IDF 2017 National Patient Survey

Immune Deficiency Foundation

Introduction

- 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.

- As a result, the Immune Deficiency Foundation (IDF) has sought to fill this void by administering a national patient experience survey within the PI community. Beginning in 1995, IDF has administered four National Patient Surveys (1995, 2002, 2007, 2012) exploring the state of primary immunodeficiency diseases in the United States.

- The data gathered from the National Patient Survey provides clinicians, researchers and most importantly, patients and their families with the only national estimates of the characteristics, treatment and experiences of patients with primary immunodeficiency diseases.

National Patient Survey

- From these National Patient Surveys, we have learned the following:

    1. The magnitude of primary immunodeficiency in the United States;

    2. General population characteristics (e.g., age, gender, type of PI) of persons with primary immunodeficiency;

    3. The current health status of persons with primary immunodeficiency diseases;

    4. Difficulties in access to treatment for this population.

- As with previous administrations of this survey, our goal is to gather data on patient diagnosis, family history of PI, health issues (e.g., infections, hospitalizations) prior to and after diagnosis, treatment (e.g., immunoglobulin replacement therapy, bone marrow transplant, prophylactic antibiotics) and current health status.

Patient Health Questionnaire-2, Brief Fatigue Inventory, SF-12 and SF-10

- In addition to administering the fifth National Patient Survey, IDF also asked participants to complete, where appropriate, the Patient Health Questionnaire-2, Brief Fatigue Inventory, SF-12 and SF-10.

- The **Patient Health Questionnaire-2** (PHQ-2) includes the first two items of the PHQ-9. The purpose of the PHQ-2 is to screen for depression. Patients who screen positive (a score of three or higher) should be further evaluated with the PHQ-9 to determine whether they meet criteria for a depressive disorder.
  - IDF hopes to measure the prevalence of possible depression among adults with PI.

- The **Brief Fatigue Inventory** (BFI) assesses the severity and impact of fatigue participants' activities of daily living.
  - Originally created for cancer patients, IDF will undertake the first national administration of the BFI to persons with primary immunodeficiency.

- 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 2017 National Patient Survey.

- The 2017 National Patient survey contained up to 56 questions, depending on the respondent's experiences.
  - Including the BFI and SF-12, Adults with PI answered up to a total of 77 questions
  - Including the SF-10, Parents of children with PI answered up to a total of 66 questions

- The study protocols were reviewed by Schulman IRB Associates and received an exemption from IRB review and approval.

- In field: October 23, 2017 – April 5, 2018
  - 4,500 mailed surveys
  - 190 undeliverable surveys
  - 1,121 surveys completed

Topics

- Diagnosis
  - Type of Respondent
  - Type of PI
  - Repeated, Unusual or Serious Infections Prior to Diagnosis
  - Time to Diagnosis
  - Physician at Time of Diagnosis
  - Reason for Testing
  - Family History and Diagnosis

- Comorbidities
  - Permanent Impairments
  - Fatigue
  - Other Chronic Conditions
  - Infections

- General Health and Healthcare
  - SF-12 and SF-10
  - General Health
  - Physical Limitations
  - Treating Physician
  - Specialists
  - Hospitalizations
  - Insurance

- Mental Health
  - PHQ-2
  - Limitations Due to Emotional Problems
  - Seeing a Mental Health Professional
  - Currently Taking Prescription Medications for Anxiety, Depression or Stress
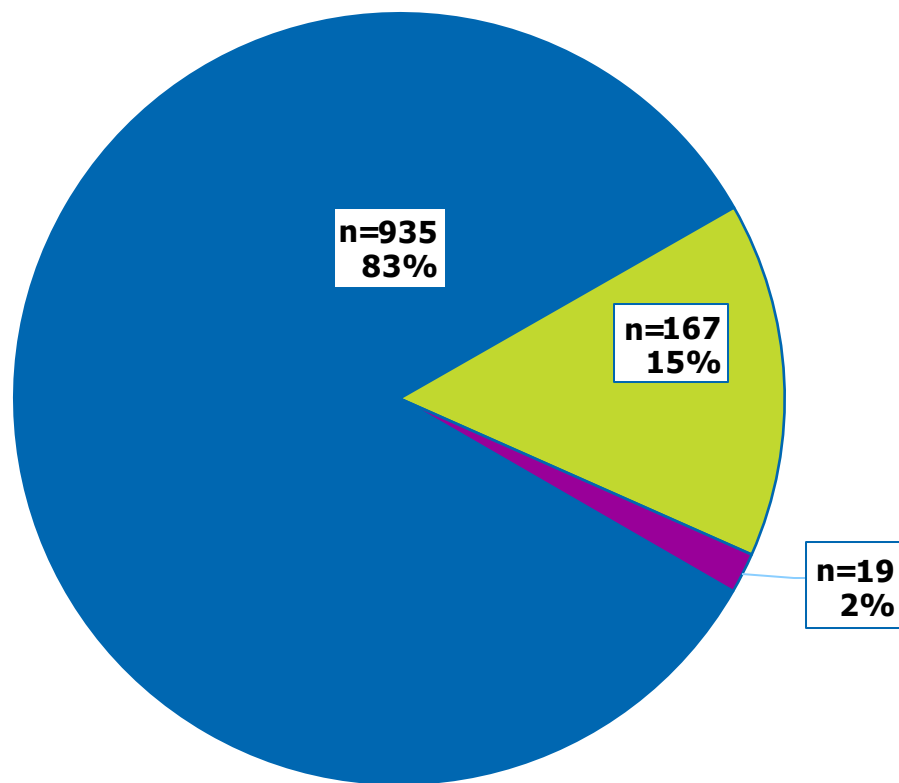 - Currently Taking Prescription Medications for Pain

- Treatment
  - Ever
  - Current
  - Ig Replacement Therapy
  - Wear Off Effect
  - Prophylactic Antibiotic Therapy
  - Prophylactic Antifungal Therapy
  - Other Medications
  - Additional Treatments

Diagnosis

Type of Respondent and Age

■ Adult person with PI   ☐ Parent/caregiver of child in household with PI   ■ Both

n=935
83%

n=167
15%

n=19
2%

	Overall N=985	Child with PI N=167	Adult with PI N=818
Mean	46.7	9.8	54.3
Median	52.00	10.0	56.0
Min	1.0	1.0	18.0
Max	92.0	18.0	92.0

Q1. Are you an adult person with a primary immunodeficiency disease (PI) or a parent/caregiver of a child in the household with PI? N=1,121
Q3. What is the age and gender of these persons with PI?
Base: All respondents

Source: IDF 2017 National Patient Survey

Type of PI

■ Antibody Deficiencies   ■ Cellular Immunodeficiencies   ■ Innate Immune Disorders

Antibody Deficiency Disorders
CVID
Hypogammaglobulinemia
XLA
IgG Subclass Deficiency
Selective IgA Deficiency
Specific Antibody Deficiency
Selective IgM Deficiency
Good's Syndrome

Cellular Immunodeficiency Disorders
SCID
CID
WAS
Hyper IgM Syndrome
Ataxia-Telangiectasia
DiGeorge Syndrome
XLP

Innate Immune Disorders
CGD
Complement Deficiency
Hyper IgE
Hereditary Angioedema
Severe Congenital Neutropenia
IRAK4
Natural Killer Cell Deficiency

n=979
92%

n=45
4%

n=35
3%

Q5. What is the specific diagnosis of the patient's PI (current diagnosis if more than one)? N=1,059
Base: All respondents with only 1 PI diagnosis

9

Repeated, Unusual or Serious Infections Prior to Diagnosis

■ Yes   ■ No

n=1,018
92%

n= 89
8%

Age when experience repeated, unusual or serious infections began (N=947):
56% between infancy to 17 years old
44% between ages 18 to 75 years old
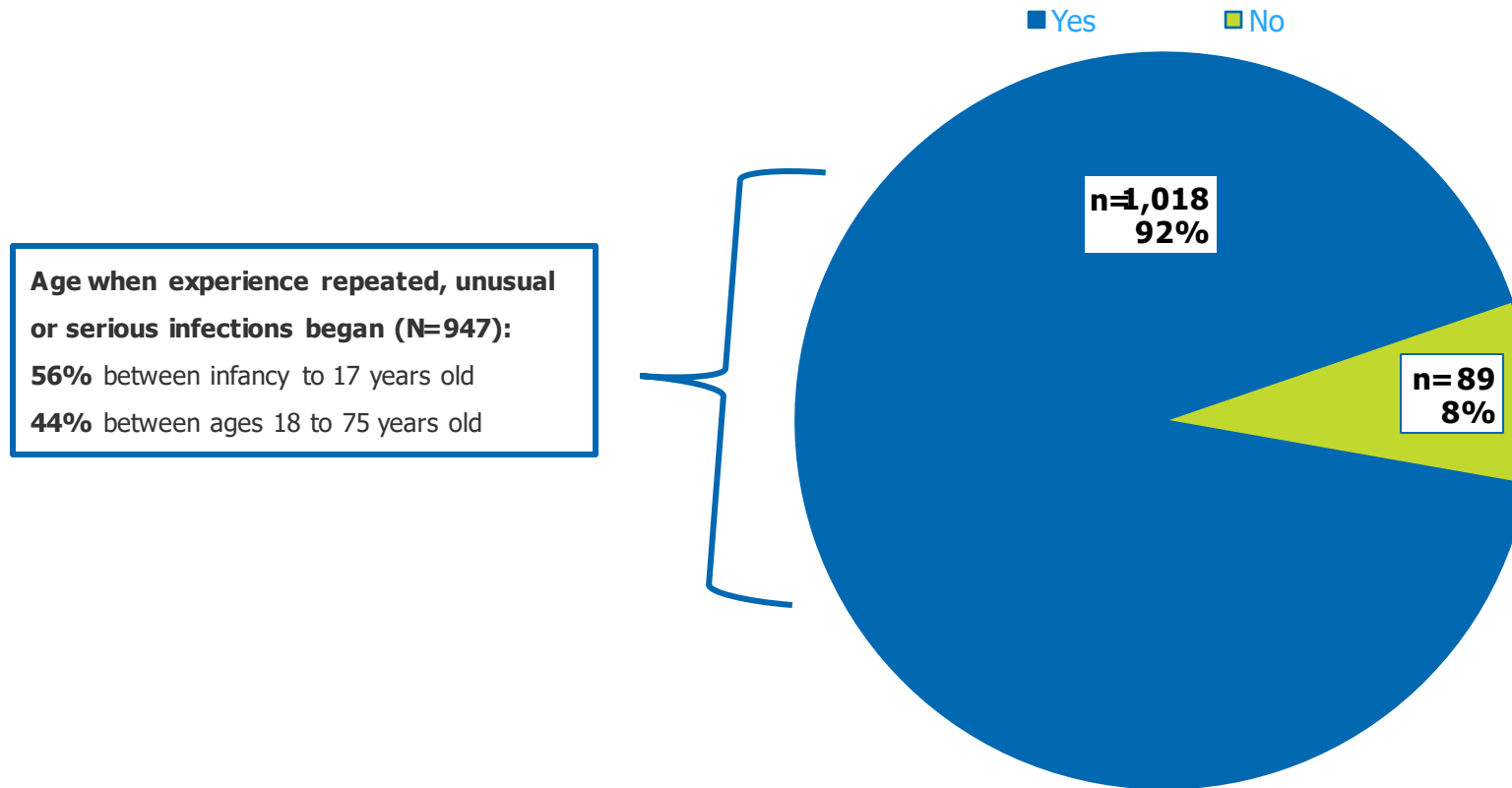
Q7a. Did the patient with PI experience repeated, serious or unusual infections prior to initial diagnosis as immune deficient? N=1,107
Base: All respondents

10

Age at Diagnosis

22% were diagnosed before age 18

78% were diagnosed after age 17

% Individuals with PI

- Younger than 1 year old: n=47, 4%
- 1 to 6: n=97, 9%
- 7 to 12: n=59, 5%
- 13 to 17: n=41, 4%
- 18 to 34: n=117, 11%
- 35 to 49: n=282, 26%
- 50 to 64: n= 346, 32%
- 65 and older: n=101, 9%
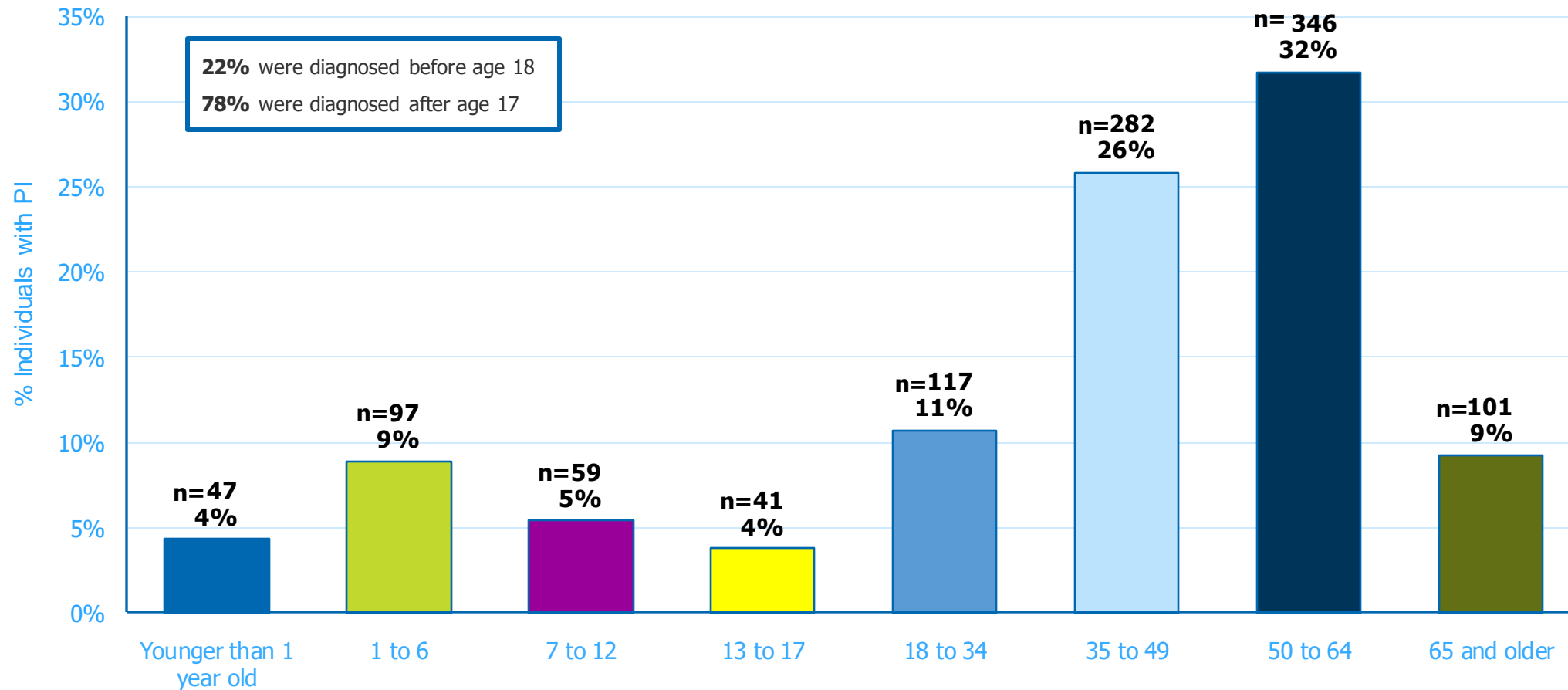
Q4. At what age (in years) was the patient first diagnosed with a PI? N=1,090
Base: All respondents
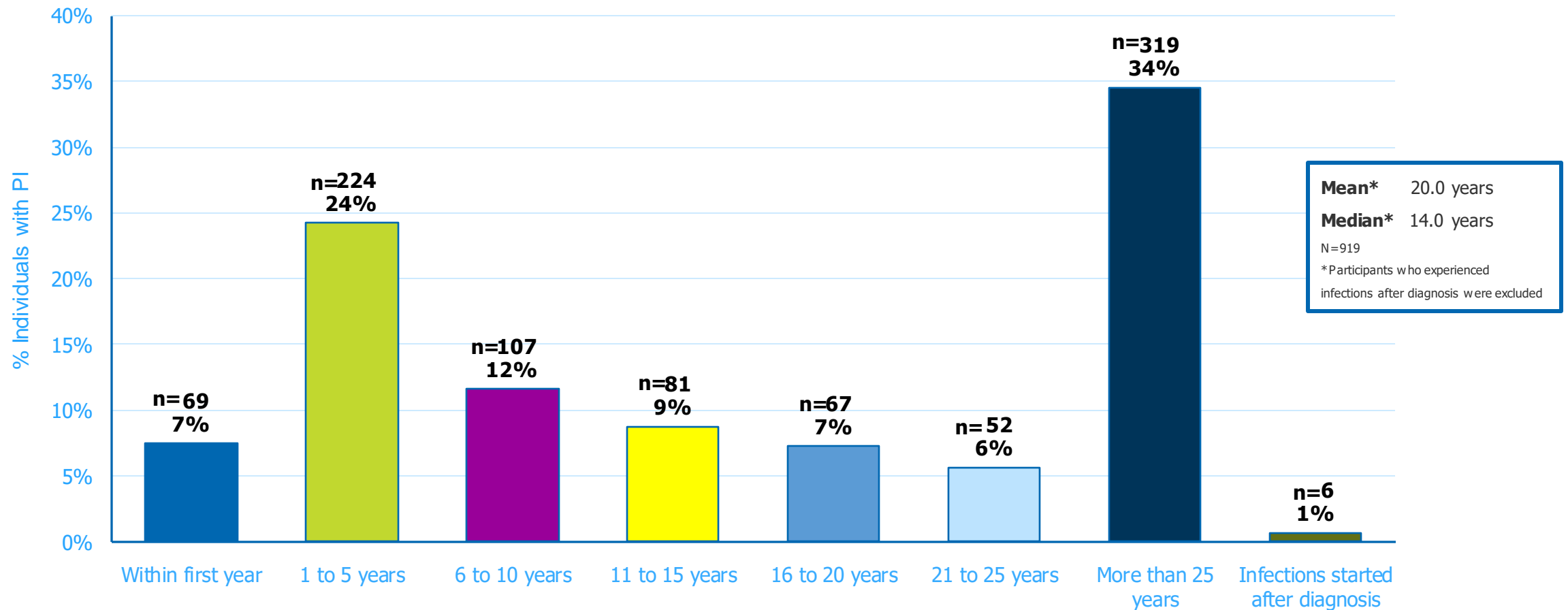
Source: IDF 2017 National Patient Survey

11

Time to Diagnosis

% Individuals with PI

- Within first year: n=69, 7%
- 1 to 5 years: n=224, 24%
- 6 to 10 years: n=107, 12%
- 11 to 15 years: n=81, 9%
- 16 to 20 years: n=67, 7%
- 21 to 25 years: n=52, 6%
- More than 25 years: n=319, 34%
- Infections started after diagnosis: n=6, 1%

Mean* 20.0 years
Median* 14.0 years
N=919
*Participants who experienced infections after diagnosis were excluded

Q4. At what age (in years) was the patient first diagnosed with a PI?
Q7b. At what age (in years) did these repeated, serious, or unusual infections begin?
N=925
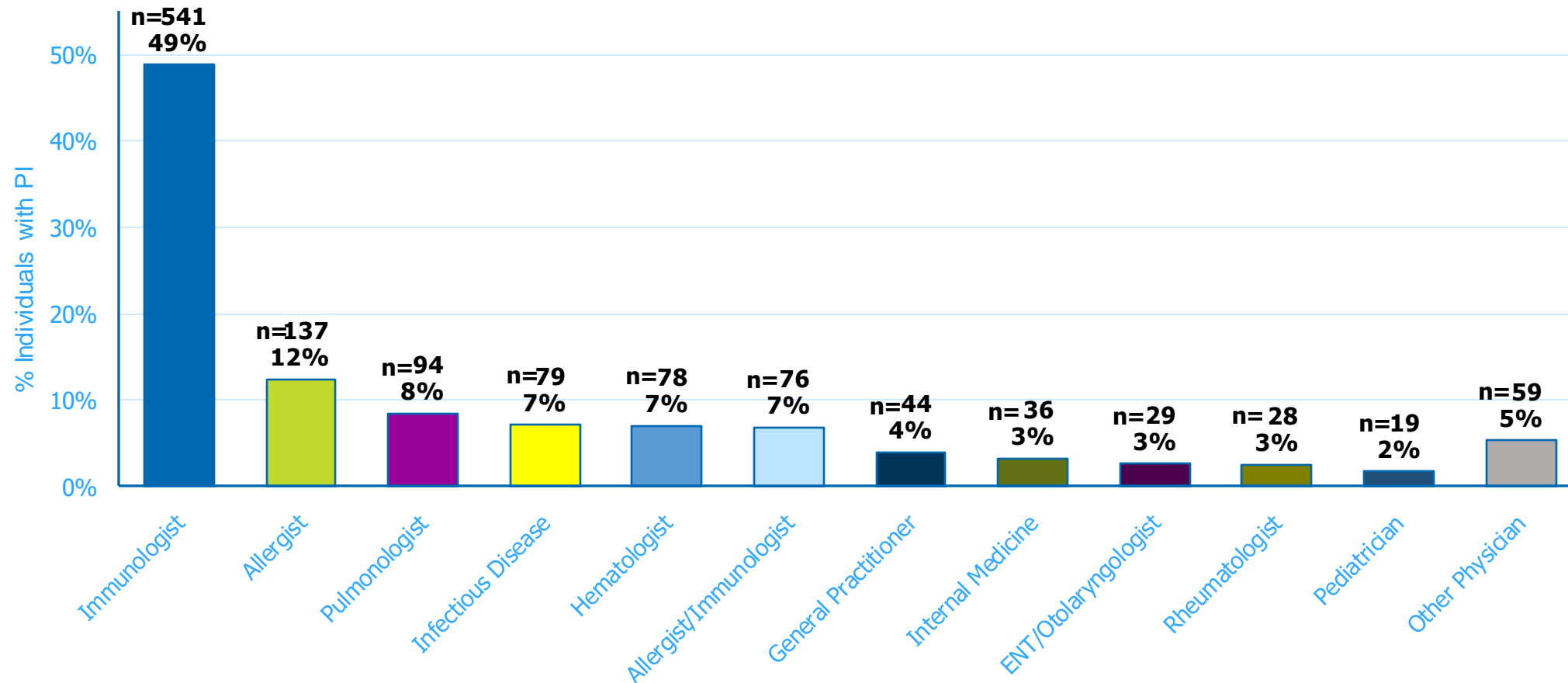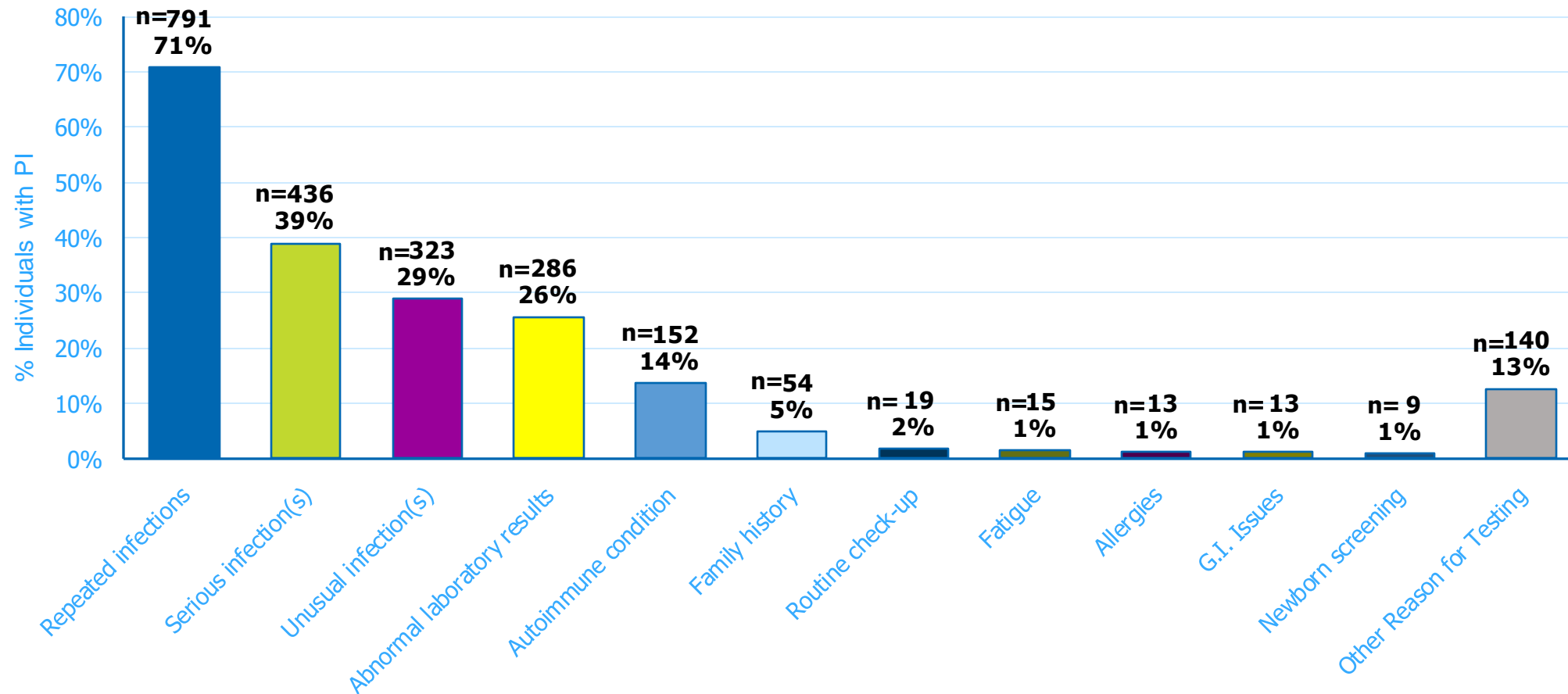Base: All respondents who experienced repeated, unusual or serious infections prior and after diagnosis

