

2018 National Treatment Survey

Immune Deficiency Foundation



National Treatment Experiences and Preferences Survey

- To date, no federally funded health surveys in the United States (i.e., the National Health Interview Survey and the National Health and Nutrition Examination Survey) collect information on primary immune deficiency diseases nor do these surveys collect data on the use of Ig therapy as a lifelong treatment of PI.
- As a result, the Immune Deficiency Foundation (IDF) has sought to fill this void by administering a national treatment experience and preference survey within the PI community. Beginning in 1995, IDF has administered four National Treatment Surveys (1996, 2003, 2008, 2013) exploring the state of immunoglobulin replacement therapy for primary immunodeficiency diseases in the United States.
- In addition to administering the fifth National Treatment Survey in 2018, IDF also asked participants to complete, where appropriate the Ig Patient Experience with Treatment (IgPET) Scale, the Treatment Satisfaction Questionnaire for Medication (TSQM-9), SF-12 and SF-10.
- The data gathered from the National Treatment Experiences and Preferences Survey provides clinicians, researchers and most importantly, patients and their families with the only national estimates of treatment experiences of patients with primary immunodeficiency diseases.

Ig Patient Experience with Treatment (IgPET) Scale

- In addition to administering the fifth National Treatment Survey, IDF also asked participants to complete, where appropriate the Ig Patient Experience with Treatment (IgPET) Scale, the Treatment Satisfaction Questionnaire for Medication (TSQM-9), SF-12 and SF-10.
- The IgPET Scale assesses Ig treatment experiences in persons with PI across three domains: **Convenience, Control, and Impacts and Interference**.
 - The **Convenience** domain covers treatment frequency, scheduling, planning, time to obtain treatment and needle sticks (six items).
 - The **Control** domain covers treatment experiences that persons with PI cannot control (four items).
 - The **Impacts and Interference** domain covers side effects, interference with daily activities, impact on travel, and worry about treatment (six items).
- In total, the IgPET Scale is comprised of 19 questions.

Treatment Satisfaction Questionnaire for Medication (TSQM-9)

- The TSQM-9 is a nine item questionnaire developed to measure patients' satisfaction with their medication and includes three scale scores: Effectiveness, Convenience and Global Satisfaction.
 - The three **Effectiveness** items measure patient's satisfaction with their medication's ability to treat their condition, relieve symptoms and their satisfaction with the amount of time it takes for the medication to begin working.
 - The three **Convenience** items measure difficulty of use, ease of planning when the medication will be used and how convenient is it to take the medication as instructed.
 - The three **Global Satisfaction** items measure the patient's confidence in the benefit of taking the medication and in the belief the benefits of taking the medication outweigh any possible difficulties the patient may experience.
- The recall period is “over the last two to three weeks, or since you last used it [the medication]”.
- The TSQM-9 has been applied in numerous conditions, including hypertension, psoriasis, multiple sclerosis, depression, diabetes, cancer, rhinitis, and arthritis.

SF-12 and SF-10

- In addition to administering the fifth National Treatment Survey, IDF also asked participants to complete, where appropriate the Ig Patient Experience with Treatment (IgPET) Scale, the Treatment Satisfaction Questionnaire for Medication (TSQM-9), SF-12 and SF-10.
- Both the SF-12 and SF-10 are designed to assess functional health and well-being.
 - The **SF-12** measures physical functioning, role physical, bodily pain, general health, vitality, social functioning, role emotional, and mental health for adults age 18 years older or older.
 - The **SF-10** measures a child's (age 5 years old to 18 years old) physical and psychological health using 10 items and must be completed by a parent.
 - IDF aims to capture a snapshot of both physical and psychological well-being among persons with PI.

Methodology

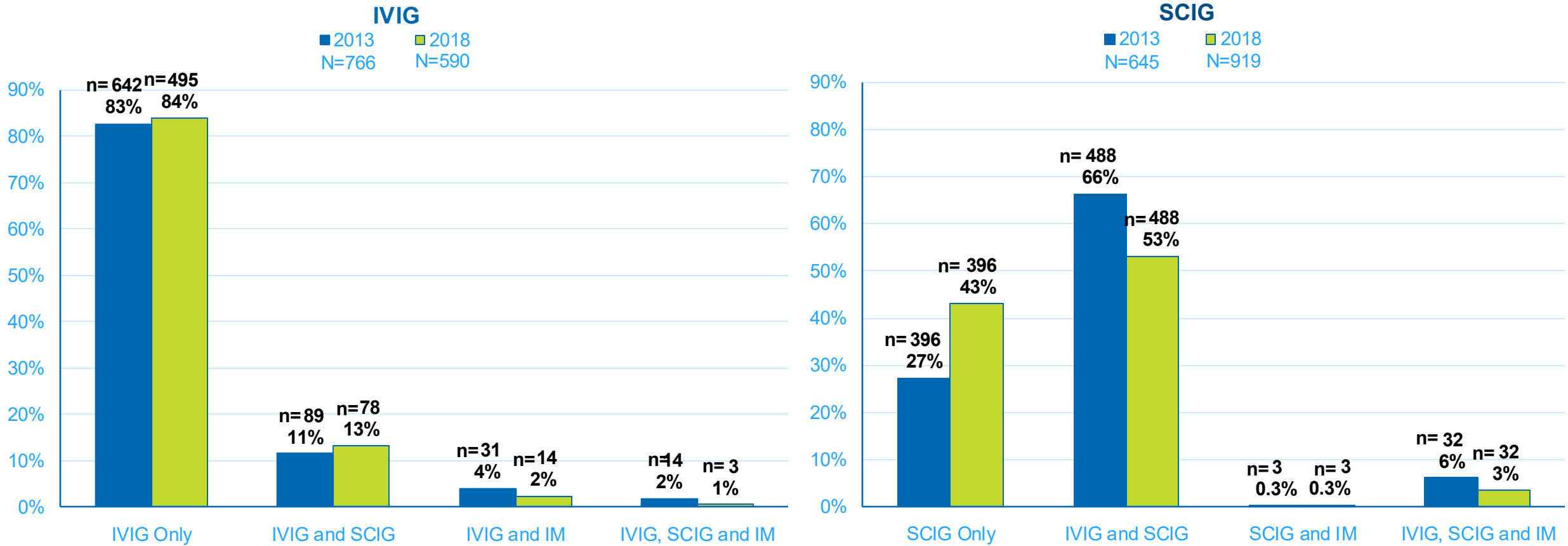
- The IDF Survey Research Team, with input from the IDF Medical Advisory Committee, developed the IDF 2018 National Treatment Survey.
- The 2018 National Treatment Survey contained up to 94 questions, depending on the respondent's experiences.
 - Including the Ig PET, TSQM-9 and SF-12, Adults with PI answered up to a total of 134 questions
 - Including the SF-10, Parents of children with PI answered up to a total of 104 questions
- The study protocols were reviewed by Advarra and received an exemption from IRB review and approval.
- 4,500 pre-notification postcards were mailed prior to the survey launch.
- In field: October 1, 2018 –February 1, 2019
 - 4,500 surveys mailed
 - 101 undeliverable surveys
 - 1,596 surveys completed
 - Completed surveys were entered into a raffle for one of three Amazon.com eGiftCards: \$100, \$200, and \$700. Three households were selected (one per gift card).
- This is a non-probability sample of individuals with PI and all data obtained represents members of the IDF member database.

Topics

- Treatment
 - Ever
 - Current
 - SCIG
 - IVIG
- Comorbidities
 - Permanent Impairments
 - Other Chronic Conditions
 - Infections
- General Health and Healthcare
 - SF-12 and SF-10
 - General Health
 - Physical Limitations
 - Treating Physician
 - Specialists
 - Hospitalizations
 - Insurance
- Ig PET
- TSQM-9
- Demographics

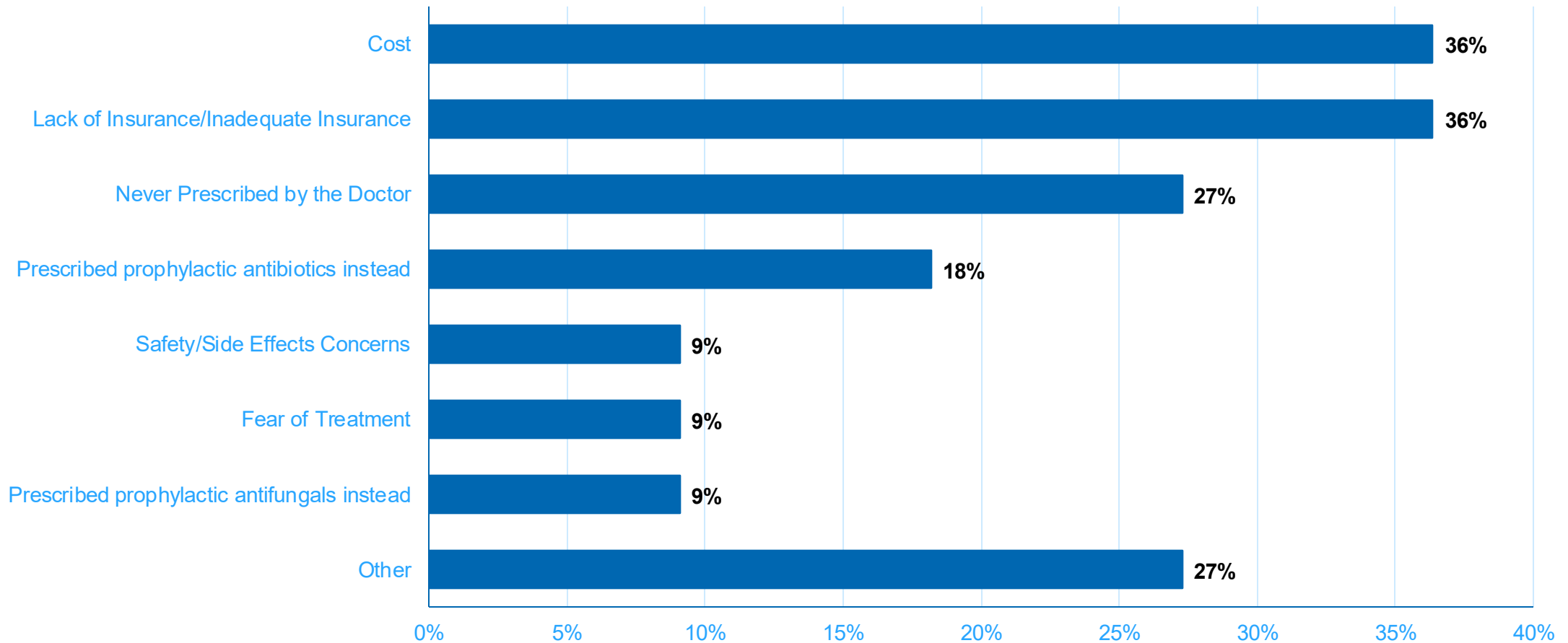
Treatment

Ig Therapy: Has the Patient EVER Used... IVIG vs. SCIG



Q18a. Has the patient EVER been treated with intravenous immunoglobulin (IVIG), subcutaneous immunoglobulin (SCIG) therapy or intramuscular (IM) immunoglobulin therapy on a regular basis? SELECT ALL THAT APPLY
 Base: Individuals who currently use IVIG or SCIG

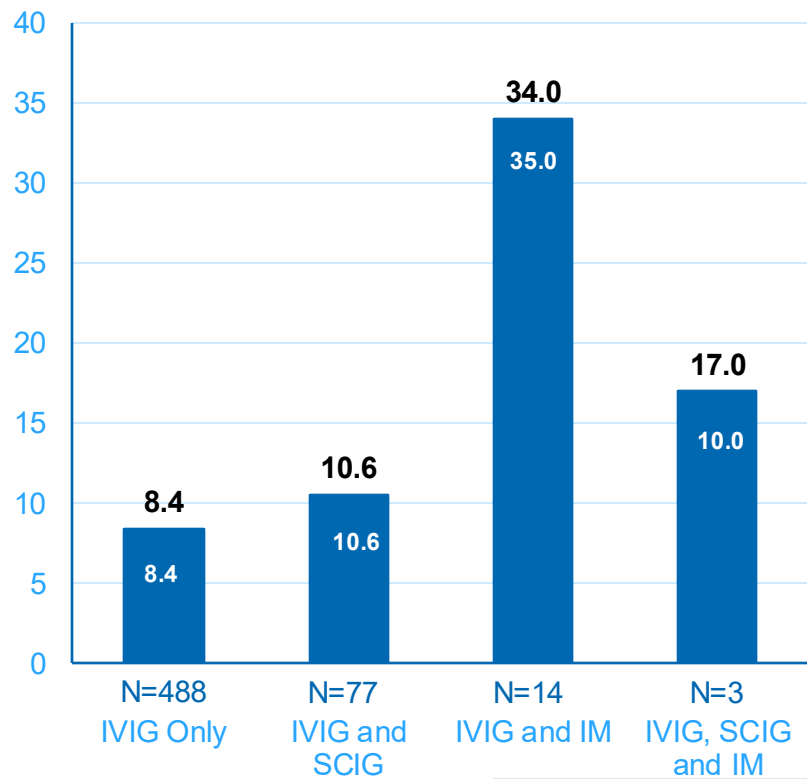
Ig Therapy: Never Treated



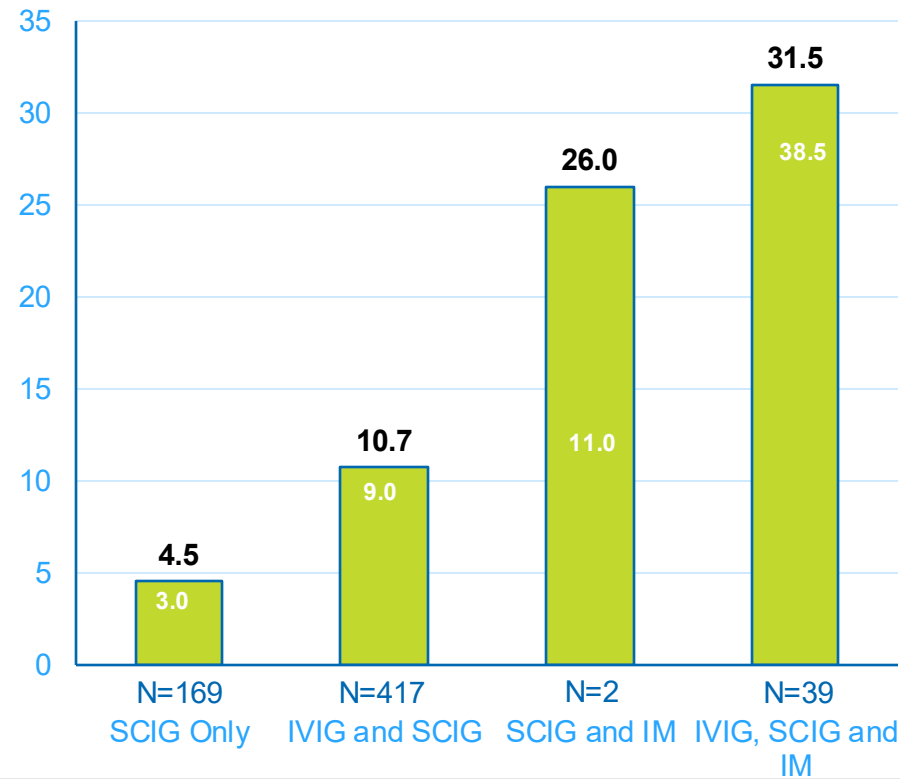
Q18b. Is there any reason why the patient has never been treated with immunoglobulin replacement therapy? N=11
Base: Individuals who were never treated with Ig Therapy

Average Years on Ig Therapy by All Ig Therapies Ever Used

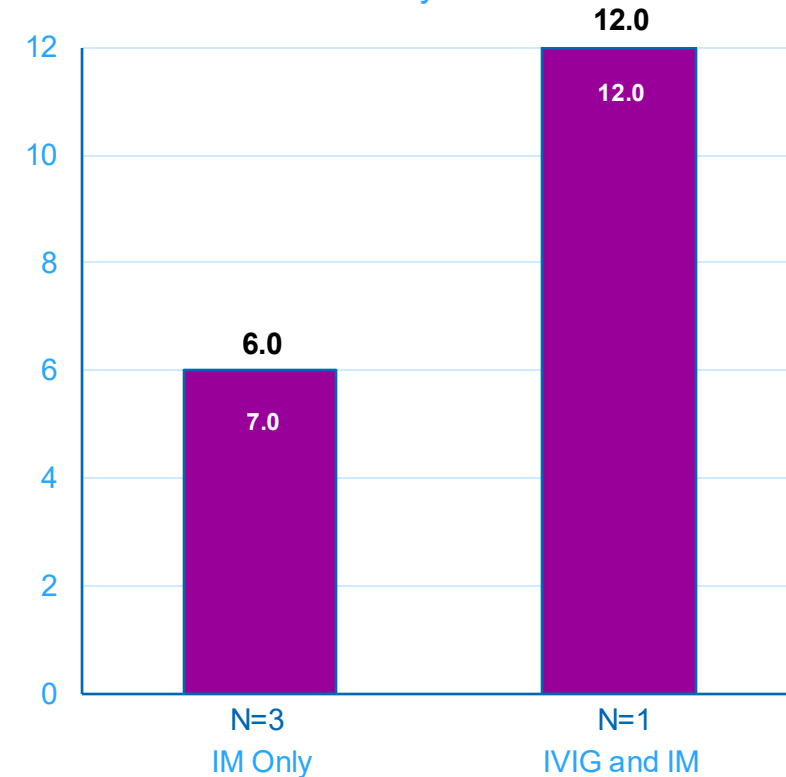
Currently on IVIG



Currently on SCIG



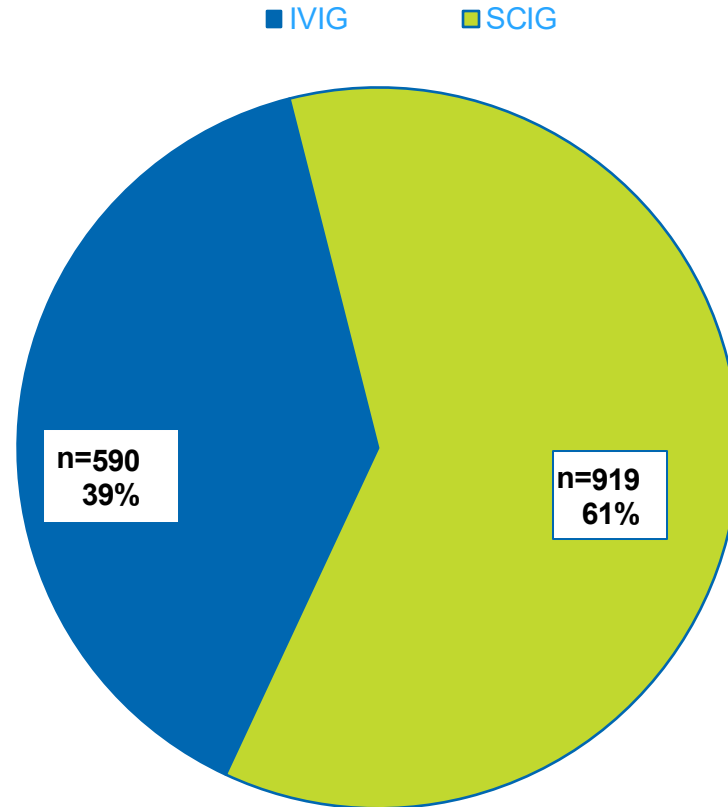
Currently on IM



Q18a. Has the patient EVER been treated with intravenous immunoglobulin (IVIG), subcutaneous immunoglobulin (SCIG) therapy or intramuscular (IM) immunoglobulin therapy on a regular basis? SELECT ALL THAT APPLY
 Q19. How many years, in total, has he/she been treated for immunodeficiency (Ig therapy) on a regular basis?
 Base: Individuals who currently use IVIG, SCIG or IM

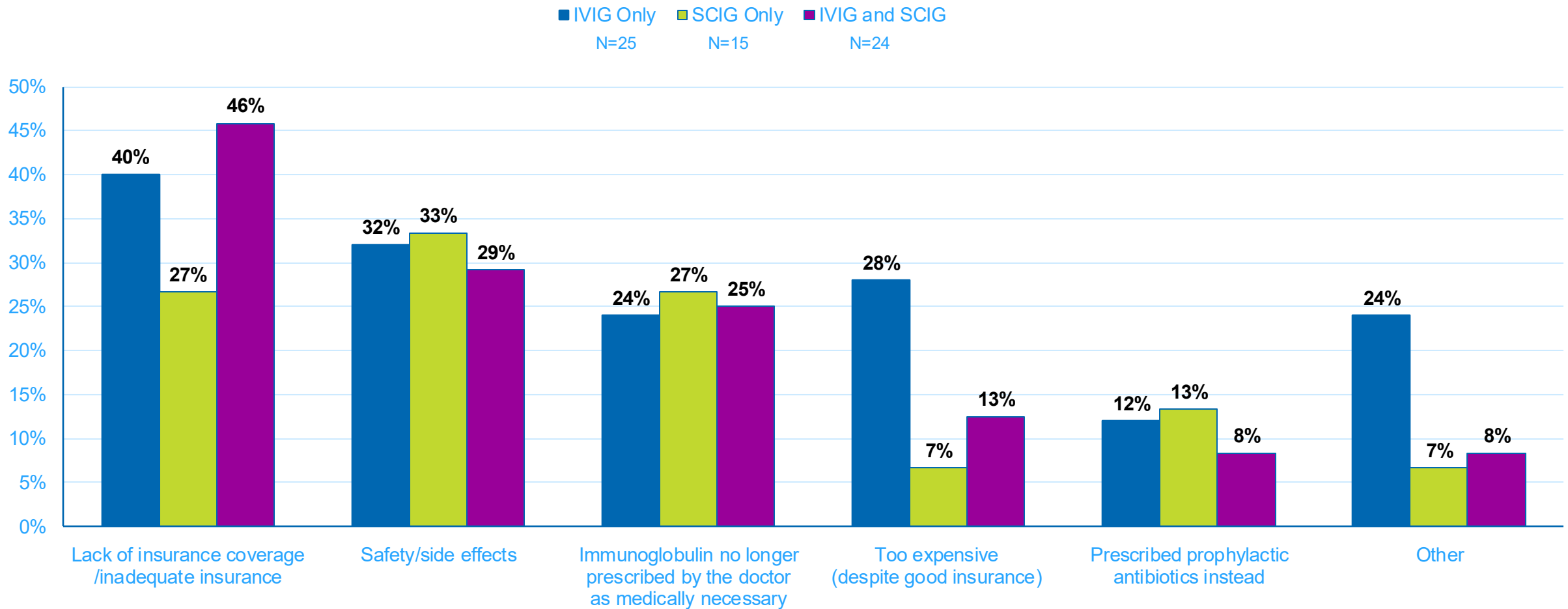
Average Median

Type of Ig Therapy Currently Used



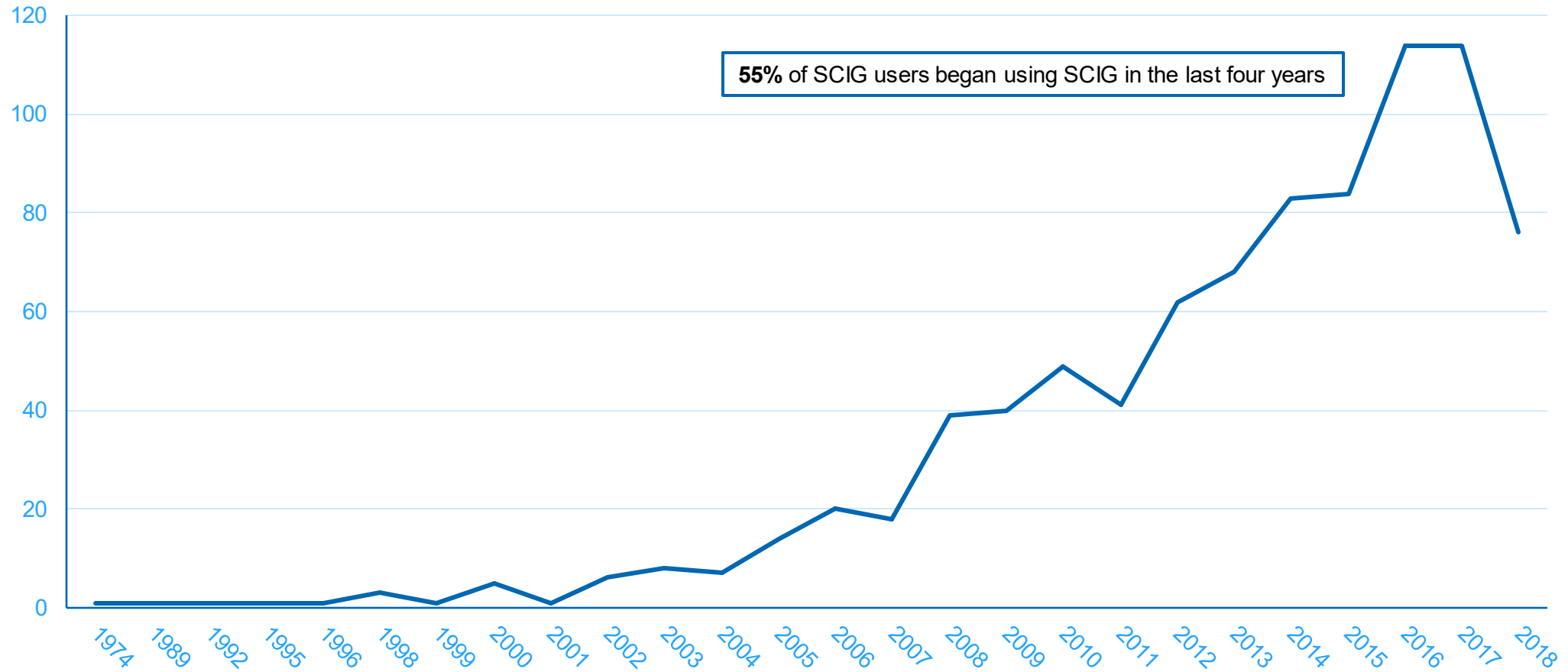
Q20a. Is he/she currently being treated with SCIG, IVIG or IM for his/her immunodeficiency disease? N=1,509
Base: Individuals who currently use IVIG or SCIG

No Longer Using Ig Therapy



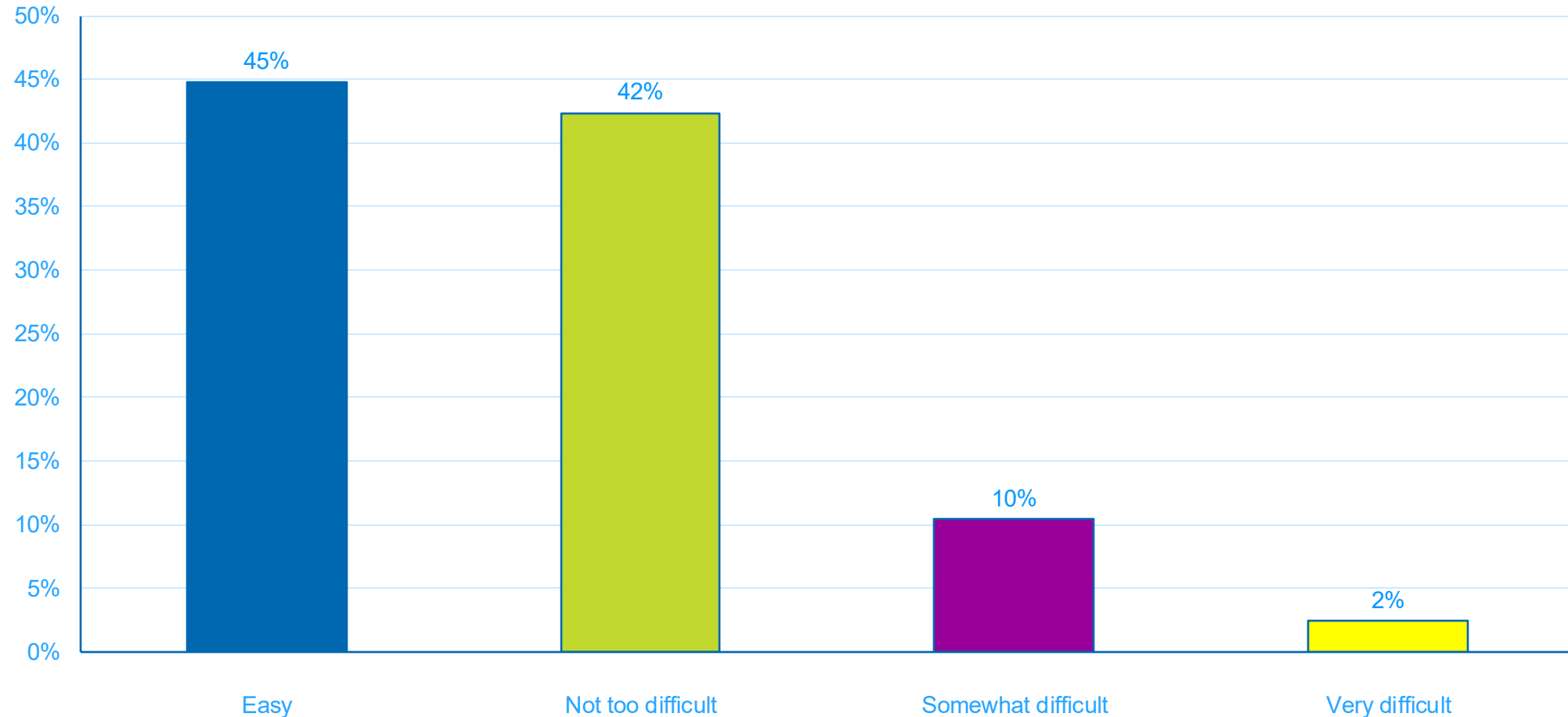
Q20b. Why is the patient no longer being treated with immunoglobulin? SELECT ALL THAT APPLY
Base: Individuals who stopped using Ig therapy

Year Started Using SCIG Therapy



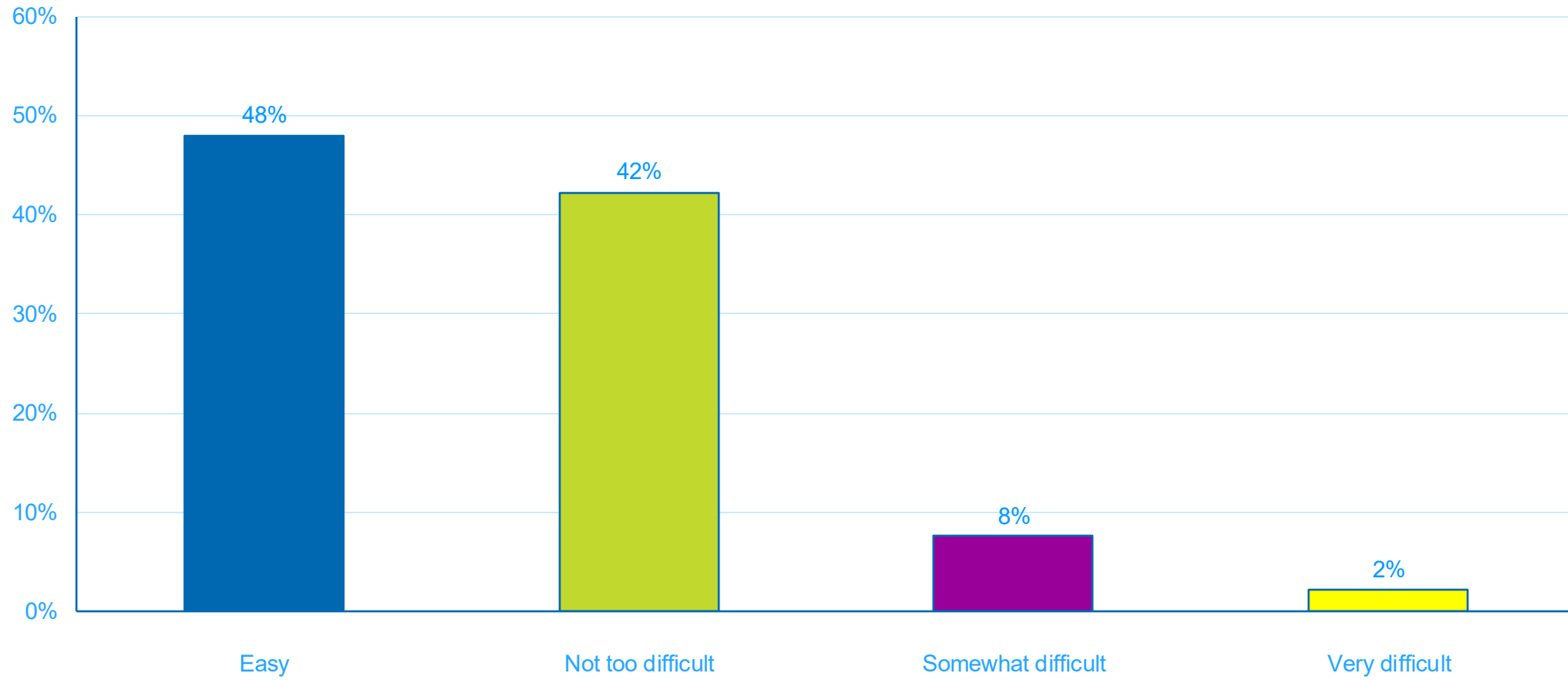
Q21. What year did you start SCIG? N=858
Base: Individuals who currently use SCIG

Difficulty Learning to Administer SCIG



Q22a. How difficult was it learning to administer SCIG? N=871
Base: Individuals who currently use SCIG

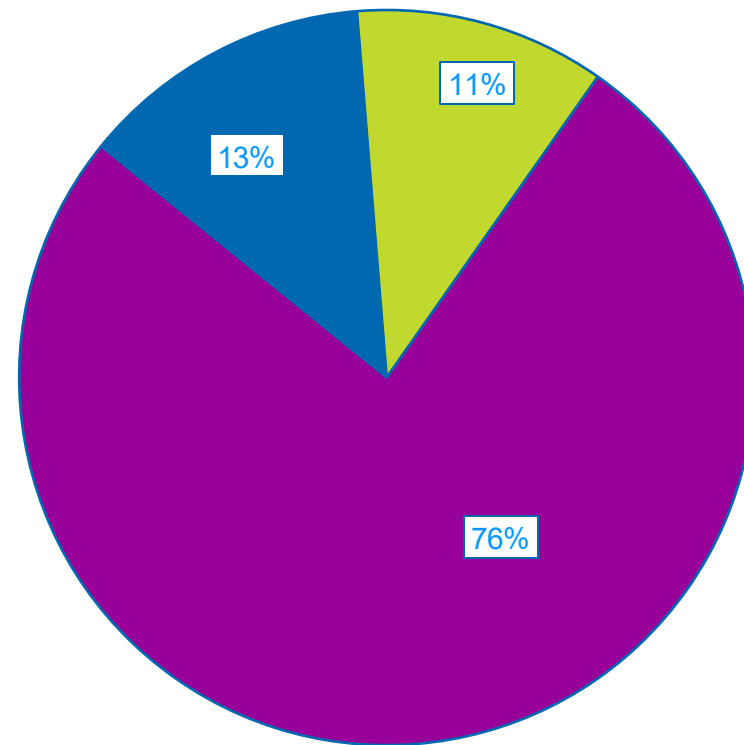
Difficulty Administering SCIG



Q22b. How difficult is it to administer SCIG? N=873
Base: Individuals who currently use SCIG

