

PRIMARY IMMUNE DEFICIENCY DISEASES IN AMERICA: 2002

THE SECOND NATIONAL SURVEY OF PATIENTS



IMMUNE DEFICIENCY FOUNDATION

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

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Overview

The Immune Deficiency Foundation commissioned the Second National Survey of Patients with Primary Immune Deficiency Diseases in the fall of 2002. A total of more than 1,500 patients with primary immune deficiency diseases completed a four-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 estimates of the characteristics and experiences of patients with primary immune deficiency diseases were 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 this survey. This landmark survey provided the first estimates of the size and distribution of the population with primary immune deficiency diseases in the United States. Unfortunately, the information on the health outcomes and treatment characteristics of this population was more than five years old by 2002. Moreover, some events since 1997, such as the shortage in IVIG supplies during 1998-99, may have significantly altered the characteristics of the population.

Consequently, the Immune Deficiency Foundation commissioned a Survey of the Treatment Experiences and Preferences of Patients with Primary Immune Deficiency Diseases in the fall of 2002. A sample of the patients who participated in the first national patient survey was included in the Treatment Experiences Survey. However, in order to avoid limiting the findings of the treatment survey to patients who had been diagnosed at the time of the first survey in 1996-7, the Treatment Experiences Survey was designed to include two strata: patients from the 1996 survey, and new patients who were not included in that 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 the 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 by the end of January 2003.

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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 even 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 1995, 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. 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 their report for the Foundation.

A national sample of 1,500 specialists who reported treating patients with primary immune deficiency diseases had been identified in 1996. The physician sample was sent questionnaires 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 in 1996 and 1997. 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.

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 two page (four sided) 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 (Figure 1).

A total of 1587 completed short form questionnaires were returned by eligible respondents from the 5922 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).

Characteristics of the Patient Population

The Second National Patient Survey confirms that primary immune deficiency diseases are no longer a pediatric condition. Only 38% of the patients with primary immune deficiency diseases in the households participating in the Second National Patient Survey were aged 18 or younger. A third (33%) were young adults aged 18-44.

A quarter (25%) was middle-aged, 45-64 years old. And, a small proportion (4%) of persons with primary immune deficiency diseases was aged 65 or older (Figure 2).

The age distribution of persons in the household with primary immune deficiency diseases in the 2002 survey is virtually identical with the age distribution for the first patient survey in 1996-1997. The first patient survey found 40% of patients were under age 18, compared to 38% in the second patient survey. The first patient survey found 35% of patients were 18-44, compared to 33% in the second patient survey. The proportion of patients aged 65 and older was 5% in 1996-7, compared to 4% in the survey conducted in 2002.

In the majority of households (57%) with any patients, there is one or more adult patients and no children with a primary immune deficiency disease living in the household. In another 6% of households, there are both adults and children with primary immune deficiency diseases in the household. By contrast, there are children, but no adults, with primary immune deficiency diseases in 37% of households (Figure 3).

In cases where there was more than one person with a primary immune deficiency disease in a household, it was necessary to direct the survey recipient on how to select a single designated respondent for the survey. Although random assignment is preferable for unbiased population estimates, the average survey respondent does not know how to make a random or systematic selection. Hence, the questionnaire specified that if an adult patient had children with immune deficiency diseases the adult patient should answer the survey questions concerning their own health and treatment, rather than their affected children. If there were multiple children with primary immune deficiency diseases, and no adult patients, the parent/caregiver was directed to answer the survey questions about the oldest child. This is an easily implemented selection procedure and tends to provide a sample with the longest diagnosis and treatment experience.

These survey procedures do introduce some bias towards older respondents in households with multiple persons with immune deficiency disease. In six percent of households with both adults and children with primary immune deficiency diseases, the adults were selected with certainty. In another five percent of households with more than one affected child, but no adults with a primary immune deficiency disease, the oldest affected child was selected with certainty. However, in almost nine out of ten households, there was only one eligible respondent, so the impact of the selection bias in the age of the sample is limited.

Among the completed sample of patients with a primary immune deficiency disease in the Second National Patient Survey, only 9% of the study subjects were six years of age or less. Thirteen percent were aged seven to twelve. Another 9% were adolescents, aged thirteen to seventeen. In total, about a third of the patient sample for the second national survey was under 18 years of age. Fourteen percent were young adults, aged 18 to 29. One in five (21%) of the patient population was 30 to 44 years old. Another 29% was middle aged, 45 to 64 years old. Only 5% of immune deficient patients are aged 65 or older (Figure 4).

The gender distribution of persons with primary immune deficiency diseases is similar to that of the general population. Among the national sample of patients with primary immune deficiency diseases, 42% were male and 57% were female (Figure 5). This is slightly different than the gender distribution of 48% male and 52% female in the first national survey, as well as the general population as a whole.

Despite the non-probability sampling procedures for the patient survey, the geographic distribution of the patient sample closely mirrors the total population of the United States. Among the nearly 1,500 patients in the sample who were born in the United States, 49 out of the 50 states plus the District of Columbia are represented in their place of birth. In comparing the proportion of the US born patients who were born in a particular Census division to the percent of the US population currently living in that division, the rates are almost identical for New England (7% to 5%), Mid-Atlantic (16% to 15%), South Atlantic (15% to 18%), East South Central (5% to 6%), West South Central (10% to 11%), and Mountain (5% to 6%). There is a higher proportion of patients to population in the second national survey from East North Central (21% to 17%) and West North Central (13% to 7%). There is a lower proportion of patients to population in the Pacific division (9% to 16%). (Figure 6)

In addition, 3% of the patient sample from the Second National Survey was born outside of the United States. Most commonly, these patients were born in Canada, Germany, England or Israel. However, patients with primary immune deficiency diseases in the United States were also born in Bolivia, Cuba, Italy, India, Iraq, Japan, Korea, Norway, Panama, Russia, Singapore, Taiwan, Uganda and Vietnam.

Diagnosis

The prevalence of specific diagnoses in the patient sample is similar to the distribution reported in the 1996 specialist survey. The most common diagnosis is Common Variable Immune Deficiency, which accounts for a majority (52%) of the patient sample (Figure 7). The next most common diagnoses are IgG Subclass Deficiency (12%) and IgA Subclass Deficiency (10%). It should be noted that when a patient reported both IgG and IgA deficiency in the current survey, the diagnosis was treated as common variable immunodeficiency. (In the first national survey, all diagnoses were reported separately.)

X-linked agammaglobulinemia (XLA) is the fourth most common of the primary immune deficiency diseases in the patient sample. The proportion of patients with XLA was 8% in both the first and second national surveys. Smaller proportions of patients report Severe Combined Immune Deficiency (2%), Chronic Granulomatous Disease (2%), Hyper IgM (2%), DiGeorge Anomaly (1%), and Wiskott-Aldrich Syndrome (1%). Seven percent reported other diagnoses and 2% reported no specific diagnosis.

Only 36% of patients were initially diagnosed with a primary immune deficiency disease before age six. And, a majority (50%) was diagnosed before they were eighteen. Nonetheless, 40% of persons with primary immune deficiency diseases were not diagnosed until they were aged 30 or older (Figure 8).

One reason for late diagnosis is the absence of a family history of these disorders. Only 22% of persons with primary immune deficiency disease report that any other family member has been diagnosed as having a primary immune deficiency disease (Figure 9). Only 1% of patients had a father with a primary immune deficiency disease and 4% had a mother with one of these diseases. It is somewhat more common for patients to have either a brother (6%) or sister (4%) with this disorder than a parent. Indeed, about the same proportion have a son (3%) or daughter (2%) with the condition as a parent. In addition, 2% of patients report an uncle, 1% an aunt, 2% a cousin, and 2% some other family member with a primary immune deficiency disease (Figure 10).

While 22% of all patients with primary immune deficiency diseases have a family history of this disorder, the likelihood of family history of primary immune deficiency disease varies with the specific diagnosis. The proportion of patients with a family history is much higher among those diagnosed with severe combined immune deficiency (44%), agammaglobulinemia (42%), Wiskott-Aldrich (40%) and Hyper IgM (39%). By contrast, some of the more common forms of primary immune deficiency have lower than average rates of family history, including common variable (18%) and IgG subclass deficiency (15%). (Figure 11)

Approximately nine out of ten patients with primary immune deficiency diseases (89%) report that they experienced repeated, serious or unusual infections prior to initial diagnosis as immune deficient (Figure 12). About one in four patients (25%) either had no symptoms prior to diagnosis or were diagnosed within a year of onset of repeated, serious or unusual infections. Another 20% were diagnosed within one to two years of symptom onset. Nonetheless, the time between the onset of symptoms and diagnosis was 3-5 years for 16% of patients; 6-9 years for 10% of patients; 10-14 years for 8% of patients; and 15-19 years for 5% of patients. One in seven patients with primary immune deficiency diseases (17%) in this sample report 20 years or more between the onset of repeated, serious or unusual infections and their initial diagnosis as immune deficient. The average time between symptom onset and initial diagnosis was 9.2 years (Figure 13).

The second national survey of primary immune deficiency diseases suggests that the time to diagnosis for immune deficient patients is not improving. The average number of years between symptom onset and diagnosis was 4.7 years for those initially diagnosed before 1970. It was 7.0 years for those initially diagnosed in the 1970's. It was 6.2 years for those initially diagnosed in the 1980's. The number of years between symptom onset and initial diagnosis was 10.6 years in the 1990's. And, among those patients in the sample who were first diagnosed since 2000, the average number of years from symptom onset to diagnosis was 9.9 years (Figure 14).

The time to diagnosis varies considerably by the specific primary immune deficiency disease. The average time from onset to diagnosis was a year or less for severe combined immune deficiency (.4 years), DiGeorge syndrome (.2 years), Wiskott-Aldrich (.6 years), and Chronic Granulomatous Disease (1 year). The average number of years between symptom onset and diagnosis was 3 years for Agammaglobulinemia. By contrast, the average time to diagnosis for the three most common diagnoses was 11.4 years for Common Variable Immunodeficiency, 11.6 years for IgG subclass deficiency and 9.3 years IgA deficiency. Despite a relatively high proportion of cases with family histories of immune deficiency, the average time to diagnosis for Hyper IgM disease was 8.7 years (Figure 15).

When asked why the patient was initially tested for immune deficiency disease, the majority of patients (57%) say it was due to repeated infections. By contrast, 17% say that they were initially tested for immune deficiency because of serious infections, and 9% say that they were tested due to unusual infections. Although 22% report that they now have a family history of primary immune deficiency disease, only 6% say that they were initially tested because of a family history of the condition. It is far more common for a patient to be diagnosed with immune deficiency as a result of a test for some other illness (16%). (Figure 16)

The importance of early diagnosis of these diseases is demonstrated by medical history prior to diagnosis. Although nearly two out of five patients are diagnosed before age six, the majority of patients (61%) report being hospitalized prior to diagnosis. Seventeen percent were hospitalized only once prior to diagnosis. Another 18% were hospitalized 2 to 3 times prior to diagnosis. But a quarter of immune deficient patients reported being hospitalized 4 or more times before diagnosis. Nearly one in ten persons with primary immune deficiency diseases (9%) report 10 or more hospitalizations before diagnosis (Figure 17).

Not surprisingly, then, the initial diagnosis as immune deficient is frequently made during a hospitalization. Nearly a quarter of immune deficient patients (23%) report that their diagnosis as immune deficient was first made during a hospitalization (Figure 18).

Also, despite the relatively early age of diagnosis, many patients report permanent functional impairment prior to initial diagnosis as immune deficient. Nearly a quarter (23%) report that they had suffered permanent loss of lung function prior to initial diagnosis as immune deficient. About one in ten reported permanent loss of hearing (11%) or digestive function (9%) prior to initial diagnosis as immune deficient. Smaller proportions report permanent loss of mobility (7%), vision (2%), neurological (2%) or other (6%) function by the time of initial diagnosis. In total, 37% of patients with primary immune deficiency disease report some type of permanent functional impairment prior to diagnosis (Figure 19).

The length of time between symptom onset and diagnosis as immune deficient affects the likelihood of permanent impairment before diagnosis. The average time to

diagnosis is more than twice as long for those who suffered permanent loss of digestive function (18.2 years) compared to those who did not (8.3 years). The average time to diagnosis is more than fifty percent longer for persons with permanent loss of vision (16.4 to 9.0 years), mobility (14.5 to 8.8 years) and lung function (13.9 to 7.8 years) prior to diagnosis. The time to diagnosis is about a third longer for persons with permanent hearing loss before diagnosis (12.3 years) compared to those without (8.8 years). The one exception to this pattern is neurological disorders in which those with these impairments have a shorter average time to diagnosis (5.9 years) compared to those who have not suffered a permanent neurological disorder (9.2 years) prior to diagnosis. This probably indicates that diseases that cause serious neurological disorders may lead to earlier diagnosis of immune deficiency (Figure 20).

The longer the time to diagnosis from symptom onset, the greater the number of permanent functional impairments. Those who reported no permanent functional impairments by the time of initial diagnosis as immune deficient reported an average of 7.4 years between symptom onset and diagnosis. The time to diagnosis increases to 10.2 years for those with one area of functional impairment, to 13.8 years for those with two areas of functional impairment, and 15.1 years for those with three areas of functional impairment prior to diagnosis. The average length of time to diagnosis increases to 22.3 years for those with four areas of permanent impairment and 25.9 years for those with five areas of permanent impairment prior to diagnosis as immune deficient (Figure 21).

Co-Morbidities

Since the current health and quality of life of patients with primary immune deficiency diseases may be affected by medical conditions other than the primary immune deficiency disease, the national sample of patients was asked about other serious or chronic health conditions. These conditions may or may not be related to the immune deficiency disease. But, at minimum, they represent important co-morbidities with the immune deficient condition.

About one in twelve patients with a primary immune deficiency disease (8%) report that they have ever had cancer or leukemia (Figure 22). Although more than nine out of ten immune deficient patients have never had cancer, the prevalence of cancer reported in this population is higher than the rate expected in the general public. This is particularly notable because nearly two out of five immune deficient patients are under age 18.

The lifetime prevalence of cancer is 1.4% for immune deficient patients under age 6, compared to 3.1% of those aged 7 to 12, and 1.4% for those aged 13 to 17. The cancer prevalence among immune deficient patients aged 18 to 29 is basically the same (1.4%) as the rate among those under age 18. There is also almost no difference between the cancer prevalence among 18 to 29 year olds with a primary immune deficiency disease (1.4%) and the general population of the United States aged 18-29 (1.1%) based on the National Health Interview Survey (Figure 23).

However, when the prevalence of cancer is compared between the adults aged 30 and older from the Second National Patient Survey and the National Health Interview Survey, immune deficient patients report higher lifetime rates of cancer. Among persons aged 30 to 44 years old, the prevalence of cancer is 5.1% for immune deficient patients, compared to 2.8% for the general public. Among persons aged 45 to 64 years old, the prevalence of cancer is 14.3% for immune deficient patients, compared to 7.3% for the general public. Among persons aged 65 or older, the prevalence of cancer is 28.4% for immune deficient patients, compared to 17.8% for the general public.