Source: IDF 2017 National Patient Survey

Physician at Time of Diagnosis

% Individuals with PI

- Immunologist: n=541, 49%
- Allergist: n=137, 12%
- Pulmonologist: n=94, 8%
- Infectious Disease: n=79, 7%
- Hematologist: n=78, 7%
- Allergist/Immunologist: n=76, 7%
- General Practitioner: n=44, 4%
- Internal Medicine: n=36, 3%
- ENT/Otolaryngologist: n=29, 3%
- Rheumatologist: n=28, 3%
- Pediatrician: n=19, 2%
- Other Physician: n=59, 5%

Q10. What kind of doctor diagnosed the PI? N=1,109
Base: All respondents

Source: IDF 2017 National Patient Survey

13

Reason for Testing

% Individuals with PI

Category	n	%
Repeated infections	n=791	71%
Serious infection(s)	n=436	39%
Unusual infection(s)	n=323	29%
Abnormal laboratory results	n=286	26%
Autoimmune condition	n=152	14%
Family history	n=54	5%
Routine check-up	n=19	2%
Fatigue	n=15	1%
Allergies	n=13	1%
G.I. Issues	n=13	1%
Newborn screening	n=9	1%
Other Reason for Testing	n=140	13%

Q8. Why was the patient initially tested for PI? SELECT ALL THAT APPLY N=1,118
Base: All respondents

Source: IDF 2017 National Patient Survey

Family History of PI

■ Yes   ■ No

n=201
18%

n=907
82%
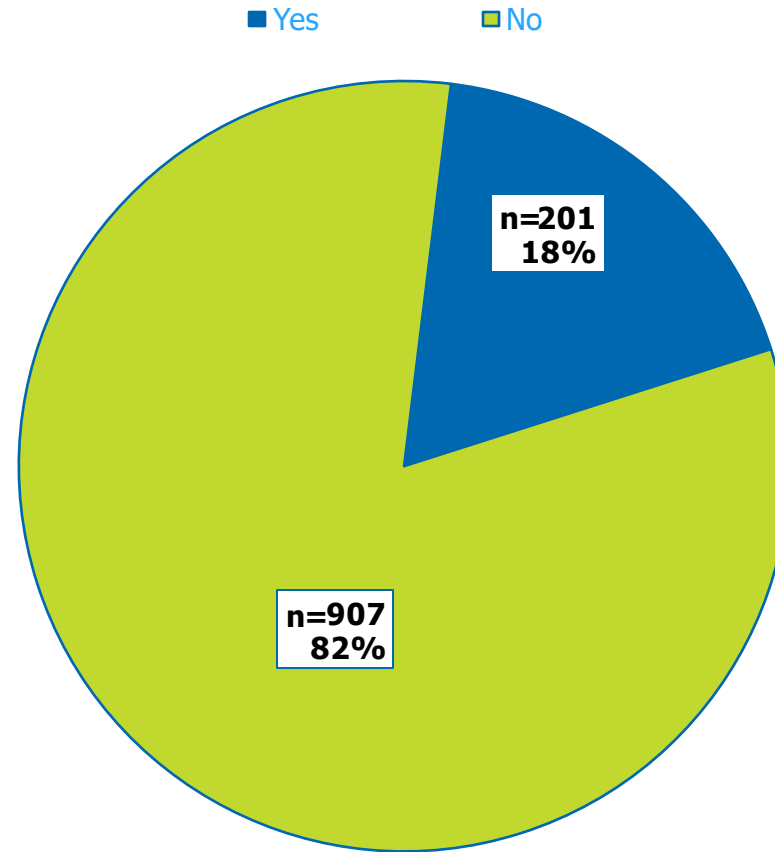
Q6a. Have any other family members of the patient been diagnosed as having a PI (even if they are not living now)? N=1,108
Base: All respondents

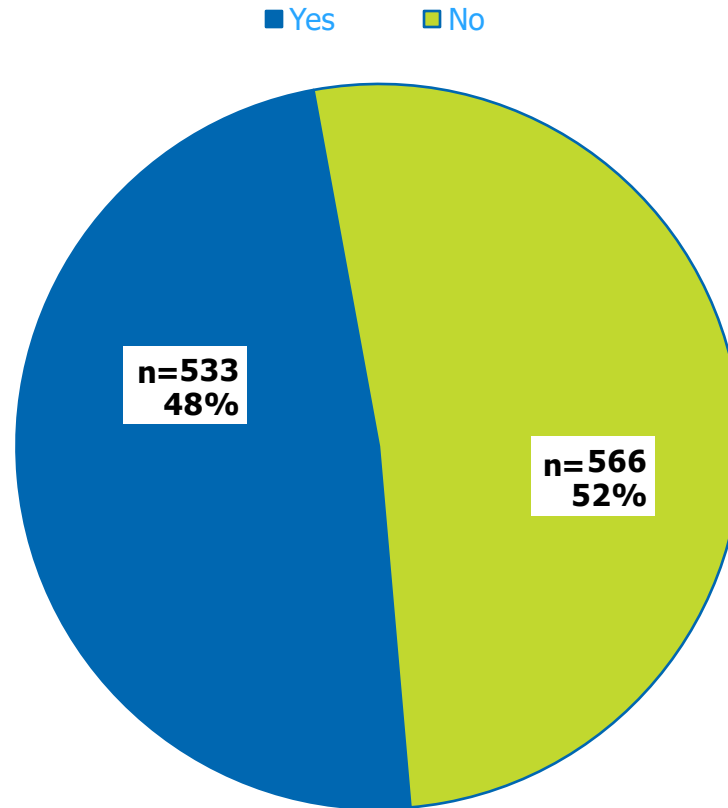Source: IDF 2017 National Patient Survey

Type of Relatives with PI

% Individuals with PI

- Father: n=17, 9%
- Mother: n=38, 19%
- Brother(s): n= 40, 21%
- Sister(s): n=39, 20%
- Uncle(s): n=11, 6%
- Aunt(s): n=12, 6%
- Cousin(s): n=26, 13%
- Son(s): n=23, 12%
- Daughter(s): n=29, 15%
- Grandmother(s): n=7, 4%
- Other: n= 21, 11%
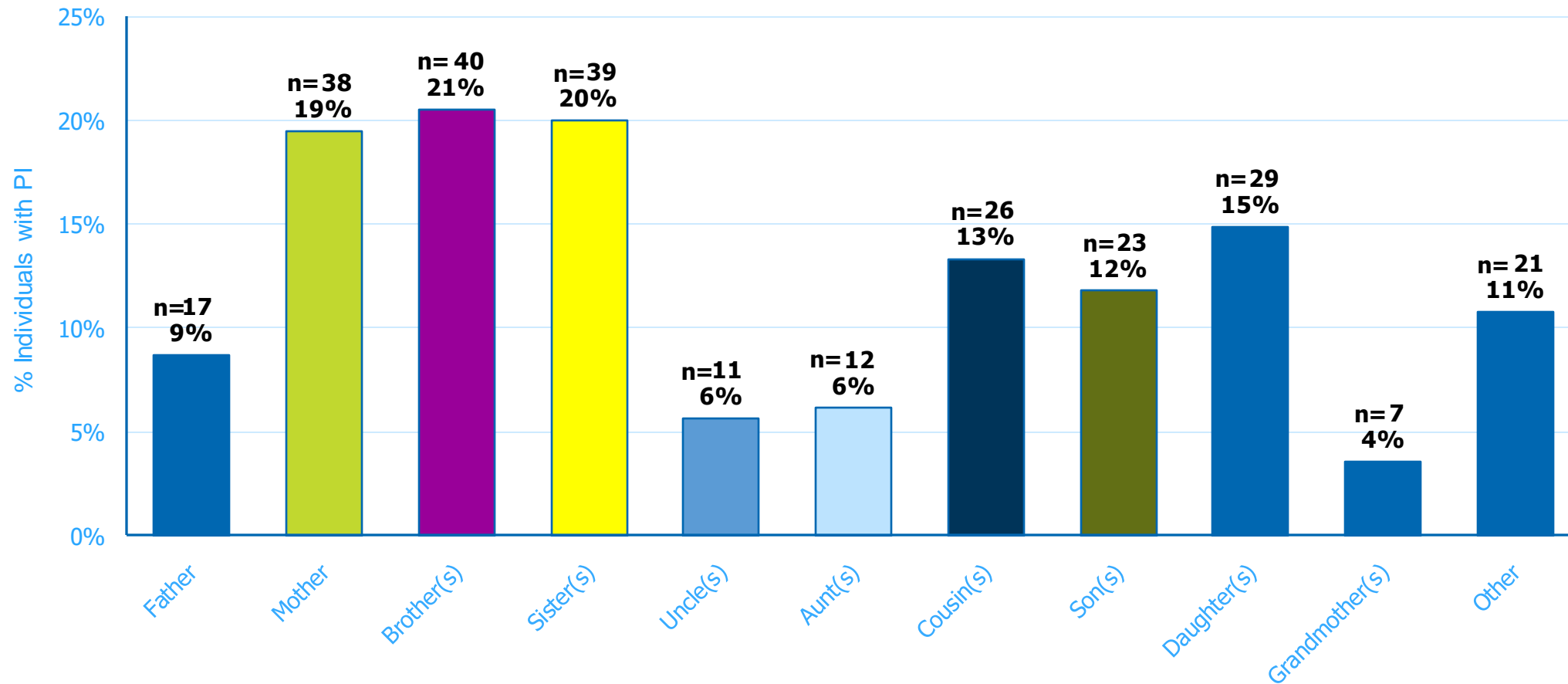
Q6b. Which patient's family member(s) have been diagnosed with a PI? SELECT ALL THAT APPLY N=195
Base: All respondents who have a family member(s) also diagnosed with PI
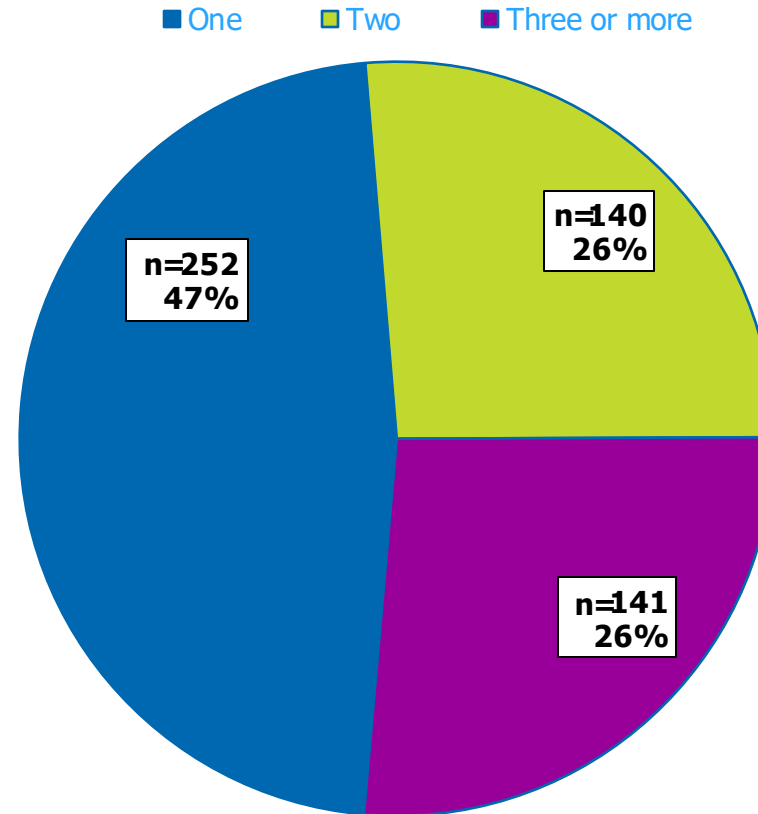
Source: IDF 2017 National Patient Survey

16

Comorbidities

Permanent Impairments

■ Yes   ■ No

n=533
48%

n=566
52%

Source: IDF 2017 National Patient Survey

Number of Permanent Impairments
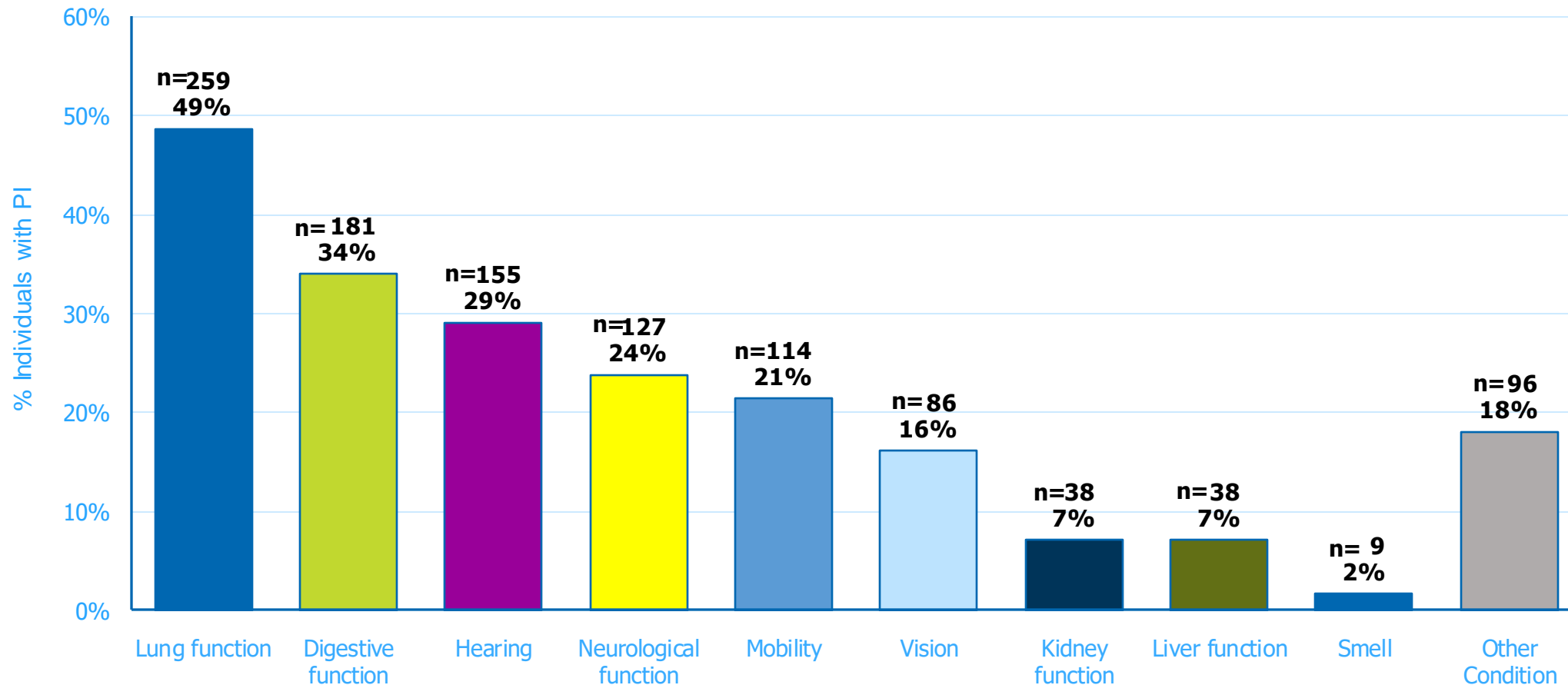

One   Two   Three or more

n=252
47%

n=140
26%

n=141
26%

Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of…? SELECT ALL THAT APPLY N=533
Base: All respondents who have at least one permanent impairment

Permanent Impairments: One or More Impairments

Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of...? SELECT ALL THAT APPLY N=533
Base: All respondents who have at least one permanent impairment

Permanent Impairments by Time to Diagnosis*

Q4. At what age (in years) was the patient first diagnosed with a PI?
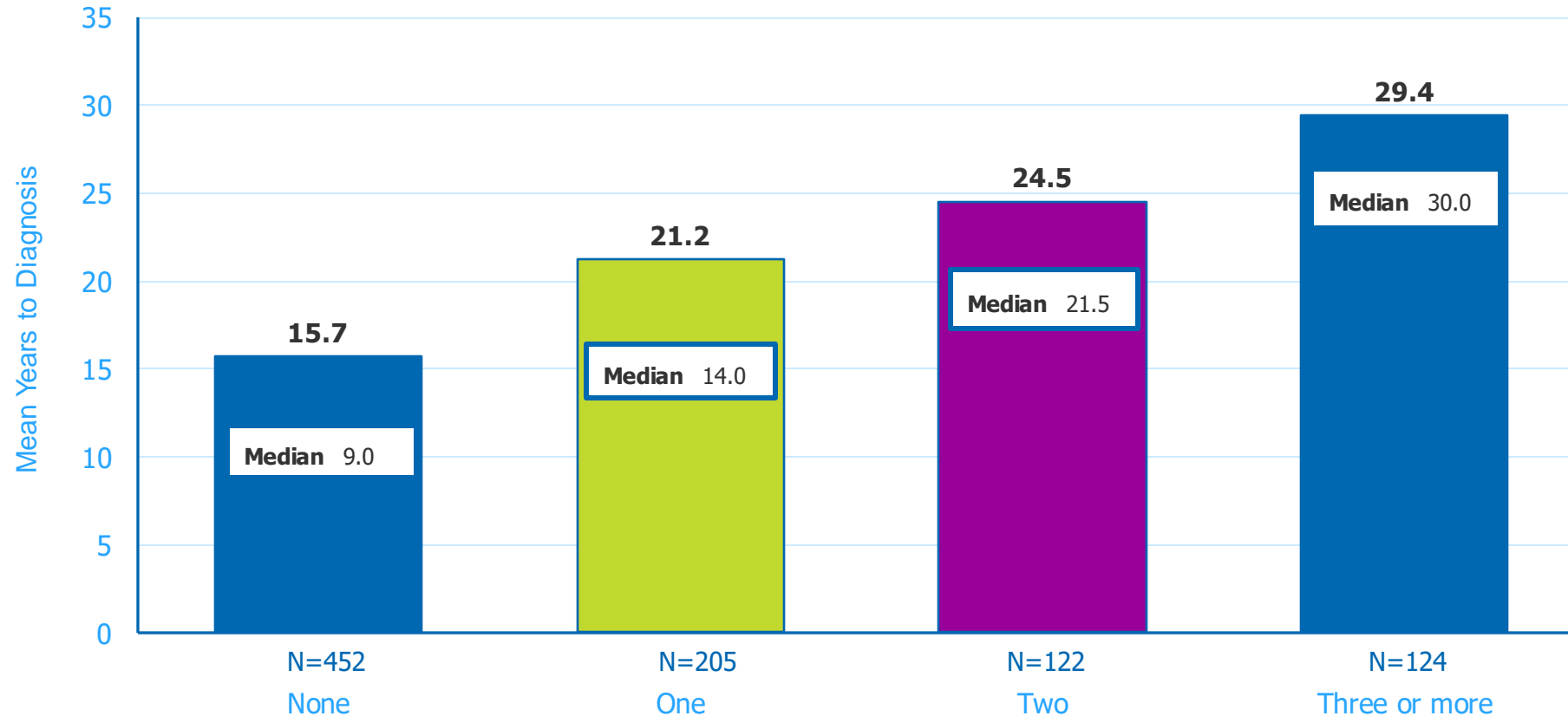Q7b. At what age (in years) did these repeated, serious, or unusual infections begin?
Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of...? SELECT ALL THAT APPLY
Base: All respondents who have at least one permanent impairment

* p<.00

BFI Severity

Mild
1 to 4

Moderate
5 to 6

Severe
7 to 10

n=211
25%

n=227
26%

n=421
49%

Global BFI Score N=859
Base: All Adults with PI age 18 years old and older

Source: IDF 2017 National Patient Survey

Global BFI by Time to Diagnosis*
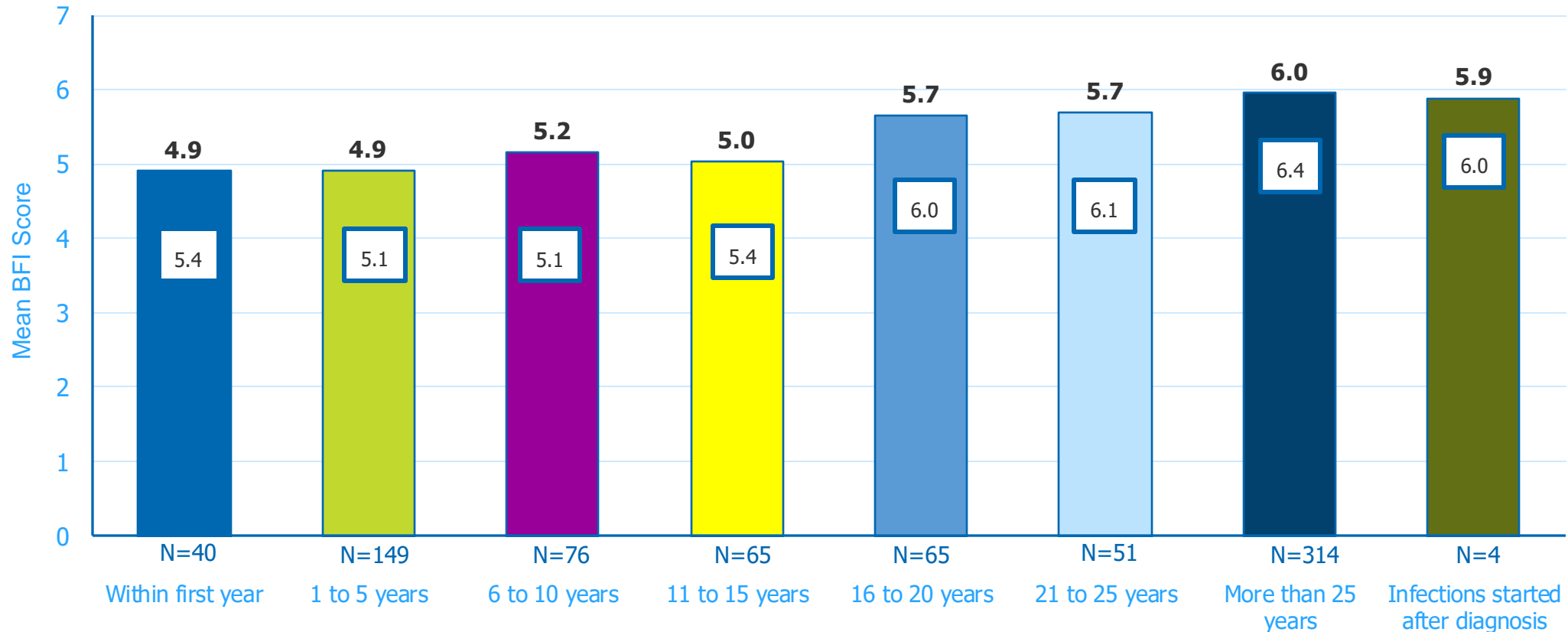
Mean BFI Score

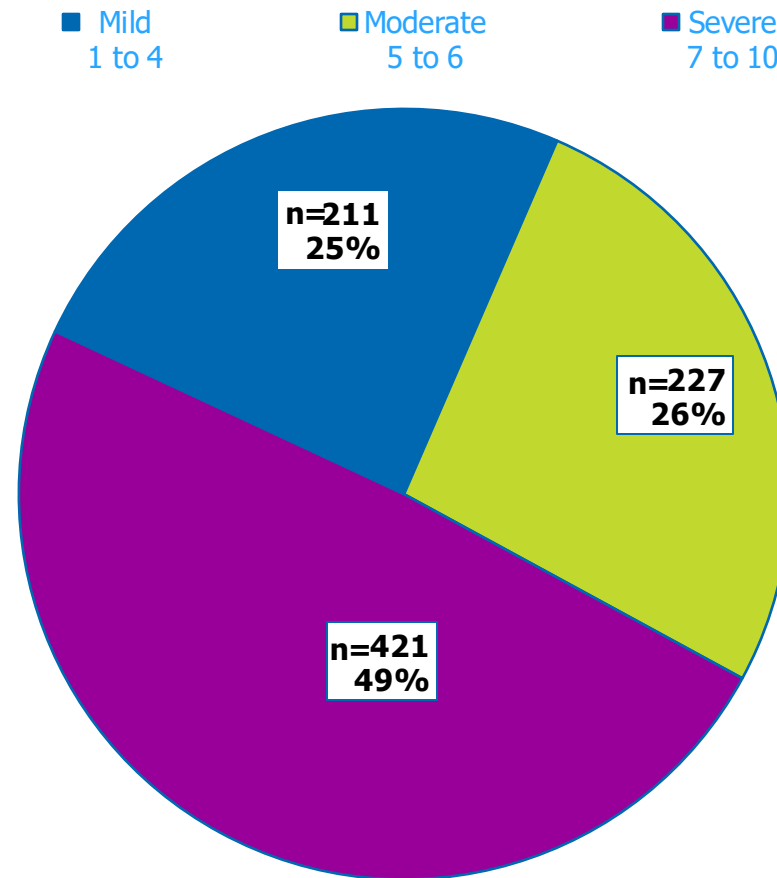| Category	Mean	Median	N
Within first year	4.9	5.4	N=40
1 to 5 years	4.9	5.1	N=149
6 to 10 years	5.2	5.1	N=76
11 to 15 years	5.0	5.4	N=65
16 to 20 years	5.7	6.0	N=65
21 to 25 years	5.7	6.1	N=51
More than 25 years	6.0	6.4	N=314
Infections started after diagnosis	5.9	6.0	N=4

Median

* p=.001

Q4. At what age (in years) was the patient first diagnosed with a PI?
Q7b. At what age (in years) did these repeated, serious, or unusual infections begin?
Global BFI Score
Base: All Adults with PI age 18 years old and older
Note: Mild BFI Score 1 to 3, Moderate BFI Score 4 to 6, Severe BFI Score 7 to 10

