (28%) from an infusion of IGIV, which has happened to immune deficient patients in this country (Figure 60).

Concerns about product safety caused a small proportion of patients to switch off a specific product (5%) or switch to another product (9%). Safety concerns have also caused some patients to refuse a product (6%) or delay a scheduled infusion (5%). However, nearly four out of five IGIV using immune deficient patients (77%) did none of these things because of concerns of product safety (Figure 61).

When asked about the most reliable source of information about the safety and efficacy of IGIV products and therapy, patients most often cited their doctor (72%). Remarkably, however, more than half of immune deficient patients (58%) cited patient organizations, generally, or the Immune Deficiency Foundation, specifically, as the most reliable source of information about IGIV safety and effectiveness. A significant number of IGIV users indicated their nurses (40%) and the Internet (31%) as reliable sources of information about product effectiveness and safety. Books and magazines (14%) and other patients (11%) were also mentioned by more than one in ten patients as the most reliable sources of information on IGIV safety and effectiveness (Figure 62).

Although 31% of immune deficient patients using IGIV indicated that the Internet was among the most reliable sources of information about product safety and effectiveness, only one in five patients used the Internet either weekly (6%) or monthly (14%) to get information about immune deficiency diseases and their treatment. The majority (52%) said that they use the Internet occasionally for information about their condition and treatment. More than a quarter (27%) said that they never use the Internet for this type of information (Figure 63).

Reactions and Side Effects

Most IGIV using immune deficient patients have experienced rate related (speed of infusion) reactions. Nearly three in ten IGIV users (29%) reported experiencing rate-related reactions from IGIV treatment in the past 12 months. Another 32% reported having rate related reactions from IGIV, but not in the past 12 months. Only 38% of IGIV users in this population said that they have never had a rate related reaction (Figure 64).

Nine out of ten patients with rate related reactions (90%) slowed down the infusion rate in response to the reaction. Nearly one in five (19%) switched IGIV products in response to the reaction. Relatively small proportions of those experiencing rate related reactions said they limit where they infuse (7%) or avoid switching products (6%) as a result of this experience. Among those who have experienced a rate related reaction to IGIV, 3% said that they have done none of these things, including slowing the rate, as a result of the experience (Figure 65).

Aside from rate related reactions, more than two out of five IGIV using immune deficient patients said that they have had a serious side effect or reaction from IGIV. One in five IGIV users (22%) reported experiencing serious side effects or reactions from IGIV treatment in the past 12 months. Another 22% of patients reported having serious side effects or reactions from IGIV, but not in the past 12 months. Nonetheless, a majority of IGIV users in this population (55%) said that they have never had a serious side effect or reaction from IGIV, aside from rate related reaction (Figure 66).

What do patients consider as serious side effects or reactions from IGIV? Those who have experienced what they consider to be serious side effects most commonly reported headaches (28%), fever (19%), nausea (14%), vomiting (12%) and chills (12%) as the side effect or reaction from IGIV. They also reported shortness of breath (10%), hives (10%), and migraines (9%). Somewhat fewer reported skin rashes (8%), high blood pressure (7%), or joint swelling (7%) as side effects or reactions to their IGIV. Other patients volunteered anaphylaxis (5%), chest pain (5%), back pain (6%), shaking (5%) and fatigue (5%) as the side effects of their IGIV (Figures 67-68).

More than nine out of ten patients who felt that they had experienced a serious side effect or reaction from their IGIV (93%) said that they told their doctor about the side effect or reactions (Figure 69). In about half of these cases, the doctor either gave the patient medicine (54%) and/or slowed the rate of infusion (51%). In a substantial proportion of cases (30%), however, the doctor switched the IGIV product used by the patient. In a small proportion of cases (4%) the doctor reduced the dosage. A minority of patients reported that the doctor told them it was normal (6%) and/or did nothing (8%) when told about the reaction to the IGIV (Figure 70).

A third of patients who had experienced a side effect or reaction to IGIV (34%) indicated that this had occurred when trying a new product for the first time. Only 23% said that it occurred when using a product with no previous problems. About one in ten (12%) said they experienced side effects or reactions when switching back to a product used before (Figure 71).

Since patients may or may not correctly attribute symptoms to product usage, all IGIV using patients were asked whether they had experienced specific symptoms after an IGIV infusion in the past year. The majority of patients (55%) reported experiencing headaches after infusion in the past year. About one in five IGIV users reported fever (22%) or nausea (23%) after infusion in the past year. More than one in ten IGIV using

immune deficient patients (12%) reported shortness of breath after an infusion in the past year. Smaller proportions of patients reported cough (8%), sore throat (7%) or fainting (2%) after an infusion in the past year. Only 37% of patients reported none of these symptoms after infusions in the past year (Figure 72).

Those who acknowledged experiencing side effects in the past year were more likely to report each of the specific symptoms after infusion in the past year. However, it is notable that among those who reported no serious side effects or reactions from their IGIV in the past year, headaches (50%), fever (19%), nausea (16%), and shortness of breath (7%) were reported after IGIV infusion. Indeed, only 52% of those who said that they have had no serious side effects or reactions to IGIV in the past year reported none of these specific symptoms after infusion in the past year (Figure 73).

The majority (55%) of immune deficient patients using IGIV said they tolerate all IGIV products about the same. However, two in five IGIV users (39%) felt that they tolerate some IGIV products better than others (Figure 74). Those who said that they tolerate some products better than others were about twice as likely than others to report fever (33%-21%), nausea (35%-19%), shortness of breath (20%-11%), sore throat (12%-6%) and fainting (4%-1%) after infusion in the past year (Figure 75).

Concerns about product tolerability have caused some patients to switch off one product (8%) or switch to another product (16%). Tolerability concerns have also caused some patients to refuse a product (11%) or delay a scheduled infusion (7%). Nonetheless, although nearly half of patients said that they tolerate some products better than others, seven in ten IGIV using immune deficient patients (70%) said they have done none of these things because of concerns of product tolerability (Figure 76).

Availability

More than nine out of ten IGIV users (92%) in the primary immune deficiency community were aware that there have been shortages in the availability of IGIV in the past few years (Figure 77). Awareness of past IGIV shortage varied with the length of time the patient had been using the product. Almost all (96%) of those using IGIV for four years or longer were aware of the shortage. Awareness of the product shortage declined to 84% among those using IGIV for only three years, and 78% of those using IGIV for only two years. Among those who have been using IGIV for one year or less, only 64% were aware that there was a shortage in IGIV availability in the past (Figure 78).

A majority of current users (54%) reported that they personally have had difficulties in getting their regular IGIV infusions. Ten percent said that their most recent difficulty in getting an infusion was more than three years ago. However, 13% of IGIV using patients reported having difficulty in getting an infusion 2-3 years ago. Another 19% reported a problem within the last 1-2 years. And, 12% of immune

deficient patients who currently infuse IGIV reported having a problem getting their regular IGIV infusion within the past year (Figure 79).

Shortages in product availability have had a measurable effect on the treatment experience of many persons using IGIV for primary immune deficiency diseases. Nearly three out of ten IGIV users reported (28%) having to postpone transfusions as a result of shortages. About one in five patients (19%) reported switching to a less preferred brand as a result of shortages in IGIV supply. Nearly one in six (16%) reported increased time between infusions as a result of shortages. Nearly one in thirteen (6%) reported reduced dosages of IGIV at infusion as a result of shortages. One in seven persons using IGIV for primary immune deficiency diseases (14%) reported having to pay more for the product as a result of shortages. Just half of current IGIV using patients with primary immune deficiency diseases (50%) said they have experienced none of these as result of shortages in IGIV supply (Figure 80).

More than a third of those who personally experienced shortages in IGIV supplies said that these shortages have had negative effects on their health. In total, 20% of all persons still using IGIV for primary immune deficiency diseases reported experiencing negative health effects as a result of shortages. By contrast, 34% of IGIV users experienced shortages in supply, but reported no negative effects on their health as a result (Figure 81).

Those patients who reported negative health impacts of the product shortage were asked to describe what had happened. Most commonly, they reported more infections (35%) and more illness (30%) as a result of shortages. Some patients reported increased fatigue (7%) as a negative health effect of the shortage. Relatively few (8%) reported increased stress and anxiety about treatment as the negative health impact of the shortage. A number of patients (15%) reported that they experienced side effects, presumably as a result of switching to a different brand, as a negative health consequence of the shortage. Five percent of those with a negative health outcome, and one percent of all primary immune deficient patients using IGIV, reported hospitalization as a result of shortages in IGIV supply (Figure 82).

As noted above, more than one in ten (12%) patients reported a problem in getting their regular IGIV infusion in the past year. However, this could reflect issues unrelated to product shortages. Consequently, patients were asked specifically how many times in the past 12 months a scheduled infusion had been cancelled, rescheduled or delayed because the product was not available. Ten percent of IGIV using immune deficient patients reported one or more delays in infusion due to product unavailability in the past 12 months. Four percent of patients reported two or more infusion delays in the past year as a result of product not being available (Figure 83).

The nature and reason for product unavailability was not assessed in the survey. A slightly higher rate of past year problems in product availability was found among those who avoided some products (14%), tolerated some better than others (13%) or had preferences for certain products (11%), compared to those who had none of these types of

product discrimination (7%). Nonetheless, it seems that product availability at time of scheduled transfusion remains a problem for those without product preferences, as well as those who preferred, tolerated better or avoided certain products (Figure 84).

Although relatively few patients were currently experiencing difficulties with IGIV supplies, the majority of IGIV users were concerned about the reliability of IGIV supplies. Indeed, 64% of immune deficient patients using IGIV said reliability of supply is a major concern for them. Another 23% said that reliability of supply is a moderate concern. Only 6% of patients said that reliability of supply is only a minor concern, while only 5% said that it is not a concern for them (Figure 85).

When asked in an open-ended fashion what comes to mind when thinking about reliability of supply, 21% volunteered availability and 16% said no shortages. Many patients thought of reliability in terms of the health consequences of shortages: getting sick (19%), a matter of life and death (4%), and keeping well (3%). Others thought of product consequences of shortages, including safety of products (11%), purity (6%), and cost (3%). A relatively small proportion of patients thought of reliability of IGIV supply, top-of-mind, in terms of profiteering (3%), non immune deficiency uses (1%) and FDA (1%). (Figure 86)

While relatively few patients were currently experiencing difficulties with IGIV supplies, the majority of IGIV users felt that it is likely that there will be another serious shortage in the next two years. Indeed, 26% felt that a serious shortage in the next two years is very likely. Another 41% felt that a serious shortage in the next two years is somewhat likely. Only 12% of IGIV users thought a serious shortage in the next two years is very or somewhat unlikely (Figure 87).

Since future shortages of IGIV were anticipated by most patients, it is not surprising that IGIV users overwhelmingly approved of manufacturers developing more efficient IGIV products. Nearly four out of five patients (78%) using IGIV approved of manufacturers developing new IGIV products that produce more IGIV from the same amount of blood compared to older products. Indeed, 61% strongly approved of the development of more efficient IGIV products. Only 2% of IGIV users actually disapproved of such products, while the remainder was indifferent to them (Figure 88).

Given the expectation of future shortages, it was also not surprising that the overwhelming majority of patients approved of the Immune Deficiency Foundation working with industry to assure an adequate supply of IGIV for persons with immune deficiency diseases. Indeed, 92% of IGIV users strongly approved of IDF working with industry to this end. Another 4% somewhat approved. Less than 1% of patients said that they disapproved (strongly or somewhat) of IDF working with industry to assure an adequate supply of IGIV (Figure 89).

Product Experience

The sample of IGIV users were asked which of eight currently licensed IGIV preparations, plus any other, they had ever used. More than a quarter of current users (29%) reported using only one IGIV product. Another 32% reported lifetime use of only two products. One fifth (19%) had only ever used three products. Less than one in ten (9%) had used four products. And only 5% reported lifetime use of five or more IGIV products (Figure 90).

The number of products used varies somewhat by number of years on IGIV. For example, nearly half of those who have been using IGIV for three years or less (49%) have used only one product. The proportion of single product users dropped to 33% among those who have used IGIV for 4 to 6 years. It dropped to 25% of those using IGIV for 7 to 9 years. However, among those using IGIV for ten or more years, the proportion of single product users remained relatively constant at 21% for the 10-12 year users, 23% for the 13-15 year users, 17% for those 16-20 year users, and 24% of those who have used IGIV for 21 years or more (Figure 91).

Product Differentiation

The IGIV users were asked how much difference they felt there was between IGIV products available in the United States on eight specific attributes. A majority of users felt that there was at least some difference between products in availability (59%), tolerability (55%), price (53%) and side effects (52%). More than four out of ten users felt that there was at least some difference between products on safety margin (46%), purity (46%), infusion rate (44%), and effectiveness/keeping you healthy (43%). Between 20% and 25% of users felt that there was a lot of difference between products on six of these attributes. Somewhat fewer (16%) thought that there was a lot of difference between products in infusion rates. Somewhat more (30%) thought there was a lot of difference between products on price (Figure 92).

The patients were then asked what, based on what they know or have heard, the most important differences were between IGIV products. In answer to this open-ended question, 6% said none and another 15% said that they were not sure. The most commonly volunteered differences between IGIV products were side effects (15%), purity (14%), and production methods (12%). Patients being treated with IGIV also cited tolerability (11%), cost/price (10%), availability (10%), and safety (9%) as important differences between products. Comparatively fewer users said effectiveness (6%) or rate of infusion (2%) was the most important difference between IGIV products, based on what they know or have heard (Figure 93).

The immune deficient IGIV users were also asked which of six factors would be most important to them in deciding to switch to a new FDA approved IGIV product that was recommended by their doctor. A majority of users said both the effectiveness of the

product (57%) and the safety margin of the process (53%) would be the most important to them in deciding whether or not to switch to a new product. The tolerability of the product (42%) was third in importance in terms of switch to a new product. A quarter of current users (25%) indicated that the reliability of supply was one of the most important factors to them in switching to a new product. By contrast, cost (16%) and ease of preparation (9%) were among the most important factors in product decisions for relatively few users (Figure 94).

Nearly six out of ten immune deficient IGIV users (58%) reported that they did have preferences among IGIV products (Figure 95). Those who had preferences among IGIV brands were asked why they preferred those products. Overall, side effects were the most often volunteered reason (30%) for preferring certain brands over others. Tolerability (12%) was the reason given second most often for preferring certain products. One in ten (9%) with product preferences said that it was the only product they have ever tried. Effectiveness (9%) or the product simply works well (6%) was given by other users with a preference among IGIV products. The convenience of a pre-mixed form (3%), safety (3%) and availability (3%) were comparatively rare reasons for preferences among specific brands or manufacturers (Figure 96).

IGIV users were asked if there were IGIV products they try to avoid. Although a majority of users said that they had preferences among products, only one third (34%) indicated that there were any licensed products that they try to avoid (Figure 97).

Users who avoided certain IGIV brands were asked why they avoided those products. Side effects are volunteered by more than half of those who avoided certain products (53%) for avoiding those products. By contrast, doctor's recommendation (4%), effectiveness (4%), purity (4%), company reputation (3%), safety (3%), tolerability (3%) and recalls (2%) were given as reasons for avoiding certain products by a relatively small proportion of users who avoided certain IGIV products (Figure 98).

Current Product and Product Satisfaction

When asked how often the patient gets the IGIV product that is currently being infused, 65% say always. Another 20% say they get the specific product most of the time. Relatively few say that they get the specific product they infused most recently only some of the time (3%), occasionally (1%), or this is the first time using the product (1%). More than one in ten users (10%) did not answer the question, approximately the proportion who does not know which product they infused (Figure 99).

One reason for not switching to new products was a high level of satisfaction with the current product among IGIV users. Nearly two thirds (65%) said that they were very satisfied with their current product. Another 21% said that they were somewhat satisfied with their current IGIV product. By contrast, only 3% said that they were dissatisfied with their current product, while another 4% were neither satisfied nor dissatisfied with the product (Figure 100).

Choice in IGIV Products

The majority of immune deficient patients using IGIV (57%) said that the doctor was primarily responsible for the selection of the IGIV product that the patient used. Another 12% said that the medical plan or provider was primarily responsible for the selection of IGIV products. Only 7% of IGIV users reported that the patient was primarily responsible for the selection of the specific product (Figure 101).

Patients using IGIV for their immune deficiency were divided in how much choice they felt their doctor or health plan had among specific IGIV products. More than a quarter (29%) felt their doctor or health plan had complete choice in products. An equal number (29%) felt that their doctor or health plan had no choice among IGIV products. And, nearly a third (31%) felt that their doctor or health plan had some choice among specific IGIV products (Figure 102).

Nearly seven out of ten patients using IGIV (69%) said that they usually stick with one particular IGIV product. Another 11% of patients reported using several specific products. Only 11% reported that they use whichever products are available (Figure 103).

IGIV users were asked what their provider would be most likely to do if a particular product was not available at the time of a scheduled transfusion. A third (33%) thought that their provider would simply substitute an equivalent without asking them about their preference. Nearly a quarter (22%) felt that their provider would be most likely to ask them their preference, if their normal product was not available. However, 16% of IGIV using immune deficient patients thought that their provider would most likely delay the infusion to get the regular product. One in ten (10%) felt their provider would pursue some combination of these options if the particular product was not available. Nearly one in five (19%) were not sure or didn't answer the question (Figure 104).

There was some difference in how a provider was expected to react, if a particular IGIV product was not available, based on infusion location. About the same proportion of patients expected that their provider would simply substitute an equivalent product, if they usually infused at an infusion suite (32%), in a hospital clinic (34%) or a doctor's office (32%). However, those who usually infused in a hospital outpatient department were more likely to expect their provider to simply substitute an equivalent product (42%) without asking their preference (Figure 105).

These same IGIV users were asked what they would be most likely to do if their usual product was not available at the time of a scheduled transfusion. The sample was almost evenly divided between the three response options. A third (33%) said that they would accept any licensed product. Nearly a third (31%) would accept only certain products as substitutes. And, more than a quarter (28%) of IGIV using immune deficient

patients said that they would be most likely to delay the infusion to get the regular product. Less than one in ten (8%) were not sure what they would do or didn't answer the question (Figure 106).

There was relatively little difference between those who had ever suffered a rate related reaction (29%) and those who had not (26%) in the likelihood of delaying an infusion if their usual product was not available. Similarly, there was little difference between those who had ever suffered serious side effects from an IGIV product (30%) and those who had not (26%) in the willingness to delay a scheduled infusion when their usual product was not available. But those who had a preference among IGIV products were twice as likely to delay an infusion (35%) than those who did not have a product preference (18%). And, those who tried to avoid certain IGIV products (42%) were also twice as likely as those who did not try to avoid certain products (20%), to delay a scheduled infusion if their usual product was not available (Figure 107).

Willingness to Switch

Although most IGIV users said that they always got their current product, most were willing to switch to a new product that was simply equal to their product on effectiveness, safety, tolerability, reliability and cost. Indeed, 30% said that they would be very willing to switch to a new, equivalent product. Another 44% would be somewhat willing to switch to the new product. By contrast, 13% said that they would be somewhat unwilling to switch, and 8% would be very unwilling to switch (Figure 108).

The survey suggests that immune deficient patients currently using IGIV gave price a relatively low priority, compared to other product attributes, in product switching decisions. A majority of current users said that they would probably switch to a new IGIV product at a somewhat higher price if it had any of eight feature improvements. The percent of current users who said they would probably switch to a new product at a somewhat higher price increased from 60% with a faster rate, 65% for fewer side effects, 70% for better tolerability, 71% for fewer infections, 71% for guaranteed availability, to 74% for greater purity, and 76% for better safety margin. The proportion who said that they would definitely switch to a new product at a somewhat higher price increased from 32% for faster rate and 35% for fewer side effects to 49% for better safety margin (Figure 109).

However, when asked what feature of a new IGIV product would be most likely to make you switch to it from your current product, a more mixed picture emerges. Safety (27%) was the most commonly volunteered reason for switching to a new product. However, side effects (23%) were a relatively close second among top-of-mind reasons for switching. Then, purity (13%), effectiveness (13%), rate of infusion (12%), and cost (11%) were given by approximately the same proportion of IGIV users as the feature most likely to make them switch to a new product. Easier administration (2%) and convenient form (1%) were clearly not features that, all other things being equal, were likely to cause switching to new products among this population. However, it appears

that some features appeal more than others to certain segments of these consumers (Figure 110).

The immune deficient patients currently being treated with IGIV for their condition were asked their reaction to replacing their current product with an equal or better product from the same manufacturer. The majority of patients (54%) said that they would be pleased if their current product was replaced by an equal or better product from the same manufacturer. Indeed, 29% said that they would be very pleased. Another 30% said that they would be neither pleased nor displeased by the replacement of their current product by an equal or better product from the same manufacturer. Only eleven percent of users said that they would be very unhappy (4%) or somewhat unhappy (7%) to have their current product replaced by an equal or better product, even from the same manufacturer (Figure 111).

All patients were asked how long they would insist their current product be kept available for transition, if the manufacturer was replacing it with a new improved product. A majority (56%) said it should be kept available for six months or less for their transition to the new product or another product. A transition period of one year met the requirements of nearly three quarters (73%) of the IGIV using patients. Indeed, only 14% indicated that they would insist on a transition period of longer than one year (Figure 112).

New IGIV Products

The immune deficient patients using IGIV products were asked about their understanding of what must be demonstrated for the approval of a new IGIV product by the FDA. Three quarters of patients (76%) selected the correct response --- the manufacturer must demonstrate that a product is both safe and effective before it can be sold in the United States. A minority (13%) thought that the product only had to be demonstrated to be safe, to be sold in the U.S. An even smaller minority (2%) thought that the manufacturer only had to demonstrate that the product was effective to be licensed. Only 3% of IGIV users felt that the government did not require manufacturers to demonstrate either safety or effectiveness of an IGIV product before it could be sold in the United States (Figure 113).

Nearly two thirds of IGIV using immune deficient patients (65%) expected that new IGIV products would be more effective than currently licensed products. Indeed, 30% expected new products to be a lot more effective than currently licensed IGIV products. About a third (31%) expected new IGIV products to be about equally effective as currently licensed products. Only 1% of IGIV users expected new products to be less effective than currently licensed products (Figure 114).

Almost three quarters of IGIV using immune deficient patients (73%) expected that new IGIV products would be safer than currently licensed products. Indeed, 41% expected new products to be a lot safer than currently licensed IGIV products. About a

quarter (24%) expected new IGIV products to be about equally safe as currently licensed products. Less than 1% of IGIV users expected new products to be less safe than currently licensed products (Figure 115).

