MADRID
22ND CONGRESS
JUNE 22 - 25 | 2017
European Hematology Association

PRIMARY ITP IN ADULTS TREATED WITH ELTROMBOPAG: A RETROSPECTIVE STUDY USING DATA FROM THE UNITED KINGDOM ADULT IMMUNE THROMBOCYTOPENIA REGISTRY

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BACKGROUND

Primary ITP is an autoimmune disorder associated with a reduced peripheral blood platelet count. Although many patients are relatively asymptomatic, many suffer with bruising, mucosal bleeding and quality of life issues. The first line treatment has remained unchanged for decades and until recently, second-line therapy has been unsatisfactory, using empirical treatments. The recently approved thrombopoietin receptor agonists eltrombopag and romiplostim have transformed patient care and these agents are licensed second-line therapies in adults.

OBJECTIVES

To describe the adult patients receiving eltrombopag using data from the UK Adult ITP Registry. In particular we were interested in understanding the mean dose used, number of prior therapies, median length of treatment with eltrombopag, median counts at baseline before treatment and at six months following treatment, and sustained response in patients who have received eltrombopag.

METHODS

The UK Adult ITP Registry involved more than 70 UK collaborating centres, coordinated by The Royal London Hospital. In this study we analysed data from all patients receiving eltrombopag and analysed these using various statistical techniques. Given that an individual had several platelet count measurements done during a time period, the mean platelet count was obtained for this particular individual and the pooled mean platelet using random effects model [with 95% confidence interval (CI)] were obtained for the cohort by the relevant time period. Pooled mean platelet counts were used to show changes over time after initiation of eltrombopag.

RESULTS

The total number of patients evaluable was 129. The median age at diagnosis was 49.4 years (26.9-66.4) [Table 1]. There were 74 males (57.4%) and 55 females (42.6%). 29 patients (22.4%) had undergone prior splenectomy. The median age at eltrombopag initiation was 59.5 years (37.0-70.7 years). The median time from ITP diagnosis to eltrombopag initiation was 1.6 years (0.7-2.3 years). The majority of patients started eltrombopag between 2013 and 2016 (93%). 10 (8%) and 8 (6%) started eltrombopag within the first 6 months and between 6 to 12 months of ITP diagnosis, respectively.

RESULTS (cont.)

Table 1. Demographic and clinical characteristics of participants at baseline (n=129)

Median (IQR) age at ITP diagnosis	49.4 (26.9, 66.4) years
Female , n(%)	55 (42.6)
Splenectomised before eltrombopag , n(%)	29 (22.4)
Age group at diagnosis , n(%)	
<18	15 (11.6)
18 to < 30	24 (18.6)
30 to < 45	18 (14.0)
45 to < 65	37 (28.7)
≥ 65	35 (27.1)
Median (IQR) age enrolled on the Registry	59.2 (38.2, 71.0) years
Median (IQR) age at eltrombopag initiation	59.5 (37.0, 70.7) years
Since 2010, median (IQR) time from ITP diagnosis to Eltrombopag initiation (years)	1.6 (0.7, 2.3)
Whole cohort median (IQR) time from ITP diagnosis to Eltrombopag initiation (years)	3.6 (1.5, 10.3)
Period of Eltrombopag initiation , n(%)	
2010-12	9 (6.9)
2013-15	113 (87.5)
2016	8 (6.2)

Most patients had received prior ITP therapies. Some 10 patients (7.8%) had received one prior ITP therapy and 99 patients (77%) had received three or more prior therapies before starting eltrombopag. The commonest prior therapies were corticosteroids in 110 patients (87%); IVIg 91 patients (72%); rituximab 68 patients (54%); romiplostim 47 patients (37%); and immunosuppressants 71 patients (56%). The median dose of eltrombopag used was 50mg/day. The median course length on eltrombopag was 14.7 (IQR: 4, 67) weeks. After initiation, 53 (41%) remained on eltrombopag as a monotherapy whereas 27 (21%) had other ITP treatment concurrently with eltrombopag. Forty nine (38%) changed treatment after eltrombopag, of which prednisolone (47%), IVIg (33%), mycophenolate (18%) and rituximab (14%) were the commonest and 10% underwent a splenectomy). Response to eltrombopag was assessed for 106 patients with adequate follow up time and platelet counts. 81 (76%) had a response, of which 54(51%) were above 100x109/L and 27 (25%) had a partial response (platelet counts between 30 to 100 x 10^9/L). Among those that had a response, 15 (14%) became unresponsive after some time whereas 2 (2%) patients were unresponsive soon after a brief episode of response. In short, 64 (60%) had a sustained response to eltrombopag (among patients who remained or came off eltrombopag).

At baseline, prior to starting eltrombopag, the median platelet count was 21×10^9 /L (10-54) and the majority of patients (64.5%) had platelets less than 30 × 10^9/L. The mean of the maximum platelet count achieved between 6 months to a year was 206.2 × 10^9/L and in the 1st year was 288 × 10^9/L. Pooled mean platelet count after 6 months to a year was 122.0 (95% CI: 71.0, 173.1) × 10^9/L and in the 1st year was 127.8 (95% CI: 91.4, 164.1) × 10^9/L

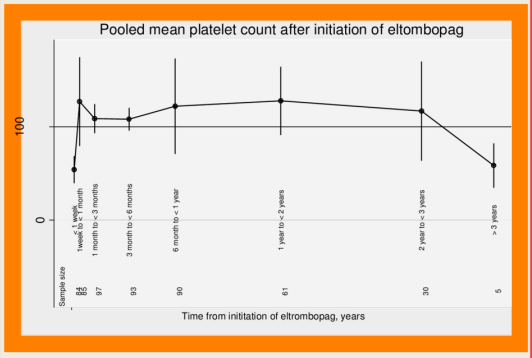

Table 2. Treatment* received before and after initiation of eltrombopag (n=129)

By type of therapeutic agent, n (%)	Before	After
Steroids	110 (87.3)	45 (34..9)
IVIg	91 (72.2)	31 (24.0)
Tranfusion	41 (32.3)	12 (9.3)
Rituximab	68 (53.9)	14 (10.9)
Splenectomy	29 (22.4)	6 (4.7)
Romiplostin	47 (37.3)	26 (20.2)
Anti-D	8 (6.4)	0 (0)
Immunosuppressants	71 (56.4)	22 (17.0)
Danazol/ Dapsone	17 (13.5)	5 (3.9)
Chemotherapy	11 (8.7)	4 (3.1)
Number^ of different types of treatment		
0	3 (2.3)	0 (0)
1	10 (7.8)	82 (63.6)
2	17 (13.2)	24 (18.6)
≥3	99 (76.7)	24 (18.6)

*an individual may have received more than 1 type of treatment

CONCLUSION

The patient characteristics of those receiving eltrombopag appear to be typical of adult ITP. Only 10 patients (7.8%) received eltrombopag as a second line therapy. Three quarters had received 3 or more prior therapies before starting eltrombopag despite its licence as a second line therapy. As clinicians become more familiar with its use, a greater proportion of patients are likely to receive eltrombopag as a second line therapy