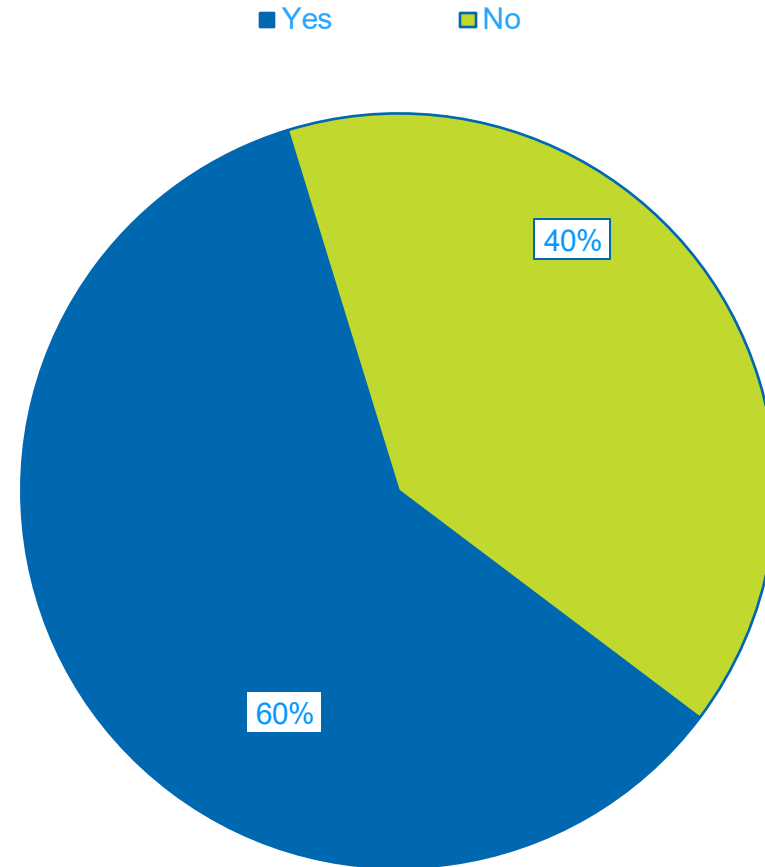
Last Time HCP Evaluated SCIG Technique

■ Less than six months ■ 6-12 months ■ 1 year or longer



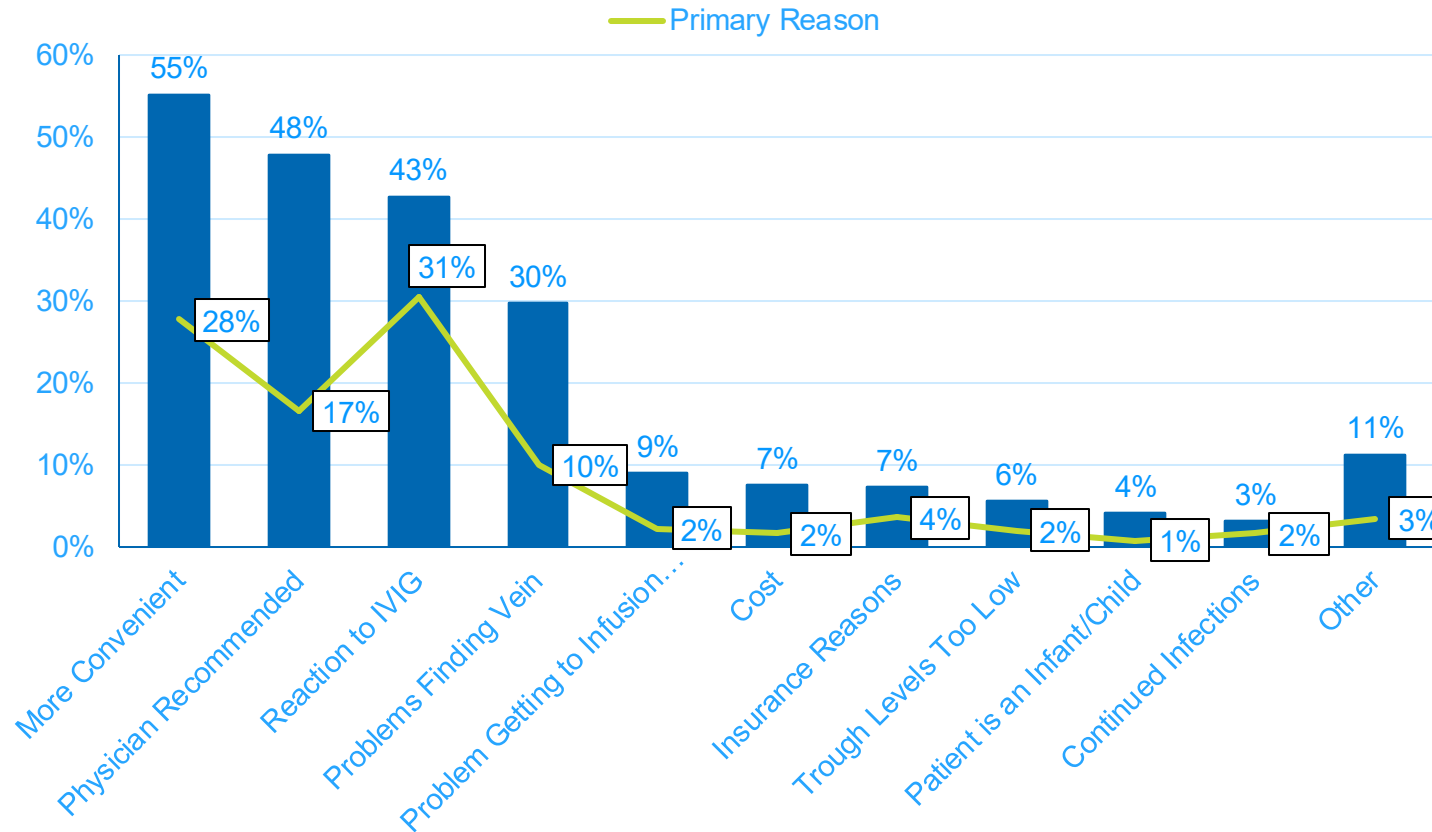
Q23. When was the last time a health professional, such as a nurse or doctor, observed and evaluated the patient's SCIG infusion technique? N=870
Base: Individuals who currently use SCIG

IVIIG Prior to SCIG



Q25. Prior to SCIG therapy did the patient receive IVIG therapy? N=887
Base: Individuals who currently use SCIG

Why Did the Patient Switch from IVIG to SCIG

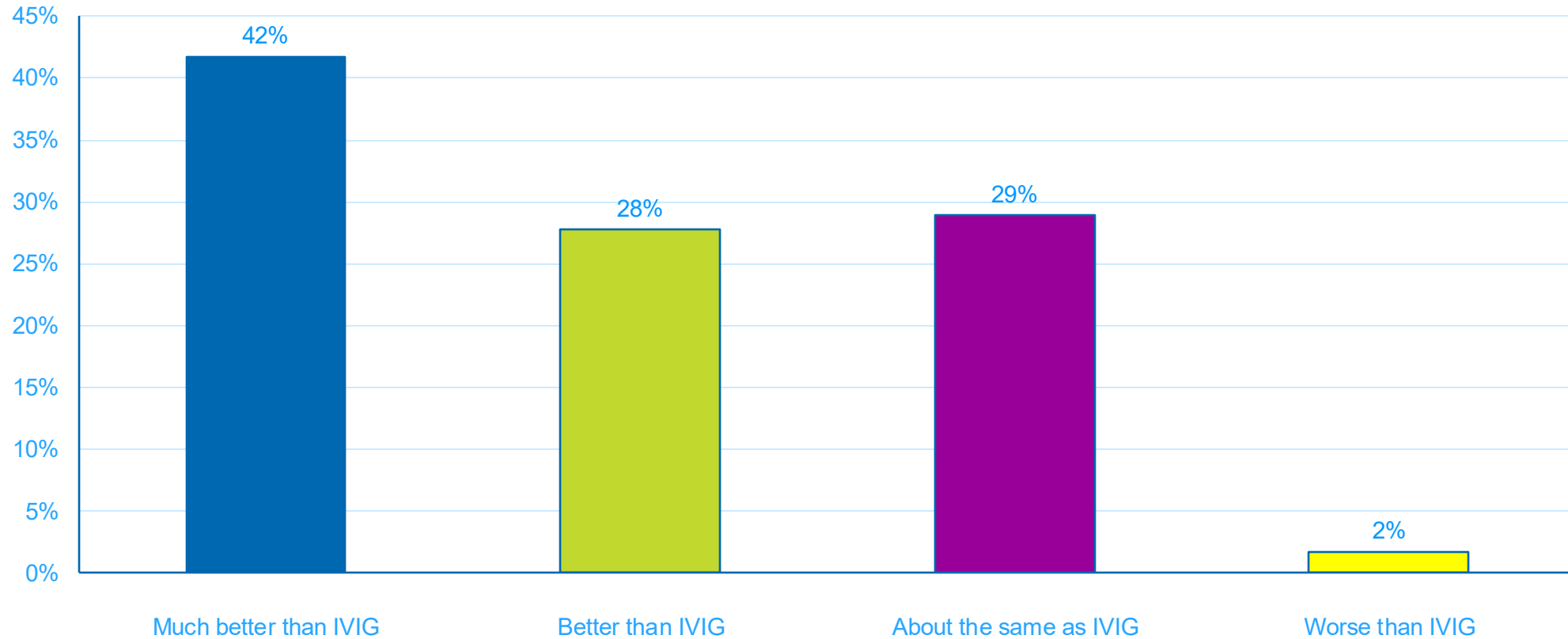


Q26a. Why did the patient switch from IVIG to SCIG? SELECT ALL THAT APPLY N=526

Q26b. Of the reasons selected above, please choose the primary reason why the patient switched from IVIG to SCIG below (select only one) N=501

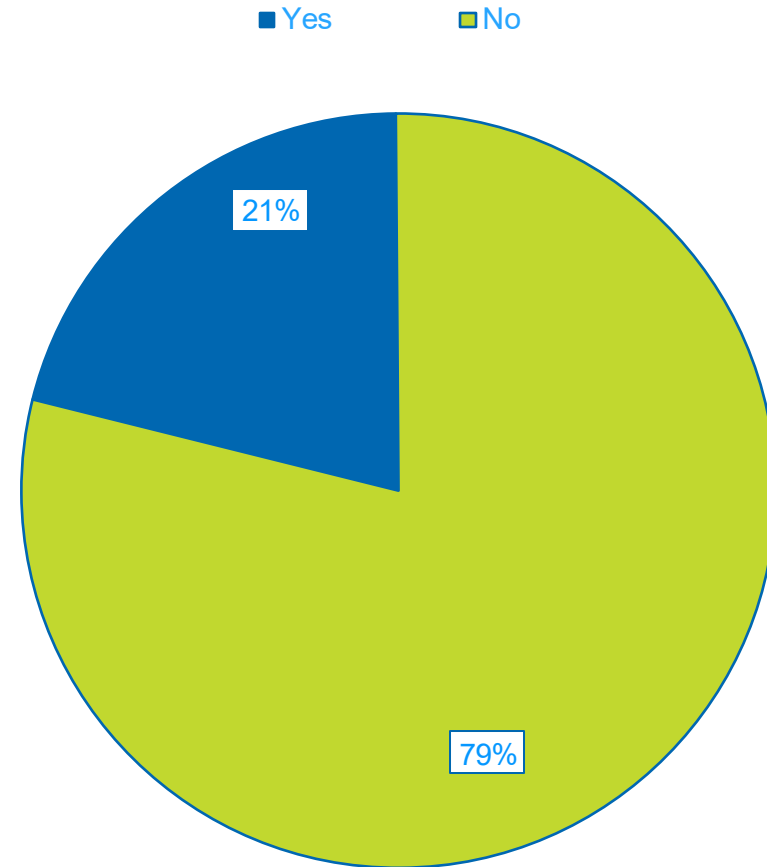
Base: Individuals who received IVIG before using SCIG

Perceived SCIG Effectiveness



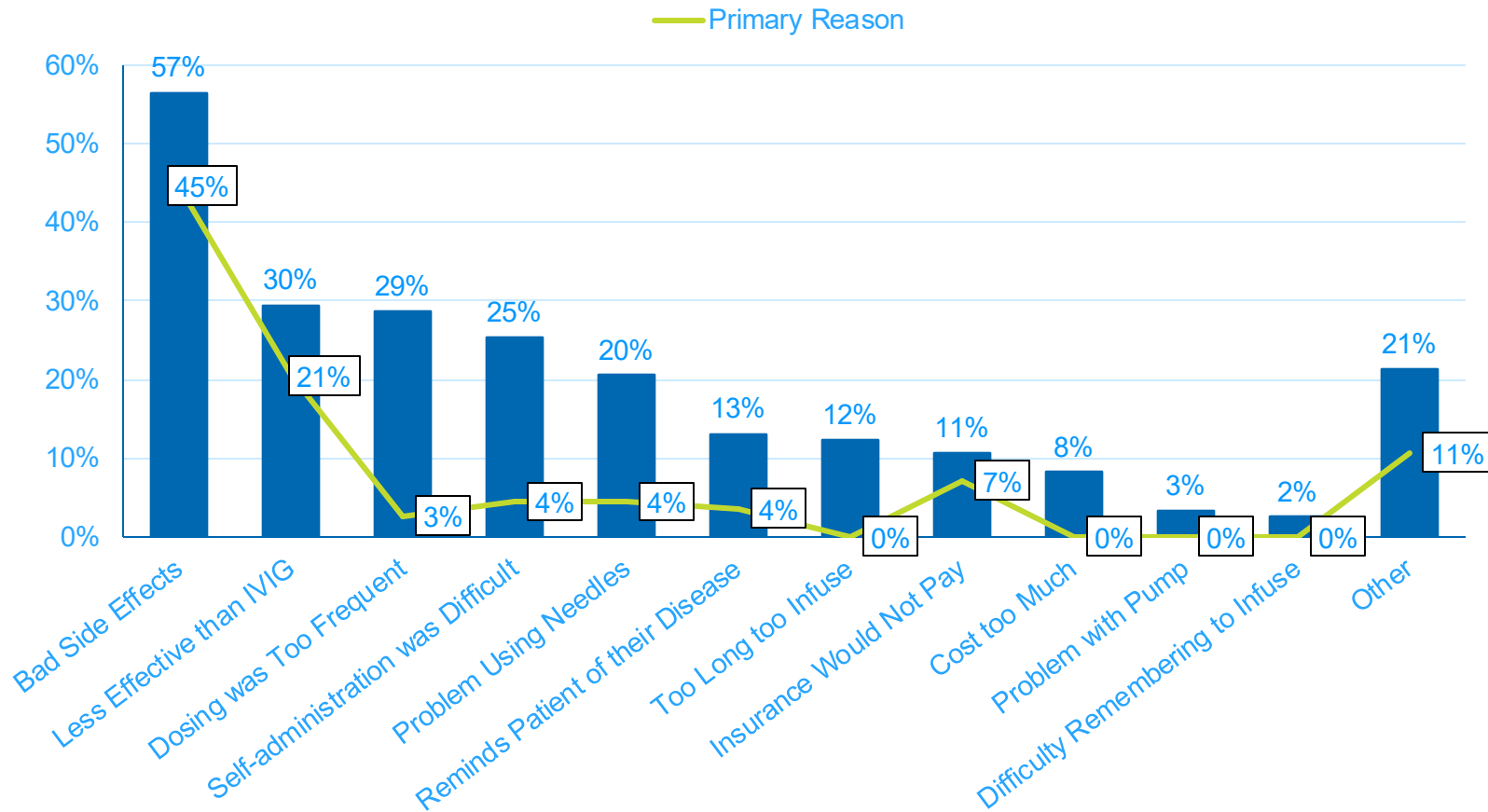
Q28. Compared to IVIG therapy how well do you feel SCIG therapy controls the patient's PI? N=523
Base: Individuals who received IVIG before using SCIG

Patient EVER Used SCIG



Q28. Has the patient ever been on SCIG therapy? N=581
Base: Individuals who currently use MIG or IM

No Longer Using SCIG



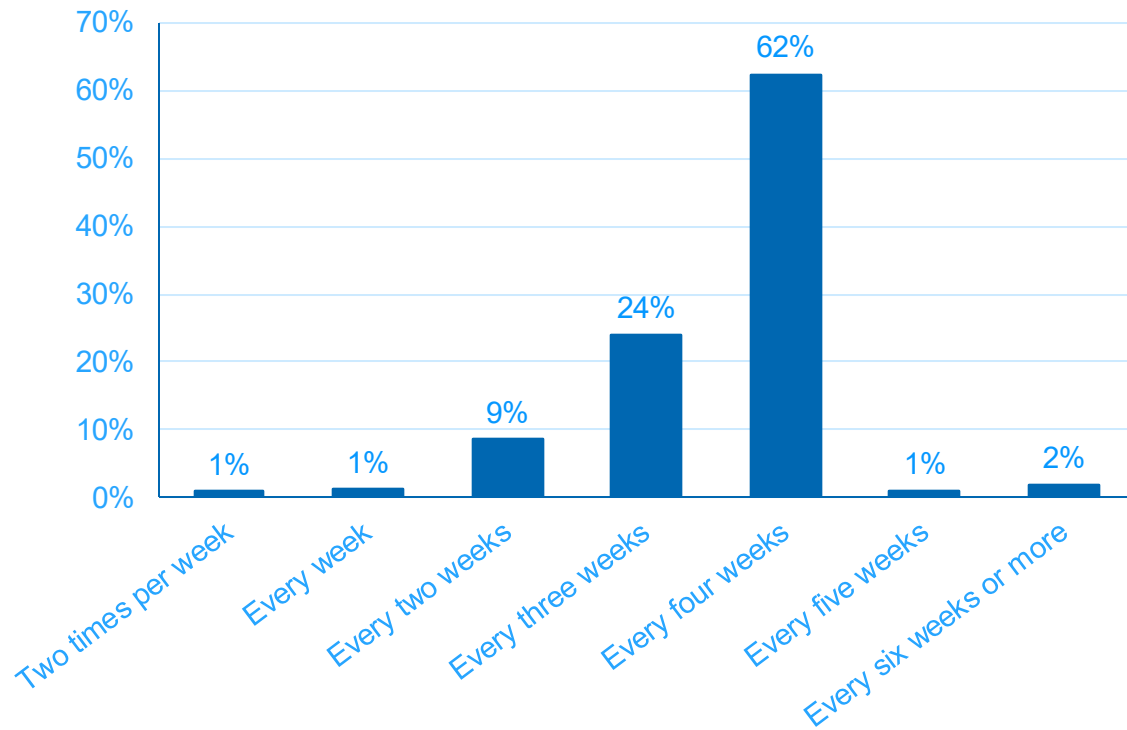
Q29a. Why did the patient switch from SCIG to SCIG? SELECT ALL THAT APPLY N=122

Q29b. Of the reasons selected above, please choose the primary reason why the patient switched from SCIG to IVIG below (select only one) N=11

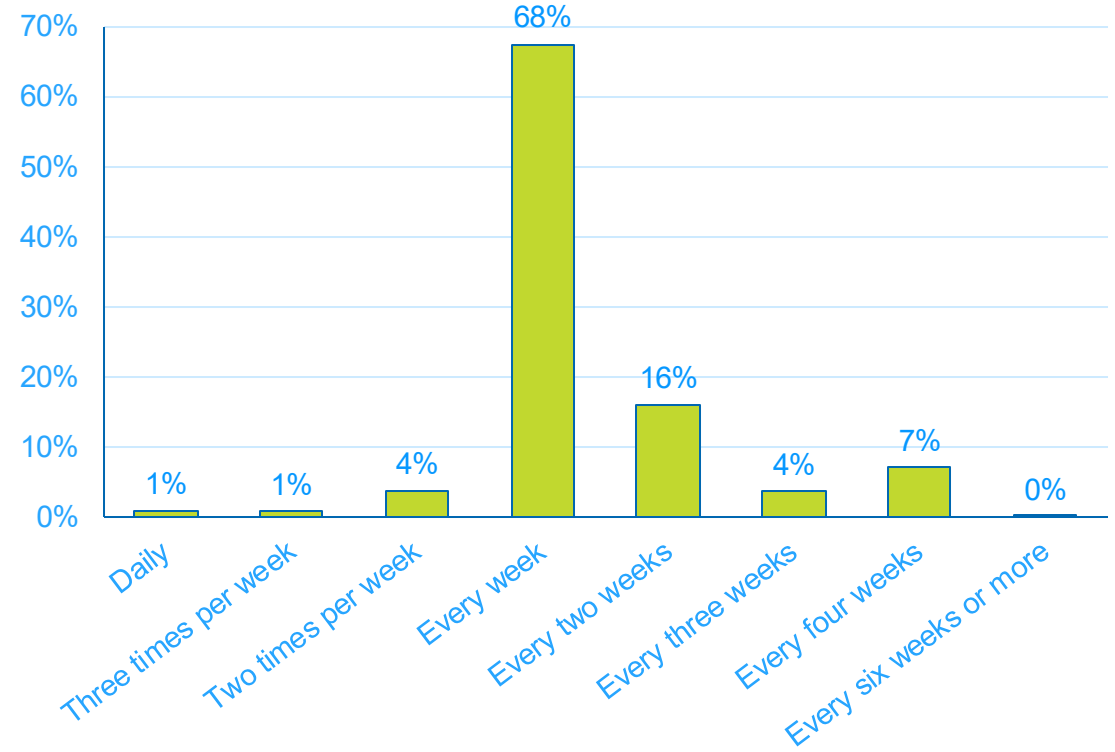
Base: Individuals who received IVIG before using SCIG

Frequency of Ig Treatment

IVIg
N=583



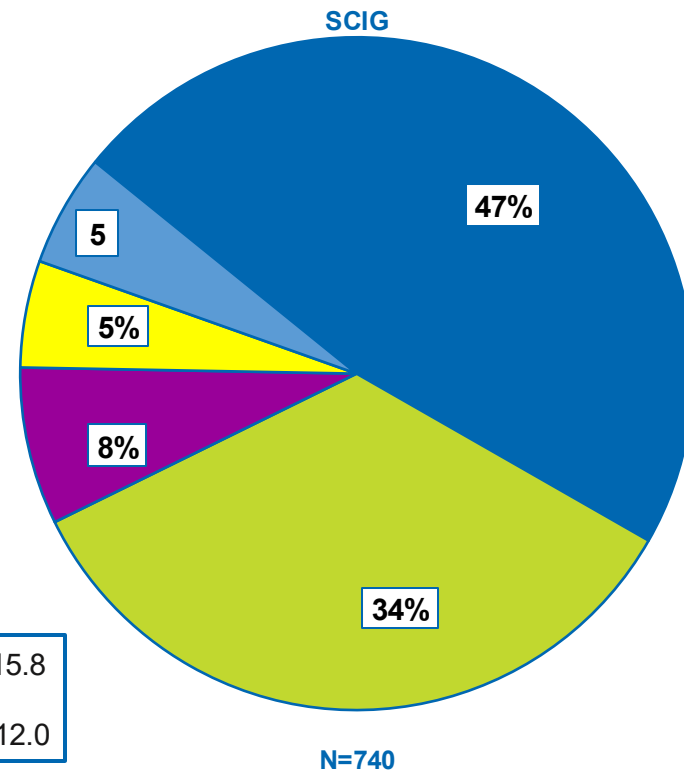
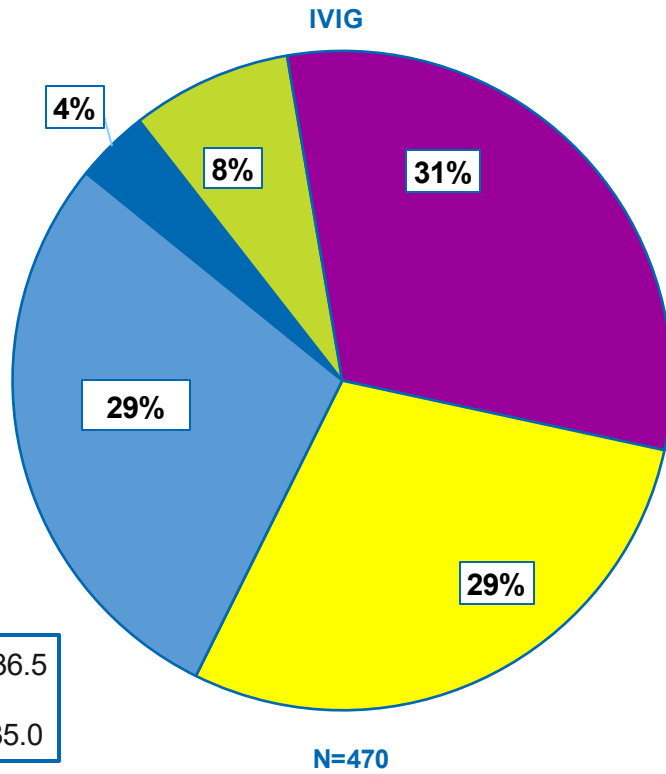
SCIG
N=915



Q30. On average, how often does he/she get their MG or SCIG therapy?
Base: Individuals who currently use MG or SCIG

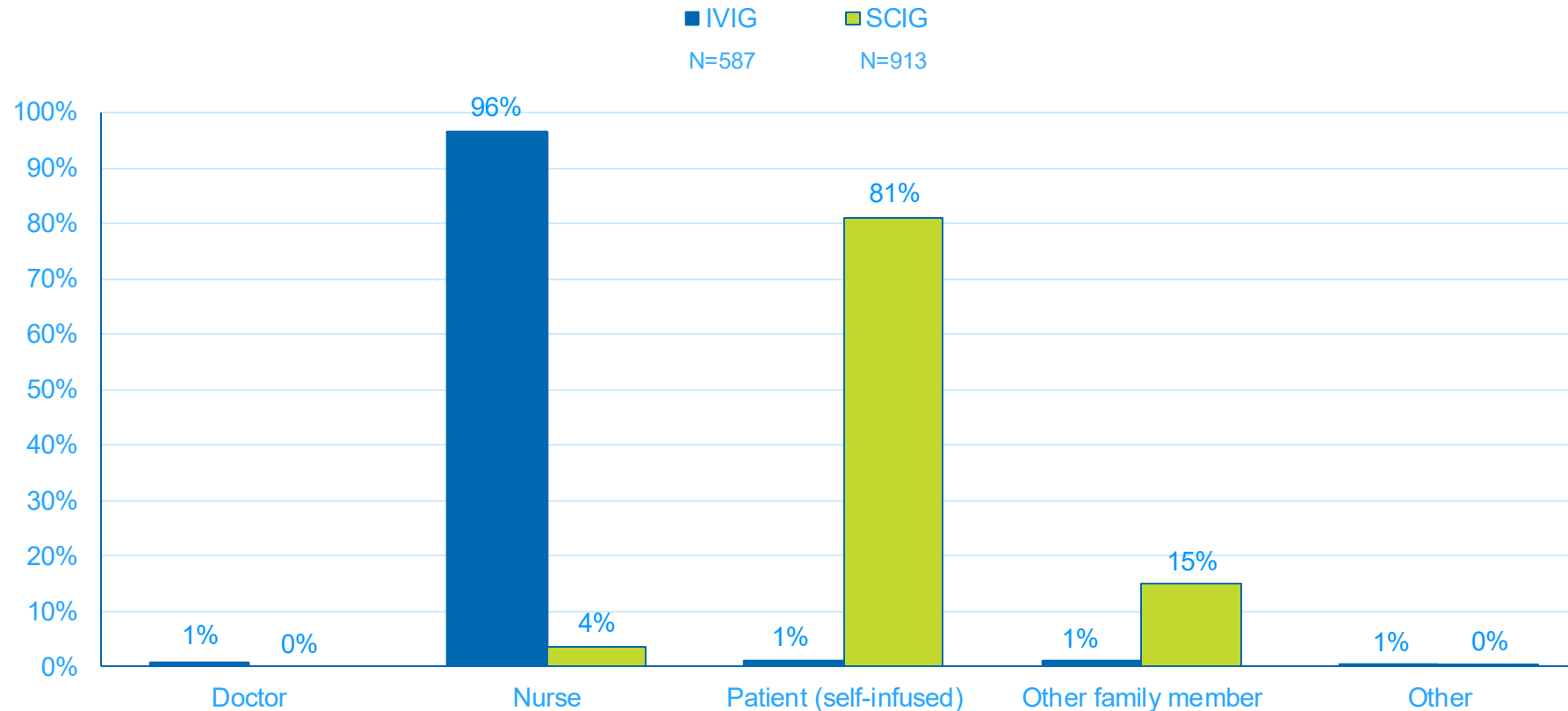
Grams of Ig per Infusion for Adult Patients

■ 1 to 10 grams ■ 11 to 20 grams ■ 21 to 30 grams ■ 31 to 40 grams ■ More than 40 grams



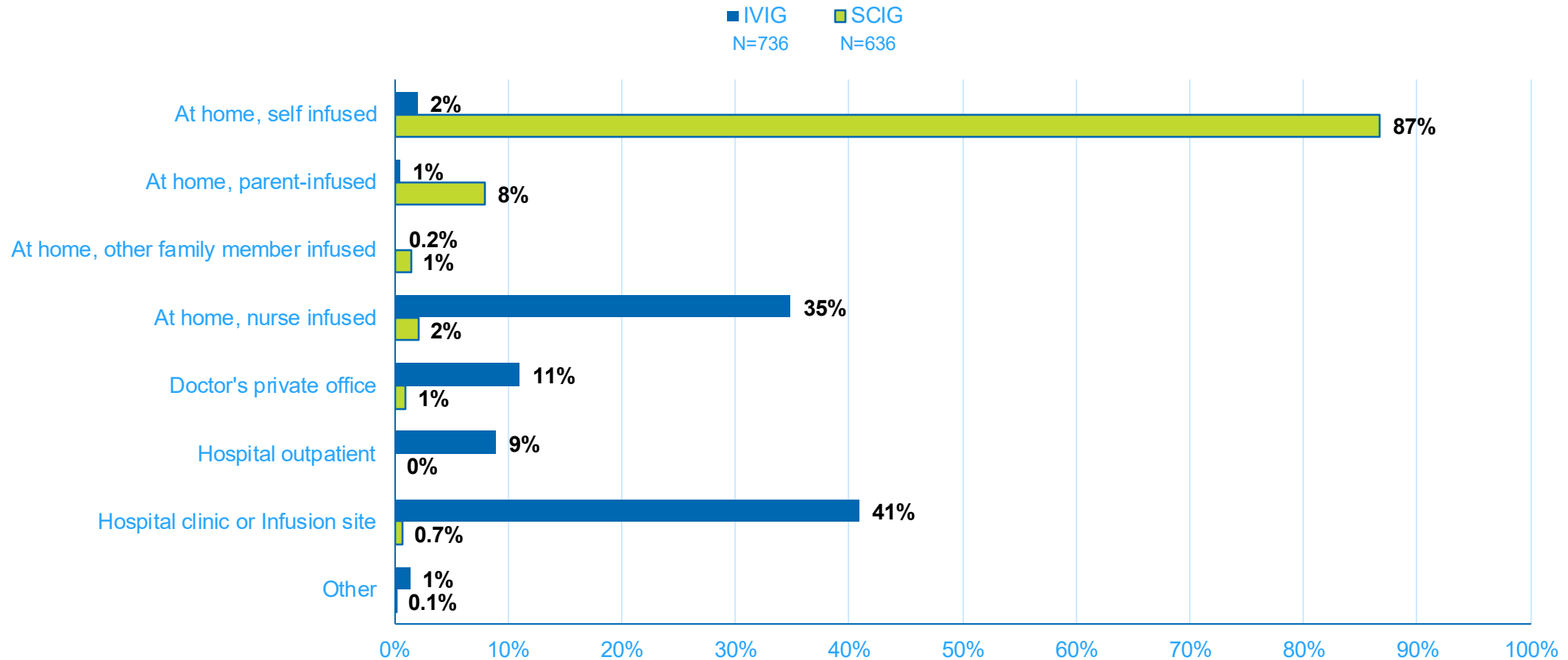
Q31. About how many grams of Ig per infusion does he/she normally receive?
Base: Adult Individuals who currently use IVIG or SCIG

Who Administers Ig Therapy



Q32. Who usually administers the therapy?
Base: Individuals who currently use IVIG or SCIG

Where is Ig Therapy Administered



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

Time to Complete Ig Therapy

| | IVIG | SCIG |
|---------------------|-----------|----------|
| Preparation* | n=509 | n=894 |
| Mean | 56min | 36min |
| Median | 30min | 20min |
| Infusion* | n=590 | n=899 |
| Mean | 4hr 18min | 2hr 0min |
| Median | 4hr 0min | 2hr 0min |
| Clean up | n=491 | n=889 |
| Mean | 37min | 24min |
| Median | 15min | 10min |

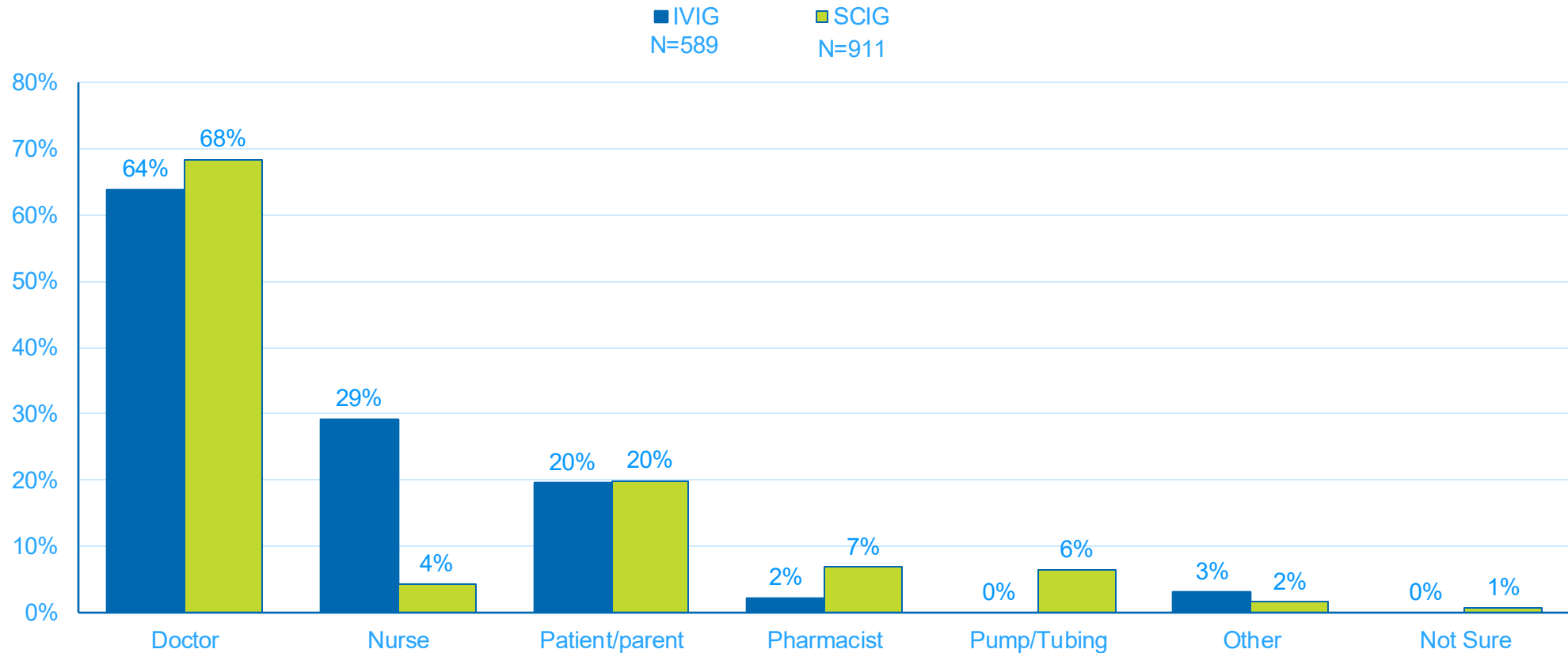
Q34a. How long does the preparation time before each infusion usually take for the patient or the patient's parent/caregiver?