New IGIV processes, as well as products, were seen as having a distinct advantage compared to older ones. When asked to compare the safety of manufacturing processes between newly approved products, and those that are currently on the market, two thirds of users (67%) expected the new processes to be safer than the older processes. Indeed, 38% of users expected new processes to be a lot safer than older processes. Only 21% of IGIV users thought the processes for newly approved products would be about as safe as products currently on the market. Only seven percent of users expected the processes of newly approved products to be less safe than those already on the market (Figure 116).

Although patients expected that the processes for newly approved products would be safer than those used by products already on the market, they did not prefer new plants for their products. When asked if they would prefer to get their product from a plant that had been producing IGIV for fifty years, or a newly designed plant producing IGIV for a year, nearly half (46%) said that they would prefer the existing plant, even though both had been inspected and approved by the FDA. Only 12% of IGIV users said that they would prefer the new plant over the existing plant. However, 36% of IGIV users said that they would have no preference between old and new plants that had been inspected and approved by the FDA (Figure 117).

The sample of IGIV users were asked if they would be willing to try a new FDA approved IGIV product from each of the nine current or past IGIV manufacturers. Only a quarter (25%) of IGIV users failed to indicate they would be willing to try a new product from any of the manufacturers. Fifteen percent indicated their willingness to try a new product from only one of the manufacturers. Another ten percent were willing to try new products from two and nine percent from three manufacturers. Sixteen percent indicated their willingness to try new products from four to eight of the manufacturers. A quarter (25%) indicated that they would be willing to try a new FDA approved IGIV product from any of the nine manufacturers (Figure 118).

Cost and Coverage

Most persons with primary immune deficiency diseases being treated with IGIV had some form of health insurance coverage. The majority (69%) had insurance through an employer group policy. Another 6% belonged to another group policy, while 9% had an individual policy. Only 19% had Medicare coverage, while 10% had Medicaid coverage, and 3% were covered by a state or county health program. Three percent had health coverage through COBRA. Seven percent reported other forms of coverage, such as military or veterans. Less than one percent reported none of these (Figure 119).

The immune deficient patients on Medicare faced certain regulations concerning treatment that those with other forms of health coverage did not. Consequently, it is important to note that four out of five immune deficient patients on Medicare (80%) had other forms of health insurance or health coverage as well. Indeed, less than four percent of patients with primary immune deficiency disease were exclusively dependent upon Medicare for their health care coverage.

Conclusions: Immune Deficient Patients using IGIV

The recognition of primary immune deficient diseases and the treatment of those diseases with immunoglobulin replacement therapy are barely fifty years old. The value of this therapy and its refinement in intravenous immunoglobulin (IGIV) is demonstrated by a relatively large, and relatively healthy population of patients with primary immune deficiency diseases in the United States. Although not designed as an epidemiologic survey, the survey identified relatively few cases of mortality between 1997 and 2002 among the old patient population.

Nonetheless, the survey found that patients with primary immune deficiency diseases, being treated with IGIV, were less healthy than other persons of the same age in the general population. Two out five immune deficient patients being treated with IGIV reported that their current health was only fair, poor or very poor. Although more than three-quarters had not been hospitalized in the past year, the rate of hospitalization was twice as high as the general public, even with IGIV therapy.

At least some of the health limitations among this patient population stem from late diagnosis and delayed treatment. Previous research has demonstrated that time to diagnosis from symptoms averages more than eight years in this population. The rate of permanent functional impairment as a result of disease increases in this population with the length of time between symptoms and diagnosis. Treatment with IGIV to prevent infections cannot correct chronic conditions caused by infection prior to treatment.

The value of early diagnosis and treatment to the immune deficient patient and his/her family cannot be underestimated. The survey data suggests that there is also a strong reason for industry to support IDF efforts in improving early diagnosis. The average time between symptom onset and first diagnosis for patients who are currently treated with IGIV is approximately the same as the average treatment time on IGIV since diagnosis. This means that early diagnosis could potentially double the market for IGIV among immune deficient patients alone.

Aside from problems of delayed diagnosis, the survey raises questions about the standards for treatment among patients diagnosed with primary immune deficiency diseases. Thirteen to fourteen percent of patients with primary immune deficiency diseases, and fifteen to eighteen percent of those ever treated with IGIV, had discontinued treatment by the time of the survey. Some of these had discontinued treatment for good reasons --- misdiagnosis, treatment with an alternative form of immunoglobulin replacement therapy, or successful treatment of the underlying immune deficiency with bone marrow transplantation. Others had a specific immune deficiency disease where the effectiveness of IGIV therapy is more problematic. However, many had a diagnosis for which IGIV is recognized as the therapy of choice, but nonetheless discontinued treatment for reasons other than medical indication.

Even among those currently being treated with IGIV, there seems to be a wide range of treatment practices. Some get infusions every two weeks, while others get

infusions every five weeks, with most getting their infusions every three or four weeks. The average number of grams infused does not vary with frequency of infusion. Overall, the dosage rate for IGIV compared to body weight falls within currently accepted standards. Nonetheless, a significant proportion of patients report current dosage rates below those standards.

The patient reaction to IGIV demonstrates that this product is not a commodity. Nearly three out of ten (29%) have had what they consider to be a rate-related reaction to IGIV in the past year. One in five (22%) have had what they consider to be a non-rate related serious side effect or reaction to IGIV in the past year. The majority (63%) reported one or more of a list of symptoms after infusing IGIV in the past year.

The reactions reported by patients to their IGIV may not meet medical standards for serious side effects. They may be related to rate or product purity or some other factor. Nonetheless, these reactions are widespread in this patient population and they affect the health and quality of life of these persons.

Not surprisingly, then, many patients discriminated among IGIV products. The majority of IGIV users thought there was either a lot (24%) or some (31%) difference between IGIV products in their tolerability. Almost the same proportion said that there was either a lot (21%) or some (31%) difference between IGIV products in side effects. Two out of five IGIV users (39%) said that they tolerated some products better than others. Almost three out of five (58%) said that they had a preference for specific IGIV products. A third (34%) said that they tried to avoid using certain IGIV products. This degree of discrimination among products is particularly notable because 29% of IGIV users have only used one product.

While only 7% of immune deficient patients currently treated with IGIV thought IGIV controls their condition less than adequately, there was a wide sense that some products may be more effective than others. Only 23% of IGIV users believed that there is a lot of difference between products in keeping you healthy, but another 20% think that there are some differences in effectiveness among products. The hallmarks of non-effectiveness of IGIV for most patients were frequent illnesses or infections, rather than specific symptoms, infections, or serious illnesses. A quarter of patients have switched or refused a product because of concerns about effectiveness.

The safety of the product that they infused is a major concern for nearly half (46%) of patients with primary immune deficiency diseases who currently infuse IGIV. Safety in IGIV means the risk of disease in general or specific transfusion transmitted diseases (HIV, HCV, CJD, etc.) to most of these patients. Over a quarter of patients (28%) feel that there is a moderate or strong risk of getting hepatitis from an IGIV infusion. Although only 22% of IGIV users believed that there was a lot of difference between products in their safety margin, another 24% thought that there were some differences in safety margin among products. Nearly one in five IGIV users said that they have switched or refused a product because of concerns about product safety.

Availability is an important criterion for IGIV users, along with effectiveness, safety and tolerability. There is almost universal awareness of the IGIV shortage (96%) among persons who were using these products four or more years ago. Even among those who have started using IGIV since the shortage, the majority is aware that there have been shortages in the recent past. More than half (54%) were personally affected by the shortage. One in five current IGIV users (20%) reported that they suffered negative effects on their health from the shortage.

One in ten current IGIV users reported having a scheduled IGIV infusion cancelled, rescheduled or delayed because the product was not available in the past year. The majority of IGIV users felt that there was a lot (26%) or some (33%) difference among products in availability. Moreover, more than two-thirds of IGIV users (67%) felt that another serious shortage of IGIV in the next two years was likely.

Nearly all IGIV users (96%) approved of the Immune Deficiency Foundation working with industry to assure an adequate supply of IGIV for persons with immune deficiency disease. However, the survey found an equally, if not more important role of the foundation in providing information to patients about the safety and efficacy of IGIV therapy and products. When asked to identify the most reliable sources of information about the safety and efficacy of these products, more than half (58%) said patient organizations, generally, or IDF specifically. For patients with primary immune deficiency diseases who use IGIV, patient organizations rank second only to their own doctor (72%) as the most reliable source of information on this therapy and these products.

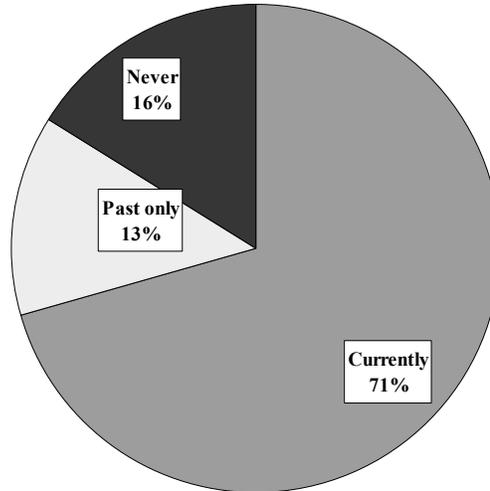
**Treatment Experiences
and Preferences
of
Patients with
Primary Immune Deficiency Diseases**

(Figures)

Conducted for the Immune Deficiency Foundation
by
Schulman, Ronca and Bucuvalas, Inc.

June 20, 2003

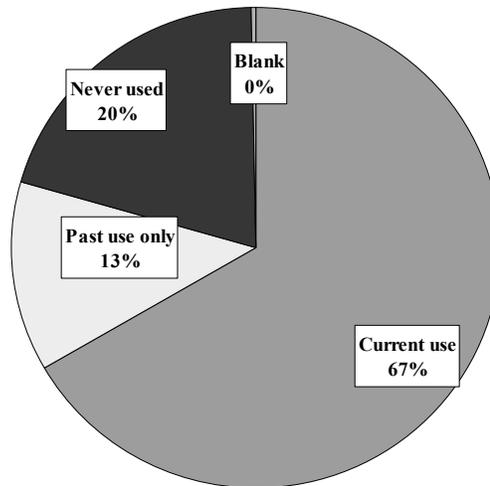
Current Use of IGIV: Old Patients



Q10a. Is he/she currently being treated with intravenous gammaglobulin (IGIV)? N=759

1

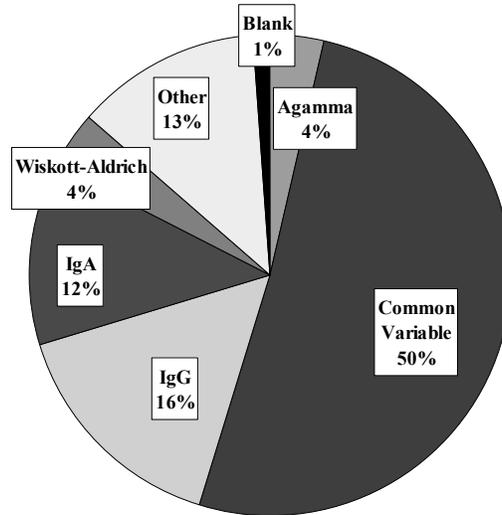
Current Use of IGIV: New Patients



Q31. Has he/she ever been treated with intravenous gammaglobulin (IGIV) on a regular basis. Q33a. Is he/she currently being treated with intravenous gammaglobulin (IGIV) for his/her immune deficiency disease? (Base: N=1,526)

2

Discontinuing Users: Diagnosis



Q3. What is the current diagnosis of that person's immune deficiency disease?
N=199 (Base: New patients)

3

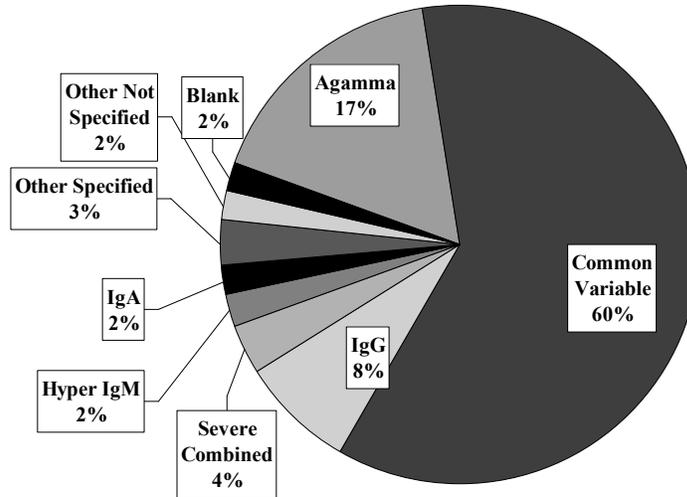
Reason No Longer Using IGIV

- Insurance /coverage 14.3%
- Side effects/reaction 12.4%
- Health improved/symptoms gone 12.4%
- Normal/near normal levels 11.8%
- Doctor doesn't think it is necessary 9.9%
- No real benefits 8.7%
- To see if body will produce antibodies 8.1%
- Can't afford/too expensive 5.0%
- Cured/Bone marrow transplantation 4.3%
- Fear of contracting diseases 3.1%
- Lack of product 2.5%
- Transient disease .6%
- Can't get a good vein/port .6%
- Other reasons 15.5%

Q33b. Why is the patient no longer being treated with IGIV?
(Base: Past users who gave reason - N=161 New Patient Survey)

4

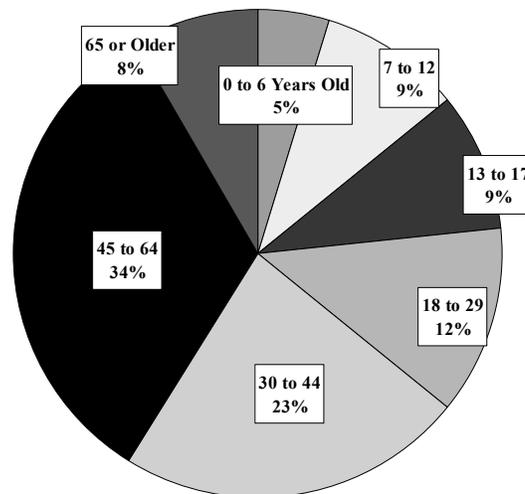
Current Users: Diagnosis



Q3. What is the current diagnosis of that person's immune deficiency disease? N=1186

5

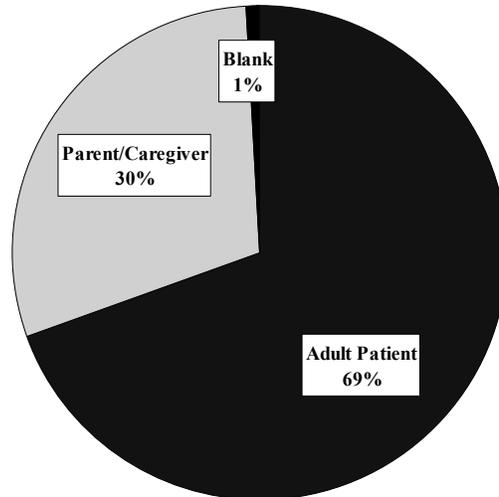
Current IGIV Users by Age



Q2. What is the date of birth of the PID patient being treated with IGIV? N=1186

6

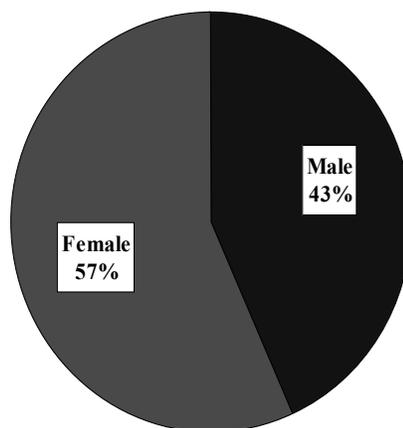
Current IGIV Users: Parents/Patients



Q1. Are you completing the survey as an adult patient with a primary immune deficiency disease or as the parent/caregiver of a child with a primary immune deficiency disease? N=1186

7

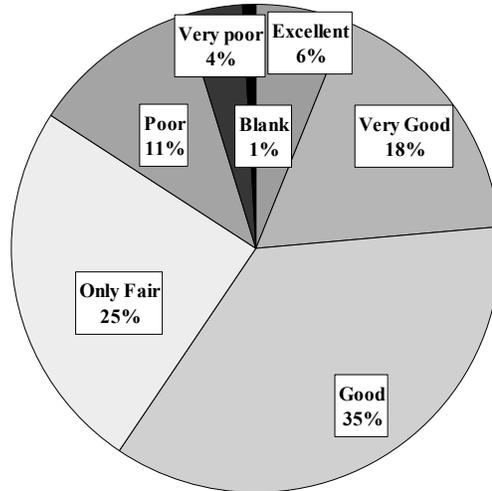
Gender of Patient



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

8

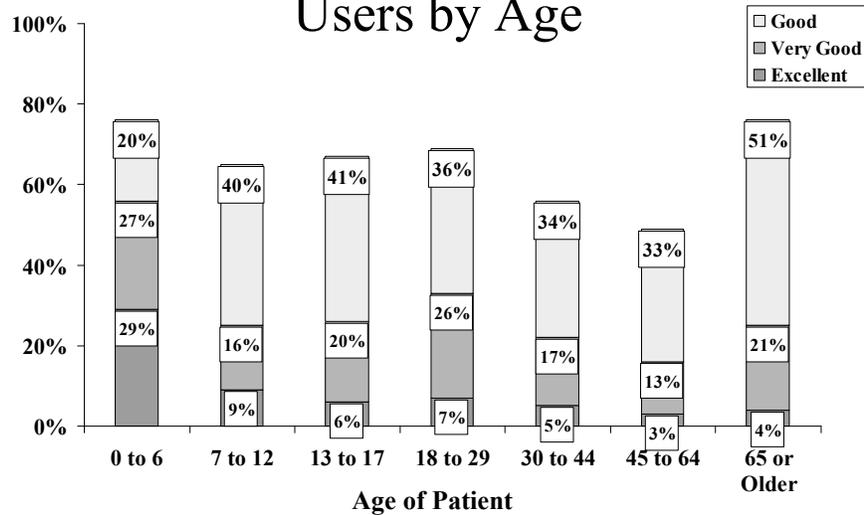
Current Health Status: IVIG Users



Q4. Compared to other persons of the same age, would you describe his/her health as?
N=1,186

9

Current Health Status of IGIV Users by Age

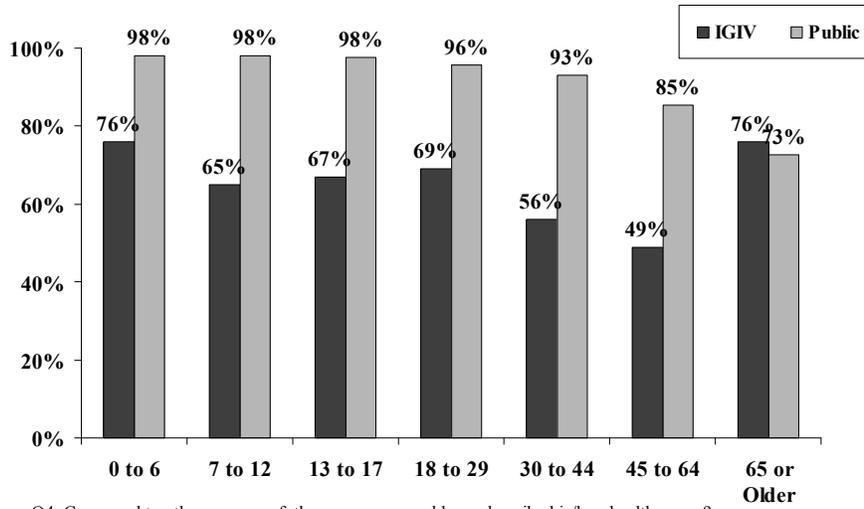


Q4. Compared to other persons of the same age, would you describe his/her health as? N=1177

Q2. What is the date of birth of the PID patient being treated with IGIV?

