

Patient and Health Care Professional Perspectives on Quality of Life and Unmet Needs of People With Chronic Neutropenia: A Survey-Based Assessment

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BACKGROUND

- Chronic neutropenia encompasses multiple blood disorders characterized by low levels of neutrophils (absolute neutrophil count [ANC] <1500/ μ L for >3 months) that substantially alter quality of life (QoL) of affected individuals owing to disease-related symptoms and increased risk of infections^{1,2}
- Granulocyte colony-stimulating factor (G-CSF) treatment can improve clinical outcomes, but chronic use can be poorly tolerated and associated with debilitating side effects such as bone pain that have negative impact on QoL and increase the risk of myelodysplastic syndromes in some cases^{1,3}
- A pilot survey that explored the experiences of people living with chronic neutropenic (CN) disorders including congenital, cyclic, and idiopathic neutropenia was recently completed in the United States that suggested a major impact of CN disorders on the daily lives of individuals living with chronic neutropenia⁴

AIM

This survey aimed to explore the experience and perspectives of patients/caregivers (P/C) and health care professionals (HCP) in the United States on unmet needs, treatment patterns, impact on daily life, and QoL of people living with CN disorders on a larger scale to confirm the results of the pilot survey

METHODS

Respondents were recruited via direct emails to patients with CN disorders, outreach to CN disorders Facebook groups by Savvy Cooperative and X4 Pharmaceuticals, and via patient advocacy groups

110 participants completed the survey

100 P/C	77% patients (P)	10 HCP ^b	60% pediatric hematology/oncology (n=6)
	18% caregivers (C)		30% pediatric/adult immunology (n=3) ^c
	5% P and C		20% adult hematology (n=2)

Participated in a virtual survey composed of

≤29 questions	≤13 questions
13 screening questions captured demographics, neutropenia type, and treatment setting	7 screening questions captured data on medical specialty and experience treating neutropenia

16 questions captured information on severity, symptoms, impact on daily life, treatment types, and unmet needs	6 questions captured patient neutropenia type, treatment patterns, and unmet needs
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^aPatients with chemotherapy-induced neutropenia were ineligible. ^bSome HCP specialize in >1 medical specialties. ^cThree providers who specialize in adult immunology also specialize in pediatric immunology.