Q34b. How long does the actual infusion treatment usually take for the patient?

Q34c. How long does the post-infusion (e.g., clean-up) time usually take for the patient?

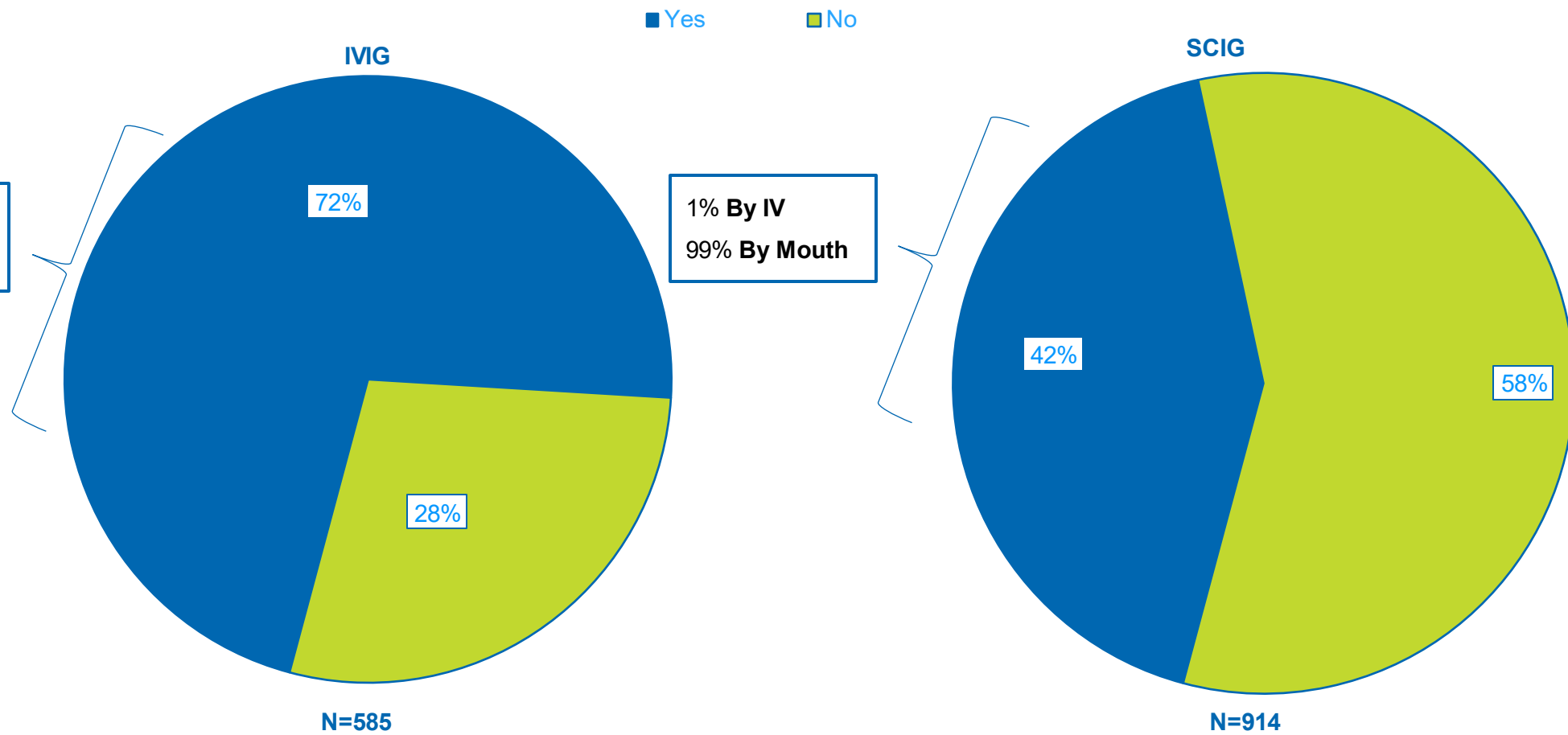
Base: Individuals who currently use IVIG or SCIG

Who Determines the Rate of Infusion



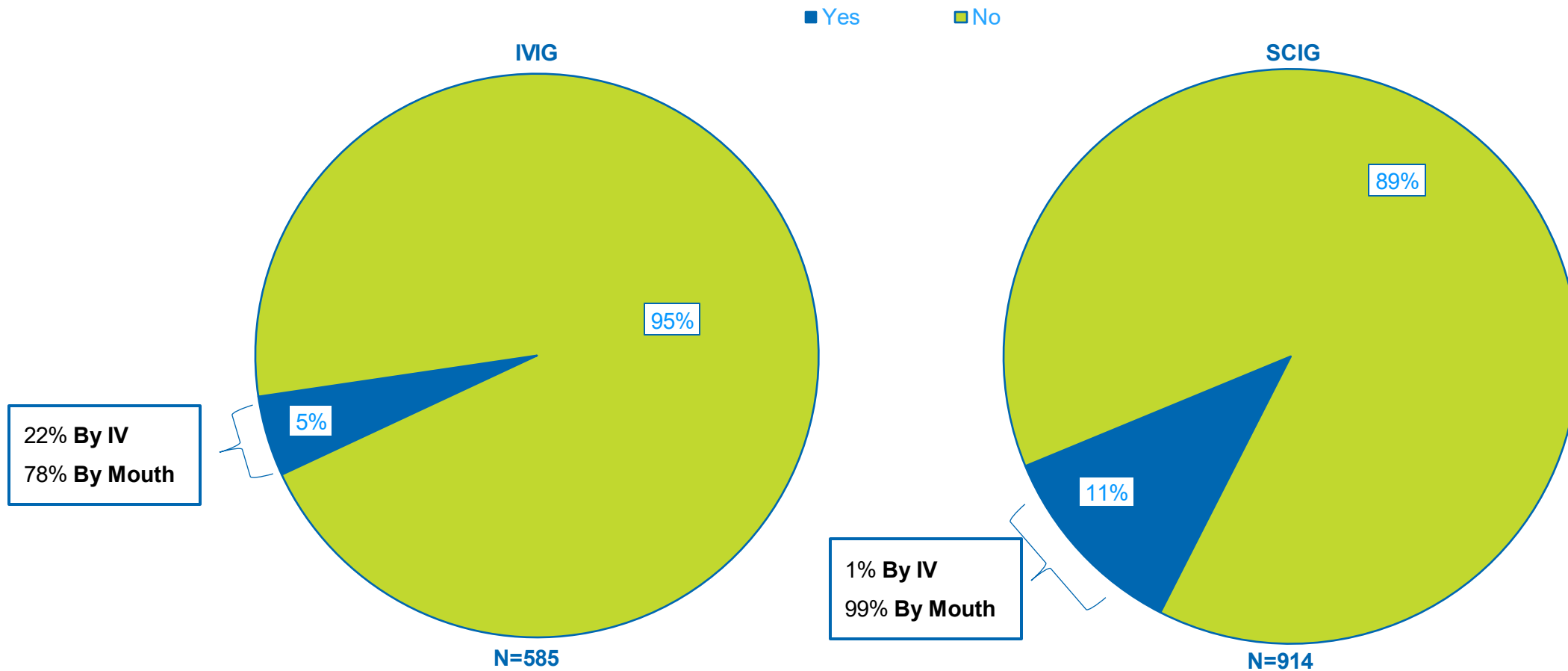
Q36. Who determines the rate of infusion?
Base: Individuals who currently use IVIG or SCIG

Antihistamine Usually Administered Before Ig Therapy Administered*



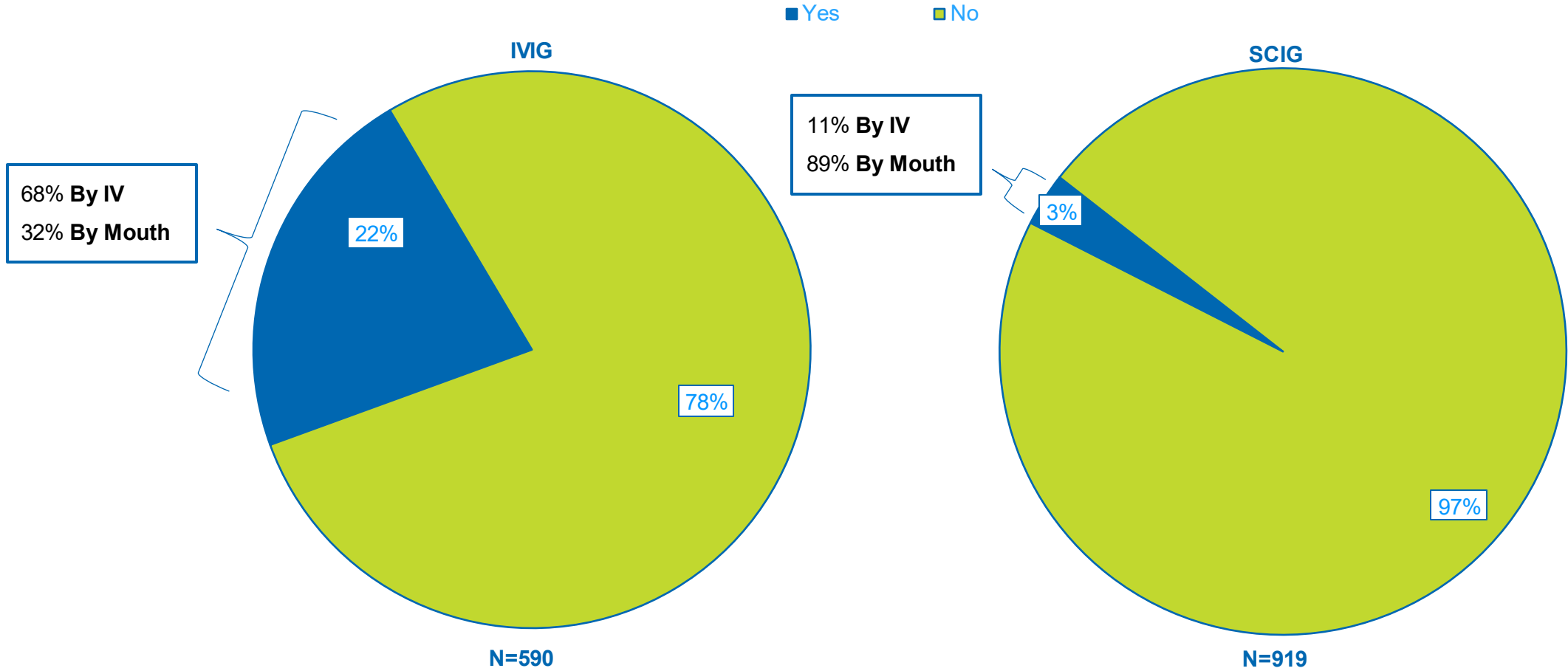
Q37a. Is the patient given medication before Ig therapy, like an antihistamine, corticosteroid or anti-inflammatory, to make it go easier or faster?
Base: Individuals who currently use IVIG or SCIG

Antihistamine Sometimes Administered Before Ig Therapy Administered*



Q37a. Is the patient given medication before Ig therapy, like an antihistamine, corticosteroid or anti-inflammatory, to make it go easier or faster?
Base: Individuals who currently use IVIG or SCIG

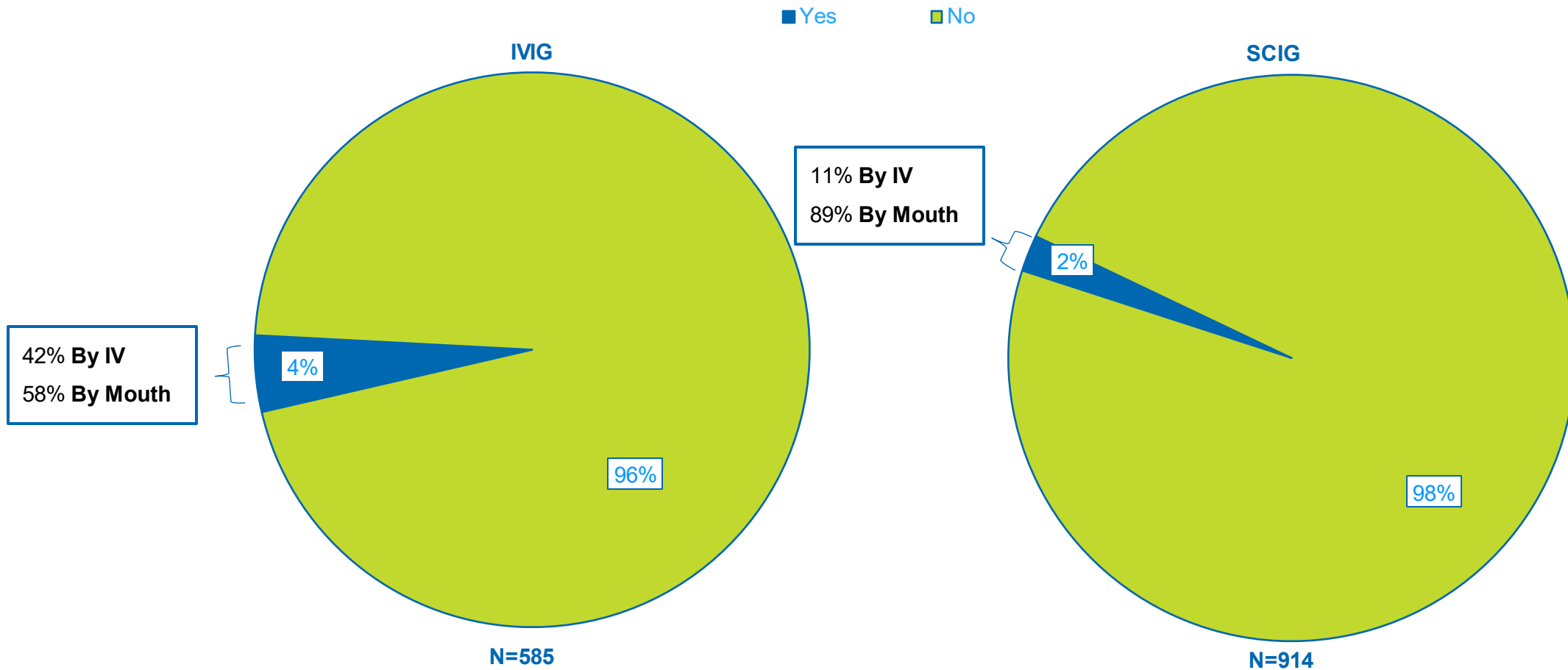
Steroid Usually Administered Before Ig Therapy Administered*



Q37a. Is the patient given medication before Ig therapy, like an antihistamine, corticosteroid or anti-inflammatory, to make it go easier or faster?
Base: Individuals who currently use IVIG or SCIG

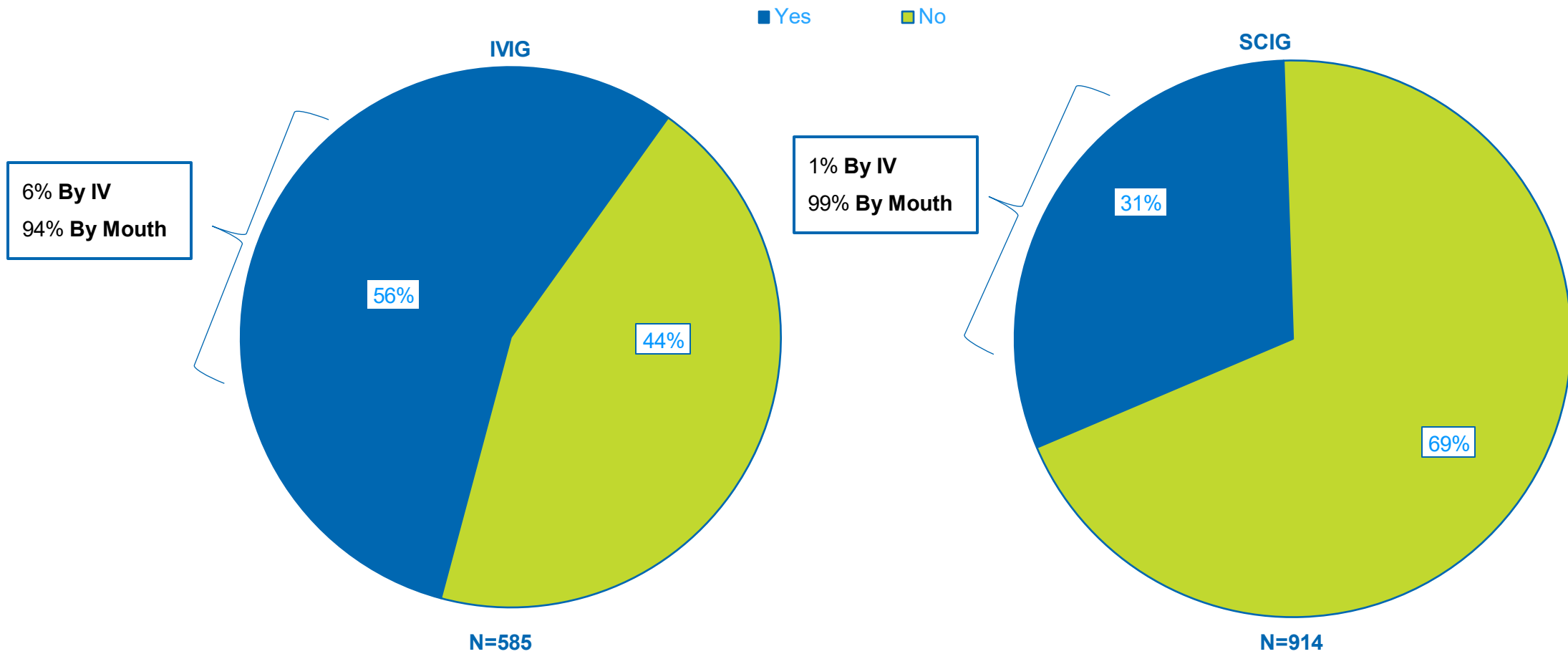
Source: IDF 2018 Treatment Survey

Steroid Sometimes Administered Before Ig Therapy Administered*



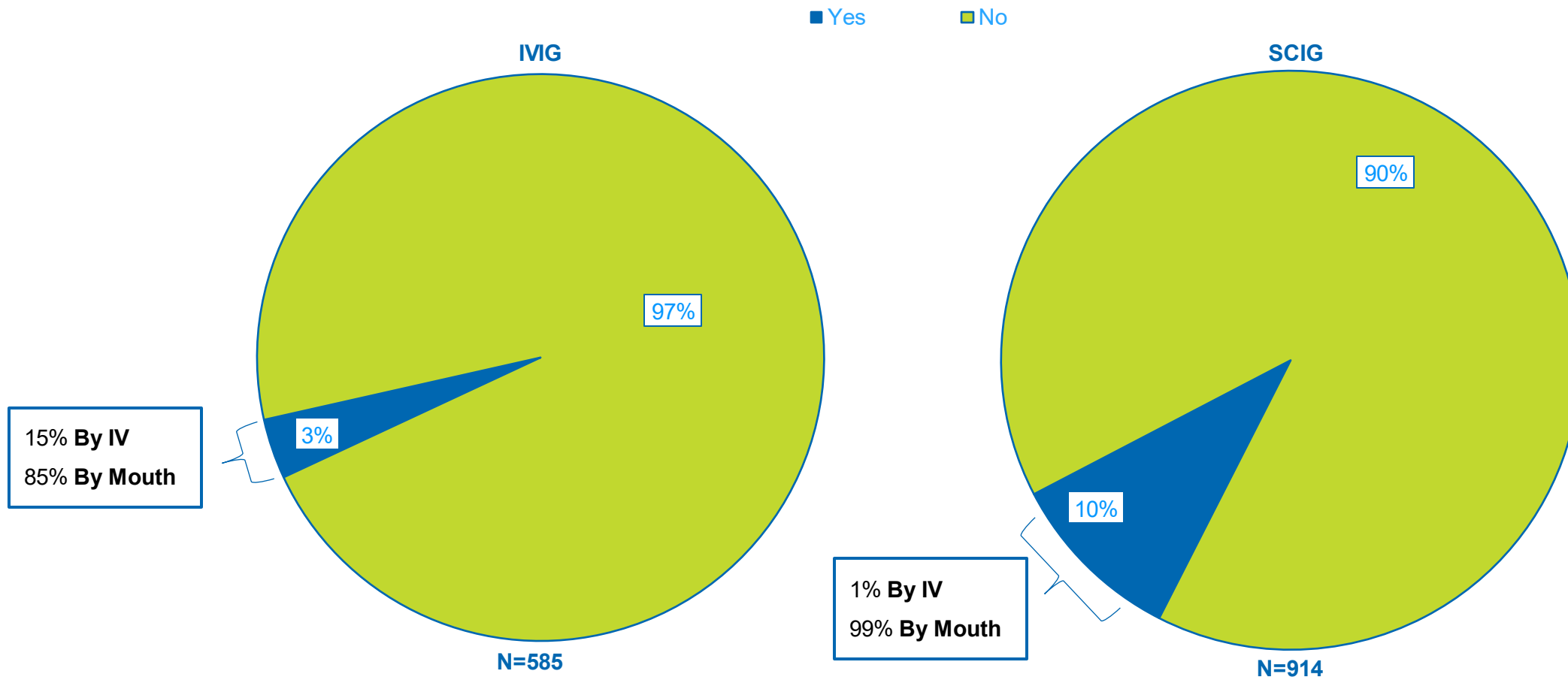
Q37a. Is the patient given medication before Ig therapy, like an antihistamine, corticosteroid or anti-inflammatory, to make it go easier or faster?
Base: Individuals who currently use IMG or SCIG

Anti-inflammatory Usually Administered Before Ig Therapy Administered*



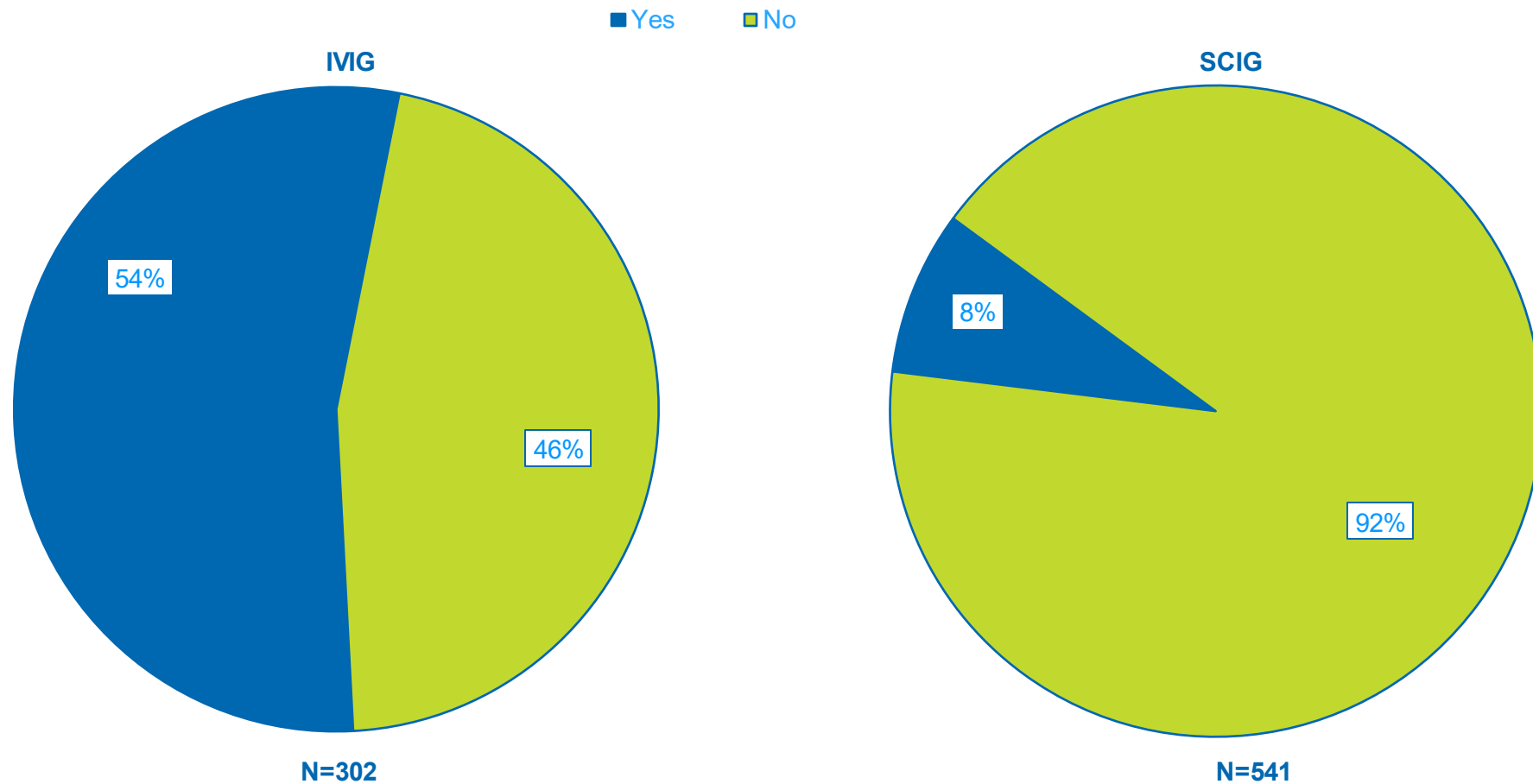
Q37a. Is the patient given medication before Ig therapy, like an antihistamine, corticosteroid or anti-inflammatory, to make it go easier or faster?
Base: Individuals who currently use IVIG or SCIG

Anti-inflammatory Sometimes Administered Before Ig Therapy Administered*



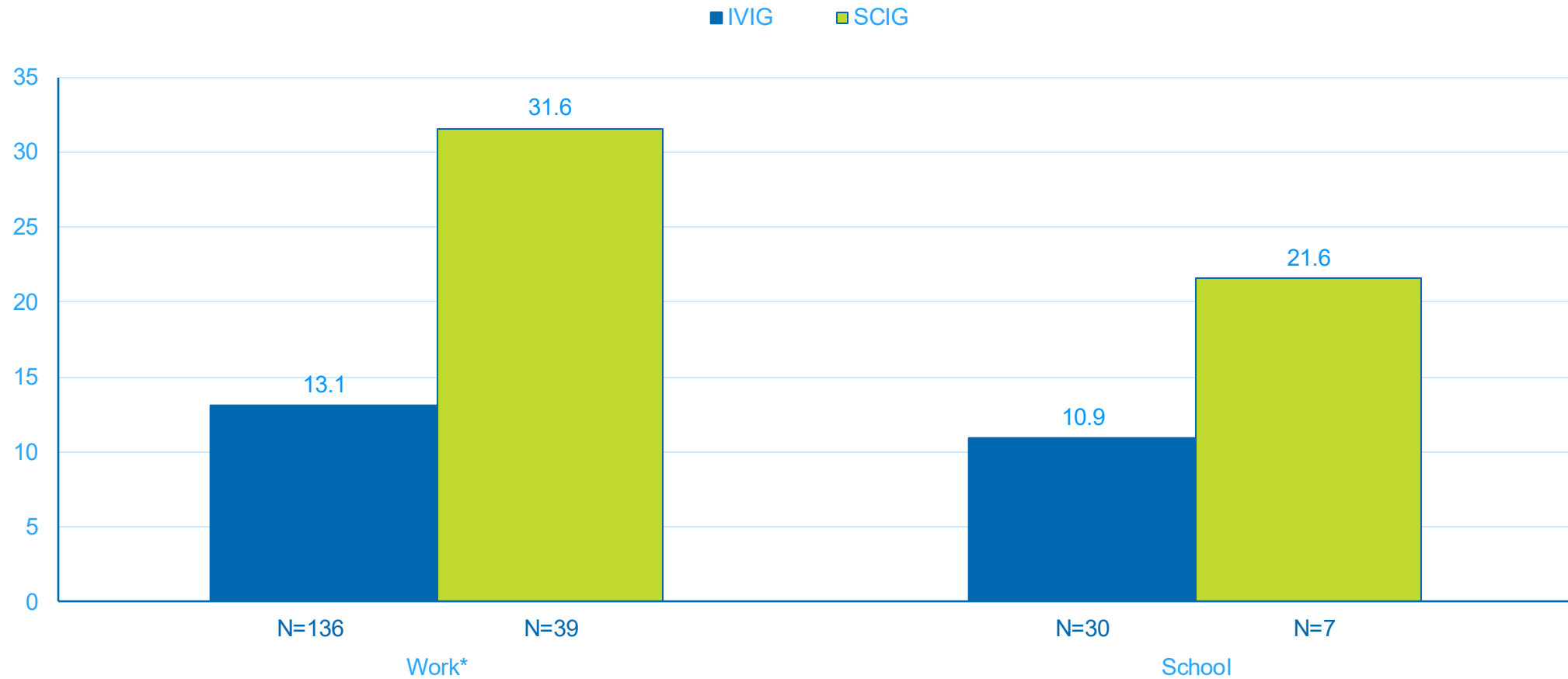
Q37a. Is the patient given medication before Ig therapy, like an antihistamine, corticosteroid or anti-inflammatory, to make it go easier or faster?
Base: Individuals who currently use IVIG or SCIG

Missed Work or School Due to Ig Therapy*



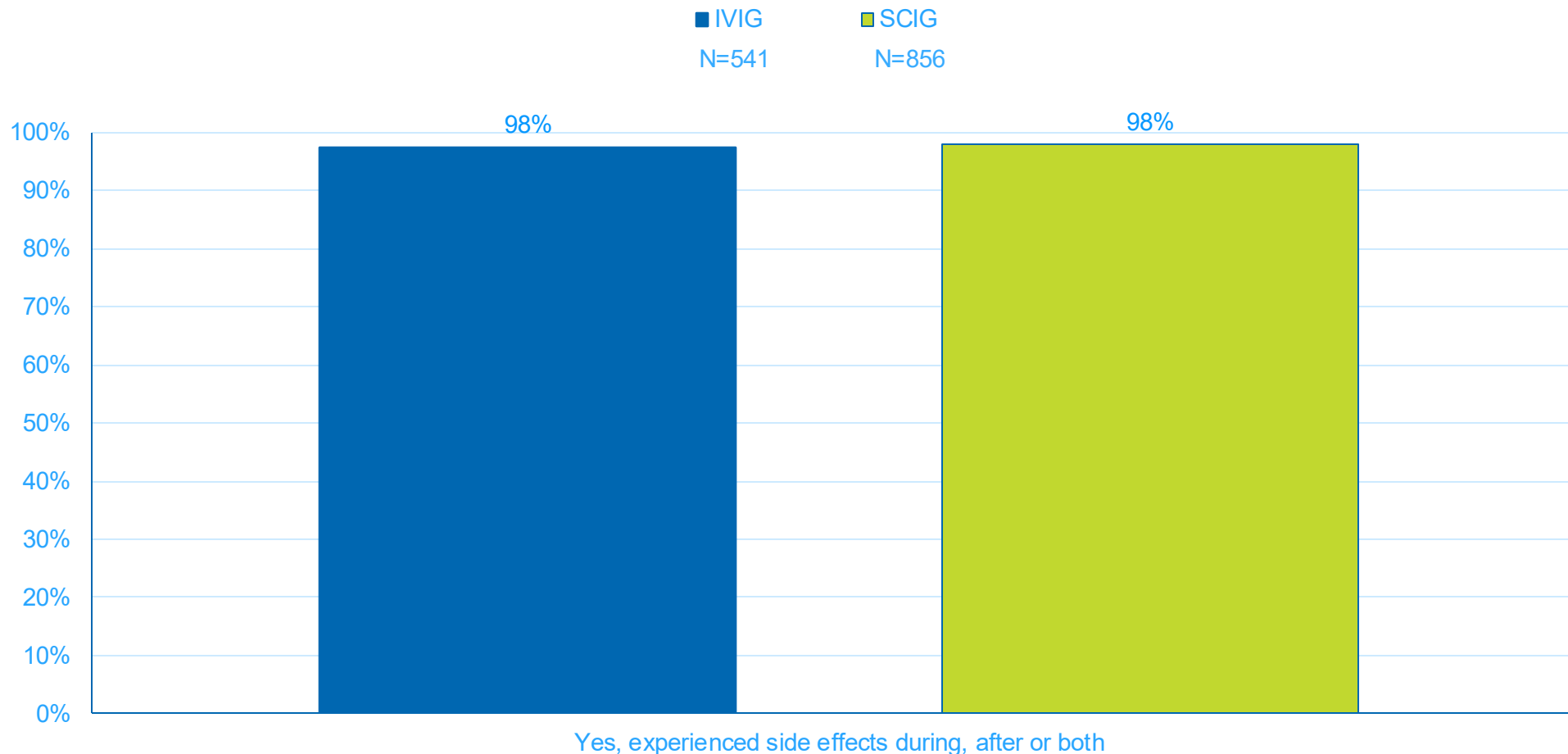
Q38a. Does the patient (parent/caretaker) need to take off from school or work to get their Ig therapy?
Base: Individuals who currently use IVIG or SCIG
Note: Patients with PI and Parents/Caregivers who are too disabled to work or retired were excluded

Missed Work or School Due to Ig Therapy



Q38a. Does the patient (parent/caretaker) need to take off from school or work to get their Ig therapy?
Base: Individuals who currently use IVIG or SCIG
Note: Patients with PI and Parents/Caregivers who are too disabled to work or retired were excluded

