

# Patients' and Physicians' Perspectives on Treatment in ITP – A Multi-Country Perspective: Results From the ITP World Impact Survey (I-WISH)

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## Background

- Treatment strategies for immune thrombocytopenia (ITP) aim to achieve a platelet count associated with adequate hemostasis while minimizing treatment-emergent toxicities and maximizing the patient's quality of life (QoL).
- The I-WISH survey gathered data on the burden of ITP and its impact on QoL. Here, we describe patients' and physicians' perceptions of treatment goals and patients' prescribed ITP treatments, across multiple countries.

## Methods

- I-WISH was an exploratory, cross-sectional survey of 1,507 patients with ITP and 472 physicians across 13 countries; patients were recruited via physicians and patient advocacy groups.
- Patients and physicians completed a 30-minute online survey, designed and endorsed by a steering committee of expert physicians and patient advocacy group ITP specialists.
- Patients and physicians were asked: "Other than a cure for your ITP condition, what are your 3 most important treatment goals?" Multiple choice answers were allowed. Patients ranked from 1 to 3 a set of 10 treatment goals, with 1 being the treatment goal considered the most important.
- Patients were also asked to indicate all received treatments, such as corticosteroids (CSs), thrombopoietin receptor agonists (TPO-RAs), anti-CD20 monoclonal antibodies (anti-CD20), and intravenous immunoglobulin, at any time prior to or at the time of survey; responses were used to determine prescribed treatment patterns for ITP.

## Results

### Patient Characteristics

- Mean patient age was 47 years, and 65% of patients were female.
- Symptom burden was reported as low to moderate by 43% of patients, high to very high by 39%, and unknown for 18% of responders.
- Median length of time with ITP varied among the countries, and it was highest in Canada, the USA, and the UK (13, 10, and 9 years, respectively).
  - Median duration of ITP in the other countries surveyed was: France (5 years); India (4.2 years); Germany (3.7 years); Italy, China, and Egypt (3 years); Spain (2.8 years); Colombia (2 years); Turkey (1 year); and Japan (0.3 years).

### Differences in Perceptions of Treatment Goals Between Patients and Physicians: Multi-Country

- Patients: Across all 13 countries, the top 3 treatment goals for patients were healthy blood counts (64%), preventing episodes of worsening ITP (44%), increasing energy levels (41%), reducing spontaneous bleeds/bruising (38%), and improving QoL (38%).
  - 43% and 17% of patients selected healthy blood counts or reducing spontaneous bleeds, respectively, in their top 3 goals; 21% of patients selected both answer choices and 19% of patients neither option.
  - 29% and 26% of patients selected increasing energy levels or improving QoL, respectively, in their top 3 goals; 12% of patients selected both answer choices and 33% of patients neither option.
- Physicians: The most frequently perceived top 3 treatment goals for physicians across all countries were: reduction in spontaneous bleeds (72%), improving QoL (64%), and healthy blood counts (51%).
  - 18% and 39% of physicians selected healthy blood counts or reducing spontaneous bleeds, respectively, in their top 3 goals; 33% of physicians selected both answer choices and 10% of physicians neither option.
  - 7% and 56% of physicians selected reducing fatigue or improving QoL, respectively, in their top 3 goals; 7% of physicians selected both answer choices and 29% of physicians neither option.

- Overall, across all 13 countries, physicians overestimated the importance of the following treatment goals to patients: reduction in spontaneous bleeds/bruising (72% vs 38%) and improving QoL (64% vs 38%). They underestimated the importance of healthy blood counts (51% vs 64%) and fatigue/increasing energy levels (15% vs 41%). These results perhaps reflect different viewpoints on the same goals.

