

# Use of Eltrombopag in Patients with Chronic Idiopathic Thrombocytopenic Purpura: A Single Center Experience\*

Emre HAFIZOĞLU<sup>1</sup>, Vildan ÖZKOCAMAN<sup>2</sup>, Fahir ÖZKALEMKAŞ<sup>2</sup>

<sup>1</sup> Afyonkarahisar State Hospital, Department of Medical Oncology, Afyonkarahisar, Türkiye.

<sup>2</sup> Bursa Uludağ University Faculty of Medicine, Department of Internal Medicine, Division of Hematology, Bursa, Türkiye.

## ABSTRACT

This study aimed to evaluate the efficacy and safety of eltrombopag in the outpatient clinic of the Department of Hematology, Uludağ University Faculty of Medicine. Treatment responses and demographic characteristics of 25 patients diagnosed with refractory chronic immune thrombocytopenia (ITP) and receiving eltrombopag between January 2011 and March 2014 were analyzed retrospectively. Both platelet  $\geq 30,000/\text{mm}^3$  and  $\geq 50,000/\text{mm}^3$  values were considered as responses. Prolonged response was defined as the maintenance of platelet  $50,000/\text{mm}^3$  values for 12 weeks or longer without the need for any additional treatment, and sustained response was defined as the maintenance of platelet  $50,000/\text{mm}^3$  and at least 2 times the baseline values without the need for additional treatment. Twenty-five patients were evaluated. Ten patients (40%) had an initial platelet value of  $<15,000/\text{mm}^3$ , and 15 (60%) had  $\geq 15,000/\text{mm}^3$ . The baseline median platelet value increased from  $15,700/\text{mm}^3$  to  $30,000/\text{mm}^3$  at week 2 and to  $51,000/\text{mm}^3$  at week 6, and this level was maintained throughout the 85-week observation period. Thirteen (52%) patients had a sustained response, with a median follow-up of 18 months. In the study, participants were grouped according to splenectomy status, platelet  $<15,000/\text{mm}^3$ , and gender, and there was no statistically significant difference in response. Eltrombopag is an effective and well-tolerated therapeutic agent in patients with chronic ITP refractory to other therapies and at increased bleeding risk. Responsiveness is independent of splenectomy, concomitant therapy, or baseline platelet values.

**Keywords:** Chronic immune thrombocytopenia. Refractory disease. Treatment. Eltrombopag.

## Kronik İdiyopatik Trombositopenik Purpura Tanılı Hastalarda Eltrombopag Kullanımı: Tek Merkez Deneyimi

## ÖZET

Bu çalışmada, Bursa Uludağ Üniversitesi Tıp Fakültesi Hematoloji Bilim Dalı polikliniğine başvuran hastalarda eltrombopag tedavisinin etkinliği ve güvenilirliğini değerlendirmeyi amaçladık. Ocak 2011 ile Mart 2014 tarihleri arasında refrakter kronik immün trombositopeni (ITP) tanısı konan ve eltrombopag alan toplam 25 hastanın tedavi yanıtları ve demografik özellikleri retrospektif olarak analiz edildi. Hem trombosit  $\geq 30.000/\text{mm}^3$  hem de  $\geq 50.000/\text{mm}^3$  değerleri yanıt olarak kabul edilmiştir. Uzamış yanıt, trombosit  $50.000/\text{mm}^3$  değerlerinin herhangi bir ek tedaviye ihtiyaç duyulmadan 12 hafta veya daha uzun süre korunması ve sürekli yanıt, trombosit  $50.000/\text{mm}^3$  ve başlangıç değerlerinin en az 2 katının ek tedaviye ihtiyaç duyulmadan korunması olarak tanımlandı. Yirmi beş hasta değerlendirildi. Başlangıç median trombosit değerinin  $15,700/\text{mm}^3$  den 2. haftada  $30,000/\text{mm}^3$  e, 6. haftada  $51,000/\text{mm}^3$  e yükseldiği ve 85 haftalık gözlem periyodu süresince bu düzeyin korunduğu görüldü. Hastaların 13'ünün (%52) devam eden yanıtı sahip olduğu; median izlem sürelerinin 18 ay, eltrombopag kullanım sürelerinin ise 11 ay olduğu görüldü. Çalışmada katılımcılar splenektomi durumuna, trombosit  $<15,000/\text{mm}^3$  olup olmamasına ve cinsiyete göre gruplandırıldı ve yanıt açısından istatistiksel olarak anlamlı fark olmadığı görüldü. İlaç genel olarak iyi tolere edildi. Eltrombopag, diğer tedavilere dirençli ve artmış kanama riski olan kronik ITP hastalarında etkili ve iyi tolere edilen bir terapötik ajandır. Çalışmamızın sonuçları, yanıt durumunun splenektomi, eşzamanlı tedavi alma veya başlangıç trombosit değerlerinden bağımsız olduğunu ortaya koymuştur.