Side Effects of Ig Therapy



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

Side Effects of Ig Therapy

IVIG

N=584

| Side Effect | % | Side Effect | % |
|----------------------------|-----|---------------------------|------|
| Fatigue | 71% | Drop in blood pressure | 15% |
| Headache | 71% | Muscle spasms | 15% |
| Muscle aches | 43% | Abdominal pain | 14% |
| Chills | 39% | Redness at infusion site | 13% |
| Migraine headache | 35% | Itching at infusion site | 12% |
| Nausea | 30% | Swelling at infusion site | 9% |
| Weakness | 30% | Wheezing | 8% |
| Fever | 26% | Vomiting | 7% |
| Dizziness | 25% | Hives | 7% |
| Anxiety | 23% | Kidney problems | 5% |
| Increase in blood pressure | 22% | Blood in tubing | 3% |
| Diarrhea | 21% | Hepatitis | 0.4% |

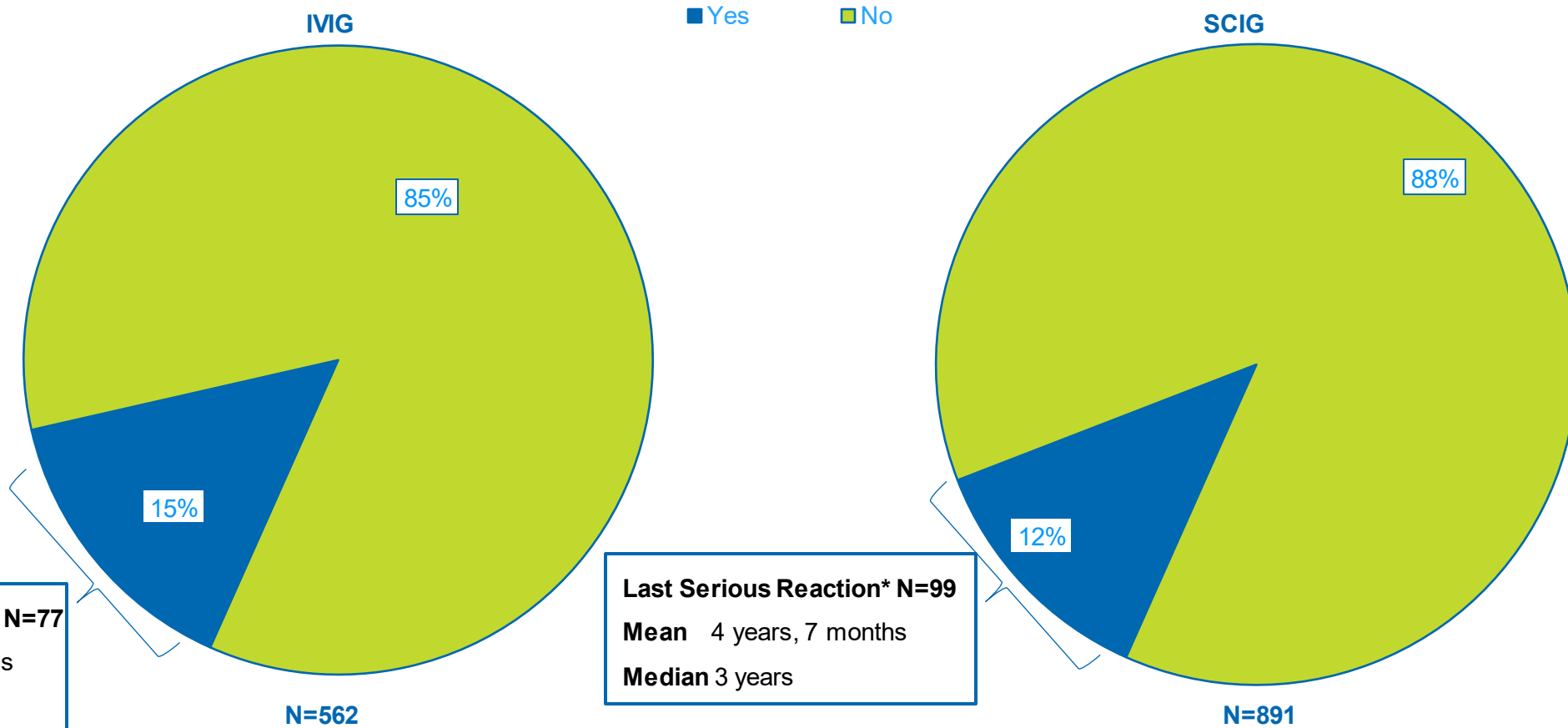
SCIG

N=839

| Side Effect | % | Side Effect | % |
|---------------------------|-----|----------------------------|-----|
| Redness at infusion site | 73% | Weakness | 18% |
| Swelling at infusion site | 64% | Diarrhea | 17% |
| Fatigue | 59% | Fever | 16% |
| Itching at infusion site | 50% | Dizziness | 14% |
| Headache | 50% | Muscle spasms | 11% |
| Blood in tubing | 39% | Increase in blood pressure | 8% |
| Muscle aches | 30% | Hives | 7% |
| Chills | 26% | Drop in blood pressure | 5% |
| Abdominal pain | 23% | Wheezing | 4% |
| Migraine headache | 22% | Vomiting | 3% |
| Anxiety | 22% | Kidney problems | 2% |
| Nausea | 20% | Hepatitis | 1% |

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

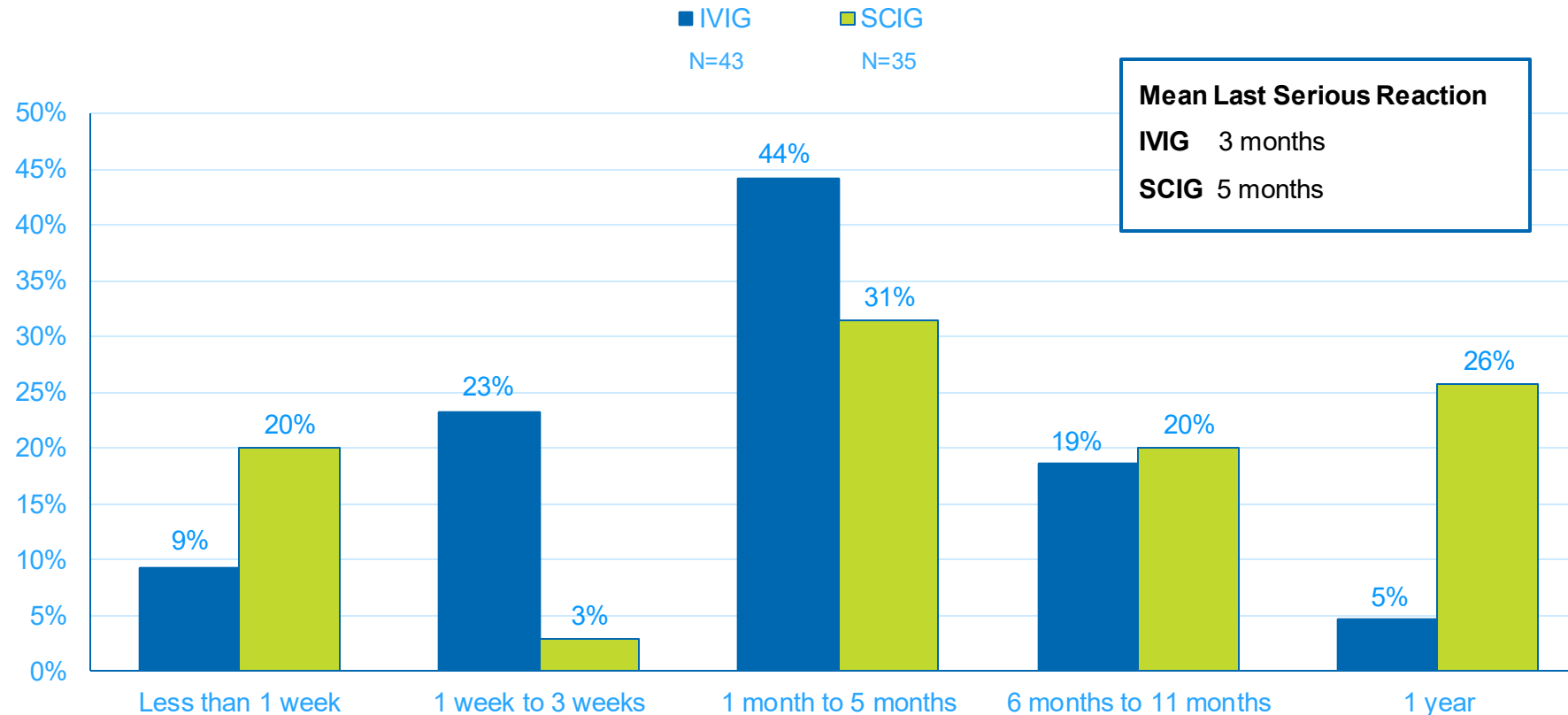
Serious Side Effects or Reactions



Last Serious Reaction* N=77
Mean 2 years, 9 months
Median 6 months

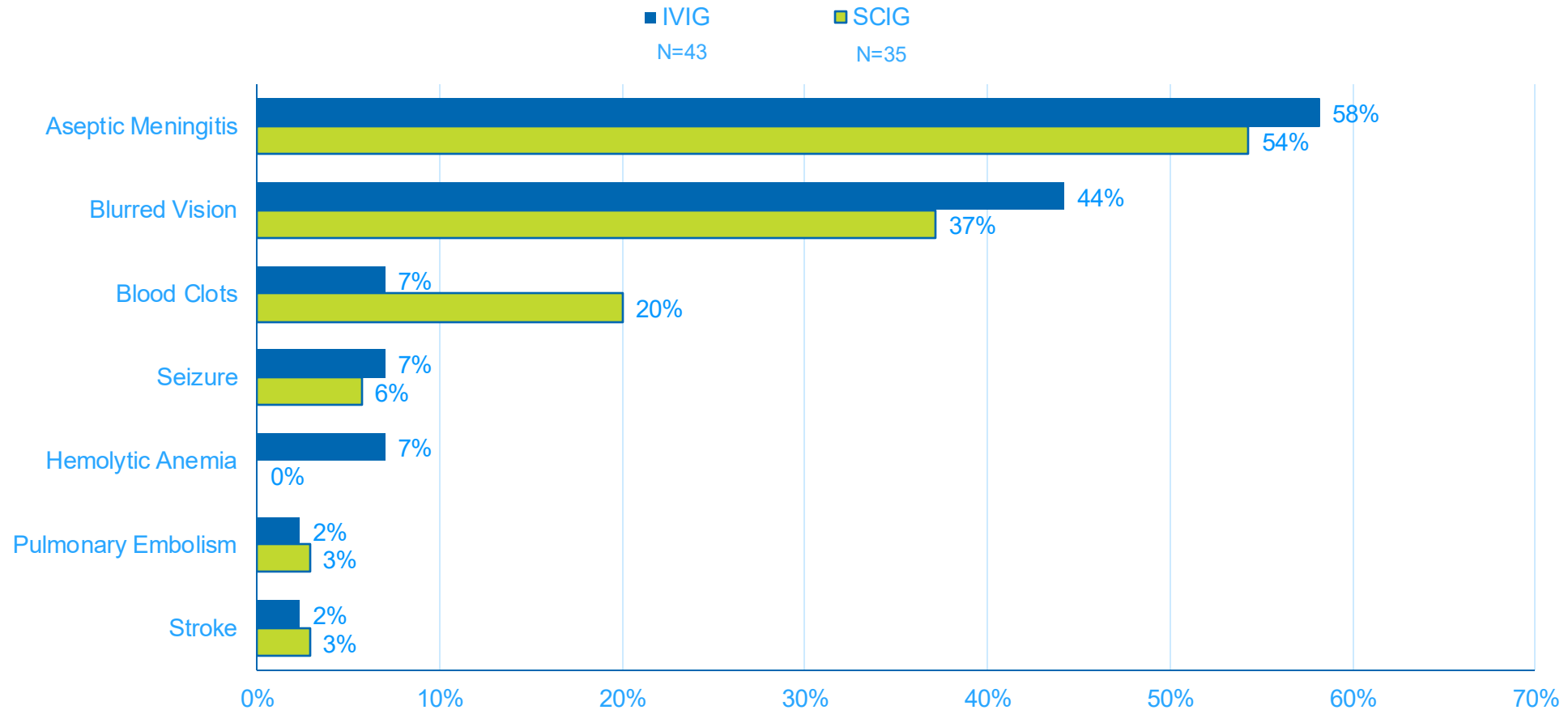
Q40a. Has the patient ever had any of the following serious side-effects or reactions from their Ig?
Q40b. When was the most recent time that he/she had a serious side effect or reaction from their Ig?
Base: Individuals who currently use IVIG or SCIG
Note: Only time since last reaction was significant

In the Past 12 Months Last Time Serious Side Effects or Reactions Occurred*



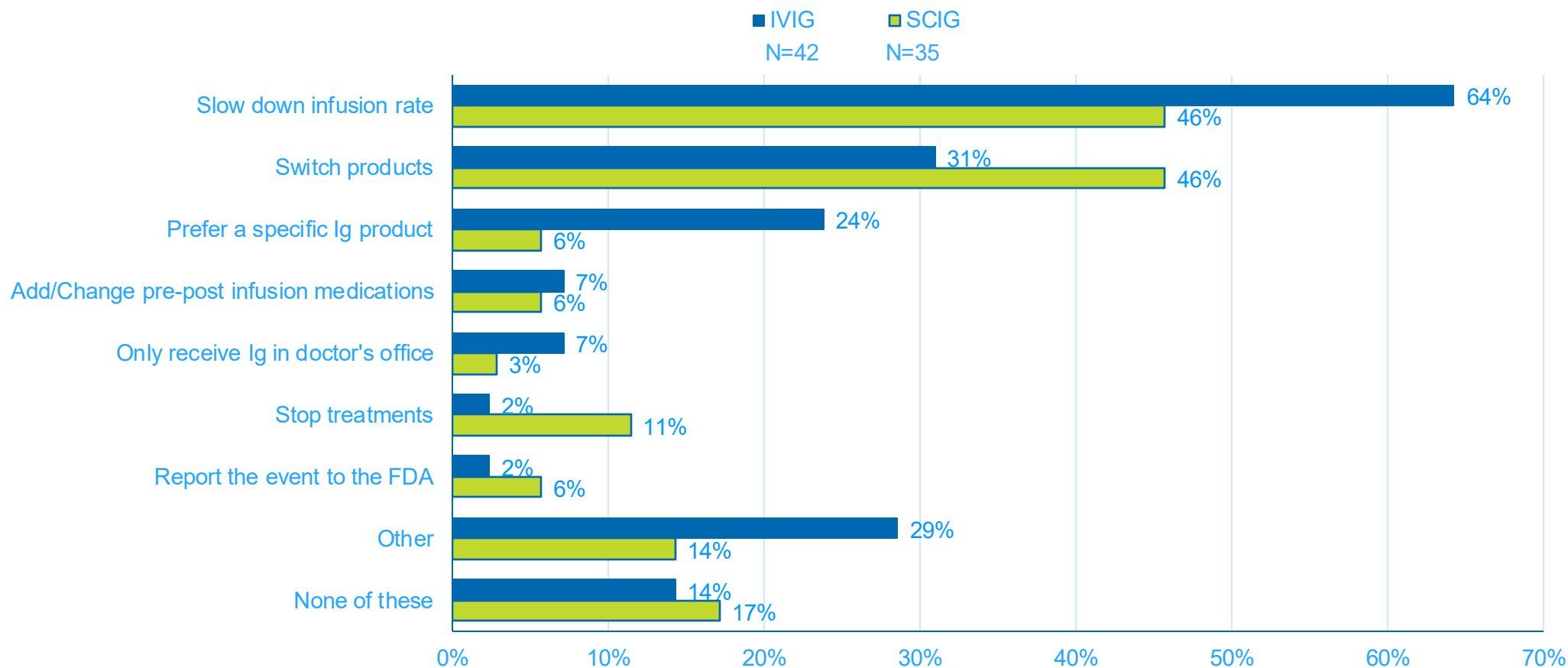
Q40b. When was the most recent time that he/she had a serious side effect or reaction from their Ig?
Base: Individuals who currently use IVIG or SCIG who experienced a serious side effect or reaction in the past 12 months

In the Past 12 Months Serious Side Effects or Reactions



Q40a. Has the patient ever had any of the following serious side-effects or reactions from their Ig? PLEASE SELECT ALL THAT APPLY
Base: Individuals who currently use IVIG or SCIG who experienced a serious side effect or reaction in the past 12 months

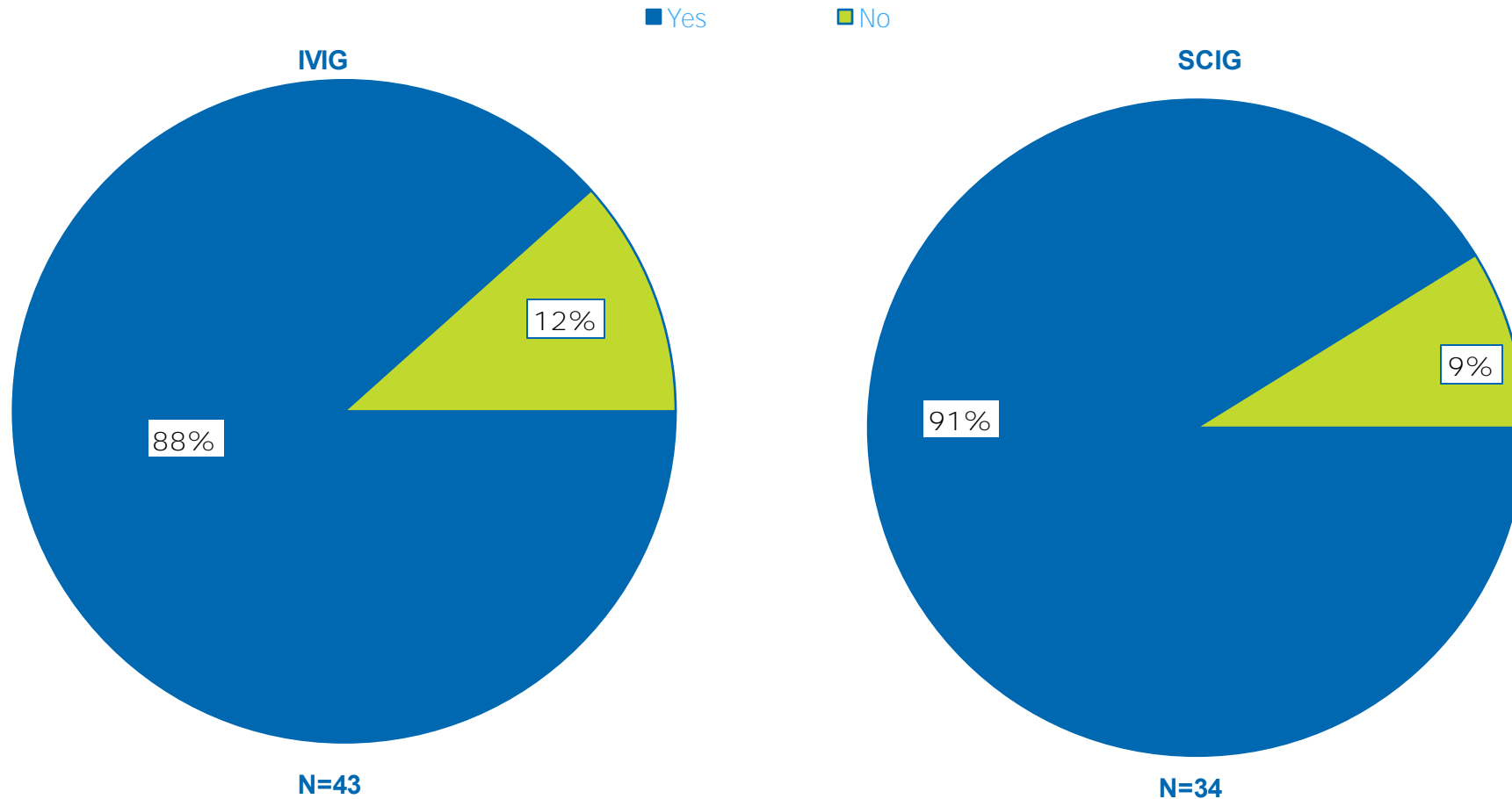
In the Past 12 Months Serious Side Effects or Reactions Caused the Patient to...



Q40c. Did the patient's side effect or reaction cause him/her to ... SELECT ALL THAT APPLY

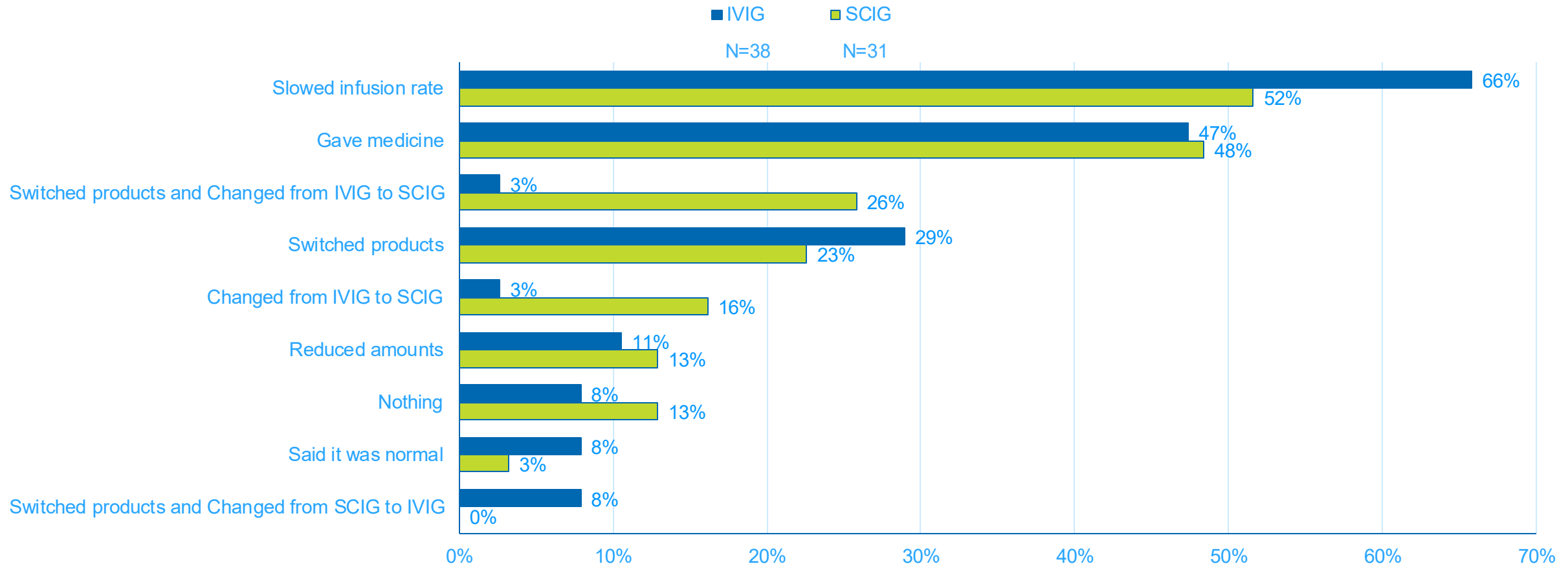
Base: Individuals who currently use IVIG or SCIG IM who experienced a serious side effect or reaction in the past 12 months

In the Past 12 Months Was Doctor Told the Patient had a Serious Side-Effect or Reaction?



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

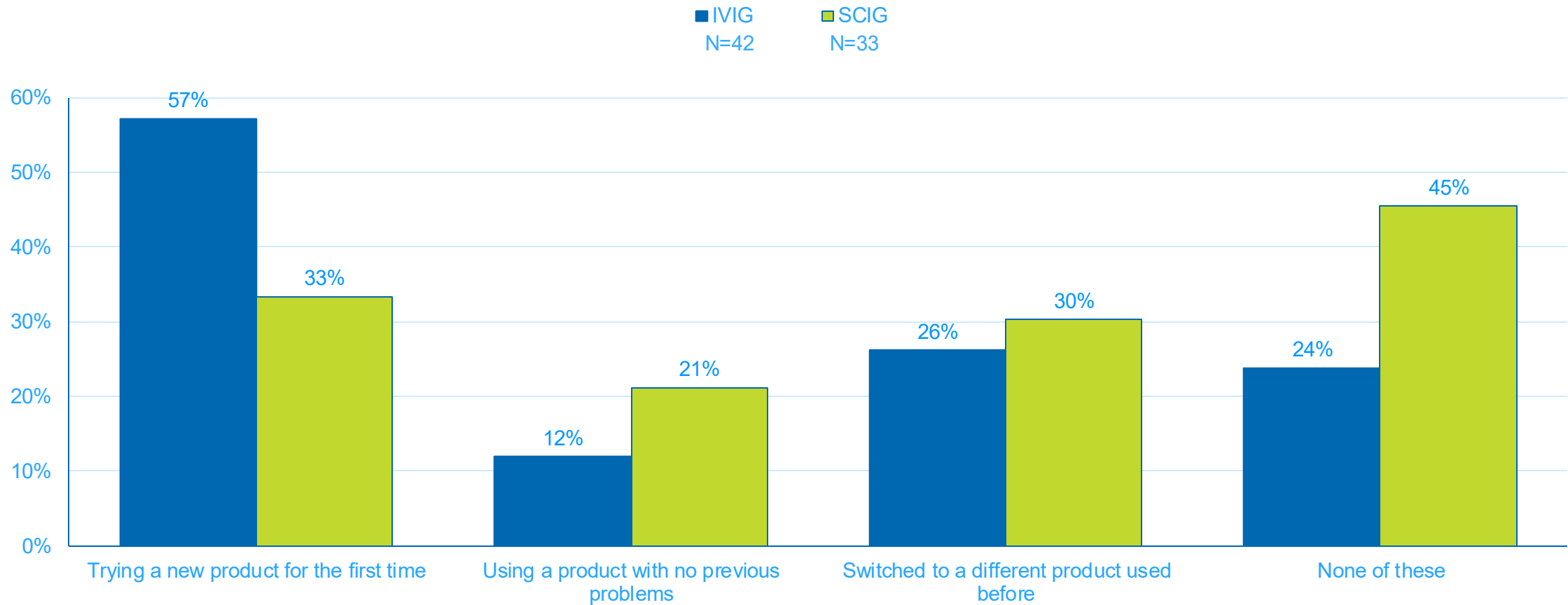
In the Past 12 Months What Did the Doctor Do?



Q40e. What did the doctor do?

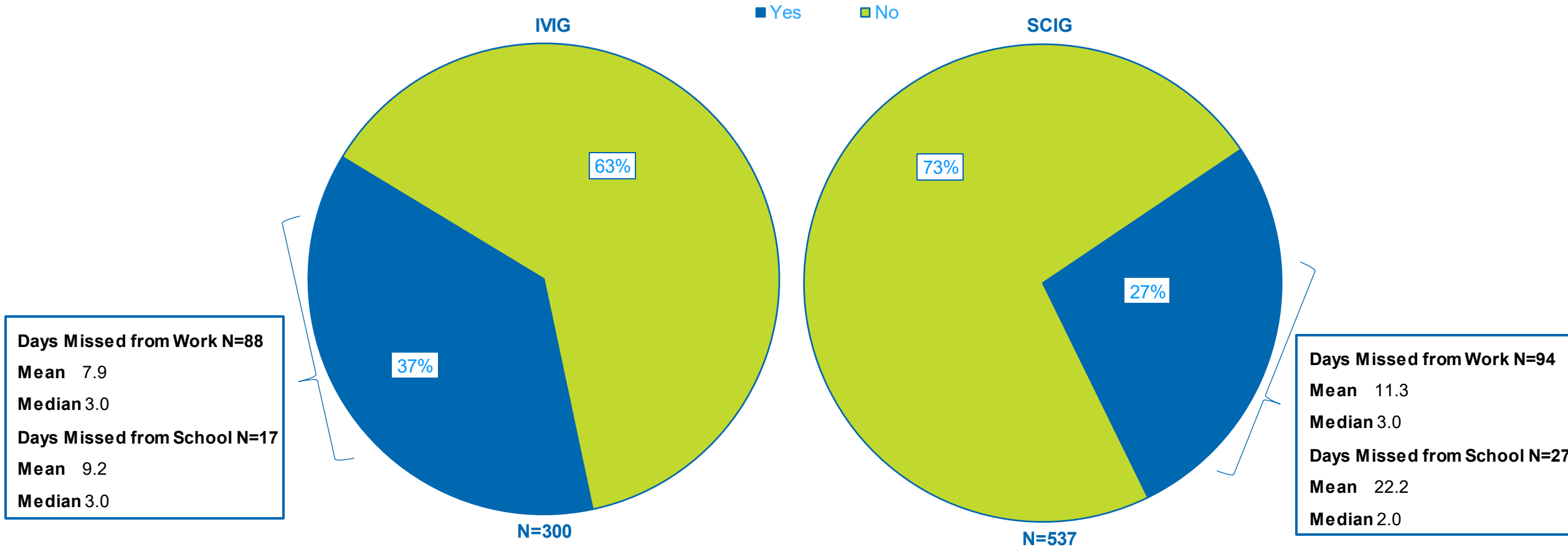
Base: Individuals who currently use IVIG who experienced a serious side effect or reaction

In the Past 12 Months Patient had a Serious Side-Effect or Reaction When...



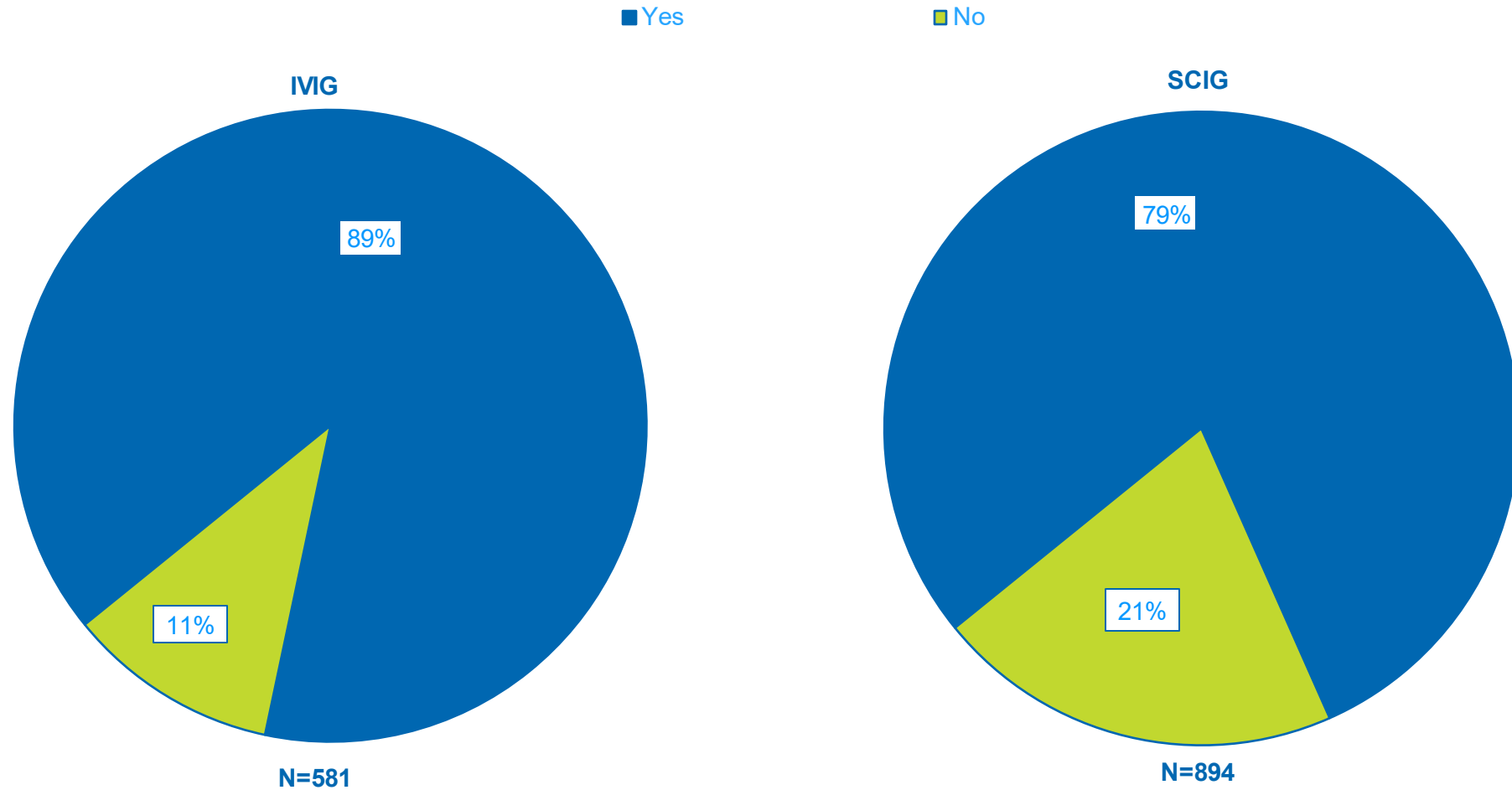
Q40f. Has the patient ever had a serious side effect or reaction from Ig therapy when... SELECT ALL THAT APPLY
Base: Individuals who currently use MG or SCIG who experienced a serious side effect or reaction

Missed Work or School Due to Reaction from Ig Therapy*



Q41a. Has the patient (parent/caretaker of a patient) ever missed school or work due to a reaction from their Ig therapy?
 Q41b. How many days in the past 12 months has the patient (parent/caretaker of a patient) missed school or work due to reactions from Ig therapy?
 Base: Individuals who currently use IMG or SCIG
 Notes: Patients with PI and Parents/Caregivers who are too disabled to work or retired were excluded, Number of days missed was not significant