### Differences in Perceptions of Treatment Goals Between Patients and Physicians in Different Countries

#### Reduction in Spontaneous Bleeds

- The highest proportions of patients who rated reduction in spontaneous bleeds as 1 of their top 3 treatment goals were from China (59%), Italy (58%), Spain (54%), and Egypt (50%) (**Figure 1A**).
- The highest proportions of physicians who rated reduction in spontaneous bleeds as 1 of their top 3 treatment goals were from France (82%), India and Turkey (both 81%), Egypt (80%), and China (75%) (**Figure 1B**).
- The largest differences between physicians' and patients' perceptions of the importance of reduction in spontaneous bleeds/bruising were observed in France (82% vs 24%), India (81% vs 28%), Canada (73% vs 31%), the UK (74% vs 33%), and the USA (66% vs 26%).
  - Physicians in all countries overestimated the importance of this goal compared with the rating given by patients (**Figure 1A-C**).

#### Healthy Blood Counts

- The highest proportions of patients who rated healthy blood counts as 1 of their top 3 treatment goals were from the USA (76%), the UK (73%), Canada (69%), France (66%), Turkey (65%), and India (65%) (**Figure 1A**).
- The highest proportions of physicians who rated healthy blood counts as 1 of their top 3 treatment goals were from Colombia (88%), Turkey (76%), the USA (75%), Canada (63%), and Japan (61%) (**Figure 1B**).
- The largest differences between physicians' and patients' perceptions of the importance of healthy blood counts were observed in Colombia (88% vs 45%), Egypt (30% vs 13%), and Turkey (76% vs 65%), where physicians overestimated the importance of this goal, and in the UK (29% vs 73%), India (29% vs 65%), France (41% vs 66%), and Germany (37% vs 61%), where physicians underestimated the importance of this goal (**Figure 1A-C**).

