

Patient preferences regarding thrombopoietin-receptor agonists for immune thrombocytopenia in Italy (TRAPeze ITA study)

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INTRODUCTION

- Immune thrombocytopenia (ITP) is a rare autoimmune disorder characterised by impaired platelet production and increased platelet destruction, resulting in an overall reduction in platelet count and increased bleeding risk¹
- Thrombopoietin-receptor agonists (TPO-RAs [eltrombopag, romiplostim and avatrombopag]) are standard of care for patients lacking optimal response to first-line therapies (e.g. corticosteroids, immunoglobulins)²
- It has previously been determined that choice of TPO-RA can be patient-led, based on preferred route of administration³
- The ongoing TRAPeze (Thrombopoietin-Receptor Agonist Patient experience) survey explores specific factors influencing ITP patient preference towards TPO-RAs, particularly regarding product attributes⁴
- Findings from the TRAPeze study in the UK and Netherlands were published in 2021⁴ and 2022⁵, respectively
- The Italian arm of the study ran from January 17th through February 28th 2022, and results are presented here

OBJECTIVES

- In the Italian cohort:
 - Explore patient preference towards TPO-RAs
 - Capture the demographics of ITP patients, the characteristics of ITP disease and its clinical and social impact

METHODS

TRAPeze

- A descriptive, cross-sectional, exploratory observational study of adults with ITP in Europe, who are currently receiving, or have previously received, a TPO-RA
- Delivered via web platform SurveyEngine®, as an online questionnaire, comprising a discrete choice experiment and a section on patient burden

Participant recruitment

- Via the Associazione Italiana Porpora Immune Trombocitopenica (AIPIT) website and mailing list
- Inclusion criteria:
 - ≥18 years
 - Formal diagnosis of primary ITP according to ICR⁶ and ASH⁷ guidelines
 - Received a TPO-RA for a period of at least 3 months in the last 12 months

Patient burden questionnaire

- Questions related to respondent demographics, disease characteristics, direct healthcare resource utilisation and wider social impact
- Questions on current and previous treatments and overall therapy satisfaction were included in UK and NL TRAPeze questionnaires, but not in the Italian questionnaire.

- The Italian version ensured relevance and cultural sensitivity of the questions

Discrete choice experiment

- Elicited respondent attitudes towards existing TPO-RA treatments
- Respondents were presented with 10 sets of scenarios and, for each set, they were asked to choose between two hypothetical therapy scenarios
- Each hypothetical therapy scenario was depicted as a list of TPO-RA product attributes and levels (Table 1) that alternated between sets

Table 1: List of discrete choice experiment attributes and levels to measure respondent preference for TPO-RA treatment in ITP

Attributes	Levels
Method of administration	Oral
	Subcutaneous injection
Frequency of dosing	Twice daily
	Once daily
	Once weekly
Drug and food interactions	Must be taken 2 hours before or 4 hours after food containing dairy products or calcium; indigestion remedies (antacids); or mineral supplements
	No restrictions
Monitoring	Twice a month (measuring platelet count only)
	Once a month (measuring platelet count only)
	Twice a month (two separate tests: one for measuring platelet count and one for liver function)
Platelet response	Speed of response – platelet count improves soon after starting treatment (within 1 to 2 weeks)
	Sustainability of treatment – safe and stable platelet count on this treatment
Potential side effects	Headache (affects more than 1 in 10 people) ^{8, 9}
	Fatigue (affects up to 1 in 10 people) ^{8, 9}
	Thromboembolic event (affects more than 1 in 100 people but less than 1 in 10 people) ^{8, 9}
	Nausea (affects up to 1 in 10 people) ^{8, 9}

Data analysis

- Responses to patient burden questions were summarised with descriptive statistics
- Responses from the discrete choice experiment were analysed using a panel-data mixed logit model to infer a pattern of attributes that respondents prioritise

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RESULTS

Survey participation

- A total of 76 Italian participants completed the survey, of which all completed the discrete choice experiment and 69 completed the questionnaire on patient burden

Patient burden questionnaire

Demographics and disease characteristics

- 80% (n=55) of respondents were female and ages ranged from 18–73 years (mean [standard deviation (SD)] 45.4 years [14.0 years])
- Mean (SD) age at ITP diagnosis was 31.7 years (16.3 years) and mean time since diagnosis was 13.7 years (14.5 years)
- Mean (range) health score was 7.1 (1–10) out of 10 (10 being excellent health) and mean (range) platelet count was 98.2x10⁹/L (4.0–400x10⁹/L)
- Bruising was the most common ITP symptom experienced, but fatigue was ranked as most negatively impactful on quality of life (Fig. 1)

Figure 1: Signs and symptoms experienced by ITP patients and ranking of ITP signs and symptoms by most negatively impactful on quality of life (n=69).