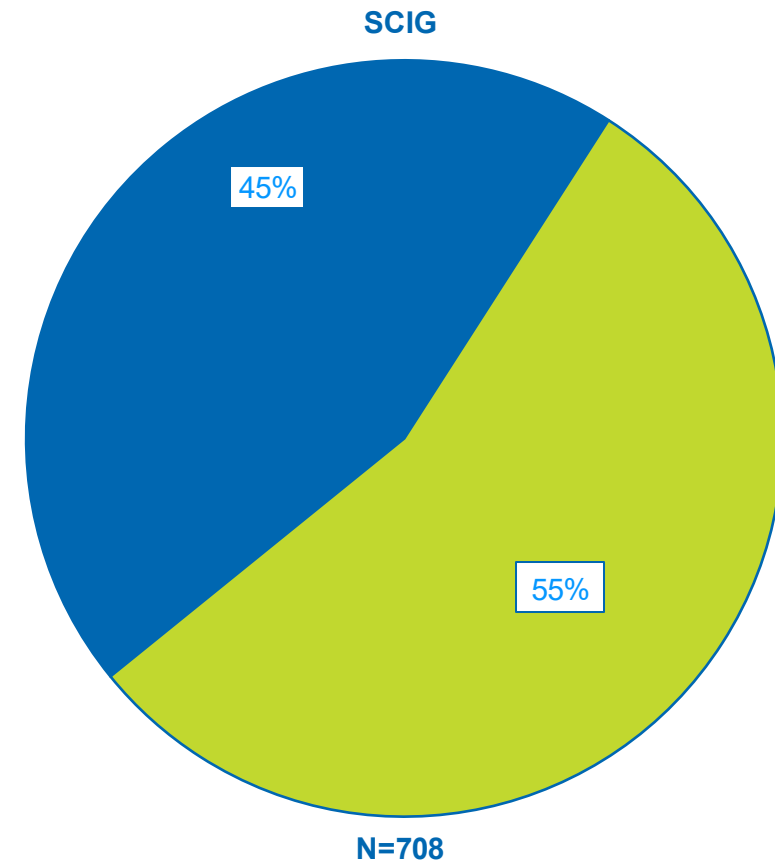
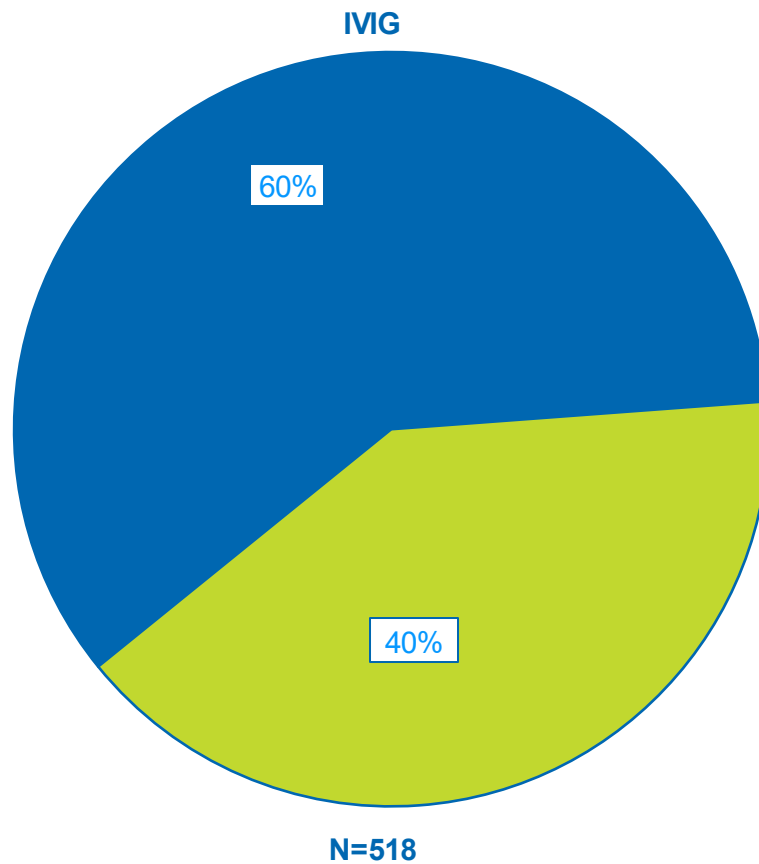
Does the Patient Experience Wear Off Effect?*



Q43a. Does the patient experience periods of fatigue or low energy between Ig therapy treatments (wear off)?
Base: Individuals who currently use IVIG or SCIG

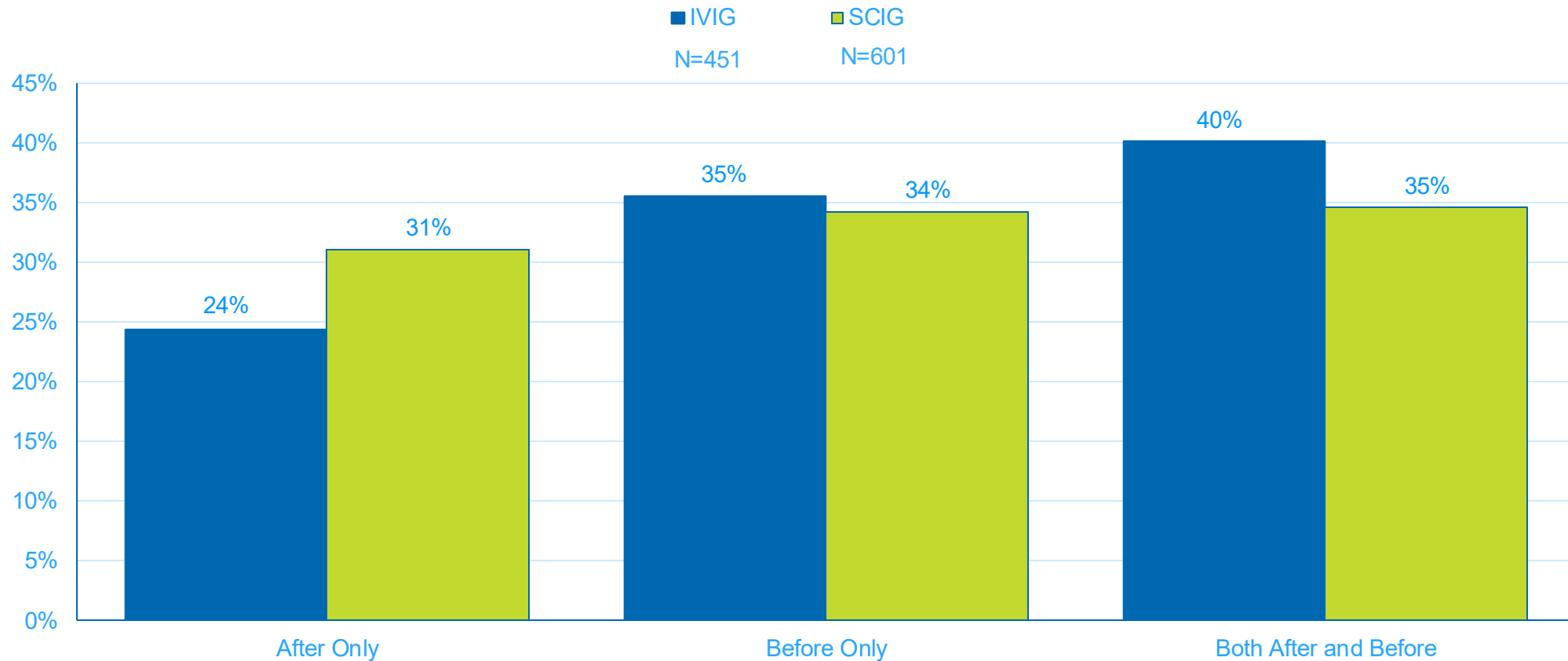
Frequency of Wear Off Effect?*

■ Always ■ Occasionally



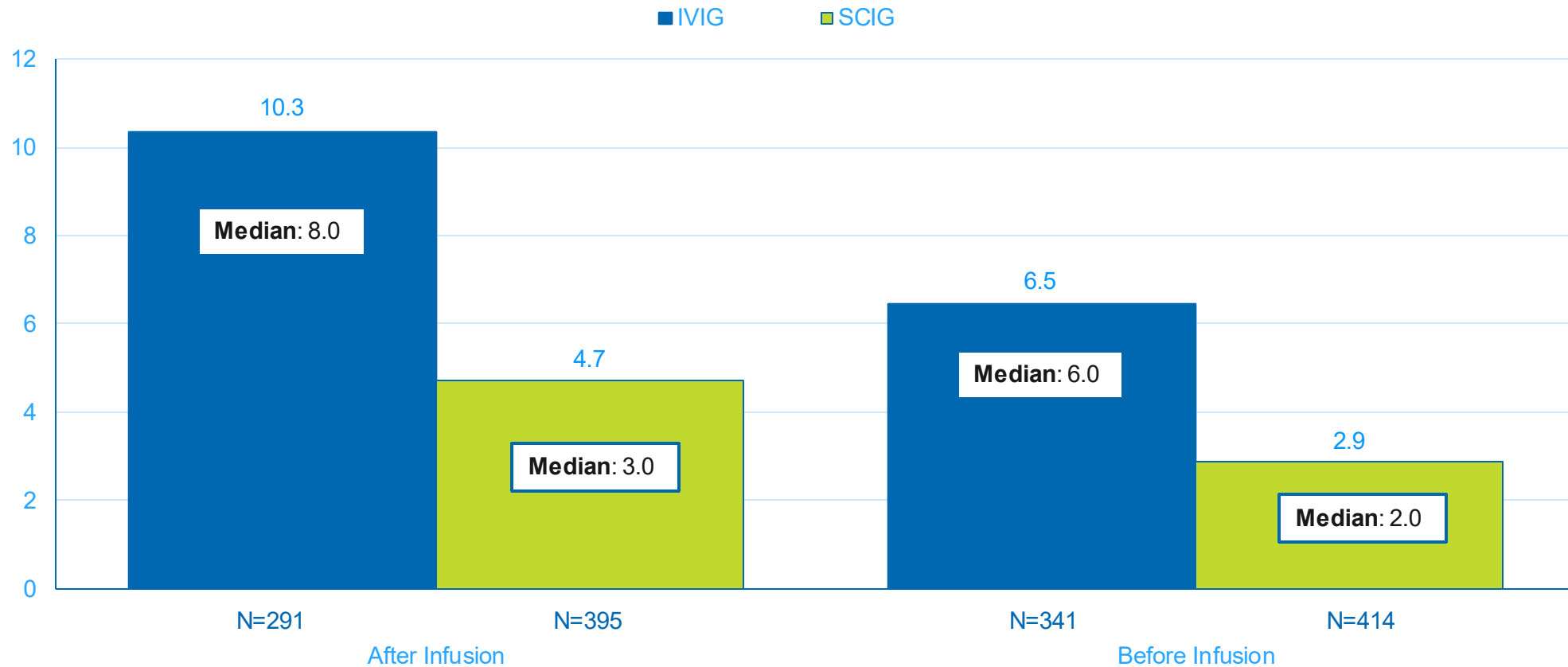
Q43a. Does the patient experience periods of fatigue or low energy between Ig therapy treatments (wear off)?
Base: Individuals who currently use IVIG or SCIG and experience a wear off effect

When Does Wear Off Effect Occur?*



Q43b. How long after infusion and how long before the next infusion does the patient feel this wear off?
Base: Individuals who currently use IMG or SCIG and experience wear off effect

Duration of Wear Off Effect*

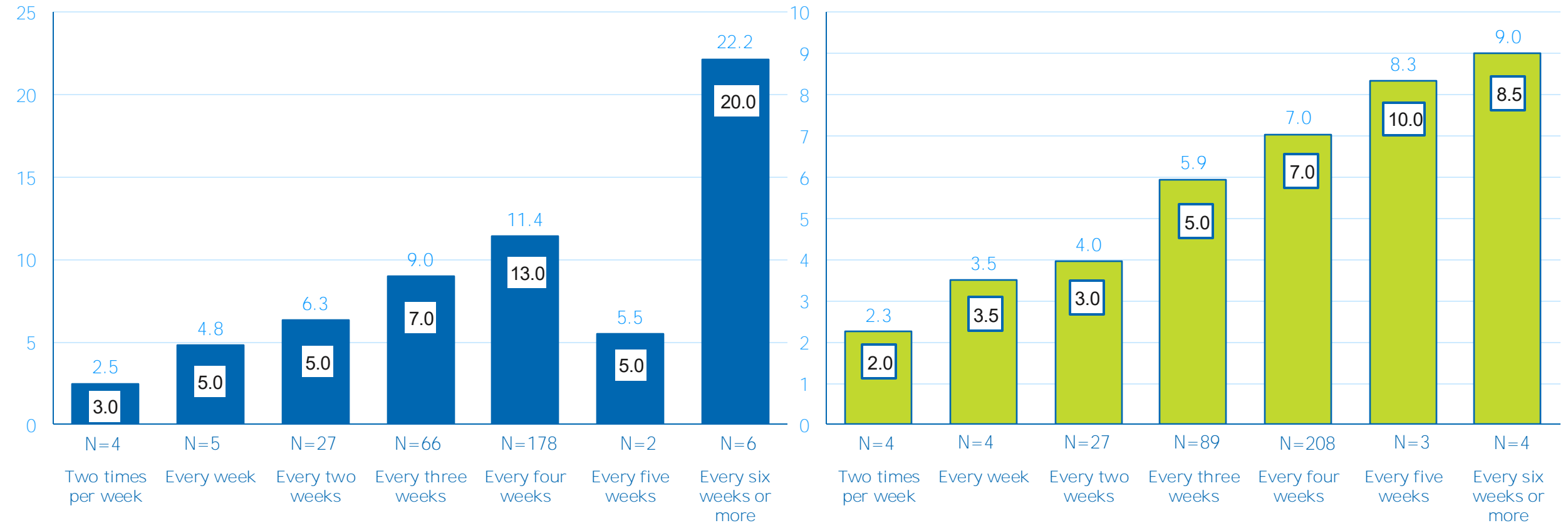


Q43b. How long after infusion and how long before the next infusion does the patient feel this wear off?
Base: Individuals who currently use IVIG or SCIG and experience wear off effect

IVI G: Wear Off Effect by Frequency of Ig Treatment

After*

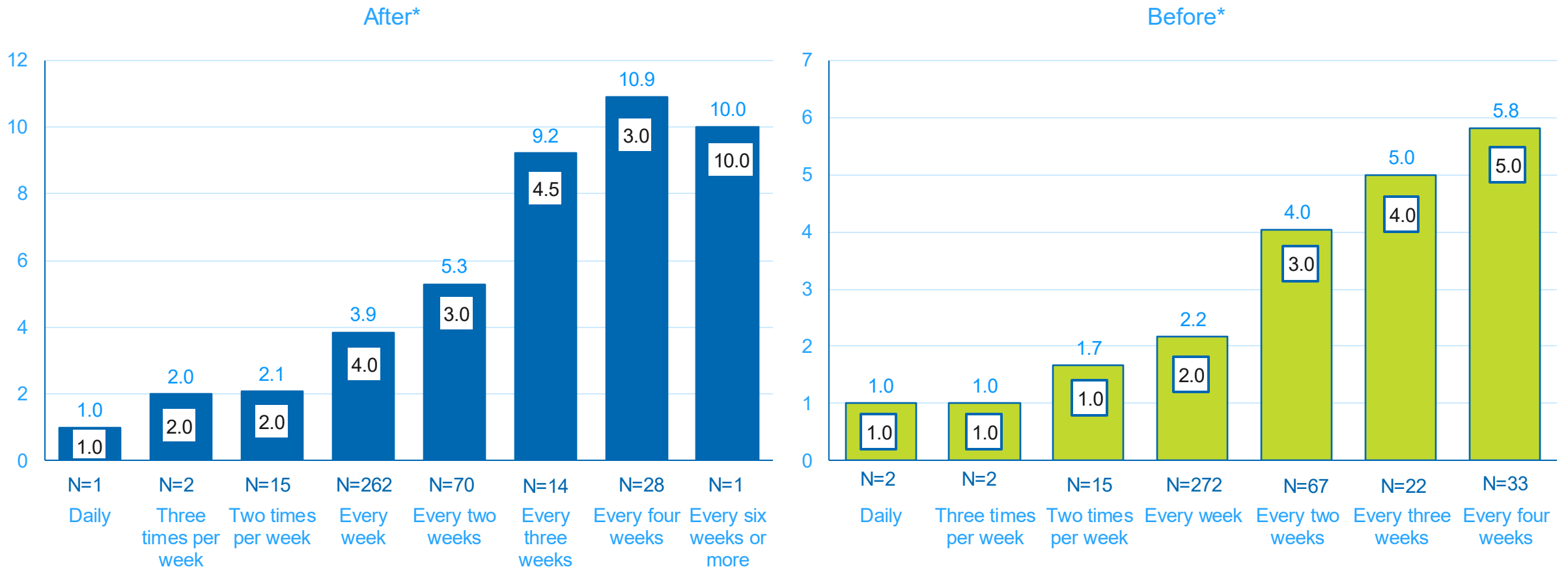
Before*



Median

Q30. On average, how often does he/she get their IVIG or SCIG therapy?
 Q43b. How long after infusion and how long before the next infusion does the patient feel this wear off?
 Base: Individuals who currently use IVIG or SCIG and experience wear off effect

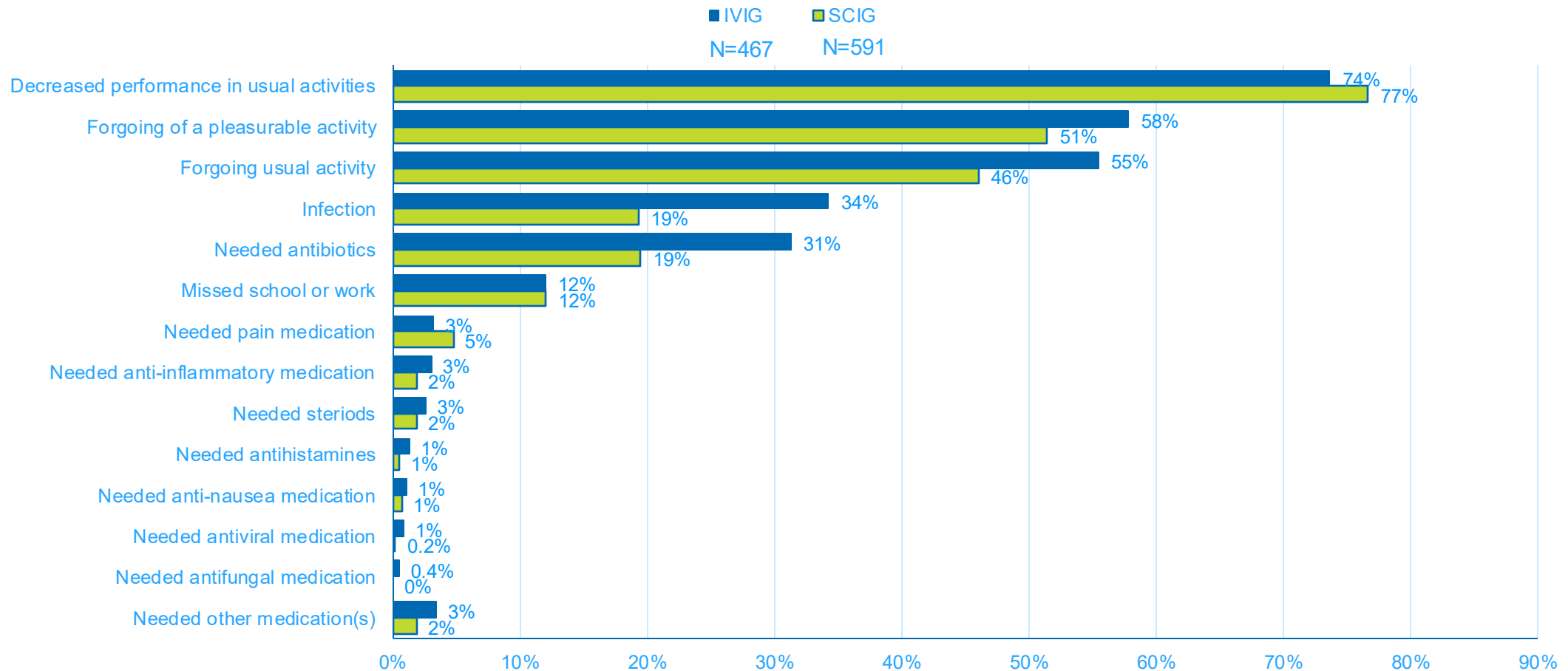
IVIg: Wear Off Effect by Frequency of Ig Treatment



Median

Q30. On average, how often does he/she get their IVIG or SCIG therapy?
 Q43b. How long after infusion and how long before the next infusion does the patient feel this wear off?
 Base: Individuals who currently use IVIG or SCIG and experience wear off effect

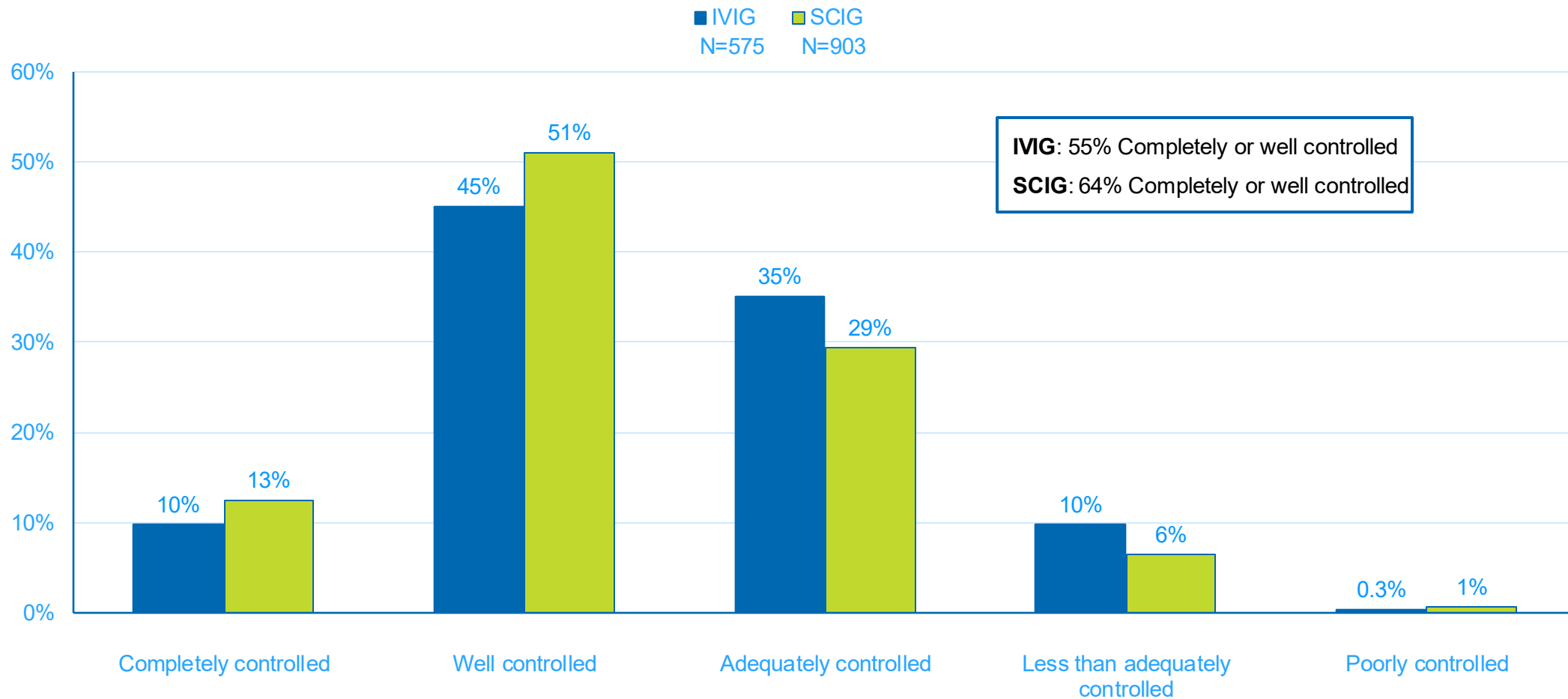
The Wear Off Effect Leads to... IVIG vs. SCIG



Q43c. Does the “wear off” result in any of the following? SELECT ALL THAT APPLY
Base: Current Ig therapy users who experienced wear off effect

How Well Does Immunoglobulin Control the Patient's Immunodeficiency?*

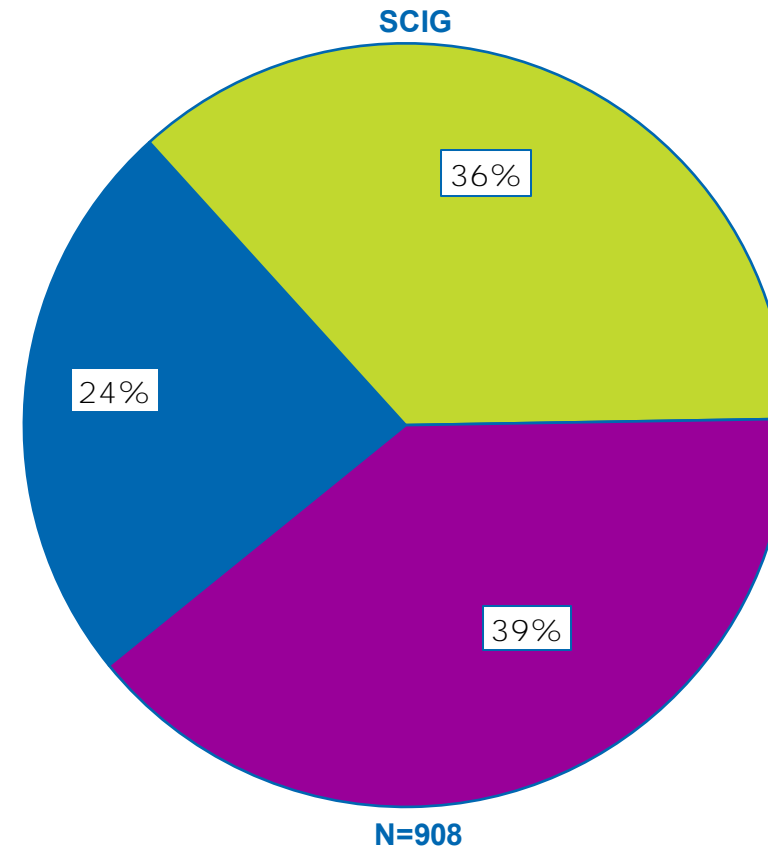
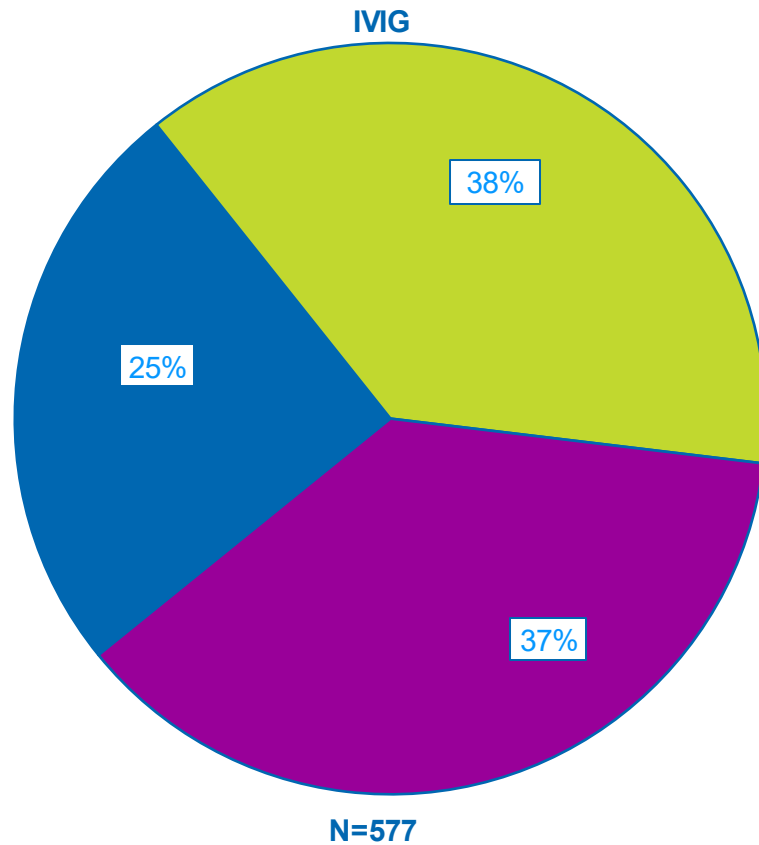
IVIG vs. SCIG



Q42. How well does immunoglobulin control the patient's immunodeficiency?
Base: Individuals who currently use IVIG or SCIG

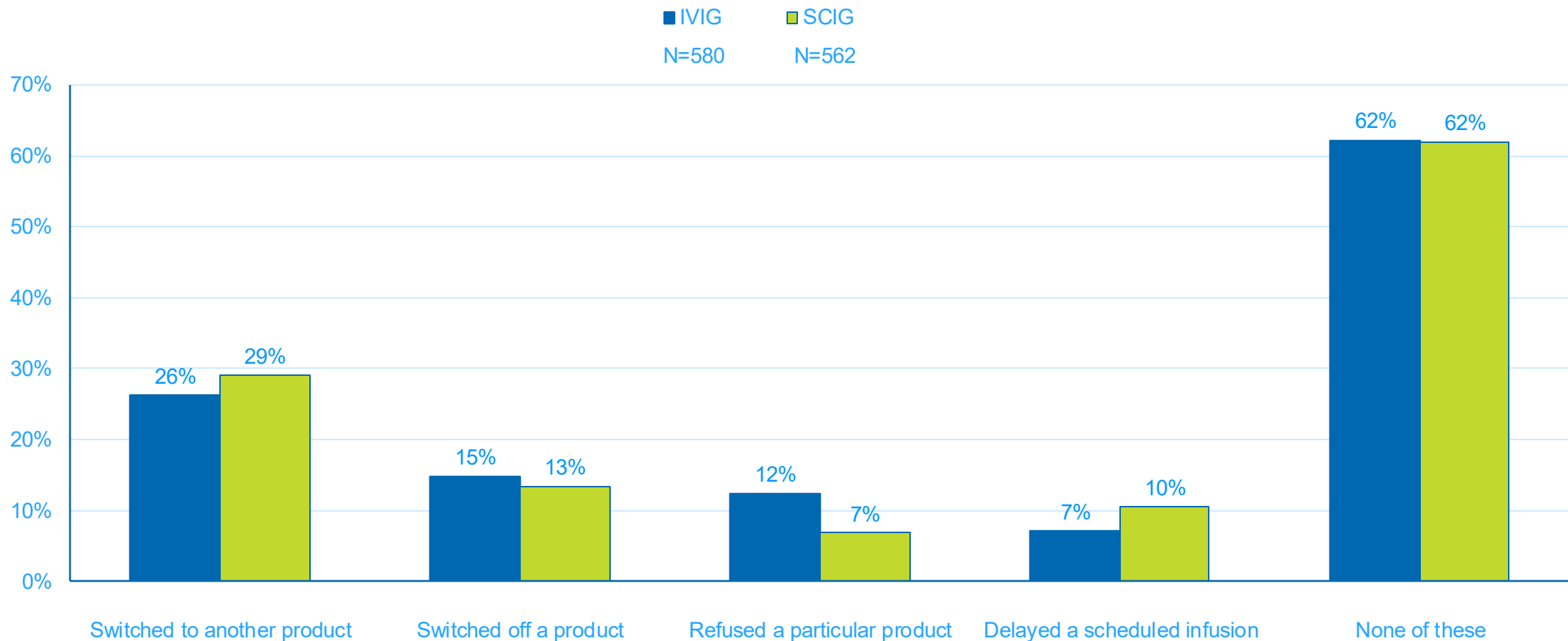
Does the Patient Tolerate Some Ig Products Better Than Others?

■ All about the same ■ Some better than others ■ Have only been on one product



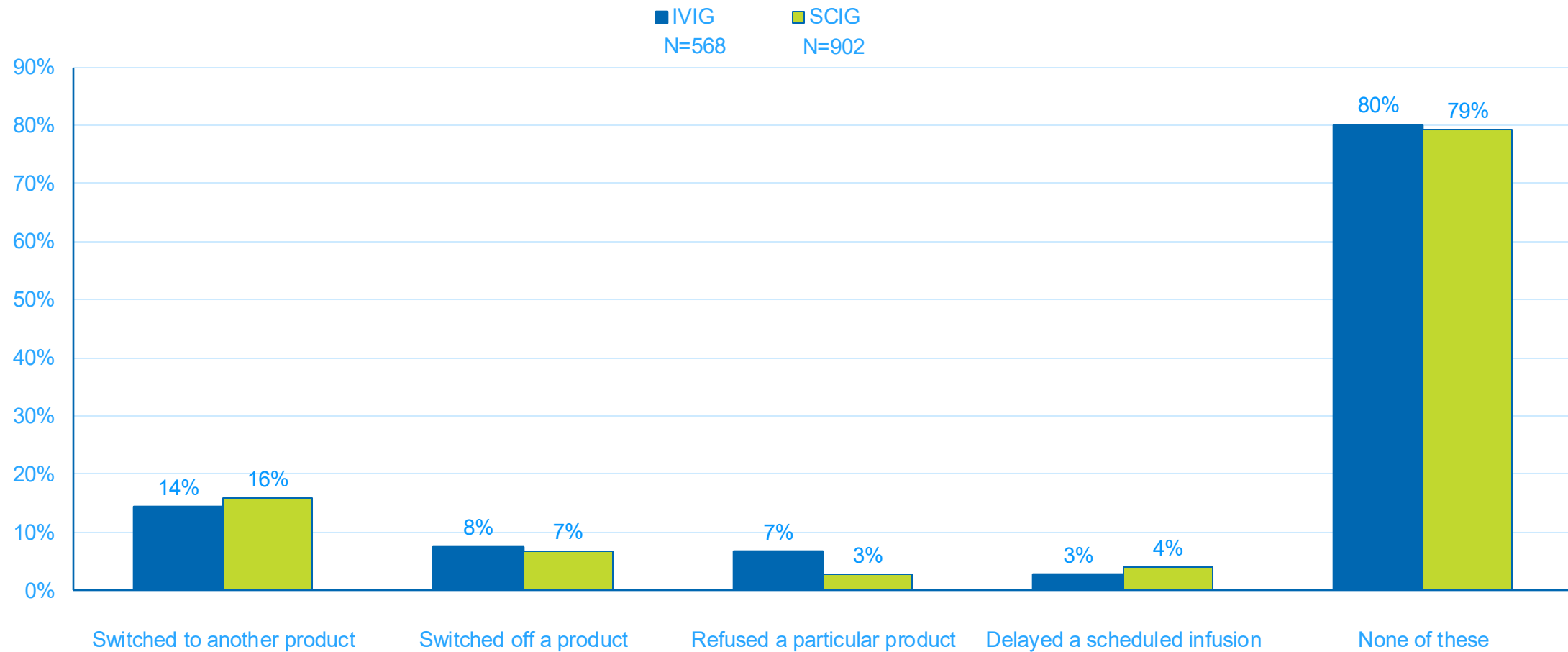
Q44. Does he/she tolerate any immunoglobulin products better than others, or are they all about the same?
Base: Individuals who currently use MIG or SCIG

Due to Tolerability Concerns, Patients...