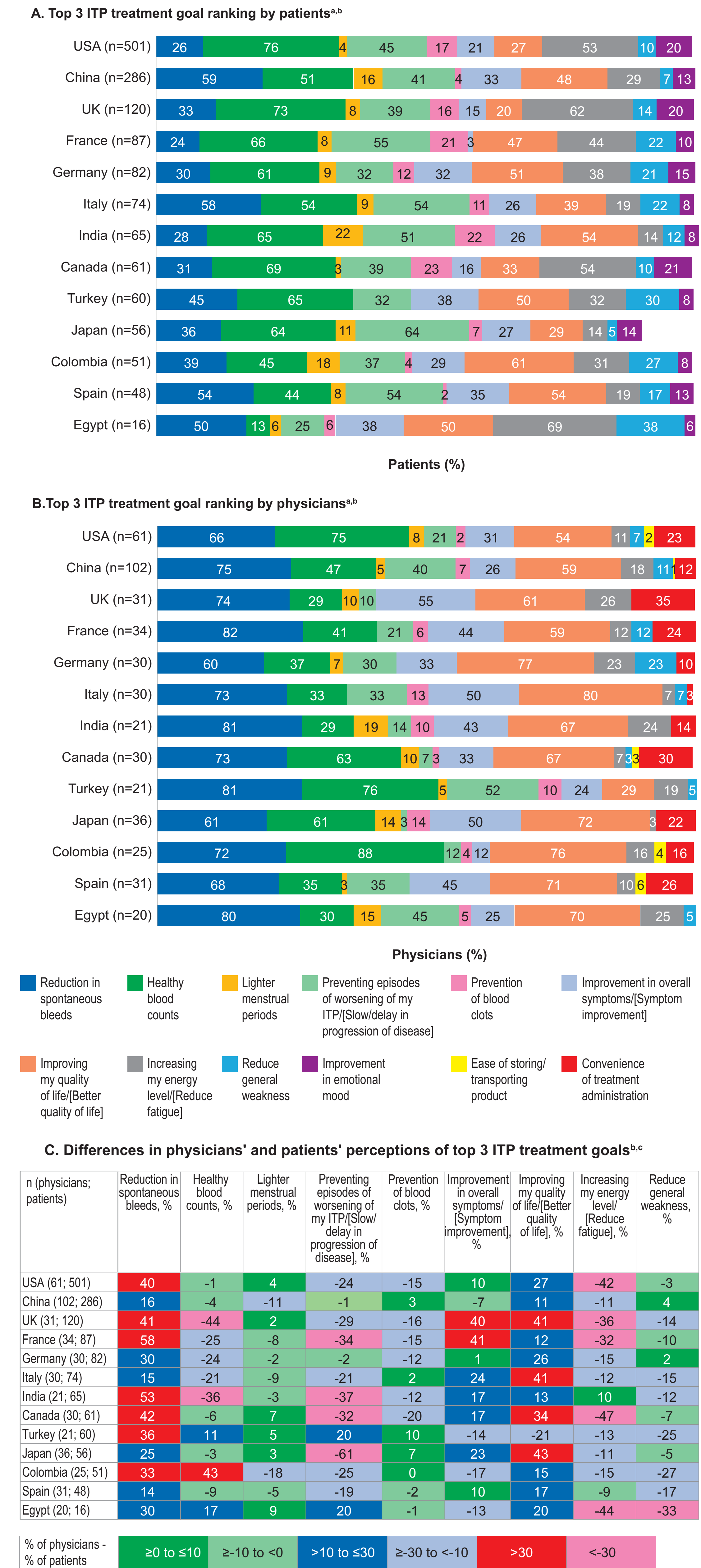
#### Improvement in Quality of Life

- The highest proportions of patients who rated improving QoL as 1 of their top 3 treatment goals were from Colombia (61%), India and Spain (both 54%), Germany (51%), and Turkey and Egypt (both 50%) (**Figure 1A**).
- The highest proportions of physicians who rated better QoL as 1 of their top 3 treatment goals were from Italy (80%), Germany (77%), Colombia (76%), Japan (72%), and Spain (71%) (**Figure 1B**).
- The largest differences between physicians' and patients' perceptions of the importance of QoL were observed in Japan (72% vs 29%), Italy (80% vs 39%), the UK (61% vs 20%), and Canada (67% vs 33%). The importance of QoL was overestimated by physicians across all countries except for Turkey, where physicians underestimated it compared with patients (29% vs 50%) (**Figure 1A-C**).

#### Fatigue Reduction/Increase in Energy Levels

- The highest proportions of patients who rated increase in energy levels as 1 of their top 3 treatment goals were from Egypt (69%), the UK (62%), Canada (54%), and the USA (53%) (**Figure 1A**).
- The highest proportions of physicians who rated reduction of fatigue as 1 of their top 3 treatment goals were from the UK (26%), Egypt (25%), India (24%), and Germany (23%) (**Figure 1B**).
- The largest differences between physicians' and patients' perceptions of the importance of reduction of fatigue/increasing energy levels were observed in Canada (7% vs 54%), Egypt (25% vs 69%), the USA (11% vs 53%), and the UK (26% vs 62%), with physicians underestimating it as a treatment goal across all countries except for India (24% vs 14%), compared with the rating given by patients (**Figure 1A-C**).

### Figure 1. Differences in Patients' and Physicians' Perspectives of Top-Ranking ITP Treatment Goals



\*Physicians and patients were asked: "Other than a cure for ITP, what are your 3 most important treatment goals?" Responders were asked to assign rankings (1 to 3) to the treatment goals listed, with 1 being the most important. The same treatment goals were listed for both physicians and patients except for ease of storing/transporting product and convenience of treatment administration (physicians only) and improvement in emotional mood (patients only). In some cases, the language was adapted to the target audience, i.e. increase my energy levels/reduce fatigue for patients/physicians, respectively. For questions that were asked only to physicians or patients, goals are only shown in the relevant chart. \*\*Countries are listed from the highest to the lowest number of patients who participated in the survey. Treatment goals are listed based on the following categories: clinical symptoms (reduction of spontaneous bleeds, healthy blood counts, lighter menstrual periods), disease prevention (preventing episodes of worsening ITP, prevention of blood clots, improvement in overall symptoms) and health-related QoL (improving my quality of life, increasing my energy, reduce general weakness). \*Negative numbers represent greater % of patients compared with the % of physicians ranking the respective goal in the top 3. ITP, immune thrombocytopenia; QoL, quality of life.