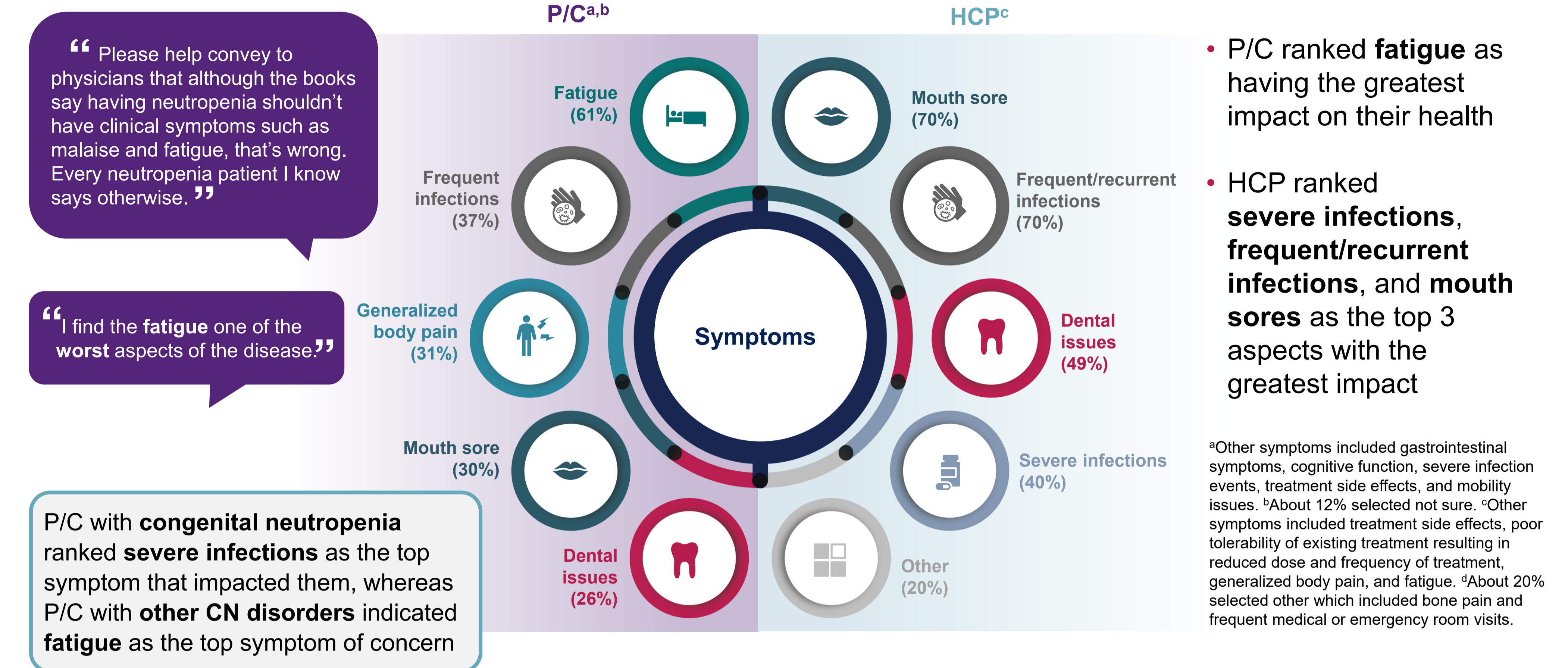
RESULTS

HCP Baseline Demographics	n (%)
Types of neutropenic disorders treated ^a	
Idiopathic	9 (90)
Cyclic	9 (90)
Autoimmune	9 (90)
Congenital	8 (80)
Secondary neutropenia	8 (80)
Medications prescribed ^b	
G-CSF	8 (80)
Ig replacement therapy	4 (40)
Prophylaxis antibiotics	1 (10)
Others ^b	2 (20)