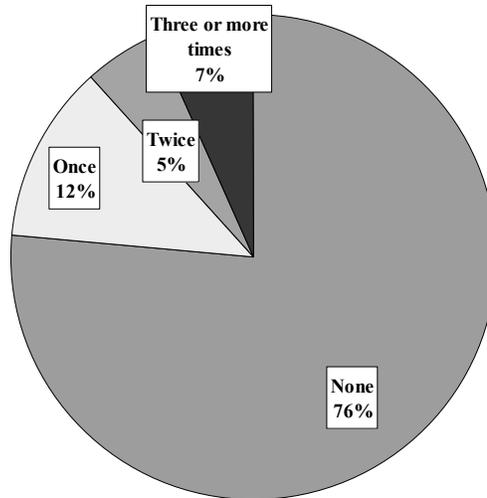
10

Health Status Good or Better



11

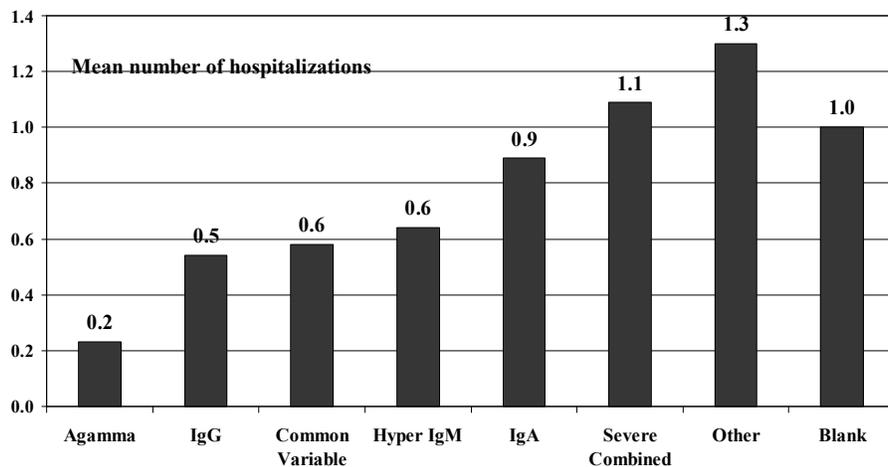
Past Year Hospitalization



Q5. How many times, if any, has he/she been hospitalized overnight or longer in the past 12 months?
N=1,186

12

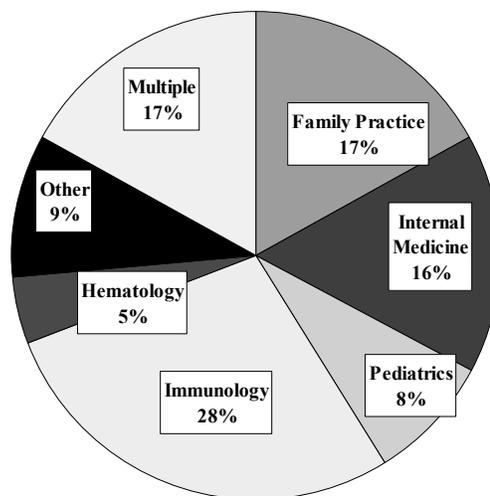
Past Year Hospitalizations by Diagnosis



Q5. How many times, if any, has he/she been hospitalized overnight or longer in the past 12 months?
N=1184

13

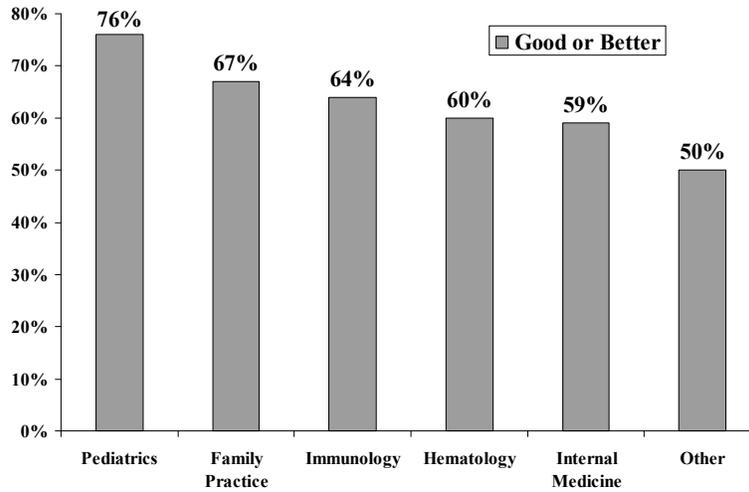
Type of Doctor Seen Most Often



Q7. What kind of doctor does the patient see most often for his/her health care? N=1,186

14

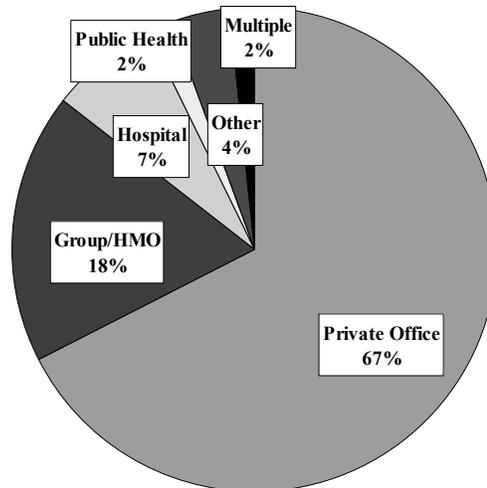
Good Health by Type of Doctor



Q7. What kind of doctor does the patient see most often for his/her health care? N=1,186

15

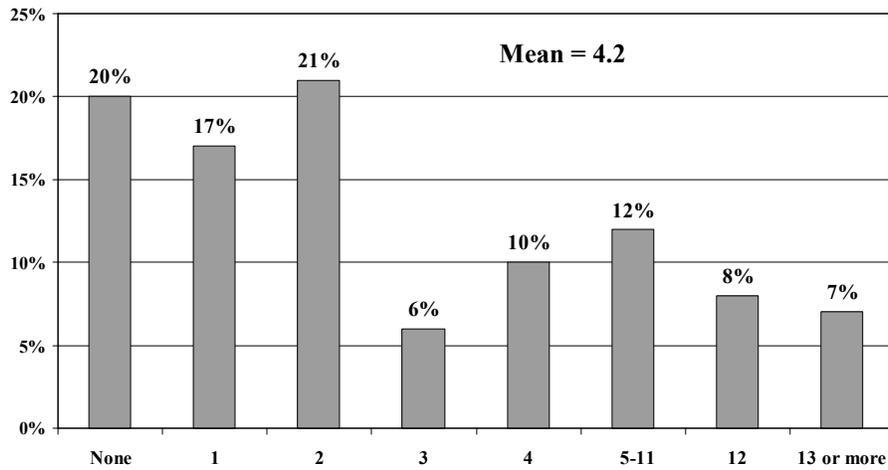
Location of Primary Doctor



Q8. Where does the patient usually visit his/her primary doctor? N=1,186

16

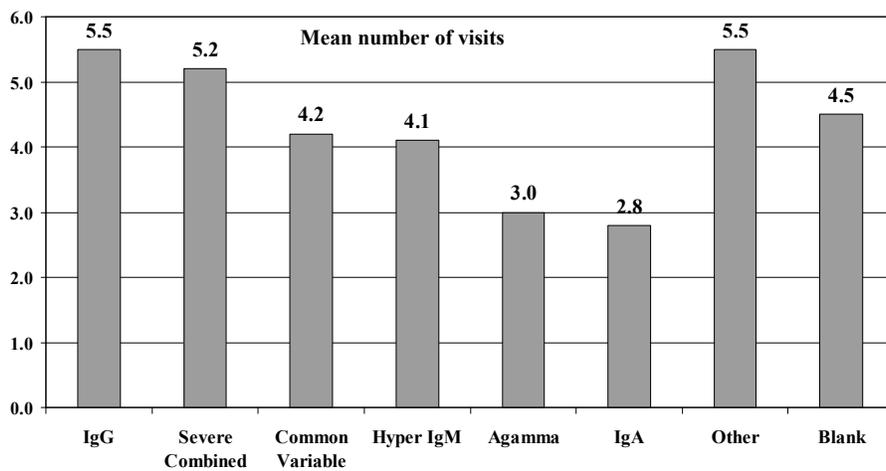
Immunologist Visits in Past Year



Q6. How many times, if any, has he/she been seen by an immunologist in the past 12 months?
N=1162

17

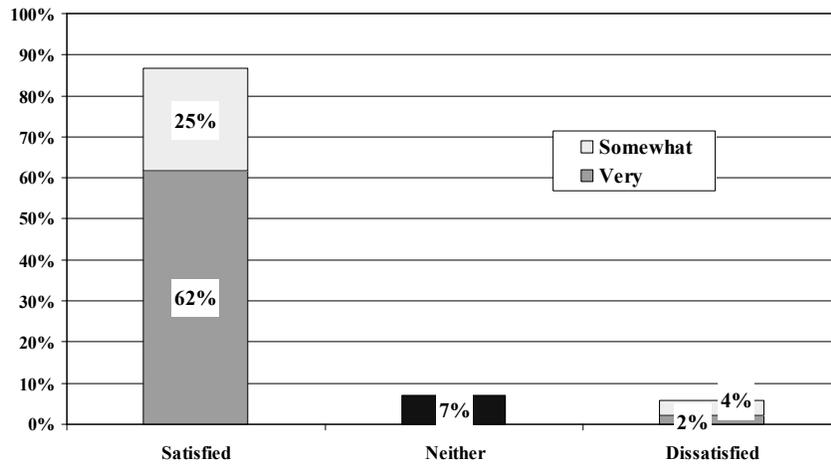
Immunologist Visits by Diagnosis



Q6. How many times, if any, has he/she been seen by an immunologist in the past 12 months? N=1162

18

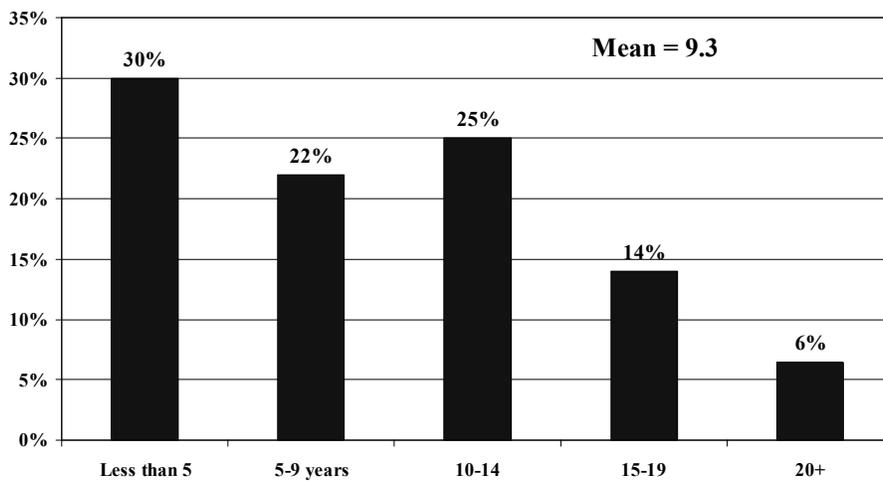
Patient Satisfaction with Doctor's Management of Condition



Q9. Overall, how satisfied are you with the primary doctor's management of the patient's primary immune deficiency disease? N=1,186

19

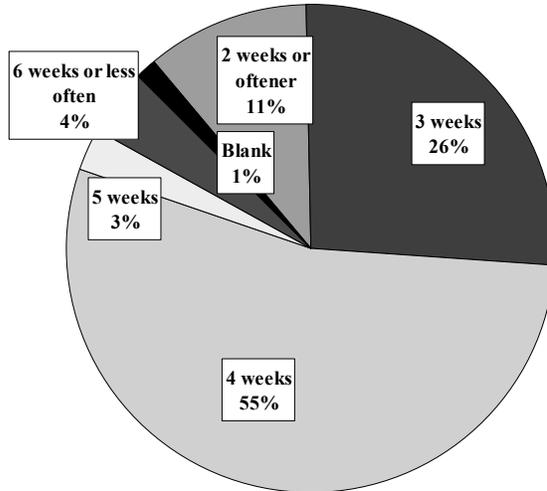
Number of Years on IGIV



Q11. How many years has the patient taken IGIV on a regular basis for his/her immune deficiency? N=1170

20

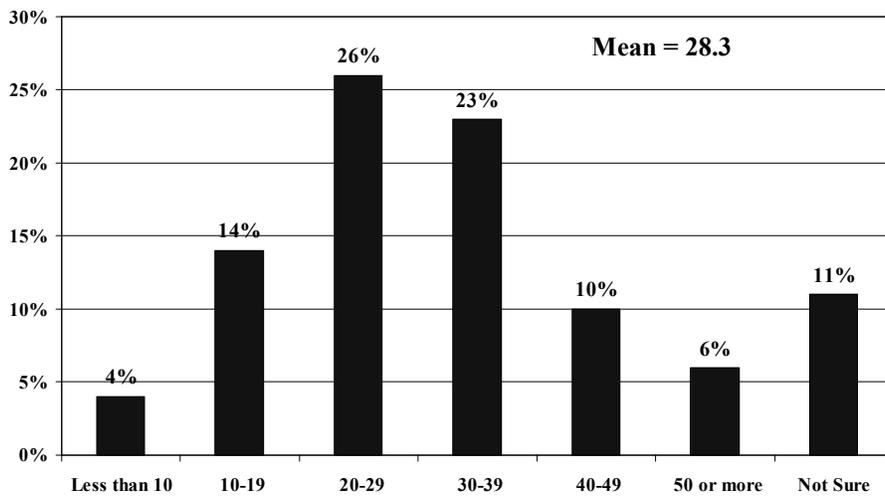
Frequency of IGIV Infusions



Q12. On average, how often does he/she get an infusion of IGIV? N=1,170

21

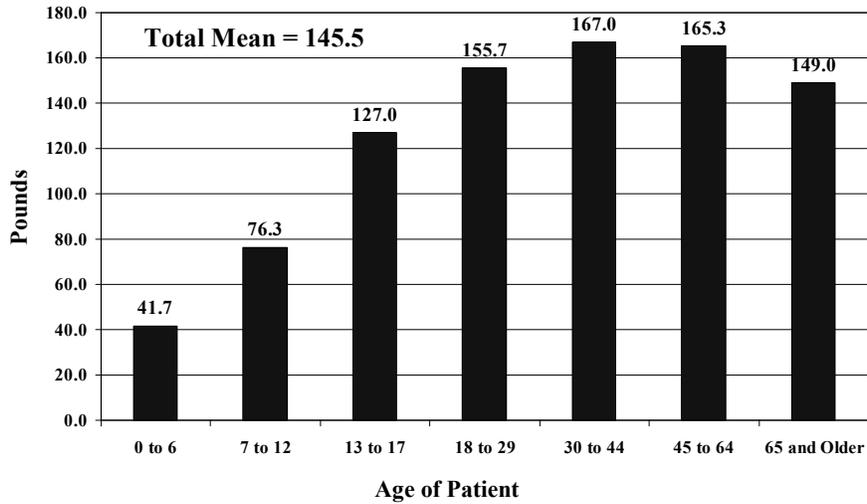
Grams of IGIV per Infusion



Q14. About how many grams of IGIV per infusion does he/she normally receive? N=1170

22

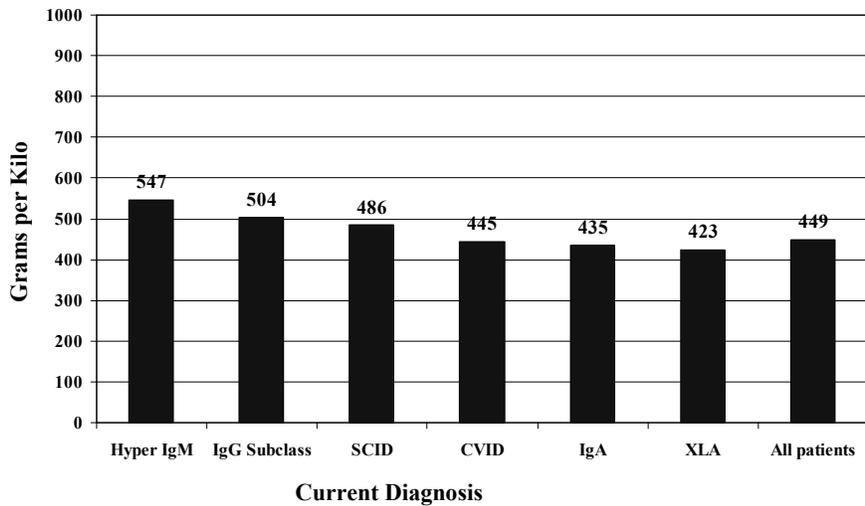
Average Weight of Patient



Q13. About how much does he/she weigh? N=1170

23

Average IVIG Dosage by Diagnosis



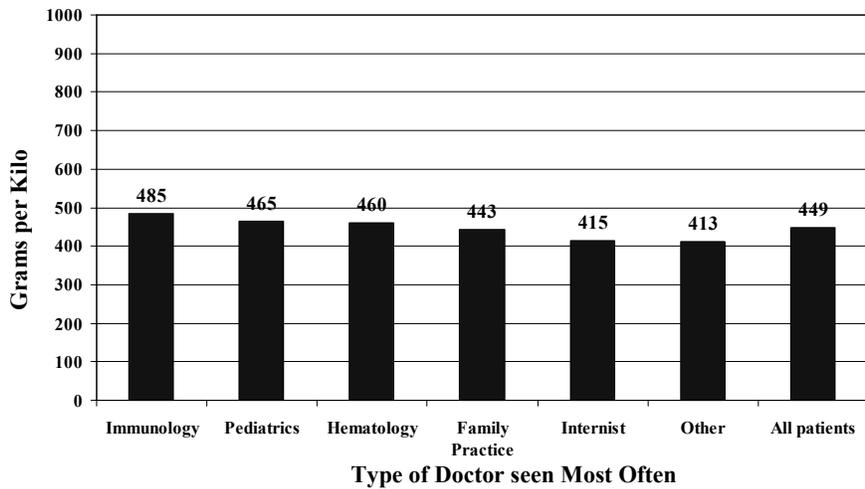
Q3. What is the current diagnosis of that person's immune deficiency disease?

Q13. About how much does he/she weigh?

Q14. About how many grams of IGIV per infusion does he/she normally receive? N=985

24

Average IVIG Dosage by Specialty



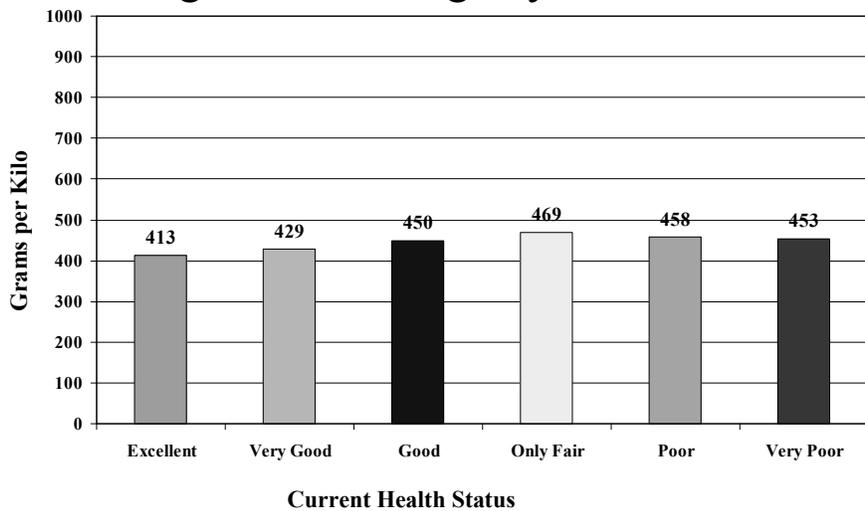
Q7. What kind of doctor does the patient see most often for his/her health care?

Q13. About how much does he/she weigh?

Q14. About how many grams of IGIV per infusion does he/she normally receive? N=985

25

Average IVIG Dosage by Health Status



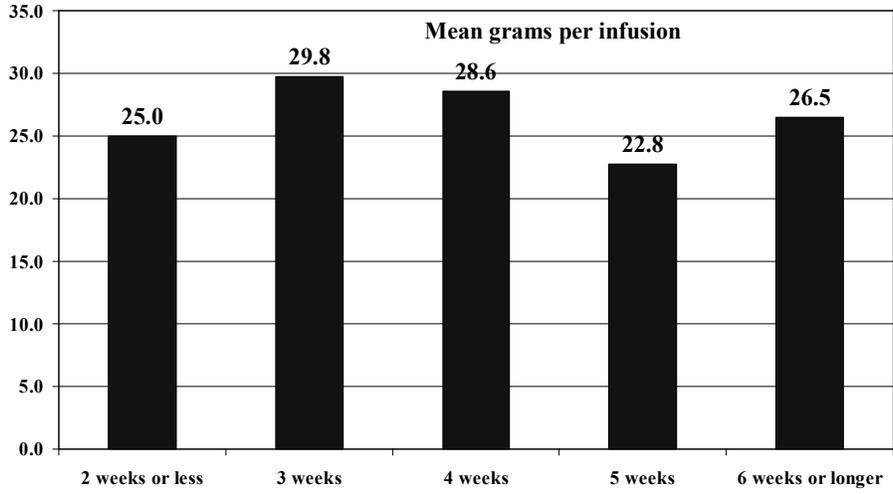
Q4. Compared to other persons of the same age, would you describe his/her health as?

Q13. About how much does he/she weigh?

Q14. About how many grams of IGIV per infusion does he/she normally receive? N=985

26

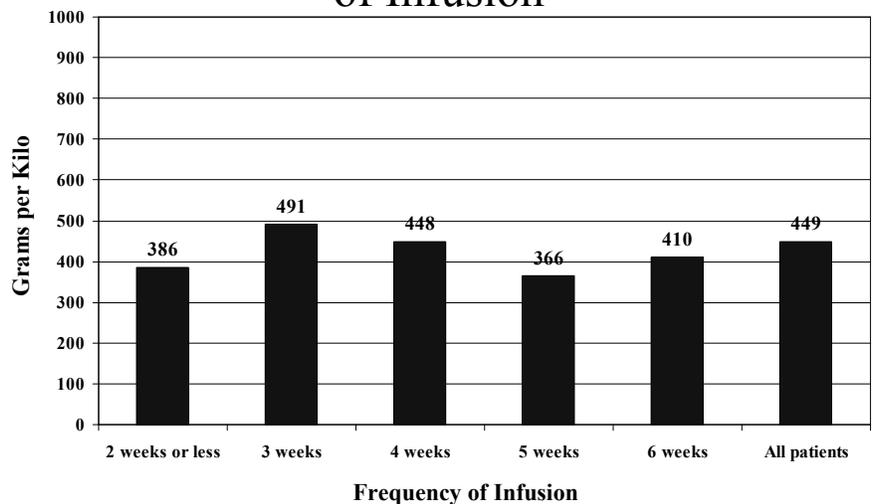
Grams Infused by Frequency of Infusion



Q12. On average, how often does he/she get an infusion of IGIV?

Q14. About how many grams of IGIV per infusion does he/she normally receive? N=989

Average IVIG Dosage by Frequency of Infusion

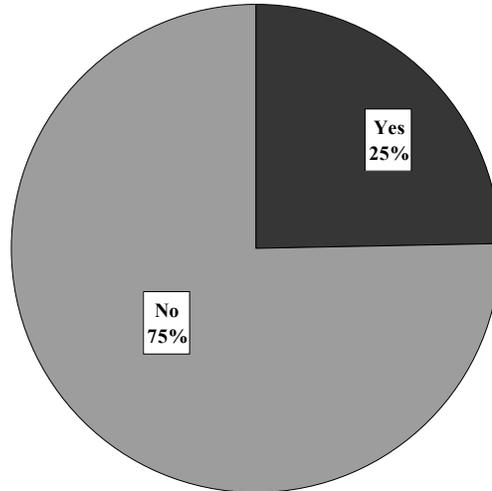


Q12. On average, how often does he/she get an infusion of IGIV?

Q13. About how much does he/she weigh?