**Anahtar Kelimeler:** Kronik immün trombositopeni. Refrakter hastalık. Tedavi. Eltrombopag.

**Date Received:** February 8, 2025  
**Date Accepted:** March 21, 2025

Dr. Emre HAFIZOĞLU  
Afyonkarahisar State Hospital, Department of  
Medical Oncology, Afyonkarahisar, Türkiye  
Phone: 0532 525 21 45  
E-mail: [emrehafizoglu@gmail.com](mailto:emrehafizoglu@gmail.com)

\* Presented as an oral presentation at the 5. Uluslararası & 9. İlaç ve Tedavi Kongresi (December 2024, Bafra, KKTC)

**Authors' ORCID Information:**  
Emre HAFIZOĞLU: 0000-0001-6291-851X  
Vildan ÖZKOCAMAN: 0000-0003-0014-7398  
Fahir ÖZKALEMKAŞ: 0000-0001-9710-134X

Chronic immune thrombocytopenia (ITP) is characterized by increased platelet destruction and impaired production caused by autoantibodies directed against platelets and megakaryocytes.<sup>1</sup> The objective of treatment in chronic ITP is to elevate platelet levels and sustain them within a safe range to prevent bleeding; Moreover, improving health-related quality of life (HRQOL) is an important goal for most patients. The American Society of Hematology (ASH) recommends a platelet count of 30,000 to 50,000/mm<sup>3</sup> for people who don't have any other risk factors so that they don't have intracerebral hemorrhage or major gastrointestinal bleeding, which are the worst complications associated with ITP.<sup>2,3</sup>

In adults with newly diagnosed ITP, platelet counts <30,000, and asymptomatic or minor mucocutaneous hemorrhage, the ASH guideline panel recommends corticosteroid therapy over follow-up. Additionally, it strongly advocates for follow-up in adults with newly diagnosed ITP, platelet counts of 30,000 or higher, and asymptomatic or minor mucocutaneous hemorrhage. The panel suggests that prednisone (0.5-2.0 mg/kg per day) or dexamethasone (40 mg per day for four days) be used as the initial corticosteroid.<sup>2,3</sup>

The ASH guideline panel recommends splenectomy, rituximab, or a thrombopoietin (TPO) receptor agonist (RA) for adults with ITP lasting  $\geq 3$  months who are corticosteroid-dependent or unresponsive to corticosteroids. Nevertheless, it suggests a TPO-RA (eltrombopag or romiplostim) rather than rituximab. The panel observed that there was no singular optimal second-line treatment for adult patients with ITP. The selection of treatment should be individualized based on the following factors: the duration of ITP, the frequency of bleeding episodes necessitating hospitalization, comorbidities, patient age, drug compliance, patient values and preferences, cost, and availability. Each of these therapies is an effective option. The choice of treatment should be decided together with the patient in line with patient expectations. Splenectomy should be postponed for a minimum of one year following diagnosis to account for the possibility of spontaneous remission within the first year, if feasible. Nevertheless, in cases where patients are not amenable to long-term drug therapy, splenectomy or rituximab may be considered a preferable alternative.<sup>2,3</sup>

Although the ASH clinical guidelines are the most prominent, other guidelines also inform the treatment of ITP. The following treatment options are included in these guidelines and are used in subsequent steps: fostamatinib, avatrombopag, intravenous immunoglobulin (IVIg), azathioprine, cyclophosphamide, cyclosporine, danazol, dapsone, mycophenolate mofetil, and vinca alkaloids.<sup>4-7</sup>