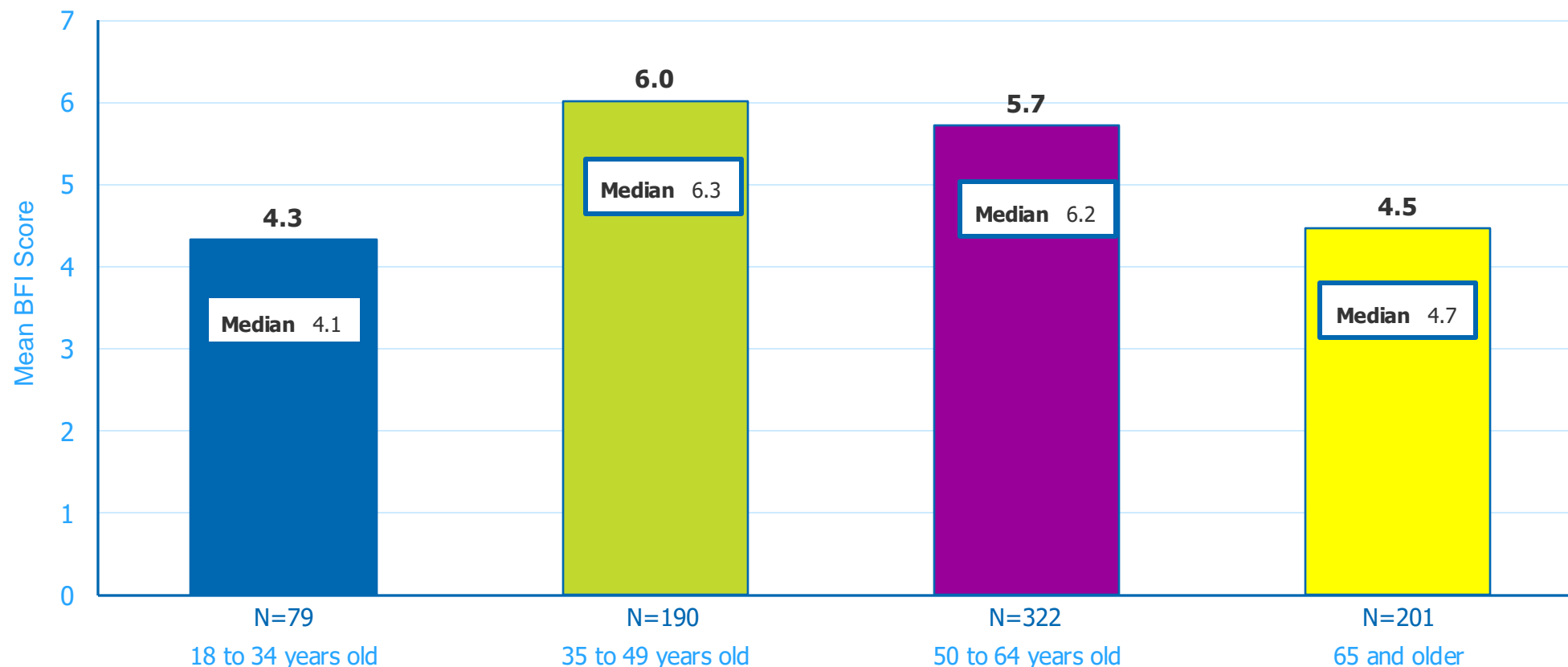# Global BFI by Age Group*

Mean BFI Score

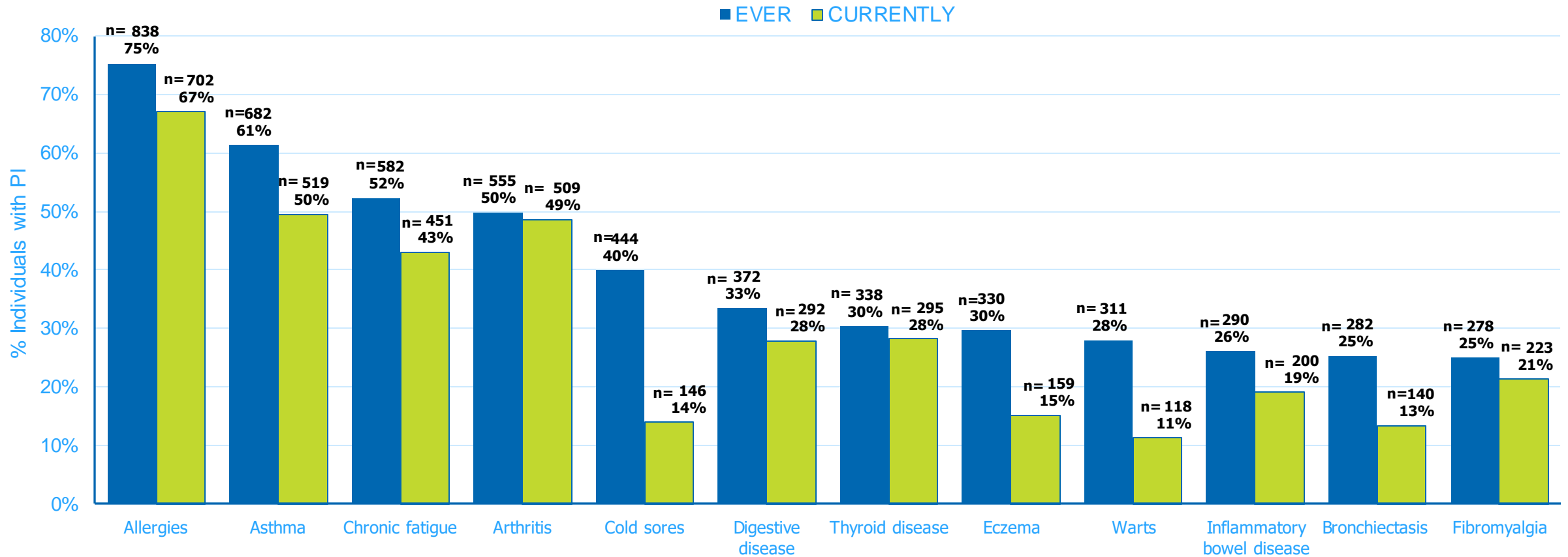|			
4.3	**6.0**	**5.7**	**4.5**
Median 4.1	Median 6.3	Median 6.2	Median 4.7
N=79	N=190	N=322	N=201
18 to 34 years old	35 to 49 years old	50 to 64 years old	65 and older

* p<.00

Q3. What is the age and gender of these persons with PI?
Global BFI Score
Base: All Adults with PI age 18 years old and older
Note: Mild BFI Score 1 to 3, Moderate BFI Score 4 to 6, Severe BFI Score 7 to 10

Most Common: Other Chronic Conditions EVER and CURRENT

■ EVER ■ CURRENTLY

% Individuals with PI

Condition	EVER	CURRENTLY
Allergies	n= 838 75%	n= 702 67%
Asthma	n=682 61%	n= 519 50%
Chronic fatigue	n=582 52%	n= 451 43%
Arthritis	n= 555 50%	n= 509 49%
Cold sores	n=444 40%	n= 146 14%
Digestive disease	n= 372 33%	n= 292 28%
Thyroid disease	n= 338 30%	n= 295 28%
Eczema	n=330 30%	n= 159 15%
Warts	n= 311 28%	n= 118 11%
Inflammatory bowel disease	n= 290 26%	n= 200 19%
Bronchiectasis	n= 282 25%	n=140 13%
Fibromyalgia	n= 278 25%	n= 223 21%

Q20a. Please tell us about any diagnosed conditions the patient has EVER had. SELECT ALL THAT APPLY N=1,113
Q20b. Please tell us about any diagnosed conditions the patient CURRENTLY has. SELECT ALL THAT APPLY N=1,048
Base: All respondents

25

Infections
Prior to and After Diagnosis

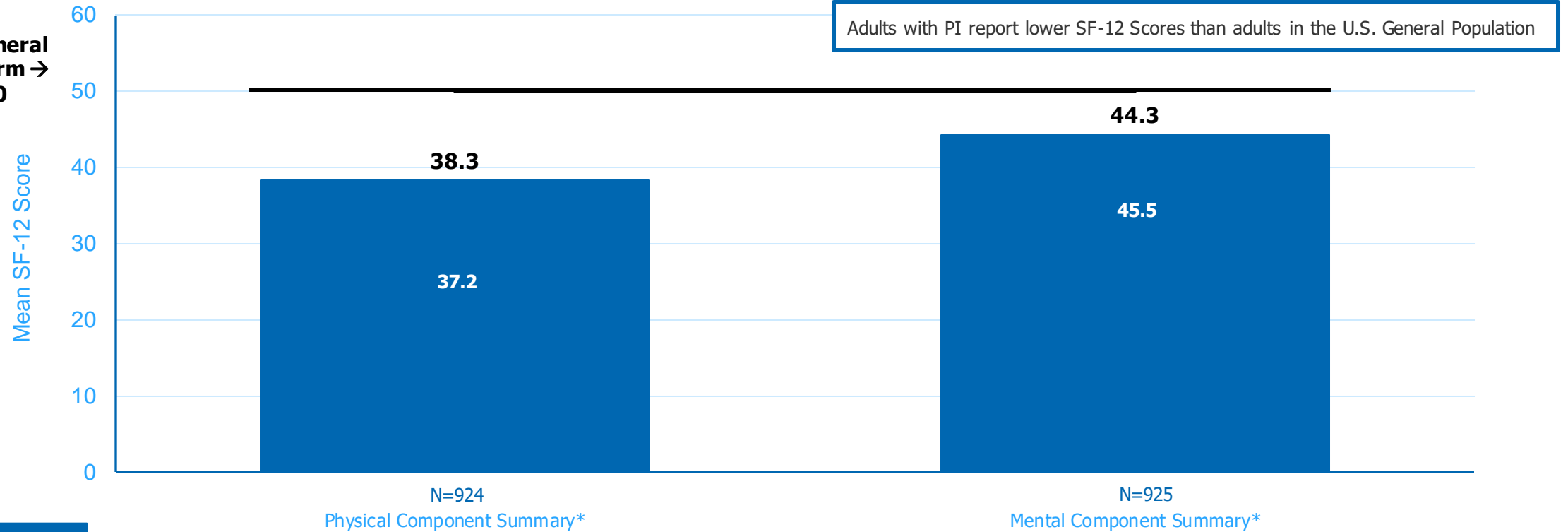■ Prior to Diagnosis   ■ Past 12 Months
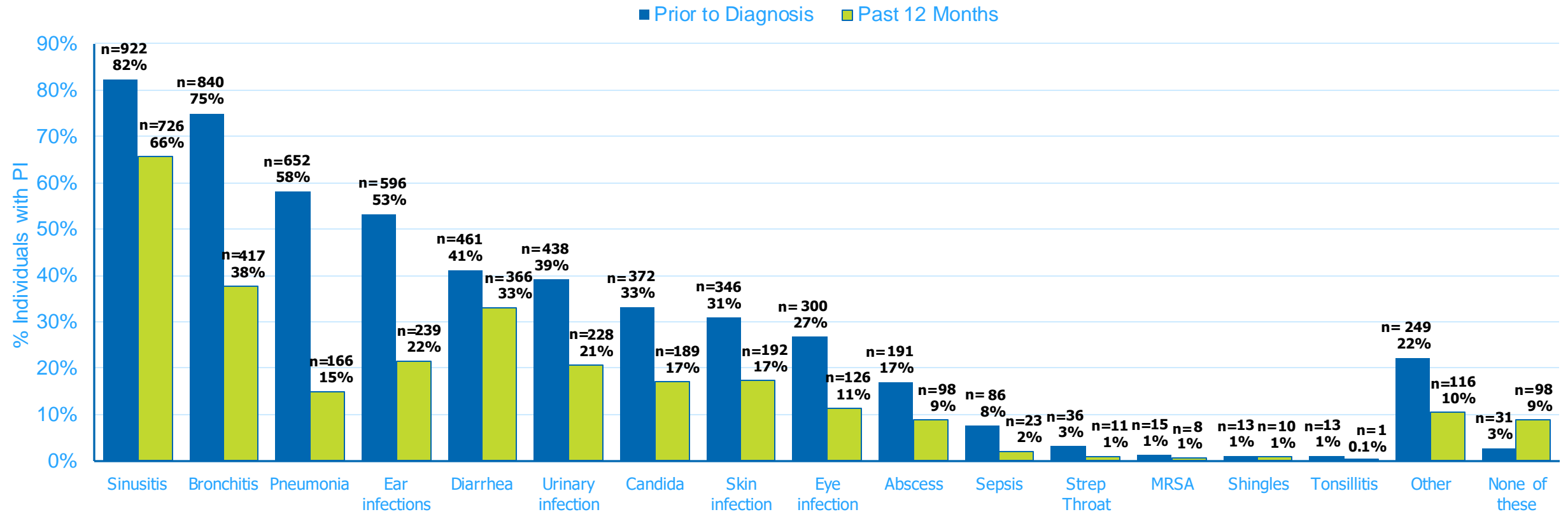
Q15. Which of the following infections, if any, did the patient have BEFORE diagnosis as immune deficient? SELECT ALL THAT APPLY N=1,120
Q21. Which of the following infections, if any, has the patient had during the past 12 months? SELECT ALL THAT APPLY N=1,107
Base: All respondents

Source: IDF 2017 National Patient Survey

General Health and Health Care

SF-12

U.S. General Pop. Norm → 50.0

Mean SF-12 Score

Adults with PI report lower SF-12 Scores than adults in the U.S. General Population

38.3

37.2

44.3

45.5

N=924
Physical Component Summary*
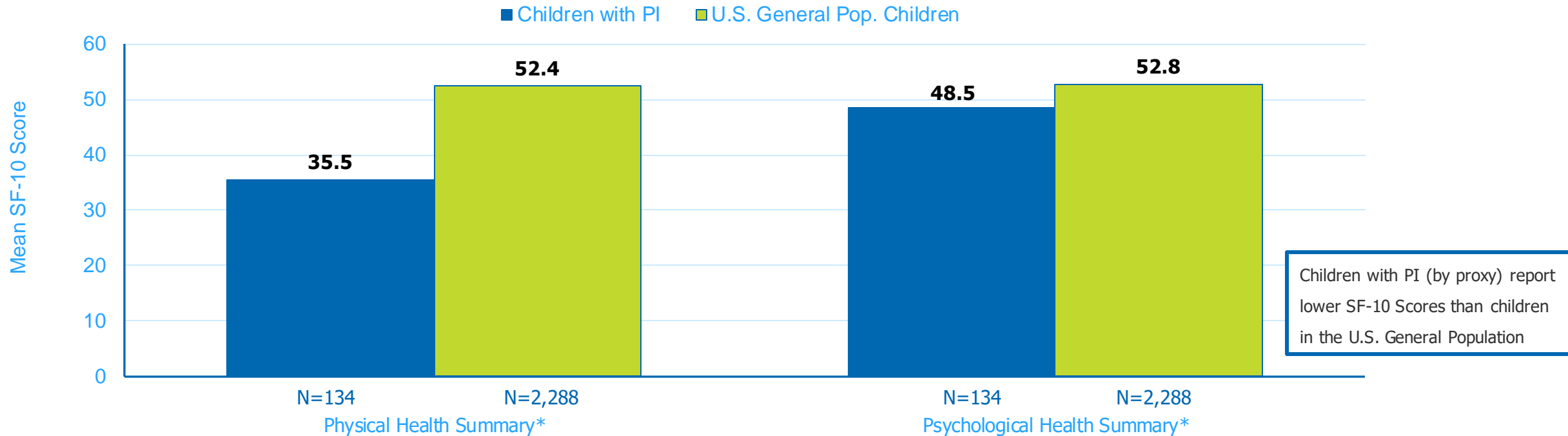
N=925
Mental Component Summary*

Median

* p<.00

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 with PI

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?

Source: IDF 2017 National Patient Survey

SF-10

■ Children with PI ■ U.S. General Pop. Children

Physical Health Summary*
- N=134 : 35.5
- N=2,288 : 52.4

Psychological Health Summary*
- N=134 : 48.5
- N=2,288 : 52.8

Mean SF-10 Score (y-axis: 0–60)

Children with PI (by proxy) report lower SF-10 Scores than children in the U.S. General Population

* p<.00

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…
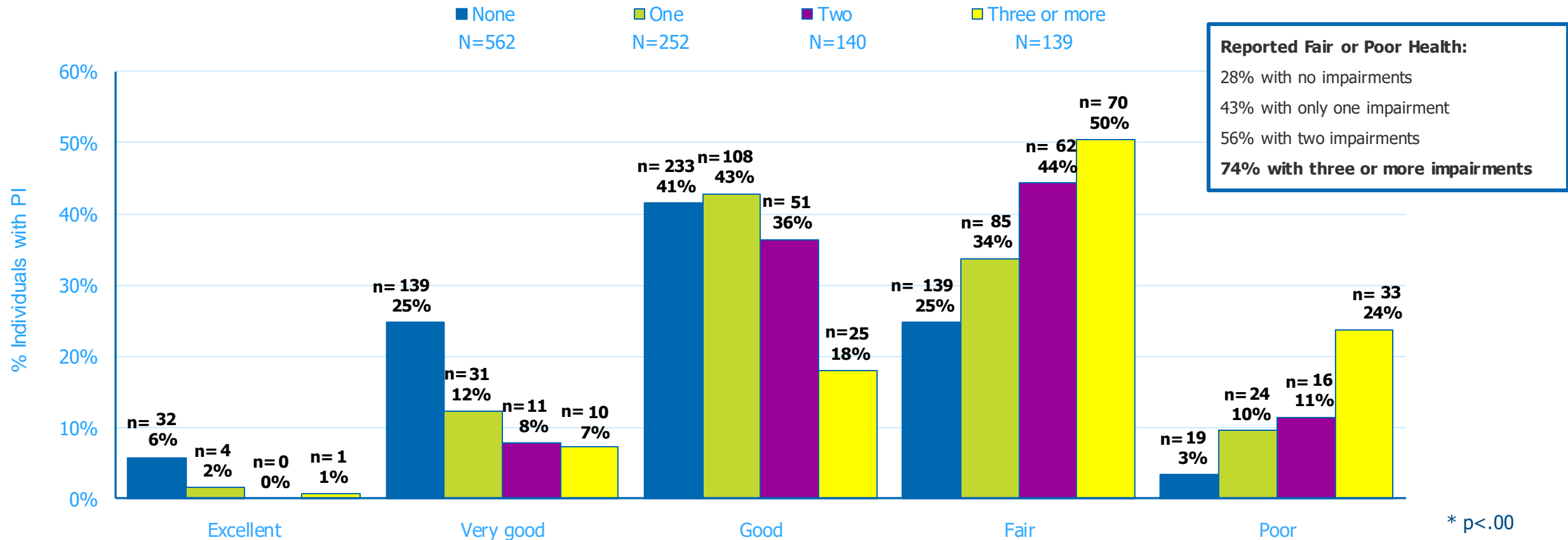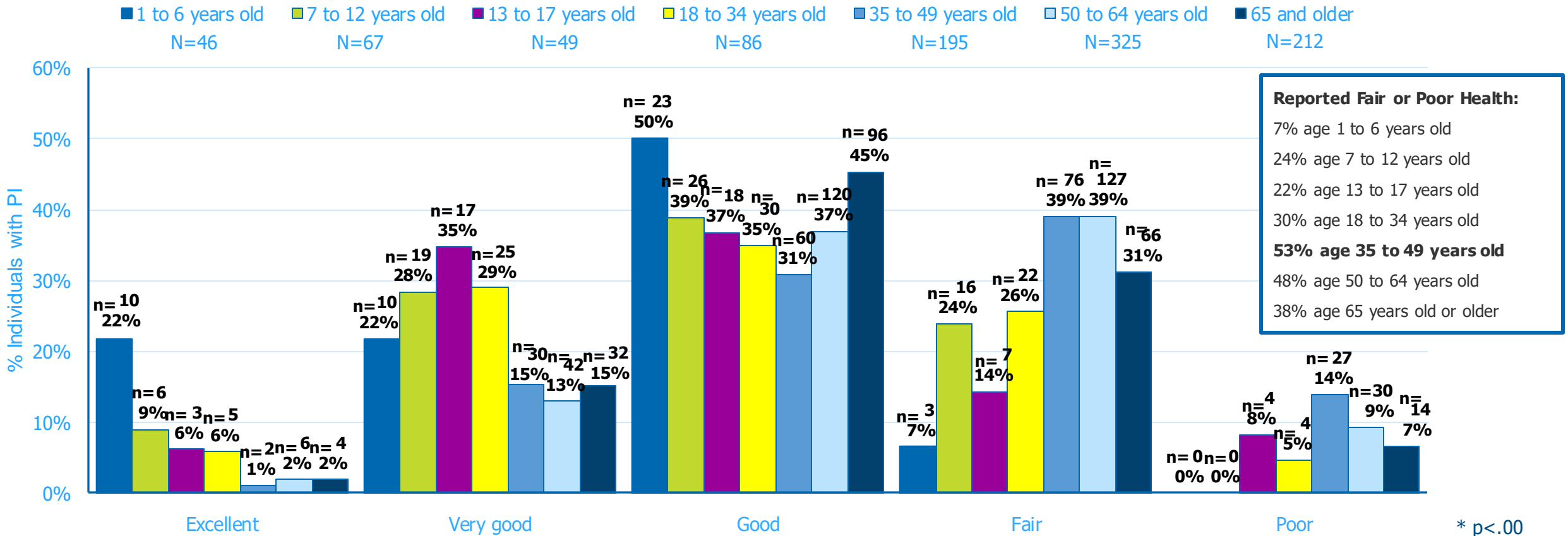Base: Children with PI

Source: IDF 2017 National Patient Survey
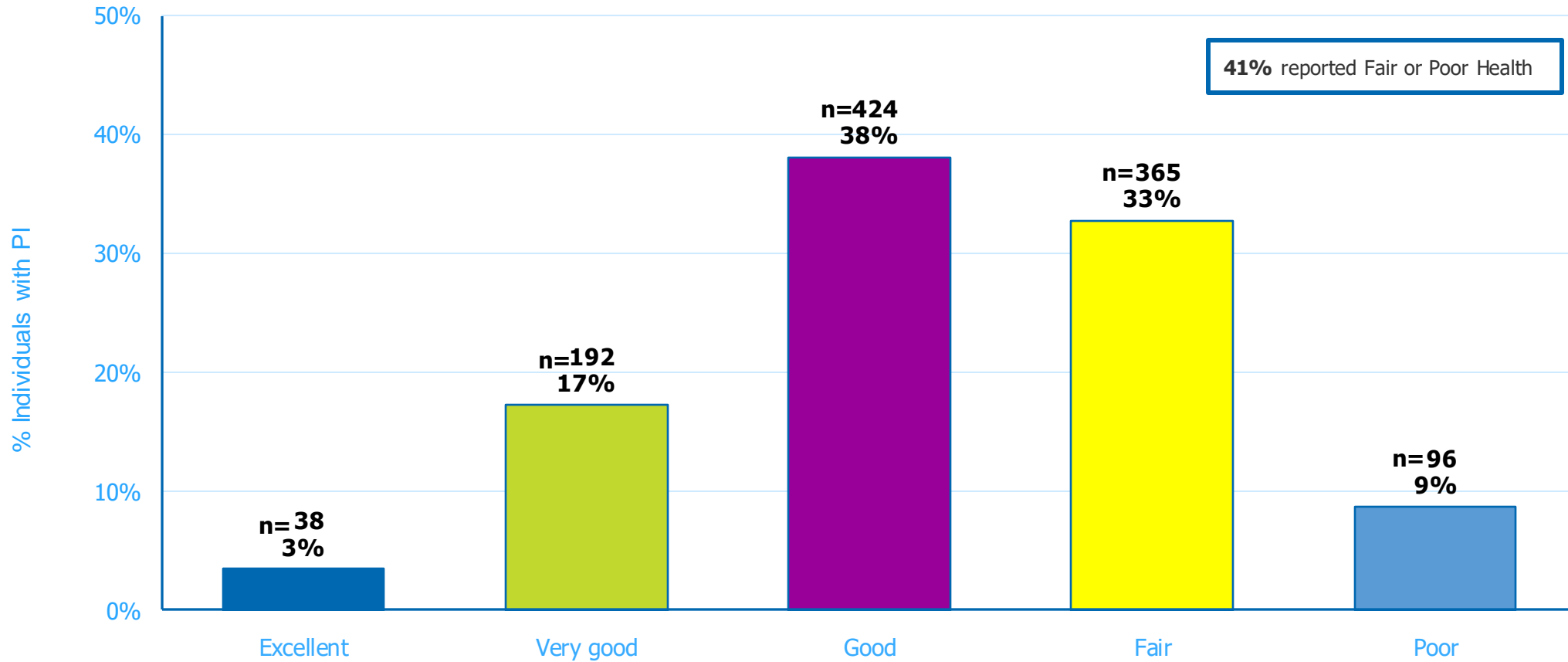
General Health

Q16. In general, would you say the patient's current health is... N=1,115
Base: All respondents

Source: IDF 2017 National Patient Survey

General Health by Permanent Impairments*

Legend:
- None N=562
- One N=252
- Two N=140
- Three or more N=139

Reported Fair or Poor Health:
28% with no impairments
43% with only one impairment
56% with two impairments
74% with three or more impairments

Y-axis: % Individuals with PI

Excellent
- None: n= 32, 6%
- One: n= 4, 2%
- Two: n= 0, 0%
- Three or more: n= 1, 1%

Very good
- None: n= 139, 25%
- One: n= 31, 12%
- Two: n= 11, 8%
- Three or more: n= 10, 7%

Good
- None: n= 233, 41%
- One: n= 108, 43%
- Two: n= 51, 36%
- Three or more: n= 25, 18%

Fair
- None: n= 139, 25%
- One: n= 85, 34%
- Two: n= 62, 44%
- Three or more: n= 70, 50%