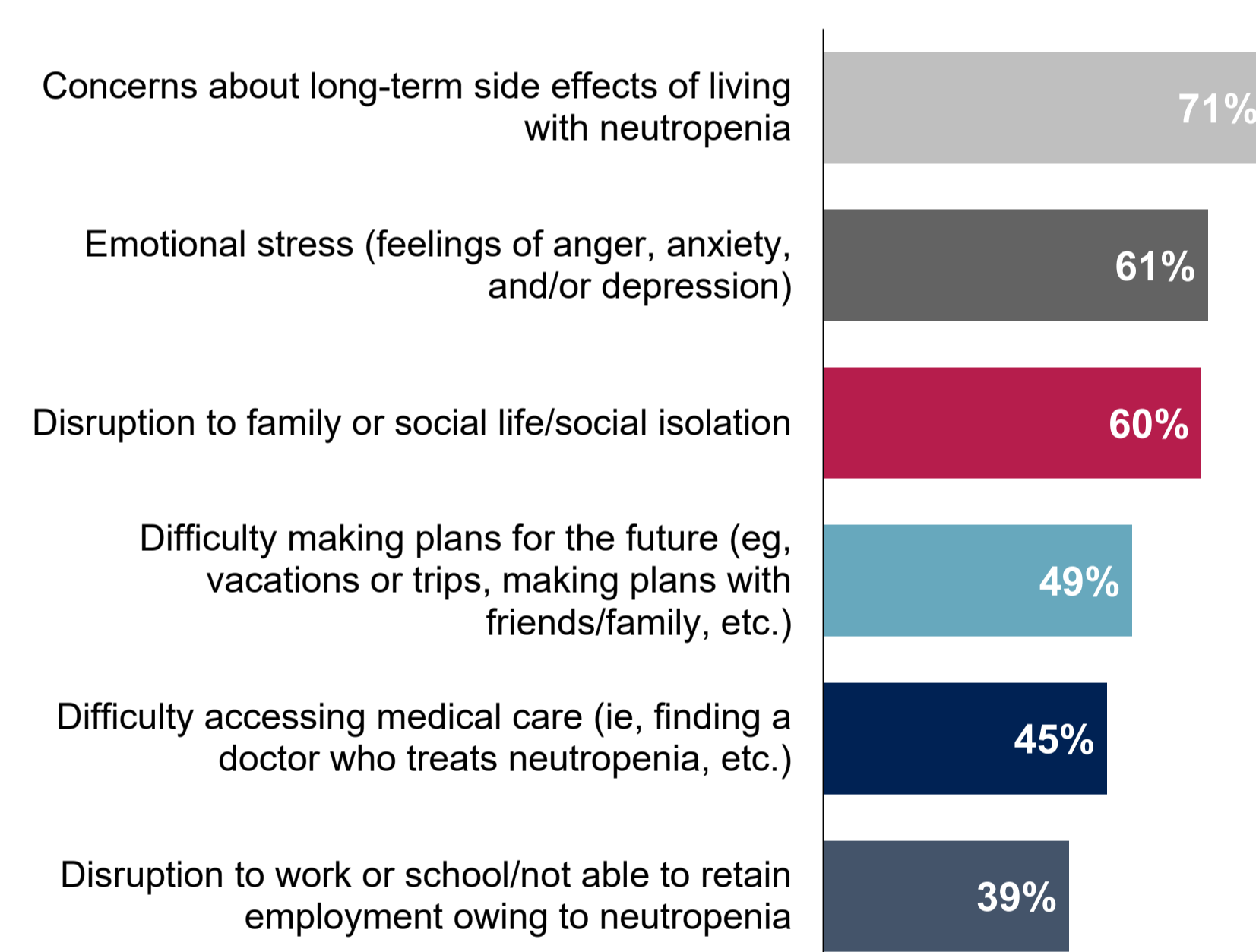
Ig, immunoglobulin. ^aRespondents were allowed to select ≥1 options, therefore, the total percentages may not add up to 100. ^bOther treatments included antibiotics only at times of infection or febrile neutropenia.

RESULTS

P/C and HCP Reported Multiple Concerns That Impact Health of a Person Living With Chronic Neutropenic Disorders