Eltrombopag is a non-peptide TPO-R agonist that has been approved for use in patients who have demonstrated an inadequate response to at least one

other form of treatment. The substance is ingested orally, and it has been demonstrated to increase platelet production by binding to the transmembrane domain of TPO-R. In vitro studies have shown that it does not compete with endogenous TPO, and it has been observed to induce proliferation and differentiation of progenitor cells in the megakaryocytic series. Following a period of up to six months of eltrombopag treatment, eighty percent of patients have shown that their platelet levels are  $\geq 50,000/\text{mm}^3$ . Still, more research on the safety and effectiveness of ITP treatments is required since patients could be subjected to these treatments for years or perhaps longer.<sup>8-11</sup>

This study aimed to contribute to existing literature by evaluating the demographic features, therapeutic response, and side effect profile in refractory cases of chronic immune thrombocytopenia undergoing eltrombopag treatment.

## Material and Method

A total of 25 patients diagnosed with chronic immune thrombocytopenia and using eltrombopag at the outpatient clinic of the Division of Hematology, Department of Internal Medicine, Bursa Uludağ University Faculty of Medicine from January 2011 to March 2014 were included in the study.

The inclusion criteria were being older than 18 years, a diagnosis of ITP, previous use of methylprednisolone, a history of splenectomy, or the absence of splenectomy due to contraindications, and refractory disease. We excluded pregnant and breastfeeding patients, and patients with severe liver disease.

Treatment responses and demographic characteristics (e.g., age, gender, date of diagnosis, other treatments received, baseline platelet value, eltrombopag initiation date, treatment response status) were analyzed.

Both platelet  $\geq 30,000/\text{mm}^3$  and platelet  $\geq 50,000/\text{mm}^3$  values were considered as responses. Statistical analyses were performed according to both values. A prolonged response was defined as the continuous maintenance of platelet levels of 50,000/mm<sup>3</sup> or more for 12 weeks or more without the need for additional treatment. A continuous (sustained) response was defined as the maintenance of platelet levels of 50,000/mm<sup>3</sup> or more and at least twice the baseline levels without the need for further treatment.

The therapeutic objective of eltrombopag treatment was not to raise platelet levels to normal levels, but rather to maintain them at a level that prevents bleeding. The treatment was initiated with a daily dose of 50 mg of eltrombopag. Concomitant use of methylprednisolone with eltrombopag was not

## Use of Eltrombopag in ITP

permitted. Patients were evaluated at two-week intervals for the purpose of dose adjustment. After four weeks of treatment with 75 mg of eltrombopag, the drug was stopped if platelet levels did not rise to a level high enough to stop bleeding, to a clinically significant level.

The statistical analysis of the data was conducted utilizing the SPSS 22.0 statistical package program. The data from our study was subjected to descriptive statistics (mean, median, standard deviation) and frequency distributions. We examined the normality assumptions of continuous variables by implementing the Shapiro-Wilk test. For the purpose of comparison between two groups with non-normal data, the Mann-Whitney U test was employed. We analyzed categorical data using both Pearson's chi-square test and Fisher's exact chi-square test.

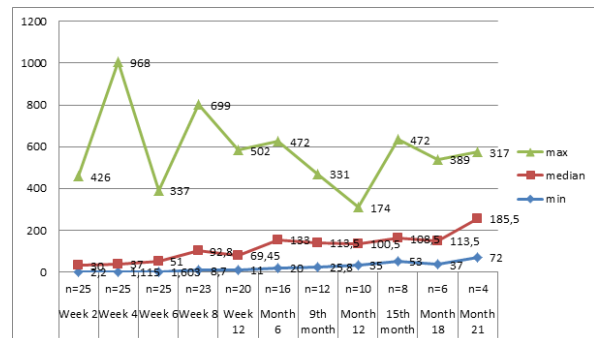
## Results

The study included nine male patients (36%) and 16 female patients (64%), with a mean age of 41 years (range 20-61). The baseline platelet value was found to be less than 15,000/mm<sup>3</sup> in 10 patients (40%) and greater than or equal to 15,000/mm<sup>3</sup> in 15 patients (60%). The median platelet value at the time of diagnosis was calculated to be 15,700/mm<sup>3</sup>. The demographic characteristics of the patients are shown in Table I.