Q14. About how many grams of IGIV per infusion does he/she normally receive? N=985

Larger or More Frequent Doses in Past

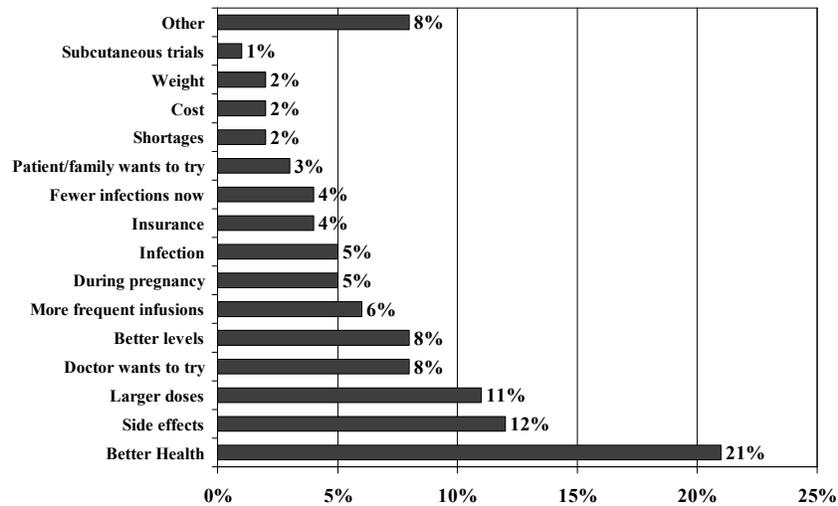


Q15a. Has he/she ever had larger or more frequent doses of IGIV on a regular basis?

N=1143

29

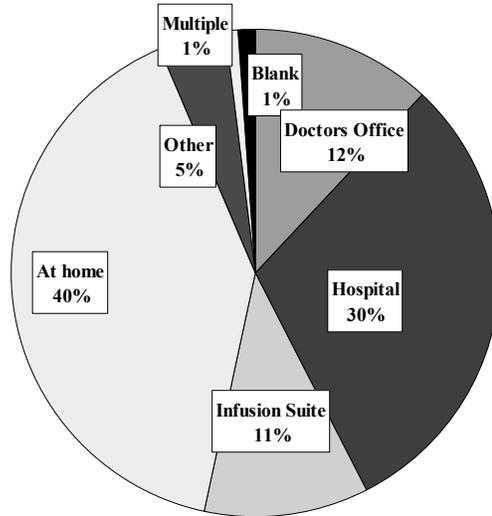
Reason Current Dose/Frequency is Less



Q15b. Why is he/she receiving smaller doses or less frequent infusions now? N=202

30

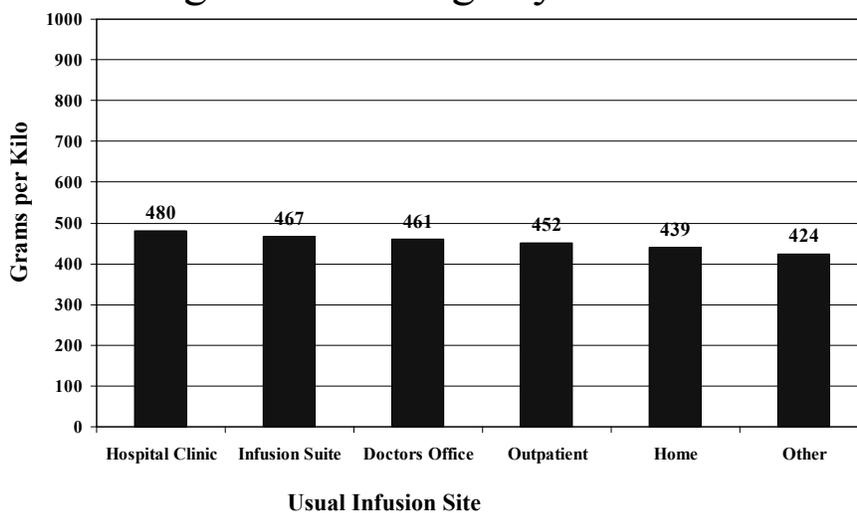
Usual Location for Infusion



Q16. Where does the patient usually receive his/her infusions? N=1170

31

Average IVIG Dosage by Infusion Site



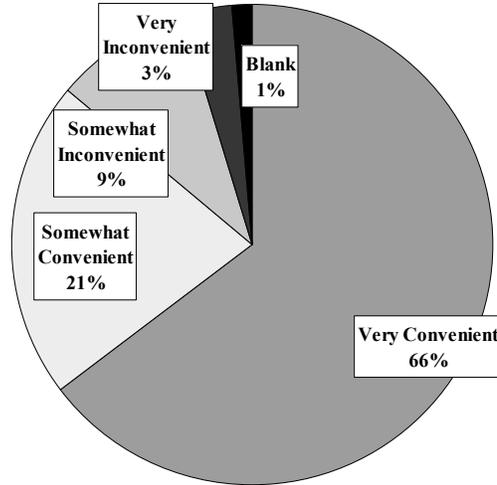
Q16. Where does the patient usually receive his/her infusions?

Q13. About how much does he/she weigh?

Q14. About how many grams of IGIV per infusion does he/she normally receive? N=985

32

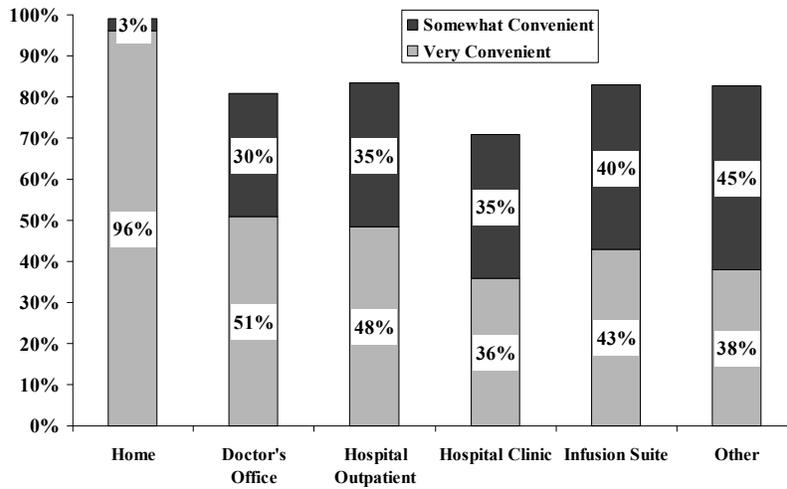
Convenience of Infusion Location



Q17. How convenient is the location of that infusion site? N=1170

33

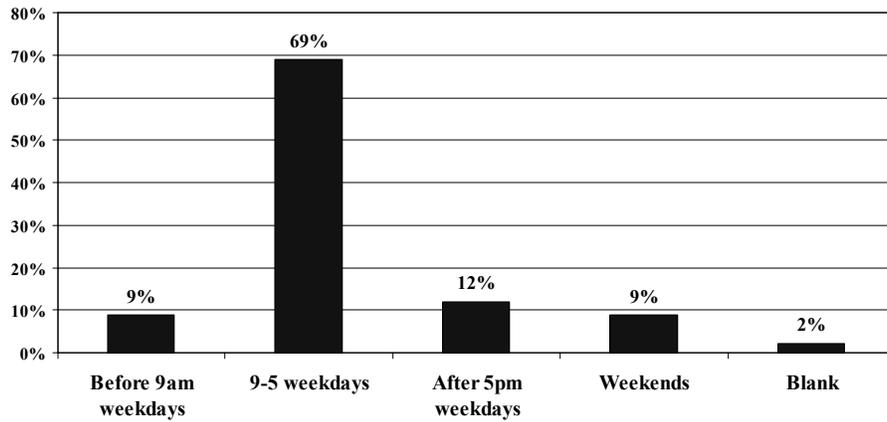
Convenience of Infusion Location



Q17. How convenient is the location of that infusion site? N=1170

34

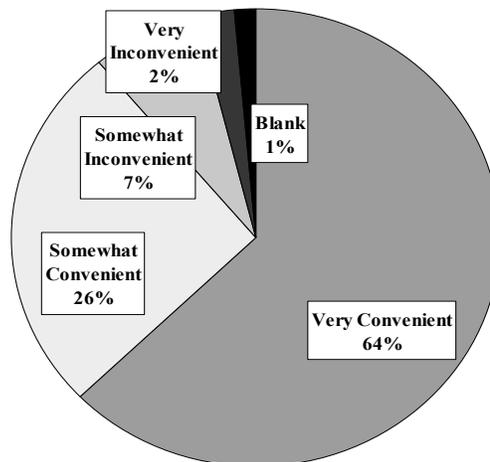
When Does Patient Get Infusion?



Q18. When does the patient usually receive his/her infusions? N=1170

35

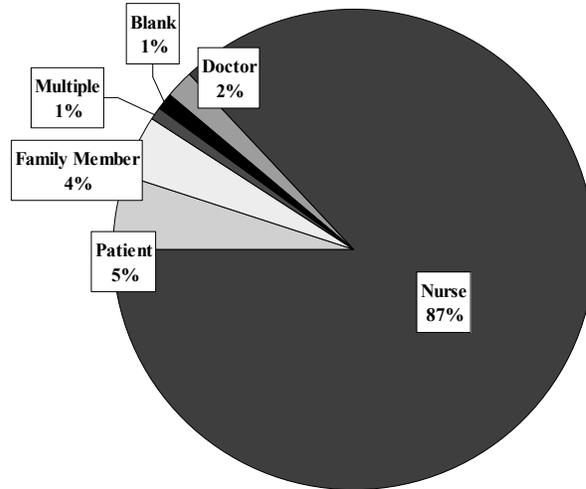
Convenience of Infusion Time



Q19. How convenient are the times he/she can get an infusion? N=1170

36

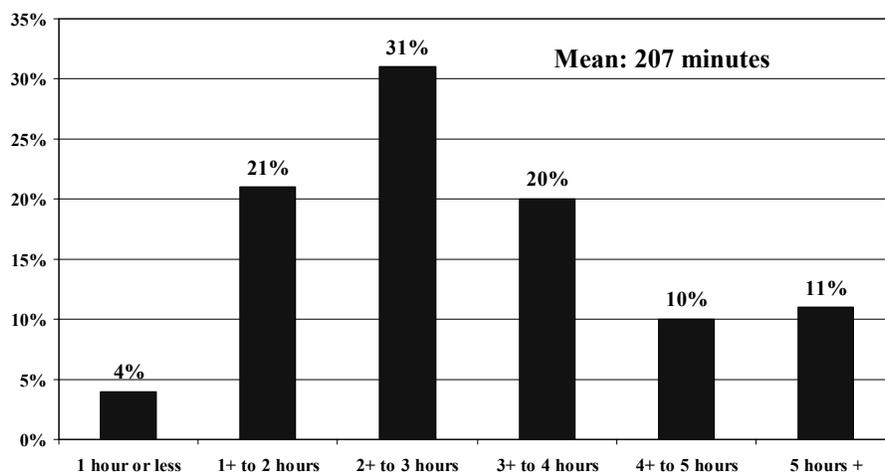
Who Administers the Infusion?



Q20. Who usually administers the infusions? N=1170

37

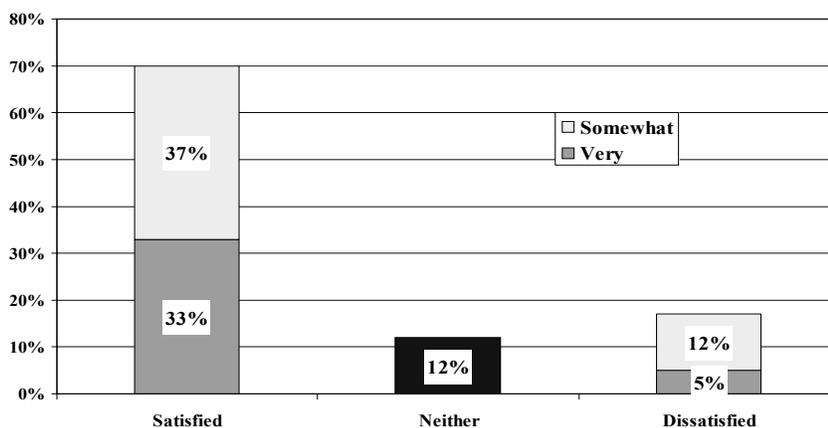
Length of Time for Infusion



Q21. How long does an infusion usually take (in minutes)? N=1170

38

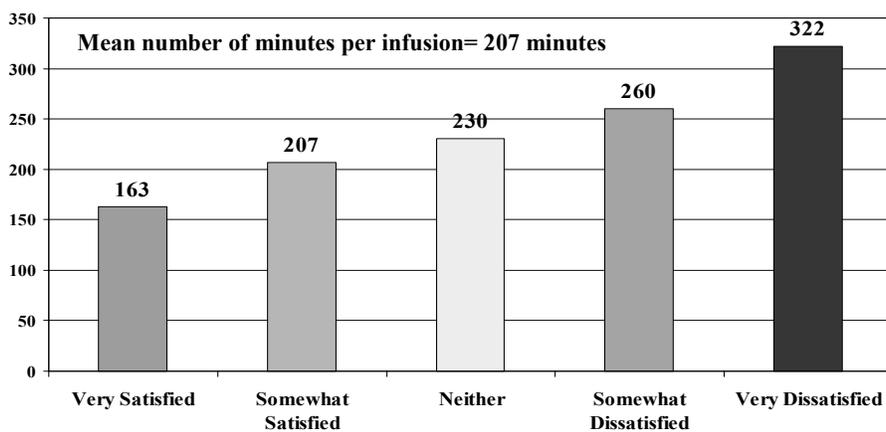
Satisfaction with Infusion Time



Q22. How satisfied is he/she with the time it takes to get an infusion? N=1170

39

Satisfaction with Infusion Time by Average Infusion Time

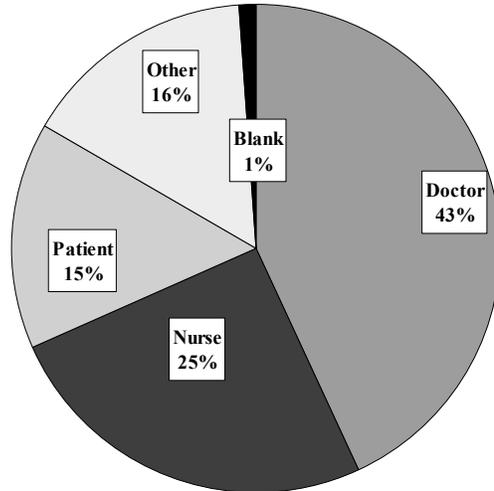


Q21. How long does an infusion usually take (in minutes)? N=1146

Q22. How satisfied is he/she with the time it takes to get an infusion?

40

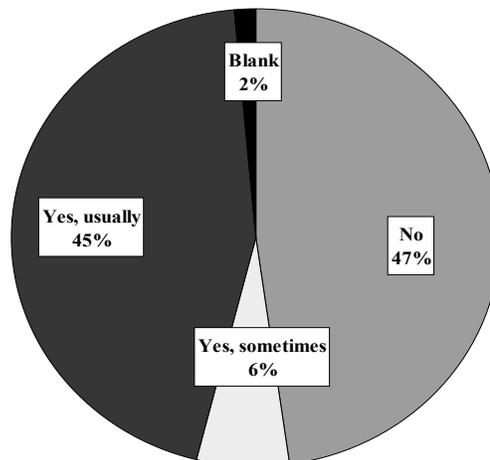
Who Determines the Infusion Rate?



Q23. Who determines the rate of infusion? N=1170

41

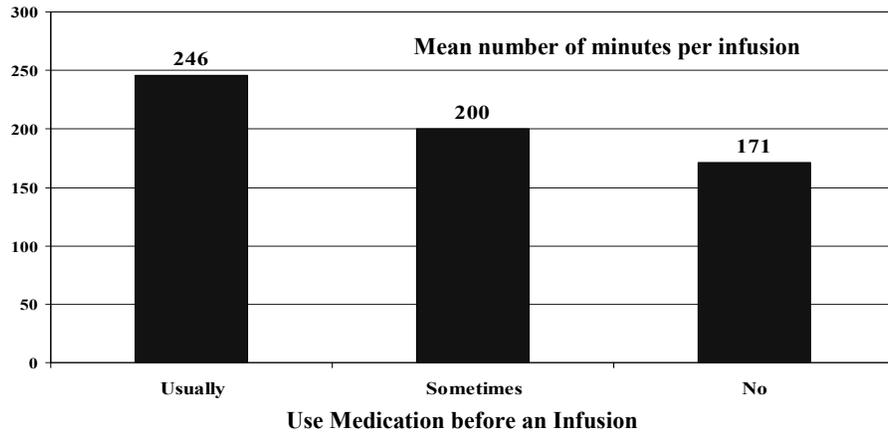
Medication Before Infusion



Q24. Is he/she given medication before an infusion, like an antihistamine, cortico-steroid or anti-inflammatory, to make it go faster or easier? N=1170

42

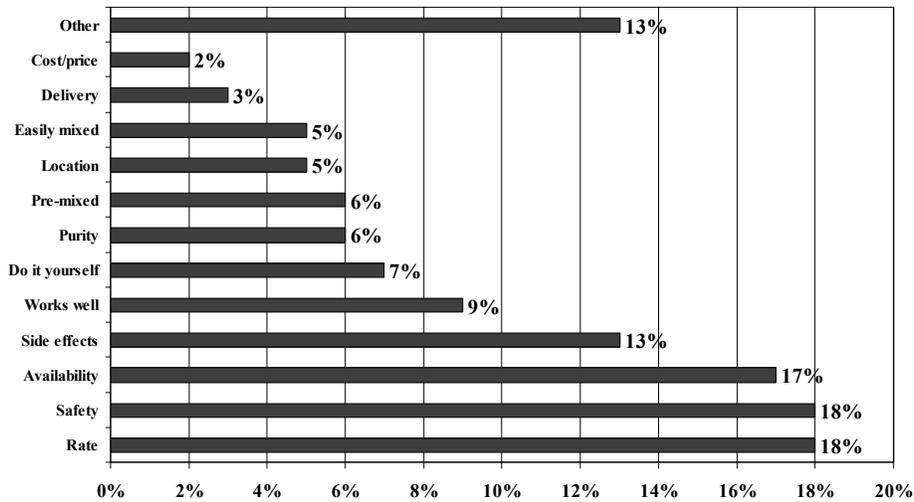
Use of Pre-Medication with IVIG by Average Infusion Time



Q24. Is he/she given medication before an infusion, like an antihistamine, cortico-steroid or anti-inflammatory, to make it go faster or easier? N=1146

43

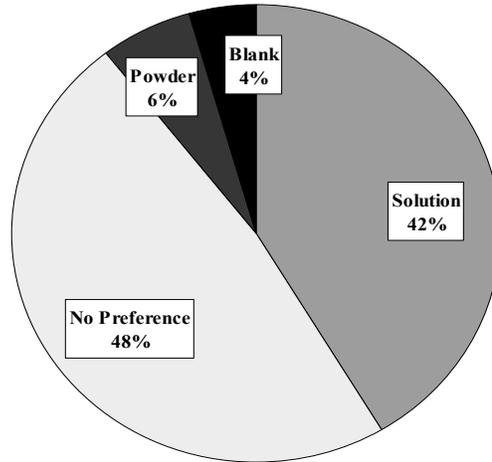
What is Convenience in IGIV?



Q25. When you think of convenience in an IGIV product, what is most important to you? N=817

44

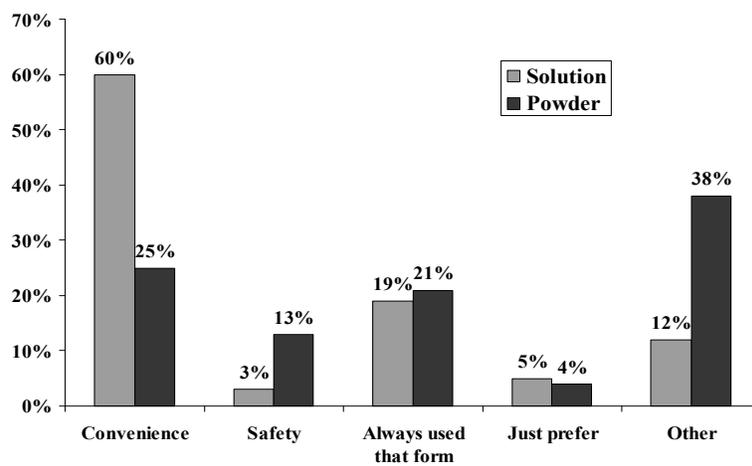
Prefer IVIG in Solution or Powder



Q26a. Does he/she prefer IGIV products that come in solution or powder? N=1170

45

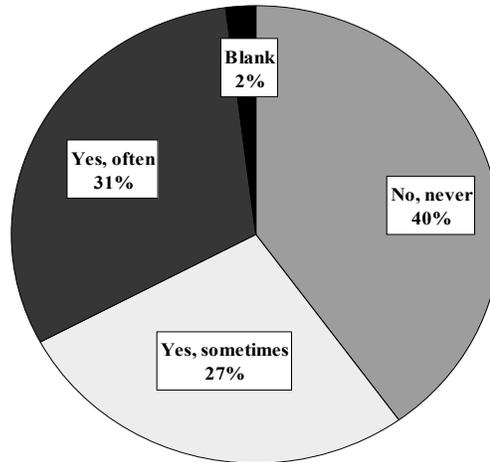
Reason for Preference by Form



Q26b. Why does he/she prefer that form? N=341 (Base: Persons with a reason for preference)

46

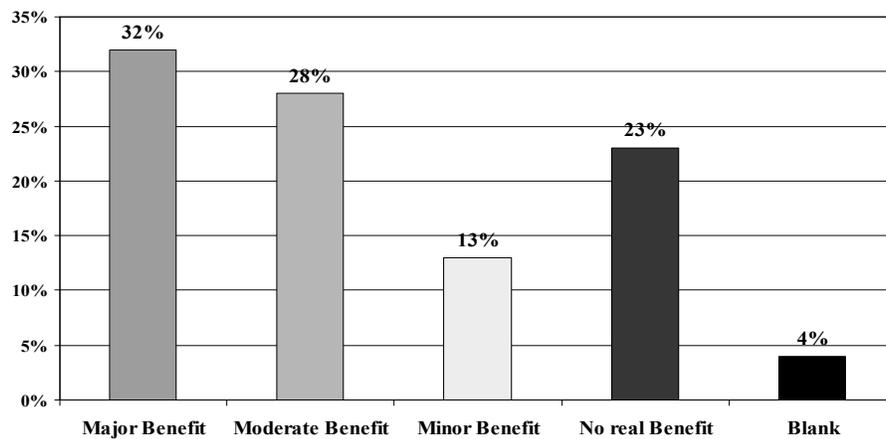
Have to Wait While IGIV Being Prepared



Q27. Does he/she ever have to wait more than a few minutes while the IGIV product is being prepared for infusion? N=1170

47

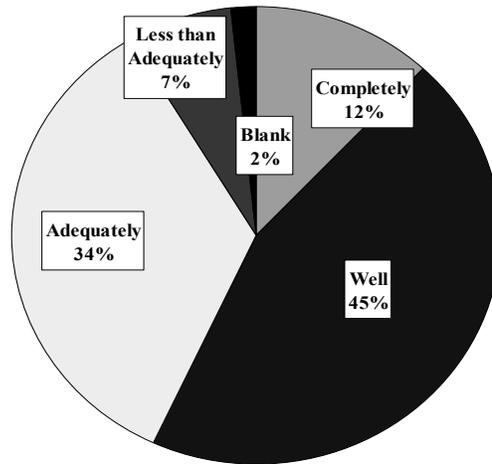
Room Temperature Product: Benefit to Patient



Q28. Some IGIV preparations are stable at room temperature, in other words they do not have to be kept in a refrigerator. For patients, would you consider this to be a... N=1170

48

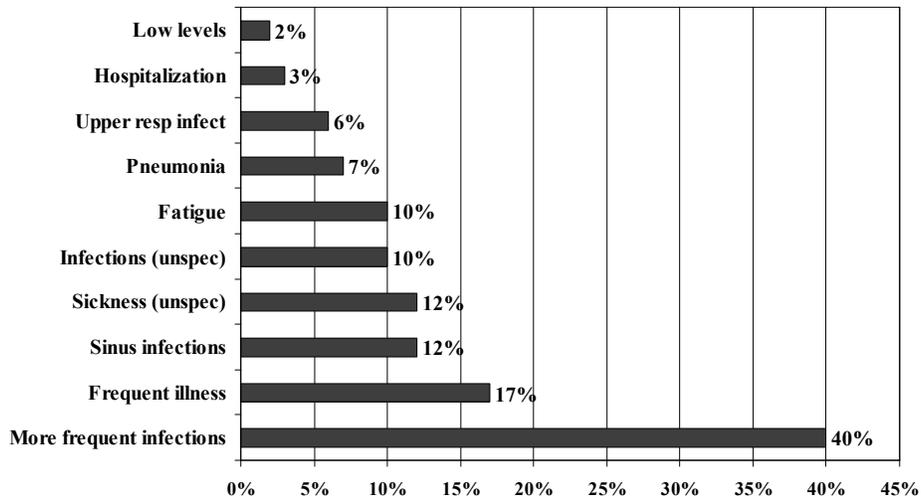
How Well Does IGIV Control PID?