The prevalence of cancer among patients with primary immune deficiency diseases varies by specific diagnosis. The highest rates of lifetime cancer are found among those with common variable immunodeficiency (9.1%), Hyper IgM (10.7%), Wiskott-Aldrich (13.3%) and those who report one of the less common diagnoses (14.0%) or no diagnosis (7.9%). By contrast, the rates of cancer are substantially lower among those diagnosed with IgA deficiency (4.6%), IgG subclass deficiency (4.5%), agammaglobulinemia (3.2%) and severe combined immunodeficiency (2.8%). None of the patients with chronic granulomatous disease or DiGeorge syndrome reported ever having cancer or leukemia (Figure 24).

In comparing the rate per thousand by cancer site with the U.S. adult population, the immune deficient population has lower rates than the public for prostate cancer (2 versus 6) and uterine cancer (2 versus 4). However, the age difference between the two populations may eliminate the apparent advantage for the immune deficient population. Similarly, although the rates per site are virtually identical between the immune deficient population and the general adult population for ovarian cancer (2 v 2), lung cancer (2 v 2), melanoma (4 v 3), cervical cancer (5 v 6), and skin cancer other than melanoma (15 v 16), the age difference between the two populations means that immune deficient patients will have higher age adjusted rates for all of these types of cancer. However, even with an age advantage, the rate per thousand for lymphomas is five times higher among immune deficient patients (10) than among the general adult population (2). (Figure 25)

The prevalence of lymphomas is also distributed unevenly by specific diagnosis among patients with primary immune deficiency disease. In addition to the two diagnoses with no cases of cancer of any type, there were no cases of lymphoma reported by patients with severe combined immunodeficiency, Wiskott-Aldrich syndrome, other types of immune deficient, and those who reported no diagnosis. By contrast, rates of lymphoma that exceeded the population rates were reported by persons with IgA deficiency (.7%), agammaglobulinemia (.8%), IgG subclass deficiency (1.1%), and common variable immunodeficiency (1.3%). But patients with a diagnosis of Hyper IgM had the highest rates (3.6%) of lymphoma (Figure 26).

One in fourteen patients with a primary immune deficiency disease (7%) reports that they have had hepatitis (Figure 27). Most commonly, they report Hepatitis C (1.6%). Smaller proportions report having had Hepatitis B (1.0%) and Hepatitis A (.6%). They also report viral hepatitis (.3%), infectious hepatitis (.2%), mono-related hepatitis (.2%), and auto-immune hepatitis (.2%). Some patients (.3%) specifically describe their type of

hepatitis as drug or medication induced hepatitis. In many cases, the type is not specified (.9%), cannot be classified (.9%) or the patient is not sure of the type (.5%). (Figure 28)

The prevalence of reported hepatitis among patients with primary immune deficiency diseases is nearly twice as high among current users of IVIG (8.0%) compared to patients who have never used IVIG (4.6%). The prevalence of hepatitis among those who have used IVIG in the past, but not currently, is similar to the rate reported by those who have never used IVIG (5.5%). (Figure 29)

There is a striking difference in the rate of hepatitis by age among immune deficient patients. The proportion of patients reporting hepatitis (of any type) increases from less than 1% for those aged 6 or younger, to 2% for those aged 7 to 12, to 4% for those aged 13 to 17, and 5% for those aged 18 to 29. Then the prevalence of hepatitis increases substantially to 8% for those aged 30 to 44, to 11% for those aged 45 to 64, and to 14% for those aged 65 and older (Figure 30).

One in five patients with a primary immune deficiency disease (21%) reports that they have had a neurological disorder (Figure 31). The most common types of neurological disorder reported by immune deficient patients are epilepsy or seizures (3.6%) and migraines (3.4%). Neuropathy (1.3%), peripheral neuropathy (.8%), extremity numbness (.7%) and tremors (.7%) are also reported. A small proportion of immune deficient patients report meningitis (.6%) and encephalitis (.2%). (Figure 32)

A substantial proportion of immune deficient patients aged 6 and under (10%) report some form of neurological disorder (Figure 33). However, the prevalence of reported neurological disorders increases to 16% of those aged 7 to 12 years old, to 22% of those aged 13 to 17 years of age. The prevalence of neurological disorders remains virtually unchanged for those aged 18 to 29 (23%), 30 to 44 (24%), 45 to 64 (25%), and 65 or older (22%).

The majority of patients with primary immune deficiency disease (53%) report they suffer from other serious, chronic disease, not counting the primary immune deficiency (Figure 34). Most commonly, they report asthma (17.8%) and sinusitis (8.3%) as the other serious or chronic condition. However, chronic lung disease (3.9%), arthritis (3.5%), diabetes (2.8%), GERD (2.2%) and hypothyroidism (1.6%) are reported by smaller but significant subsets (Figure 35).

Nearly two out of five (38%) immune deficient patients, aged 6 or younger, are reported to suffer from other serious chronic disease, not counting their immune deficiency. The prevalence of other chronic diseases is about the same for patients aged 7 to 12 (45%), 13 to 17 (46%), and 18 to 29 (46%). The proportion of patients reporting other serious chronic disease increases to 55% of those aged 30 to 44, and 66% of those aged 45 to 64. The rate of serious chronic disease falls slightly to 61% of those aged 65 and older (Figure 36).

The development of permanent functional impairments as a result of disease prior to diagnosis as immune deficient contributes to the prevalence of chronic conditions among the patient population. Less than half of patients with no permanent loss of function prior to diagnosis (46%) report any other serious chronic diseases. The prevalence of other serious chronic diseases increases to 61% of those with one functional impairment before diagnosis, to 65% for those with two permanent impairments, and to 87% of those with three or more (Figure 37). Nonetheless, nearly half of immune deficient patients who did not suffer any permanent impairment prior to diagnosis report having other serious chronic conditions besides their immune deficiency.

Current Health

Nearly three out five patients with primary immune deficiency diseases describe their current health status as good or better. Specifically, 8% describe their current health status as excellent, 21% as very good, and 30% as good. Twenty-eight percent describe their current health as only fair. Only 12% of patients report their current health status as poor or very poor (Figure 38).

If the current health rating of immune deficient patients is compared to the general public, using an equivalent measure from the National Health Interview Survey, it is clear that patients do not feel as healthy as the general public of the same age. For example, 98% of the general public under the age of 18 rates their health as excellent, very good or good, compared to 72%-76% of persons with primary immune deficiency disease less than 18 years of age. Among persons aged 45 to 64 in the general public, 85% rate their health as good or better, compared to only 43% of immune deficient patients in that age range. Among persons aged 65 and older in the general public, 73% rate their health as good or better, compared to only 45% of immune deficient patients in that age range (Figure 39).

The comparatively low rating of current health by immune deficient patients is strongly affected by the development of permanent functional impairments prior to diagnosis. Nearly seven out of ten patients with no permanent impairments prior to diagnosis (69%) rate their health as good or better. The proportion of patients who rate their current health as good or better declines to 51% of those with one permanent impairment, to 35% of those with two permanent impairments, to 24% of those with three or more permanent impairments (Figure 40).

Most patients with primary immune deficiency diseases report only slight (29%) or no physical limitations (30%) as a result of health. A quarter (25%) reports moderate limitations as a result of their health. One in seven patients (14%) reports severe physical limitations as a result of their health (Figure 41).

Current activity limitation, like current health rating, is strongly affected by the development of permanent functional impairments prior to diagnosis. Less than three out of ten patients with no permanent impairments prior to diagnosis (28%) say their activities are severely or moderately limited as a result of their health. The proportion of

patients who say their activities are severely or moderately limited by their health increases to 47% of those with one permanent impairment, to 63% of those with two permanent impairments, to 85% of those with three or more permanent impairments (Figure 42).

The national sample of patients with primary immune deficiency disease was also asked about their experience with acute conditions in the past 12 months. Nearly half of the patients (45%) report having bronchitis in the previous 12 months. A third (34%) report repeated diarrhea in the past 12 months. A quarter of immune deficient patients in the second national survey report repeated ear infections in the past 12 months. About one in six report pneumonia (17%), urinary infections (17%), candida (17%), and eye infections in the past 12 months. Nearly one in ten immune deficient patients (8%) reports malabsorption in the past 12 months. A relatively small proportion reports lymphopenia (3%), neutropenia (3%), and sepsis (2%) in the past 12 months. A third (33%) of the immune deficient patients volunteered other conditions in the previous 12 months. Only 17% reported none of these conditions in the past year (Figure 43).

Three out of ten patients with primary immune deficiency diseases (30%) report they had been hospitalized overnight or longer in the past year (Figure 44). The nature of the hospitalization was not determined in this survey. There is a relationship between the rate of hospitalization in the past year and permanent functional impairment prior to diagnosis. Among those persons with immune deficiency disease who had no permanent impairment prior to diagnosis, 27% reported a hospitalization in the past 12 months. The rate of past year hospitalization increases to 33% for those with one functional impairment, 39% of those with two impairments, and 47% of those with 3 or more permanent impairments prior to diagnosis (Figure 45).

It should be noted that the general health measures among persons with primary immune deficiency diseases appear to be somewhat poorer for the national patient sample in 2002, compared to the 1996-7 survey. More than two thirds (68%) of the immune deficient patients in 1996-97 survey reported their health as good or better, compared to 59% in the 2002 survey. Seventy percent of the patients in the 1996-97 survey reported slight or no physical limitation, compared to 59% in the current survey. And, 76% of immune deficient patients reported no hospital nights in the past year in 1996-97, compared to 69% in 2002. These differences are statistically significant, meaning the differences in reported health status between the First Patient Survey and the Second Patient Survey exceed the expected differences from sampling variability.

Health Care Access and Utilization

The type of doctor seen most often by the patient for his or her health care is split between primary care and specialists. Nearly two out of five patients report that the doctor they see most often for their health care is in pediatrics (10%), family practice (17%) or internal medicine (10%). Almost half report that the doctor they see most often for their health is a specialist in immunology (24%), allergy (13%), hematology (3%), or

other medical specialty (9%). About one in seven patients (14%) reports seeing more than one kind of doctor most often for their health (Figure 46).

Most patients usually visit their primary care doctor in a private office (67%). About one in five patients (19%) usually see their doctor in a group practice or HMO setting. About one in seven immune deficient patients (13%) usually see their doctor in a hospital outpatient or hospital clinic. Very few report that they usually see their doctor in a public health clinic (2%) or somewhere else (1%). (Figure 47)

Most persons with primary immune deficiency diseases have some form of health insurance coverage. The majority (69%) has insurance through an employer group policy. Another 9% have their health insurance from a non-employer group policy, while 6% have an individual policy. Only 15% have Medicare coverage, 11% have Medicaid coverage, and 4% are covered by a state or county health program. A handful has their health care coverage through COBRA (3%), VA/Military health programs (2%), or other health insurance (3%). Only one percent reports none of these (Figure 48).

The vast majority of immune deficient patients (70%) report that they have been seen by an immunologist in the past 12 months (Figure 49). Insurance and type of insurance has some impact on the likelihood that an immune deficient patient will have seen an immunologist in the past 12 months. Those who report no insurance have a substantially lower likelihood of seeing an immunologist (44%) than the average patient. Those whose health coverage is through the VA or military (54%) and COBRA (58%) are also well below average. Immune deficient patients on Medicare (65%) are also less likely than those with health insurance through work (72%), other group (73%) or individual policies (72%) to have seen an immunologist in the past year (Figure 50).

Almost nine out of ten patients (88%) say that they are able to see a specialist as often as needed. Nonetheless, 11% of this national sample of persons with primary immune deficiency diseases says that they are not able to see a specialist as often as needed (Figure 51). Insurance and type of insurance has some impact on the ability of an immune deficient patient to see a specialist as often as needed. Those who report no insurance have a substantially less access to a specialist when needed (44%) than the average patient. Those whose health coverage is through the VA or military (79%) and Medicaid (79%) are less likely than those with health insurance through work (92%), other group (89%), individual policies (88%) or COBRA (91%) to feel they have access to a specialist when needed (Figure 52).

Although most patients are covered by some form of health insurance or public health program, many have experienced insurance problems. Ten percent have had a health insurance application denied, while 4% have had a policy cancelled. Nine percent have had conditions excluded from their coverage. Twenty-one percent have had treatment delayed by their insurance carrier, while 17% have had treatment denied by their carrier. Three percent of patients with primary immune deficiency disease say that they have exceeded the lifetime cap of the insurance coverage. The majority of persons

with primary immune deficiency diseases (62%), however, have not experienced any of these health insurance problems (Figure 53).

Treatment

Four out of five patients with primary immune deficiency disease in the Second National Patient Survey report that they have been treated with intravenous gammaglobulin (IGIV) for their disorder. Two thirds (67%) are currently being treated with IGIV. However, one in eight patients with a primary immune deficiency disease (13%) have stopped taking IGIV (Figure 54).

Nearly half of the patient sample for the 2002 Patient Survey who had ever been treated with IGIV on a regular basis, had been treated for less than five years (46%). A quarter (25%) had been treated with IGIV for five to nine years. Sixteen percent had been treated for 10-14 years. But, 13% of the IVIG users had been treated with the product for fifteen years or more (Figure 55).

The discontinuing IGIV users were asked why they were no longer being treated with IGIV. Some discontinuing IGIV users reported that better health (12%) and/or normal to near normal immunoglobulin levels (12%) were the reasons that stopped the therapy. Others did so because their doctors didn't think it was necessary (10%), they felt that there were no real benefits (9%) or they wanted to see how they did without IGIV treatment (8%). A small proportion had been cured by bone marrow transplantation (4%). Nonetheless, some patients diagnosed with primary immune deficiency disease reported that they had discontinued this treatment because of insurance coverage (14%), side effects or reactions to IGIV (12%), fear of contracting disease through the product (3%), costs or coverage of treatment (5%), or lack of product availability (3%). Nearly half of patients with primary immune deficiency diseases who had discontinued using IGIV had one of these "bad" reasons for not using IGIV (Figure 56).

Current IVIG use varies by diagnosis among primary immune deficiency diseases. It is most common for X-linked Agammaglobulinemia (93%). It is also commonly reported by four out of five of those with Common Variable Immunodeficiency (78%), Hyper IgM (82%) and severe combined immunodeficiency (83%). It is also reported by a majority of patients with IgG Subclass Deficiency (60%). It is least commonly used for DiGeorge Anomaly (33%), IgA deficiency (16%) and Chronic Granulomatous Disease (4%). (Figure 57)

The frequency of IGIV infusions varies considerably among persons with primary immune deficiency diseases. A majority (54%) reports that, on average, they get an infusion every four weeks. Nearly three in ten (29%) reports they get an infusion every three weeks. One in ten (9%) say they get their infusion every two weeks or more often. A slightly smaller proportion says that they get their infusion every five weeks or less often (5%). (Figure 58)

Most commonly, patients report that the average number of grams per infusion is either 20-29 grams (21%) or 30-39 grams (21%). One in eight (13%) patients report 10-19 grams per infusion. Only a handful (5%) reports infusions of less than ten grams. However, nearly one in five patients (18%) report infusion doses of 40 grams or more. Two out of five patients or caregivers (22%) did not know the average number of grams of IGIV they used per infusion. Among those who did how many grams they were receiving, the average number of grams per infusion was 31.3 (Figure 59).