### ITP Treatment Trends

#### Corticosteroids

- Average CS use was 79% at any time prior to the survey across all countries but had been reduced substantially to 26% at the time of survey.
  - Median duration of ITP for patients on CSs compared with those not on CSs at the time of survey was 3.0 years and 5.9 years, respectively.
  - Countries with the highest CS use at any time during ITP management were Colombia (90%), France (85%), and the USA (82%) (**Figure 2A**).
- Lowest CS use at any time prior to the survey was reported by patients in Japan (64%), whereas the highest usage was reported by patients in Colombia (90%) (**Figure 2A**).
- CS use at the time of survey was reduced compared with CS use at any time prior to the survey; the highest proportions of patients on CSs at the time of survey were in Colombia (59%), India (58%), and Egypt (44%) (**Figure 2B**).
  - Countries with the highest proportion of patients on CSs at the time of survey had a median ITP duration of 1 year in Colombia and 3 years in both India and Egypt.
  - 13% of surveyed patients in Canada were on CSs at the time of survey and had the highest median ITP duration (16.5 years) among all countries studied.

#### Thrombopoietin Receptor Agonists

- Average use of TPO-RAs was 27% across all countries and at any time prior to the survey; at the time of survey, 17% of patients across all countries were on TPO-RAs.
- Countries with the highest TPO-RA use at any time during ITP management were Turkey (40%), the UK and Egypt (both 31%), and the USA (29%) (**Figure 2A**).
- At the time of survey, TPO-RAs were most used by patients in Egypt (31%) and were least used in Japan (7%) (**Figure 2B**).
- TPO-RA use was surprisingly high in several of the countries, perhaps owing to the limited numbers of patients who completed the survey.

#### Anti-CD20 Monoclonal Antibodies

- Average use of anti-CD20s was 29% across all countries and at any time prior to the survey; at the time of survey, 5% of patients across all countries were receiving anti-CD20s.
- Countries with the highest anti-CD20 use at any time during ITP management were the USA (45%), France (43%), and Canada (36%) (**Figure 2A**).
- At the time of survey, anti-CD20s were most used by patients in India (14%); no patients used anti-CD20s in Turkey and Egypt (**Figure 2B**).