Poor
- None: n= 19, 3%
- One: n= 24, 10%
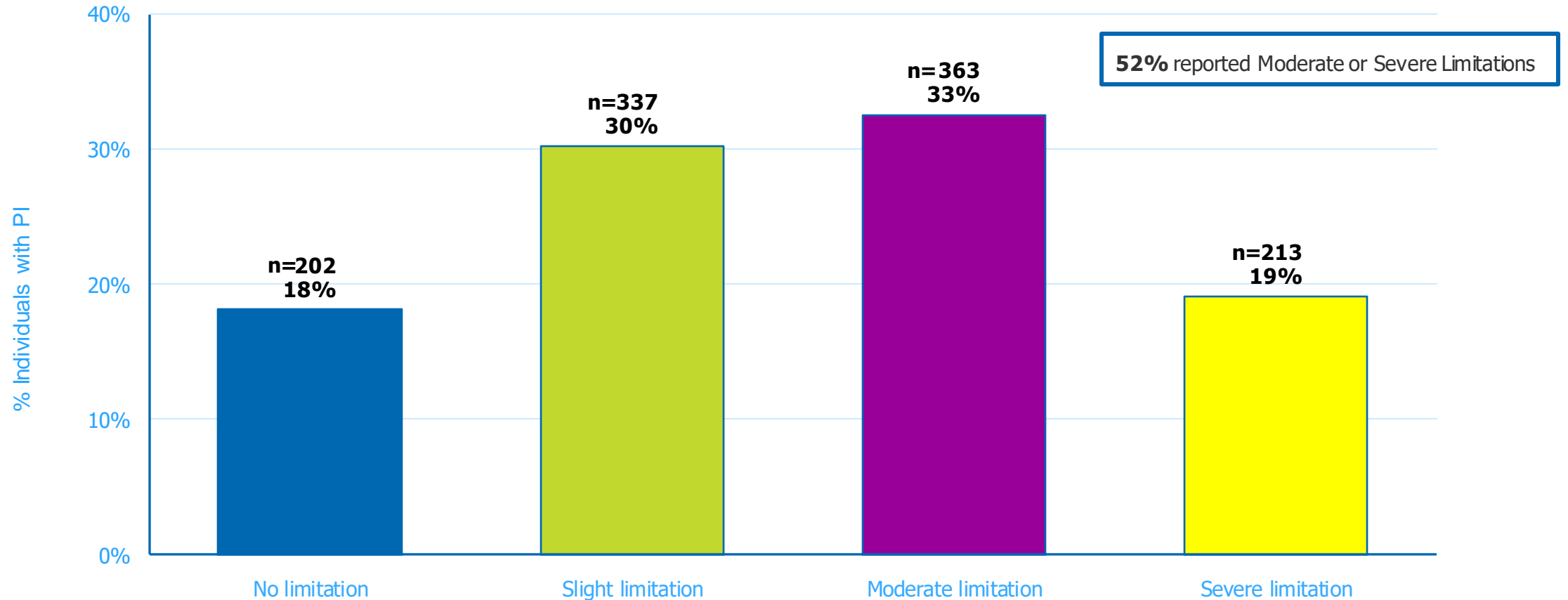- Two: n= 16, 11%
- Three or more: n= 33, 24%

* p<.00

Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of…? SELECT ALL THAT APPLY
Q16. In general, would you say the patient's current health is…
Base: All respondents

Source: IDF 2017 National Patient Survey

General Health by Age Group*

Source: IDF 2017 National Patient Survey

Physical Limitations

% Individuals with PI

- No limitation: n=202, 18%
- Slight limitation: n=337, 30%
- Moderate limitation: n=363, 33%
- Severe limitation: n=213, 19%

52% reported Moderate or Severe Limitations

Q17. How much is the patient currently limited in work, play or normal physical activity as a result of his/her physical heath? N=1,115
Base: All respondents

Physical Limitations by Permanent Impairments*
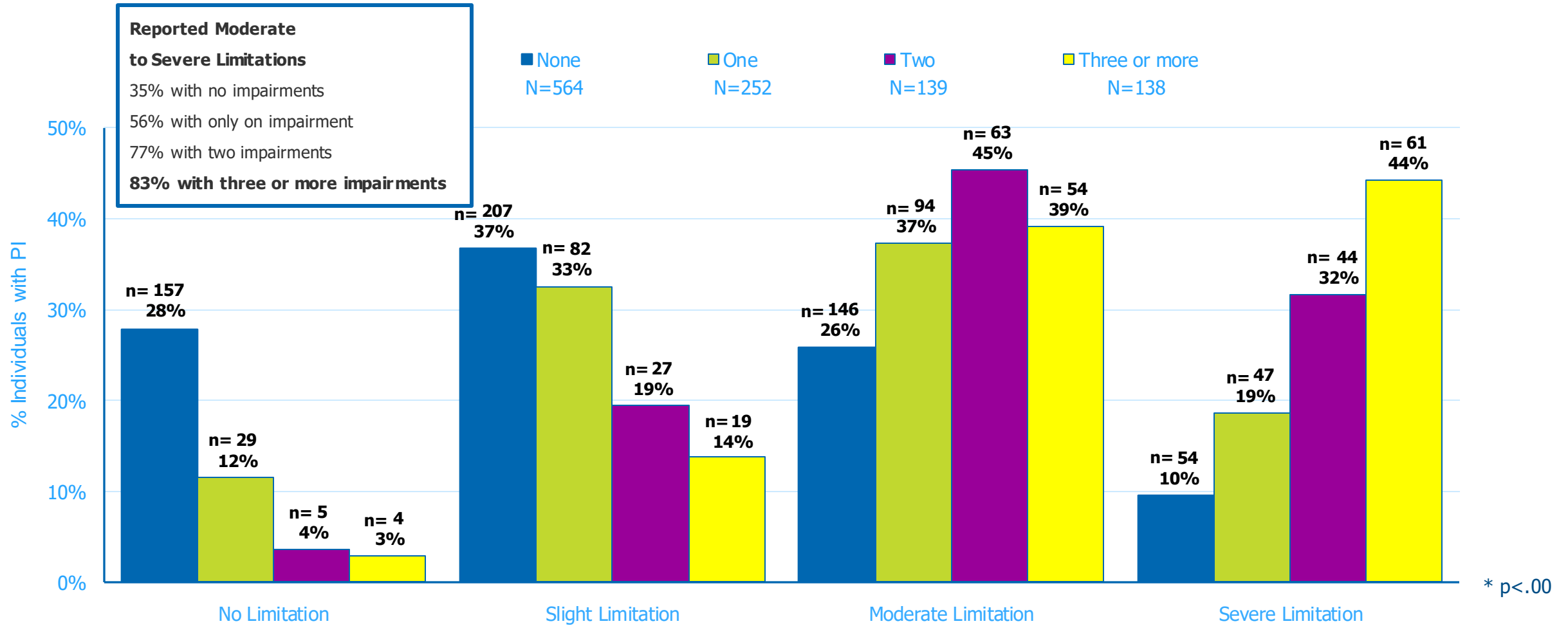
Reported Moderate to Severe Limitations

35% with no impairments
56% with only on impairment
77% with two impairments
83% with three or more impairments

■ None
N=564

■ One
N=252

■ Two
N=139

□ Three or more
N=138

% Individuals with PI

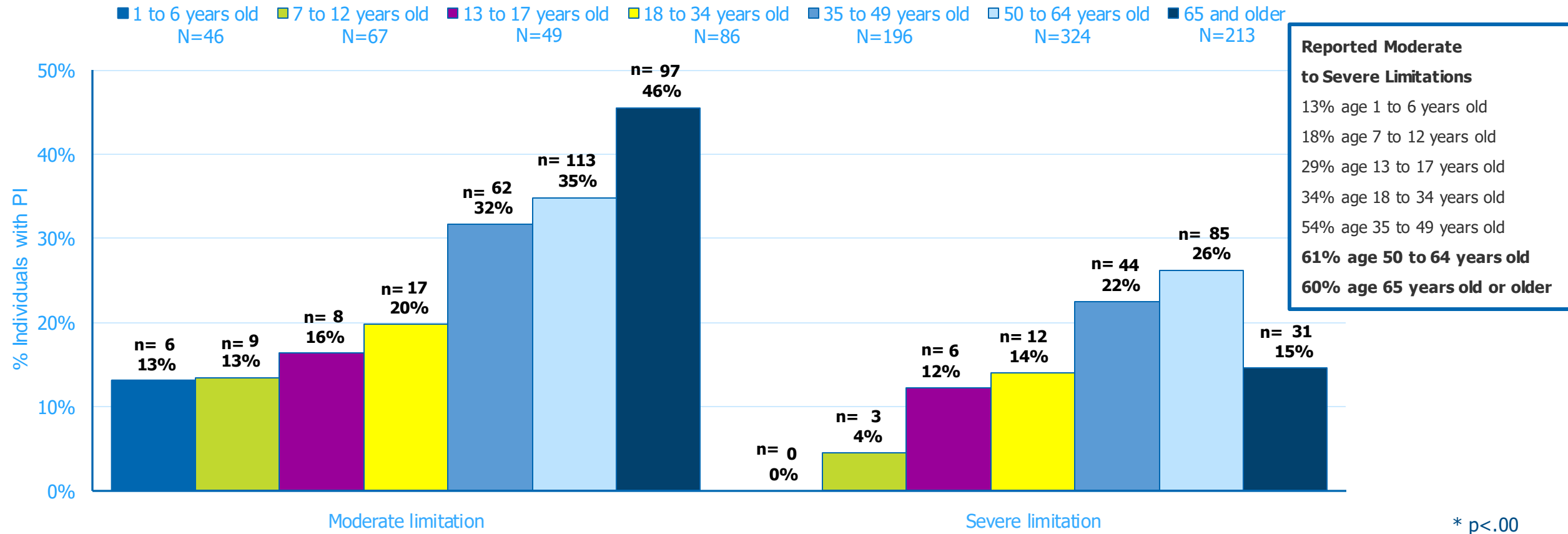**No Limitation**
- n= 157 / 28%
- n= 29 / 12%
- n= 5 / 4%
- n= 4 / 3%

Slight Limitation
- n= 207 / 37%
- n= 82 / 33%
- n= 27 / 19%
- n= 19 / 14%

Moderate Limitation
- n= 146 / 26%
- n= 94 / 37%
- n= 63 / 45%
- n= 54 / 39%

Severe Limitation
- n= 54 / 10%
- n= 47 / 19%
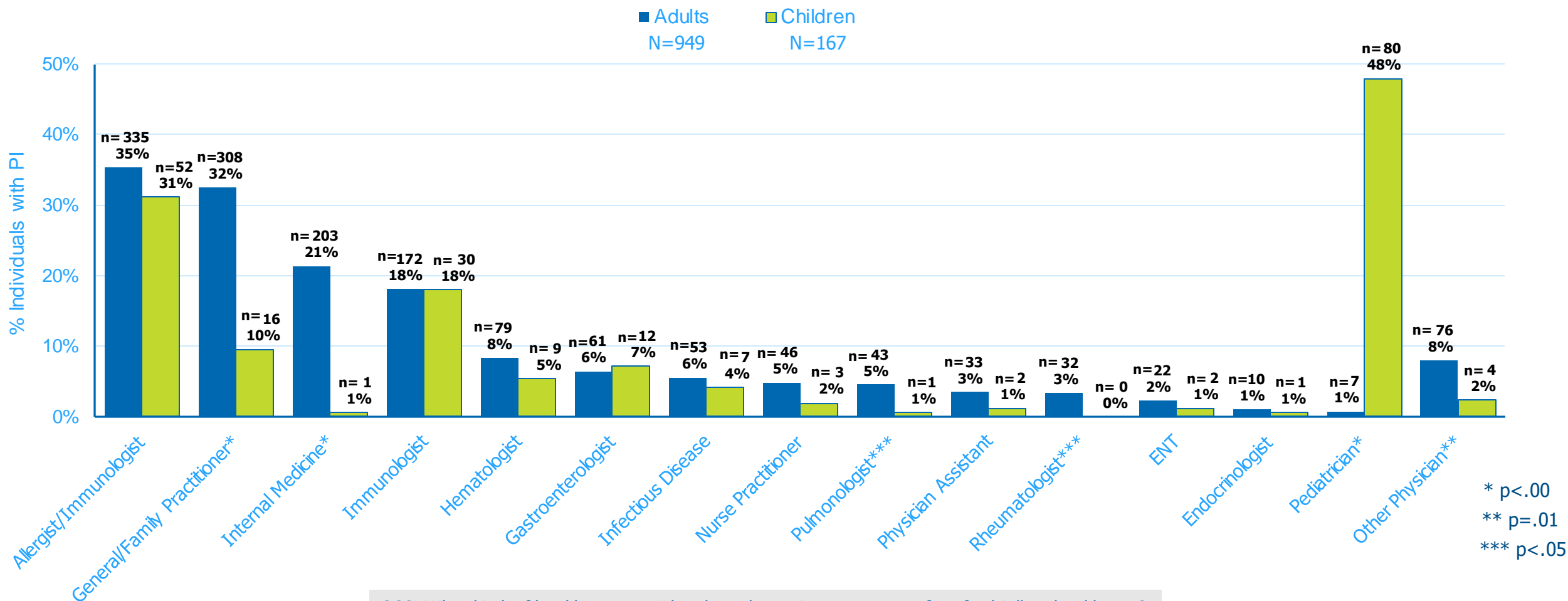- n= 44 / 32%
- n= 61 / 44%

50%
40%
30%
20%
10%
0%

* p<.00

Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of…?
Q17. How much is the patient currently limited in work, play or normal physical activity as a result of his/her physical health?
Base: All respondents

Source: IDF 2017 National Patient Survey

34

Physical Limitations by Age Group*

Legend:
- ■ 1 to 6 years old N=46
- ■ 7 to 12 years old N=67
- ■ 13 to 17 years old N=49
- ■ 18 to 34 years old N=86
- ■ 35 to 49 years old N=196
- ■ 50 to 64 years old N=324
- ■ 65 and older N=213

Reported Moderate to Severe Limitations
- 13% age 1 to 6 years old
- 18% age 7 to 12 years old
- 29% age 13 to 17 years old
- 34% age 18 to 34 years old
- 54% age 35 to 49 years old
- **61% age 50 to 64 years old**
- **60% age 65 years old or older**

Y-axis: % Individuals with PI (0% to 50%)

Moderate limitation:
- n= 6, 13%
- n= 9, 13%
- n= 8, 16%
- n= 17, 20%
- n= 62, 32%
- n= 113, 35%
- n= 97, 46%

Severe limitation:
- n= 0, 0%
- n= 3, 4%
- n= 6, 12%
- n= 12, 14%
- n= 44, 22%
- n= 85, 26%
- n= 31, 15%

* p<.00

Q3. What is the age and gender of these persons with PI?
Q17. How much is the patient currently limited in work, play or normal physical activity as a result of his/her physical heath?
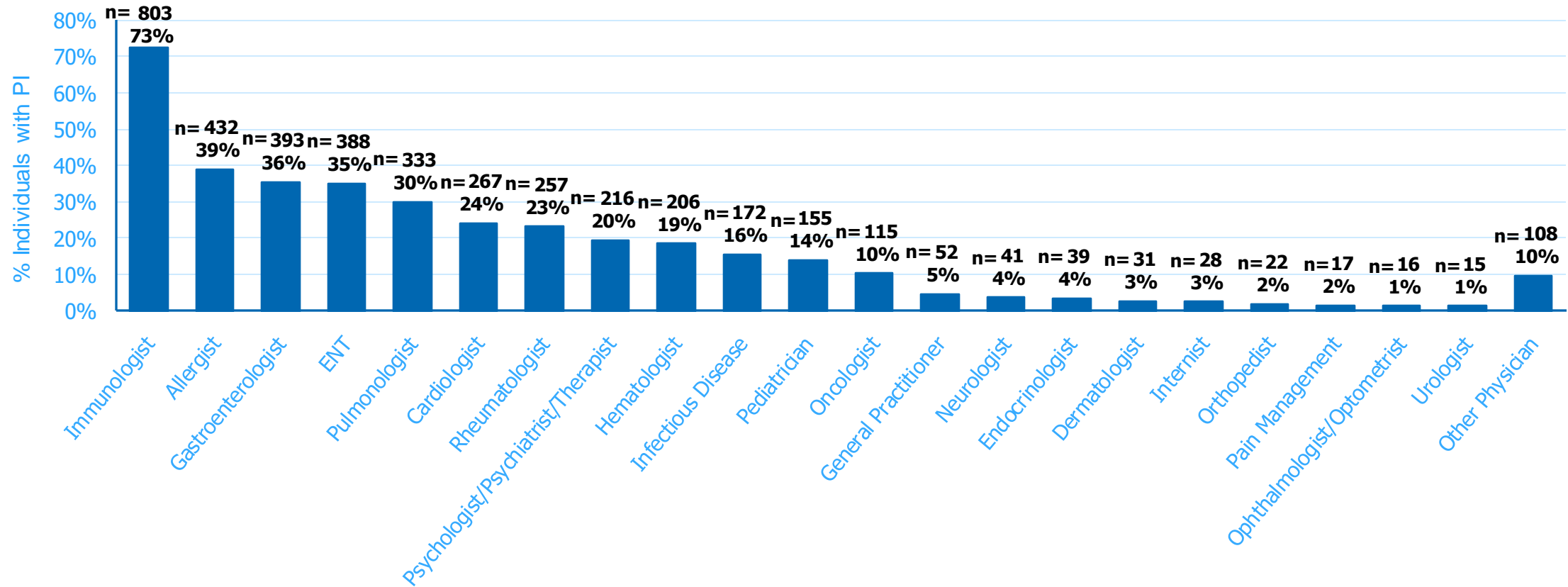Base: All respondents

Source: IDF 2017 National Patient Survey

Treating Physician by Age*

Legend: ■ Adults N=949 ■ Children N=167

Data labels by category:

- Allergist/Immunologist: Adults n= 335, 35%; Children n=52, 31%
- General/Family Practitioner*: Adults n=308, 32%; Children n= 16, 10%
- Internal Medicine*: Adults n= 203, 21%; Children n= 1, 1%
- Immunologist: Adults n=172, 18%; Children n= 30, 18%
- Hematologist: Adults n=79, 8%; Children n= 9, 5%
- Gastroenterologist: Adults n=61, 6%; Children n=12, 7%
- Infectious Disease: Adults n=53, 6%; Children n= 7, 4%
- Nurse Practitioner: Adults n= 46, 5%; Children n= 3, 2%
- Pulmonologist***: Adults n= 43, 5%; Children n=1, 1%
- Physician Assistant: Adults n= 33, 3%; Children n= 2, 1%
- Rheumatologist***: Adults n= 32, 3%; Children n=0, 0%
- ENT: Adults n= 22, 2%; Children n= 2, 1%
- Endocrinologist: Adults n=10, 1%; Children n= 1, 1%
- Pediatrician*: Adults n= 7, 1%; Children n= 80, 48%
- Other Physician**: Adults n= 76, 8%; Children n= 4, 2%

Y-axis: % Individuals with PI (0% – 50%)

* p<.00
** p=.01
*** p<.05

Q22. What kind of healthcare provider does the patient see most often for his/her healthcare?
Base: All respondents

Source: IDF 2017 National Patient Survey

36

Seen Immunologist in Past 12 Months

■ Yes ■ No

n=919
82%

n=197
18%
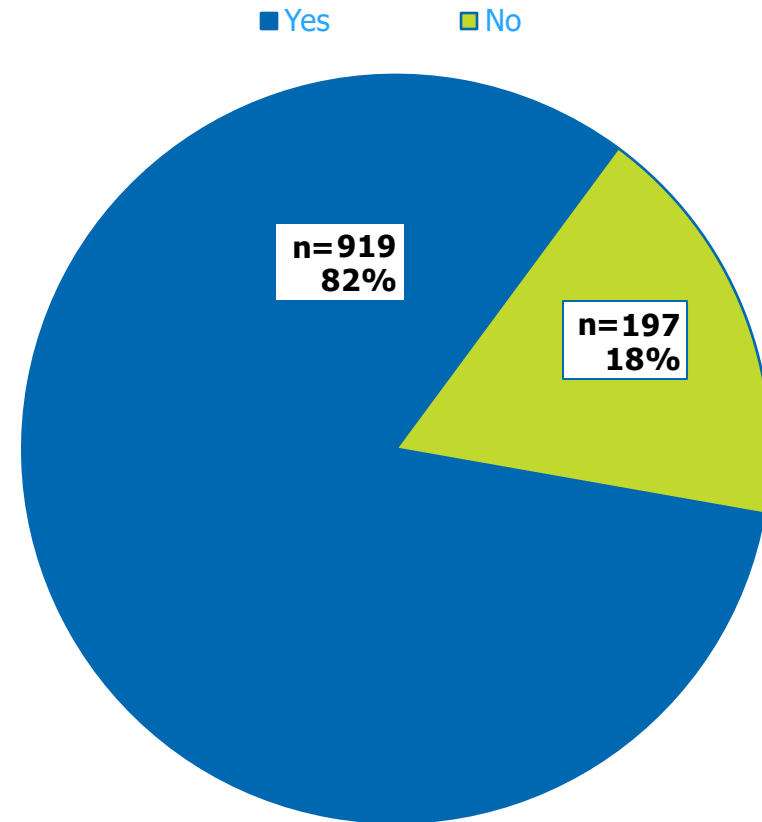
Q23a. Has he/she been seen by an immunologist during the last 12 months? N=1,116
Base: All respondents
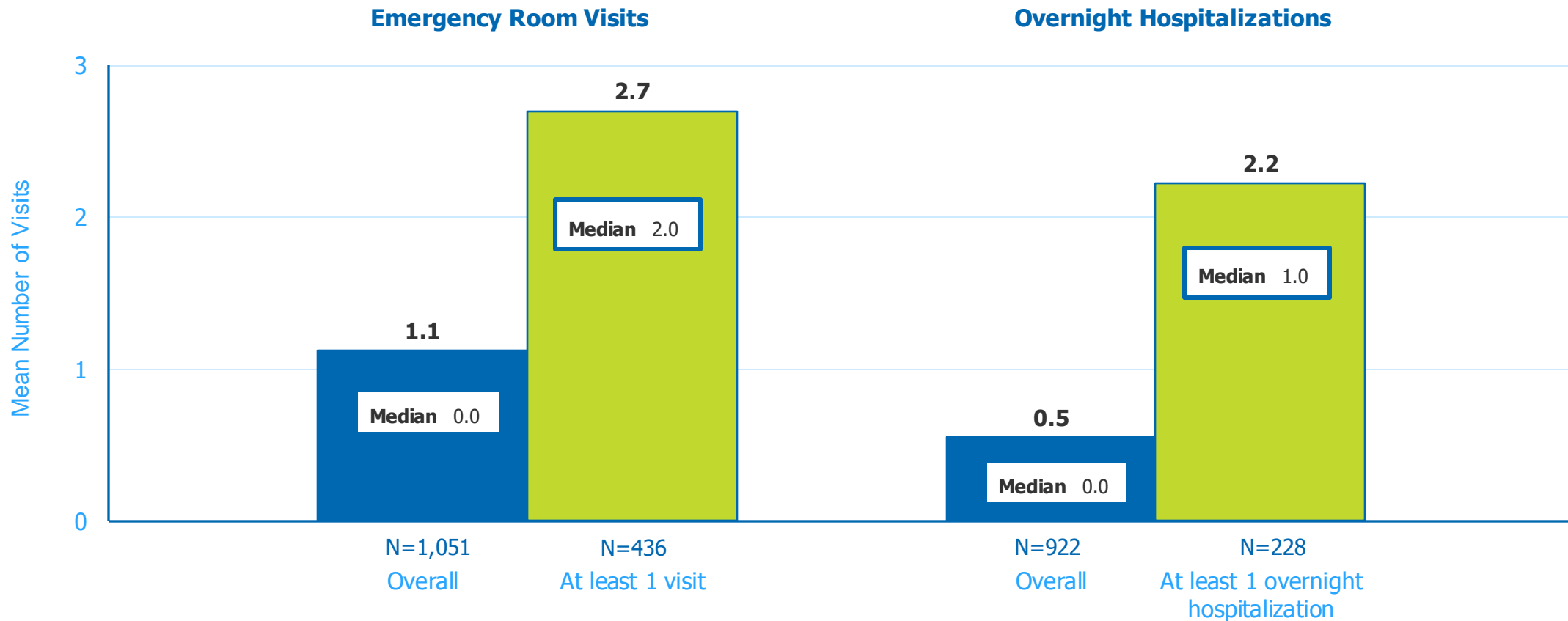
Specialists Seen in Past 12 Months

Q24a. What kind of healthcare providers, if any, has the patient seen for his/her healthcare in the past 12 months? SELECT ALL THAT APPLY N=1,105
Base: All respondents