More than a third of IGIV users in the Second National Patient Survey report that they usually receive their infusion at home, either self-infused (7%) or nurse-infused (28%). A third (32%) IGIV users usually get their infusion in a hospital outpatient setting, while a small proportion (3%) usually gets their infusion in a hospital in-patient setting. Another third of the patients says that they usually get their infusion in a doctor's private office (14%) or an infusion suite (18%). (Figure 60)

The sample of IGIV users were asked which of eight currently licensed IGIV preparations, plus any other, they had ever used. Two out of five current users in the Second National Patient Survey (40%) reported using only one IGIV product. Another quarter (27%) reported lifetime use of only two products. Less than one in five reported using three products (11%) or four products (6%). And only 3% reported lifetime use of five or more IGIV products. One in eight (13%) of these IVIG users could not name any of the products they had used (Figure 61).

More than a quarter of current IGIV users (28%) report experiencing serious side effects or reactions from IGIV treatment in the past 12 months (Figure 62). What types of side effects or reactions to users experience from IGIV? Most commonly, current IVIG users report headaches (9%) from their infusions in the past year. Somewhat less commonly, they report fever (6%), nausea (3%), chills (3%), migraines (3%) and vomiting (3%) as the side effect or reaction from IGIV in the past year. They also report shortness of breath (2%), fatigue (2%), high blood pressure (2%), skin rash (2%), hives (2%), and shaking (2%). (Figure 63)

Demographics

The vast majority of patients with primary immune deficiency diseases in the Second National Patient Survey describe their race as white/non-Hispanic (92%). Only 6% of patients describe themselves as American Indian/Alaskan Native, Asian/Pacific Islander, Black/African American, Hispanic or Latino, Mixed or Something Else. Two percent did not answer this question (Figure 64). Since there is no known racial or ethnic genetic predisposition to primary immune deficiency disease, the surfeit of non-Hispanic whites among the patient sample, compared to the general population, probably represents a problem of diagnosis among minority populations.

The majority of the adult patients or caregivers of children with primary immune deficient diseases are employed full time (44%) or part time (9%). Although the proportion of employed adults in the sample is less than the national average, it should be

noted that the sample is younger than the national average and another 12% are students. Another 7% are homemakers. However, one in seven patients or caregivers describes their employment status as disabled (16%). (Figure 65)

More than two out of five adult patients or caregivers of children with primary immune deficiency diseases have a college degree (42%), including 19% with a graduate degree. Another 30% have some college. Only 9% have less than a high school degree, while 18% are high school graduates (Figure 66).

Conclusions

Primary immune deficiency diseases are a set of comparatively rare genetic disorders. The First National Patient Survey suggests that approximately 50,000 persons in the United States have been diagnosed with one of these diseases. Hence, primary immune deficiency diseases are more common in the United States than some better known genetic disorders, including hemophilia (less than 15,000), cystic fibrosis (30,000) and Huntington's Disease (30,000), among others.

Half of all persons with primary immune deficiency diseases are not diagnosed until they are adolescents or older. One problem for early diagnosis is that the vast majority of patients have no family history of immune deficiency disease. Nine out ten patients report repeated, serious or unusual infections prior to diagnosis. Indeed, most patients report that they were initially tested for immune deficiency because of repeated infections, serious infections or unusual infections. Unfortunately, the average time between the onset of symptoms and initial diagnosis as immune deficient was 9.2 years for this patient population.

The cost of late diagnosis is a heavy burden of disease on the patient. Nearly two out of five immune deficient patients (37%) report suffering permanent functional impairments prior to diagnosis. The likelihood of permanent impairment of lung function, mobility, digestive function, vision and hearing is related to the time between symptom onset and initial diagnosis of immune deficiency.

Only three out five persons with primary immune deficiency diseases describe their current health as good, very good or excellent. Most say their health causes no limitations or only slight limitations on work, play and other activities. Seventy percent have had no hospitalizations in the past year. Nonetheless, the general health, activity limitation and hospitalization rates for persons with primary immune deficiency disease are measurably poorer than the general public. A significant proportion of this difference is related to permanent functional impairments suffered prior to diagnosis and treatment as immune deficient.

The most common form of treatment for primary immune deficiency diseases is intravenous gammaglobulin (IVIG). Seven out of ten patients report being treated with

IVIG for their condition. The proportion of PID patients being treated with IVIG has been increasing at a rate of about fifteen percent per annum in recent years.

Most patients are currently covered by some form of health insurance or health plan, most commonly through employer group insurance. Nonetheless, many immune deficient patients encounter problems with health insurance. For example, 21% have had treatment delayed by their insurance carrier and 17% have had treatment denied by their insurance carrier. Nearly two out of five immune deficient patients (38%) have experienced at least one type of health insurance problem.

Despite a generally positive outlook after diagnosis for most patients with primary immune deficiency diseases, the survey finds that a significant portion of the patient population faces barriers to timely and effective treatment of their condition. In addition, a significant number of patients with primary immune deficiency disease are only diagnosed after multiple hospitalizations. The long-term outlook for most patients with primary immune deficiency diseases, while good, could be greatly improved by earlier diagnosis and better access to appropriate care and treatment.

Figure 1

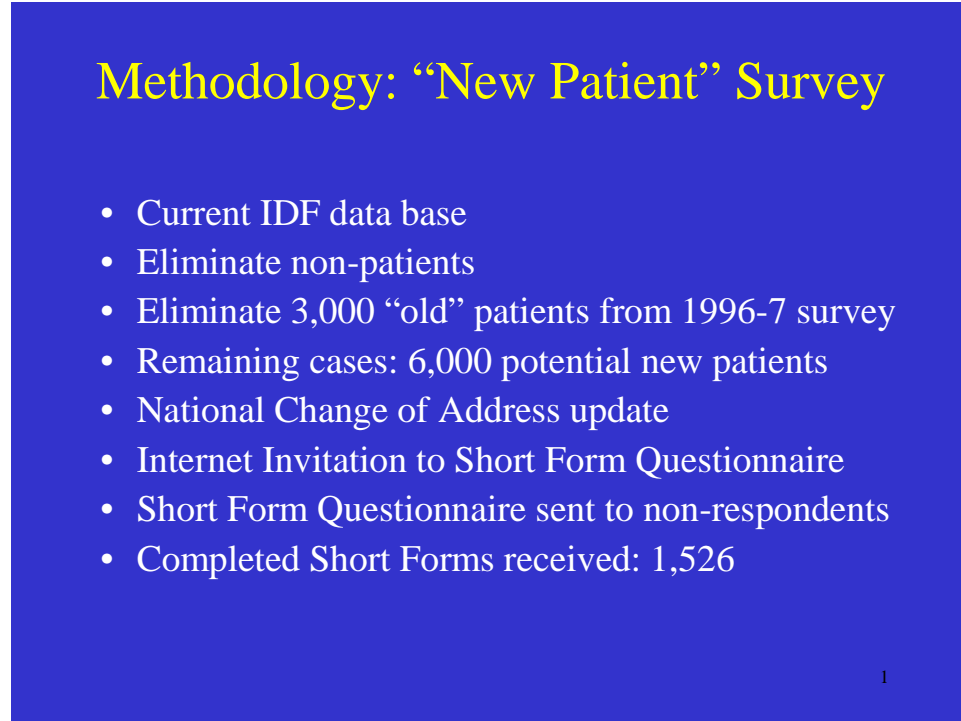
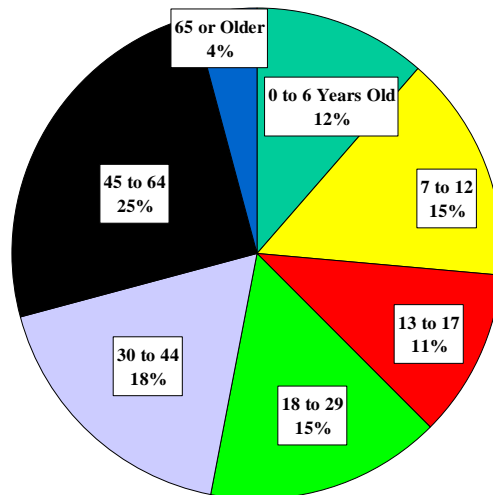


Figure 2

All Patients in Household by Age

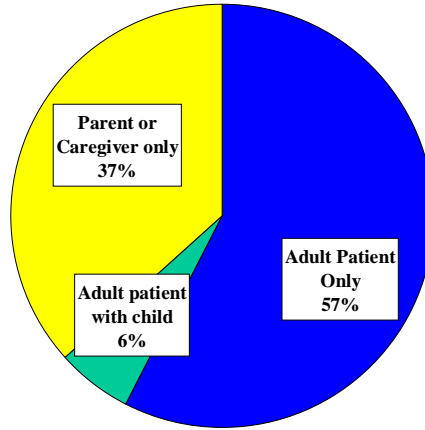


Q5. What is the date of birth of the (adult patient/oldest child) in the household with a primary immune deficiency disease? Q4. What are the ages of those children? (Base: N=1,780)

2

Figure 3

Adult or Child

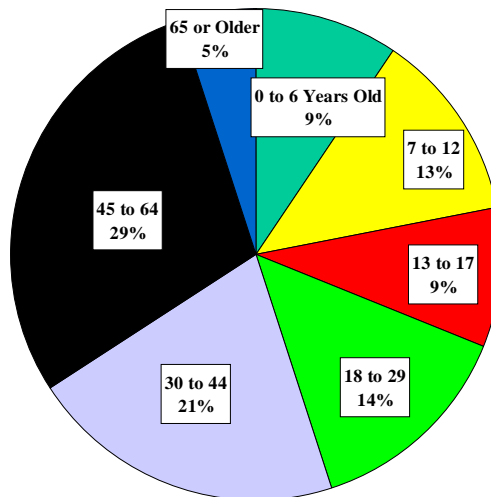


Q1. Are you a patient with a primary immune deficiency disease (PID) or a parent/caregiver of a child in the household with PID? Q3. How many children living in your household have primary immune deficiency disease (Base: N=1,526)

3

Figure 4

Patients by Age

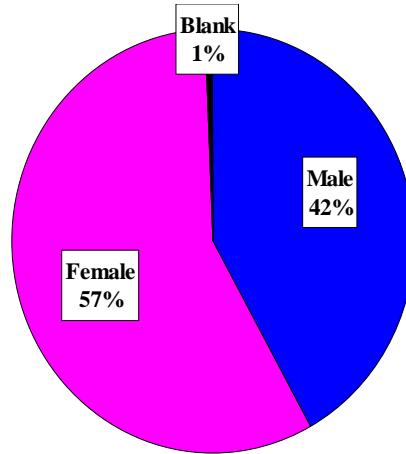


Q5. What is the date of birth of the (adult patient/oldest child) in the household with a primary immune deficiency disease? (Base: N=1,512 – excludes blanks)

4

Figure 5

Gender of Patient

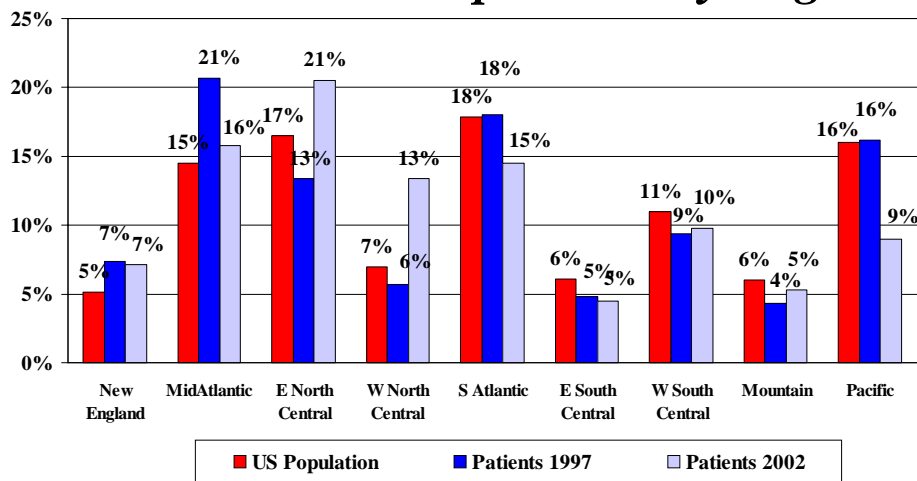


Q7. What is the gender of that person? (Base: N=1,526)

5

Figure 6

Distribution of 1997 and 2002 Patients and Overall US Population by Region

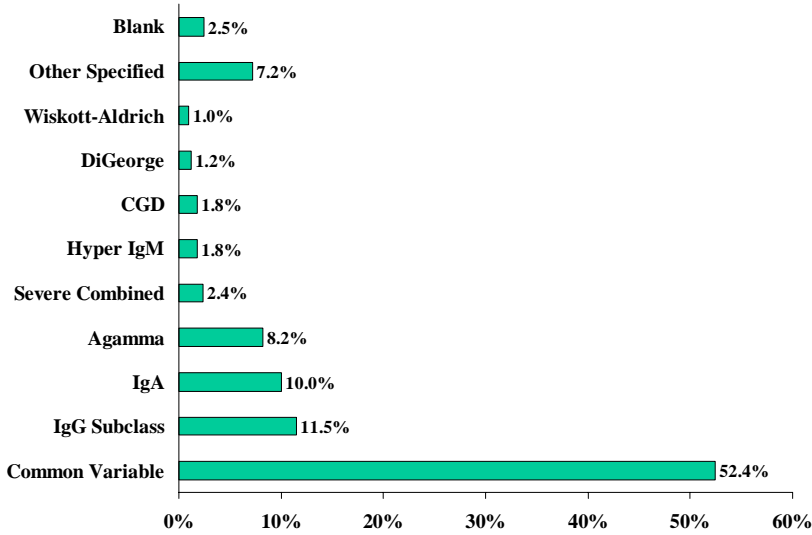


Q6. In what state was he/she born? (Base: N=1,465 - excludes 3% of cases born outside of U.S.)