**Table I.** Demographic characteristics of the 25 patients

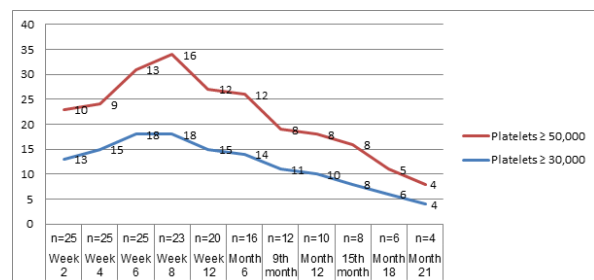
	Median (range) or n (%)
<b>Median Age</b>	41 (20-61)
<b>Gender</b>	
Female	16 (64)
Male	9 (36)
<b>Initial Platelet Value</b>	
< 15,000/mm <sup>3</sup>	10 (40)
≥ 15,000/mm <sup>3</sup>	15 (60)
<b>Previous Treatments</b>	
1	1 (4)
2	7 (28)
3	8 (32)
≥4	9 (36)
<b>Splenectomy</b>	
Yes	19 (76)
<b>Previous Treatments</b>	
Steroid	25 (100)
IVIg	21 (84)
Colchicine	15 (60)
Danazol	8 (32)
Azathioprine	3 (12)
Vincristine	1 (4)
Rituximab	5 (20)

The median duration of eltrombopag treatment was six months (min: 1.5, max: 22), and the median follow-up period was nine months (min: 1.5, max: 22). The baseline median platelet value increased from 15,700/mm<sup>3</sup> to 30,000/mm<sup>3</sup> at week 2 and to 51,000/mm<sup>3</sup> at week 6, and this level was maintained during the 85-week observation period (see Figure I).



**Figure 1.**  
Median, minimum, and maximum platelet values during eltrombopag treatment

In the course of the study, 21 (84%) patients with platelets ≥30,000/mm<sup>3</sup> and 18 (72%) patients with platelets ≥50,000/mm<sup>3</sup> were identified. The response status according to platelet ≥30,000/mm<sup>3</sup> and ≥50,000/mm<sup>3</sup> values during follow-up is shown in Figure II. The investigation revealed no statistically significant differences in response rates between the male and female demographics (see Table II).



**Figure 2.**  
Response status according to platelet ≥30,000/mm<sup>3</sup> and ≥50,000/mm<sup>3</sup> values

**Table II.** Response status by gender

	Woman	Male	P
Ongoing response	10	3	0.226
Platelets ≥ 30,000/mm <sup>3</sup>	13	8	1.0
Platelets ≥ 50,000/mm <sup>3</sup>	12	6	0.673

Thirteen patients (52%) demonstrated a continuous (sustained) response, with a median follow-up period of 18 months and a duration of eltrombopag use of 11 months. The number of patients exhibiting a prolonged response was five, who maintained reliable platelet values without the need for treatment during a median follow-up period of 10 months (3-19). This constituted 20% of all patients and 38.5% of those demonstrating prolonged responsiveness.

Tables III and IV show that platelet counts of 30,000/mm<sup>3</sup> and above are thought to mean that there has been a response. The response status is shown based on the splenectomy status.

**Table III.** Response according to splenectomy status-1

		Platelets $\geq 30,000/\text{mm}^3$ at any time		
		No	There is	P
Splenectomy	No	2	4	0.234
	Yes	2	17	

**Table IV.** Response according to splenectomy status-2

		Platelets $\geq 50,000/\text{mm}^3$ at any time		
		No	There is	P
Splenectomy	No	2	4	1.0
	Yes	5	14	

If platelet counts of 30,000/mm<sup>3</sup> and 50,000/mm<sup>3</sup> are taken as signs of a response, Tables V and VI show the response status based on the initial platelet level.