Hospitalizations

Mean Number of Times

4.2

Median 1.0

N=1,098
Prior to Diagnosis

2.8

Median 0.0

N=1,036
Past 12 Months
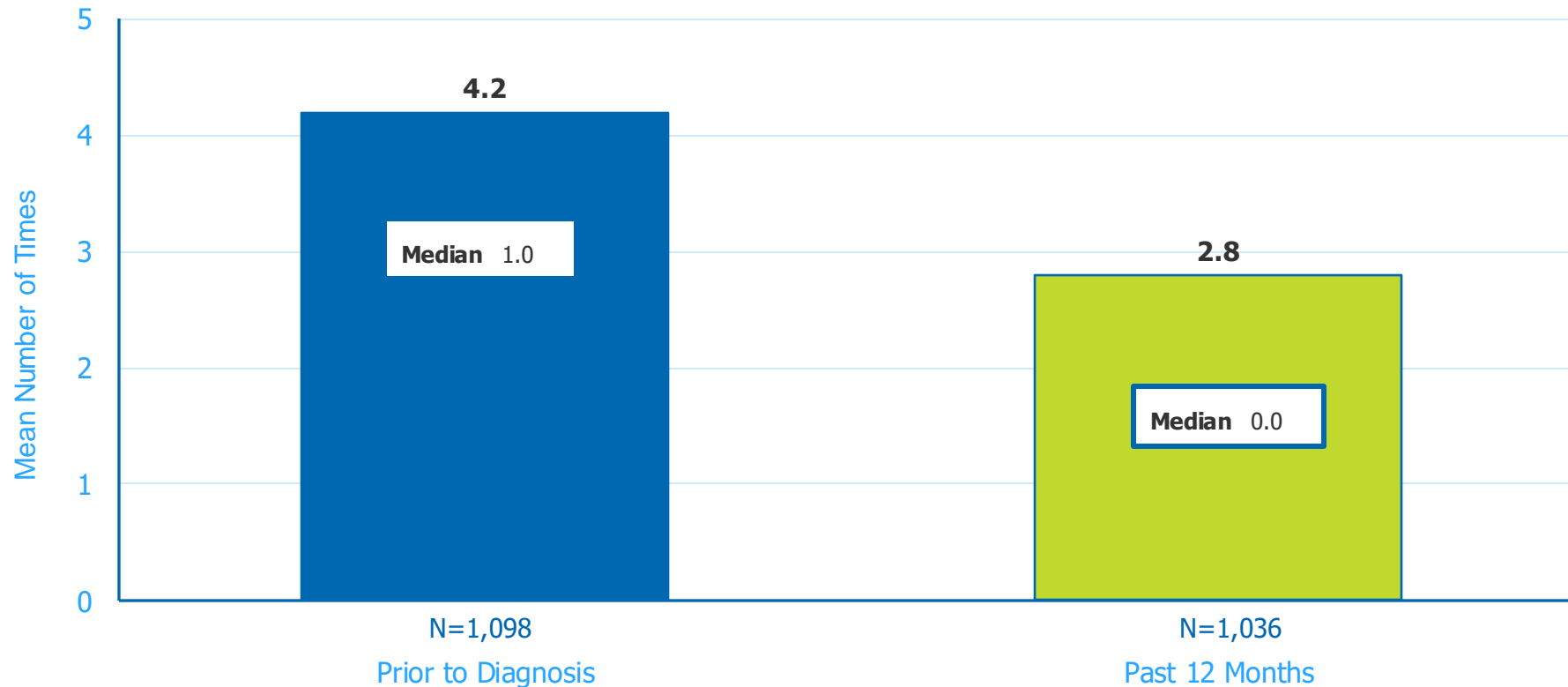
Q12. How many times had he/she been hospitalized BEFORE DIAGNOSIS as immune deficient?
Q13. How many times has he/she been hospitalized SINCE DIAGNOSIS as immune deficient?
Base: All respondents

ER Visits and Overnight Hospitalizations

Emergency Room Visits

- **2.7** — At least 1 visit
  - Median 2.0
- **1.1** — Overall
  - Median 0.0

Mean Number of Visits

N=1,051 — Overall
N=436 — At least 1 visit

Overnight Hospitalizations

- **2.2** — At least 1 overnight hospitalization
  - Median 1.0
- **0.5** — Overall
  - Median 0.0

N=922 — Overall
N=228 — At least 1 overnight hospitalization

Q25. How many emergency room visits and/or hospitalizations during the past 12 months, NOT COUNTING INFUSIONS. Please enter "0" if none. Base: All respondents

Source: IDF 2017 National Patient Survey

Insurance

■ Private   ■ Public   ■ Both Private and Public   □ Other/Not Sure   ■ No Insurance

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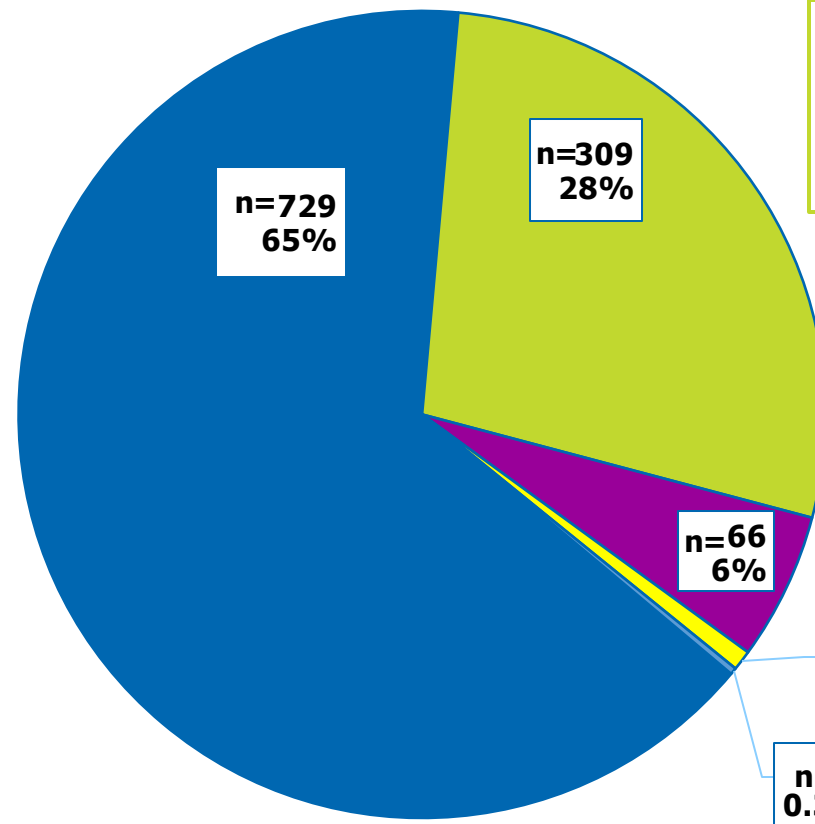
Public Insurance
Medicare
Medicare due to disability
Medicaid
TRICARE/Veterans Policy
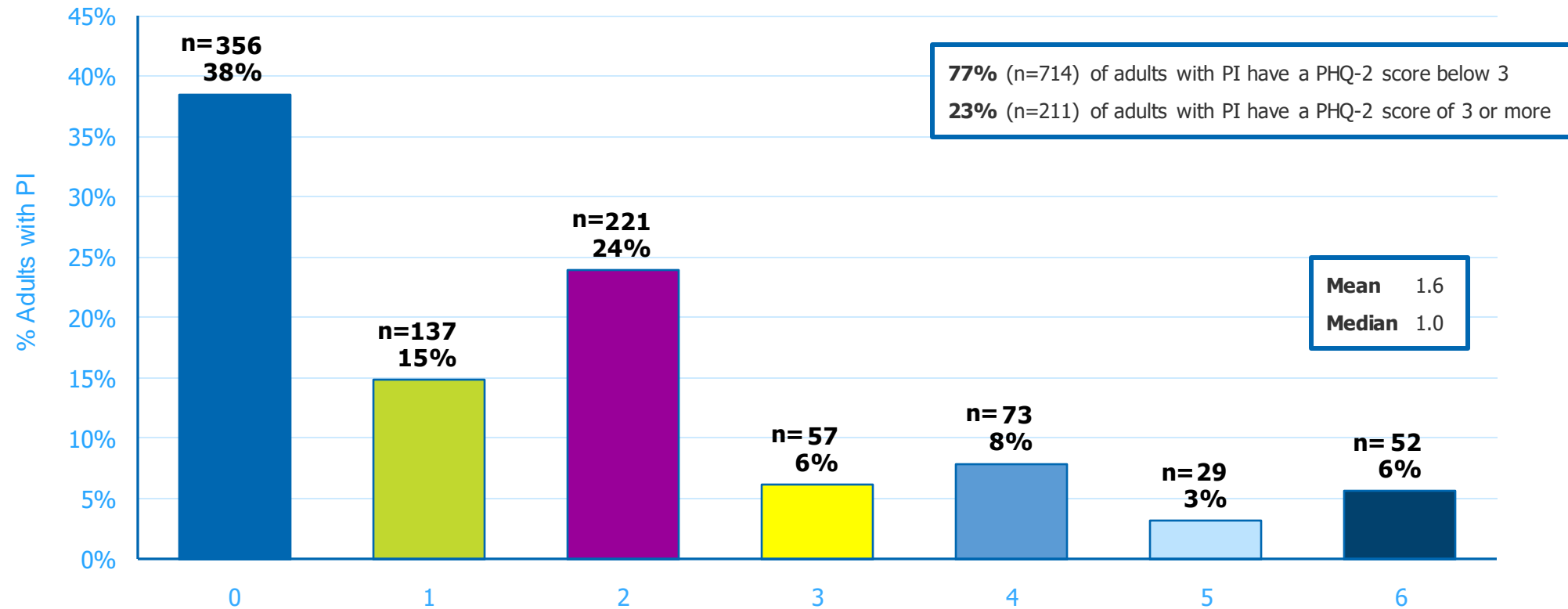Children's Health Insurance Plan

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

n=729
65%

n=309
28%

n=66
6%

n= 9
1%

n= 3
0.3%

Q42. What kind of health insurance does the patient have? N=1,116
Base: All respondents

41

Mental Health

PHQ-2

77% (n=714) of adults with PI have a PHQ-2 score below 3

23% (n=211) of adults with PI have a PHQ-2 score of 3 or more

Mean	1.6
Median	1.0

% Adults with PI

- 0: n=356, 38%
- 1: n=137, 15%
- 2: n=221, 24%
- 3: n=57, 6%
- 4: n=73, 8%
- 5: n=29, 3%
- 6: n=52, 6%

Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
N=925
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder
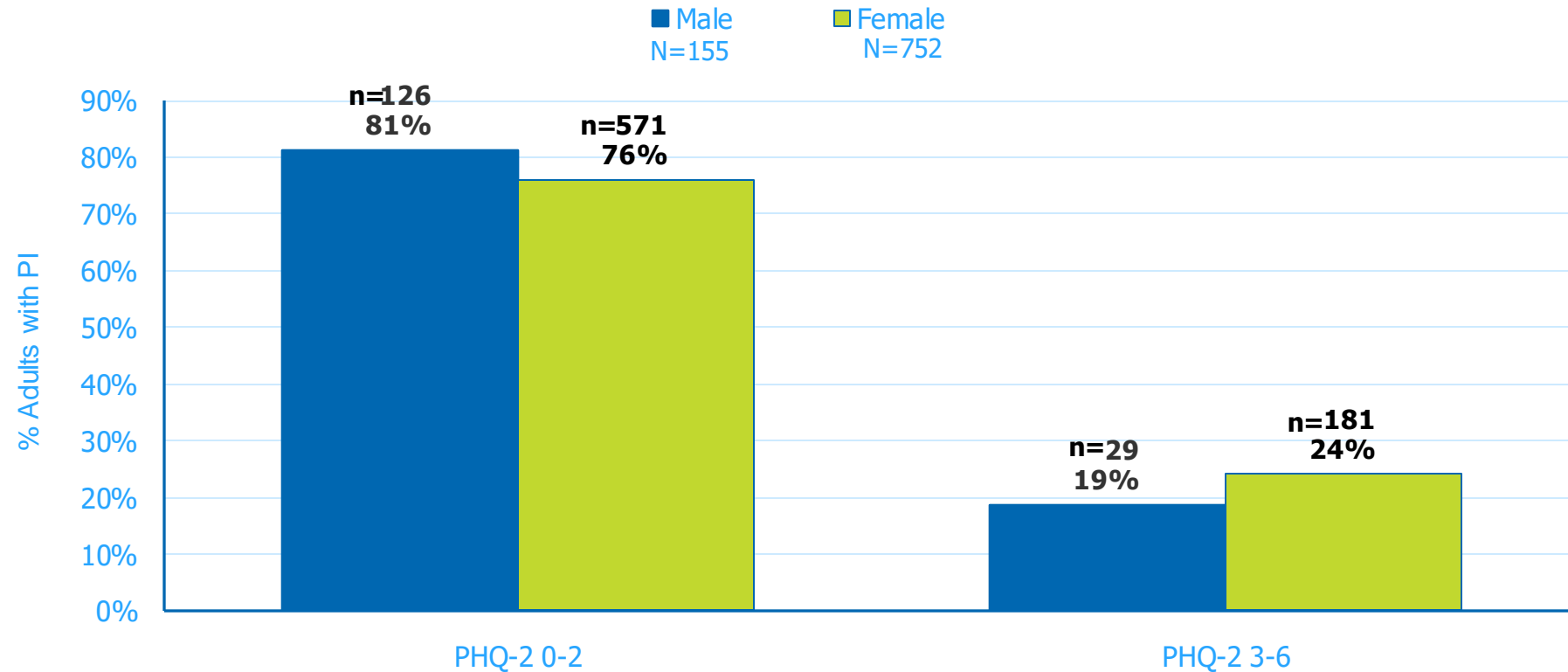
Source: IDF 2017 National Patient Survey

43

PHQ-2 Scores 3 or more
U.S. General Population versus Adults with PI*

Adults with PI were more likely to have a PHQ-2 score of 3 or more than Adults in the U.S. General Population

% Adults

30%

20%

n=211
23%

10%

n=2,139
9%

0%

N=23,770
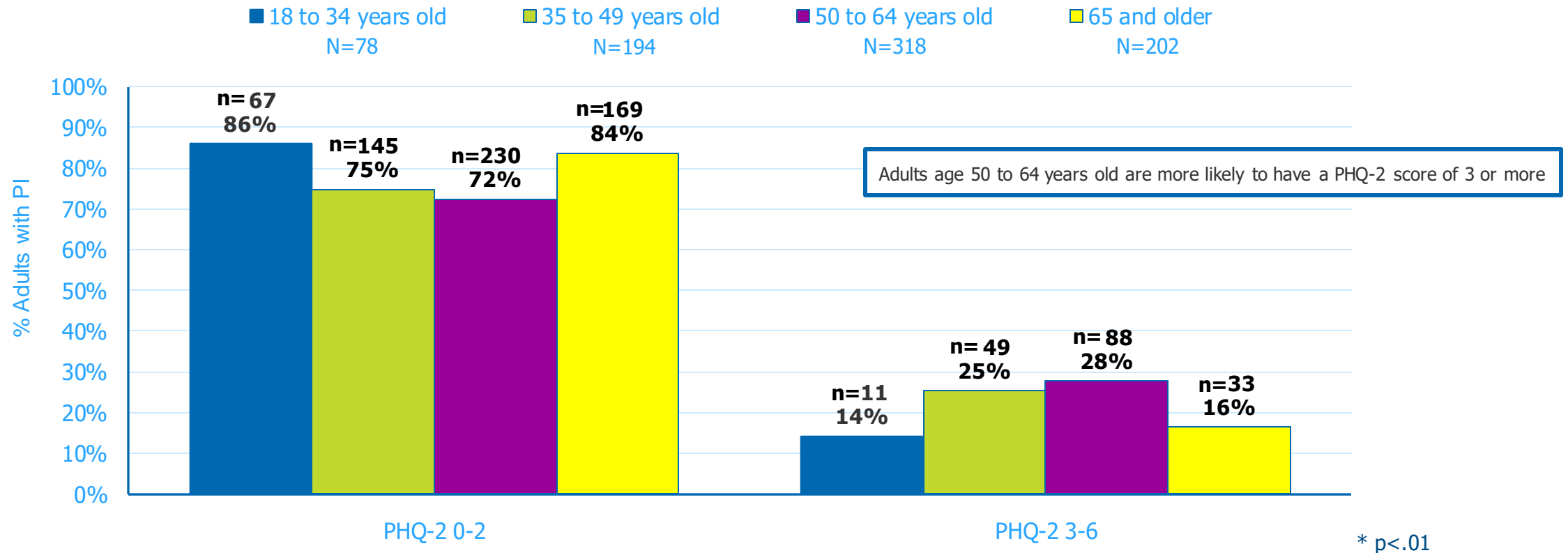U.S. General Pop.

N=925
Adults with PI

* p<.00

Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder

Source: IDF 2017 National Patient Survey

PHQ-2 Score by Gender

■ Male
N=155

■ Female
N=752

% Adults with PI

n=126
81%

n=571
76%

n=29
19%

n=181
24%

90%
80%
70%
60%
50%
40%
30%
20%
10%
0%

PHQ-2 0-2

PHQ-2 3-6

Q3. What is the age and gender of these persons with PI?
Q19a. Little interest or pleasure in doing things
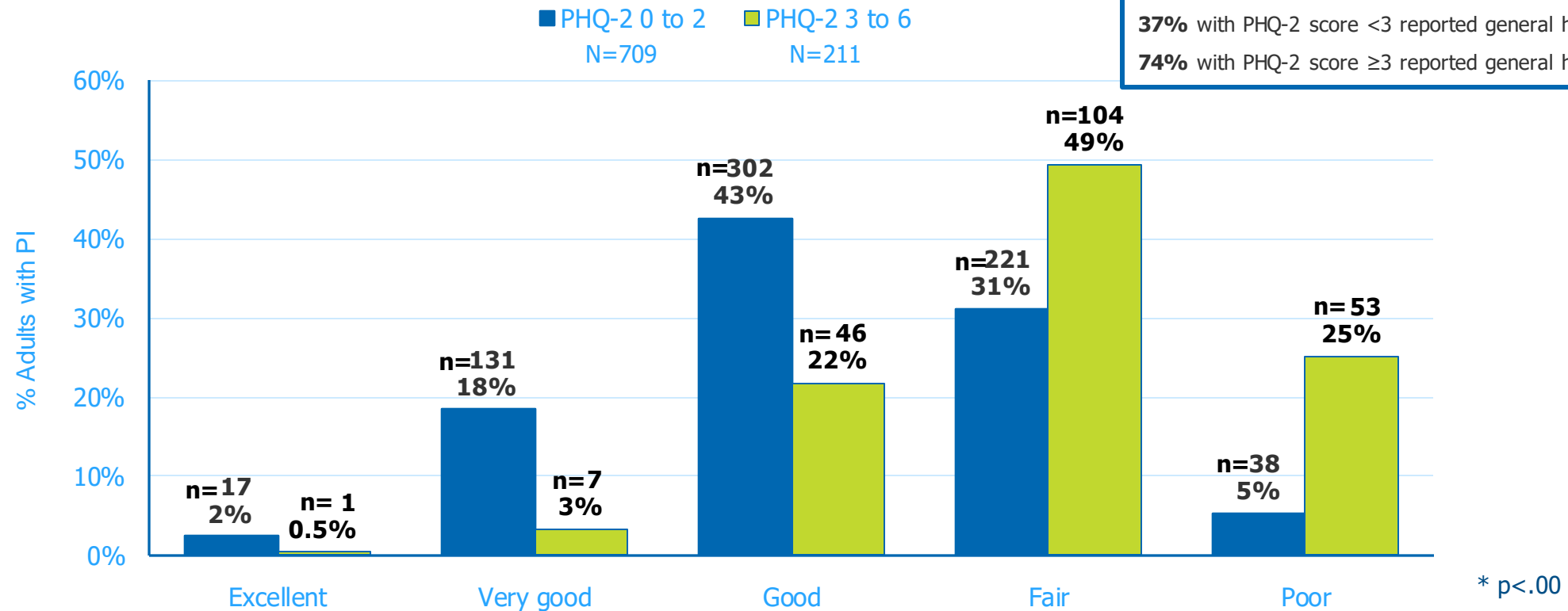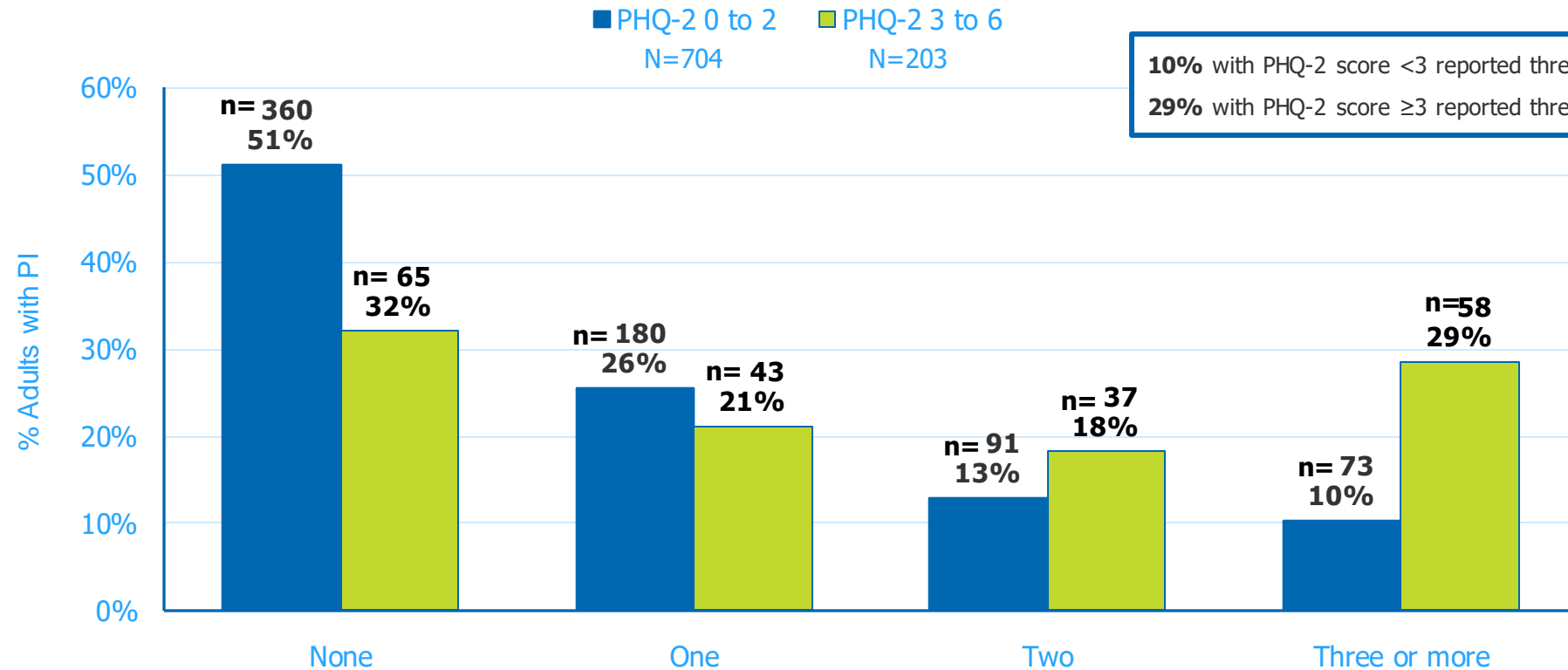Q19b. Feeling down, depressed or hopeless
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder

45

Source: IDF 2017 National Patient Survey

PHQ-2 Score by Age Group*

Legend:
- 18 to 34 years old N=78
- 35 to 49 years old N=194
- 50 to 64 years old N=318
- 65 and older N=202

% Adults with PI

PHQ-2 0-2:
- n=67, 86%
- n=145, 75%
- n=230, 72%
- n=169, 84%

PHQ-2 3-6:
- n=11, 14%
- n=49, 25%
- n=88, 28%
- n=33, 16%

Adults age 50 to 64 years old are more likely to have a PHQ-2 score of 3 or more

* p<.01

Q3. What is the age and gender of these persons with PI?
Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder
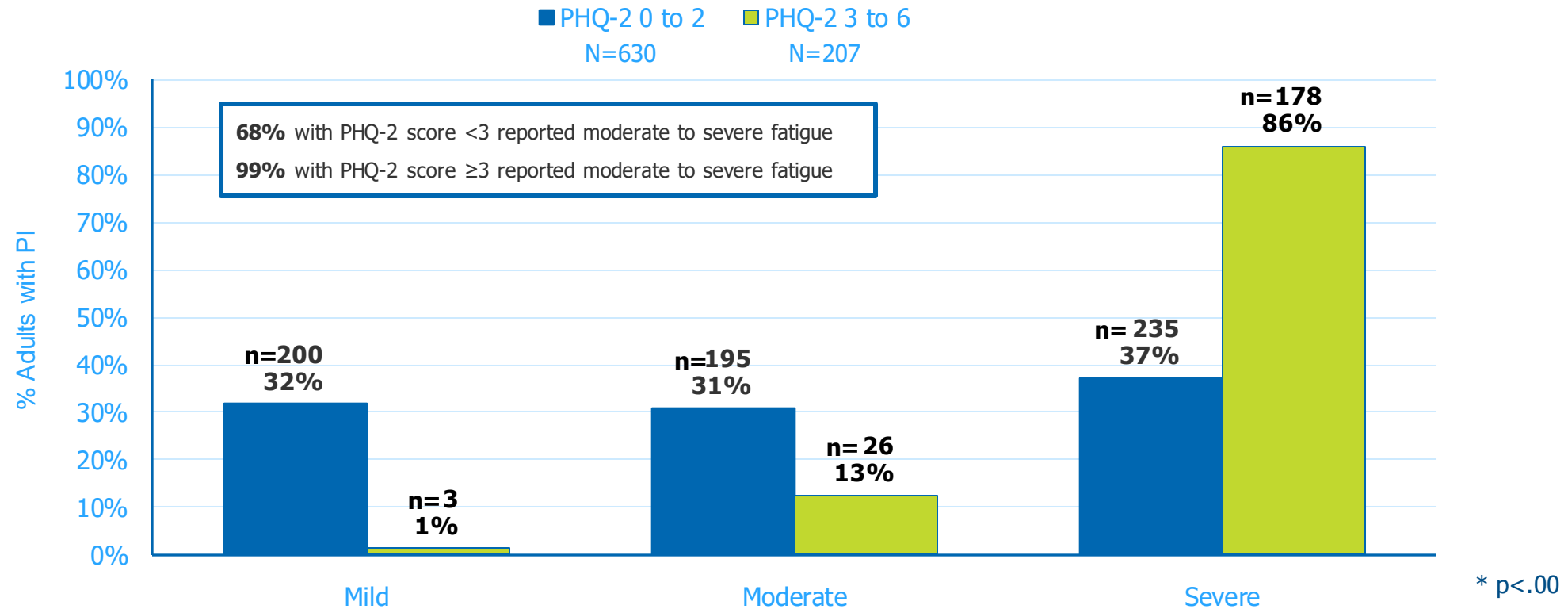
PHQ-2 Score by General Health*