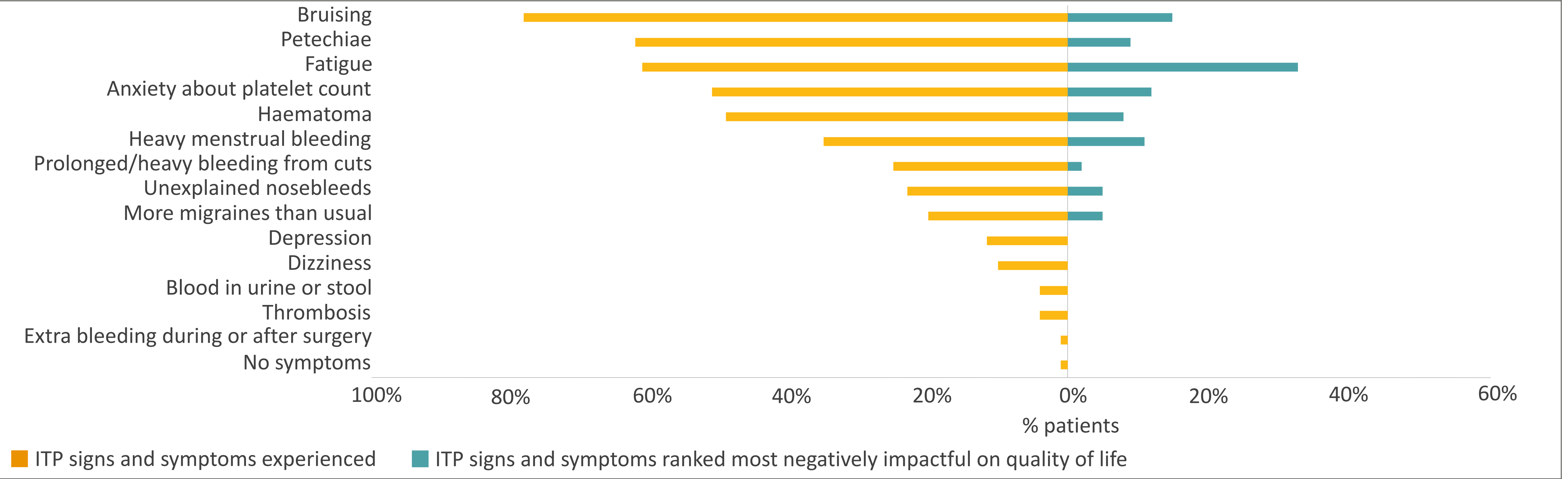
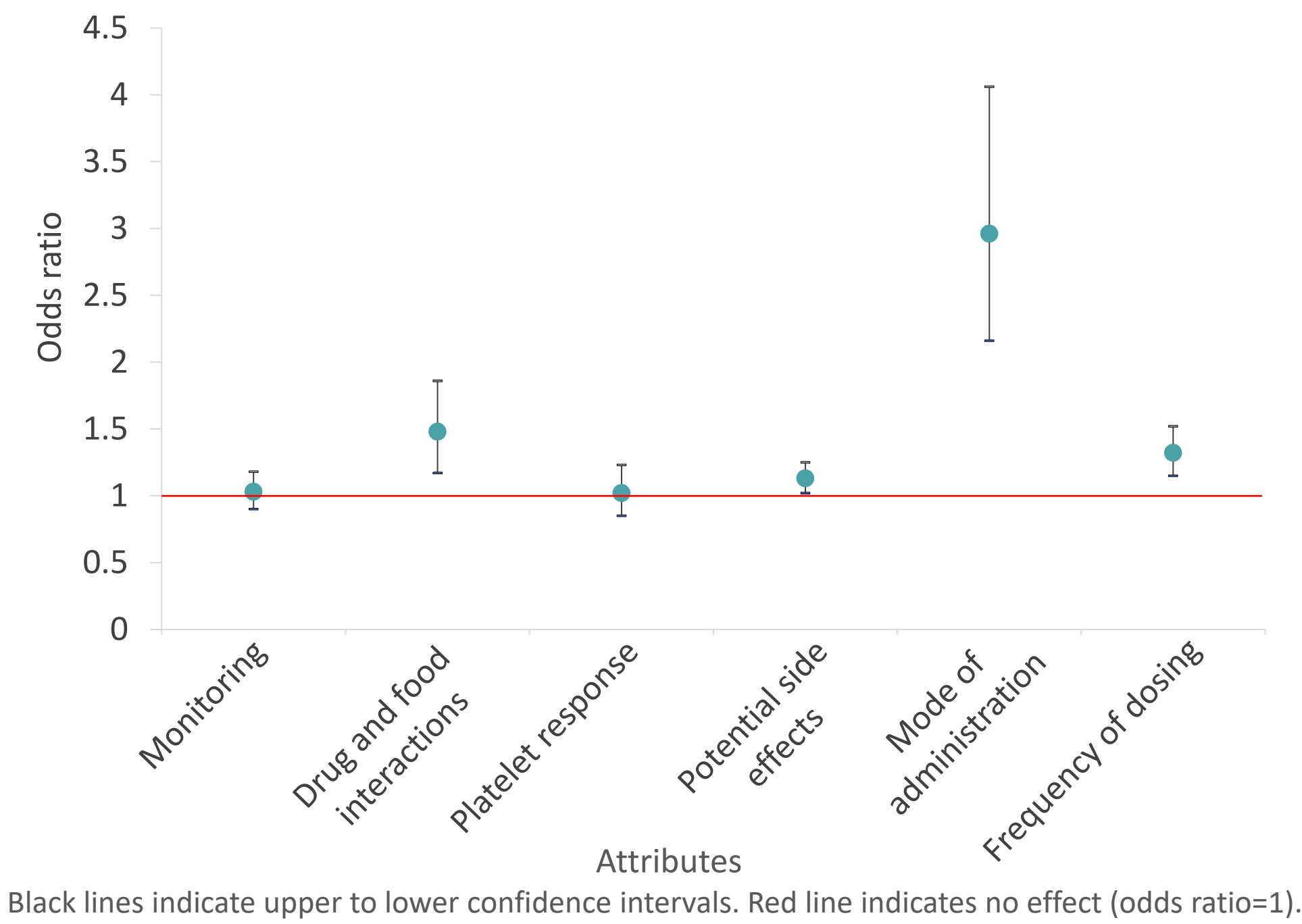