**Table V.** Response according to baseline platelet value-1

		Platelets $\geq 30,000/\text{mm}^3$ at any time		
		No	There is	P
Baseline platelets $\geq 15,000/\text{mm}^3$		1	14	0.267
$<15,000/\text{mm}^3$		3	7	

**Table VI.** Response according to baseline platelet value-2

		Platelets $\geq 50,000/\text{mm}^3$ at any time		
		No	There is	P
Baseline platelets $\geq 15,000/\text{mm}^3$		2	13	0.075
$<15,000/\text{mm}^3$		5	5	

In the present study, subjects were categorized according to splenectomy status, platelet count (platelet count of  $<15,000/\text{mm}^3$ ), and gender. The study revealed no statistically significant differences in response.

The prevalence of adverse effects was as follows: headaches (60%), weakness (40%), diarrhea and nausea (16%), upper respiratory tract infection (12%), motor weakness (4%), pain (8%), epistaxis (8%), and dizziness (4%). Bleeding of grade 1 occurred in 28% of patients during treatment. The bleeding manifested as gingival bleeding. Thromboembolic events occurred in two patients (8%), presenting as deep vein thrombosis. In one of these patients, the platelet count exceeded 1,000,000 and was successfully managed with Hydrea. Severe bleeding (grade 3-4) during treatment and post-treatment bleeding were not observed. The occurrence of hepatobiliary events, cataracts, and malignancies was not observed in the study. The study found no significant differences in the side effect profile between the sexes.

## Discussion and Conclusion

Eltrombopag is an oral, non-peptide, thrombopoietin receptor agonist that increases platelet production by stimulating megakaryocyte differentiation and proliferation. It was approved by the United States Food and Drug Administration (FDA) in 2008 for the treatment of chronic ITP and by the European Medicines Agency (EMA) in 2010 for the treatment of chronic ITP after splenectomy. In view of the fact that it does not have as long a history as other drugs used in the treatment of ITP, a significant number of international studies are currently underway to elucidate the clinical implications of the drug.

In our study conducted with twenty-five patients, the mean age of the patients was 41 years, and the mean age of starting eltrombopag was  $39 \pm 15.4$  years. The mean age of the patients was 47 years in the RAISE study<sup>10</sup>, 50 years in the EXTEND study<sup>11</sup>, and 50.5 years in the REPEAT study<sup>12</sup>. The female sex ratio was found to be 57%, 66%, and 69% in the same studies, respectively.<sup>10-12</sup> In our study, this rate was found to be 64%, compatible with the literature.

The 'Delphi-based Consensus Recommendations' panel reported from Turkey recommends the use of thrombopoietin receptor antagonists (TPO-RAs) or rituximab in patients with persistent or chronic pITP who are refractory or dependent on corticosteroids or who relapse after corticosteroids. There is a recommendation that TPO-RAs (eltrombopag or romiplostim) should be used as second-line treatment before splenectomy in these patients. Splenectomy should be performed for patients with chronic pITP ( $>12$  months) who are refractory to current treatments and who relapse.<sup>4</sup> Nevertheless, the present study revealed that 76% of patients underwent splenectomy prior to TPO-RA. This is attributable to the fact that the guidelines at the time of the study differed from those in effect at present.

## Use of Eltrombopag in ITP

In this study, 40% of patients had a baseline platelet value of  $<15,000/\text{mm}^3$ . We observed an increase in the initial median platelet value from  $15,700/\text{mm}^3$  to  $30,000/\text{mm}^3$  at week 2, to  $51,000/\text{mm}^3$  at week 6. This level was maintained during the 85-week observation period. A review of the literature indicates that platelet levels of  $\geq 50,000/\text{mm}^3$  are typically attained by week 2.<sup>10-12</sup> However, in our study, this level was achieved by week 6. This discrepancy can be attributed to the heterogeneous distribution of patients in the studies and the exclusion of platelet levels  $>30,000/\text{mm}^3$  from our study.

