

The lack of tolerable treatment options that can induce durable responses without fear of relapse after discontinuation represents a significant unmet need for patients with immune thrombocytopenia (ITP): results from the ITP World Impact Survey (I-WISH) 2.0

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KEY FINDINGS & CONCLUSIONS

- Some patients with ITP show dissatisfaction with current treatment options because of a perceived lack of efficacy, the burden of short- and long-term side effects and the need for daily, life-long administration. Physicians are generally satisfied with current treatment options, largely because of their ability to achieve treatment goals and improve quality of life (QoL) and ITP symptoms.
- Physicians report that commonly prescribed treatments for ITP, particularly corticosteroids and other immunosuppressants, are associated with fatigue
 - However, limiting immunosuppressive side effects is not an important aim for most physicians nor do many rank reducing fatigue among their top three treatment goals, indicating insufficient prioritization with respect to this significant issue for patients with ITP.
- Physicians and patients are aligned in wanting ITP treatments to achieve healthy platelet counts and a good QoL. Both would like new ITP treatments to offer a sustained period of remission or cure without the need for chronic administration, while patients wish to be able to safely discontinue them without fear of relapse.
- There is an unmet need for well-tolerated therapies with disease-modifying potential that can safely induce durable responses that are maintained beyond treatment discontinuation.

INTRODUCTION

- ITP is an acquired autoimmune disease characterized by transient or persistent thrombocytopenia caused by accelerated platelet destruction and impaired platelet production.¹
- As demonstrated in I-WISH 1.0, ITP substantially impairs patients' QoL, including daily activities, social interactions, work and emotional wellbeing;^{2,3} fatigue is the most common patient-reported symptom and has a significant impact on QoL.^{3,4}
- Corticosteroids are recommended as first-line treatment for ITP, while thrombopoietin receptor agonists (TPO-RAs), rituximab and fostamatinib are established second- or later-line therapies after failure of corticosteroids⁵⁻⁷
 - These treatments have limited sustained efficacy, resulting in a chronic treatment approach, and may be associated with short- and long-term side effects that impact QoL.^{5,6}
- We assessed patient and physician perceptions of current ITP treatment and treatment goals using data from I-WISH 2.0.