Legend: ■ PHQ-2 0 to 2 N=709   ■ PHQ-2 3 to 6 N=211

37% with PHQ-2 score <3 reported general health as fair or worse
74% with PHQ-2 score ≥3 reported general health as fair or worse

Y-axis: % Adults with PI (0% to 60%)

X-axis categories: Excellent, Very good, Good, Fair, Poor

Excellent: n=17 2% (blue), n= 1 0.5% (green)
Very good: n=131 18% (blue), n=7 3% (green)
Good: n=302 43% (blue), n= 46 22% (green)
Fair: n=221 31% (blue), n=104 49% (green)
Poor: n=38 5% (blue), n= 53 25% (green)

* p<.00

Q16. In general, would you say the patient's current health is:
Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder

Source: IDF 2017 National Patient Survey

47

PHQ-2 Score by Permanent Impairments*

■ PHQ-2 0 to 2
N=704

■ PHQ-2 3 to 6
N=203

10% with PHQ-2 score <3 reported three or more permanent impairments

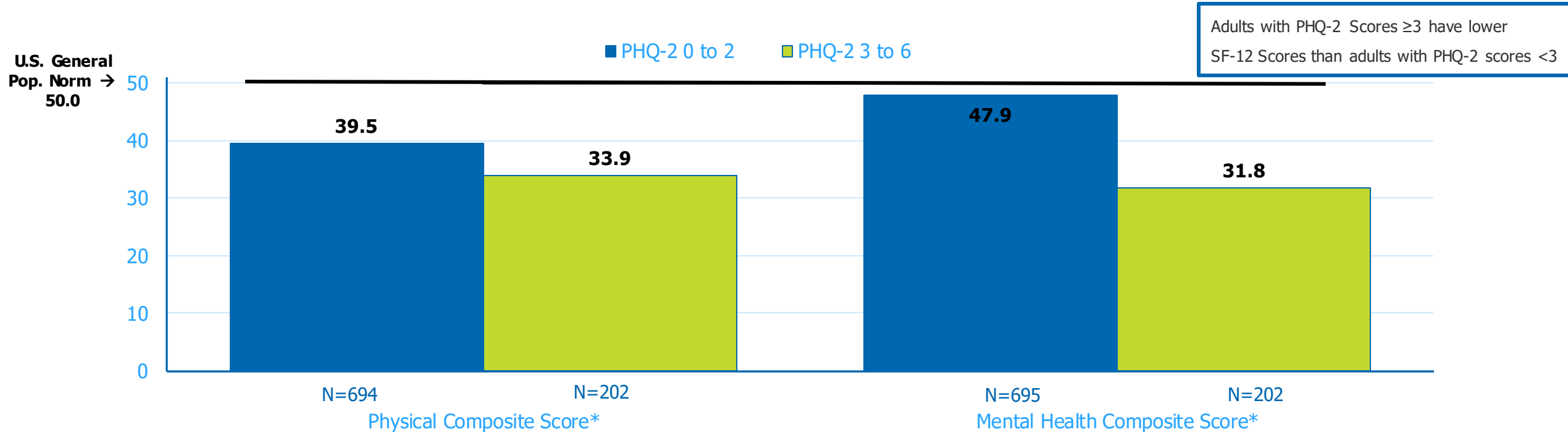**29%** with PHQ-2 score ≥3 reported three or more permanent impairments

% Adults with PI

n= 360
51%

n= 65
32%

n= 180
26%

n= 43
21%

n= 91
13%

n= 37
18%

n= 73
10%

n=58
29%

None

One

Two

Three or more

* p<.00

Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of...? SELECT ALL THAT APPLY
Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder

PHQ-2 Score by BFI*

■ PHQ-2 0 to 2
N=630

■ PHQ-2 3 to 6
N=207

68% with PHQ-2 score <3 reported moderate to severe fatigue

99% with PHQ-2 score ≥3 reported moderate to severe fatigue

% Adults with PI

n=200
32%

n=3
1%

n=195
31%

n= 26
13%

n= 235
37%

n=178
86%

Mild

Moderate

Severe

* p<.00

Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Global BFI Score
Base: Adults with PI
Note[1]: PHQ-2 scores greater than 2 indicate a possible depressive disorder
Note[2]: 1-4=Mild; 5-6=Moderate; 7-10=Severe

Source: IDF 2017 National Patient Survey

PHQ-2 Score by Mean SF-12 Scores*

U.S. General Pop. Norm → 50.0

■ PHQ-2 0 to 2   ■ PHQ-2 3 to 6

Adults with PHQ-2 Scores ≥3 have lower SF-12 Scores than adults with PHQ-2 scores <3

Physical Composite Score*
- 39.5 (N=694)
- 33.9 (N=202)

Mental Health Composite Score*
- 47.9 (N=695)
- 31.8 (N=202)

Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
VT02. Did you have a lot of energy?
SF02. How much of the time has your physical health or emotional problems interfered with your social activities?
RE02. Accomplished less than you would like
RE03. Did work or other activities less carefully than usual
MH03. Have you felt calm and peaceful?
MH04. Have you felt downhearted and depressed?
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder

* p<.00

Source: IDF 2017 National Patient Survey

50

Limitations due to Emotional Problems

26% of adults with PI report moderate to severe limitations due to emotional problems

Chart: % Adults with PI

- No limitation: n= 401, 42%
- Slight limitation: n=301, 32%
- Moderate limitation: n=159, 17%
- Severe limitation: n=88, 9%
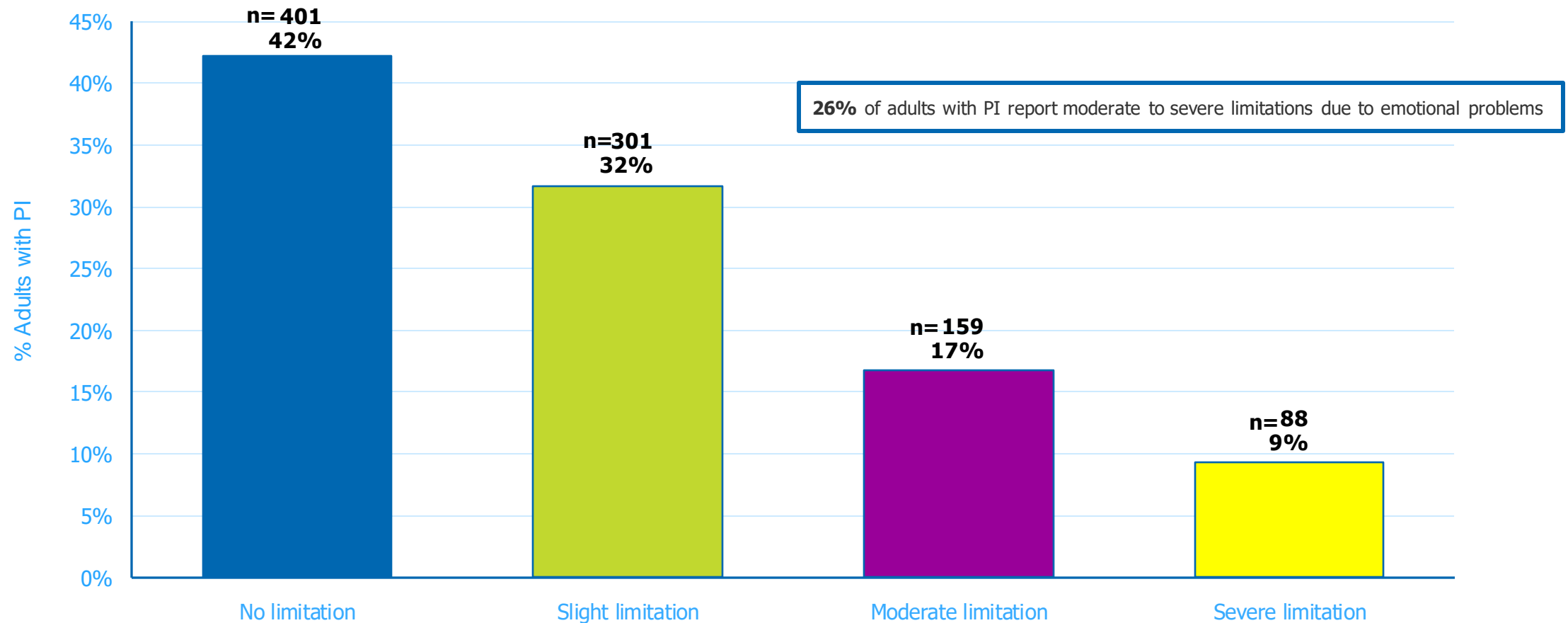
Q18. How much is the patient currently limited in work, play or normal activities as a result of any emotional problems (such as feeling depressed or anxious)? N=949
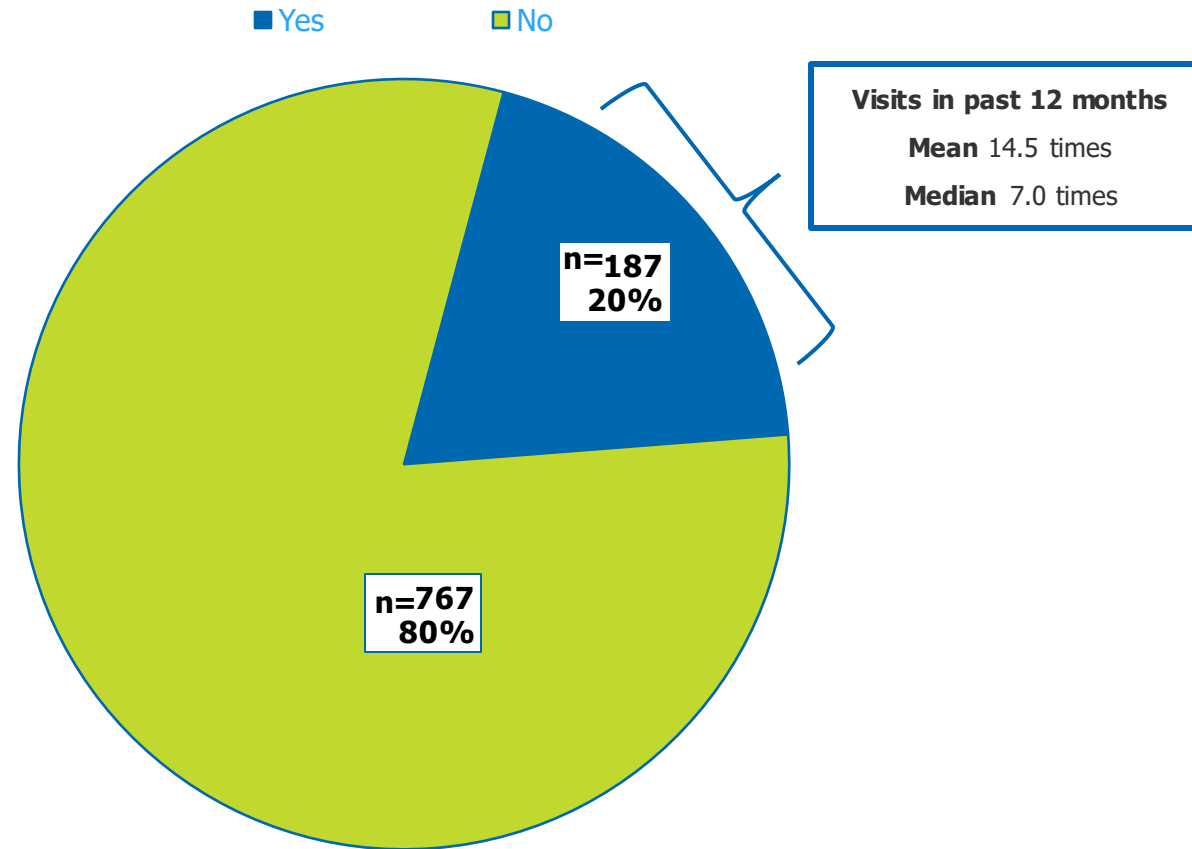Base: Adults with PI

Moderate to Severe Limitations Due to Emotional Problems by PHQ-2, Gender, Age and Permanent Impairments

	Moderate to Severe Limitation
PHQ-2*	
0 to 2 (N=712)	13%
3 to 6 (N=210)	**74%**
Gender	
Male (N=162)	22%
Female (N=769)	**27%**
Age*	
18 to 34 (N=81)	17%
35 to 49 (N=195)	**31%**
50 to 64 (N=325)	29%
65 and older (N=212)	22%
Permanent Impairments*	
None (N=436)	18%
One (N=229)	25%
Two (N=130)	33%
Three or More (N=135)	**44%**

Q3. What is the age and gender of these persons with PI?
Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of...?
Q18. How much is the patient currently limited in work, play or normal activities as a result of any emotional problems (such as feeling depressed or anxious)?
Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder

* p<.00

Seeing a Mental Health Professional

■ Yes   ■ No

n=187
20%

n=767
80%

Visits in past 12 months
Mean 14.5 times
Median 7.0 times

Q24a_10. What kind of healthcare providers, if any, has the patient seen for his/her healthcare in the past 12 months? Psychologist/Psychiatrist/Therapist N=954
Q24b_10. How many visits to these specialists in the past 12 months? N=165
Base: Adults with PI

Source: IDF 2017 National Patient Survey

Seeing a Mental Health Professional by PHQ-2, Gender, Age and Permanent Impairments

	Saw a Mental Health Professional	Mean Number of Visits
PHQ-2*/***		
0 to 2 (N=93/N=80)	13%	11.6
3 to 6 (N=88/ N=82)	**74%**	**17.4**
Gender*		
Male (N=162/N=12)	8%	13.0
Female (N=744/N=152)	**22%**	**14.6**
Age*		
18 to 34 (N=82/N=16)	22%	**21.6**
35 to 49 (N=196/N=50)	**28%**	14.6
50 to 64 (N=327/N=58)	20%	13.6
65 and older (N=213/N=19)	10%	11.5
Permanent Impairments*/**		
None (N=438/N=68)	17%	11.0
One (N=230/N=34)	17%	**20.8**
Two (N=131/N=23)	21%	17.7
Three or More (N=136/N=38)	**31%**	13.7

Q3. What is the age and gender of these persons with PI?
Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of...?
Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Q24a_10. What kind of healthcare providers, if any, has the patient seen for his/her healthcare in the past 12 months? Psychologist/Psychiatrist/Therapist
Q24b_10. How many visits to these specialists in the past 12 months?
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder

* p<.00
** p<.01
*** p<.05

Source: IDF 2017 National Patient Survey

Currently Taking Prescription Medications for Anxiety, Depression or Stress

■ Yes   ■ No

n=392
42%

n=552
58%
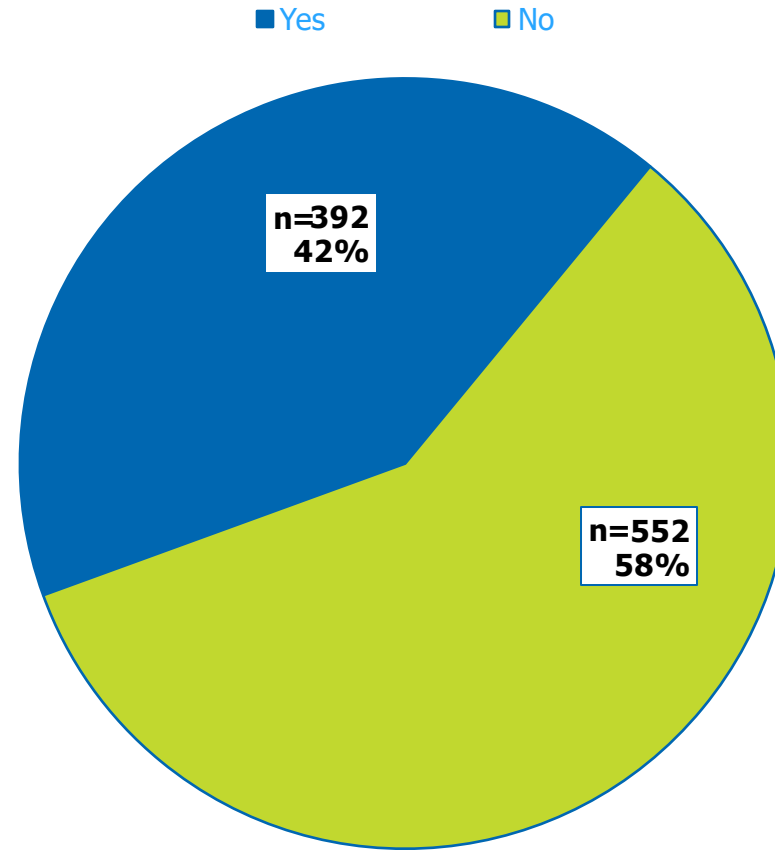
Q37. Is the patient currently taking any prescription medications for anxiety, depression or stress such as Prozac, Effexor or Wellbutrin? N=944
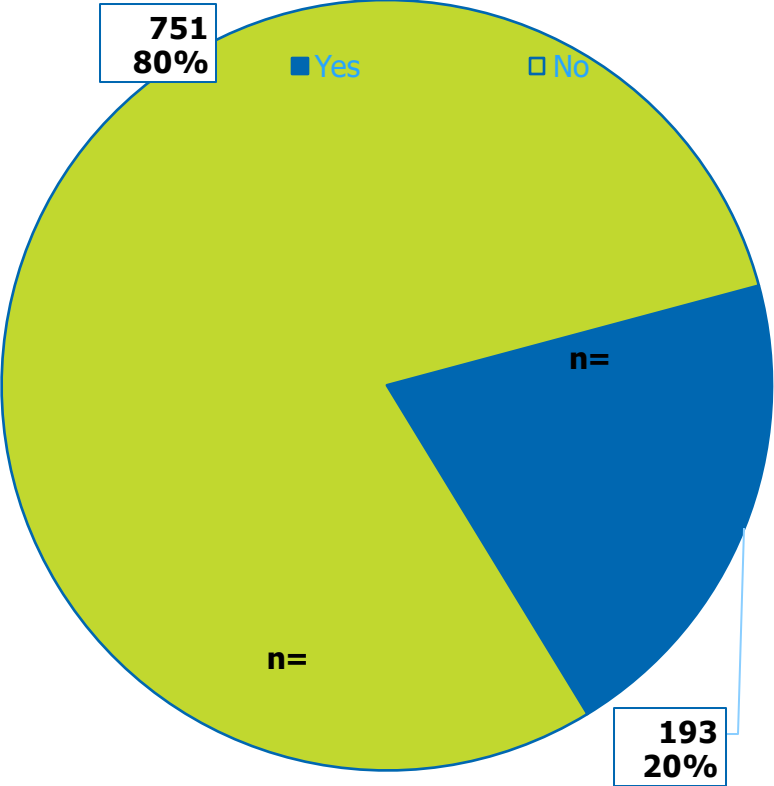Base: Adults with PI

Source: IDF 2017 National Patient Survey

Currently Taking Prescription Medications for Anxiety, Depression or Stress by PHQ-2, Gender, Age and Permanent Impairments

	Currently Taking Prescription Medications for Anxiety, Depression or Stress
PHQ-2*	
0 to 2 (N=706)	33%
3 to 6 (N=211)	**69%**
Gender*	
Male (N=161)	23%
Female (N=765)	**46%**
Age	
18 to 34 (N=82)	34%
35 to 49 (N=196)	35%
50 to 64 (N=321)	**40%**
65 and older (N=210)	**40%**
Permanent Impairments	
None (N=432)	39%
One (N=227)	43%
Two (N=131)	39%
Three or More (N=135)	**47%**

Q3. What is the age and gender of these persons with PI?
Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of...?
Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Q37. Is the patient currently taking any prescription medications for anxiety, depression or stress such as Prozac, Effexor or Wellbutrin?
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder

* p<.00

Source: IDF 2017 National Patient Survey

Currently Taking Prescription Medications for Pain

751
80%

■Yes   □No

n=

n=

193
20%

Q40. Is the patient currently taking any prescribed medications for pain such as oxycodone, codeine or hydrocodone? N=944
Base: Adults with PI

Currently Taking Prescription Medications for Pain by PHQ-2, Gender, Age and Permanent Impairments

	Currently Taking Prescription Medications for Pain
PHQ-2*	
0 to 2 (N=707)	17%
3 to 6 (N=208)	**33%**
Gender**	
Male (N=161)	11%
Female (N=761)	**22%**
Age*	
18 to 34 (N=82)	5%
35 to 49 (N=195)	**26%**
50 to 64 (N=323)	24%
65 and older (N=211)	18%
Permanent Impairments*	
None (N=433)	16%
One (N=229)	18%
Two (N=129)	25%
Three or More (N=134)	**34%**

Q3. What is the age and gender of these persons with PI?
Q14. By the time of initial diagnosis as immune deficient, had he/she suffered any permanent impairment or loss of…?
Q19a. Little interest or pleasure in doing things
Q19b. Feeling down, depressed or hopeless
Q40. Is the patient currently taking any prescribed medications for pain such as oxycodone, codeine or hydrocodone?
Base: Adults with PI
Note: PHQ-2 scores greater than 2 indicate a possible depressive disorder
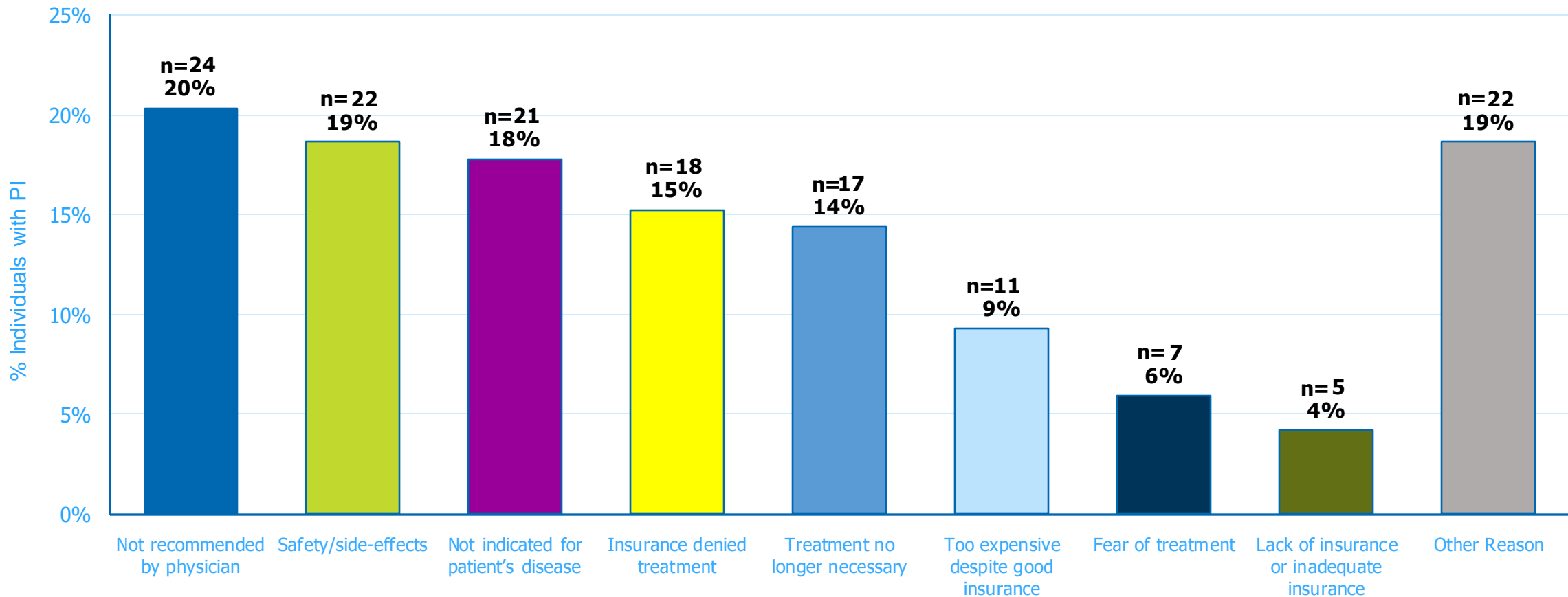
* p<.00
** p<.01

Source: IDF 2017 National Patient Survey

Treatment

Treatments EVER

% Individuals with PI

Treatment	n	%
IVIG	n=700	63%
SCIG	n=581	53%
Prophylactic antibiotics	n=203	18%
IM	n=32	3%
Bone marrow transplant	n=20	2%
Gamma Interferon	n=11	1%
Gene therapy	n=3	0.3%
PEG-ADA	n=2	0.2%
Other Treatment	n=34	3%
None of the above	n=112	10%
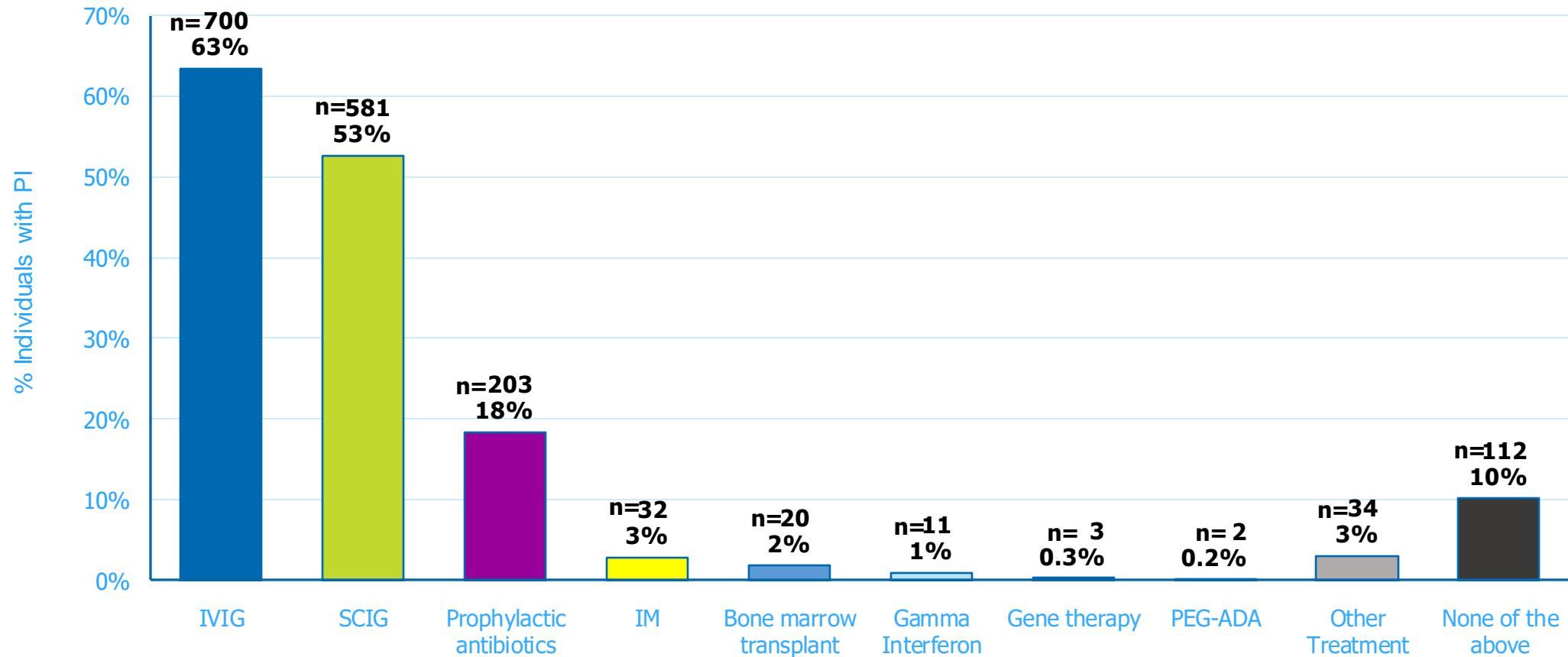
Q26. Has the patient ever received any of the following treatments for their PI? SELECT ALL THAT APPLY N=1,105
Base: All respondents