Q29 How well do you feel that IGIV controls the patients immune deficiency? N=1170

49

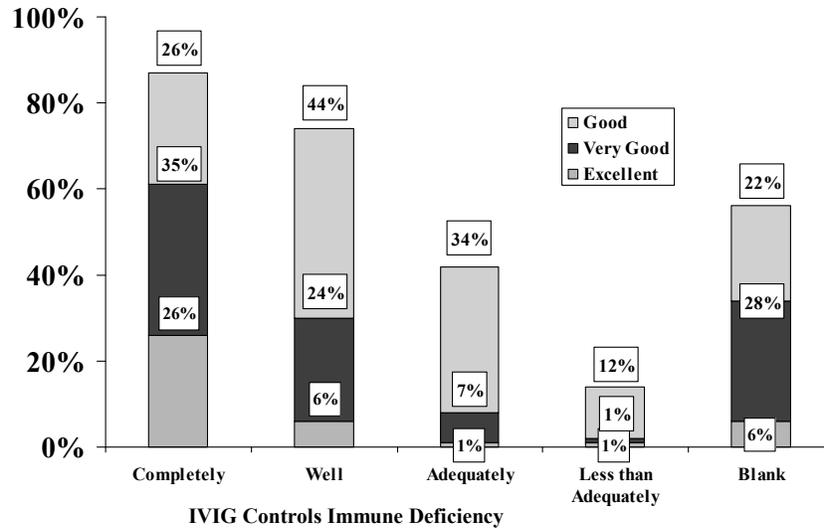
Signs of Non-Effectiveness in IGIV



Q30. What would indicate to you that the IGIV was not effective in controlling the condition? N=913

50

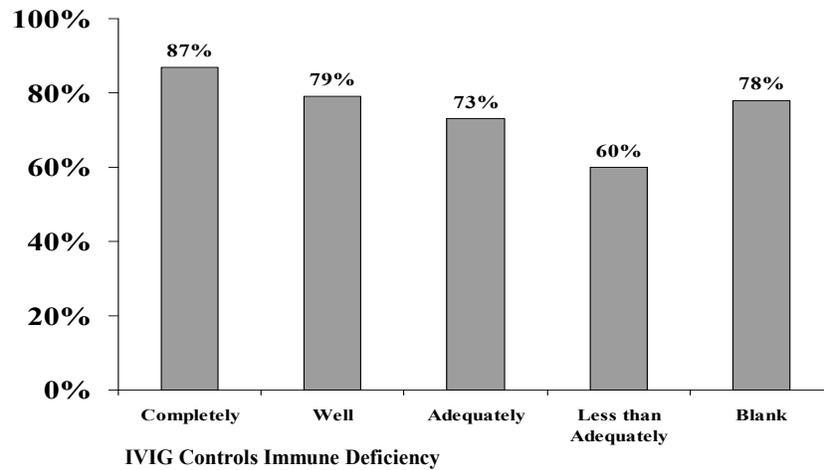
Health Status by IVIG Control



Q4. Compared to other persons of the same age, would you describe his/her health as?
 Q29 How well do you feel that IGIV controls the patients immune deficiency? N=1170

51

No Hospitalization by IVIG Control

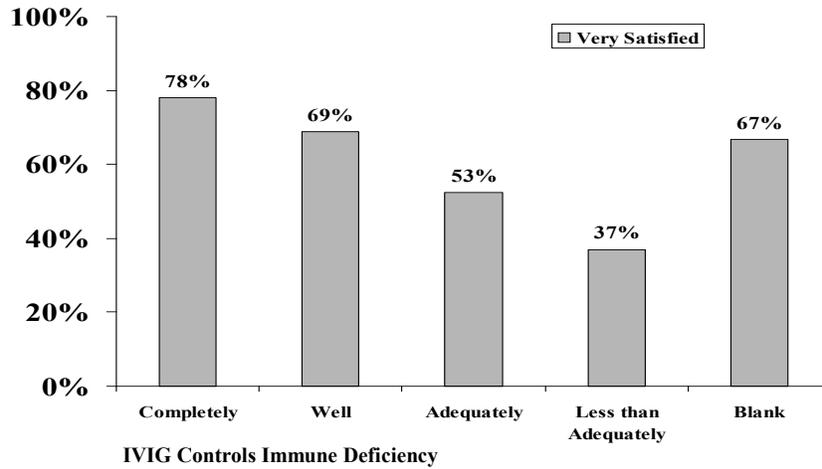


Q5. How many times, if any, has he/she been hospitalized overnight or longer in the past 12 months?

Q29 How well do you feel that IGIV controls the patients immune deficiency? N=1168

52

Doctor Satisfaction by IVIG Control

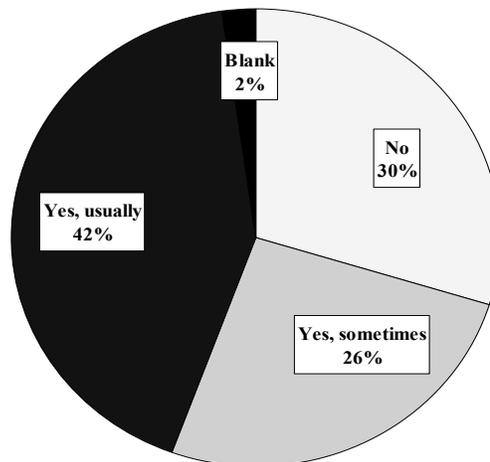


Q9. Overall, how satisfied are you with the primary doctor's management of the patient's primary immune deficiency disease?

53

Q29 How well do you feel that IGIV controls the patients immune deficiency? N=1170

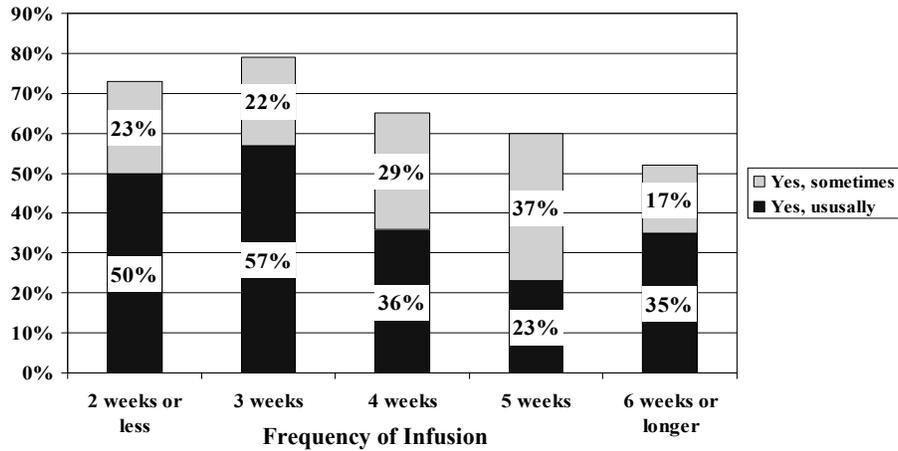
Can Patient Feel IGIV Wearing Off?



Q31a. Can the patient feel when the effects of an IGIV infusion is wearing off? N=1170

54

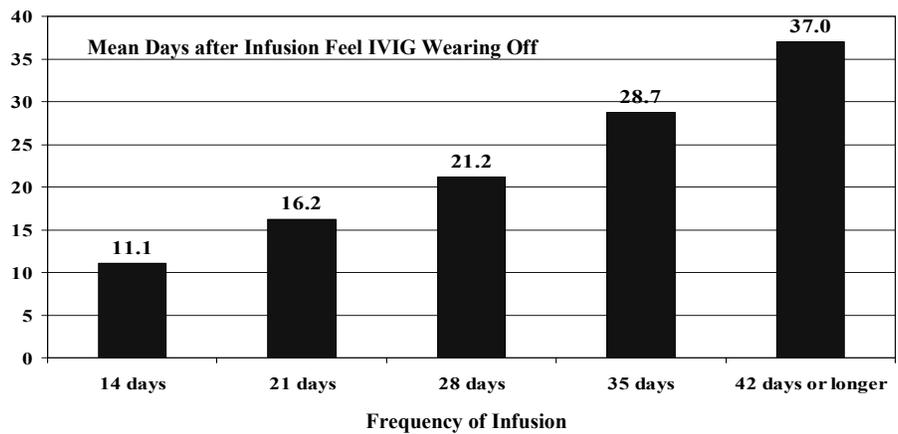
Feel Effects Wearing Off by Frequency of Infusion



Q31a. Can the patient feel when the effects of an IGIV infusion is wearing off? N=1170

55

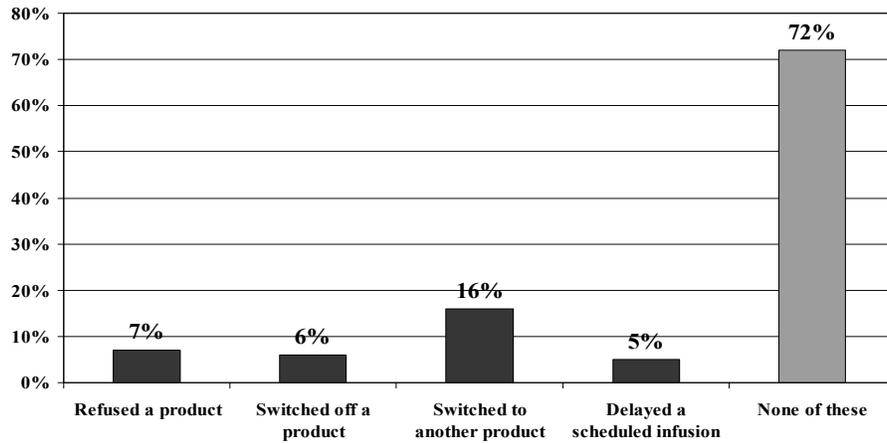
How Soon Feel Effects Wearing Off by Frequency of Infusion



Q31b. How long after infusion does he/she feel the effects beginning to wear off?
(Base: Feel Effects Wearing Off) N=757

56

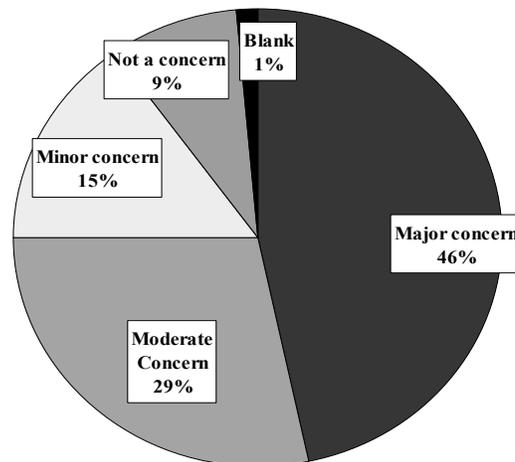
Impact of Concerns about Product Effectiveness



Q32. As a result of concerns about product effectiveness, has he/she ever.....? N=1170

57

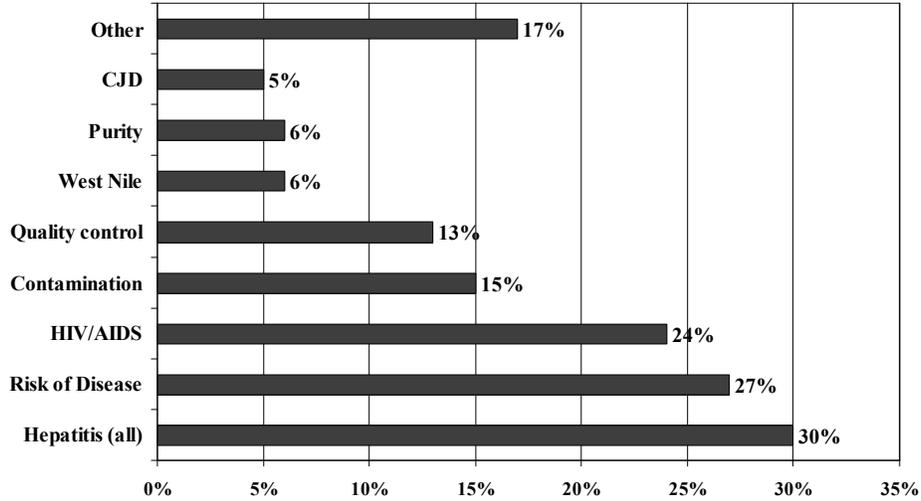
Concern about IGIV Safety



Q33. How concerned are you about the safety of the IGIV products the patient infuses? N=1170

58

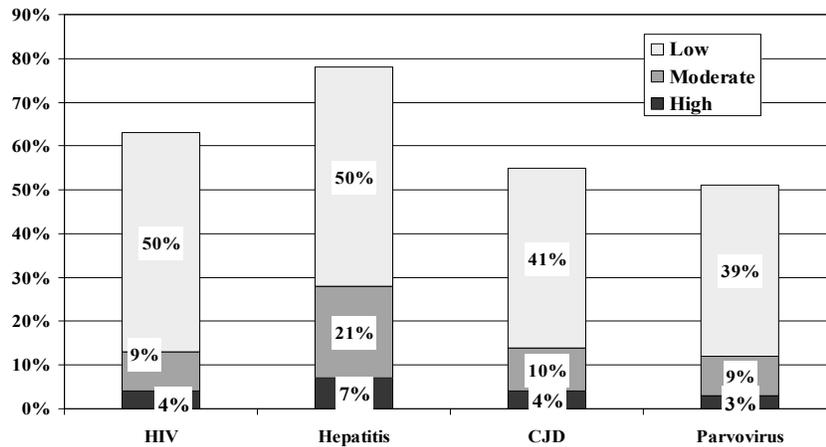
What is Safety in IGIV?



Q34. When you think of safety in IGIV, what comes to mind? N=904

59

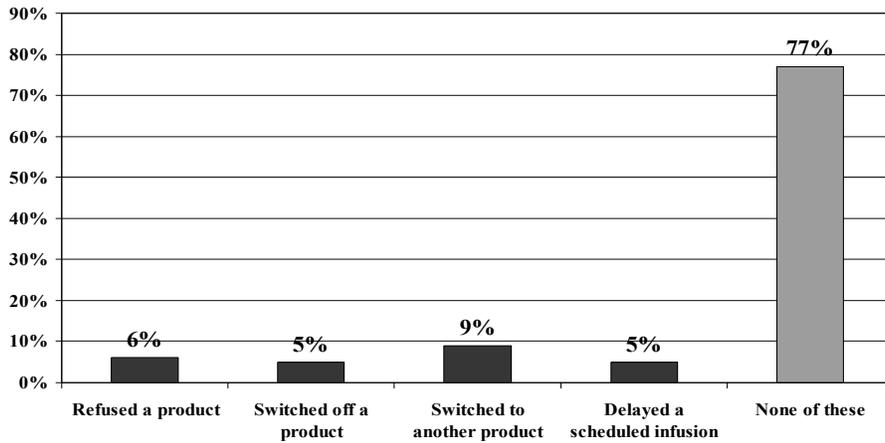
Risk of Infection from Infusion



Q49. Based on what you know or have heard, how much risk is there for PID patients getting the following diseases from an infusion of IGIV.....? N=1186

60

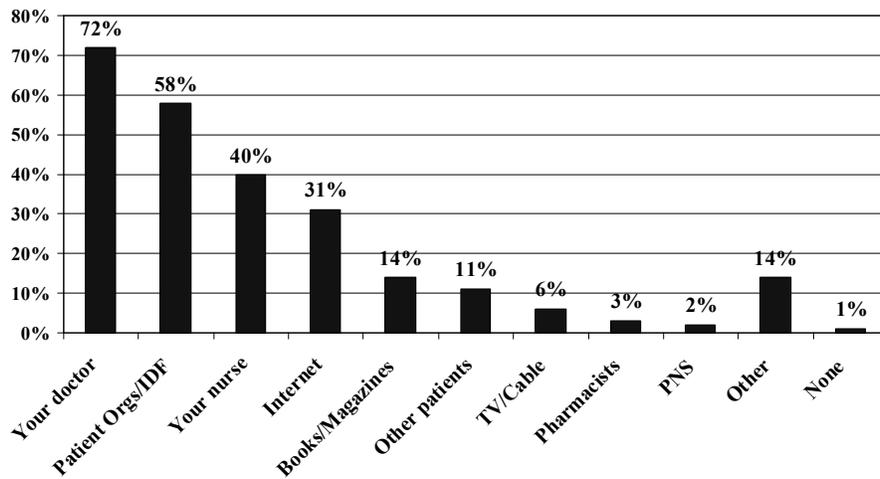
Impact of Concerns about Product Safety



Q35. As a result of concerns about product safety, has he/she ever.....? N=1170

61

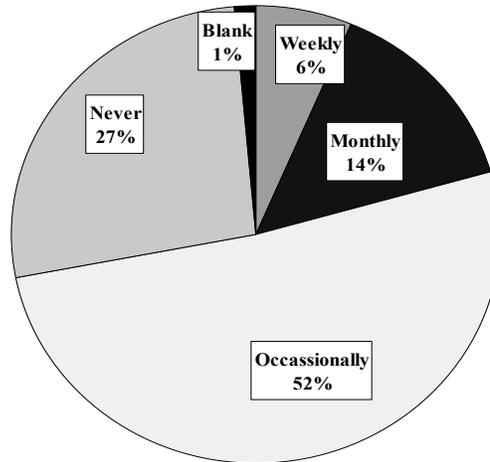
Most Reliable Sources of Information about Safety and Efficacy of IVIG



Q36. Which of the following are you most reliable sources of information about the safety and efficacy of IGIV therapy and IGIV products? N=1170

62

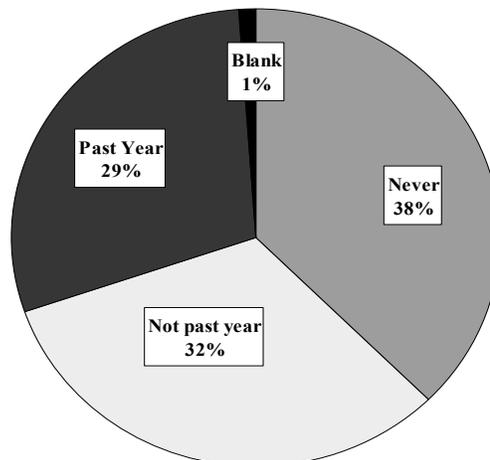
Internet Use for Information about PID



Q37. How often do you used the Internet to get information about immune deficiency diseases and/or their treatment? N=1170

63

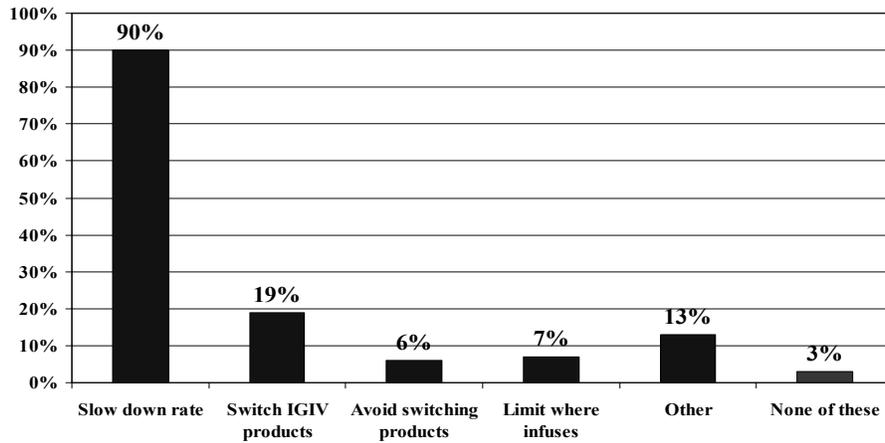
Rate Related Reaction from IGIV



Q38a. Has the patient ever had a rate related (speed of infusion) reaction from the IGIV? N=1186
Q38b. When was the most recent time that he/she had a rate related reaction from IGIV?