METHODS

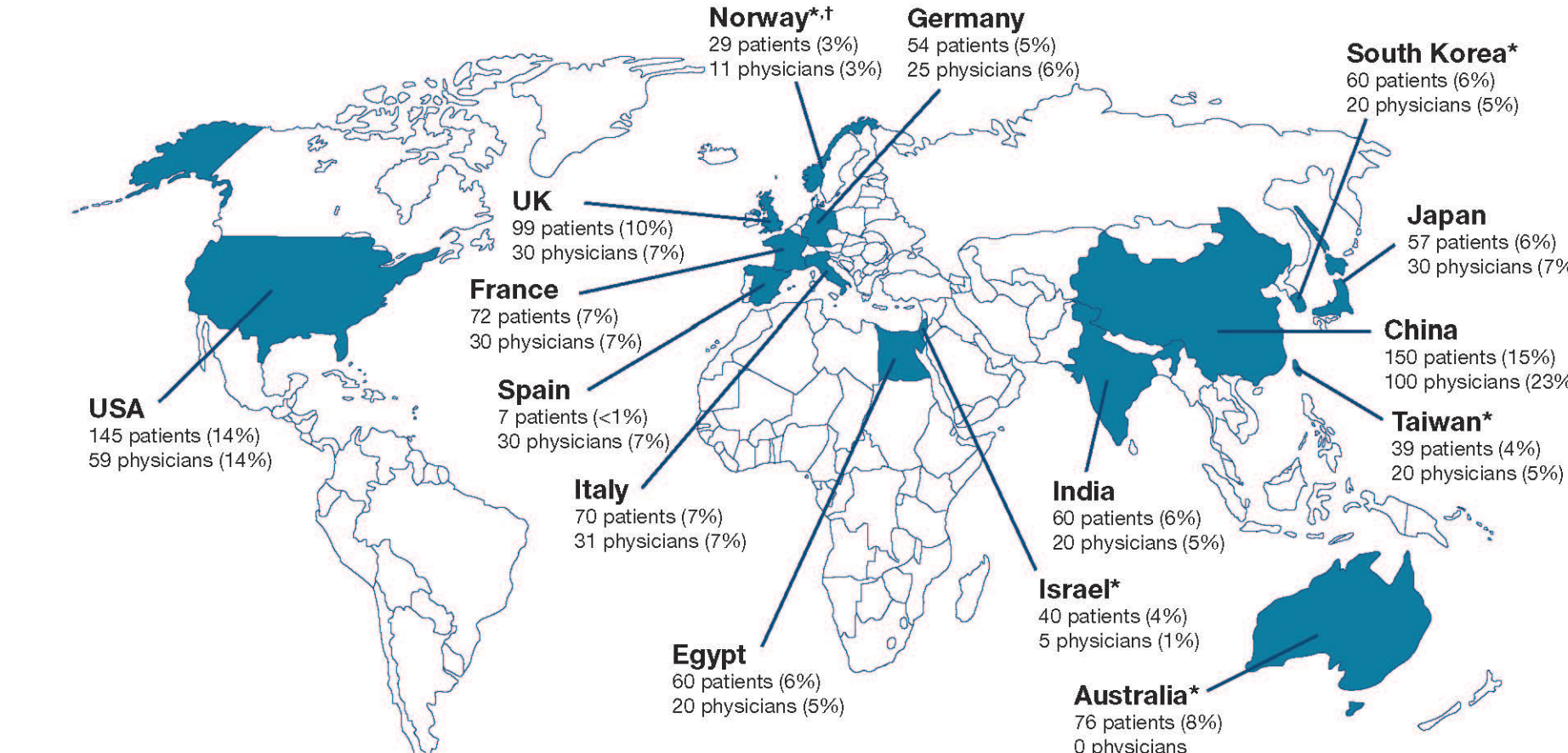
- I-WISH 2.0 was a cross-sectional survey that investigated patient and physician experiences of ITP, building on I-WISH 1.0 with additional topics and countries.
- Patients aged >18 years with a diagnosis of ITP and physicians who managed at least three patients with ITP at screening were surveyed.
- Patients were recruited by their physician or patient advocacy group, and physicians were recruited by Adelphi fieldwork partners.
- The surveys were developed by a steering committee with support from Novartis and Adelphi Real World and comprised expert ITP physicians and leading patient advocates.
- Data were collected on a range of topics, including prescribed ITP treatments, their impact on QoL, treatment satisfaction and treatment goals.
- Some responses to questions were classified on a 1–7 Likert scale (1 = no impact/agreement; 5–7 = high impact/agreement).

RESULTS

Survey disposition

- From February to July 2022, 1018 patients and 431 physicians from 15 countries completed I-WISH 2.0 (Figure 1)
- 83% of patients (n/N=844/1018) and 84% of physicians (n/N=361/431) had not completed the previous I-WISH 1.0.