In the present study, it was observed that 21 (84%) patients responded when platelet levels  $\geq 30,000/\text{mm}^3$  were taken at any time, and 18 (72%) patients responded when platelet levels  $\geq 50,000/\text{mm}^3$  were taken. Tomiyama Y et al. conducted a study in Japan with 23 participants, 15 of whom received eltrombopag and eight of whom received a placebo for six weeks. At the end of the sixth week, the response rate in the eltrombopag arm was found to be 60% according to platelet  $\geq 50,000/\text{mm}^3$ .<sup>13</sup> In a subsequent study by Yoshida M et al. in Japan, which involved 22 participants, it was determined that platelet levels should be maintained between 50,000 and  $400,000/\text{mm}^3$  for over 75% of the follow-up period. This study also reported a response rate of 65%.<sup>14</sup> Similar results were obtained in a subsequent study by Katsutani S et al. in Japan.<sup>15</sup> Tripathi AK et al. conducted a study with newly diagnosed ITP and inclusion criteria that had not responded to two-week steroid treatment.<sup>16</sup> The study found a response rate of 80% and a median platelet value of  $150,000/\text{mm}^3$  with a platelet  $\geq 50,000/\text{mm}^3$  value at the first month. 76% and a median platelet value of  $126,000/\text{mm}^3$  at the end of the third month; treatment was not discontinued at the end of the third month against the risk of rebound thrombocytopenia. In a separate study, 94% (17 out of 18) of patients treated with eltrombopag as second-line treatment attained a target platelet count of over 50,000, and their responses remained stable during the entire follow-up period.<sup>17</sup> In another study, a complete response was achieved in 72.1% of patients using eltrombopag as second-line treatment.<sup>18</sup>

If we take a look at the studies done in Turkey, in a study conducted by Eser A. et al., the response rate was found to be 93.5% according to a platelet value  $\geq 50,000/\text{mm}^3$  at any time.<sup>19</sup> Özdemirkıran F. et al. conducted a study with 32 patients in 7 different centers in the Aegean region and found that 75% of the patients had platelet values of  $\geq 100,000/\text{mm}^3$  and 6.25% had platelet values of  $30,000/\text{mm}^3$  -  $100,000/\text{mm}^3$  at the end of the first month.<sup>20</sup> Another study conducted by Çekdemir D. et al. in 11 different centers with 35 patients found the response rate to be 74% according to the platelet  $\geq 30,000/\text{mm}^3$  value at week 2.<sup>21</sup> In another study, a response rate of 87%

was reported.<sup>22</sup> In a study reported from Muğla province with the same response definition, the response rate was found to be above 65%.<sup>23</sup> As a result, the response rate in our study was found to be consistent with the literature.

In the present study, 13 (52%) of the patients demonstrated a sustained response with a median follow-up period of 18 months. The number of patients with a prolonged response was 5 (20%). Gonzalez Lopez TJ. et al. shared the sustained, prolonged response status they obtained in patients using eltrombopag in their follow-up in a report.<sup>24</sup> During a median follow-up period of 7 months (range 6-20 months), 12 patients maintained reliable platelet levels without the need for additional treatment. The median age of the patients was 24 years, the median number of prior treatments was 5, and the median duration of eltrombopag use was 5 months (range 1-13).<sup>24</sup> Mahevas M. et al. similarly reported a sustained, prolonged response. During a median follow-up of 13.5 months (range, 5-27 months), eight patients maintained reliable platelet values without the need for any treatment.<sup>25</sup> It was determined that predicting prolonged response was not feasible. However, it was hypothesized that a significant proportion of patients could maintain response status after discontinuing TPO-R agonists.<sup>25</sup> In a study, the incidence of prolonged response was documented to be 11.3%.<sup>26</sup> In the Phase II TAPER study, the proportion of patients achieving sustained response off-treatment (SRoT) by 12 months following treatment discontinuation was 30.5%, and the median SRoT duration was 8 months.<sup>27</sup> In a further study, 16 patients (17%) were reported to have prolonged response when response was considered as platelet value  $\geq 30,000/\text{mm}^3$  and 8 patients (8%) were reported to have prolonged response when response was considered as platelet value  $\geq 100,000/\text{mm}^3$ .<sup>28</sup> The higher rates of patients with prolonged response and ongoing responsiveness in our study compared to the literature may be due to individual differences in ITP, the pathogenesis of which has not been clearly elucidated, or to other reasons such as genetic structure, ethnicity, environmental effects, or the relatively small number of patients we evaluated. In our study, participants were divided into groups based on splenectomy status, platelet count ( $<15,000/\text{mm}^3$ ) and gender. Our analysis revealed no statistically significant differences in response compared to the findings reported in the literature.