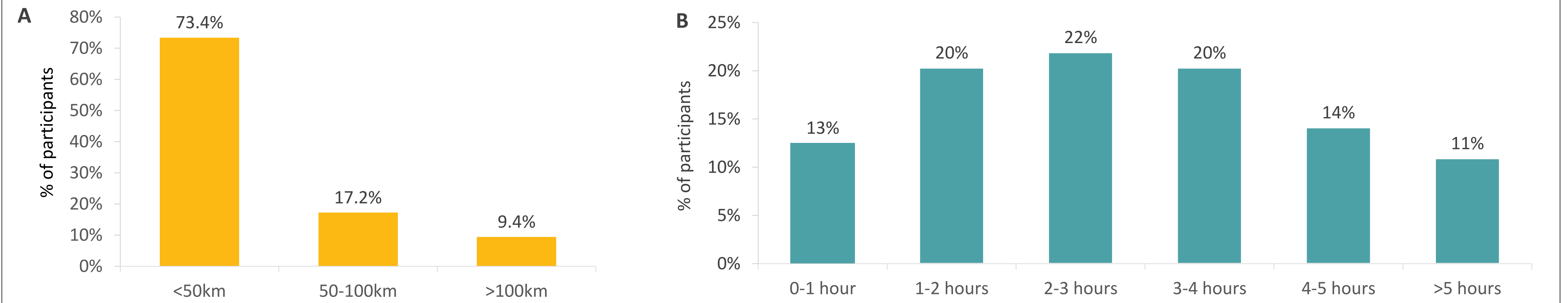
Figure 2: TPO-RA treatment preference (n=76) indicated in the discrete choice experiment.