Figure 1. Number of patients and physicians per country who completed I-WISH 2.0

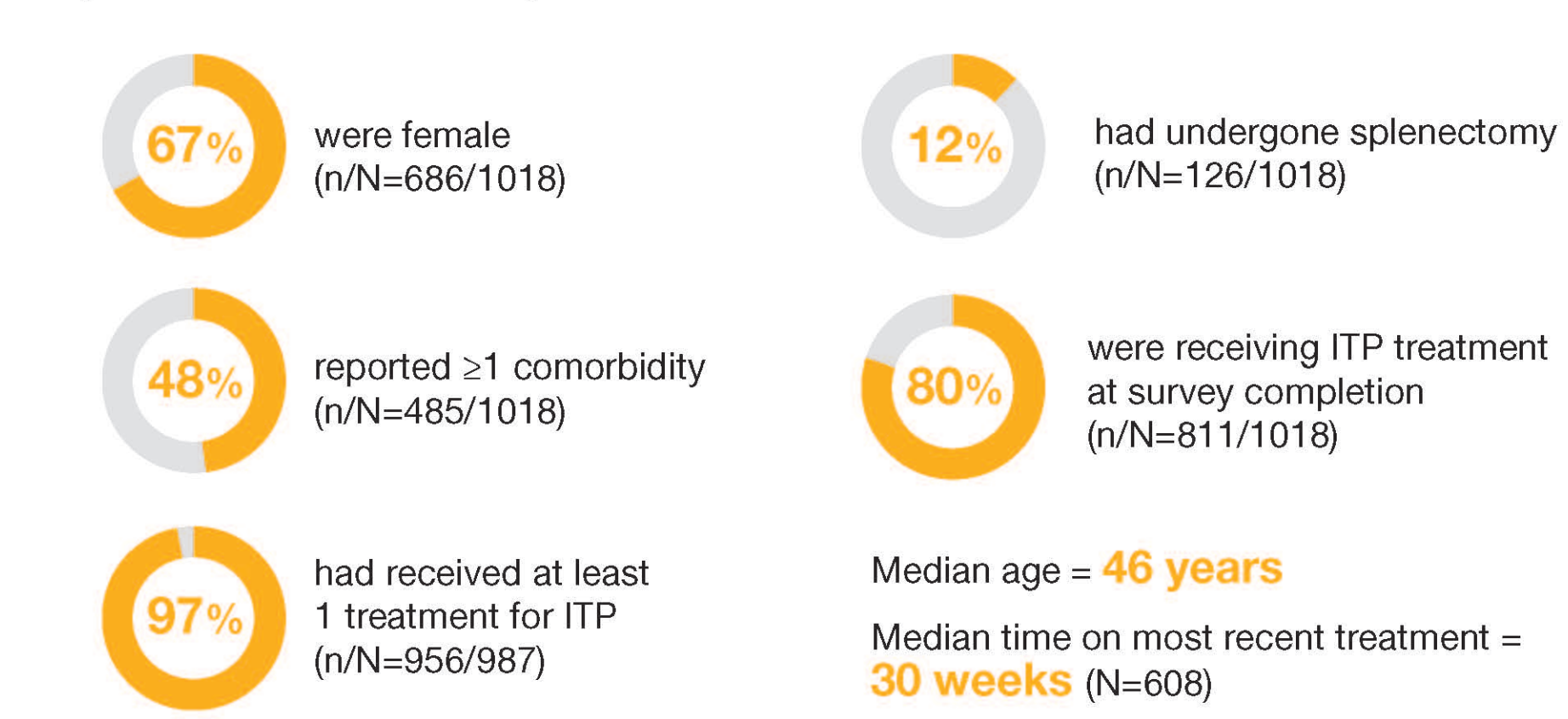


*Participated for the first time in I-WISH 2.0 (three countries that participated in I-WISH 1.0 did not participate in I-WISH 2.0: Canada, Colombia, Turkey); †The patient questionnaire in Norway did not include questions on ITP treatment

Baseline characteristics

- Figure 2 shows the key baseline characteristics of patients surveyed.
- 75% of physicians surveyed (n/N=324/431) had been treating patients with ITP for >10 years
 - The median case load was 20 (interquartile range = 10–35) patients with ITP.