6

Figure 7

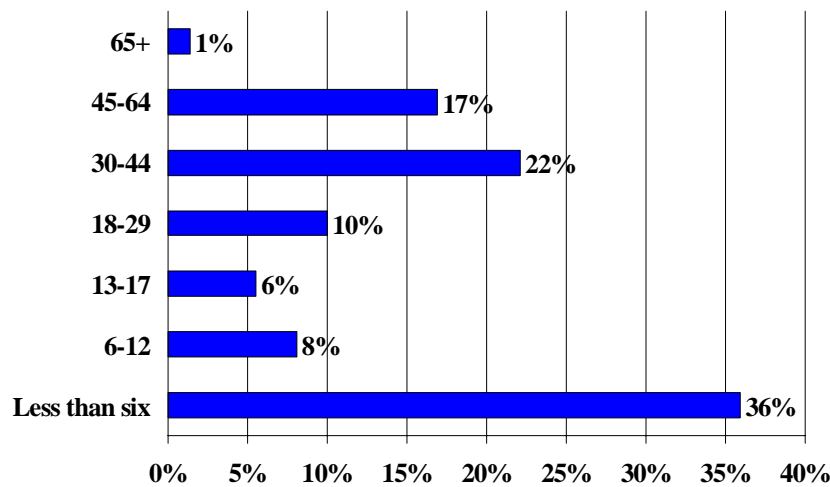
Current Diagnosis



Q10. What is the specific diagnosis of that person's immune deficiency disease (current diagnosis if more than one)? (Base: N=1,526) 7

Figure 8

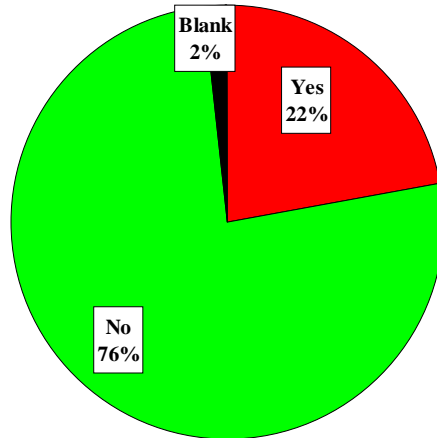
Patient Age at PID Diagnosis



Q8. At what age (in years) was that person first diagnosed with a primary immune deficiency disease? (Base: N=1,499 – excludes blank data) 8

Figure 9

Family History of Immune Deficiency

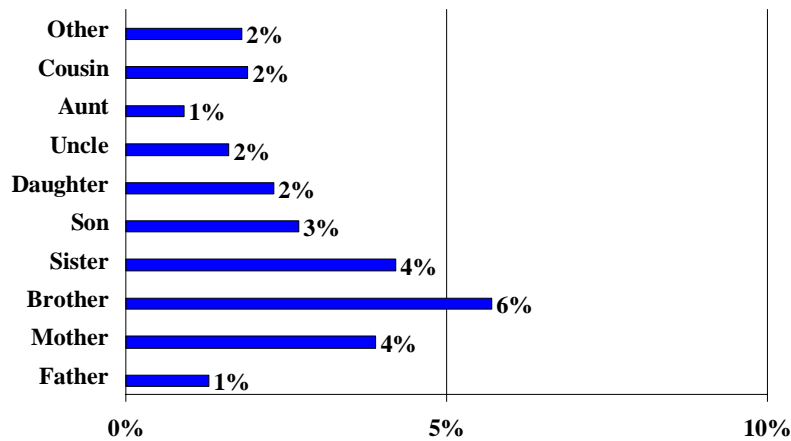


Q11a. Have any other family members been diagnosed as having a primary immune deficiency disease? (Base: N=1,526)

9

Figure 10

Family History of PID

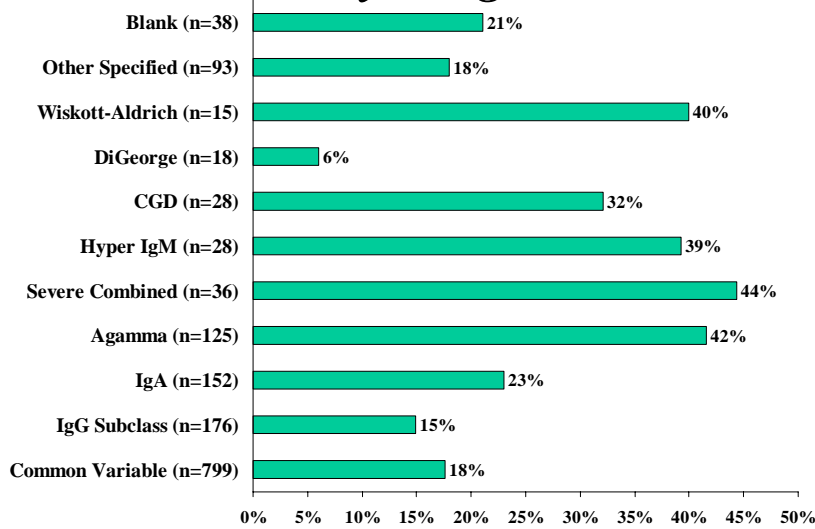


11b. Which other family member (s) have been diagnosed with a primary immune deficiency disease? (Base: N=1,526)

10

Figure 11

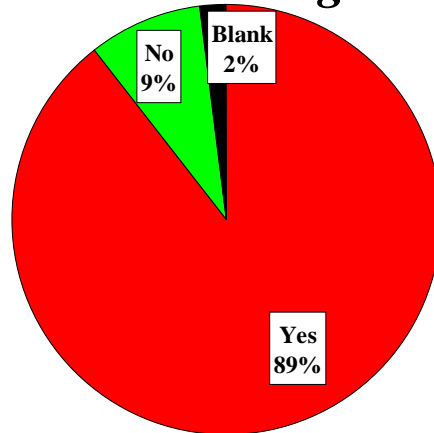
Family Member Diagnosed with PID by Diagnosis



Q11a. Have any other family member (s) been diagnosed as having an immune deficiency disease? 11

Figure 12

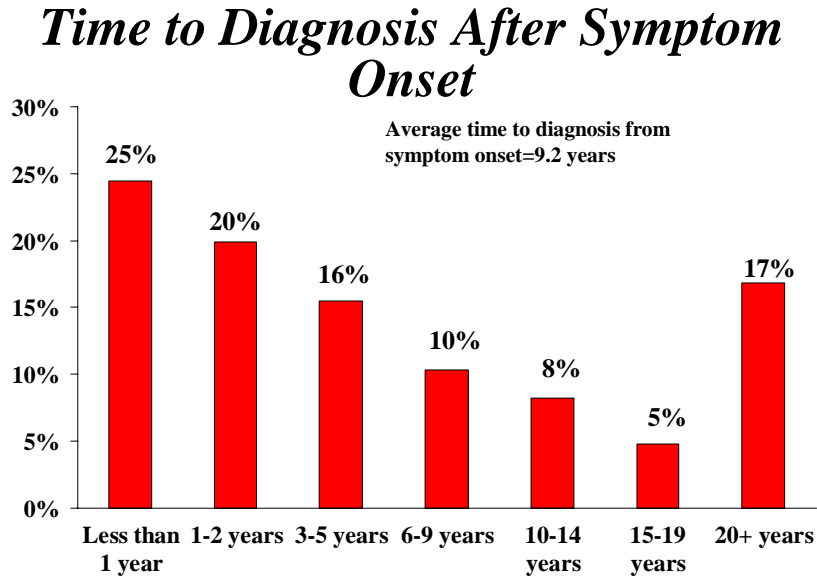
Repeated or Unusual Infections Prior to Diagnosis



Q12a. Did the PID patient exhibit repeated, serious or unusual infections prior to initial diagnosis as immune deficient? (Base: N=1,526)

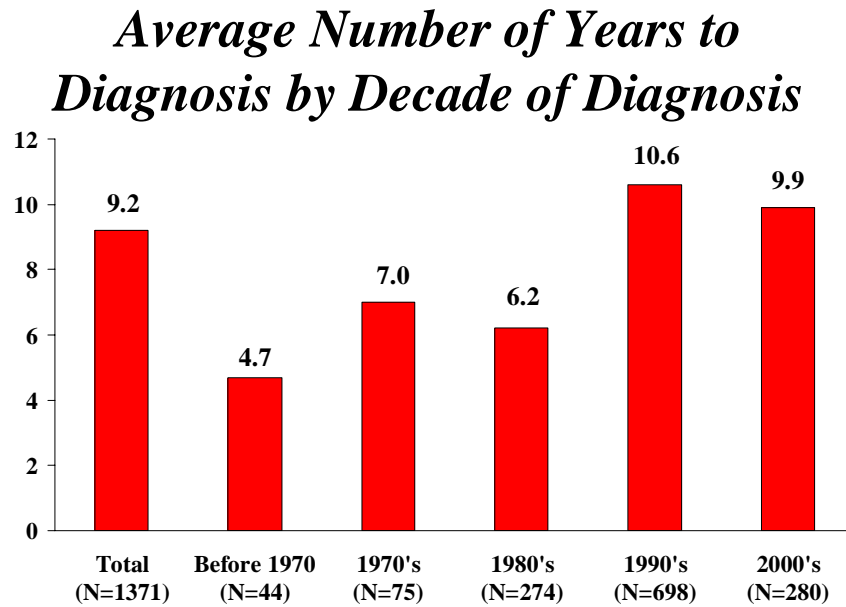
12

Figure 13



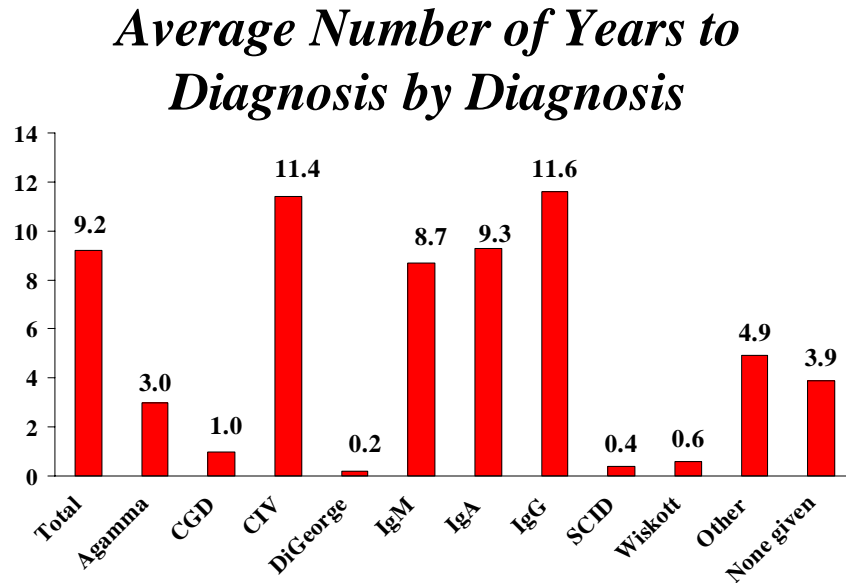
Q8. At what age (in years) was that person first diagnosed with a primary immune deficiency disease?
 Q12b. At what age (in years) did these repeated, serious or unusual infections begin? 13
 (Base: N=1,397 – excludes those with missing information)

Figure 14



Q8. At what age (in years) was that person first diagnosed with a primary immune deficiency disease? Q12b. At what age (in years) did these repeated, serious or unusual infections begin? 14

Figure 15

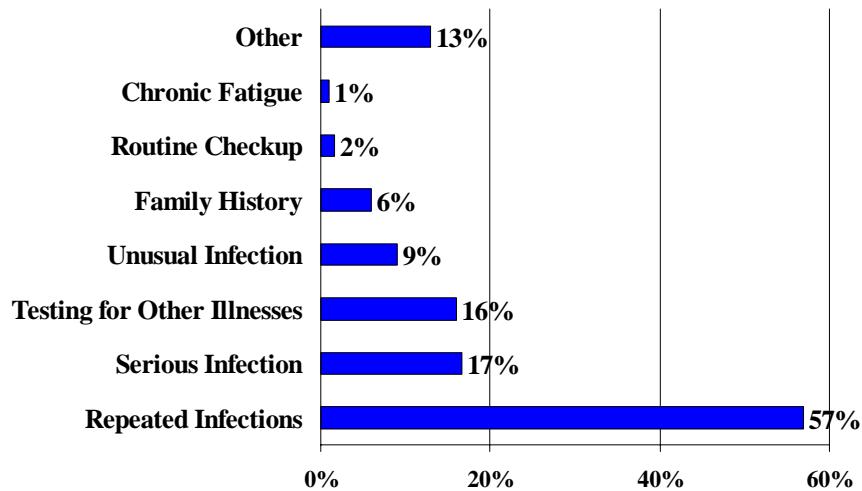


Q8. At what age (in years) was that person first diagnosed with a primary immune deficiency disease? Q12b. At what age (in years) did these repeated, serious or unusual infections begin?

15

Figure 16

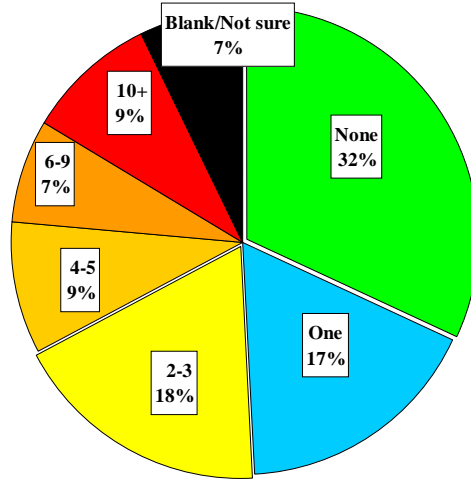
Reason Patient Initially Tested for PID



Q13. Why was the patient initially tested for immune deficiency disease? (Base: N=1,526) 16

Figure 17

Times Hospitalized before Diagnosis Made

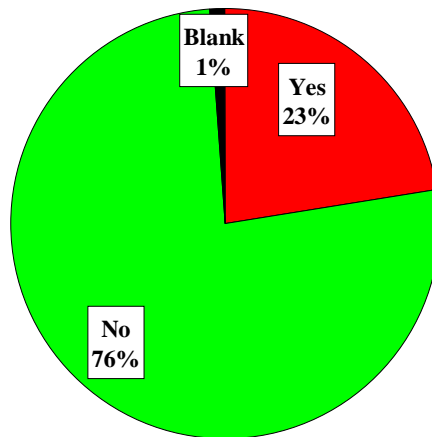


Q15. How many times had he/she been hospitalized BEFORE DIAGNOSIS as immune deficient? (Base: N=1,526)

17

Figure 18

Initial Diagnosis Made During Hospitalization

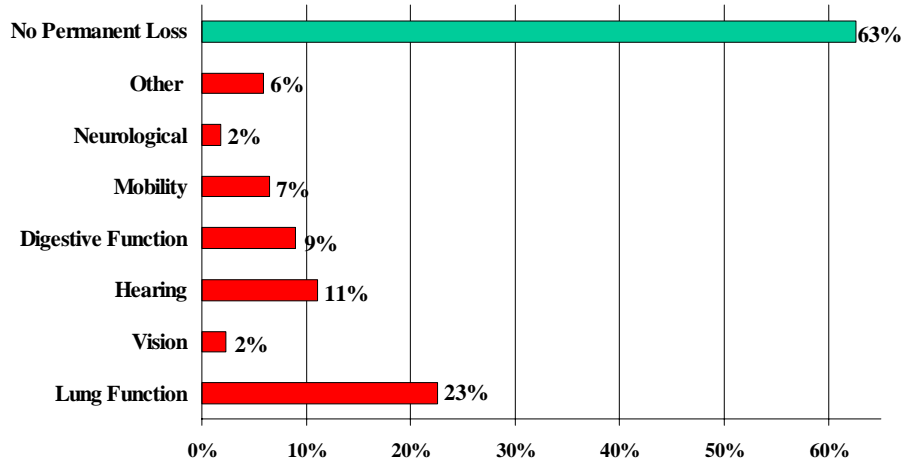


Q14. Was the diagnosis as immune deficiency disease first made during a hospitalization? (Base: N=1,526)