Healthcare resource utilisation

- Median (range) visits over 6 months prior to COVID-19 (Oct 2019–Mar 2020) was 5 (0–10). Median (range) visits since COVID-19 (Mar 2020–Feb 2022) was also 5 (0–10), despite difference of 15 months between timeframes.
- 73.4% (n=47) of respondents travelled less than 50km for specialist hospital appointments (Fig. 3A)
- 67.2% (n=43) of specialist hospital appointments lasted more than 2 hours (Fig. 3B)

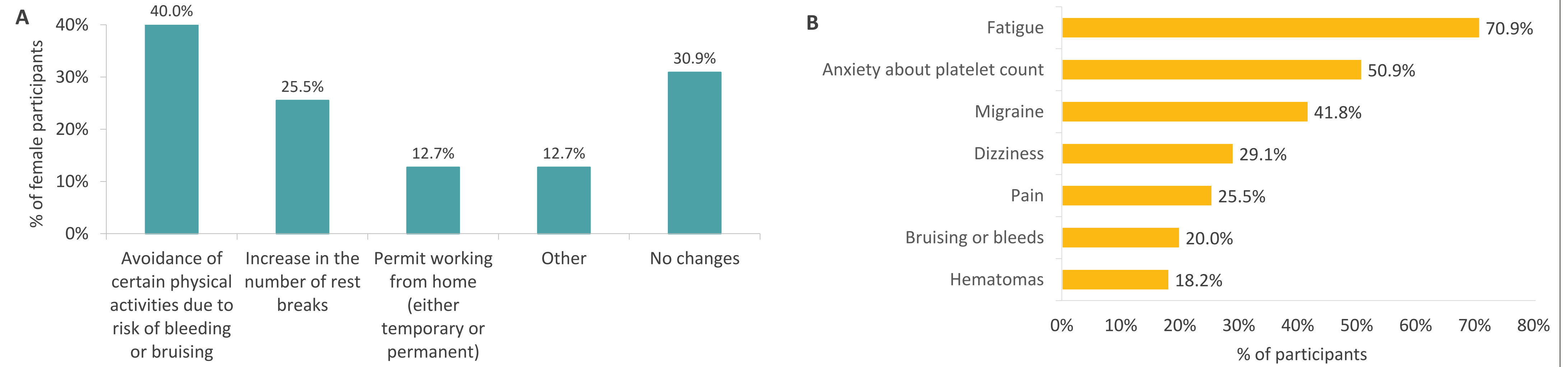
Figure 3: A) Distance travelled to attend hospital consultant appointments due to ITP (n=64). B) Typical duration of specialist hospital consultant appointments for ITP (n=64).



Work and productivity

- Some respondents reported job changes due to ITP (Fig. 4A). Avoidance of certain physical activities due to bleeding/bruising (40.0%, n=18) and increase of rest breaks (25.5%, n=14) were most commonly reported. Nearly a third (30.9%, n=17) of respondents made no changes to their job
- ITP symptoms, especially fatigue (70.9%, n=39) had an impact on respondent’s ability to fulfil employment responsibilities (Fig. 4B)
- Just over two thirds (68.6%, n=35) of respondents made no changes in weekly working hours due to ITP

Figure 4: A) Changes respondents made to their job due to ITP (n=55). B) ITP symptoms that have influenced respondents’ ability to fulfill employment responsibilities (n=55).



Wider social impact

- ITP affected some respondents decision to have children. Female respondents were most concerned about bleeding during pregnancy or childbirth (36%, n=18), their children inheriting ITP (36%, n=18) and their ITP affecting the platelet count of the child (32%, n=16)
- Most respondents felt that ITP impacted their relationships, with the most impacted relationships being that with their family (84%, n=57), partner (77%, n=48) and friends (68%, n=45)

CONCLUSIONS

- The gender distribution of the Italian cohort was representative of the wider clinical population, with a majority of respondents being female
- Primary drivers of TPO-RA preference were mode of administration, drug and food interactions, and frequency of dosing
 - Further DCE analysis also found that potential side effects were a notable driver of choice in the Italian cohort
- Though most respondents had not reduced their number of hours worked, symptoms of ITP did impact working ability. Fatigue and platelet count anxiety were the most commonly cited symptoms influencing ability to fulfil employment responsibilities
 - Of note, however, is the finding that respondents preferred fatigue to headache as a potential side effect of TPO-RA therapy
- This study highlights the burden of ITP on patients’ relationships, healthcare providers and society
- Evidence from the patient perspective on treatment preference and disease characteristics may facilitate improved ITP management