Figure 2. Patient demographics

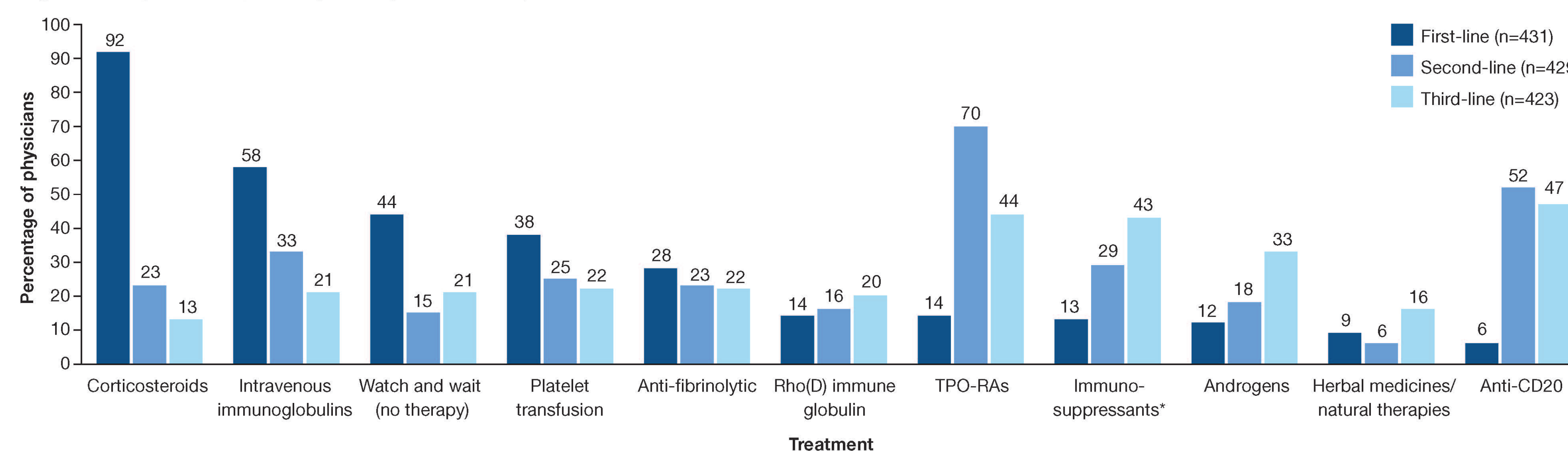


n represents the total number of participants who gave this answer, and N denotes the total number of participants who completed the question ITP, immune thrombocytopenia

Treatment patterns in ITP

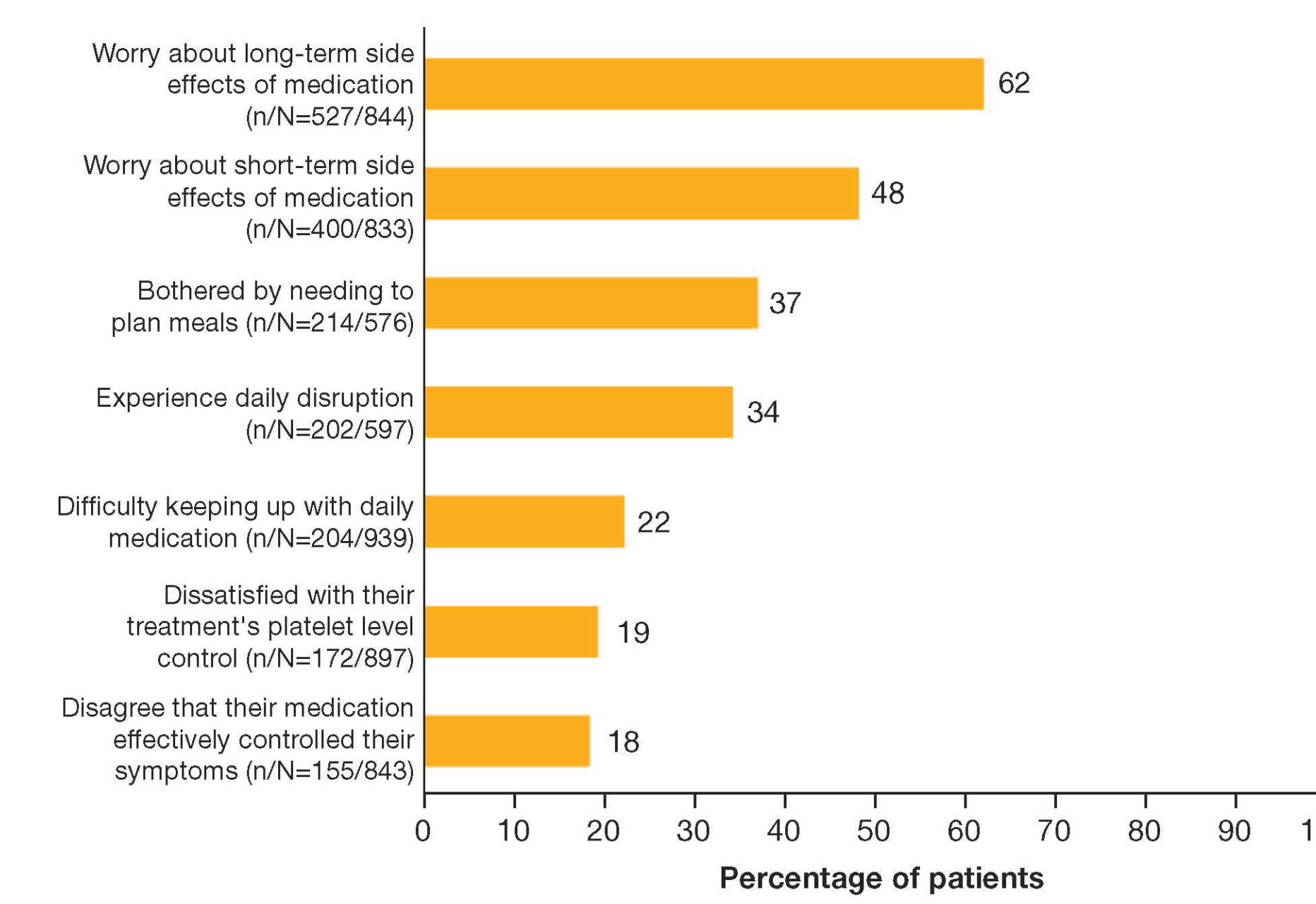
- The most common first- and second-line treatments prescribed by physicians were corticosteroids and TPO-RAs, respectively (Figure 3); for third-line treatment, the most common were immunosuppressants, TPO-RAs and anti-CD20 at similar proportions.
- Physicians estimated that 15% of their patients have opted against receiving ITP treatment (N=431).
- 73% (n/N=313/431) of physicians considered their patients' perception of medications when making treatment decisions.