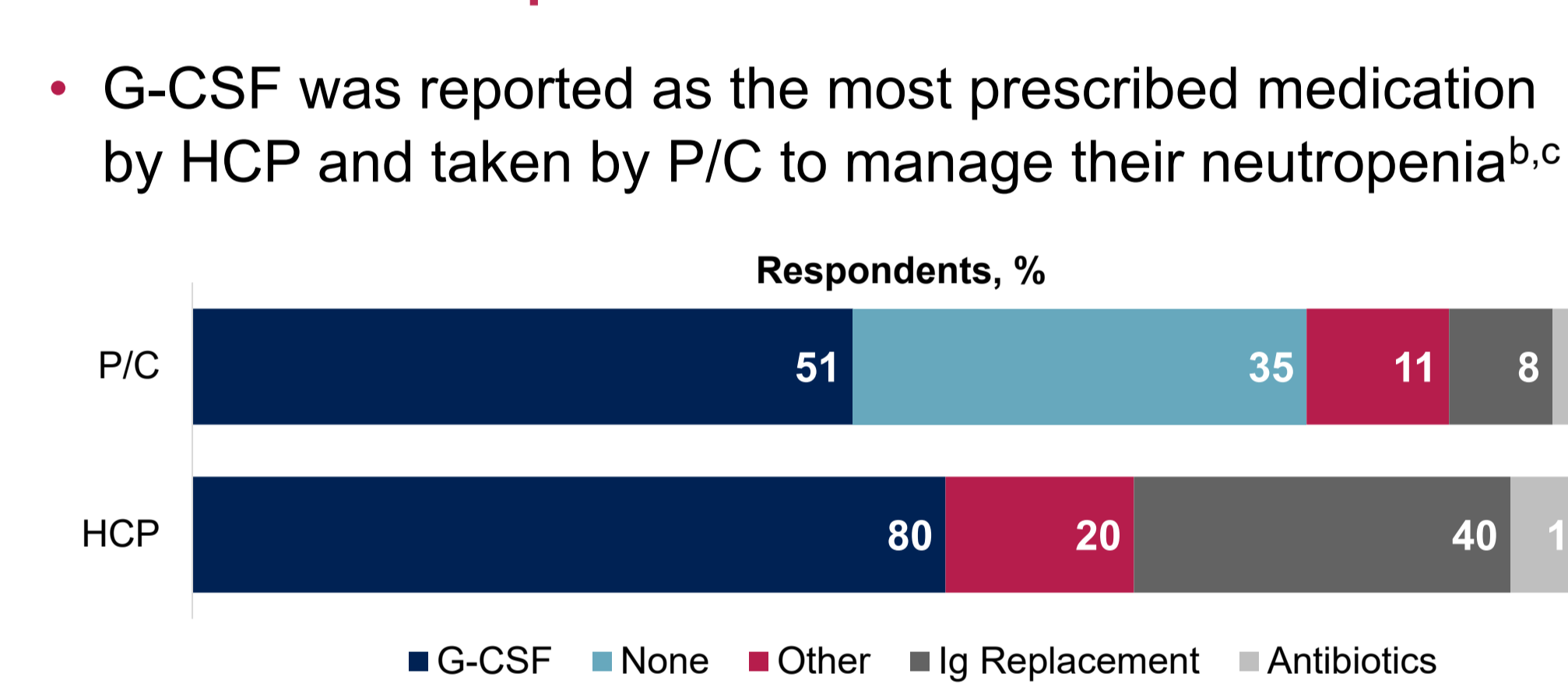
Psychosocial Experience of P/C Living With Chronic Neutropenic Disorders



P/C Baseline Demographics	n (%)
Age, y	
2–12	10 (10)
13–21	8 (8)
≥22	82 (82)
Sex	
Female	78 (78)
Diagnosis	
Idiopathic	29 (29)
Cyclic	20 (20)
Autoimmune	19 (19)
Congenital	18 (18)
Unknown	14 (14)
Severity of neutropenia	
Mild (ANC >1000–1500/ μ L)	9 (9)
Moderate (ANC 500–1000/ μ L)	16 (16)
Severe (ANC <500/ μ L)	46 (46)
Variable	24 (24)
Not sure	5 (5)
Treatments received ^a	
G-CSF	51 (51)
Ig replacement therapy	8 (8)
Prophylaxis antibiotics	4 (4)
Others ^b	11 (11)
Not taking medication	35 (35)

^aRespondents were allowed to select ≥1 options, therefore, the total percentages may not add up to 100. ^bOther treatments included iron supplements, multivitamins, and pain medication.

The Majority of P/C See Adult Hematology/Oncology Specialists for Their Care^a



^aOther specialties included pediatric hematology/oncology, adult hematology, adult primary care/general medicine, family medicine, adult rheumatology, and adult immunology. ^bFor P/C, “other” included iron supplements, multivitamins, and pain medication. ^cFor P/C, preventative long-term use of antibiotics was captured.

Regardless of the type of CN disorder, the majority of respondents indicated that they were taking G-CSF as a treatment for chronic neutropenia