64

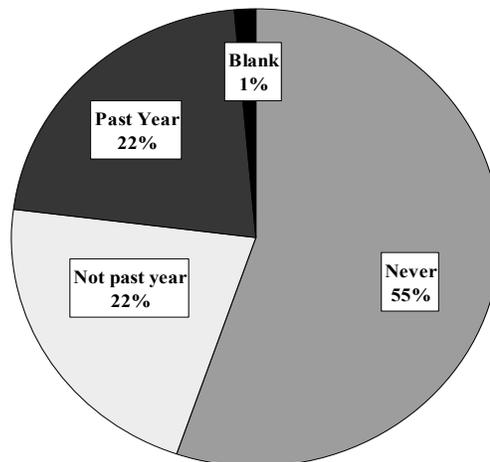
Impact of Rate Related Reactions



Q38c. Has patient's rate related reaction experience caused his/her to.....?
 Base: Has had a rate related reaction N=728

65

Serious Side Effects from IGIV

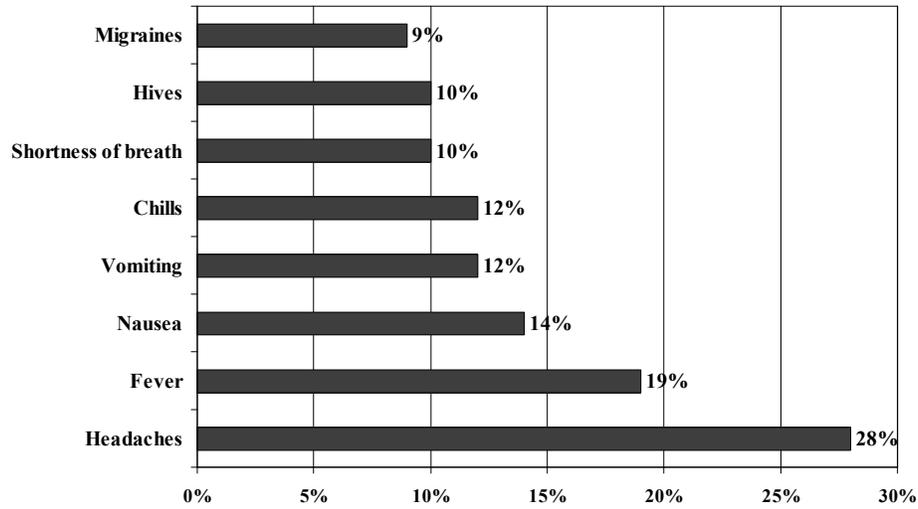


Q39a. Aside from rate related reactions, has the patient ever had any serious side effect or reaction from the IGIV?

Q39c. When was the most recent time he/she had a serious side effect or reaction from IGIV? N=1186

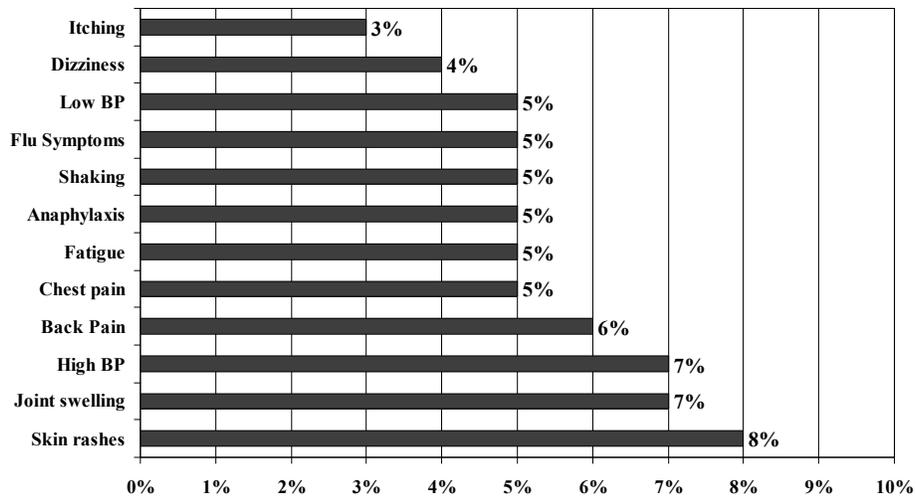
66

Most Common Side Effects from IGIV



Q39b. What types of (non-rate related) serious side effects or reactions from IGIV has he/she had? (N=397) 67

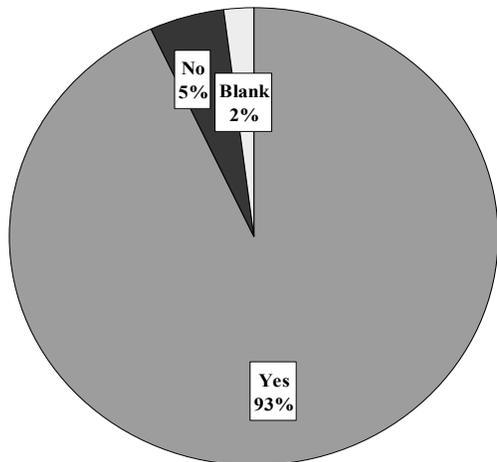
More Side Effects from IGIV



Q39b. What types of (non-rate related) serious side effects or reactions from IGIV has he/she had?

68

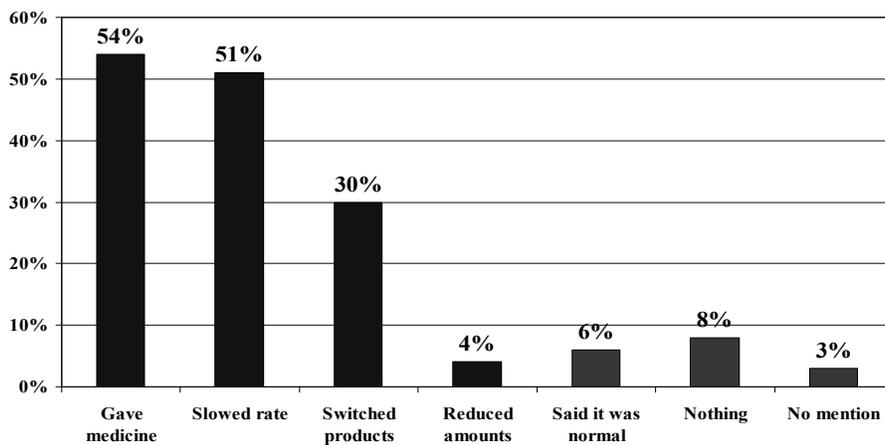
Told Doctor about Side Effects



Q39d. Was the doctor told about the side effect or reactions? (Base: Had side effect) N=515

69

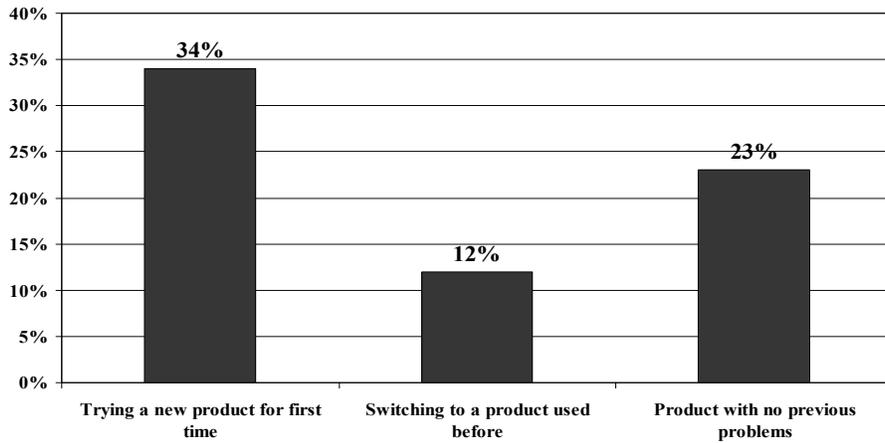
Doctor Response to Reported Side Effects from IVIG



Q39e. What did the doctor do (when told about side effects).....?
Base: Doctor told about side-effects N=479

70

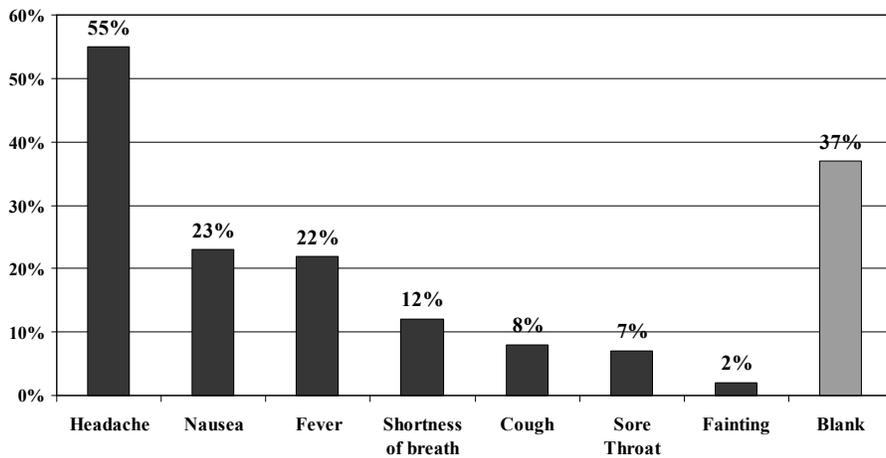
When Side Effects Have Occurred



Q39f. Has the patient ever had a serious side effect or reaction for IGIV when..? N=479

71

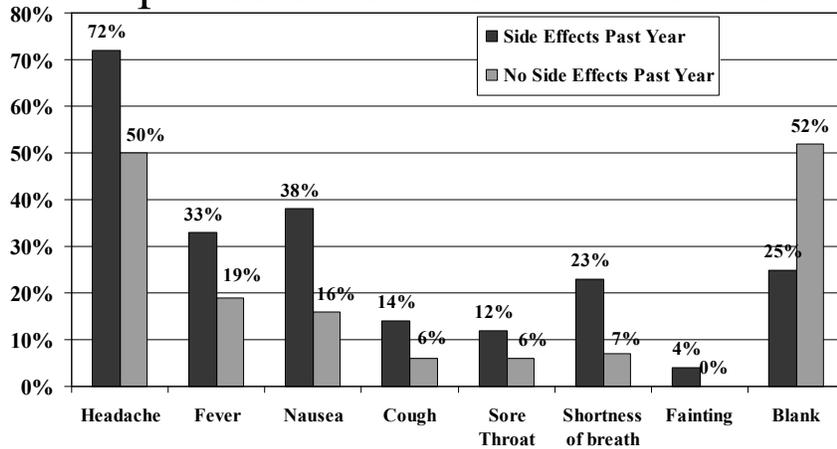
Symptoms after Infusion in Past Year



Q40. During the past year, has he/she experienced any of the following after an IGIV infusion? N=1186

72

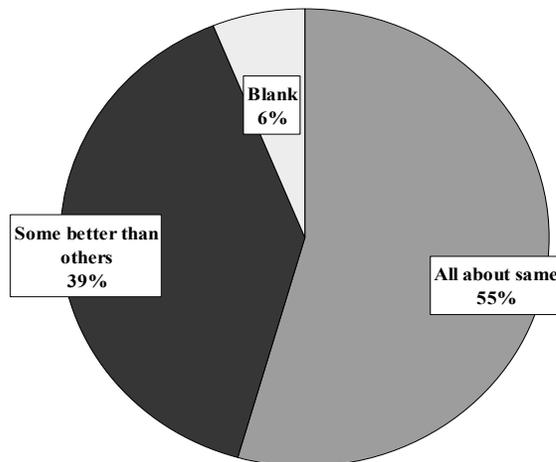
Symptoms Following Infusion by Reported Side Effects in Past Year



Q40. During the past year, has he/she experienced any of the following after an IGIV infusion? N=1186

73

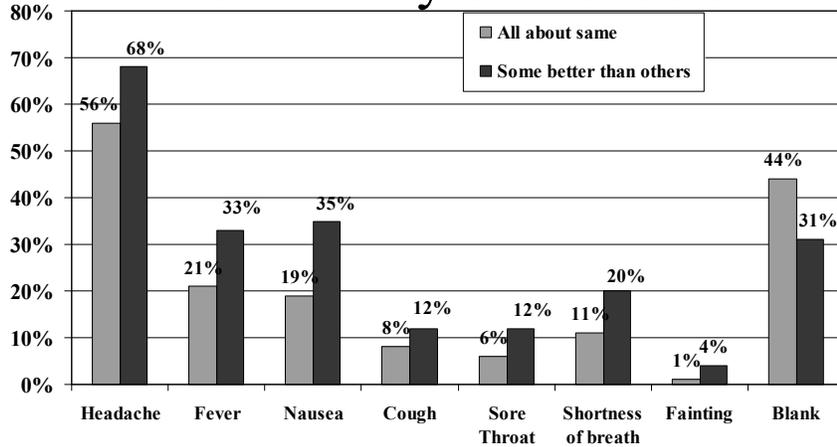
Tolerate IVIG Products Differently



Q41. Does he/she tolerate any IVIG products better than others, or are they all about the same? N=1186

74

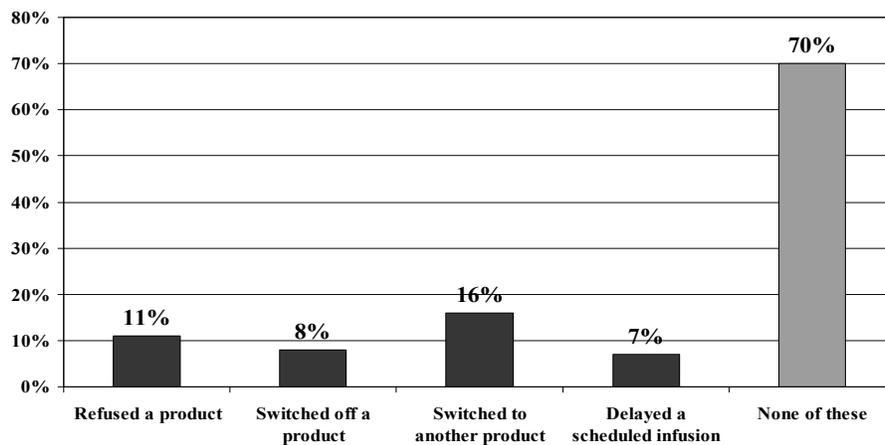
Symptoms Following Infusion by Tolerability of Products



Q40. During the past year, has he/she experienced any of the following after an IGIV infusion? N=1186

75

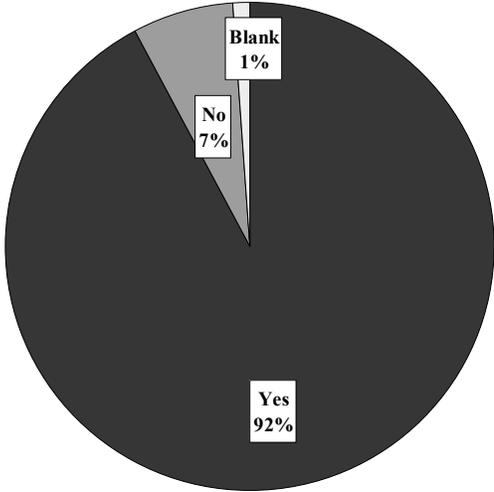
Impact of Concerns about Product Tolerability



Q42. As a result of concerns about product tolerability, has he/she ever.....? N=1186

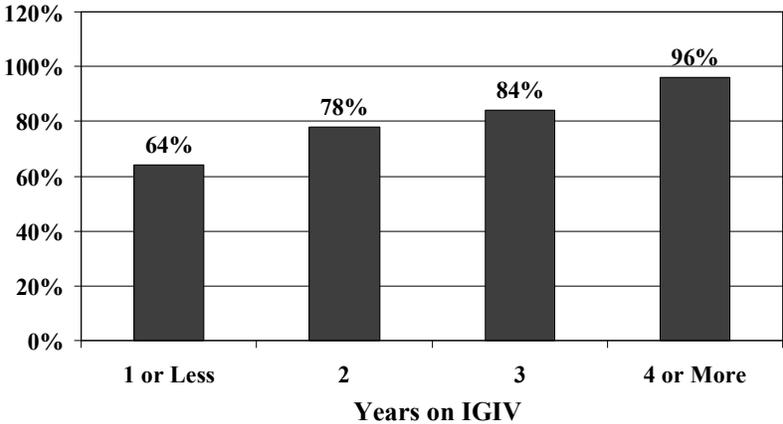
76

Aware of IGIV Shortages



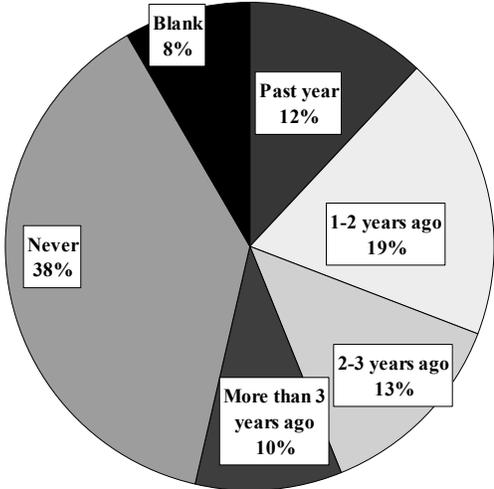
Q43a. Were you aware that there have been shortages in the availability of IGIV in the past few years? N=1186

Awareness of IGIV Shortage by Years on IGIV



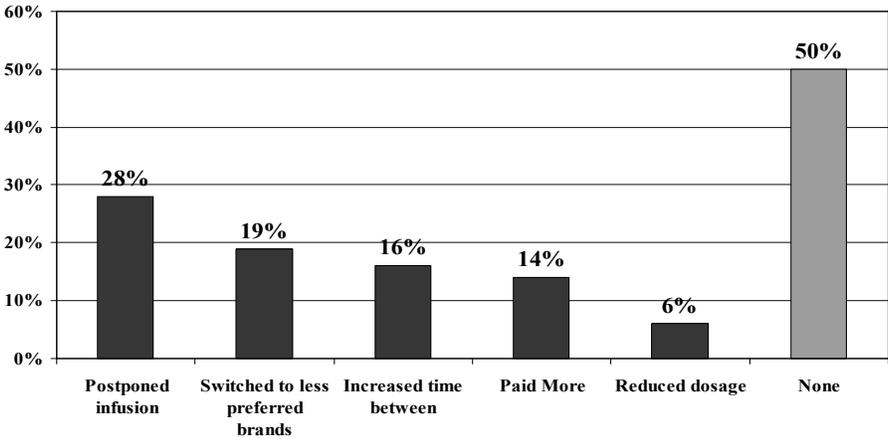
Q11. How many years has the patient taken IGIV on a regular basis for his/her immune deficiency? N=1170
 Q43a. Were you aware that there have been shortages in the availability of IGIV in the past few years?

Most Recent Problem Getting Infusion



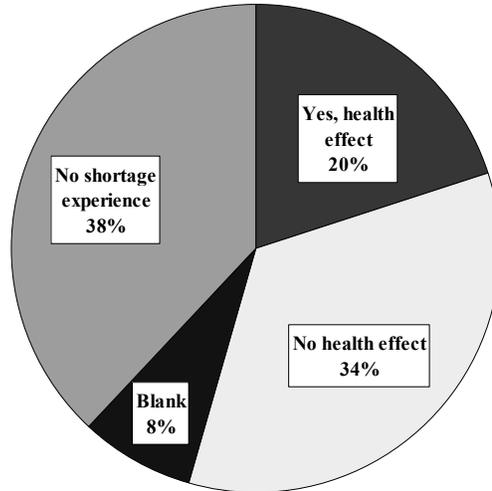
Q43b. When was the most recent time the patient had a problem in getting his/her regular IGIV infusion? N=1186

Impact of IGIV Shortages



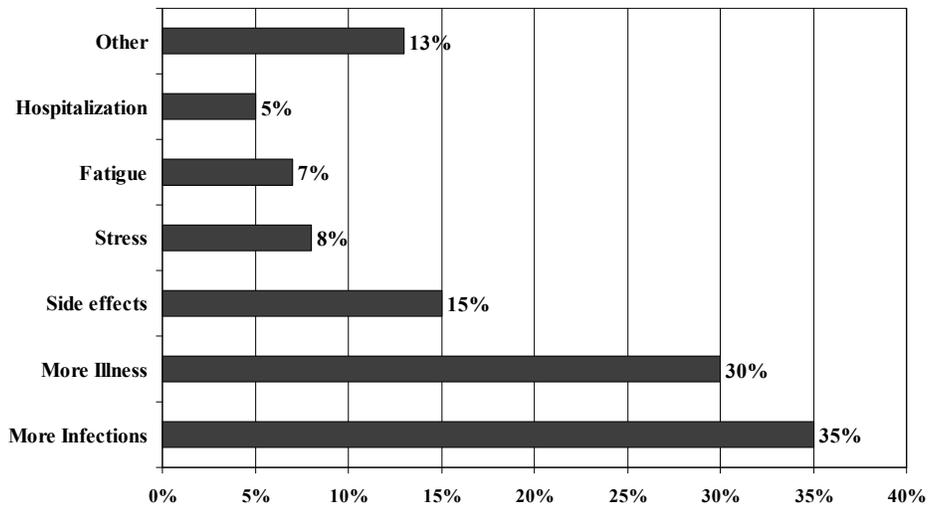
Q43c. As a result of shortages in IGIV supply, which of the following (if any) has happened to the patient? N=1186

Health Consequences of Shortage



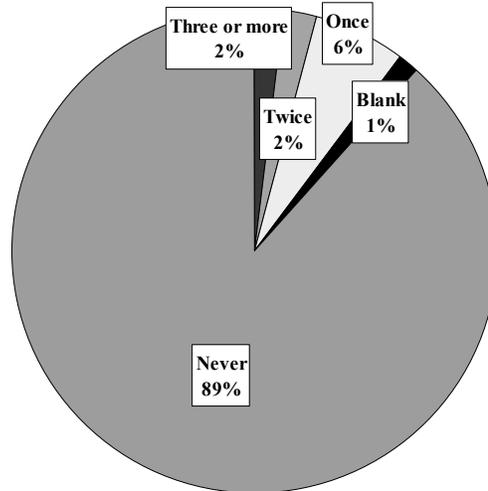
Q43d. Have these shortages in IGIV supplies had any negative effects on the patient's health? N=1186 81

Negative Health Impact of Shortages



Q43d. Have these shortages in IGIV supply had any negative effects on the patients health? Please describe. 82
Base: Had negative health effects (N=206)

