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Analysis of Second Line Treatments for Children with Persistent or Chronic Primary Immune Thrombocytopenia (ITP) in the UK.

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Abstract Content: We present the changing patterns of second line treatments for persistent or chronic primary immune thrombocytopenia (ITP) in children from 2006 to 2019 in the UK. The source of information was the UK paediatric ITP registry. To be eligible for second line therapy patients must have had a platelet count < 30 x 10^9/L at 6 months after diagnosis. Second line therapies considered were thrombopoietin agonists (TPO-RA), Rituximab and splenectomy. From 2006, 212 patients out of 1915 children on the registry at this time, would be eligible for second line therapy with 23% of the eligible patients currently receiving a second line therapy with a platelet count <30 x 10^9/L at 6 months.

The use of TPO-RA has risen in proportion to 47.6% of all therapies from January 2015 to January 2019 (Figure 1) compared to 23.8% rituximab and 9.5% splenectomy. This compares to 23.1% receiving TPO-RA, 34.6% receiving Rituximab, and 15.4% splenectomy between 2006 to January 2011. In our population, the patients most likely to receive second line therapy were female, and of non-Caucasian ethnicity. They were also more likely to be older with an average age of 9.2 vs 6.9 years, and a longer duration of disease of 54 vs 36 weeks total follow-up.

The patients receiving second line therapies are the more severe; compared to those who receive either no intervention (watch/monitor) or those receiving just rescue steroids or immunoglobulin. Based on the Bolton-Maggs severity score, patients on second line therapies had a higher bleed severity score of 2.00 vs 1.74 (P value =0.02). A total proportion of bleeds at Grade 2-3 of 71% vs 67%, at Grade 3 of 20% vs 8.6%, finally at Grade 4 of 6% vs 0%. Furthermore, a bleed frequency average 7.7 vs 5.9 (P value =0.017), platelet count average of 13.6 vs 15.7 (P value =0.12), and total follow-up period of 53 vs 36 months.

New international guidelines will recommend an increase in second line therapies for children with persistent or chronic ITP. The current data demonstrates the change in treatment trend since the start of the registry and provides a baseline analysis prior to the impact of these new recommendations.

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