18

Figure 19

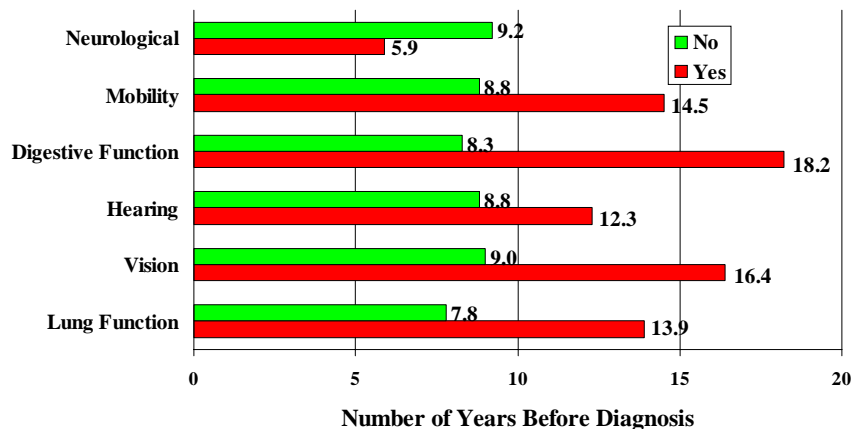
Type of Permanent Functional Impairment Prior to Diagnosis



Q16. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent loss of? (Base: N=1,526) 19

Figure 20

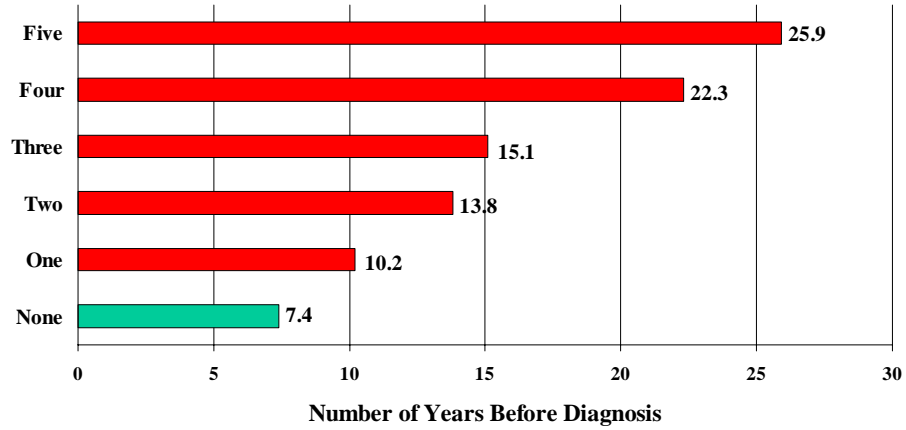
Whether Permanent Functional Impairment Prior to Diagnosis by Time to Diagnosis



Q16. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent loss of (Base: N=1,398 – excludes cases with missing information) 20

Figure 21

Number of Permanent Functional Impairments by Time to Diagnosis

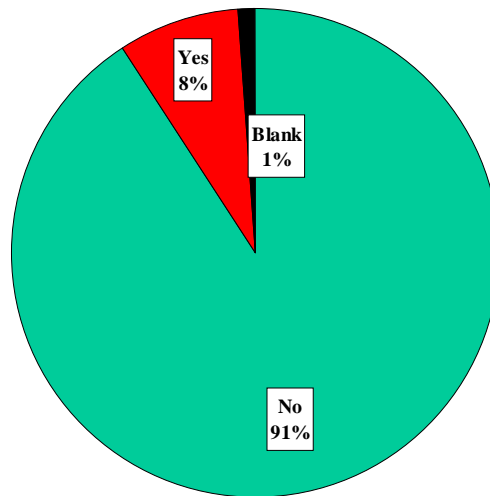


Q16. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent loss of (Base: N=1,398 – excludes cases with missing information)

21

Figure 22

Cancer or Leukemia: Lifetime

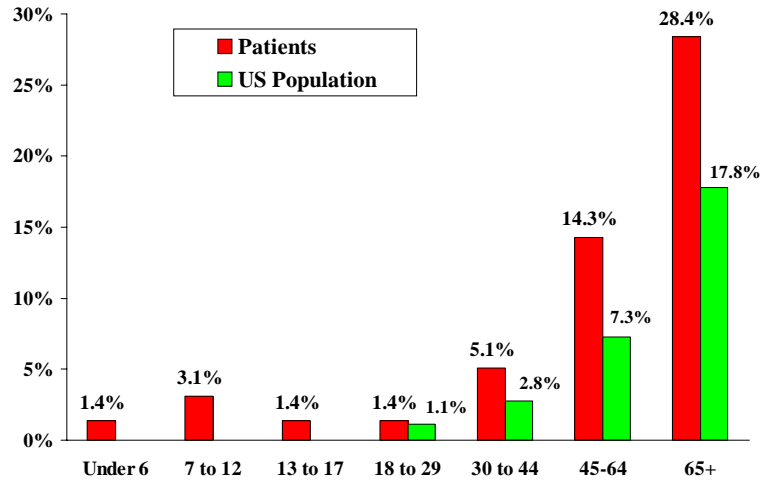


Q18. Has he/she ever had cancer or leukemia? (Base: N=1,526)

22

Figure 23

Cancer Prevalence by Age for PID Patients and US Population

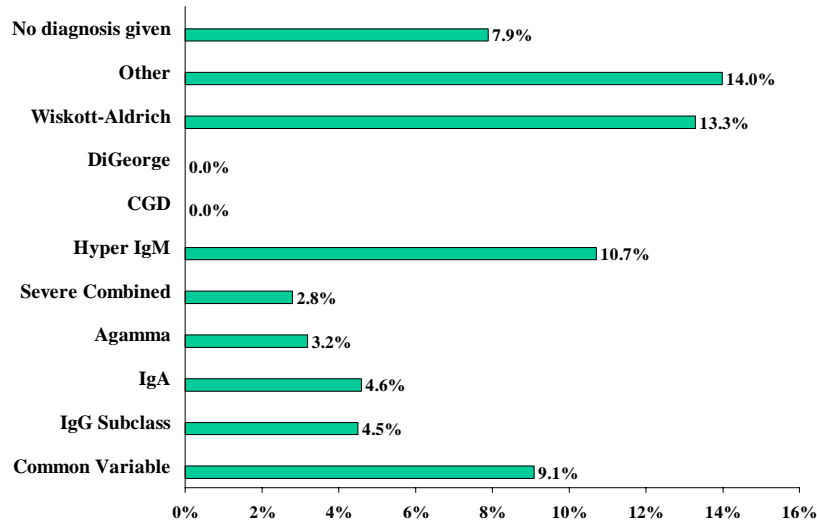


Q18. Has he/she ever had cancer or leukemia? (Base: N=1,526)
 US Population data from National Health Interview Survey

23

Figure 24

Cancer Prevalence by Diagnosis

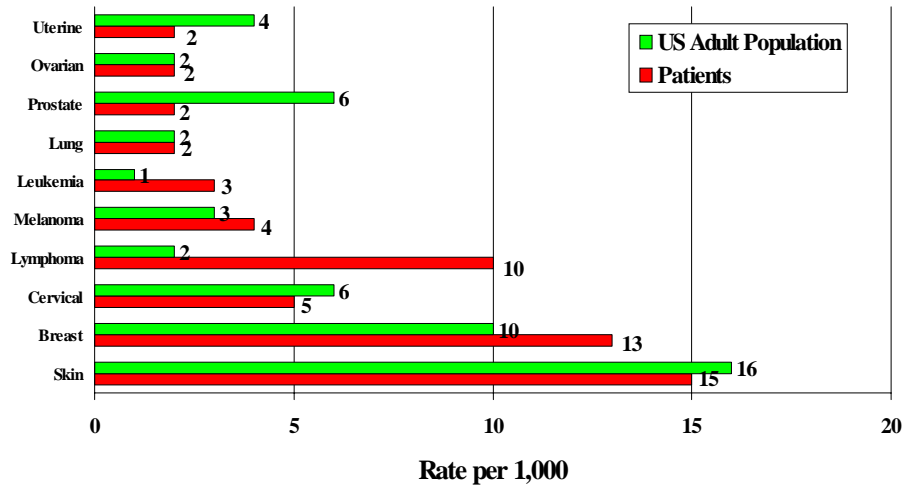


Q18. Has he/she ever had cancer or leukemia? (Base: N=1,526)

24

Figure 25

Cancer Rate per Thousand by Site

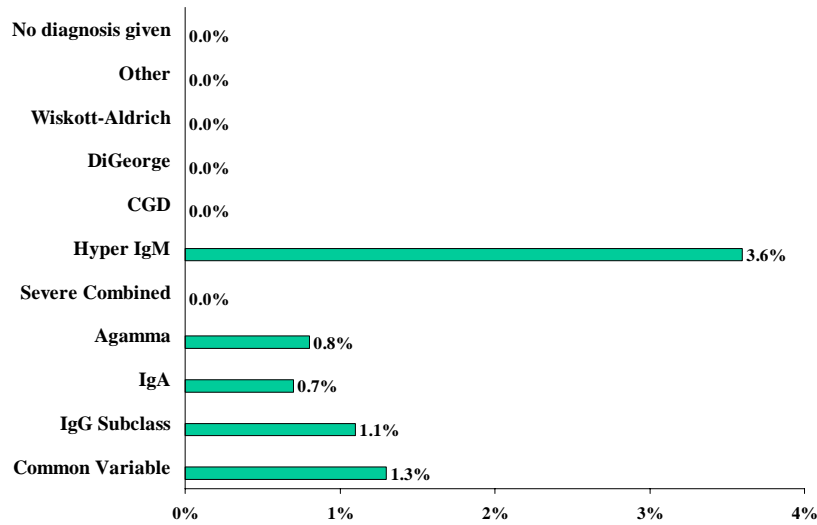


Q18. Has he/she ever had cancer or leukemia? What kind(s)? (Base: N=1,526)
US Population data from National Health Interview Survey

25

Figure 26

Lymphoma Prevalence by Diagnosis

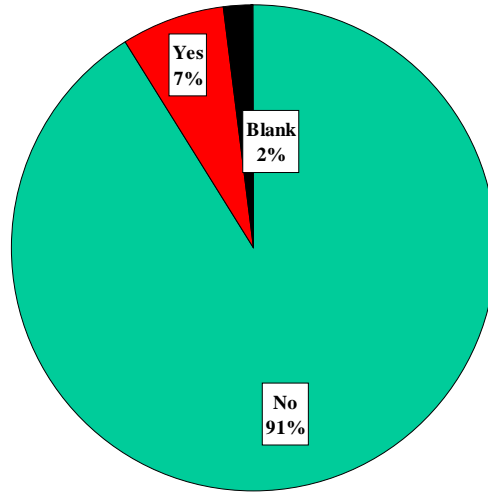


Q18. Has he/she ever had cancer or leukemia? What kind(s)? (Base: N=1,526)

26

Figure 27

Hepatitis: Lifetime

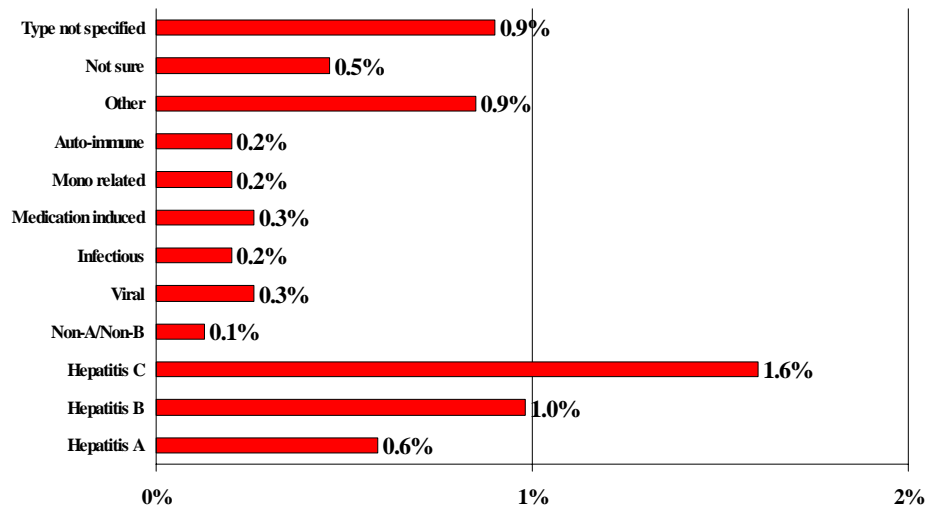


Q19. Has he/she ever had hepatitis? (Base: N=1,526)

27

Figure 28

Prevalence of Hepatitis by Type

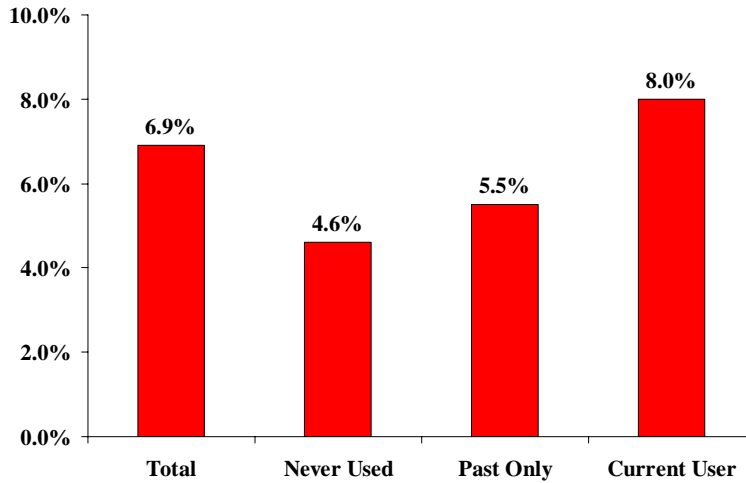


Q19. Has he/she ever had hepatitis? What type(s)? (Base: N=1,526)

28

Figure 29

Hepatitis By IVIG Use

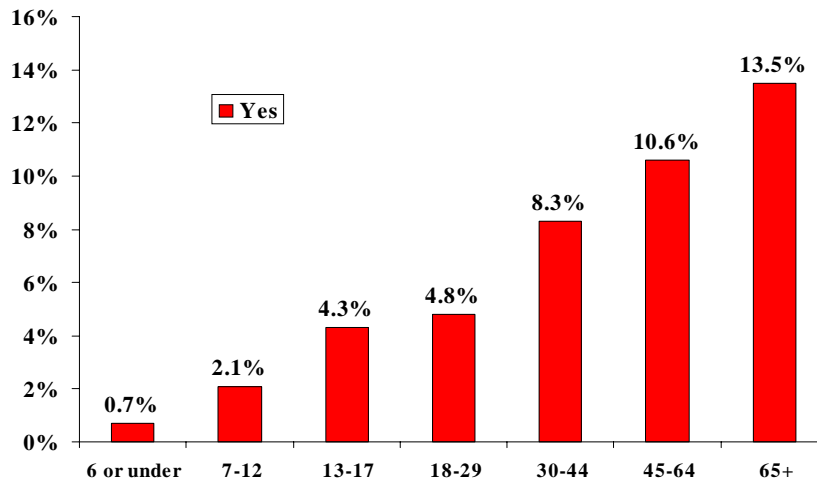


Q19. Has he/she ever had hepatitis? (Base: N=1,526)