Figure 3. Physician-reported prescription of first-, second- and third-line treatments in ITP



*Eg cyclosporine, azathioprine, cyclophosphamide or mycophenolate mofetil ITP, immune thrombocytopenia; TPO-RA, thrombopoietin receptor agonist

Figure 4. Burden of ITP treatment and treatment satisfaction for patients



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Satisfaction with current ITP treatments and their burden on patients

- Patients reported a significant burden due to their current ITP medication (Figure 4)
 - Most patients were worried about long-term side effects and almost half were worried about short-term side effects
 - Approximately one-third of patients experienced daily disruption because of their current ITP treatment.
- Almost half of physicians did not report being satisfied or very satisfied with current treatment options (Figure 5).

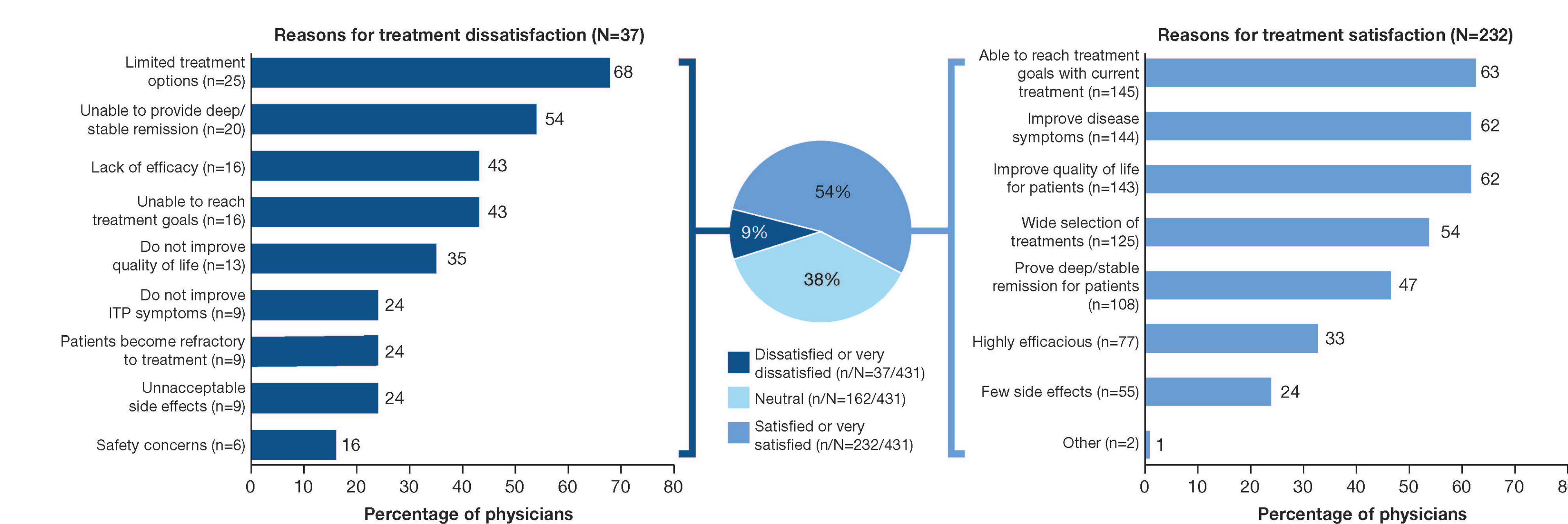
Treatment goals for ITP