When the side effect profile was analyzed, generally compatible results with the literature were observed, and it was thought that the existing percentage differences may be due to the retrospective nature of the study, inadequacies in our data records, and the lack of an objective method in the evaluation of some side effects.

In conclusion, the objective of treatment with eltrombopag should not be to normalize platelet counts but rather to maintain them above the threshold for hemorrhagic risk. Eltrombopag is an effective treatment for patients with chronic ITP who are refractory to other therapies and have an increased risk of bleeding. It is generally well tolerated in the short and long term, and responsiveness is independent of splenectomy, concomitant treatment, or baseline platelet values. Long-term efficacy and safety studies on eltrombopag with five years of experience and data are needed.

#### Ethics Committee Approval Information:

Ethical Board: Bursa Uludağ University Faculty of Medicine Clinical Research Ethics

Date: 18.03.2014

Degree No: 2014-6/13

#### Researcher Contribution Statement:

Idea and design: E.H., V.Ö., F.Ö.; Data collection and processing: E.H.; Analysis and interpretation of data: E.H., V.Ö., F.Ö.; Writing of significant parts of the article: E.H.

#### Support and Acknowledgement Statement:

This study received no financial support.

#### Conflict of Interest Statement:

The authors of the article have no conflict of interest declarations.

## References

1. Pruemer J. Epidemiology, pathophysiology, and initial management of chronic immune thrombocytopenic purpura. *Am J Health Syst Pharm.* 2009;66(2 Suppl 2):S4-S10.
2. Provan D, Stasi R, Newland AC, et al. International consensus report on the investigation and management of primary immune thrombocytopenia. *Blood.* 2010;115(2):168-186.
3. Neunert C, Terrell DR, Arnold DM, et al. American Society of Hematology 2019 guidelines for immune thrombocytopenia. *Blood Adv.* 2019 Dec 10;3(23):3829-3866.
4. Demir AM, Ümit EG, Ar MC, et al. Management of Adult Primary Immune Thrombocytopenia: Delphi-Based Consensus Recommendations. *Turk J Haematol.* 2024 May 30;41(2):97-104.
5. Neunert CE, Arnold DM, Grace RF, Kuhne T, McCrae KR, Terrell DR. The 2022 review of the 2019 American Society of Hematology guidelines on immune thrombocytopenia. *Blood Adv.* 2024 Jul 9;8(13):3578-3582.
6. Provan D, Arnold DM, Bussell JB, et al. Updated international consensus report on the investigation and management of primary immune thrombocytopenia. *Blood Adv.* 2019 Nov 26;3(22):3780-3817.
7. González-López TJ, Provan D. Proposal for a New Protocol for the Management of Immune Thrombocytopenia (ITP). *Adv Ther.* 2022 Jun;39(6):2287-2291.
8. Garnock-Jones KP, Keam SJ. Eltrombopag. *Drugs.* 2009;69(5):567-576.
9. Bussell JB, Provan D, Shamsi T, et al. Effect of eltrombopag on platelet counts and bleeding during treatment of chronic idiopathic thrombocytopenic purpura: a randomized, double-blind, placebo-controlled trial. *Lancet.* 2009;373(9664): 641-648.
10. Cheng G, Saleh MN, Marcher C, et al. Eltrombopag for management of chronic immune thrombocytopenia (RAISE): a 6-month, randomized, phase 3 study. *Lancet.* 2011;377(9763):393-402.
11. Wong RSM, Saleh MN, Khelif A, et al. Safety and efficacy of long-term treatment of chronic/persistent ITP with eltrombopag: final results of the EXTEND study. *Blood.* 2017 Dec 7;130(23):2527-2536.
12. Bussell JB, Saleh MN, Vasey SY, et al. Repeated short-term use of eltrombopag in patients with chronic immune thrombocytopenia (ITP). *Br J Haematol.* 2013 Feb;160(4):538-546.