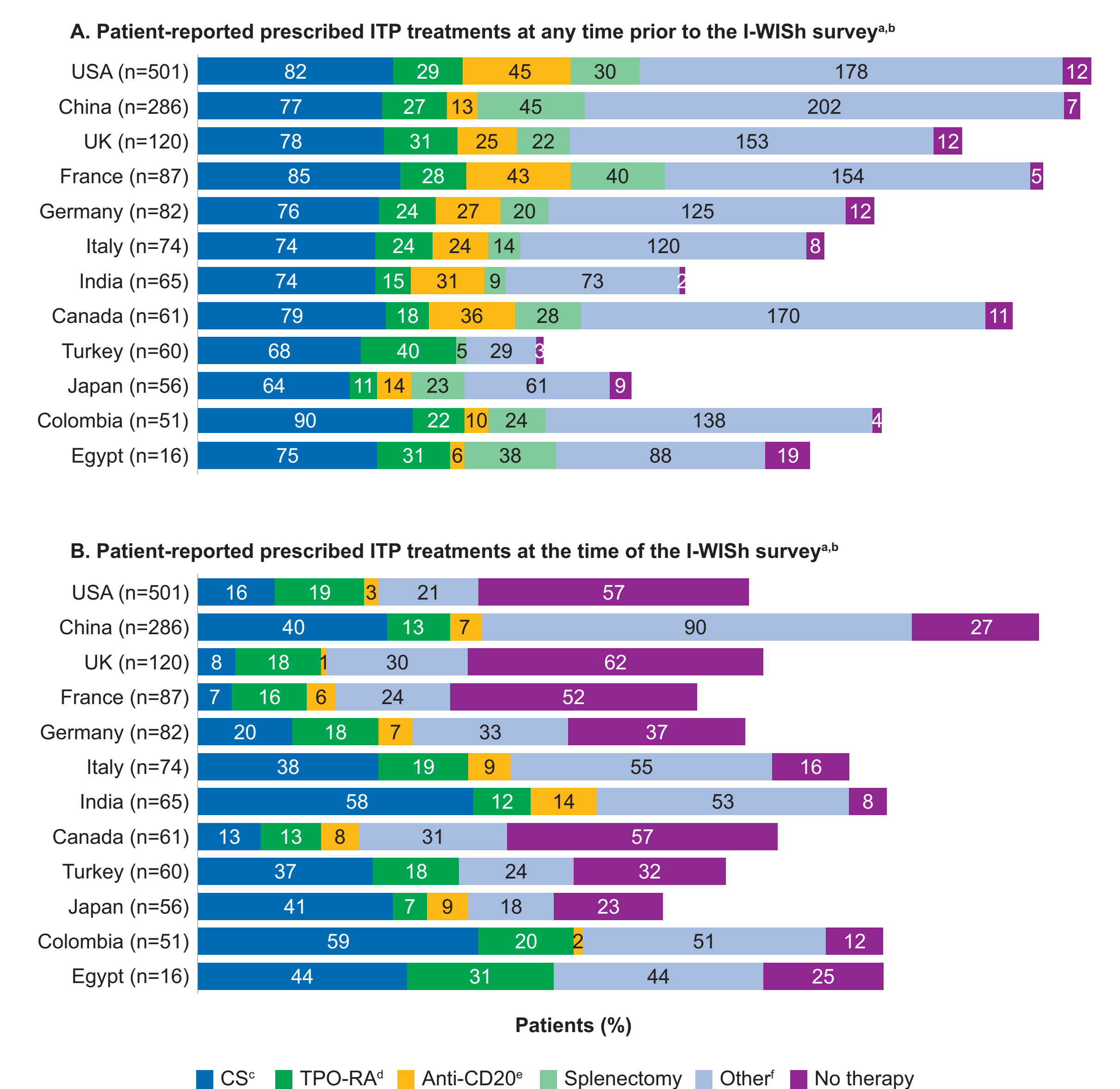
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## Disclosures

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### Figure 2. Utilization Patterns of CSs, TPO-RAs, Anti-CD20, Splenectomy, and Other ITP Treatments Between Countries



\*Patients were asked to select all treatments they had received at any time prior to/at the time of the survey to help manage their ITP. Because patients could have been receiving more than 1 treatment, the total percentages add up to more than 100%. \*\*Treatment-related questions were not allowed in Spain; \*CSs include prednisolone, methylprednisolone, or dexamethasone. \*TPO-RAs include eltrombopag and romiplostim. \*Anti-CD20s include rituximab, veltuzumab, or ofatumumab. \*Other treatments include androgens, anti-fibrinolytics, immunosuppressants, cyclosporine, intravenous immunoglobulin, platelet transfusion, Rhlg, and treatments that patients could not remember. CS, corticosteroid; ITP, immune thrombocytopenia; Rhlg, Rho(D) immune globulin; TPO-RA, thrombopoietin receptor agonist.

## Conclusions

- Physicians across all countries ranked reduction in spontaneous bleeds as the most important goal.
- Patients also noted reduction in spontaneous bleeds as an important treatment goal, but more patients believed increasing energy levels to be an important top 3 treatment goal.
- Improving QoL was selected as a top treatment goal more frequently by physicians than patients (64% vs 38%). However, fewer physicians than patients (15% vs 41%) considered fatigue to be as important.
- Limitations to some of these conclusions are the similarity between answers (e.g. healthy blood counts and reduction of spontaneous bleeds), which may have led to different responses despite analogous interpretations. Similarly, fatigue is a key component of QoL. Thus, cognate answer choices in the survey may have led to some of the apparent differences in perceptions observed between physicians and patients.
- The availability of new therapies is reflected in the treatment trends over time. Usage of TPO-RAs and anti-CD20s at any time was similar, but each was less than 30%. Access to advanced therapies may differ from country to country, health economics may change, and limited patient participation may give an unbalanced view of care in that country. All of these factors may influence both the perceived and real treatment trends observed in the clinical practice of the respective country.

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