- 32% (n/N=327/1016) and 13% (n/N=127/1016) of patients believed they were not or did not know if they were in sustained remission, respectively.
- When asked to rate the importance of offering a sustained remission or cure for ITP as a treatment goal, most patients considered it to be important (82% [n/N=823/1007]).
- Of treatment goals mentioned in their top three, the most common for patients included healthy blood counts, improving QoL and increasing energy levels, and for physicians, included reducing spontaneous bleeds, healthy blood counts and improving QoL (Figure 6).

Patient preferences for limiting time on ITP treatment

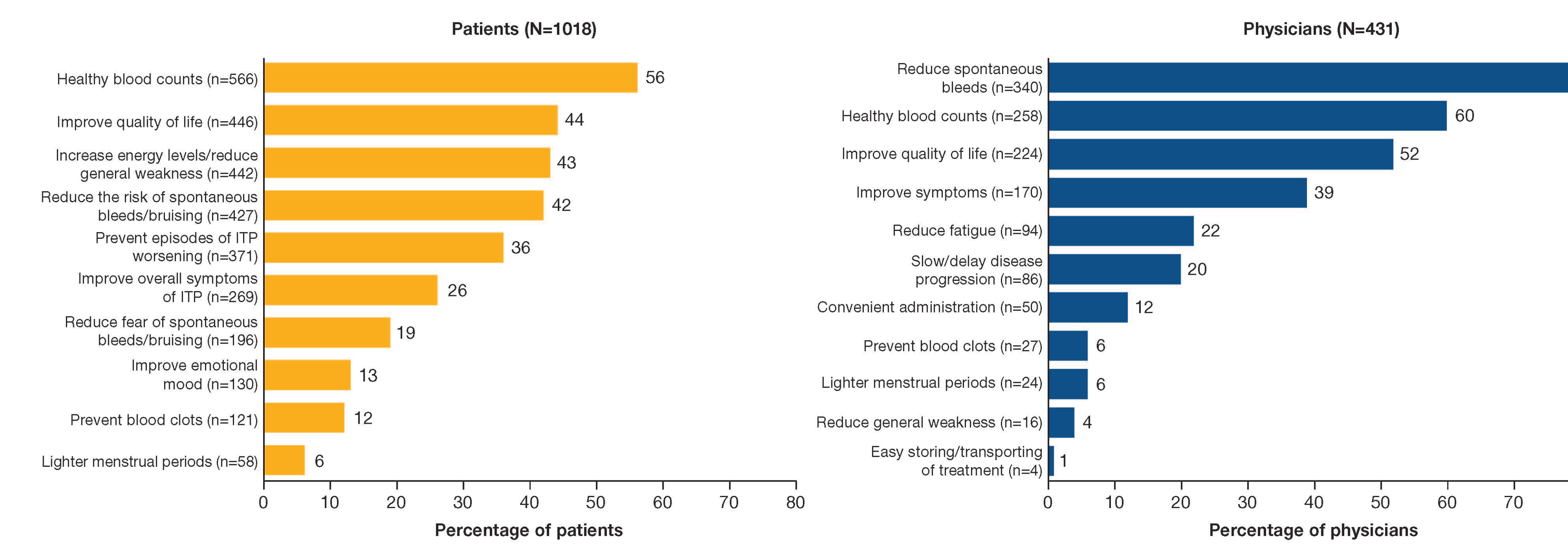
- Limiting their time on treatment would substantially affect treatment preferences for 66% (n/N=668/1009) of patients.
- 28% (n/N=241/851) of patients had paused treatment at some point during ITP management, most commonly because their disease was under control (42% [n/N=101/241]), their platelet count was stable for a long time (25% [n/N=60/241]) or they experienced side effects (22% [n/N=53/241])