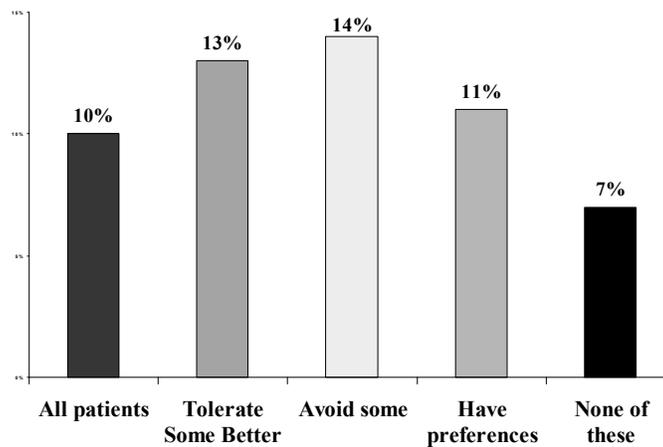
Past Year Problems Getting IGIV



Q44. How many times in the past 12 months, if any, has the patient's scheduled IGIV infusion been cancelled, rescheduled, or delayed because the product was not available? N=1186

83

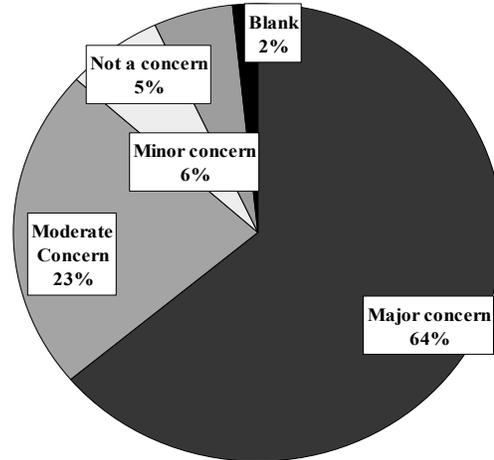
Past Year Problems Getting IGIV



Q44. How many times in the past 12 months, if any, has the patient's scheduled IGIV infusion been cancelled, rescheduled, or delayed because the product was not available? N=1186

84

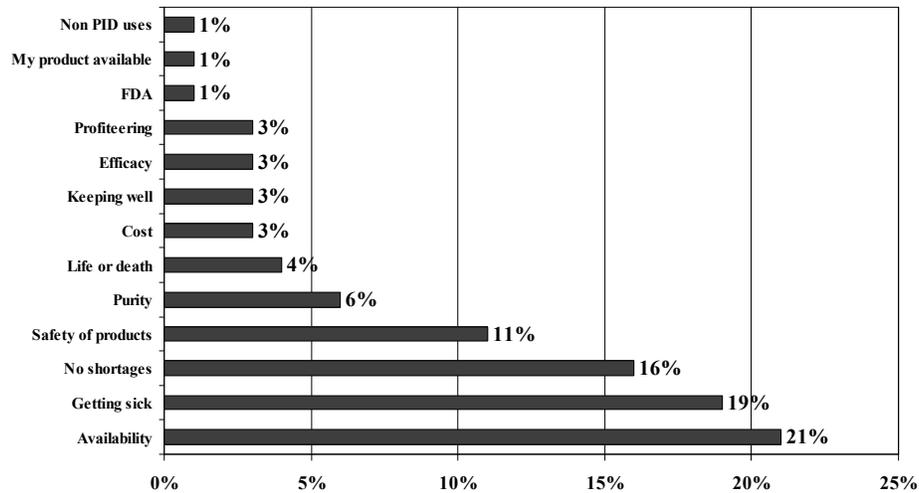
How Concerned about IGIV Supply



Q45. How much of a concern to you is the reliability of IGIV supply? N=1186

85

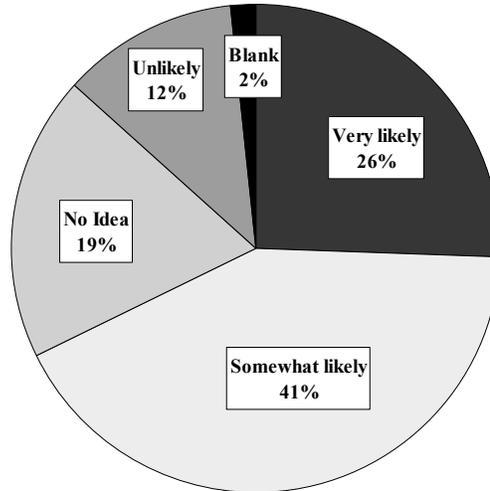
Reliability of IGIV Supply



Q46. When you think of reliability of IGIV supply, what comes to mind? N=817

86

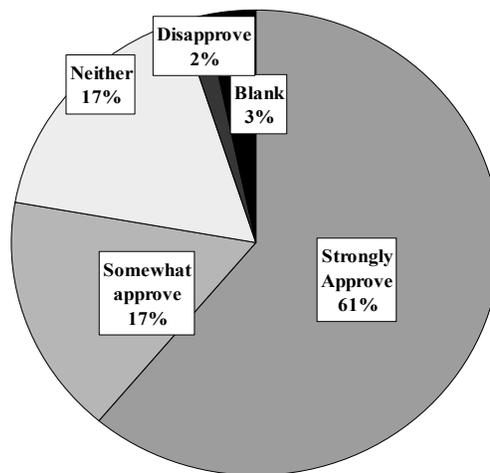
Likelihood of Shortages in Next 2 Years



Q69. Based on what you know or have heard, how likely do you think it is that there will be another serious shortage of IGIV in the next two years? N=1141

87

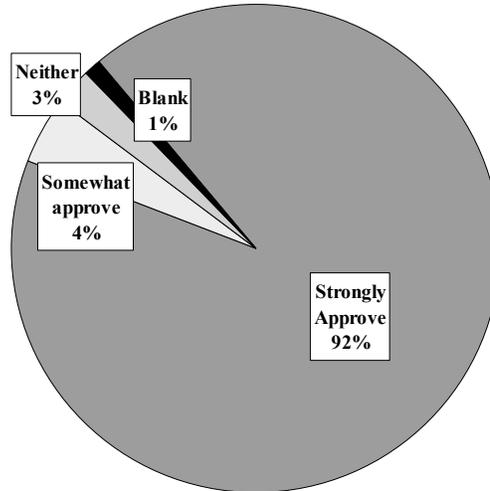
Attitudes toward Development of More Efficient Products



Q71. How do you feel about manufacturers developing new IGIV products that produce more IGIV from the same amount of blood than older products? N=1141

88

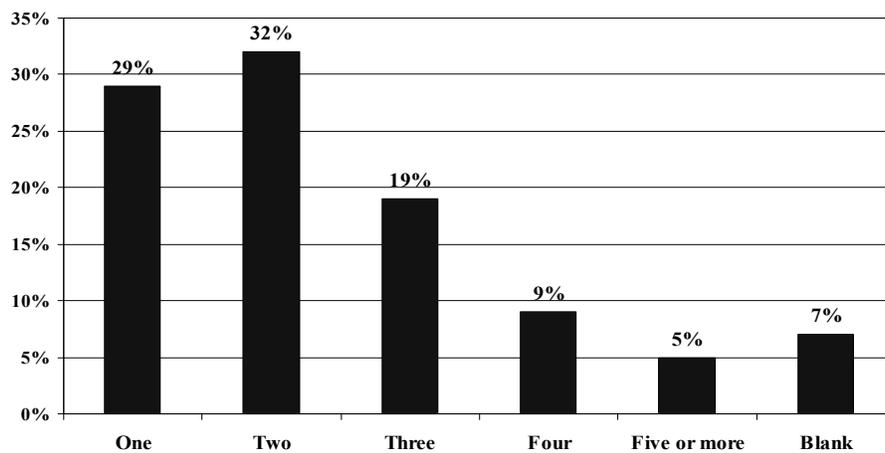
IDF Working with Industry



Q70. How do you feel about the Immune Deficiency Foundation working with industry to assure an adequate supply of IGIV for persons with immune deficiency diseases? N=1141

89

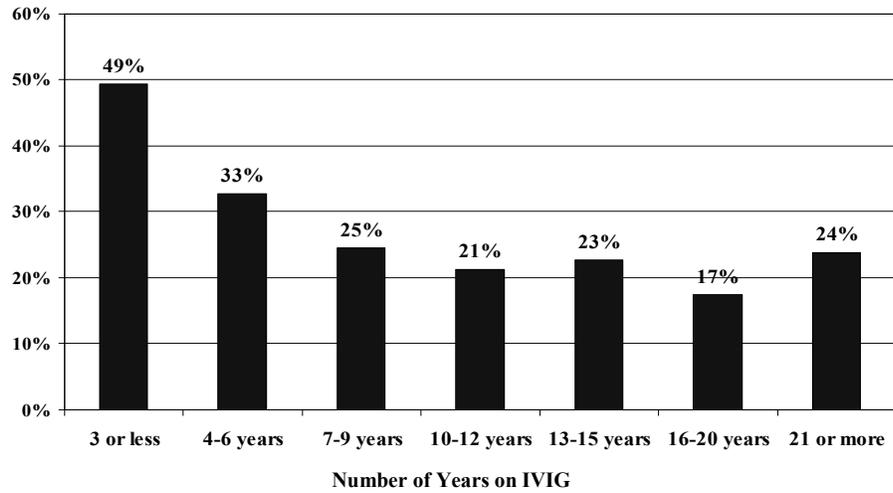
Number of Products Ever Used



Q53. Which of the following IGIV preparations has the patient ever used? N=1186

90

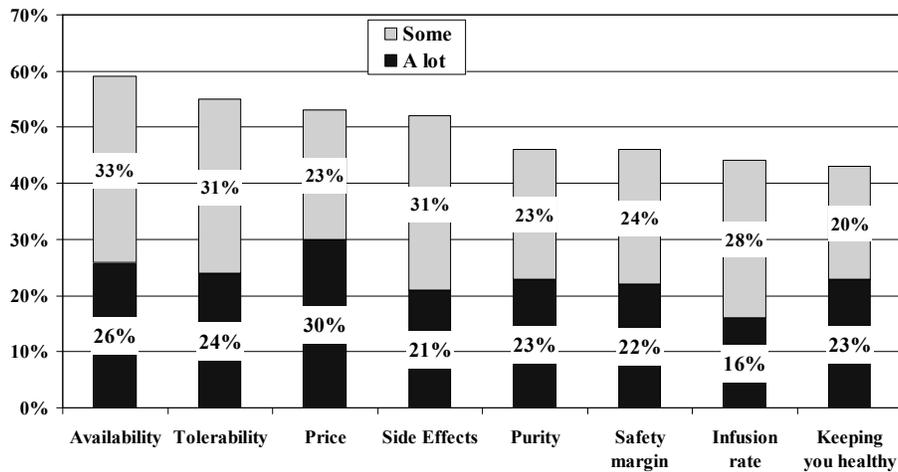
Used Only One IVIG Product



Q56. Which of the following IGIV preparations did the patient get in the most recent infusion? N=1150

91

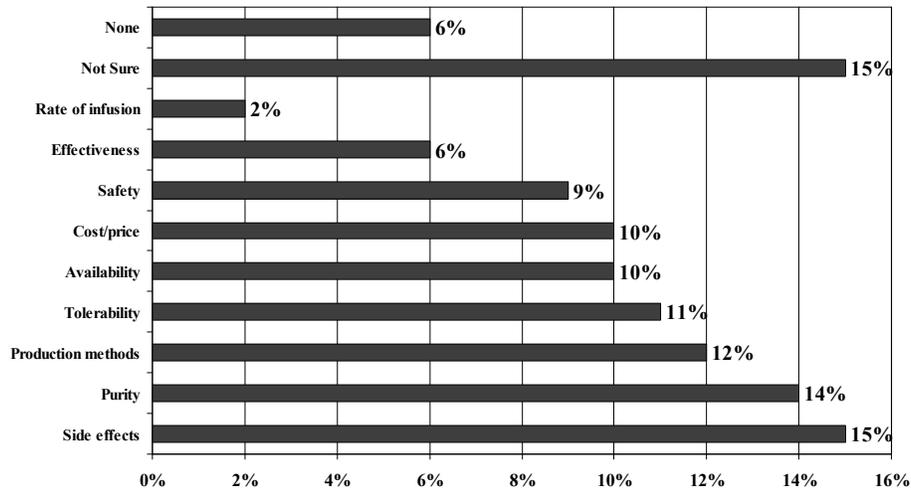
Difference between IGIV Products



Q47. Based on what you know or have heard, how much difference do you feel there is between IGIV products available in the U.S. in terms of? N=1186

92

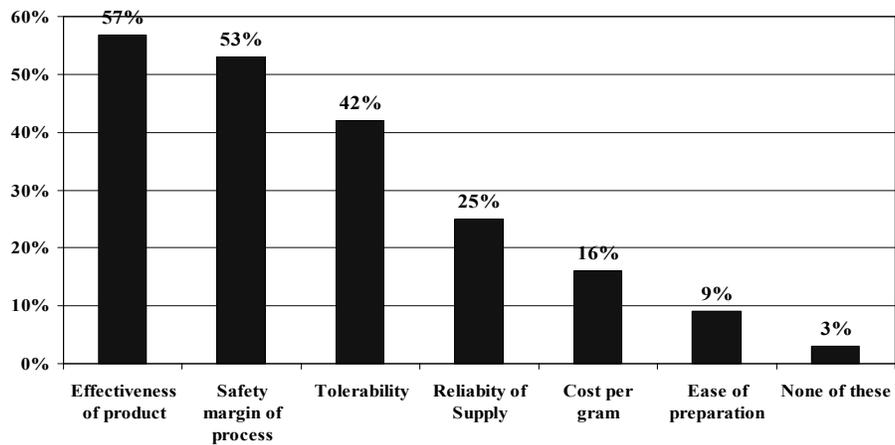
Most Important Differences Between Products



Q48. Based on what you know or have heard, what is the most important difference between IGIV products? N=732

93

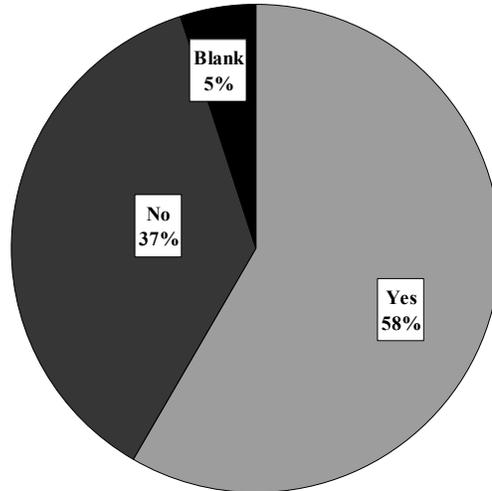
Most Important Factors in Switching Products



Q50. If your doctor recommended the patient switch to a new FDA approved IVIG product, which of the following would be most important to you in deciding whether to switch? N=1186

94

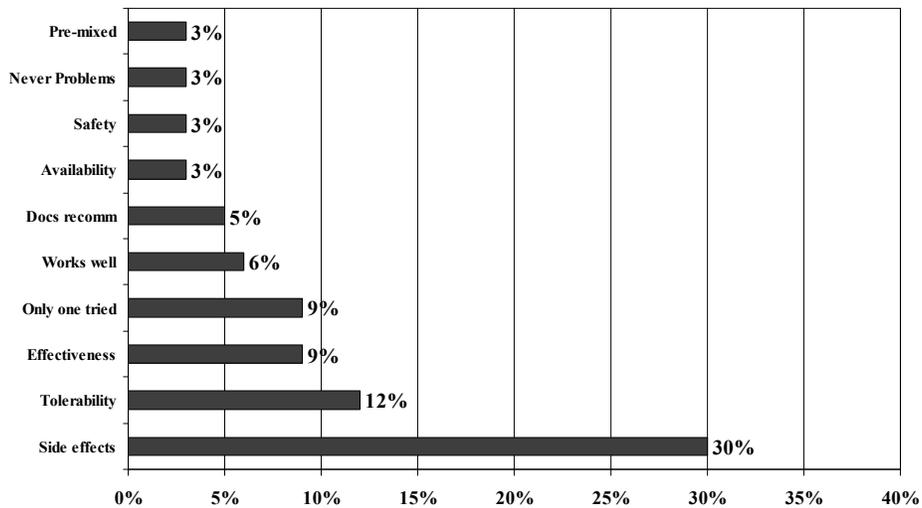
Preferences for Specific Products



Q54a. Does he/she have any preferences for specific IGIV products? N=1186

95

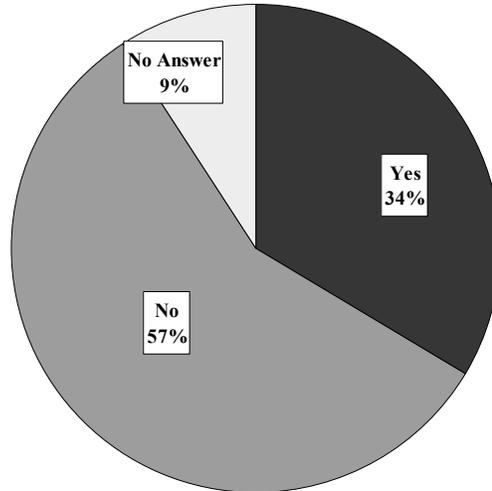
Why Prefer Specific Products



Q54c. Why do you prefer that/those product(s)? N=692

96

Avoid Any IGIV Products

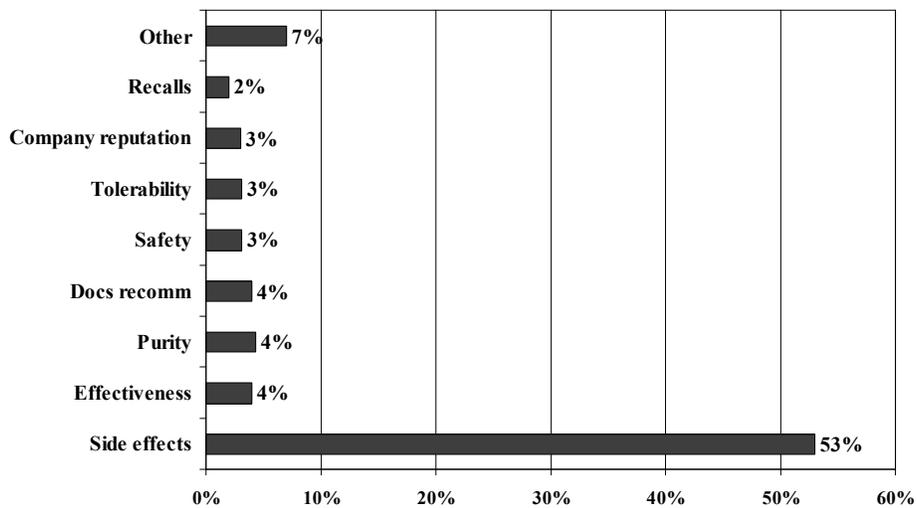


Q55a. Are there any IGIV products that you try to avoid using?

N=1186

97

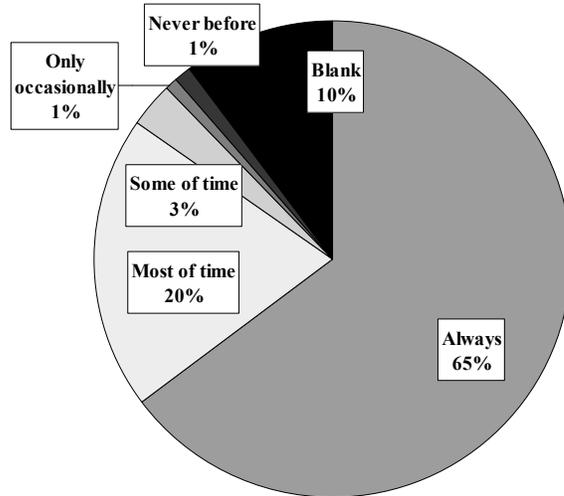
Why Avoid Specific Products



Q55c. Why do you try to avoid that/those product(s)? N=400

98

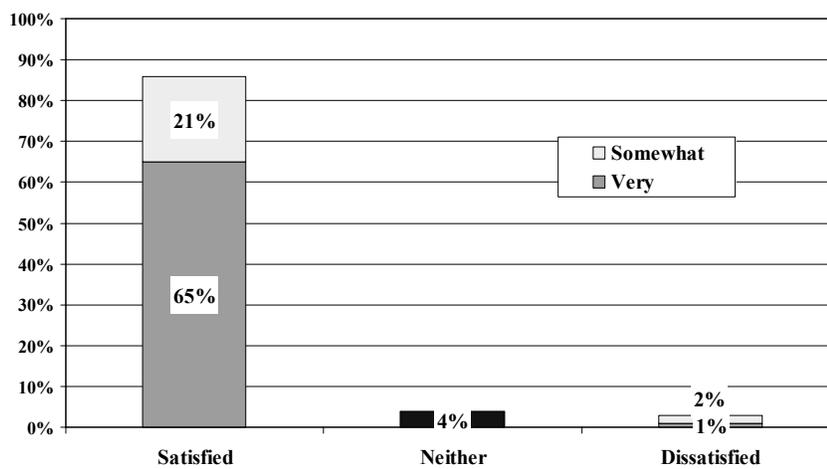
How Often Gets Current Product



Q57. How often does he/she get this specific product? N=1186

99

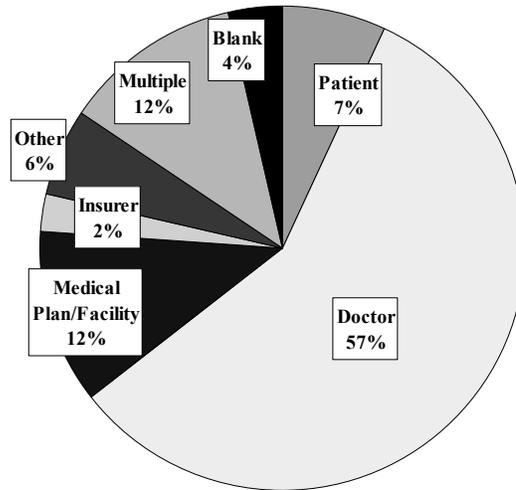
Satisfaction with Current Product



Q58. How satisfied are you with this (current) product? N=1186

100

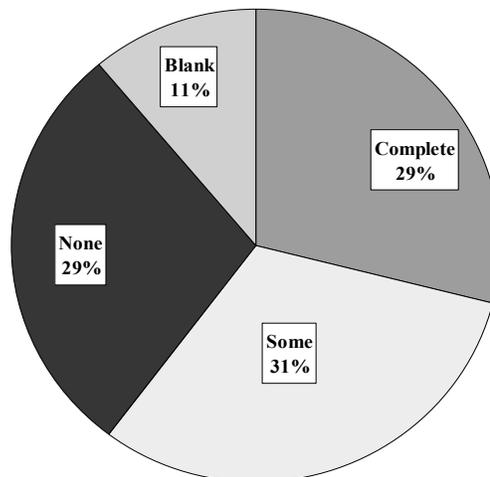
Who Selects IGIV Products



Q64. Who is primarily responsible for the selection of the IGIV product that the patient uses? (net of mentions) N=1186