29

Figure 30

Hepatitis By Age

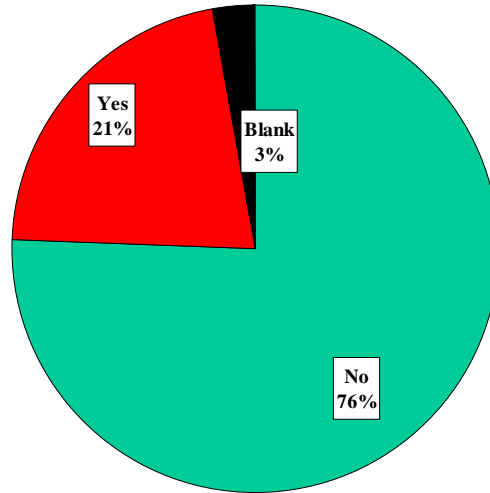


Q19. Has he/she ever had hepatitis? (Base: N=1,526)

30

Figure 31

Neurological Disorders: Lifetime

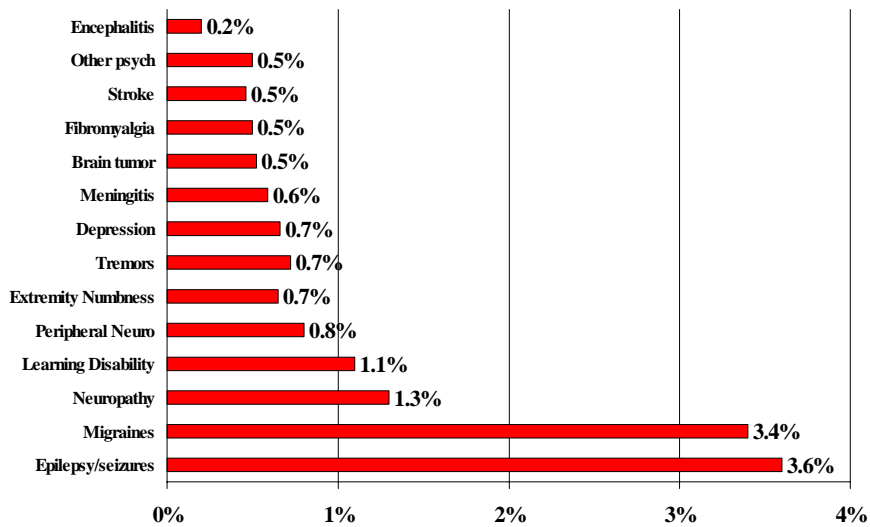


Q20. Has he/she ever had any neurological disorders? (Base: N=1,526)

31

Figure 32

Specific Rates of Neurological Disorders

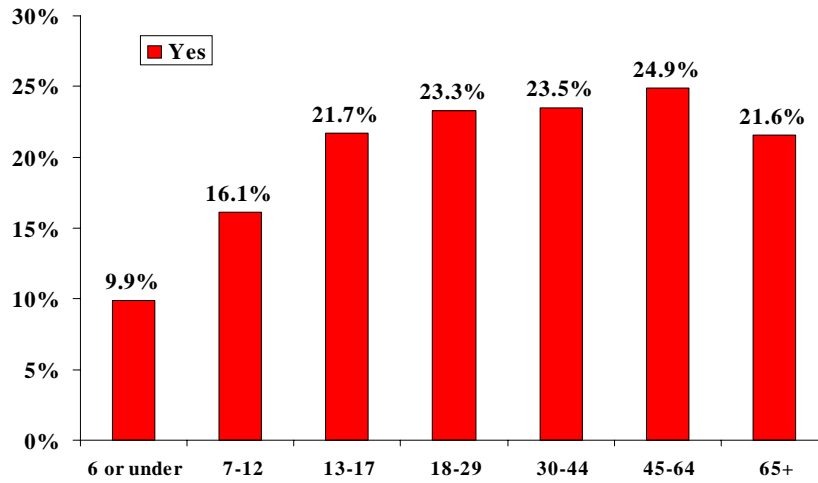


Q20. Has he/she ever had any neurological disorders? Please describe. (Base: N=1,526)

32

Figure 33

Neurological Disorders By Age

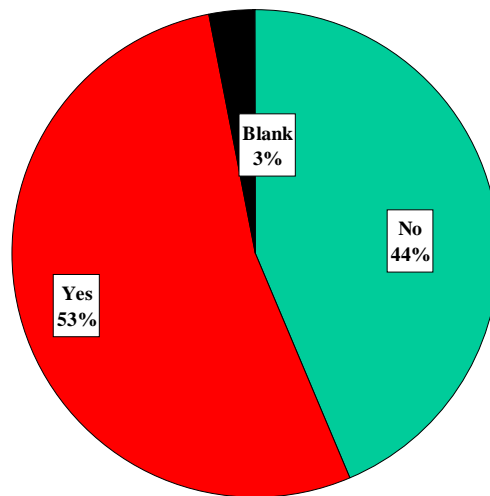


Q20. Has he/she ever had any neurological disorders? (Base: N=1,526).

33

Figure 34

Other Serious Chronic Disease

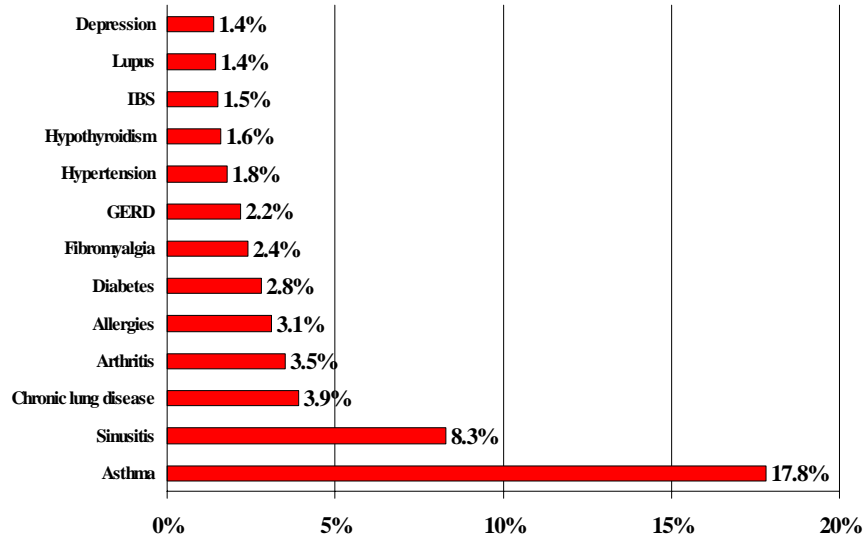


Q21. Does the PID patient suffer from any other serious, chronic disease (not counting immune deficiency)? (Base: N=1,526)

34

Figure 35

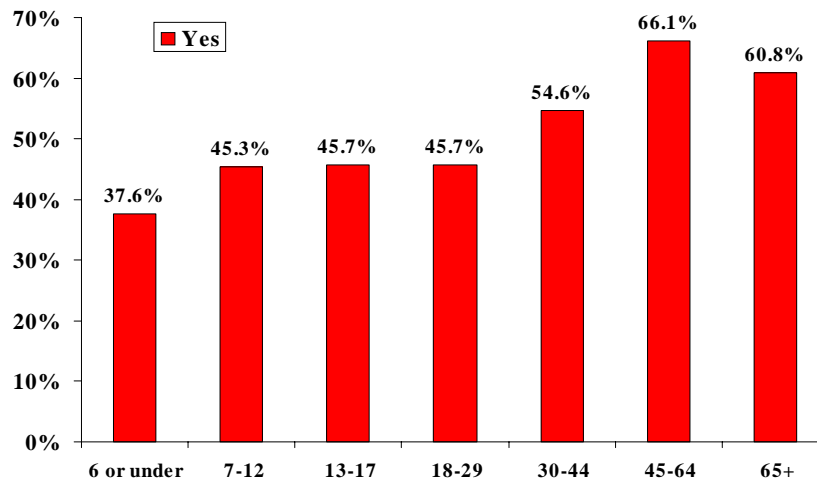
Rates of Specific Chronic Conditions



Q21. Does the PID patient suffer from any other serious, chronic disease (not counting immune deficiency)? Please describe. (Base: N=1,526) 35

Figure 36

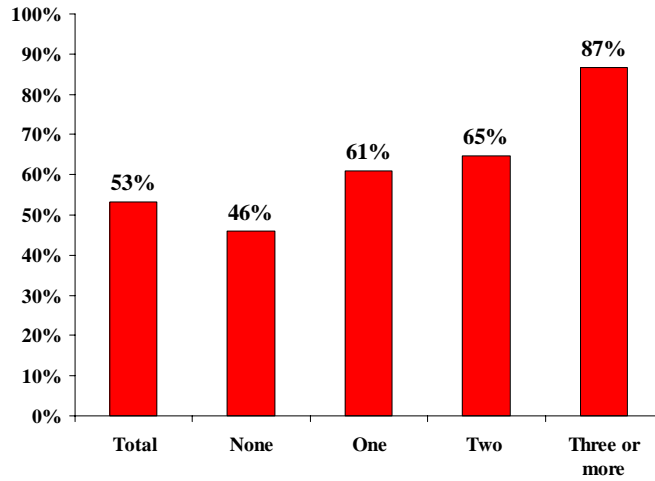
Other Chronic Condition By Age



Q21. Does the PID patient suffer from any other serious, chronic disease (not counting immune deficiency)? (Base: N=1,526) 36

Figure 37

Other Chronic Condition by Number of Impairments before Diagnosis

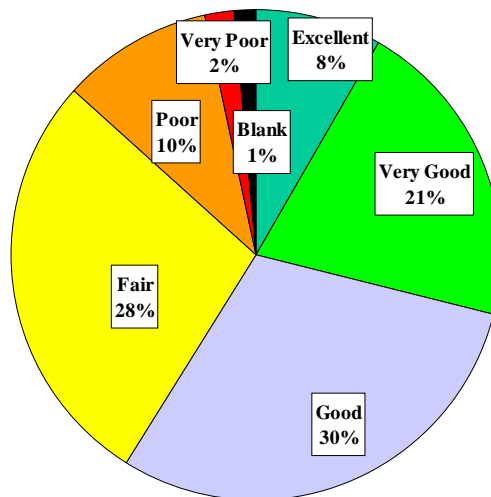


Q21. Does the PID patient suffer from any other serious, chronic disease (not counting immune deficiency)? (Base: N=1,526)

37

Figure 38

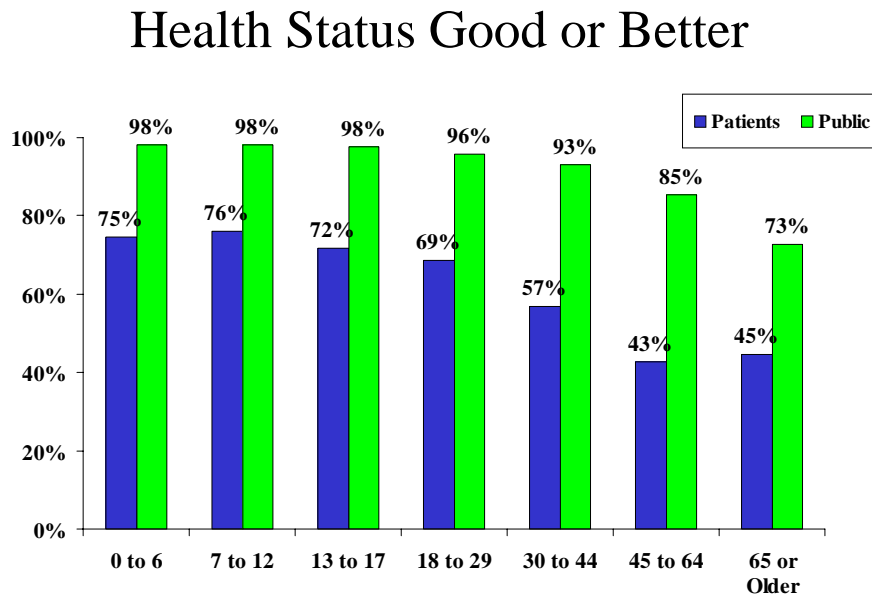
Current Health Status



Q23. Would you describe his/her current health status as? (Base: N=1,526)

38

Figure 39

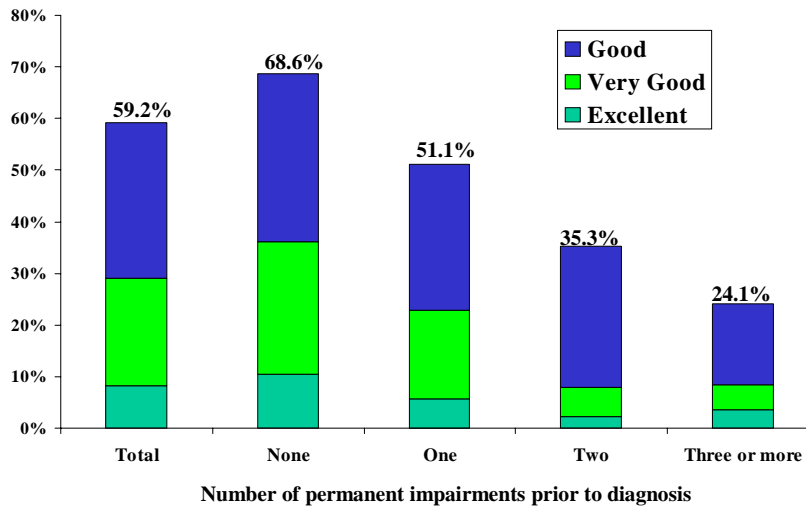


Q23. Would you describe his/her current health status as excellent, very good, good, fair, poor, or very poor?