Figure 5. Physician satisfaction with current treatment options for ITP



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Figure 6. Treatment goals mentioned in the "top three" for patients and physicians



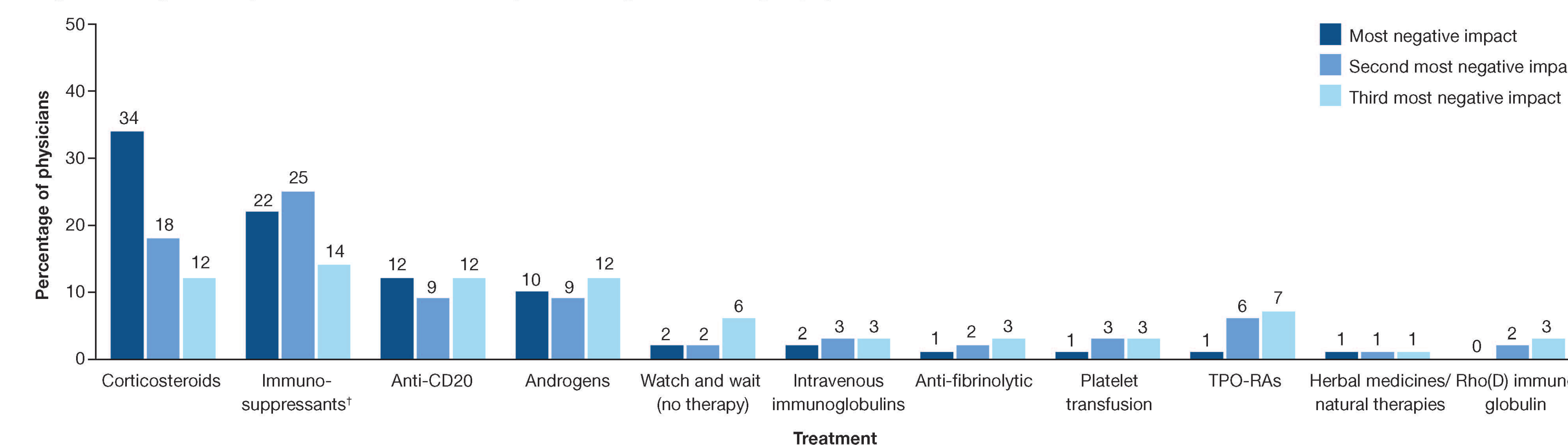
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- The median longest time off treatment reported by patients (N=225) was 26 (interquartile range = 8–104) weeks, with most patients reporting that the reason for restarting treatment was because their platelet count was getting lower (67% [n/N=161/241]) or they started to experience bleeding symptoms (26% [n/N=62/241]).
- 38% (n/N=294/775) of patients did not want to take their ITP treatment for the foreseeable future
 - However, relapse if treatment was stopped was a concern for 67% (n/N=680/1013) of patients.

Fatigue and immunosuppression

- Immunosuppression due to ITP treatment worried 53% (n/N=414/787) of patients, and 67% (n/N=679/1007) thought immunosuppression was important to consider when treatment decisions are made.
- Physicians considered corticosteroids and other immunosuppressants to have the greatest negative impact on fatigue (Figure 7)
 - Despite this, only 12% (n/N=52/431) strongly agreed they aimed to limit immunosuppressive effects.

Figure 7. Negative impact of ITP treatments on patient fatigue according to physicians* (N=431)



*Physicians were asked to rank their top three treatments for their negative impact on fatigue; †Eg cyclosporine, azathioprine, cyclophosphamide or mycophenolate mofetil ITP, immune thrombocytopenia; TPO-RA, thrombopoietin receptor agonist

Acknowledgments

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