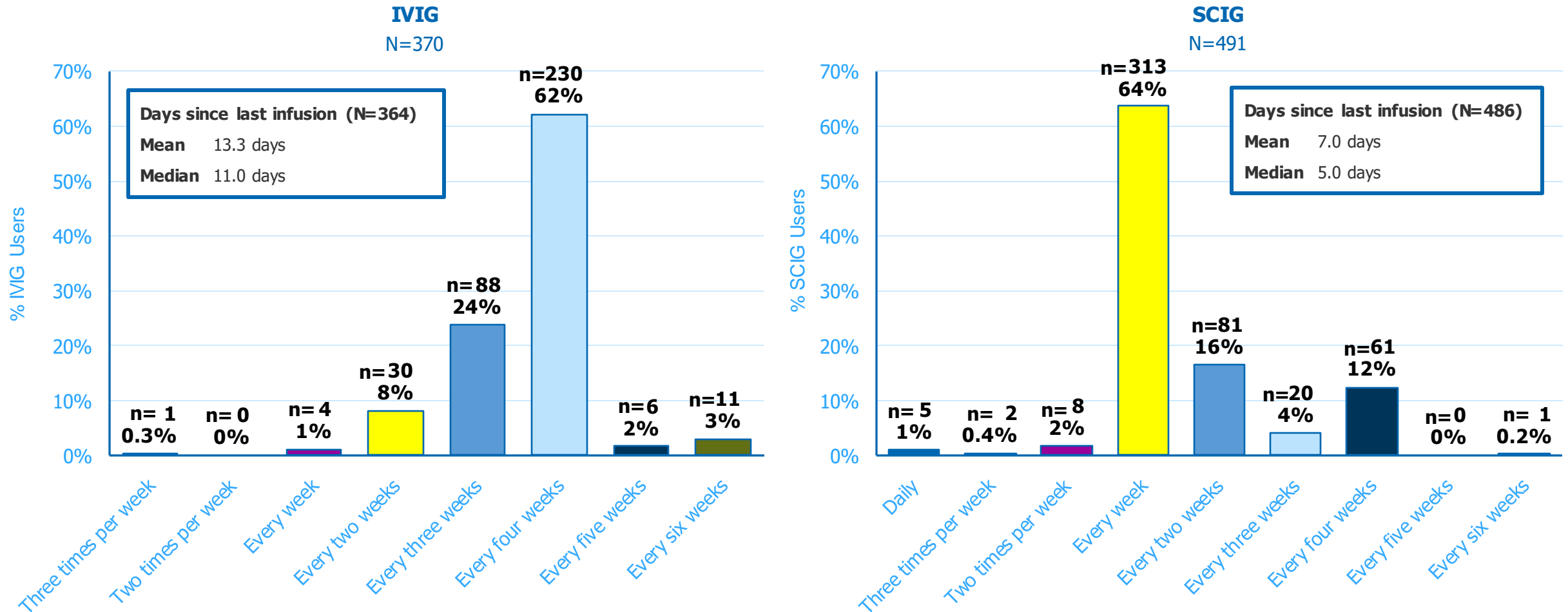Source: IDF 2017 National Patient Survey

60
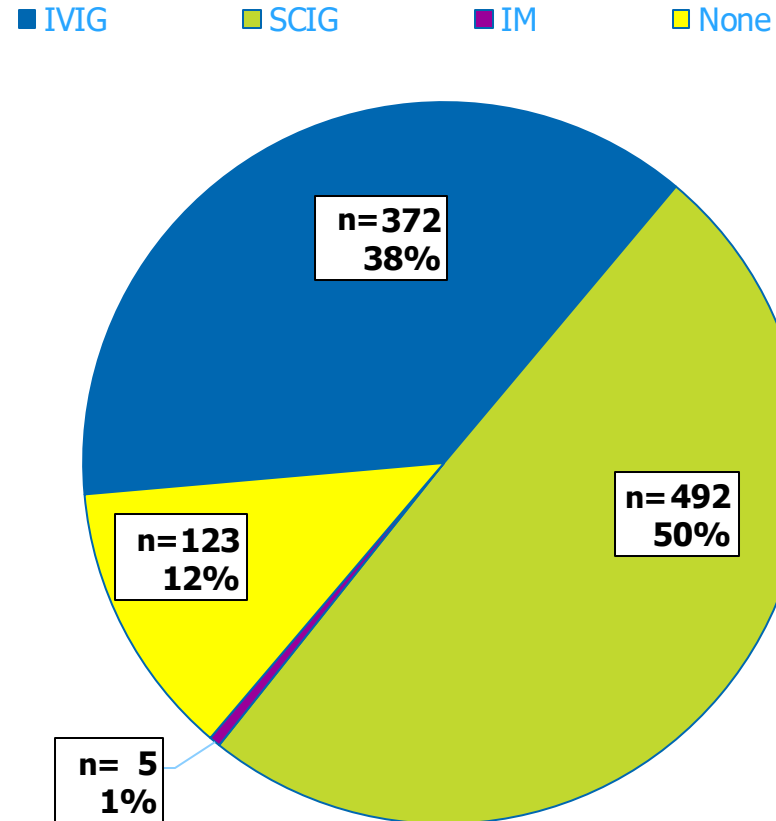
Current Treatment

Q27a. Is the patient currently being treated with IM, IVIG or SCIG? N=992
Base: All respondents who have ever been treated for PI

No Longer Receiving Treatment

% Individuals with PI

- Not recommended by physician: n=24, 20%
- Safety/side-effects: n=22, 19%
- Not indicated for patient's disease: n=21, 18%
- Insurance denied treatment: n=18, 15%
- Treatment no longer necessary: n=17, 14%
- Too expensive despite good insurance: n=11, 9%
- Fear of treatment: n= 7, 6%
- Lack of insurance or inadequate insurance: n= 5, 4%
- Other Reason: n=22, 19%

Q27b. Why is the patient not being treated with immunoglobulin? SELECT ALL THAT APPLY N=118
Base: All respondents not currently on Ig therapy
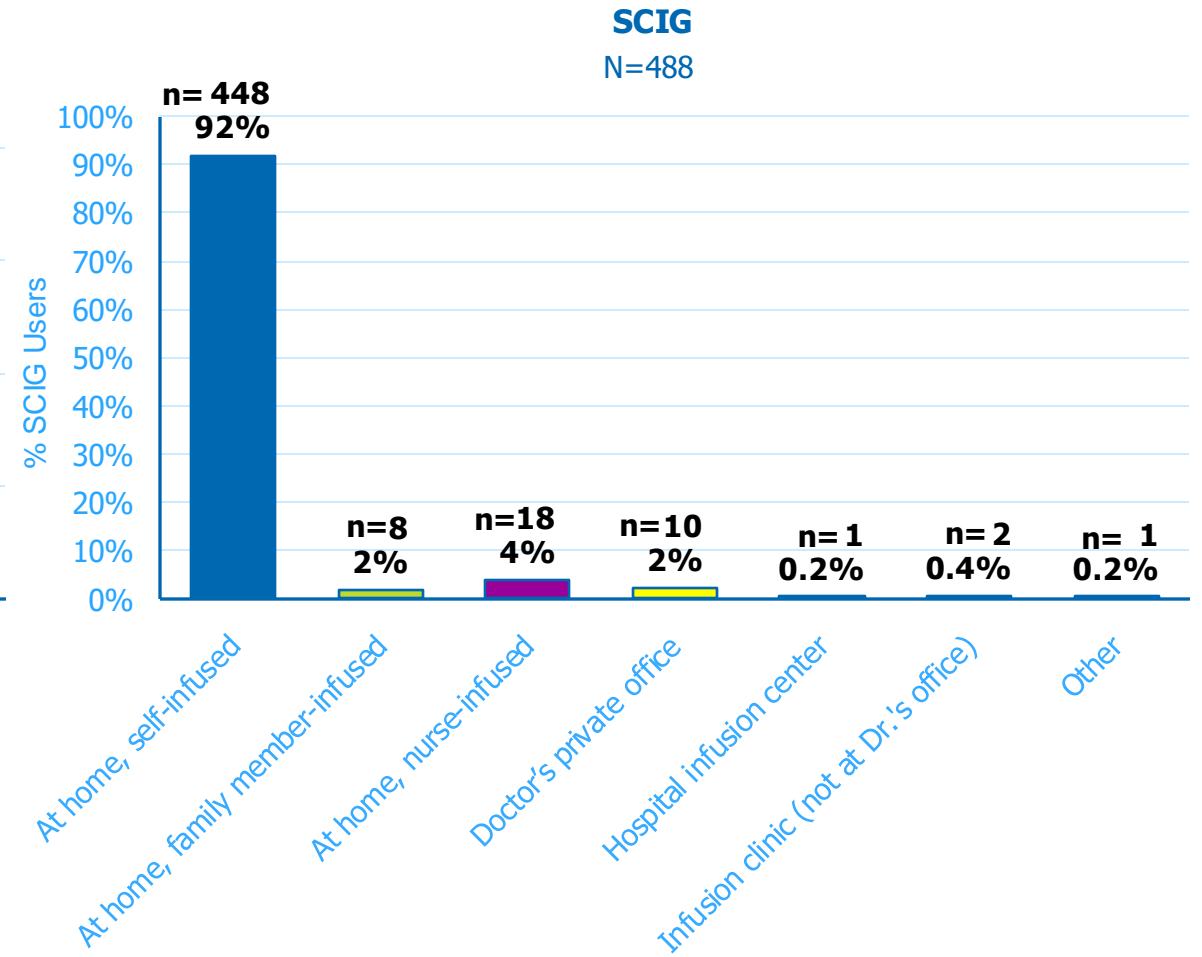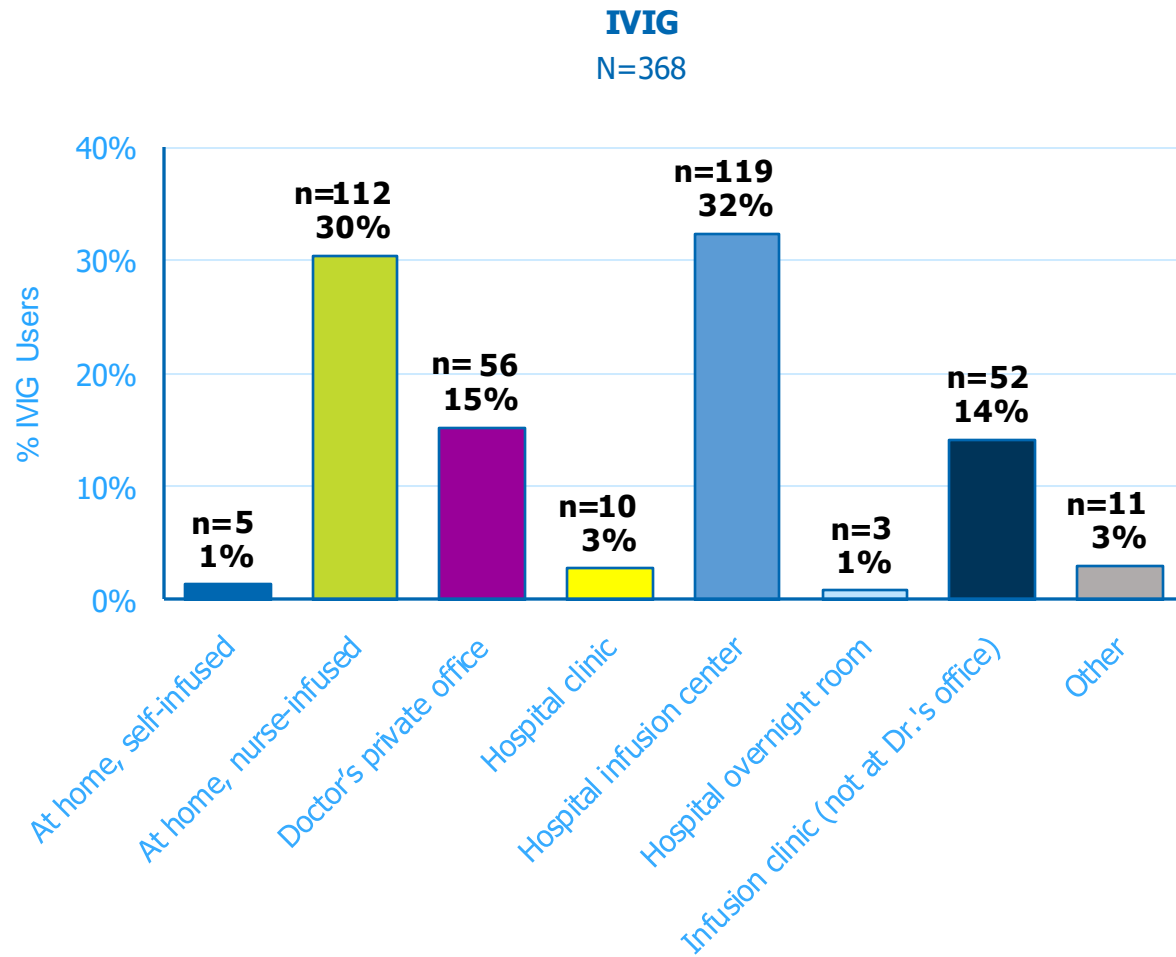
Frequency of Treatment
IVIG vs SCIG

IVIG
N=370

SCIG
N=491

Days since last infusion (N=364)
Mean 13.3 days
Median 11.0 days

Days since last infusion (N=486)
Mean 7.0 days
Median 5.0 days

IVIG chart:
- Three times per week: n=1, 0.3%
- Two times per week: n=0, 0%
- Every week: n=4, 1%
- Every two weeks: n=30, 8%
- Every three weeks: n=88, 24%
- Every four weeks: n=230, 62%
- Every five weeks: n=6, 2%
- Every six weeks: n=11, 3%

SCIG chart:
- Daily: n=5, 1%
- Three times per week: n=2, 0.4%
- Two times per week: n=8, 2%
- Every week: n=313, 64%
- Every two weeks: n=81, 16%
- Every three weeks: n=20, 4%
- Every four weeks: n=61, 12%
- Every five weeks: n=0, 0%
- Every six weeks: n=1, 0.2%

Q29a. How often does the patient receive their immunoglobulin?
Q29b. How many days ago did the patient last receive their immunoglobulin?
Base: All individuals with PI who are currently receiving IVIG or SCIG therapy

Source: IDF 2017 National Patient Survey

63

Grams or mL per infusion

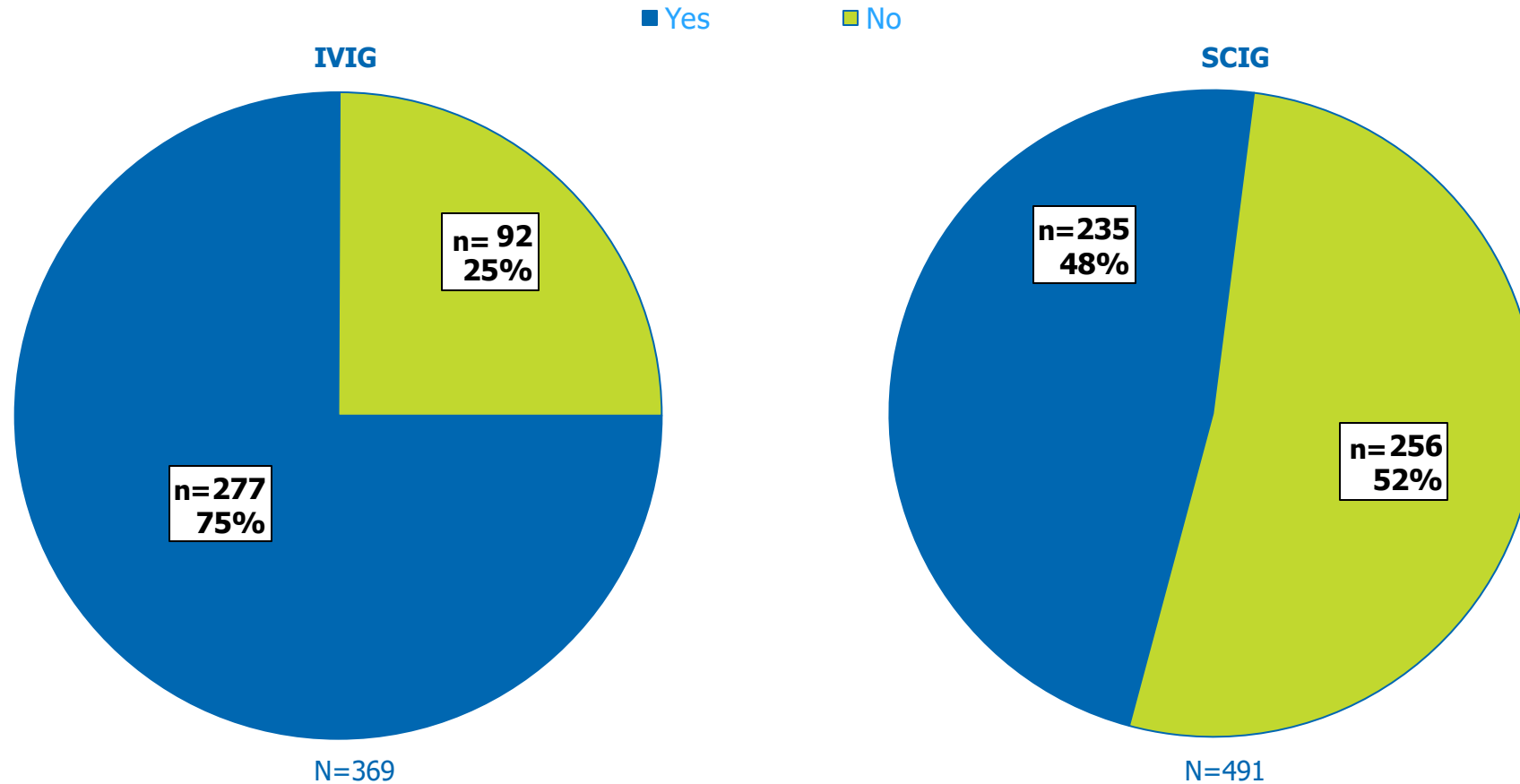|	IVIG Grams N=268	SCIG Grams N=375	SCIG mL N=49
Mean	36.5	17.7	36.5
Median	35.0	12.0	40.0
Min	4	3	3
Max	70	65	70

Q30. About how many grams of immunoglobulin does the patient normally receive?
Base: All individuals with PI who are currently receiving IVIG or SCIG therapy

Wear Off Effect
IVIG vs SCIG*

■ Yes ■ No

IVIG

n= 92
25%

n=277
75%

N=369

SCIG

n=235
48%

n=256
52%

N=491

* p<.00

Q34a. Is there a noticeable "wear off" as the patient is due for a next immunoglobulin treatment?
Base: All individuals with PI who are currently receiving IVIG or SCIG therapy

Source: IDF 2017 National Patient Survey

Wear Off Effect Resulted In…
IVIG vs SCIG
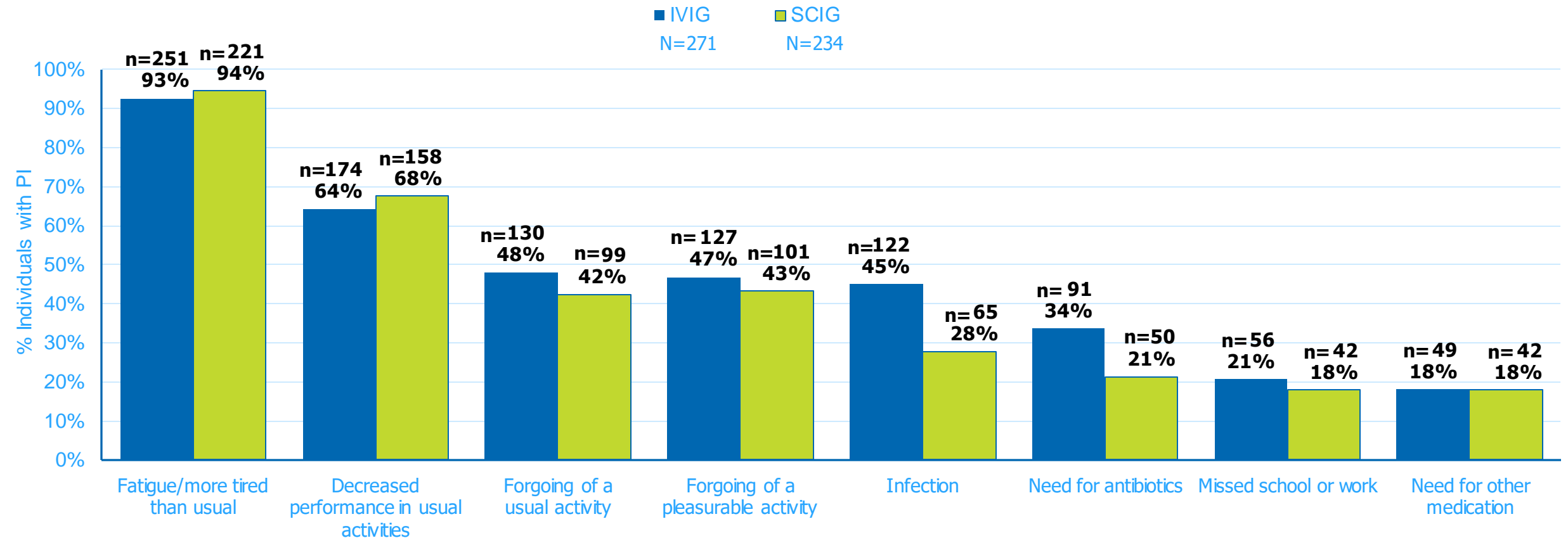
Q34b. Does the "wear off" result in any of the following? SELECT ALL THAT APPLY
Base: All individuals with PI who are currently receiving IVIG or SCIG therapy

67

Source: IDF 2017 National Patient Survey

Prophylactic Antibiotic Therapy (PAT) Medications by Ig Usage

| Type of Antibiotic Used	IVIG Users 31% (n=114, N=368) Receiving PAT	SCIG Users 23% (n=112, N=491) Receiving PAT	Not Currently Receiving Ig* 36% (n=83, N=233) Receiving PAT
	n=108	n=110	n=82
Azithromycin	31%	**36%**	34%
Trimethoprim/sulfamethoxazole (Bactrim)	24%	**27%**	33%
Amoxicillin/clavulanate (Augmentin)	21%	21%	**26%**
Amoxicillin	16%	16%	**34%**
Clarithromycin (Biaxin)	**14%**	8%	10%
Ciprofloxacin (Cipro)	13%	16%	**22%**
Doxycycline	**8%**	**8%**	5%
Levofloxacin (Levaquin)	**5%**	**5%**	2%
Ceftin	**5%**	3%	1%
Cephalexin (Keflex)	**5%**	1%	2%
Cefdinir (Omnicef)	3%	2%	**4%**
Other Antibiotic	**16%**	10%	7%

* p<.00

Q35a. Does the patient take antibiotics on a regular basis (every day or at least once a week) to prevent infections?
Q35c. What is the name of the antibiotic(s) the patient takes to prevent infection? SELECT ALL THAT APPLY
Base: All respondents who take antibiotics to prevent infection

Prophylactic Antifungal Therapy (PFT) Medications by Ig Usage

Type of Antifungal Used	IVIG Users 10% (n=38, N=365) Receiving PFT	SCIG Users 7% (n=31, N=491) Receiving PFT	Not Currently Receiving Ig* 12% (n=28, N=233) Receiving PFT
	n=37	n=30	n=27
Fluconazole	84%	87%	63%
Itraconazole	8%	0%	15%
Nystatin	5%	3%	7%
Posaconazole	3%	0%	11%
Other Antifungal	22%	23%	19%