39

Figure 40

Current Health Status by Number of Functional Impairments before Diagnosis

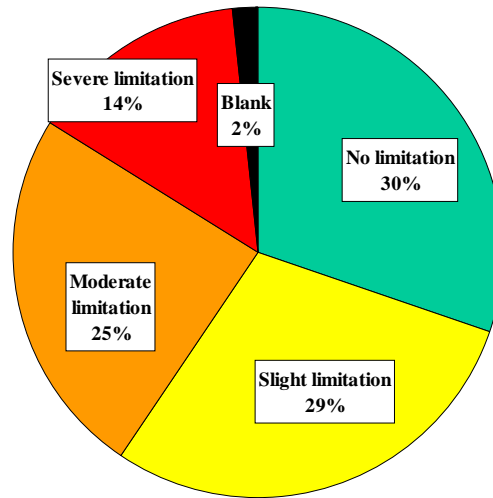


Q23. Would you describe his/her current health status as? (Base: N=1,526)

40

Figure 41

Current Activity Limitation

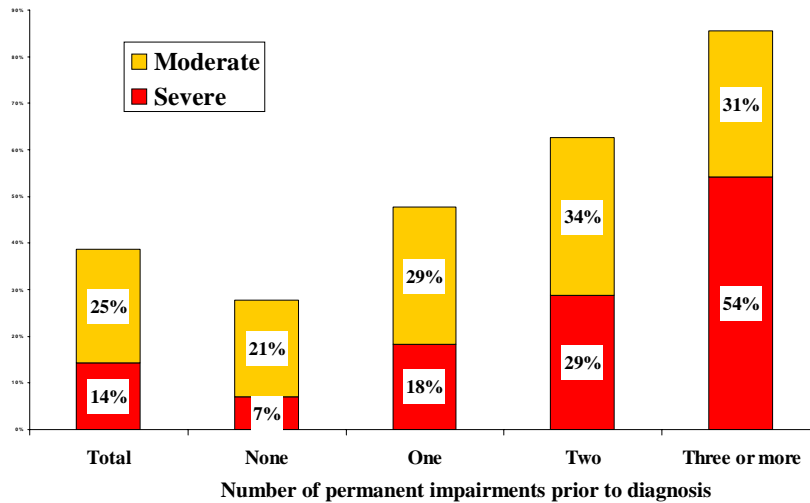


Q22. How much is he/she limited in work, play or normal physical activity as a result of his/her health? (Base: N=1,526)

41

Figure 42

Current Activity Limitation by Number of Functional Impairments before Diagnosis

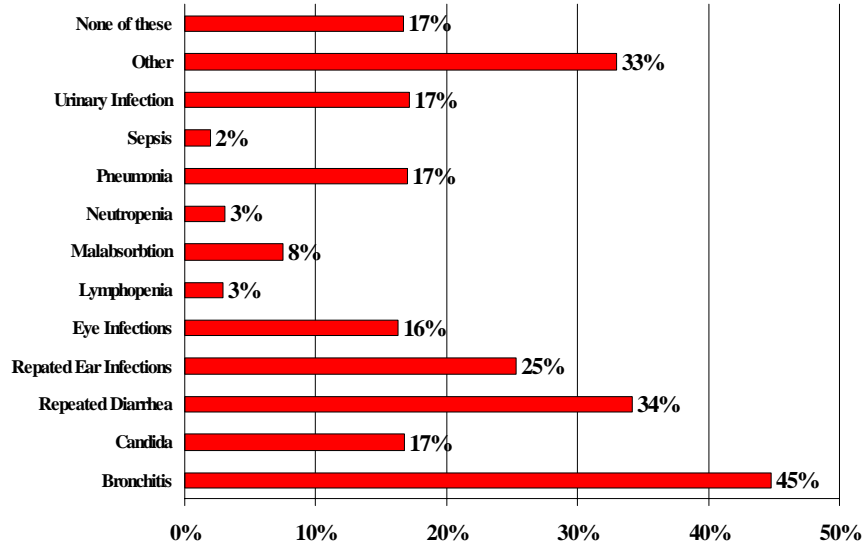


Q22. How much is he/she limited in work, play or normal physical activity as a result of his/her health? (Base: N=1,526)

42

Figure 43

Acute Conditions in Previous 12 Months

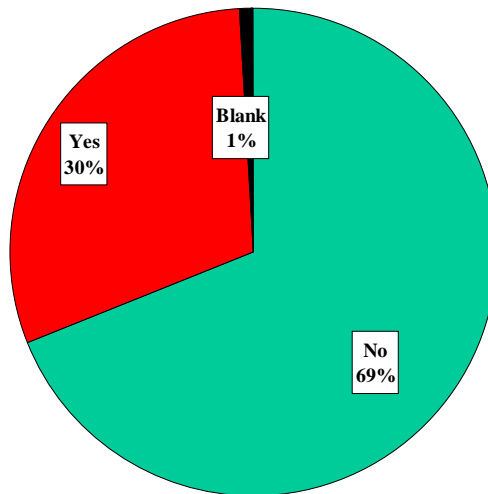


Q24. Which of the following conditions, if any, has he/she had during the past 12 months? (Base: N=1,526)

43

Figure 44

Past Year Hospitalization

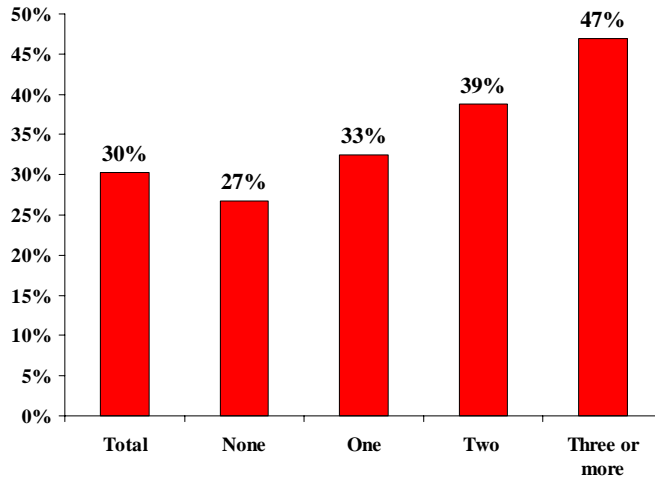


Q17. Has he/she been hospitalized overnight or longer in the past 12 months? (Base: N=1,526)

44

Figure 45

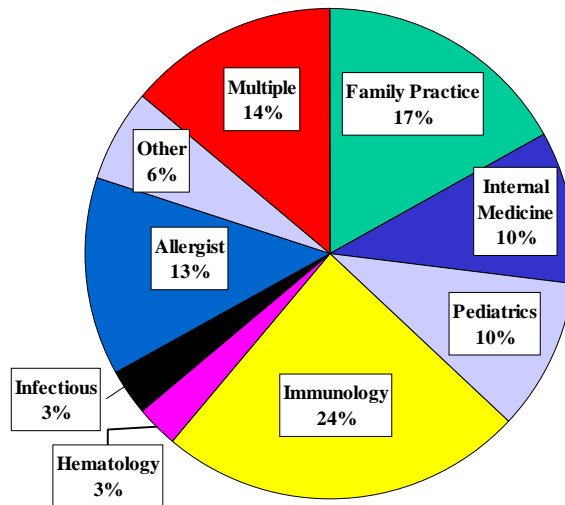
Past Year Hospitalization by Number of Impairments before Diagnosis



Q17. Has he/she been hospitalized overnight or longer in the past 12 months? (Base: N=1,526) 45

Figure 46

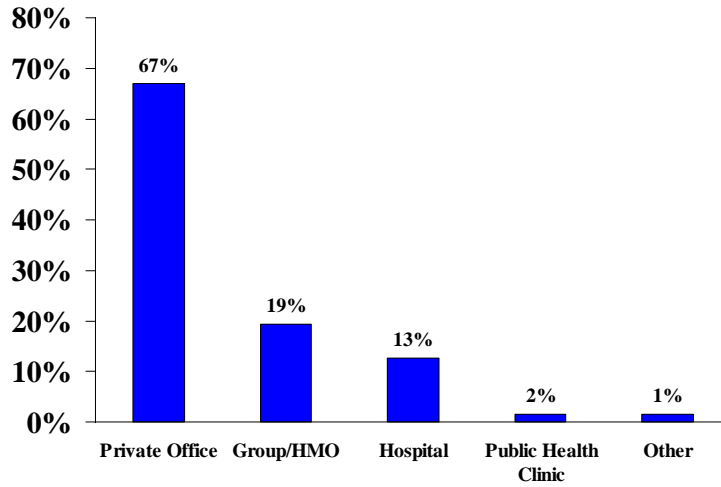
Type of Doctor Seen Most Often



Q25. What kind of doctor does the patient see most often for his/her health care? (Base: N=1, 526) 46

Figure 47

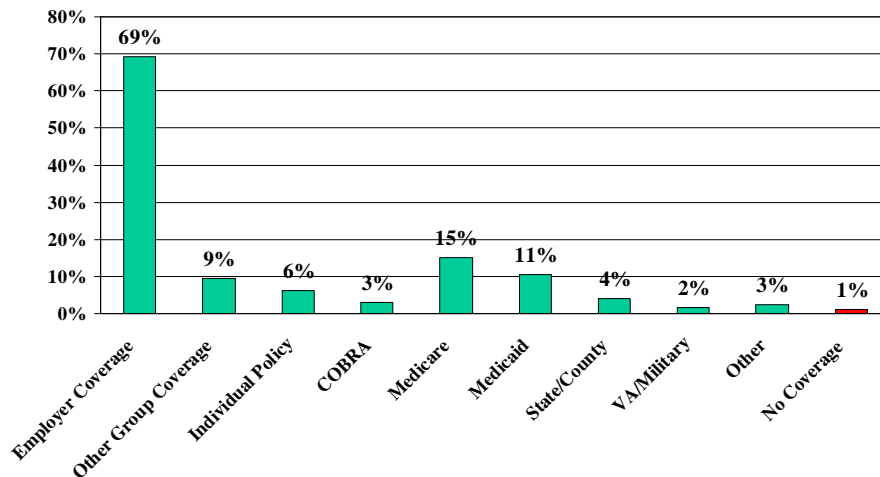
Setting of Primary Doctor Visits



Q26. Where does the patient usually visit his/her primary doctor? (Base: N=1,526) 47

Figure 48

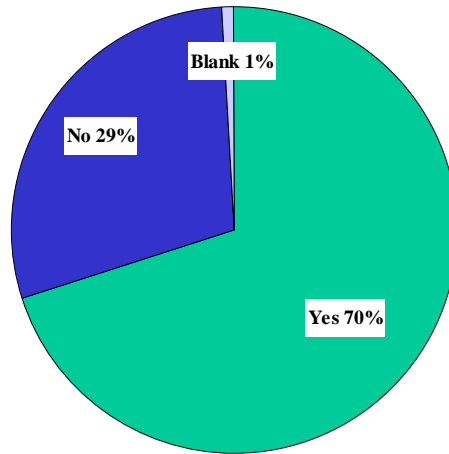
Source of Current Health Insurance



Q29. What is the current source(s) of the patient's health care coverage? (Base: N=1,526) 48

Figure 49

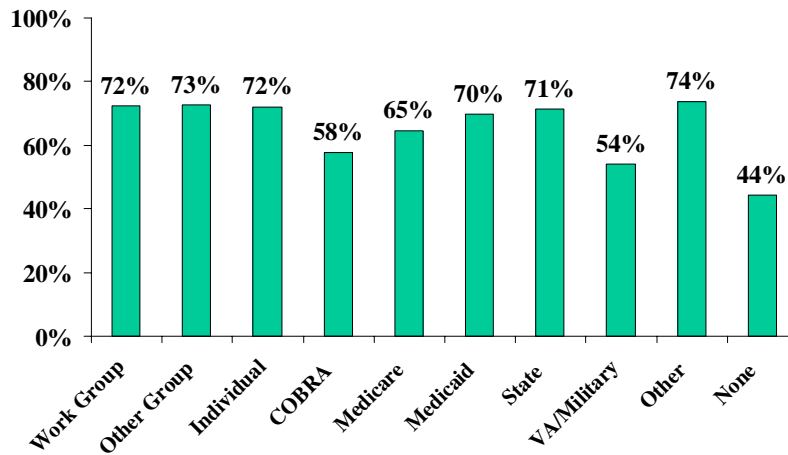
Immunologist Visit in Past Year



Q27. Has he/she been seen by an immunologist during the past 12 months? (Base: N=1,526) 49

Figure 50

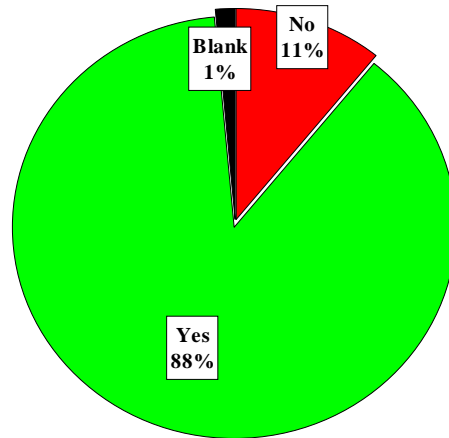
Past Year Immunologist Visit by Type of Insurance



Q27. Has he/she been seen by an immunologist during the past 12 months? (Base: N=1,526). 50

Figure 51

Able to See a Specialist as Needed

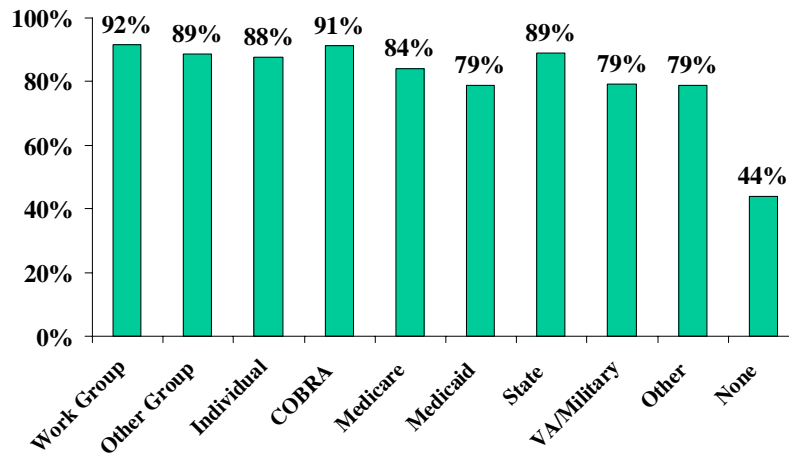


Q28. Is the patient able to see a specialist as often as needed? (Base: N=1,526)

51

Figure 52

Able to See a Specialist by Type of Insurance

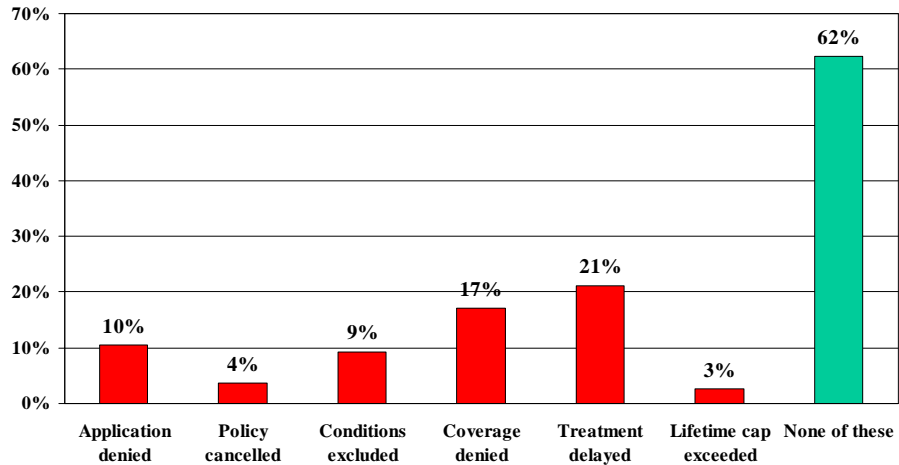


Q28. Is the patient able to see a specialist as often as needed? (Base: N=1,526)

52

Figure 53

Health Insurance Problems

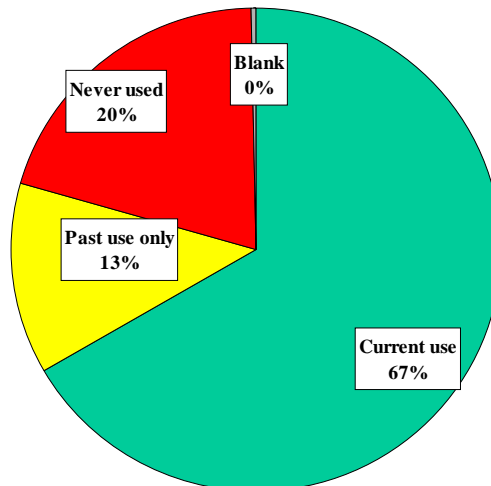


Q30. Has the patient (or his/her family) had any of the following problems with health insurance because of his/her health condition? (Base: N=1,526)