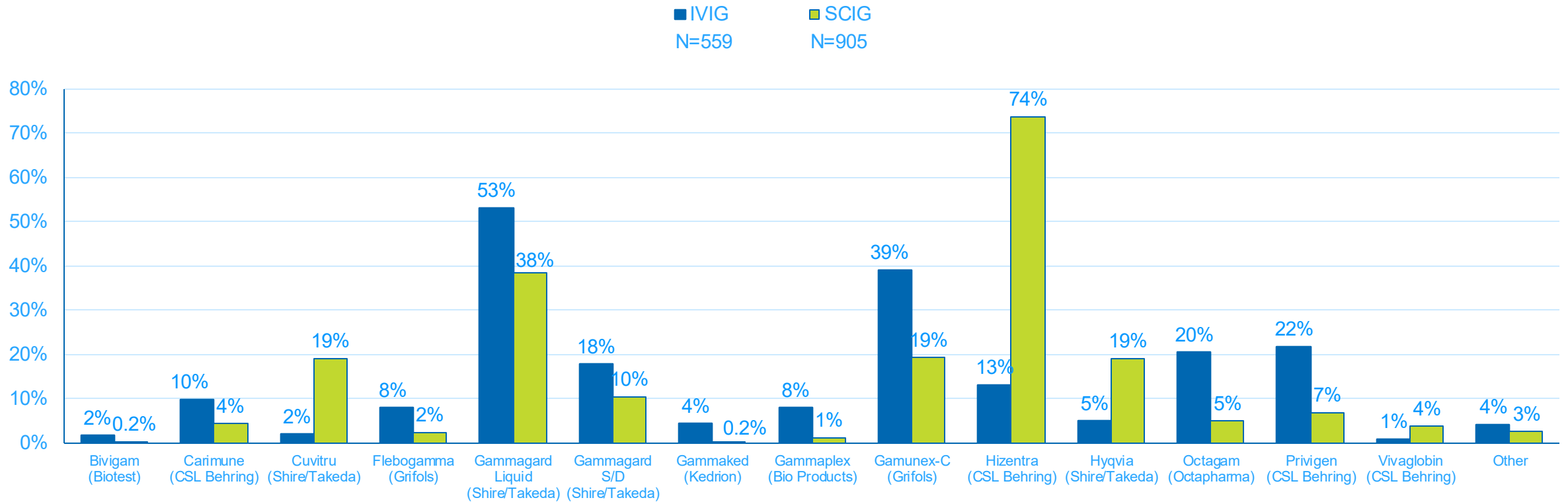
Q45. As a result of concerns about product tolerability has he/she ever ... SELECT ALL THAT APPLY
Base: Individuals who currently use MIG or SCIG

Due to Product Effectiveness, Patients...



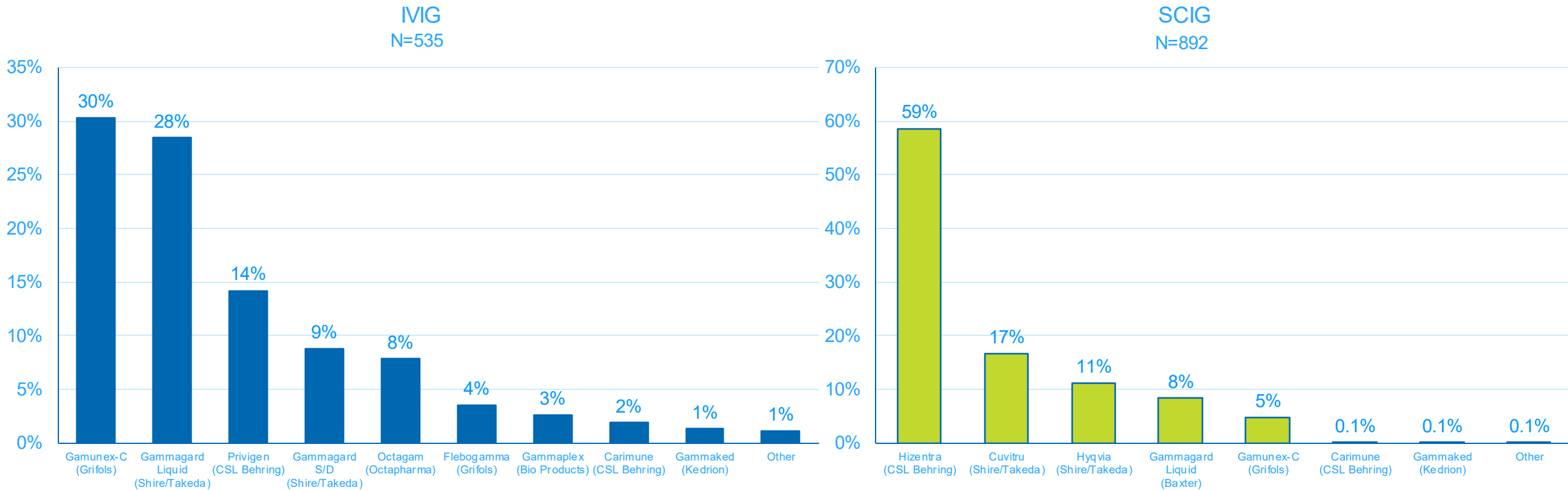
Q46. As a result of product effectiveness has he/she ever ... SELECT ALL THAT APPLY
Base: Individuals who currently use MIG or SCIG

Ig Products EVER Used IVIG vs. SCIG



Q47a. Which of the following Ig products has the patient ever used?
Base: Individuals who currently use IIG

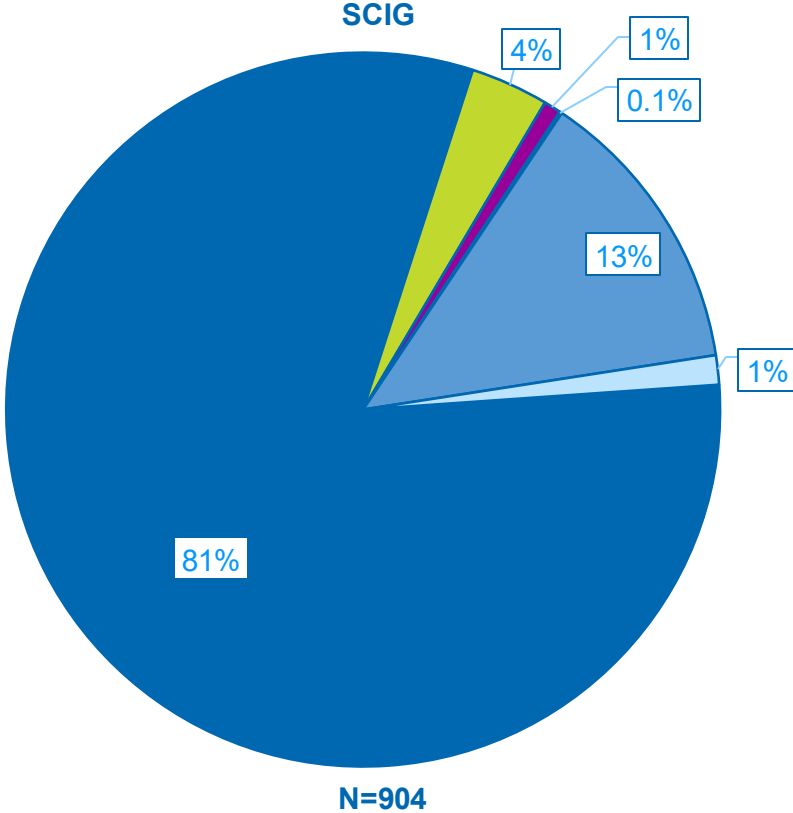
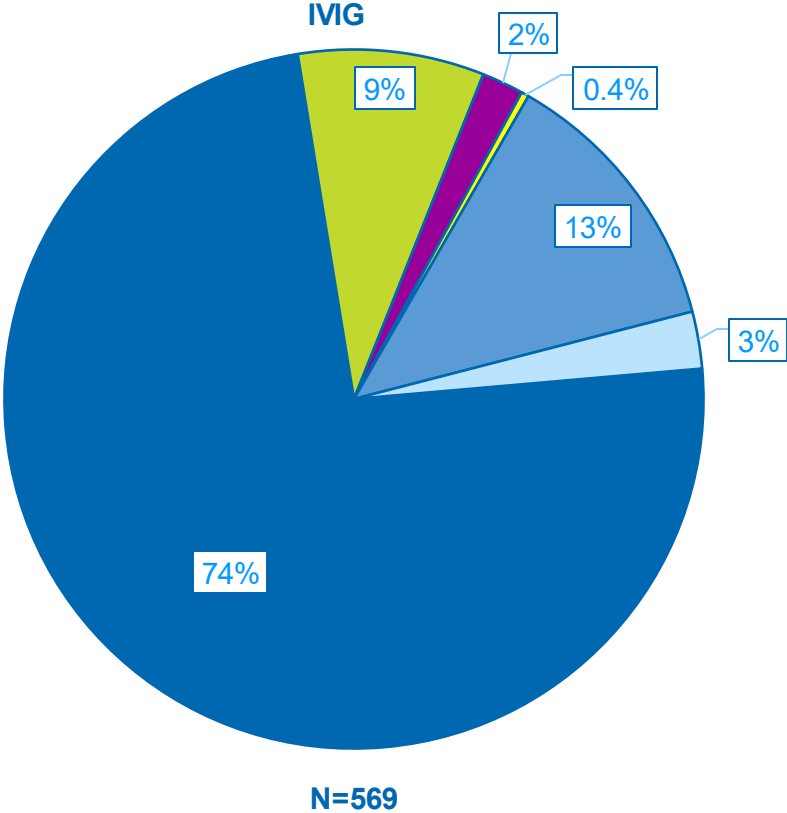
Ig Product Currently Used



Q47b. Which of the following Ig products do they currently use?
 Base: Individuals who currently use IVIG or SCIG

How Often Does the Patient Get Their Preferred Ig Product?*

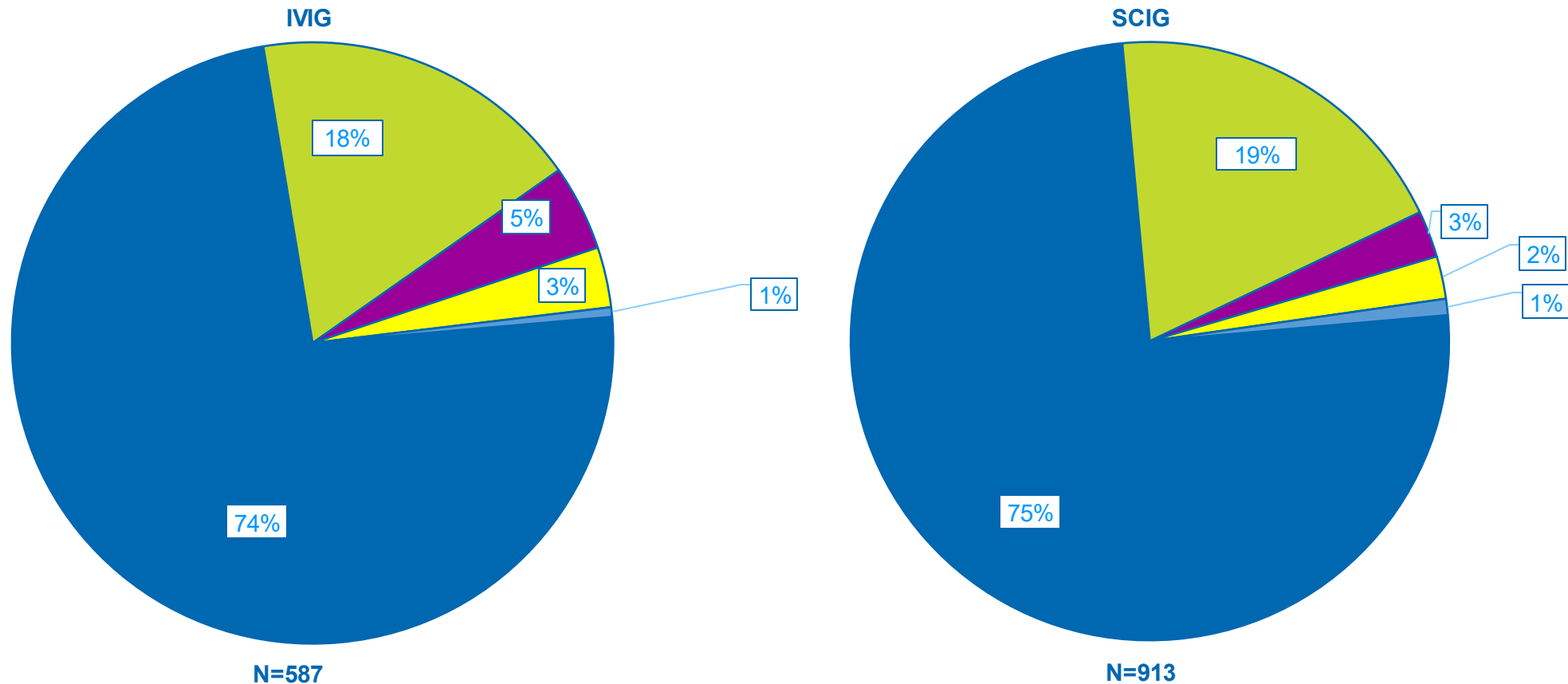
■ Always
 ■ Most of the time
 ■ Some of the time
 ■ Only occasionally
 ■ Only tried one product
 ■ Never



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

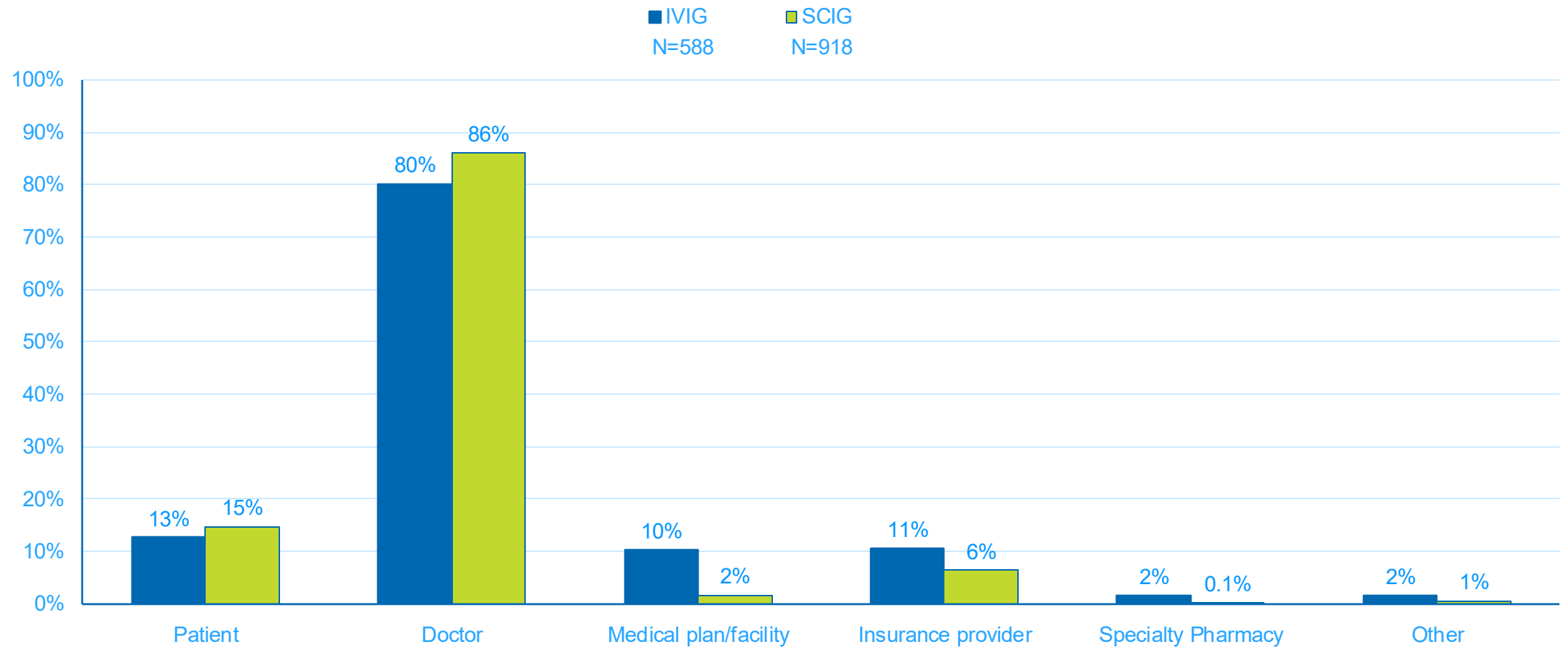
Satisfaction with Current Ig Product IVIG vs. SCIG

■ Very satisfied ■ Somewhat satisfied ■ Neither ■ Somewhat dissatisfied ■ Very dissatisfied



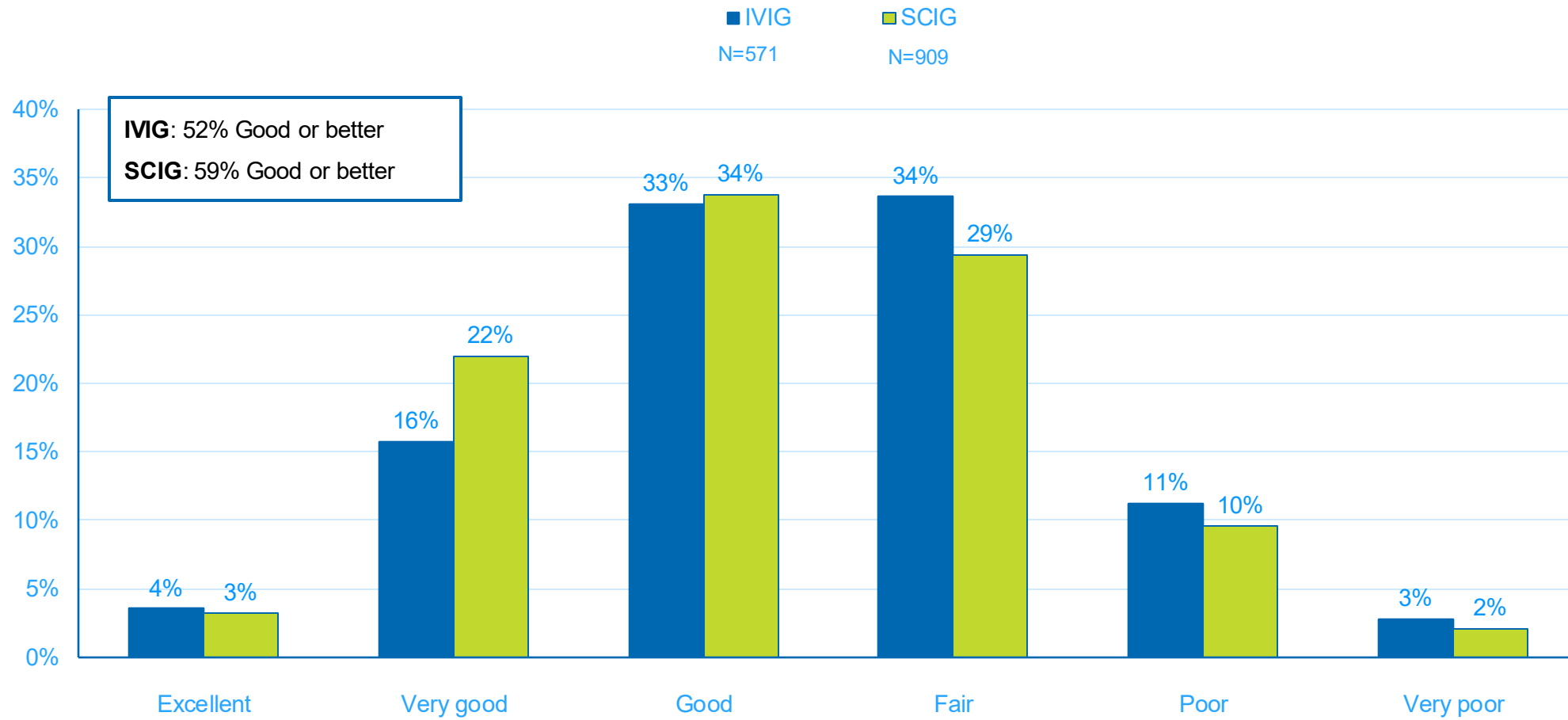
Q49. How satisfied is the patient with the Ig product currently being used?
Base: Individuals who currently use IVIG or SCIG

Who Selects the Patient's Ig Product?



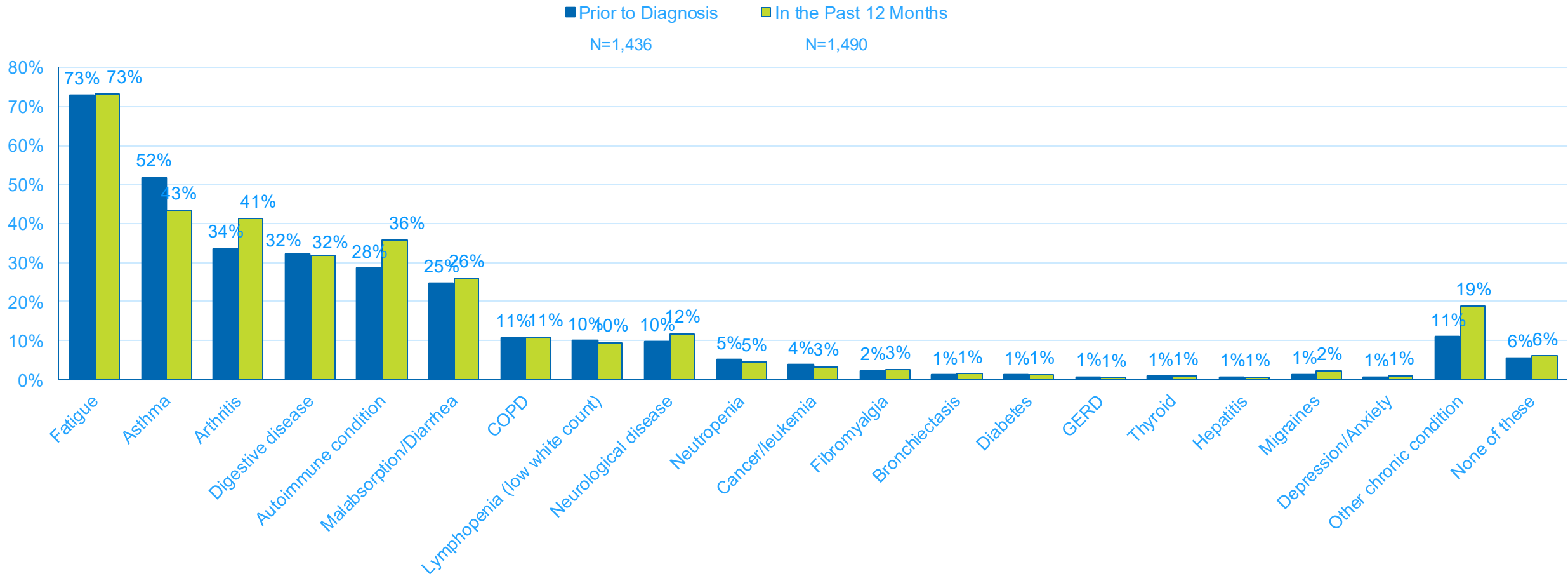
Q50. Who is primarily responsible for the selection of the Ig product that the patient uses?
Base: Individuals who currently use IVIG or SCIG

Currently: Patient's General Health IVIG vs. SCIG



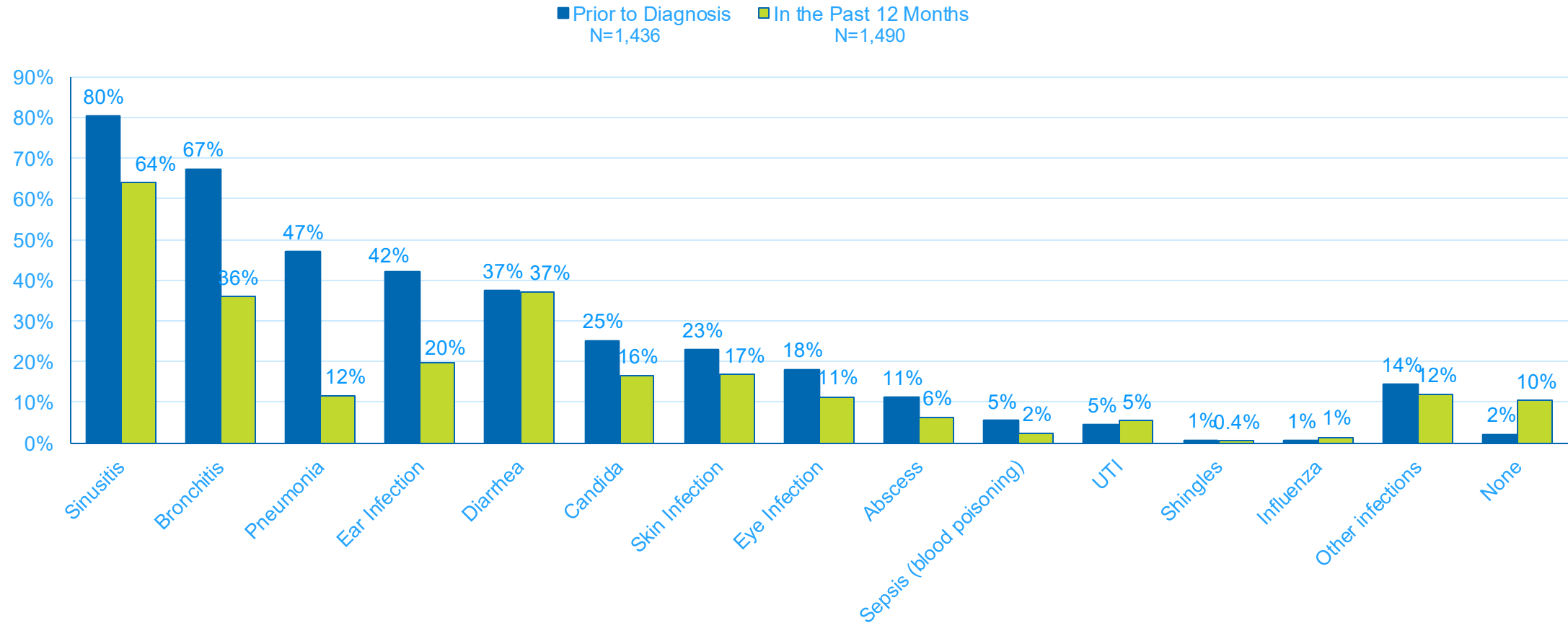
Q51. In general would you say the patient's health is...
Base: Individuals who currently use IVIG or SCIG

Other Chronic Conditions



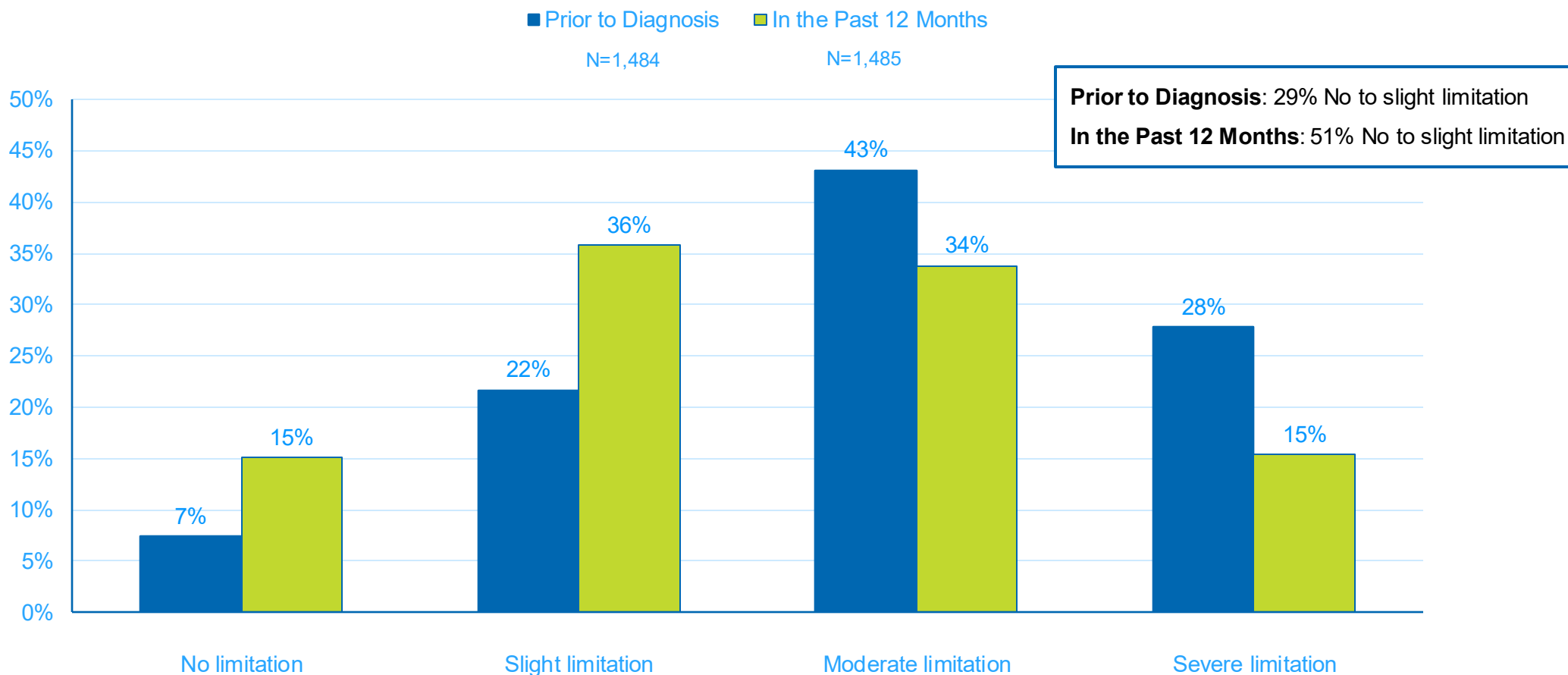
Q9. During the 12 months prior to diagnosis, did he/she have ... ? SELECT ALL THAT APPLY
 Q52. During the PAST 12 MONTHS, has he/she had...
 Base: Individuals who currently use MIG or SCIG

Infections



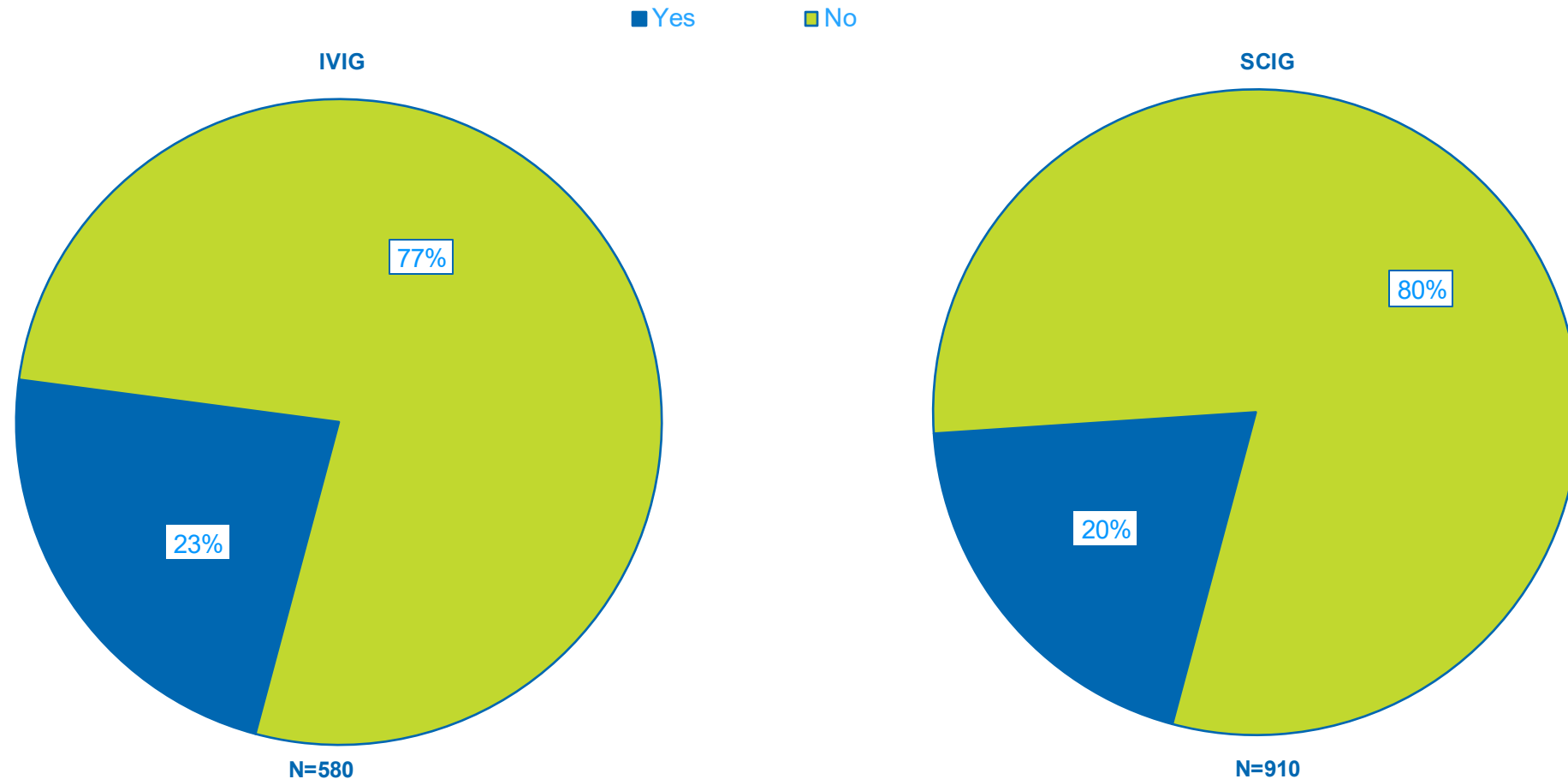
Q10. Did the patient experience any of the following infections during the 12 months prior to diagnosis?
 Q53. Did the patient experience any of the following infections during the past 12 months?
 Base: Individuals who currently use MIG or SCIG

Activity Limitation



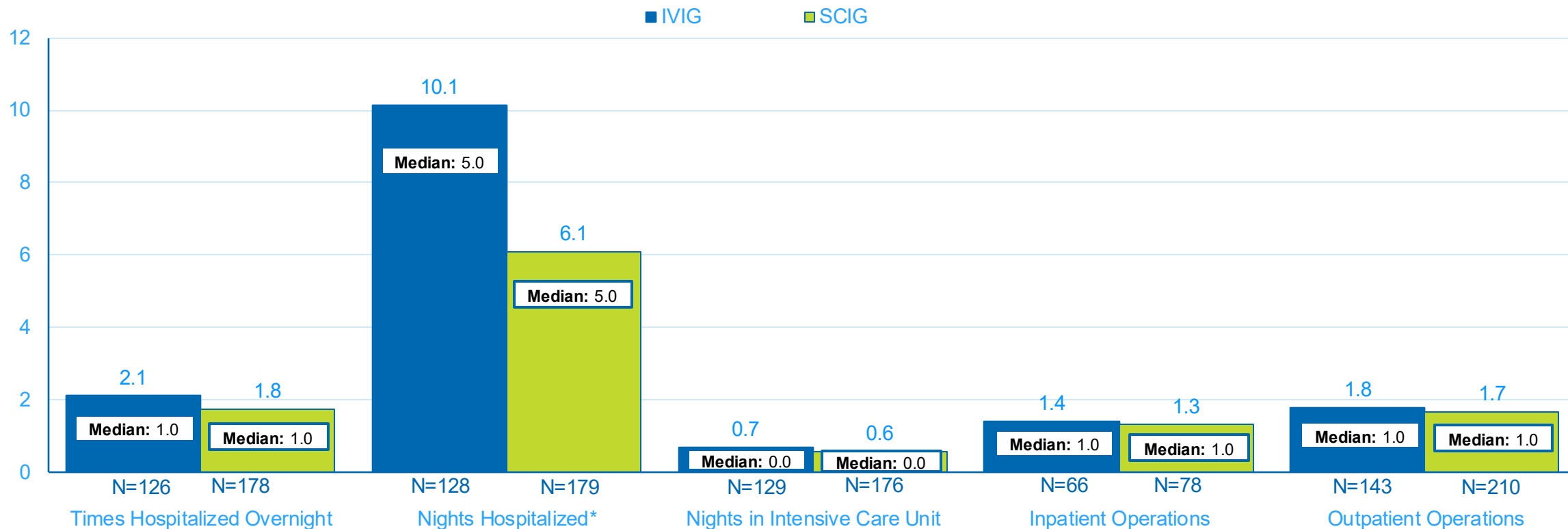
Q11. During the 12 months prior to diagnosis, how much was he/she limited in work, play or normal physical activity as a result of his/her health.
Q54. During the PAST 12 MONTHS, how much has he/she been limited in work, play or normal physical activity as a result of his/her health?
Base: Individuals who currently use MIG or SCIG