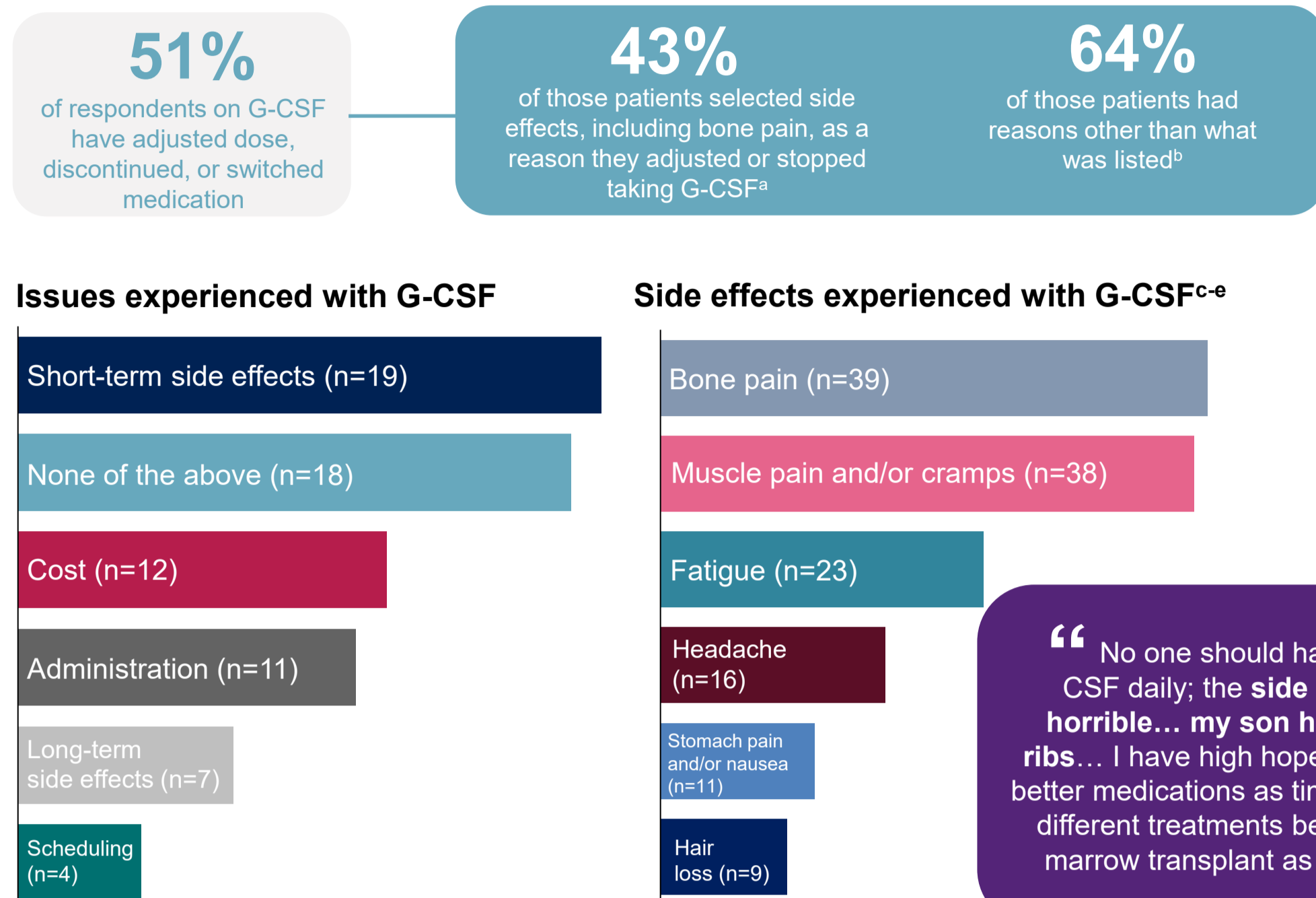
Respondents not on any medication

“I cannot tolerate the side effects of (the medication) that I previously used... Living with severe neutropenia and not being able to tolerate the commonly prescribed medication is frustrating.”

“G-CSF did not work for me; all the trials failed. It gave me more bone pain and wasn't worth it since my levels didn't stay up.”

“My insurance does not cover [G-CSF], unless I am inpatient/hospitalized.”