53

Figure 54

Use of IGIV Status

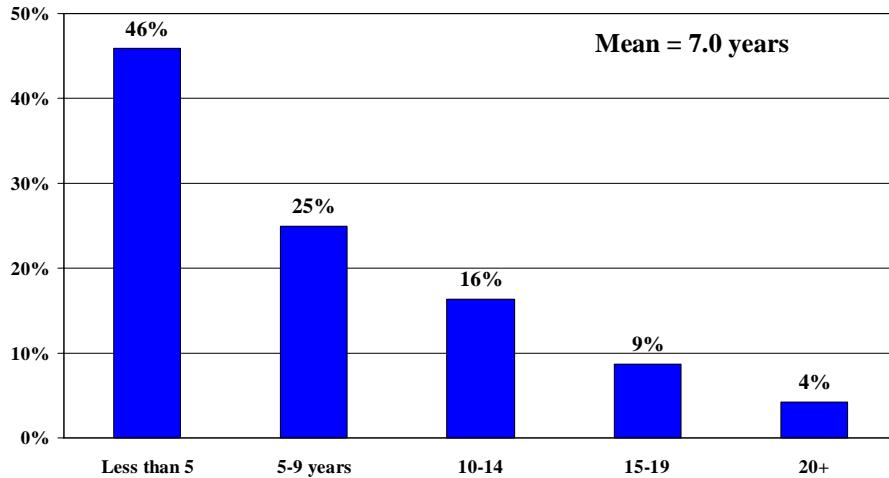


Q31. Has he/she ever been treated with intravenous gammaglobulin (IGIV) on a regular basis. Q33a. Is he/she currently being treated with intravenous gammaglobulin (IGIV) for his/her immune deficiency disease? (Base: N=1,526)

54

Figure 55

Number of Years on IGIV



Q32. How many years, in total, has he/she been treated for immune deficiency with IGIV on a regular basis? (Base: Ever users N=1,181)

55

Figure 56

Reason No Longer Using IGIV

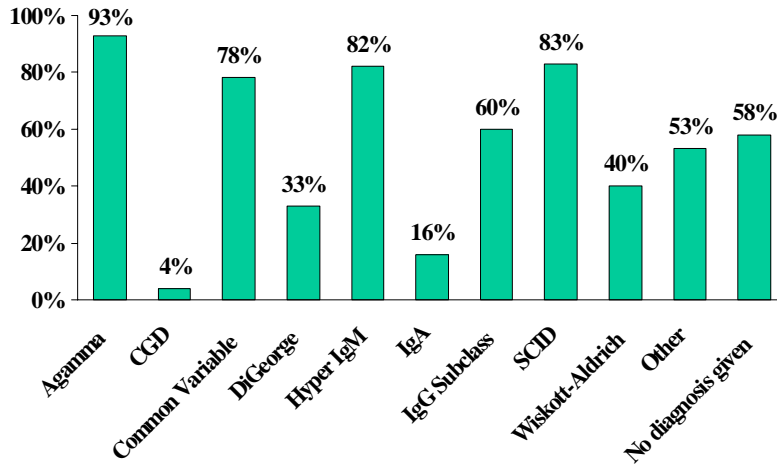
- Insurance /coverage 14.3%
- Side effects/reaction 12.4%
- Health improved/symptoms gone 12.4%
- Normal/near normal levels 11.8%
- Doctor doesn't think it is necessary 9.9%
- No real benefits 8.7%
- To see if body will produce antibodies 8.1%
- Can't afford/too expensive 5.0%
- Cured/Bone marrow transplantation 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)

56

Figure 57

Current Use of IGIV By Diagnosis

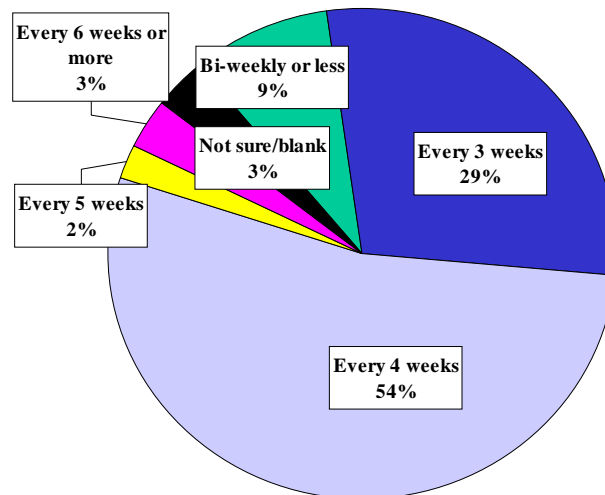


Q33a. Is he/she currently being treated with intravenous gammaglobulin (IGIV) for his/her immune deficiency disease? (Base: N=1,526)

57

Figure 58

Frequency of IGIV Infusions

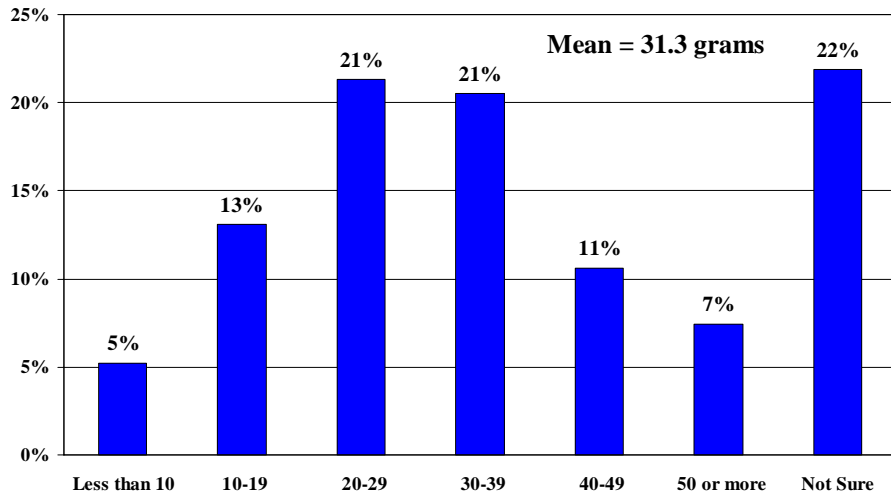


Q34. On average, how often does he/she get an infusion of IGIV? (Base: Current IGIV users N=1,015)

58

Figure 59

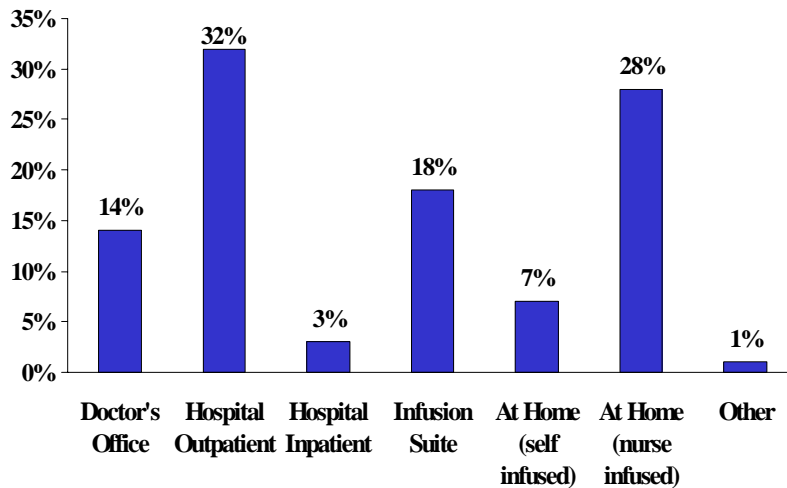
Grams of IGIV per Infusion



Q35. About how many grams of IGIV per infusion does he/she normally receive? (Base: 59 Current users N=1,015)

Figure 60

Usual Place of Infusion

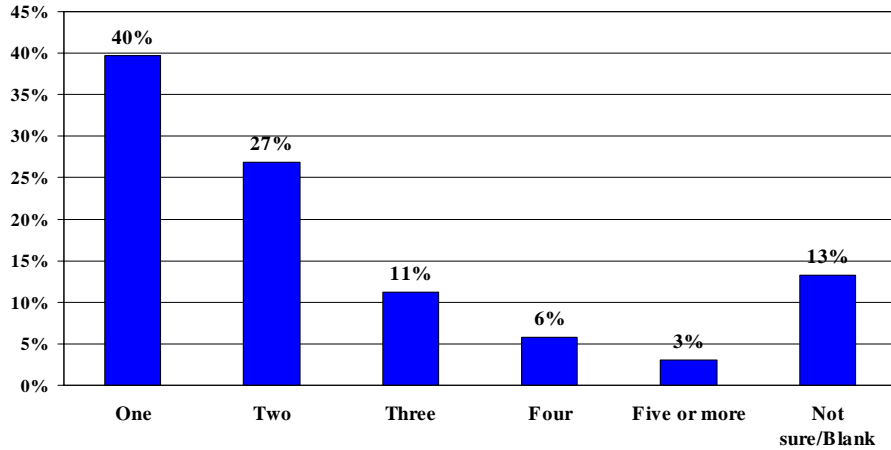


Q36. Where does the patient usually receive his/her infusions? (Base: Current users N=1,015)

60

Figure 61

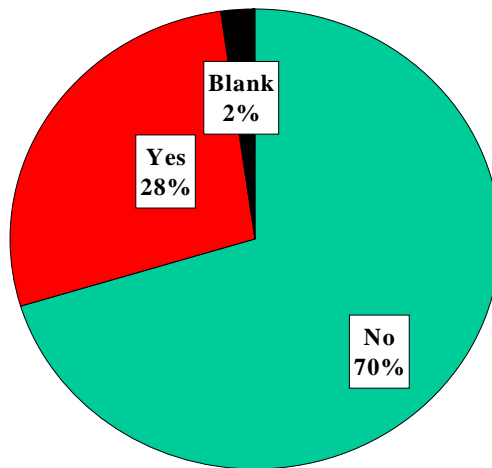
Number of IGIV Preparation Ever Used



Q37. Which of the following IGIV preparations has the patient ever used? (Base: Current users N=1,015) 61

Figure 62

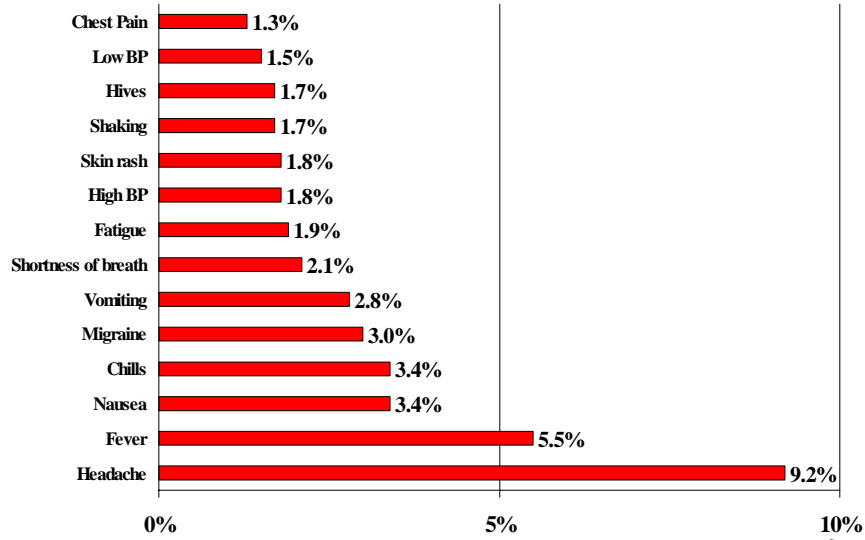
Side Effects from IGIV in Past Year



Q38a. Did he/she have any serious side effects or reactions from IGIV treatment in the past 12 months? (Base: Current users N=1,015) 62

Figure 63

Types of Side Effects from IGIV in Past Year

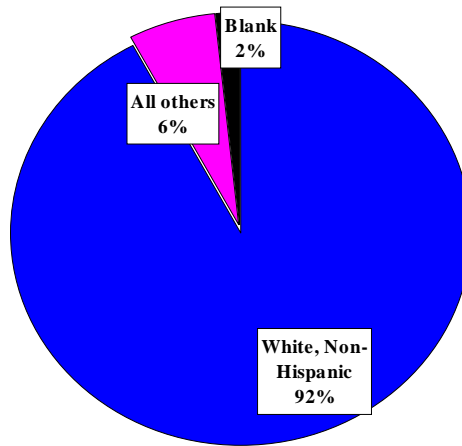


Q38a. Did he/she have any serious side effects or reactions from IGIV treatment in the past 12 months? Please describe. (Base: Current users N=1,015)

63

Figure 64

Race of PID Patients

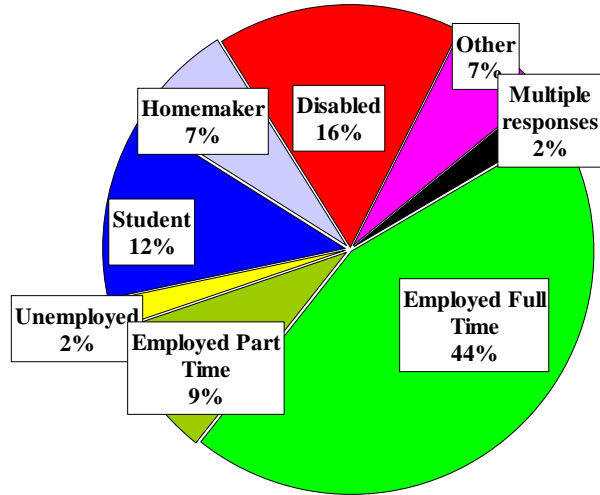


Q39. Which of the following categories would best describe the race or ethnicity of the patient? American Indian/Alaskan Native, Asian/Pacific Islander, Black/African-American, Hispanic or Latino, White/non-Hispanic, Mixed, or Other? (Base: N=1,526)

64

Figure 65

Employment Status of Patient or Caregiver

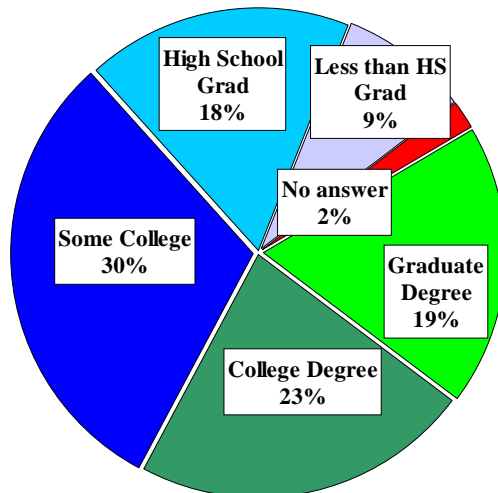


Q40. What is the current employment status of the PID patient (head of household if patient is a child)? (Base: N=1,526)

65

Figure 66

Education of Patient or Caregiver



Q41. What is the last grade or year of school completed by the PID patient (head of household if patient is a child)? (Base: N=1,526)

66