Hospitalized Overnight in Past 12 Months



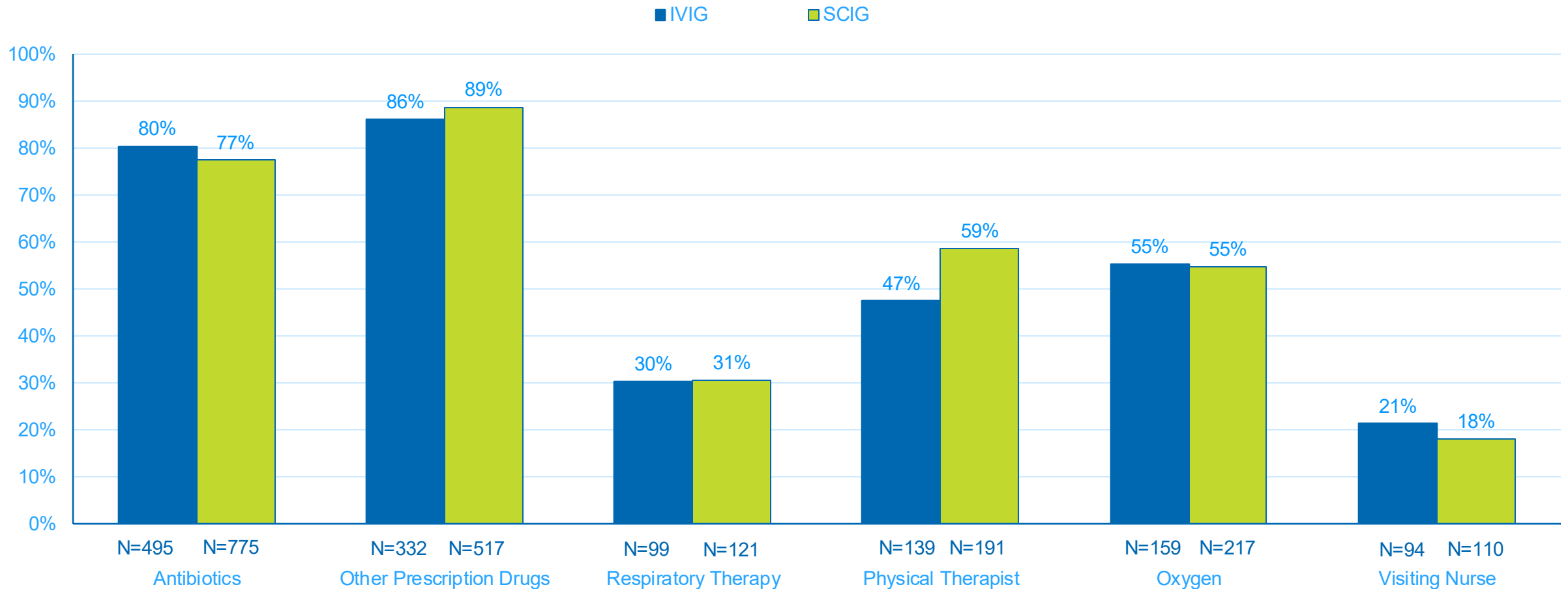
Q12a. Was he/she hospitalized overnight or longer for any reason during the 12 months prior to diagnosis?
Q55a. Has he/she been hospitalized overnight or longer for any reason during the past 12 months?
Base: Individuals who currently use IVIG or SCIG

Number of Hospitalizations and Operations in Past 12 Months



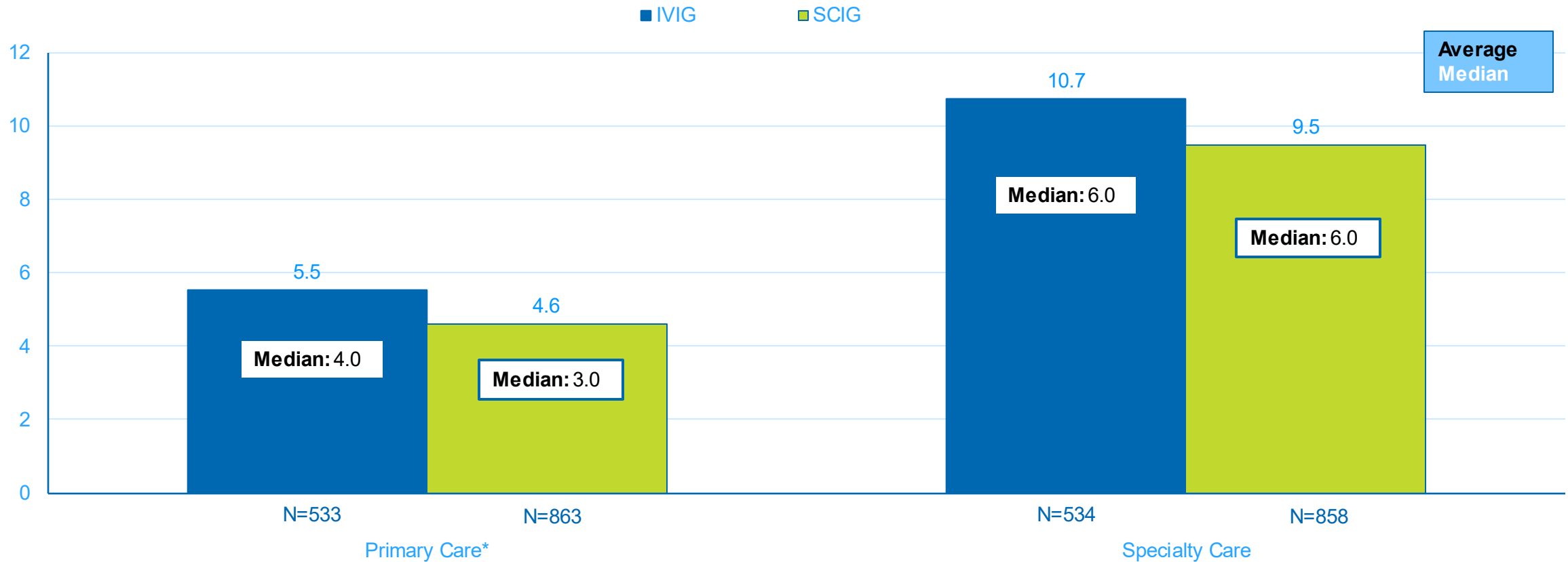
Q13b. How many TIMES was he/she hospitalized in the 12 months prior to diagnosis?
 Q13c. How many NIGHTS was he/she hospitalized in the 12 months prior to diagnosis?
 Q13d. How many NIGHTS, if any, was he/she in an INTENSIVE CARE UNIT in the 12 months prior to diagnosis?
 Q56b. How many TIMES was he/she hospitalized in the past 12 months?
 Q56c. How many NIGHTS was he/she hospitalized in the past 12 months?
 Q56d. How many NIGHTS, if any, was he/she in an INTENSIVE CARE UNIT in the past 12 months?
 Base: Individuals who currently use IVIG or SCIG who experienced an overnight hospitalization
 Q14. How many operations, if any, did he/she have in the 12 months prior to diagnosis?
 Q57. How many operations, if any, did he/she have in the past 12 months?
 Base: Individuals who currently use IVIG or SCIG

Used for More Than One Week... In the Past 12 Months



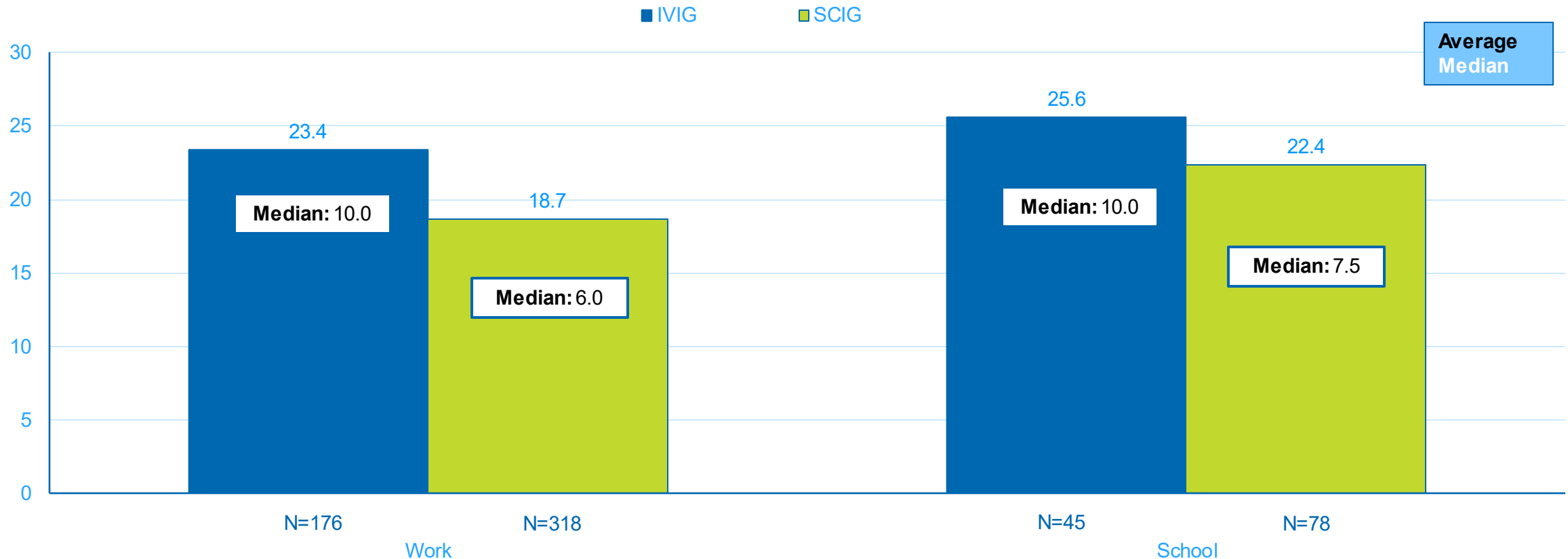
Q15. In the 12 months prior to diagnosis, approximately how many days did he/she use.....
Q58. Approximately how many days in the past 12 months did he/she use.....
Base: Individuals who currently use MIG or SCIG

Doctor Visits in Past 12 Months



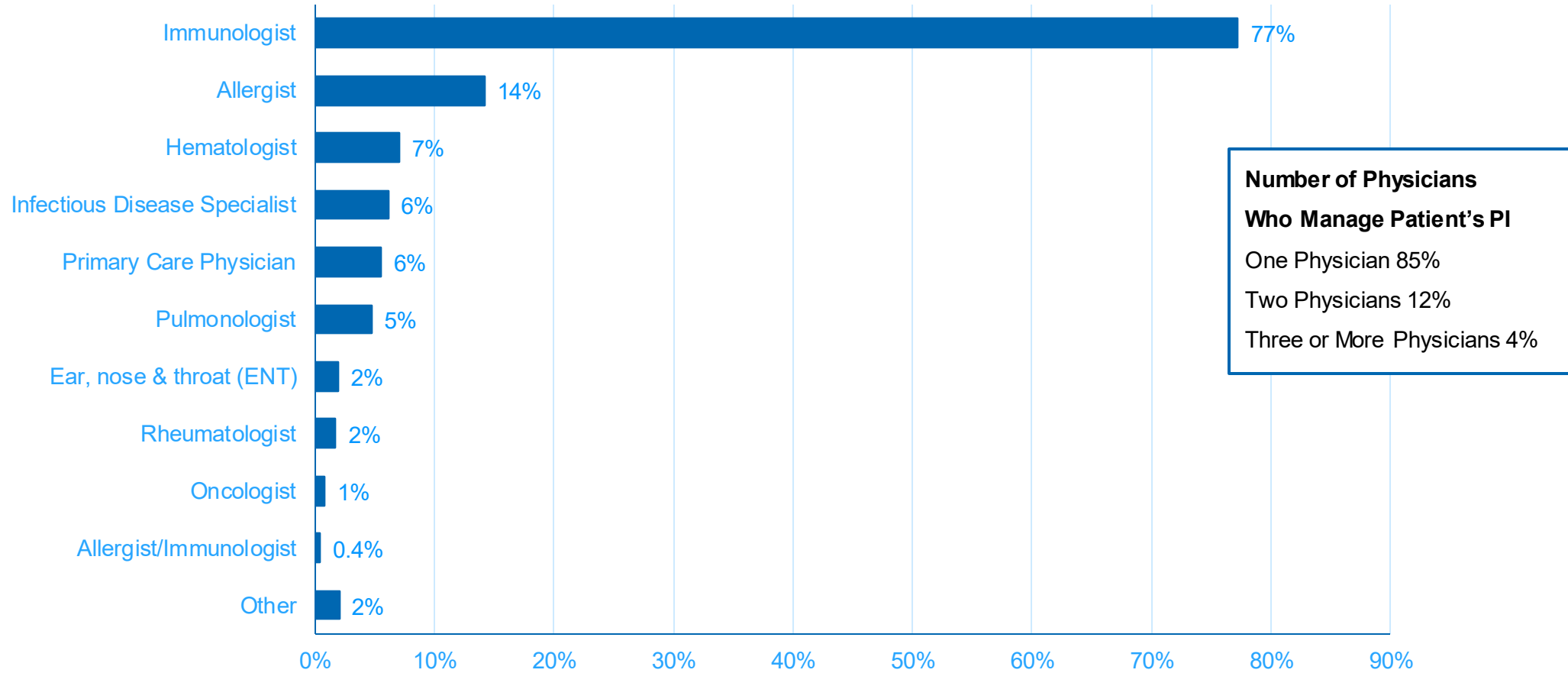
Q58. Not counting hospitalizations, about how many DOCTOR VISITS did the patient make during the past 12 months?
Base: Individuals who currently use IVIG or SCIG

Missed Days from Work or School in Past 12 Months



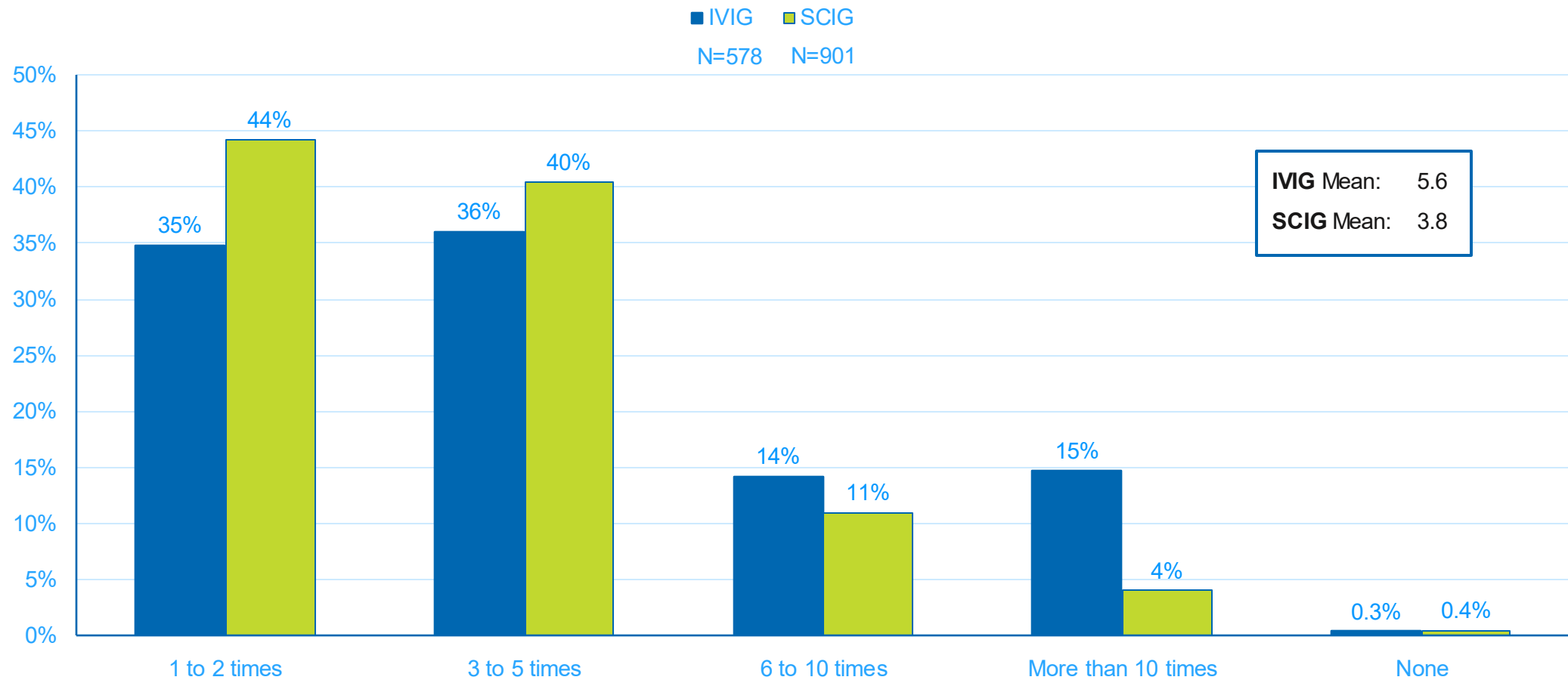
Q59. Not counting hospitalizations, how many days was the patient too sick to work, go to school or perform usual activities in the past 12 months?
Base: Individuals who currently use MIG or SCIG
Note: Patients with PI and Parents/Caregivers who are too disabled to work or retired were excluded

PI Physician



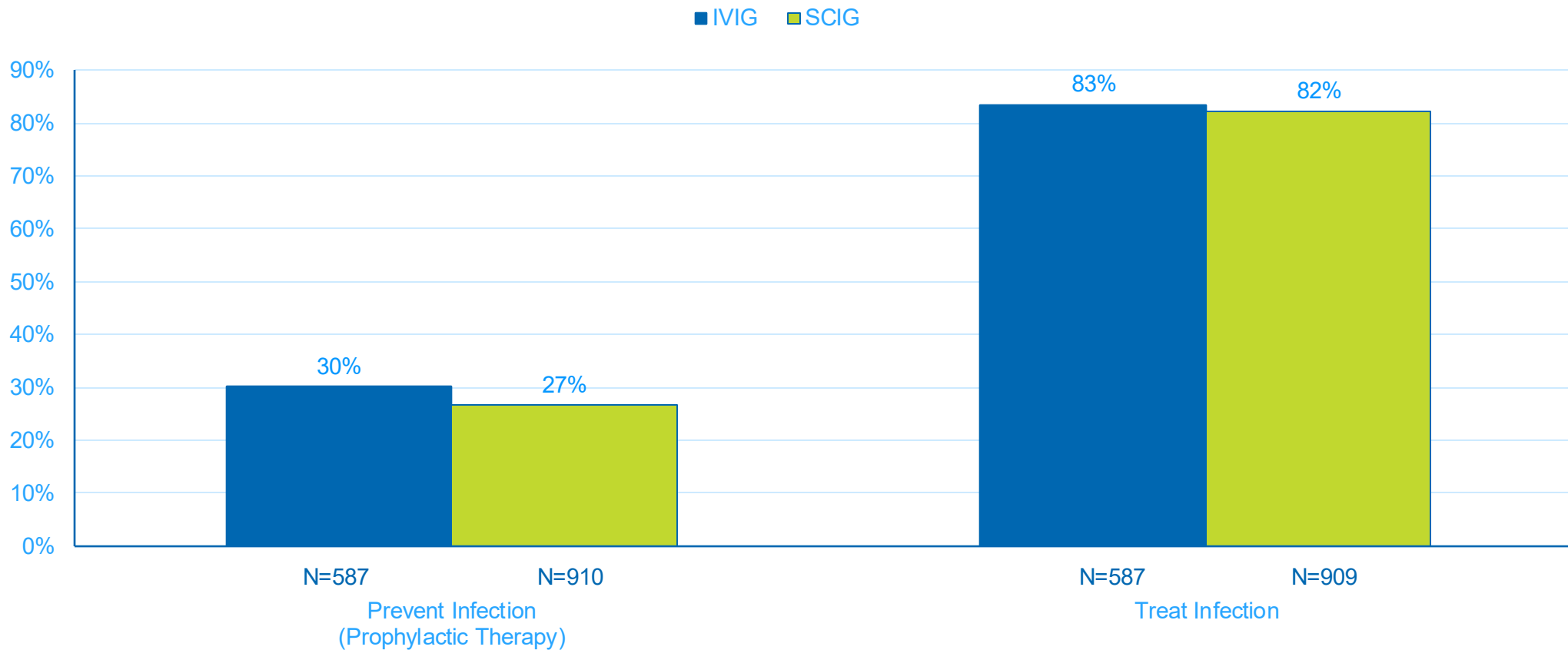
Q17a. Which type of physician is responsible for the treatment and management of the patient's PI? N=1,507
Base: Individuals who currently use MIG or SCIG

Number of Times Physician Was Seen in Past 12 Months* IVIG vs. SCIG



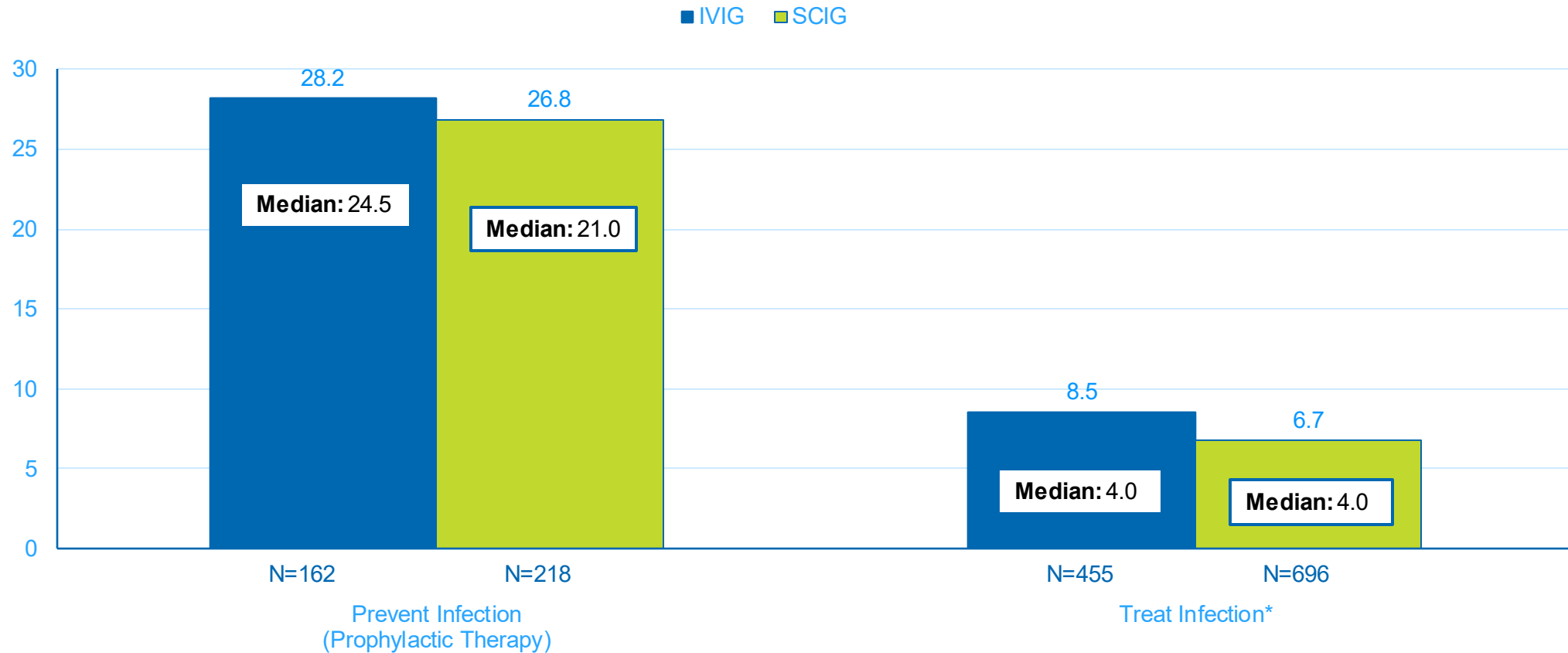
Q17b. How many times over the past 12 months has the patient seen this physician?
Base: Individuals who currently use IVIG or SCIG

Antibiotic Usage IVIG vs. SCIG



Q60a. In the past 12 months, has the patient taken antibiotics on a regular basis (every day or at least once a week) to prevent infections?
Q61a. In the past 12 months, has the patient taken antibiotics for an active infection?
Base: All respondents currently receiving MIG or SCIG

Duration of Antibiotic Treatment IVIg vs. SCIG



Q60b. How many weeks in the past 12 months did the patient take antibiotics to prevent infections?
Q61b. How many weeks in the past 12 months did the patient take antibiotics for an active infection?
Base: All respondents currently receiving IVIg or SCIG and who either received prophylactic antibiotic therapy or received an antibiotic to treat an active infection

Antibiotic Medications Prescribed to Prevent Infection

IVIG vs. SCIG

| | IVIG N=172 | SCIG N=238 |
|---|---------------|---------------|
| Azithromycin | 37% | 39% |
| Trimethoprim/sulfamethoxazole (Bactrim) | 30% | 23% |
| Amoxicillin/clavulanate (Augmentin) | 22% | 22% |
| Amoxicillin | 21% | 20% |
| Ciprofloxacin (Cipro) | 19% | 16% |
| Clarithromycin (Biaxin) | 12% | 12% |
| Doxycycline | 10% | 11% |
| Levofloxacin (Levaquin) | 3% | 8% |
| Macrobid | 2% | 3% |
| Other Antibiotic | 28% | 17% |

Q60d. What is the name of the antibiotic(s) the patient took to prevent infections? SELECT ALL THAT APPLY
Base: All respondents who take antibiotics to prevent infection

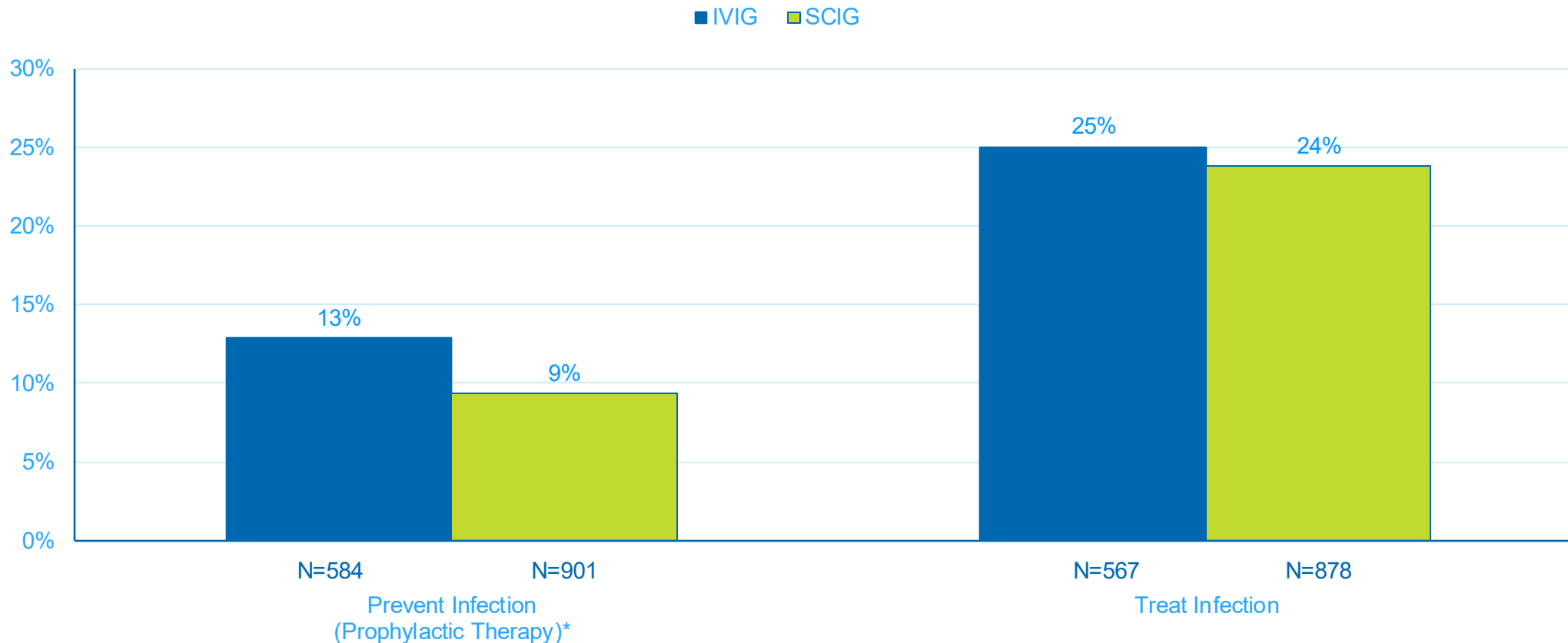
Antibiotic Medications Prescribed to Treat Infection

IVIG vs. SCIG

| | IVIG N=479 | SCIG N=735 |
|---|---------------|---------------|
| Amoxicillin/clavulanate (Augmentin) | 39% | 40% |
| Azithromycin | 36% | 31% |
| Ciprofloxacin (Cipro) | 30% | 29% |
| Amoxicillin | 25% | 25% |
| Trimethoprim/sulfamethoxazole (Bactrim) | 15% | 16% |
| Clarithromycin (Biaxin) | 12% | 13% |
| Levofloxacin (Levaquin) | 10% | 13% |
| Doxycycline | 7% | 6% |
| Cefdinir (Omnicef) | 4% | 4% |
| Cephalexin (Keflex) | 4% | 4% |
| Clindamycin | 4% | 3% |
| Moxifloxacin | 2% | 2% |
| Macrobid | 2% | 1% |
| Rocephin | 2% | 1% |
| Vancomycin | 2% | 1% |
| Rifaximin | 2% | 0% |
| Ceftin | 1% | 2% |
| Flagyl | 1% | 1% |
| Other Antibiotic | 13% | 9% |

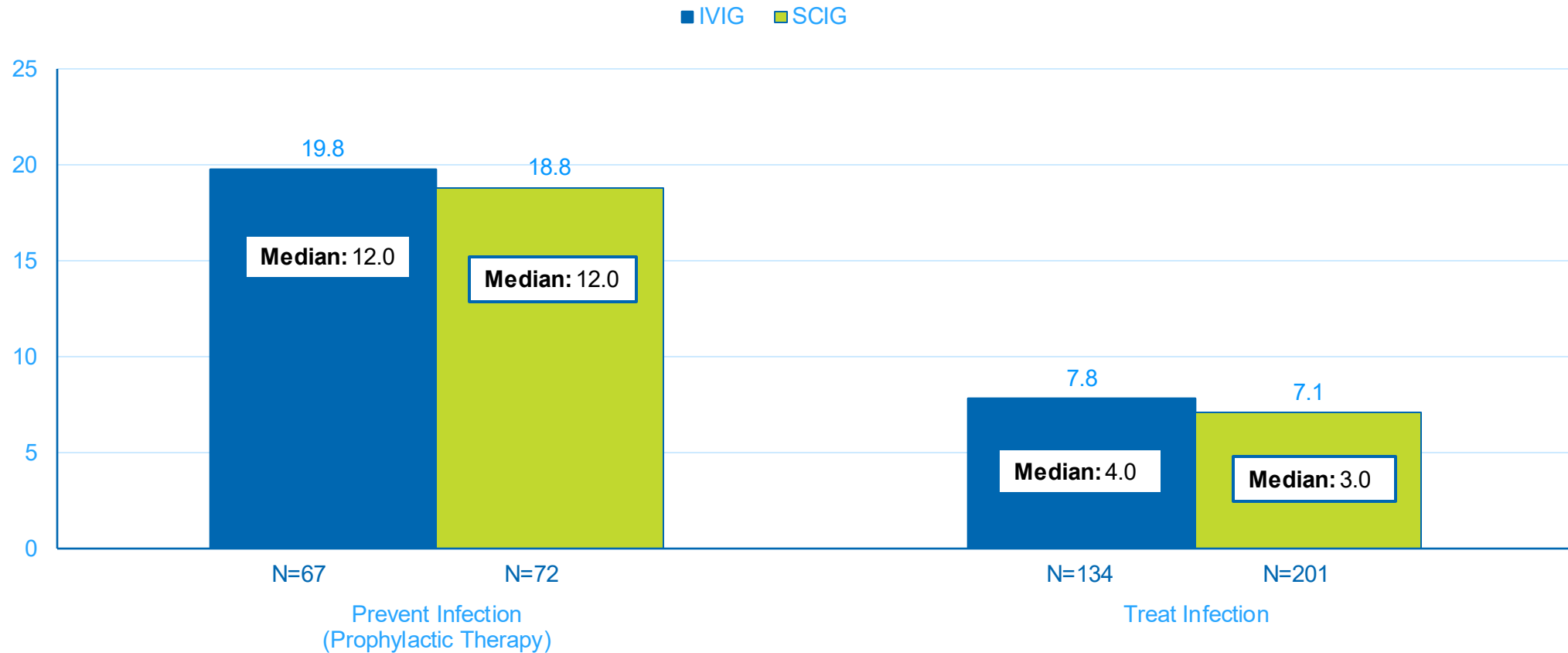
Q61c. What is the name of the antibiotic(s) the patient took to treat an active infection? SELECT ALL THAT APPLY
 Base: All respondents who take antibiotics to treat an active infection

Antifungal Medication Usage IVIIG vs. SCIG



Q62a. In the past 12 months, has the patient taken antifungal medications on a regular basis (every day or at least once a week) to prevent infections?
Q63a. In the past 12 months, has the patient taken antifungal medications for an active infection?
Base: All respondents currently receiving IVIG or SCIG

Duration of Antibiotic Treatment IVIG vs. SCIG



Q62b. How many weeks in the past 12 months did the patient take antifungal medications to prevent infections?
Q63b. How many weeks in the past 12 months did the patient take antifungal medications for an active infection?
Base: All respondents currently receiving IVIG or SCIG and who either received prophylactic antifungal therapy or received an antifungal medication to treat an active infection

Antifungal Medications Prescribed to Prevent Infection

IVIG vs. SCIG

| | IVIG N=70 | SCIG N=75 |
|------------------|--------------|--------------|
| Fluconazole | 81% | 77% |
| Nystatin | 10% | 11% |
| Itraconazole | 9% | 9% |
| Posaconazole | 1% | 0% |
| Other Antifungal | 19% | 19% |