13. Tomiyama Y, Miyakawa Y, Okamoto S, et al. A lower starting dose of eltrombopag is efficacious in Japanese patients with previously treated chronic immune thrombocytopenia. *J Thromb Haemost.* 2012 May;10(5):799-806.
14. Yoshida M, Kanashima H, Nakao T, et al. Retrospective analysis of eltrombopag for the treatment of refractory primary immune thrombocytopenia in Japan. *Rinsho Ketsueki.* 2013 May;54(5):444-450.
15. Katsutani S, Tomiyama Y, Kimura A, et al. Oral eltrombopag for up to three years is safe and well-tolerated in Japanese patients with previously treated chronic immune thrombocytopenia: an open-label, extension study. *Int J Hematol.* 2013 Sep;98(3):323-330.
16. Tripathi AK, Shukla A, Mishra S, et al. Eltrombopag therapy in newly diagnosed steroid non-responsive ITP patients. *Int J Hematol.* 2014 Apr;99(4):413-417.
17. Gardellini A, Guidotti F, Feltri M, et al. Eltrombopag as second line treatment in patients with primary immune thrombocytopenia: A single center real life experience. *Blood Cells Mol Dis.* 2021 Dec;92:102620.
18. Rosenberg A, Cashion C, Ali F, et al. Treatment of immune thrombocytopenia in Australian adults: A multicenter retrospective observational study. *Res Pract Thromb Haemost.* 2022 Sep 18;6(6):e12792.
19. Eser A, Toptaş T, Tanrikulu F, et al. Marmara University eltrombopag experience in chronic ITP patients. XXXIXth National Hematology Congress Book of Abstracts 2013;271-272.
20. Özdemirkıran F, Payzın B, Kiper H, et al. Eltrombopag in the treatment of refractory chronic immune thrombocytopenic purpura: Aegean region experience. XXXIXth National Hematology Congress Book of Abstracts 2013;67-68.
21. Çekdemir D, Hindilerden F, Güvenç S, et al. Use of eltrombopag in immune thrombocytopenia in Turkey: Experience of eleven centers. XXXIXth National Hematology Congress Book of Abstracts 2013;9.
22. Dogan EE, Turan Erkek E, Elverdi T, et al. Eltrombopag in the Treatment of Immune Thrombocytopenia: Two-Center Experience from Istanbul. *Indian J Hematol Blood Transfus.* 2022 Apr;38(2):327-332.
23. Pektaş G, Uncu İA, Dere Y, et al. Retrospective Evaluation of Survival and Prognostic Factors in Immune Thrombocytopenia: A Single-Center and Cross-Sectional Study. *Medicina (Kaunas).* 2024 Jul 17;60(7):1153.
24. González-López TJ, Sánchez-González B, Pascual C, et al. Sustained response after discontinuation of short-and medium-term treatment with eltrombopag in patients with immune thrombocytopenia. *Platelets.* 2014 Feb 5. [Epub ahead of print]
25. Mahévas M, Fain O, Ebbo M, et al. The temporary use of thrombopoietin-receptor agonists may induce a prolonged remission in adult chronic immune thrombocytopenia. Results of a French observational study. *Br J Haematol.* 2014 Jun;165(6):865-869.
26. Mishra K, Pramanik S, Jandial A, et al. Real-world experience of eltrombopag in immune thrombocytopenia. *Am J Blood Res.* 2020 Oct 15;10(5):240-251.
27. Cooper N, Ghanima W, Vianelli N, et al. Sustained response off-treatment in eltrombopag-treated adult patients with ITP who are refractory or relapsed after first-line steroids: Primary,

## Use of Eltrombopag in ITP

- final, and ad-hoc analyses of the Phase II TAPER trial. *Am J Hematol.* 2024 Jan;99(1):57-67.
28. Cooper N, Scully M, Percy C, et al. Real-world use of thrombopoietin receptor agonists for the management of immune thrombocytopenia in adult patients in the United Kingdom: Results from the TRAIT study. *Br J Haematol.* 2024 Jun;204(6):2442-2452.