101

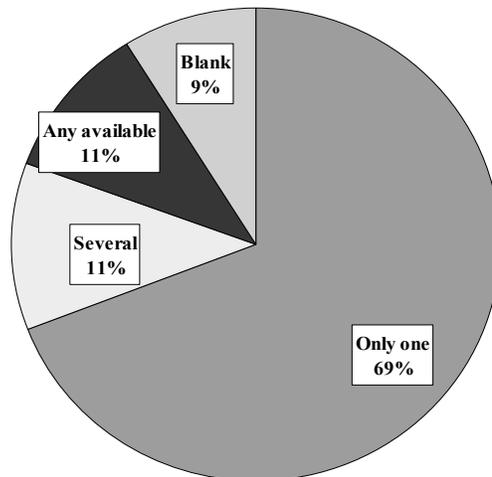
Choice in IGIV Products



Q68. How much choice does his/her doctor (or health plan) allow him/her among specific IGIV products? N=1186

102

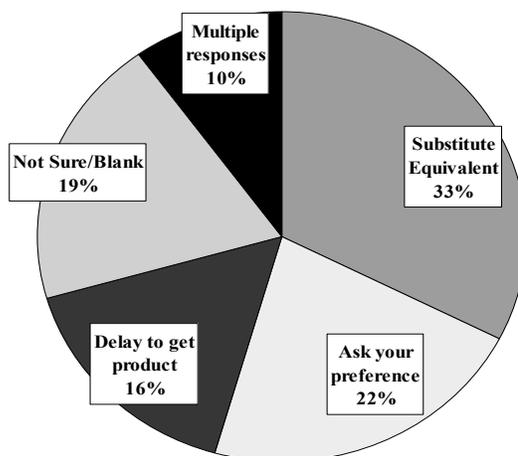
Stick with Same IGIV Products



Q65. Does he/she usually stick with one particular IGIV product, use several specific products, or use whichever products are available? N=1186

103

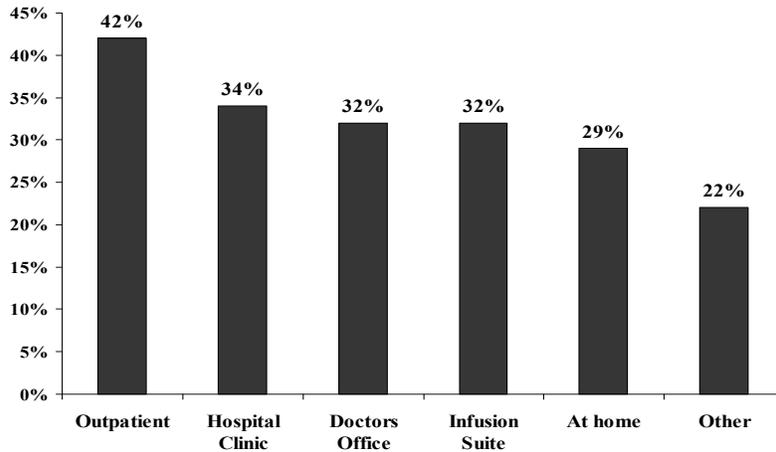
What Would Provider Do if Specific Product Not Available



Q66. If a particular product was not available at the time of a scheduled transfusion, would his/her provider be most likely to? N=1186

104

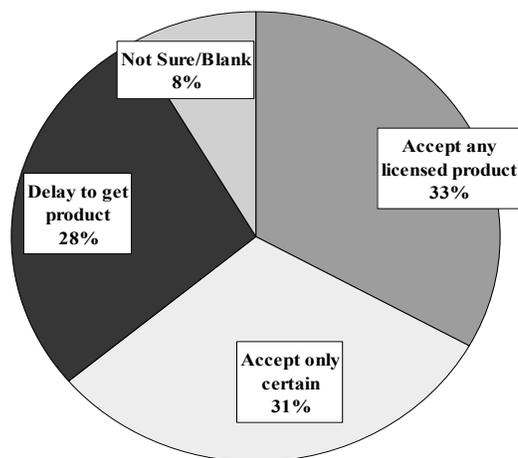
Provider Would Substitute an Equivalent If Specific Product Not Available



Q66. If a particular product was not available at the time of a scheduled transfusion, would his/her provider be most likely to? N=1170

105

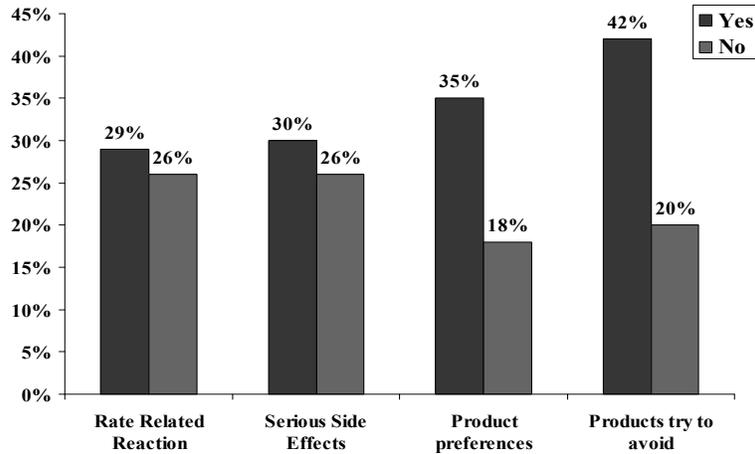
What Would You Do if Usual Product Not Available



Q67. If you knew that his/her usual product was not available at the time of a scheduled infusion, what would you most likely do? N=1186

106

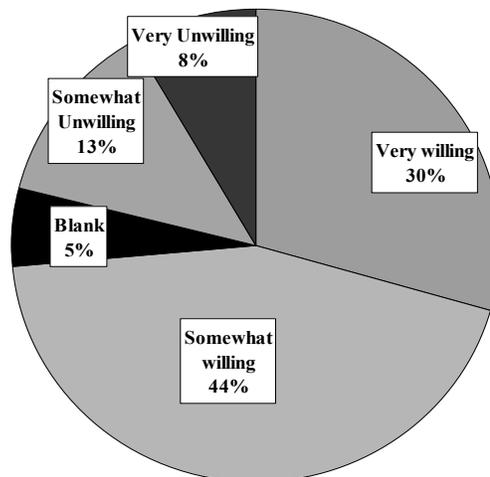
Would Delay Infusion if Usual Product Not Available



Q67. If you knew that his/her usual product was not available at the time of a scheduled infusion, what would you most likely do? N=1186

107

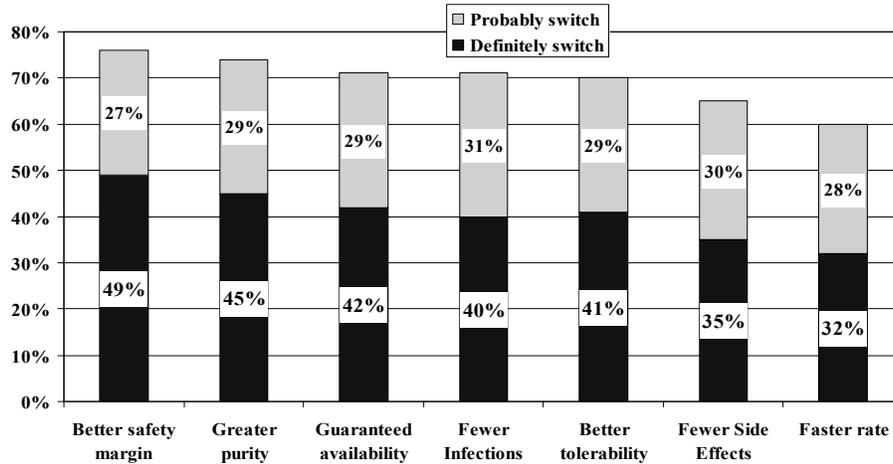
Willingness to Switch to Equal Product



Q59. How willing would you be to switch to a new product that was EQUAL to this product on effectiveness, safety, tolerability, reliability and cost? N=1186

108

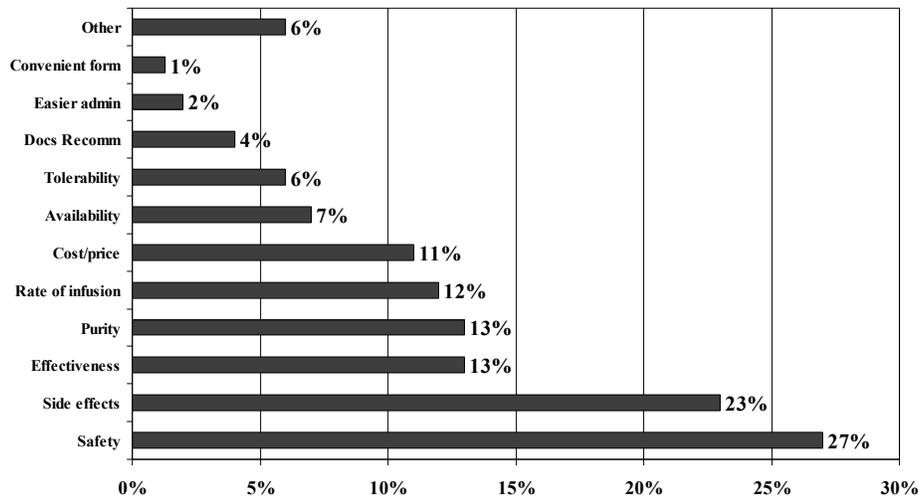
Likelihood of Switching to New Product at Higher Price by Product Attribute



Q60. How likely would you be to switch to a new IGIV product at a somewhat higher price that had? N=1186

109

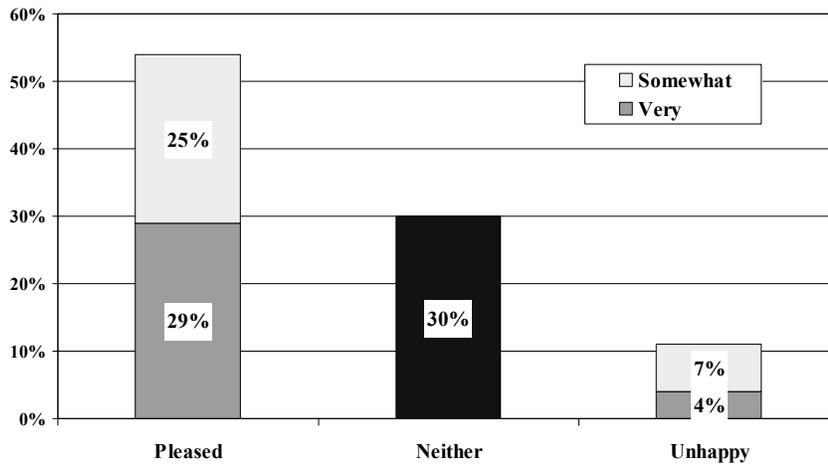
Why Switch to a New Product



Q61. What feature of a new IGIV product would be most likely to make you switch to it from your current product? N=858

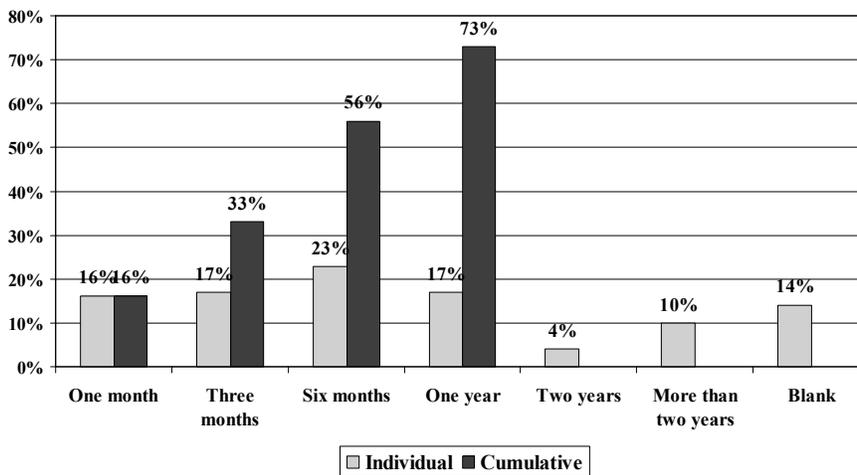
110

Reaction to Replacing Current Product with Equivalent from Same Manufacturer



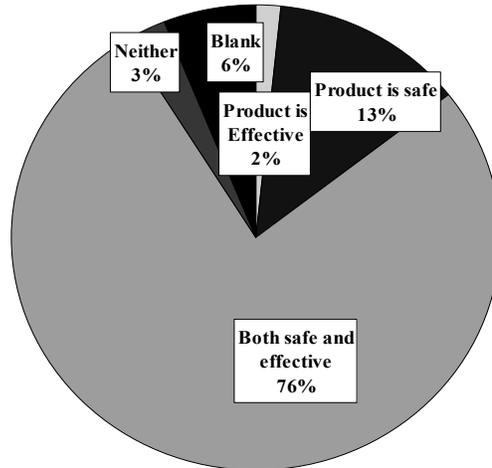
Q62a. If the IVIG product that the patient currently uses was being replaced by an equal or better product from the same manufacturer, what would be your reaction? N=1186 111

How Long a Transition Needed for Old Product



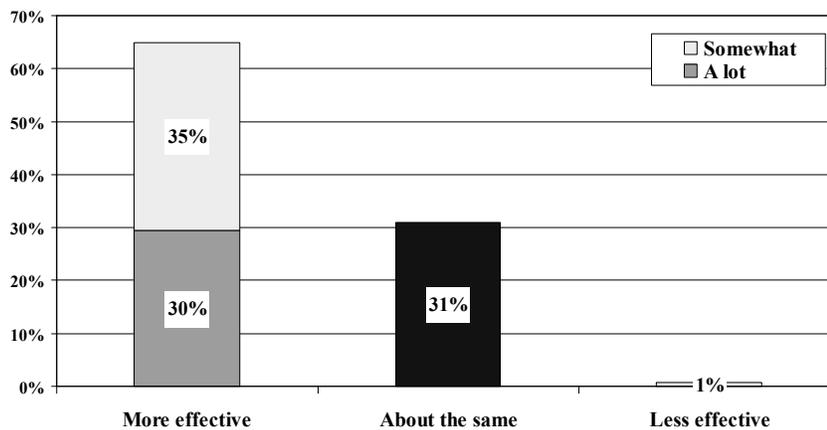
Q63. If the manufacturer of the product you currently use was replacing it with a new improved product, how long would you insist the current product be available for your transition to the new product or another product? N=1186

What Must Be Demonstrated Before IVIG Can be Sold in U.S.



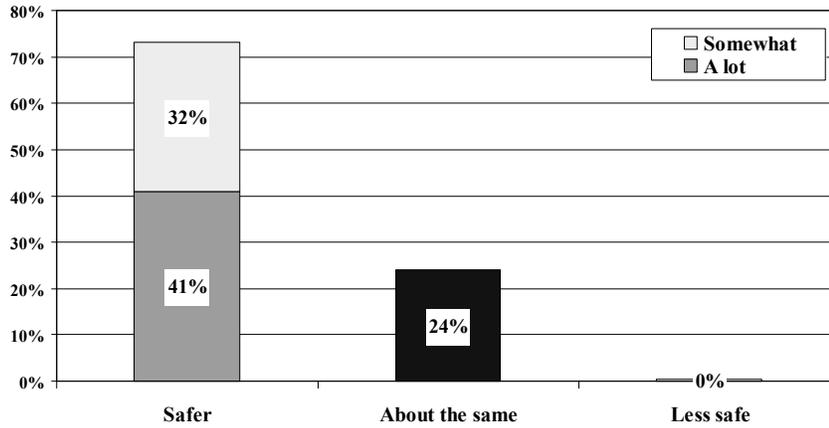
Q72. Based on what you know or have heard, what does the government require an IGIV manufacturer to demonstrate before the product can be sold in the United States? N=1141 113

New Products: More or Less Effective



Q73. Compared to currently licensed IGIV products, would you expect new products to be.... N=1141

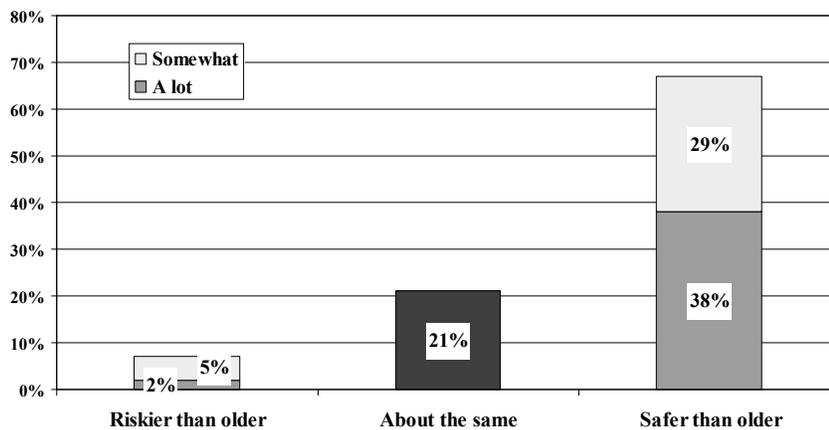
New Products: More or Less Safe



Q74. Compared to currently licensed IGIV products, would you expect new products to be.... N=1141

115

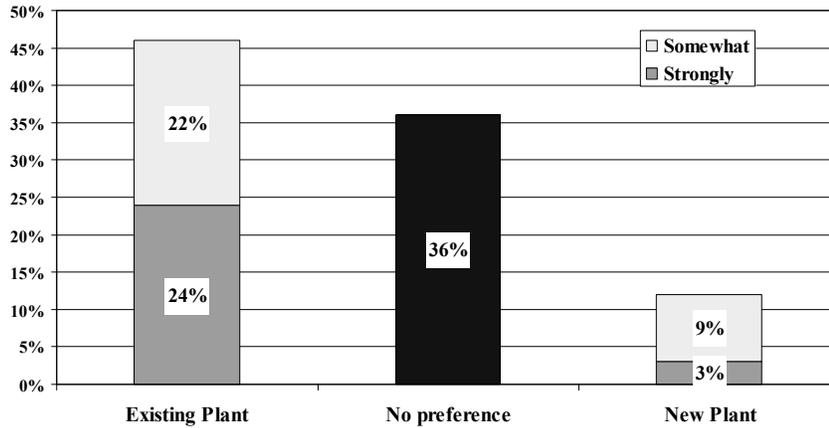
New IVIG Processes: Riskier or Safer



Q51. Compared to products currently on the market, would you expect that new IGIV products approved by the FDA would have manufacturing processes that were.... N=1186

116

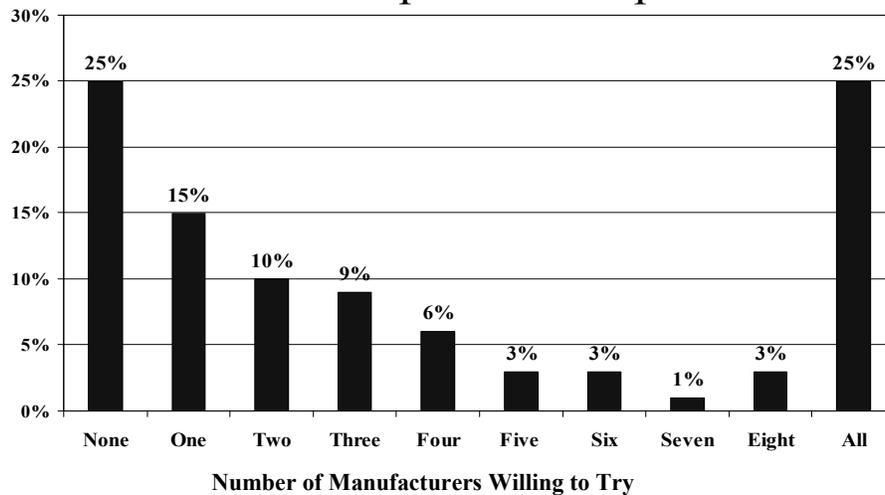
Plant Preference: Old vs. New



Q52. Assuming that both had been inspected and approved by the FDA, would you prefer to get product from an existing plant that had been producing IVIG for fifty years or a newly designed plant than had been producing ICIV for a year? N=1186

117

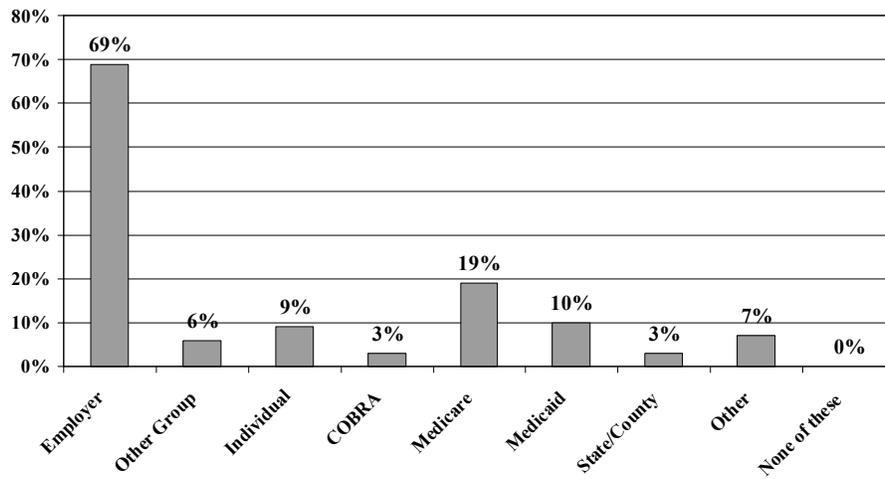
Willingness to Try New Products: Number of Specific Companies



Q75. How willing would you be to try a new FDA approved IGIV product from the following manufacturers? N=1141

118

Type of Current Health Insurance



Q76. What are the current source(s) of the patient's health insurance? N=1141



Immune Deficiency Foundation

40 West Chesapeake Avenue

Suite 308

Towson, MD 21204

800-296-4433

www.primaryimmune.org