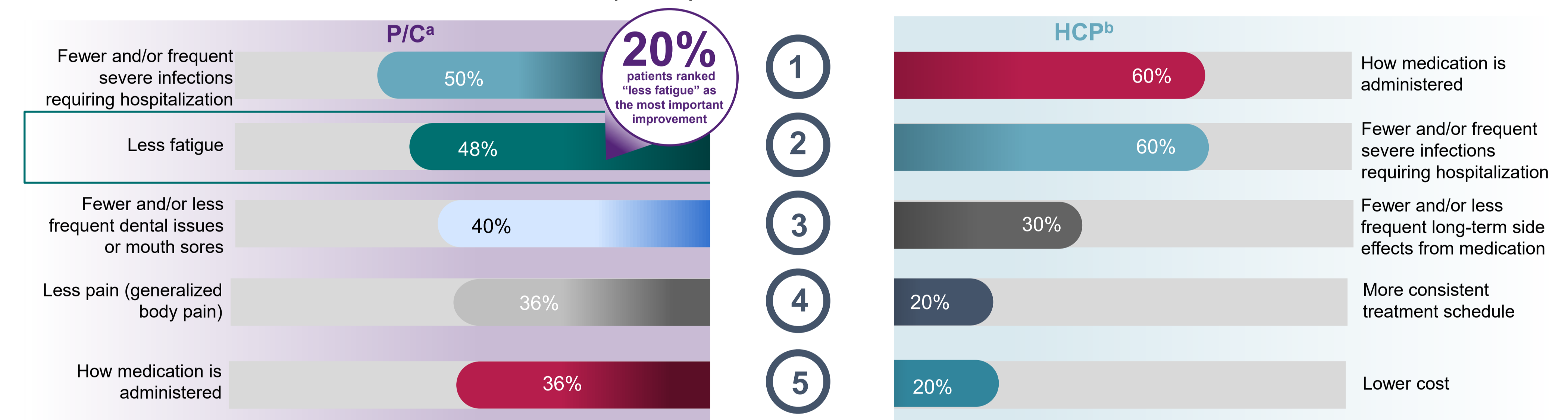
Respondents on G-CSF Reported Experiencing Several Issues and Side Effects



^aRespondents were allowed to select ≥1 options. ^bOther reasons included no longer need it, did not work, frequent dose adjustment, and not covered by insurance. ^cNone was reported by 5% of respondents. ^dFive percent of respondents also indicated others as their response. ^eOther side effects reported by respondents included vomiting, difficulty urinating, red spots on the skin, none of the above, pain, redness, or irritation at site where injected, other (please explain), dizziness or feeling faint, fever and/or chills, cough.

Despite Variable Views On The Symptoms That Have the Most Impact and Treatment Side Effects That Needed Most Improvement, Both P/C and HCP Conveyed a Strong Interest in New Treatments

Both P/C and HCP would like to see multiple improvements in a new treatment for CN disorders



^aOther improvements included lower cost, fewer and/or less frequent short-term side effects from medication, fewer and/or less frequent gastrointestinal symptoms, fewer and/or less frequent long-term side effects from medication, and easier storage. ^bOther improvements included better access to chronic therapy, fewer and/or less frequent short-term side effects from medication, fewer and/or less frequent severe infections, fewer and/or less frequent dental issues, fewer and/or less frequent mouth sores, and less generalized body pain.

CONCLUSIONS

- The results of this survey show differing perspectives between P/C and HCP on impact of chronic neutropenia and its treatment on QoL
- P/C cited fatigue as the most impactful symptom and fatigue reduction as a priority treatment need, while HCP ranked fatigue at the bottom or not at all. Additionally, HCP ranked fewer and/or less frequent severe infections requiring hospitalization, how medication is administered, and fewer and/or less frequent long-term side effects as the priority treatment needs
- Although both groups reported improvement in medication administration as a need, perceptions of patient experience with current treatment differ, with P/C more commonly citing both short- and long-term side effects as the cause of medication changes
- Survey results show considerable impact of CN disorders on the lives of people diagnosed and suggest the existing treatment paradigm is burdensome and insufficiently addresses physical health/QoL needs
- Additional studies are required to understand unmet needs of people living with CN disorders and reasons behind differing perspectives between P/C and HCP communities

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