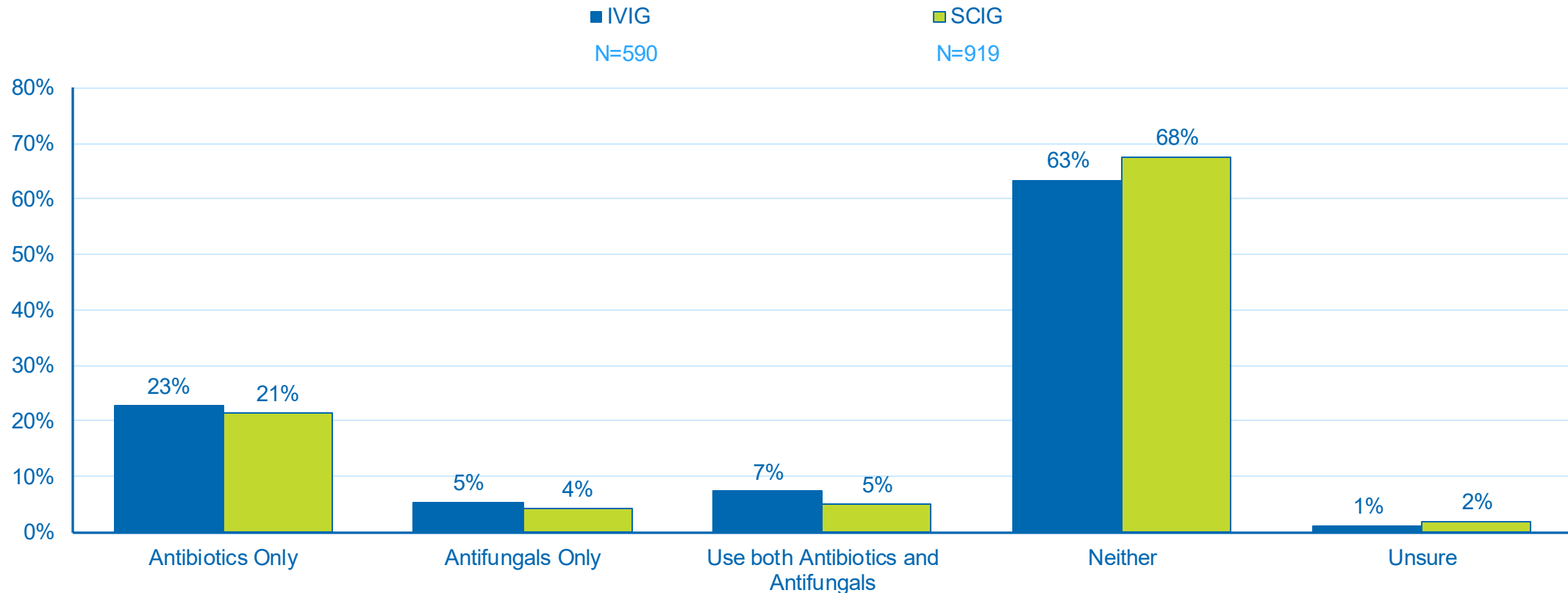
Q62d. What is the name of the antifungal medication(s) the patient took to prevent infections?
Base: All respondents who take antibiotics to prevent infection

Antifungal Medications Prescribed to Treat Infection IVIG vs. SCIG

| | IVIG N=134 | SCIG N=201 |
|------------------|---------------|---------------|
| Fluconazole | 87% | 80% |
| Nystatin | 9% | 8% |
| Itraconazole | 8% | 5% |
| Posaconazole | 1% | 0% |
| Other Antifungal | 13% | 19% |

Q63c. What is the name of the antifungal medication(s) the patient took to treat an active infection?
Base: All respondents who take antibiotics to prevent infection

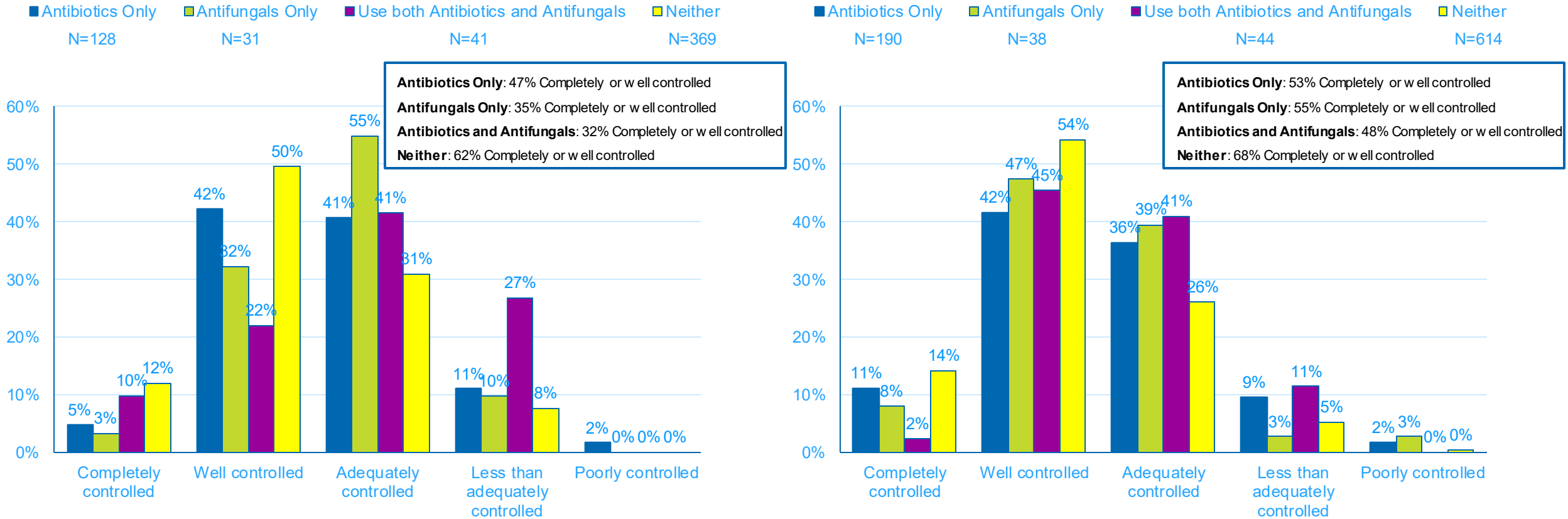
Prophylactic Antibiotic Therapy and Prophylactic Antifungal Therapy: IVIG vs. SCIG



Q60a. In the past 12 months, has the patient taken antibiotics on a regular basis (every day or at least once a week) to prevent infections?
Q62a. In the past 12 months, has the patient taken antifungal medications on a regular basis (every day or at least once a week) to prevent infections?
Base: All respondents currently receiving IVIG or SCIG and those not currently receiving any Ig replacement therapy

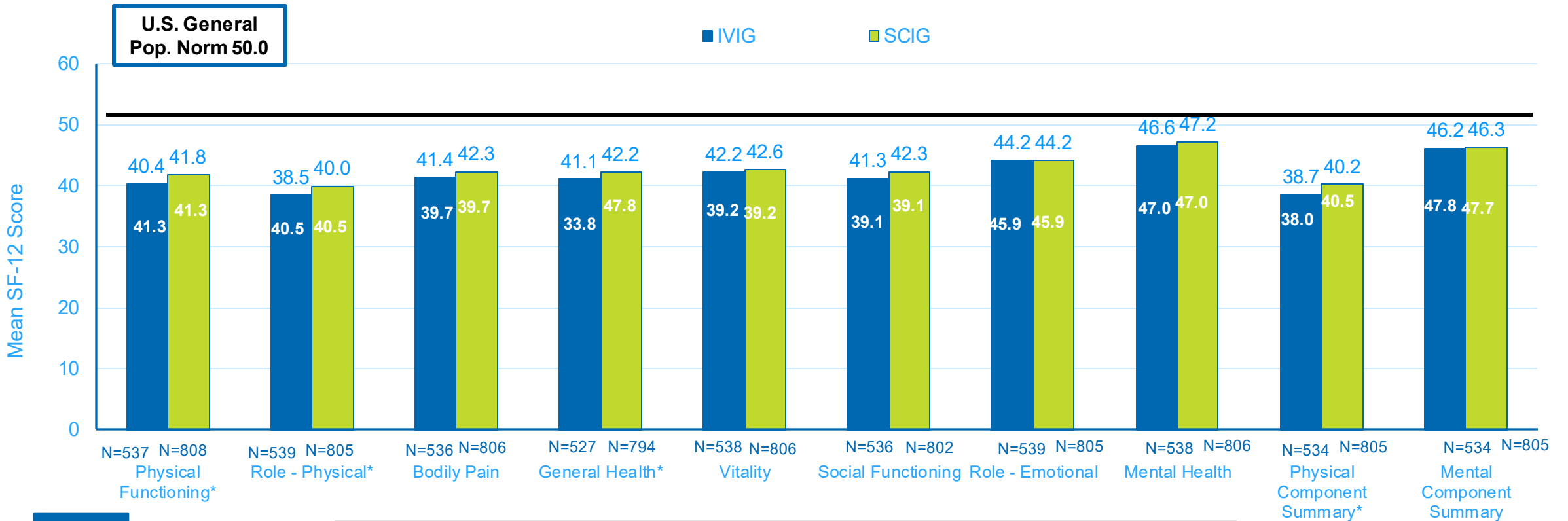
Prophylactic Antibiotic Therapy and Ig Therapy Effectiveness*

IVIG vs. SCIG



Q42. How well does immunoglobulin control the patient's immunodeficiency?
 Q61. In the past 12 months, how long has the patient taken antibiotics to prevent infections (prophylactically)?
 Base: Individuals who currently use IVIG or SCIG

SF-12



U.S. General Pop. Norm 50.0

■ IVIG ■ SCIG

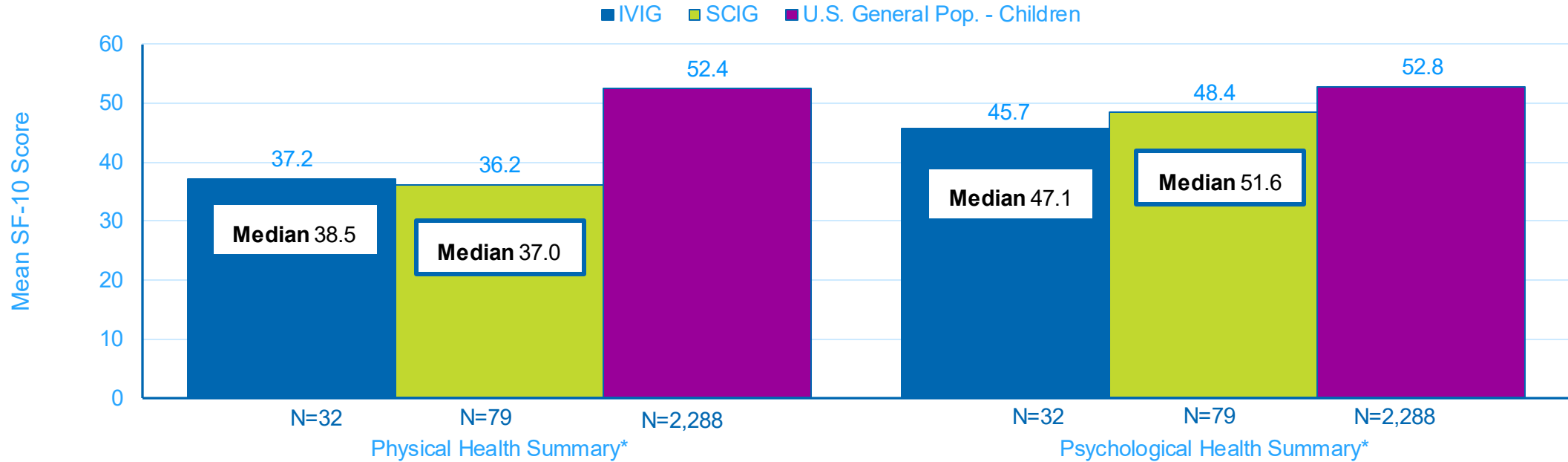
Mean SF-12 Score

Median

GH01. In general, would you say your health is...
 PF02. Moderate activities, such as moving a table, pushing a vacuum cleaner, bowling, or playing golf
 PF04. Climbing several flights of stairs
 RP02. Accomplished less than you would like
 RP03. Were limited in the kind of work or other activities
 RE02. Accomplished less than you would like
 Base: Adults who currently use IVIG or SCIG

RE03. Did work or other activities less carefully than usual
 BP02. How much did pain interfere with your normal work?
 MH03. Have you felt calm and peaceful?
 VT02. Did you have a lot of energy?
 MH04. Have you felt downhearted and depressed?
 SF02. How much of the time has your physical health or emotional problems interfered with your social activities?

SF-10



GHGLOBAL. In general, would you say your child's health is...

PFSOME. Doing things that take some energy such as riding a bike or skating?

PFBEND. Bending, lifting, or stooping?

RPKIND. Has your child been limited in the KIND of schoolwork or activities with friends he/she could do because of PHYSICAL health problems?

REBKIND. Has your child been limited in the KIND of schoolwork or activities with friends he/she could do because of EMOTIONAL or BEHAVIORAL problems?

BPGLOBAL. How much bodily pain or discomfort has your child had?

SEFRIEND. How satisfied do you think your child has felt about his/her friendships?

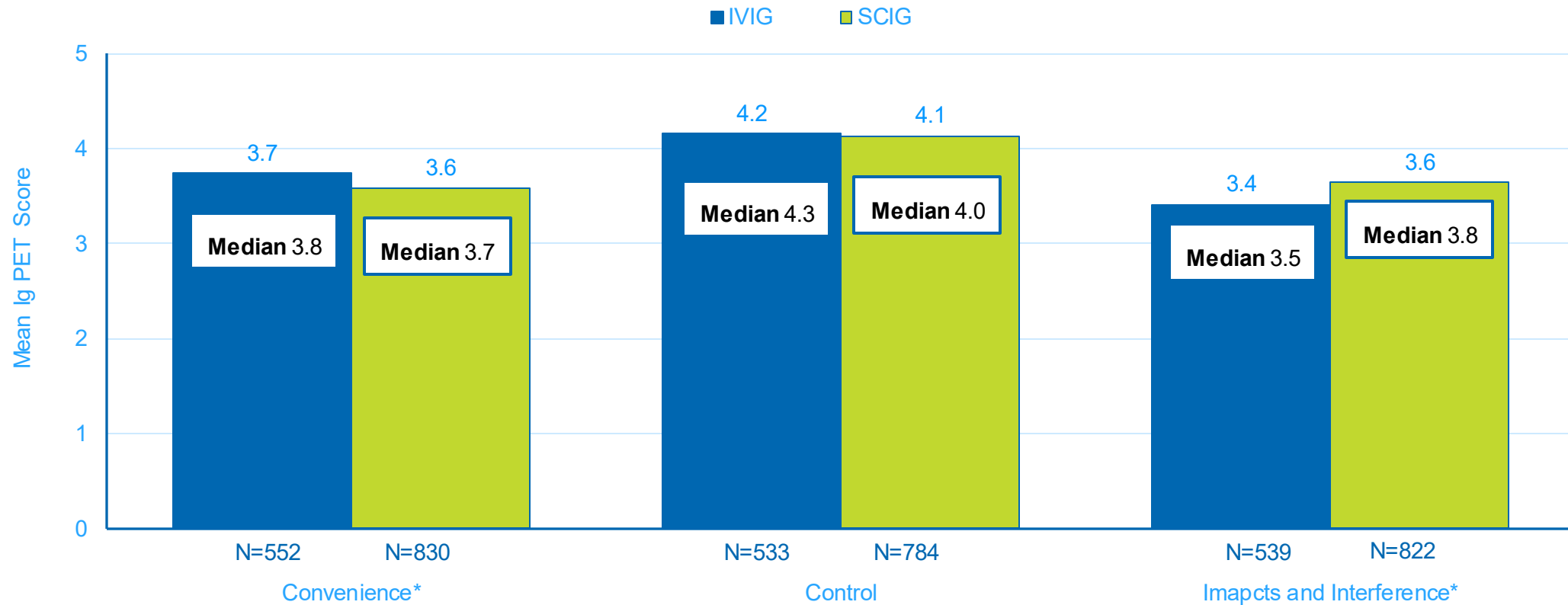
SEOVERAL. How satisfied do you think your child has felt about his/her life overall?

MHUPSET. How much of the time do you think your child acted bothered or upset?

BEGLOBAL. Compared to other children your child's age, in general would you say his/her behavior is...

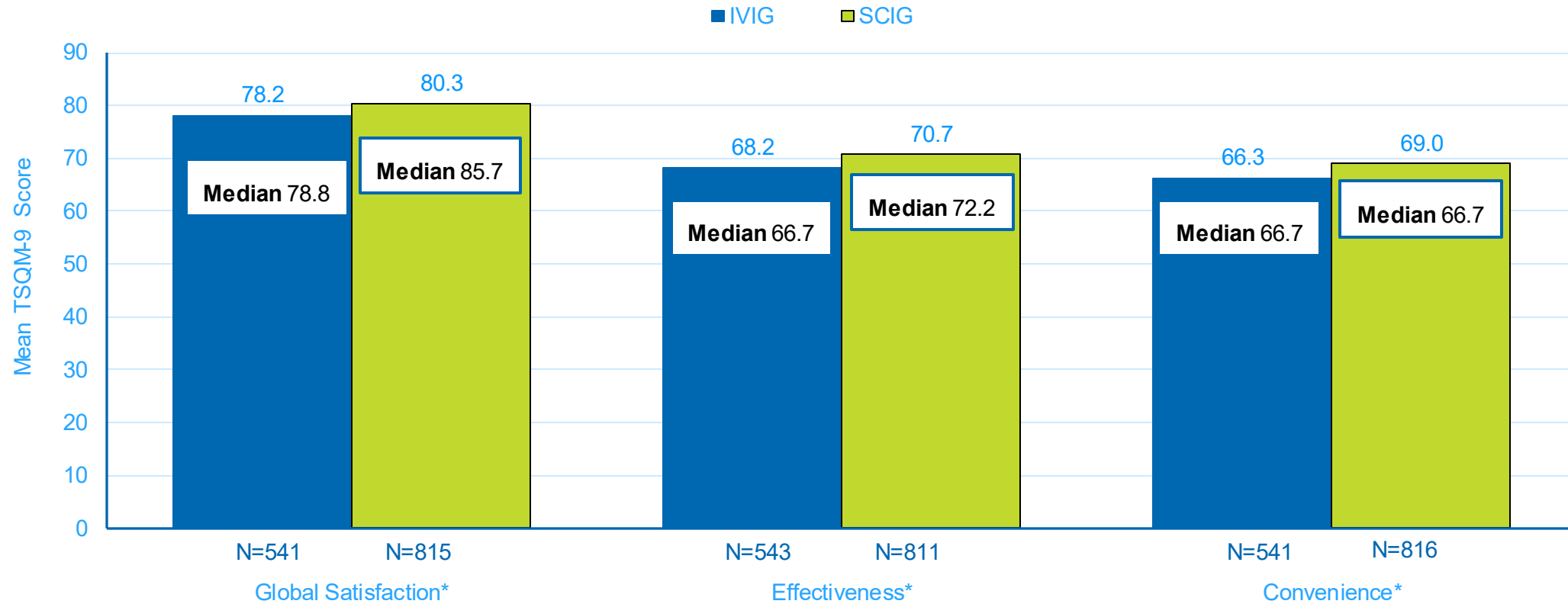
Base: Children who currently use IVIG or SCIG

Ig PET



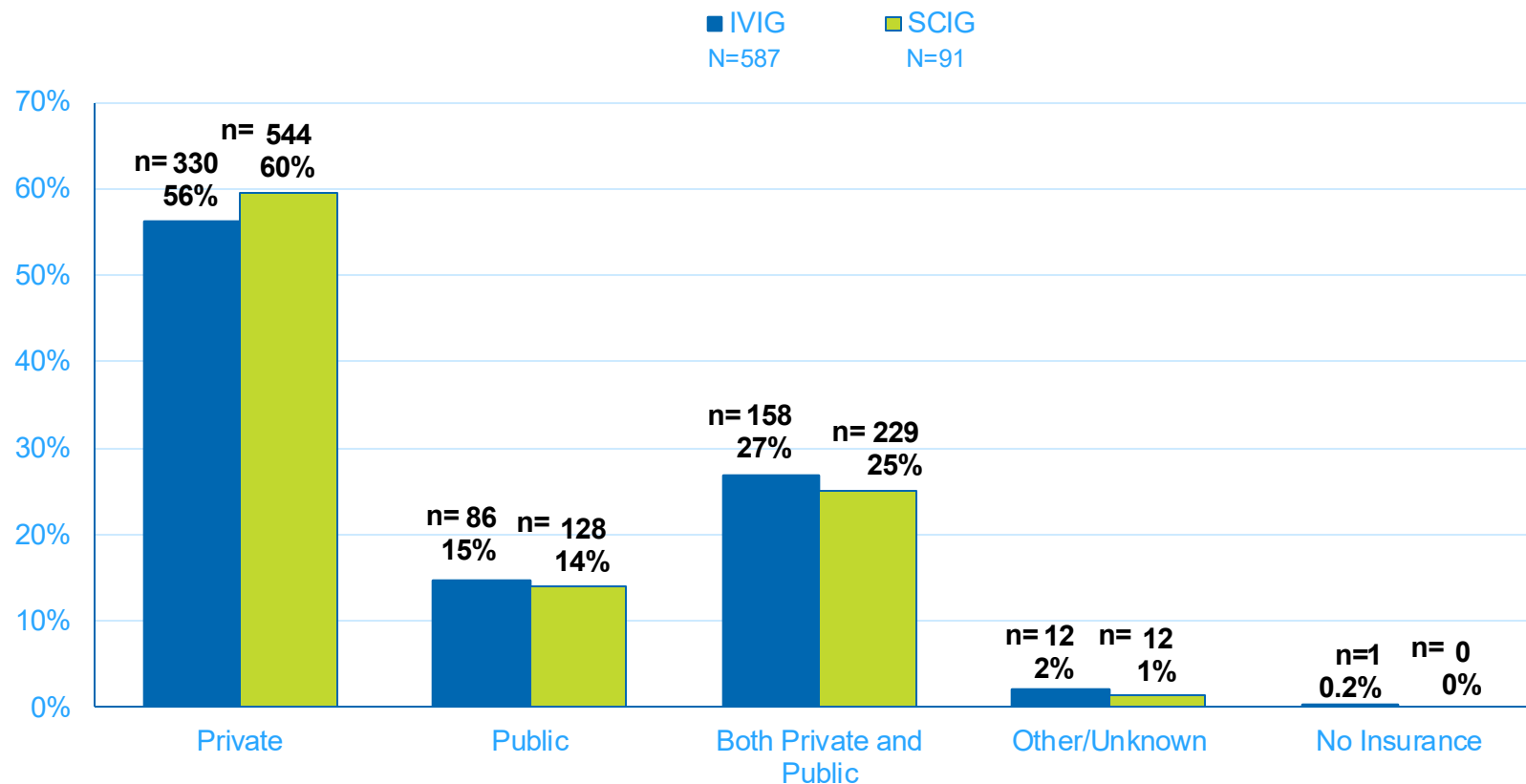
IgPET Convenience Score
IgPET Control Score
IgPET Impacts and Interference Score
Base: Individuals who currently use IVIG or SCIG

TSQM-9



TSQM Global Satisfaction Score
TSQM Effectiveness Score
TSQM Convenience Score
Base: Individuals who currently use MG or SCIG

Current Health Insurance IVIG vs. SCIG



Private Insurance

- Employer sponsored group plan
- Individual policy plan (for individuals or family)
- Cobra
- Exchange/Marketplace (State)
- Exchange/Marketplace (Federal)
- Medicare Advantage Plan
- Medicare Supplement Plan

Public Insurance

- Medicare
- Medicare due to disability
- Medicaid
- TRICARE/Veterans Policy
- Children's Health Insurance Plan

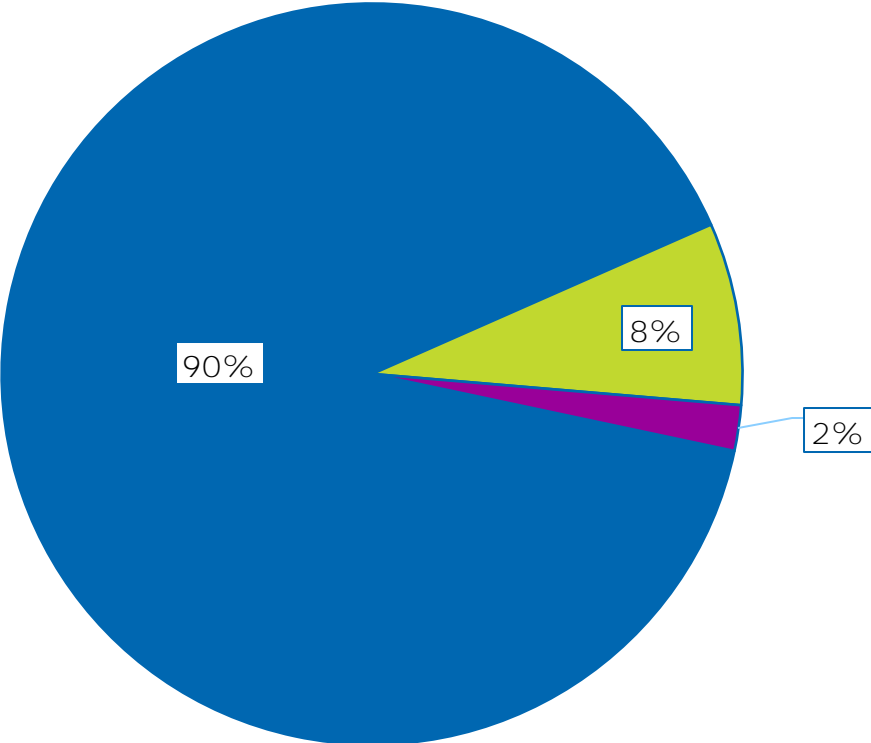
Both Private and Public Insurance

- Employer sponsored group plan/Medicare
- Employer sponsored group plan/Medicaid
- Employer sponsored group plan/TRICARE/VA
- Individual policy plan/Medicare
- Individual policy plan/Medicaid
- Individual policy plan/TRICARE/VA
- Medicaid/Medicare Advantage Plan
- TRICARE/VA/Medicare Supplement Plan

Q63. What kind of health insurance does the PATIENT currently have? SELECT ALL THAT APPLY
Base: Individuals who currently use IVIG or SCIG

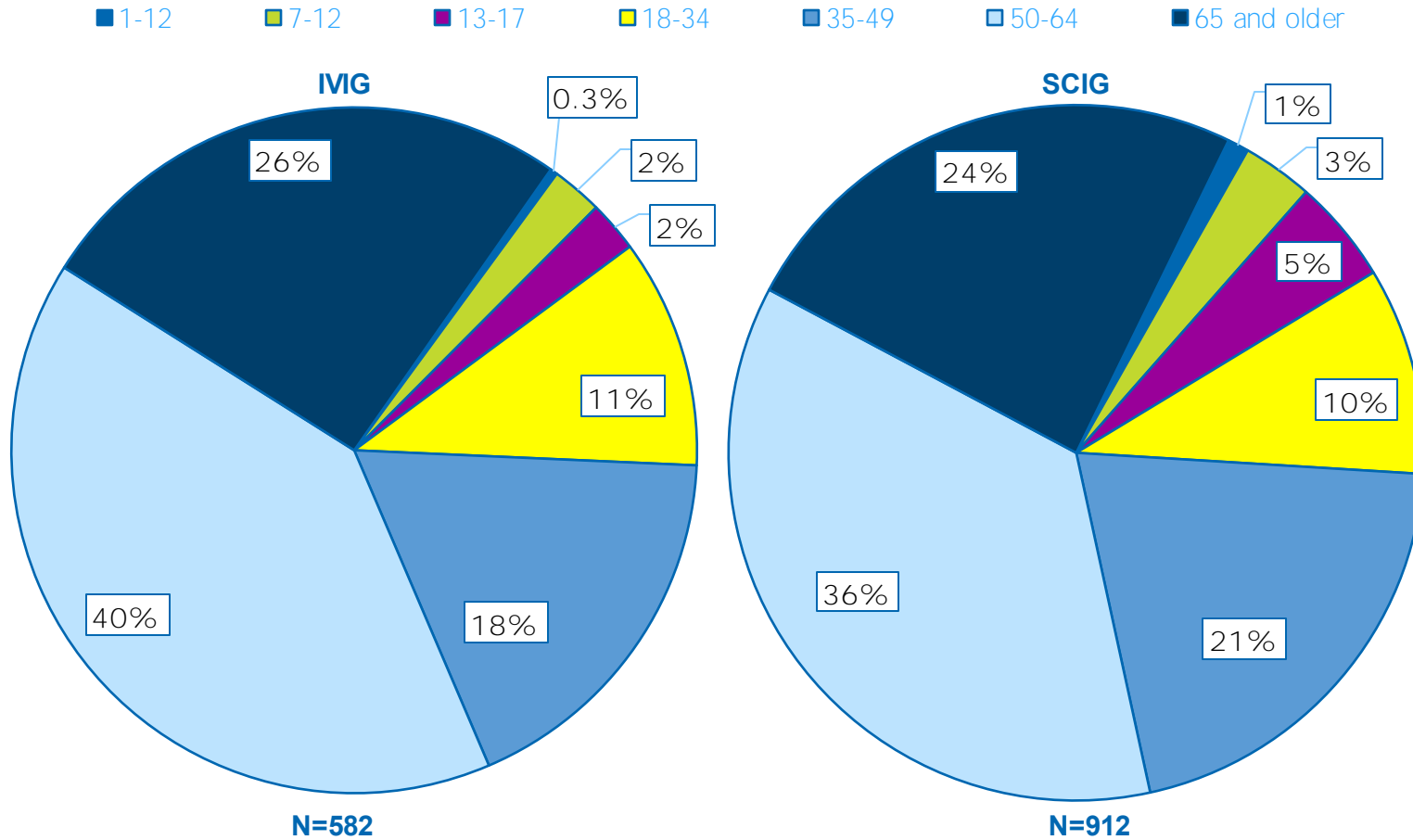
Patient or Parent/Caregiver

■ PI Patient ■ Parent/caregiver ■ Both



Q1. Are you a patient with a primary immunodeficiency disease (PI) or parent/caregiver of a child in the household with PI? N=1,509
Base: Respondents currently receiving MIG or SCIG

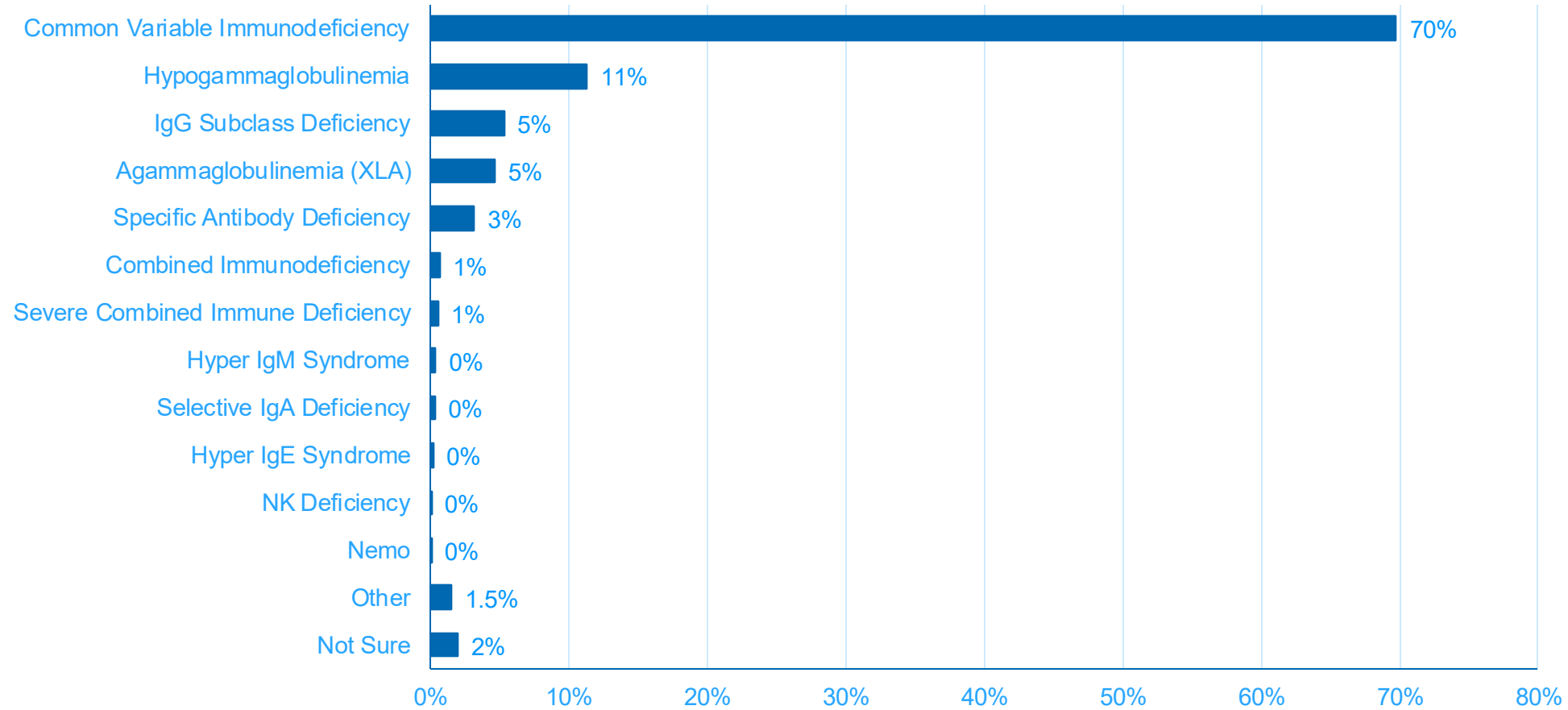
Age of Patient IVIIG vs. SCIG



| | Overall | | Child with PI | | Adult with PI | |
|--------|----------------|---------------|---------------|--------------|----------------|---------------|
| | IVIIG N=582 | SCIG N=912 | IVIIG N=34 | SCIG N=86 | IVIIG N=548 | SCIG N=826 |
| Mean | 52.6 | 50.3 | 12.6 | 12.2 | 55.1 | 54.3 |
| Median | 57.0 | 54.0 | 13.0 | 13.0 | 58.0 | 56.0 |
| Min | 5.0 | 2.0 | 2.0 | 2.0 | 18.0 | 19.0 |
| Max | 90.0 | 84.0 | 18.0 | 18.0 | 90.0 | 84.0 |

Q2. What is the date of birth of the (adult patient/oldest child) in the household with a primary immunodeficiency disease?
Base: Respondents currently receiving IVIG or SCIG

PI Diagnosis



What is the specific diagnosis of the patient's PI? (current diagnosis if more than one) N=1,503
Base: Individuals who currently use MIG or SCIG

Thank You!