Q36a. Does the patient take antifungal on a regular basis (every day or at least once a week) to prevent infections?
Q36c. What is the name of the antifungal(s) the patient takes to prevent infection? SELECT ALL THAT APPLY
Base: All respondents who take antibiotics to prevent infection
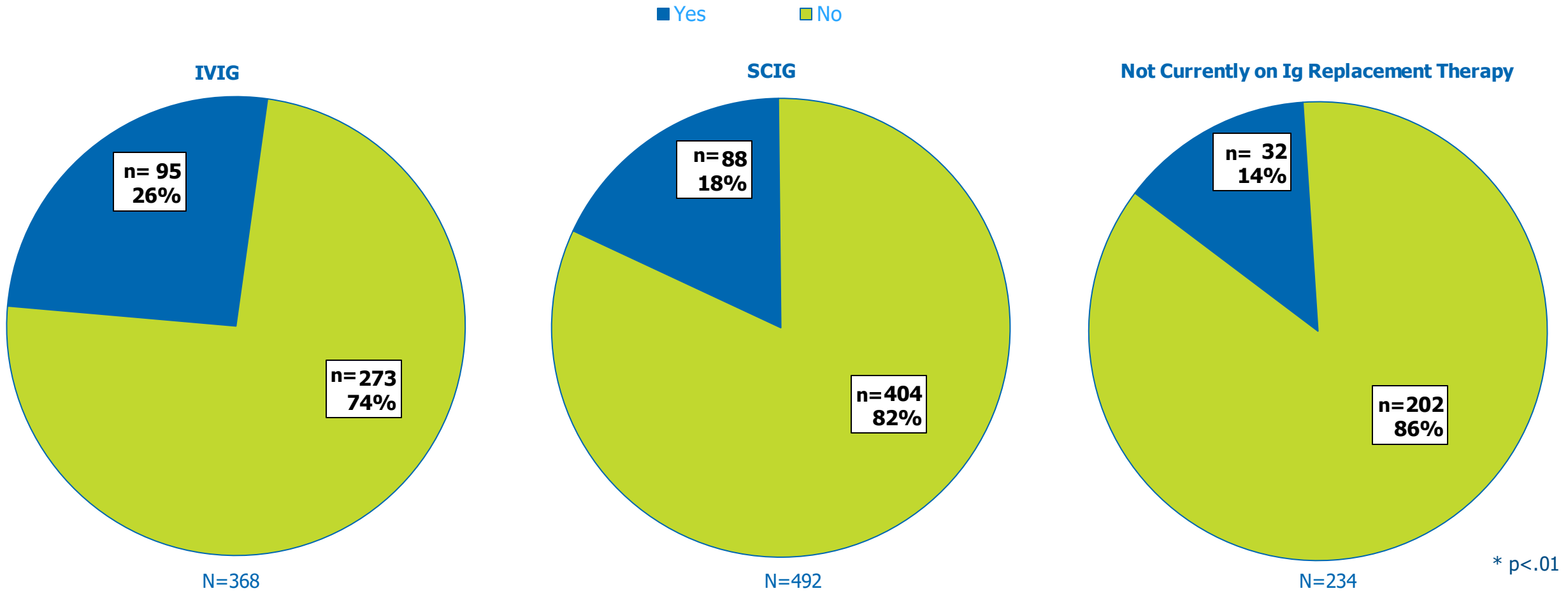
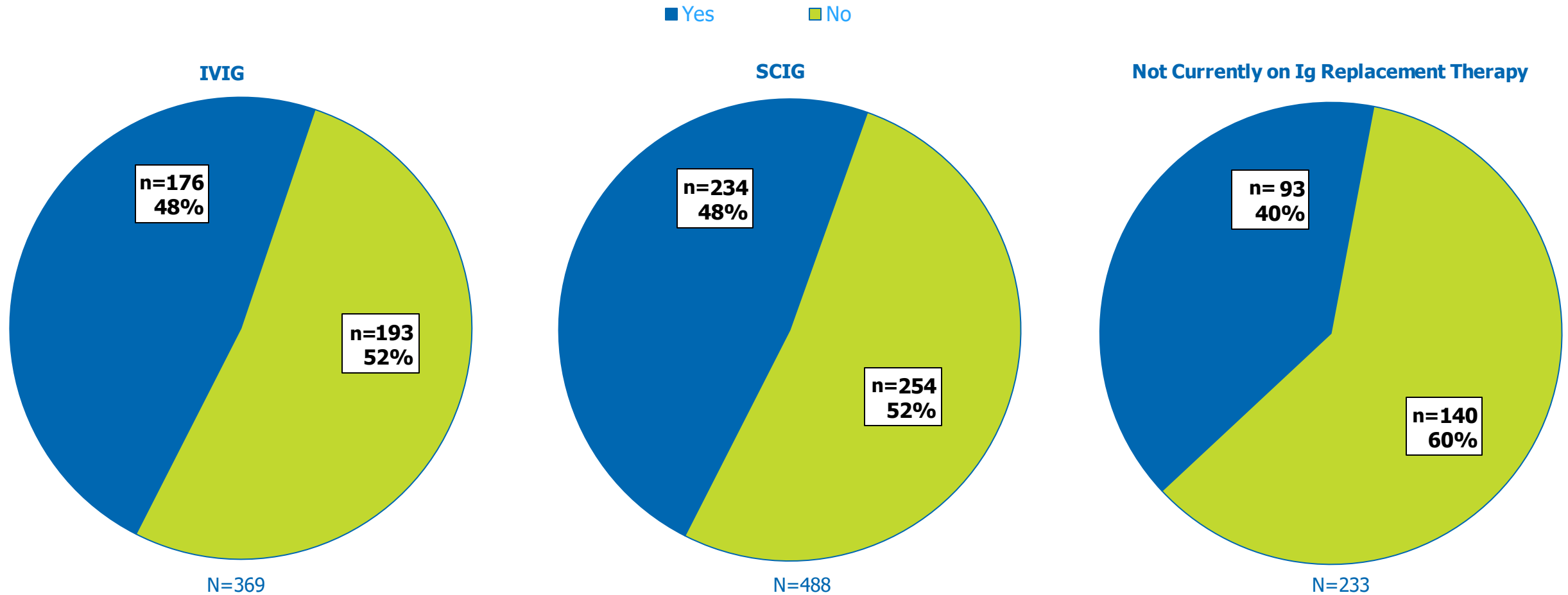# Prophylactic Antibiotic Therapy and Prophylactic Antifungal Therapy: Current Ig Replacement Therapy Use*
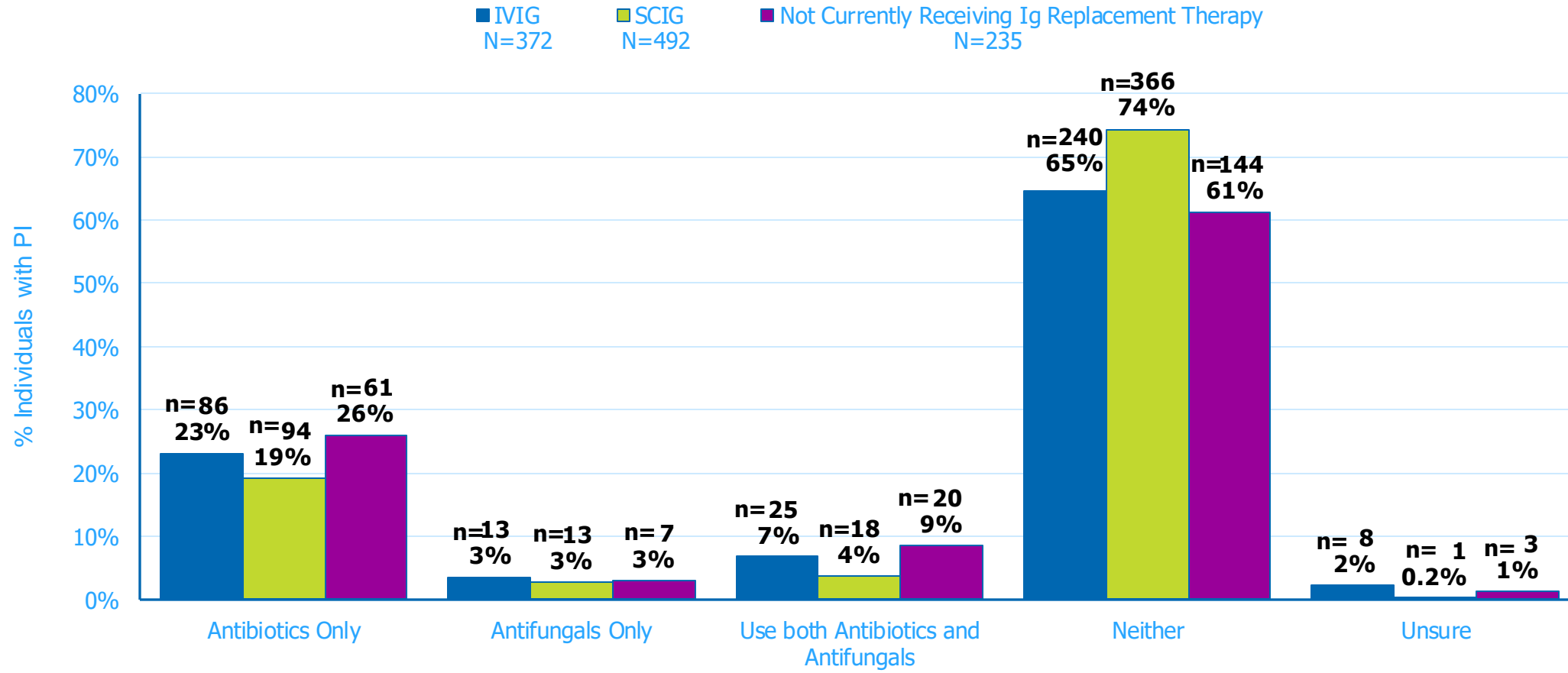
Q35a. Does the patient take antibiotics on a regular basis (every day or at least once a week) to prevent infections?
Q36a. Does the patient take antifungals on a regular basis (every day or at least once a week) to prevent infections?
Base: All respondents currently receiving IVIG or SCIG and those not currently receiving any Ig replacement therapy

* p<.01

70

Currently Taking Corticosteroids by Current Ig Replacement Therapy Use*

■ Yes   ■ No

IVIG

n= 95
26%

n=273
74%

N=368

SCIG

n=88
18%

n=404
82%

N=492

Not Currently on Ig Replacement Therapy
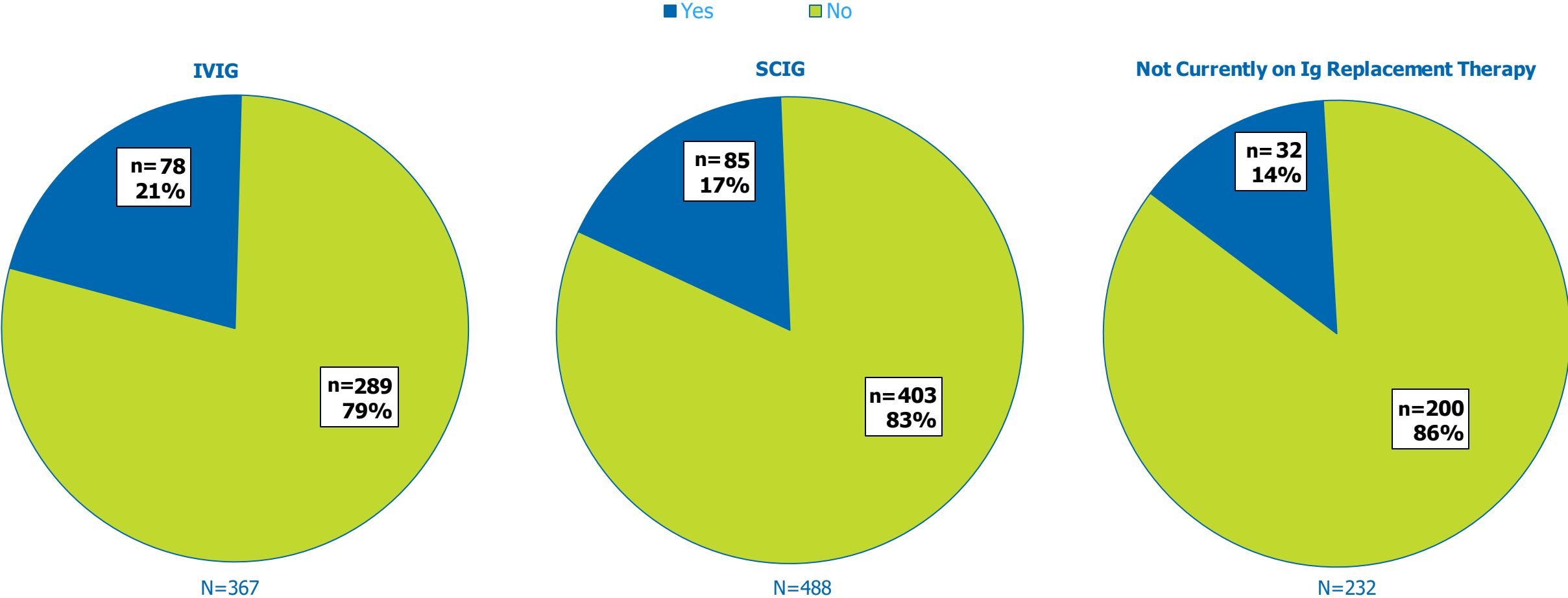
n= 32
14%

n=202
86%

N=234

* p<.01

Q38. Is the patient currently taking any corticosteroids such as Prednisone, Cortisone, Medrol or Dexamethasone?
Base: All respondents currently receiving IVIG or SCIG and those not currently receiving any Ig replacement therapy

Currently Taking Nonsteroidal Anti-inflammatory Medications by Current Ig Replacement Therapy Use

■ Yes ■ No

IVIG

n=176 48%

n=193 52%

N=369

SCIG

n=234 48%

n=254 52%

N=488

Not Currently on Ig Replacement Therapy
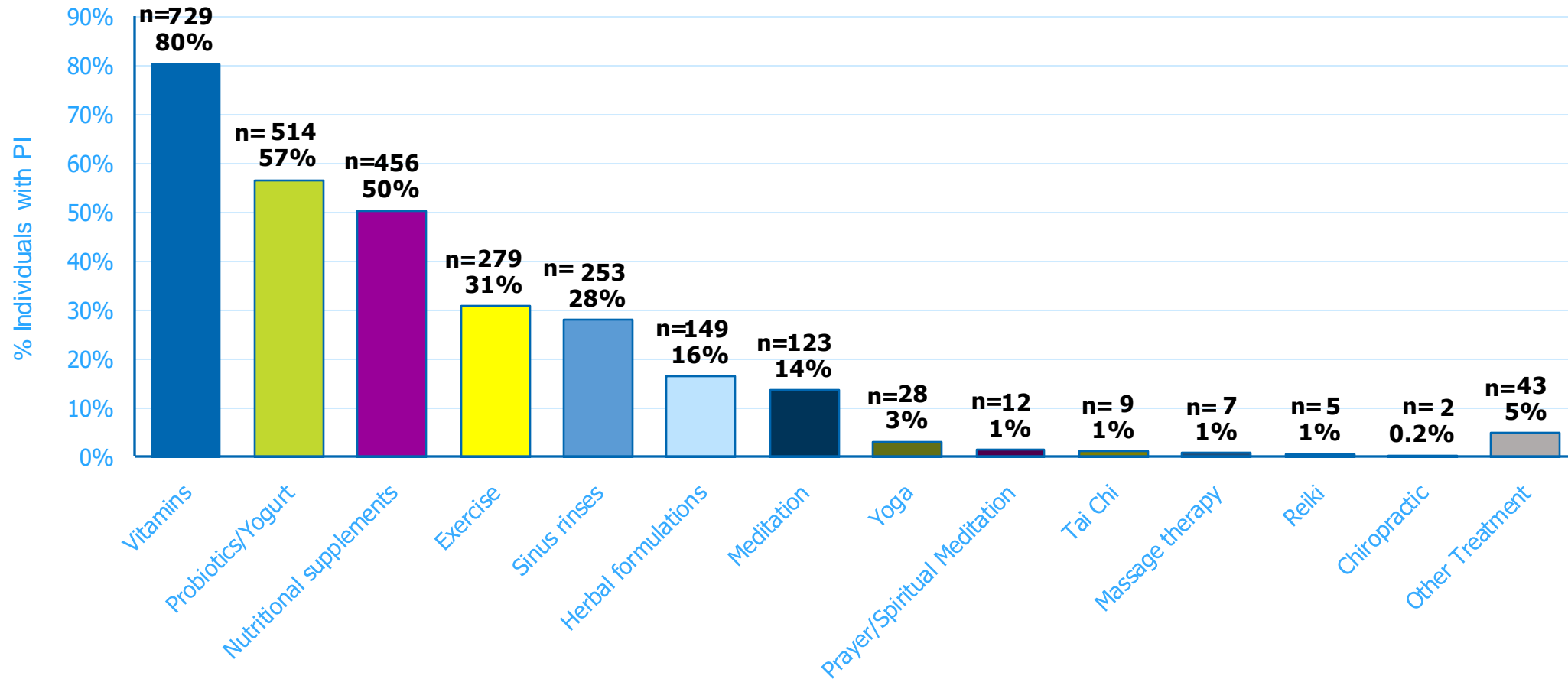
n= 93 40%

n=140 60%

N=233

Q39. Is the patient currently taking any nonsteroidal anti-inflammatory medications such as ibuprofen, naproxen or Celebrex?
Base: All respondents currently receiving IVIG or SCIG and those not currently receiving any Ig replacement therapy

Source: IDF 2017 National Patient Survey

Currently Taking Prescribed Medications for Pain by Current Ig Replacement Therapy Use

■ Yes   ■ No

IVIG

n=78
21%

n=289
79%

N=367

SCIG

n=85
17%

n=403
83%

N=488

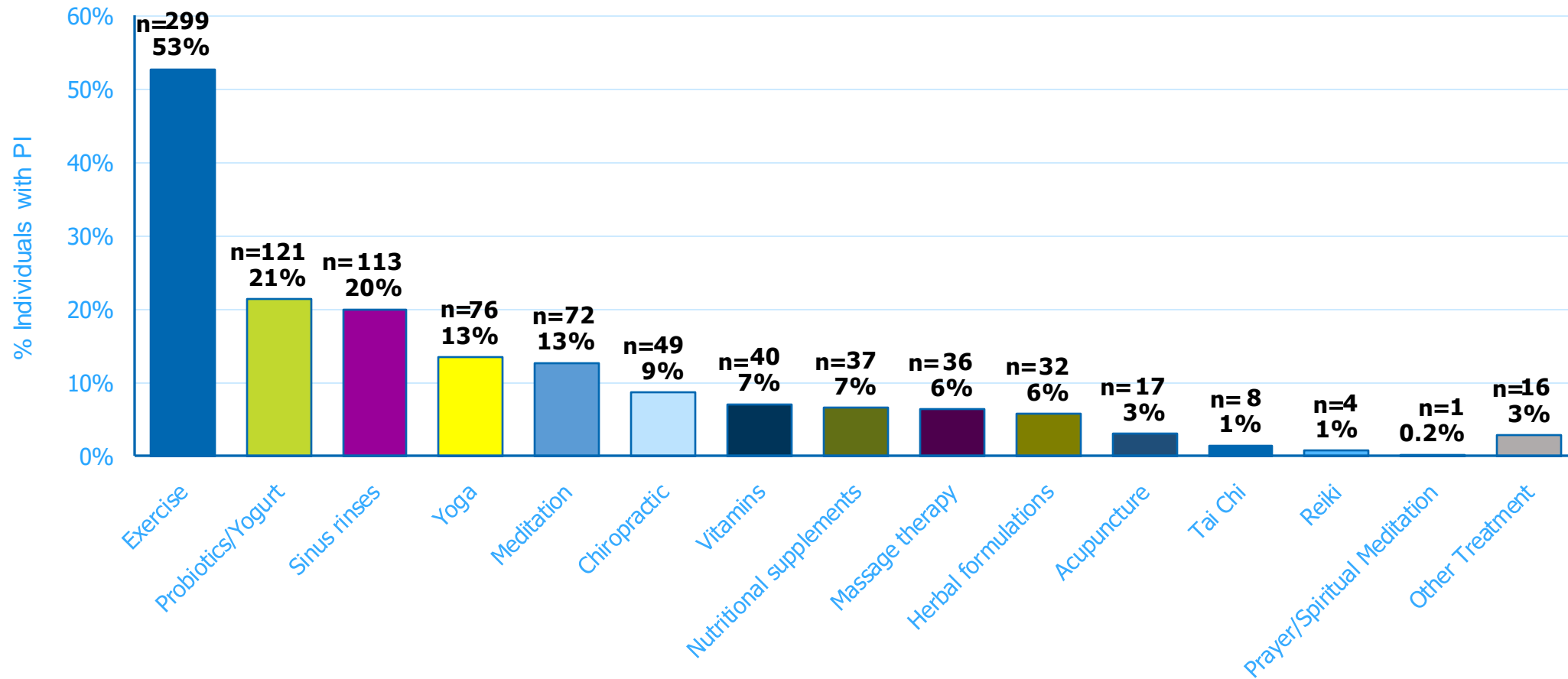**Not Currently on Ig Replacement Therapy**

n=32
14%

n=200
86%

N=232

Q40. Is the patient currently taking any prescribed medications for pain such as oxycodone, codeine or hydrocodone?
Base: All respondents currently receiving IVIG or SCIG and those not currently receiving any Ig replacement therapy
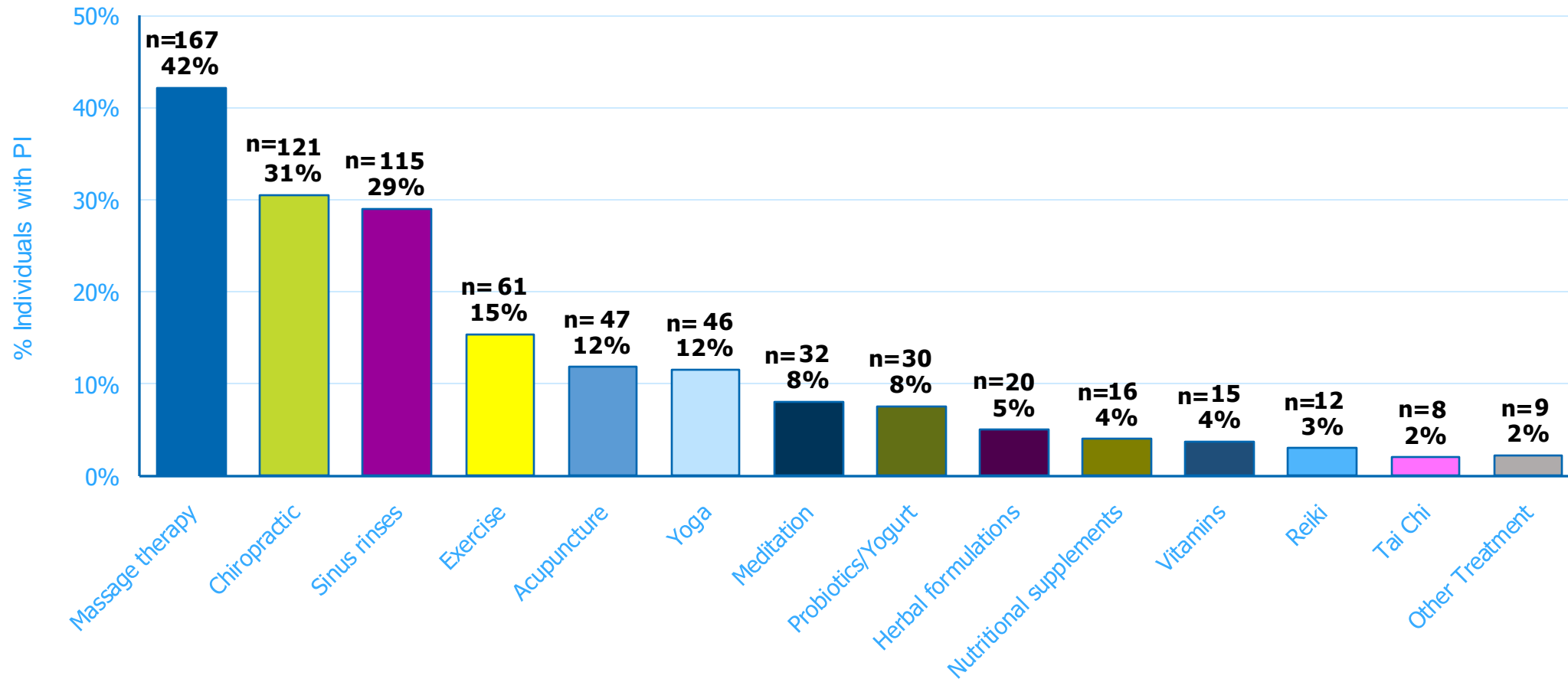
Additional Treatments Daily

% Individuals with PI

- Vitamins: n=729, 80%
- Probiotics/Yogurt: n= 514, 57%
- Nutritional supplements: n=456, 50%
- Exercise: n=279, 31%
- Sinus rinses: n= 253, 28%
- Herbal formulations: n=149, 16%
- Meditation: n=123, 14%
- Yoga: n=28, 3%
- Prayer/Spiritual Meditation: n=12, 1%
- Tai Chi: n= 9, 1%
- Massage therapy: n= 7, 1%
- Reiki: n= 5, 1%
- Chiropractic: n= 2, 0.2%
- Other Treatment: n=43, 5%

Q41. Please tell us which of these other treatments, if any, the patient uses in the management of their PI. N=908
Base: All respondents

Source: IDF 2017 National Patient Survey

Additional Treatments Weekly

Source: IDF 2017 National Patient Survey

Additional Treatments Monthly

% Individuals with PI

- Massage therapy: n=167, 42%
- Chiropractic: n=121, 31%
- Sinus rinses: n=115, 29%
- Exercise: n= 61, 15%
- Acupuncture: n= 47, 12%
- Yoga: n= 46, 12%
- Meditation: n= 32, 8%
- Probiotics/Yogurt: n=30, 8%
- Herbal formulations: n=20, 5%
- Nutritional supplements: n=16, 4%
- Vitamins: n=15, 4%
- Reiki: n=12, 3%
- Tai Chi: n=8, 2%
- Other Treatment: n=9, 2%

Q41. Please tell us which of these other treatments, if any, the patient uses in the management of their PI. N=396
Base: All respondents