BSH 2020 VIRTUAL
9 -14 NOVEMBER

British Society for Haematology
60 1960–2020
Listening • Learning • Leading

Predicting treatment response in paediatric chronic ITP patients

S. NOVINTAN [1], A. CABRERA [2], S. DEPLANO [2] and N. COOPER[2]
1 Faculty of Medicine, Haematology, Imperial College School of Medicine
2 Haematology, Imperial College Healthcare Trust, London, UK

NHS
Imperial College Healthcare
NHS Trust

INTRODUCTION

Immune thrombocytopenia purpura (ITP) is a heterogeneous disorder characterised by increased destruction and suppressed production of platelets.

There are no biomarkers to help direct treatment in children, who experience unpredictable response rates and a large disease burden.

To help direct treatment pathways, we assessed whether there were any biological features that could predict responses to treatment.

METHOD

This was a retrospective case review of 28 children with chronic ITP (>12 months) who attended St. Mary's Hospital (SMH), Imperial College NHS Trust Paediatric ITP clinic.

This cohort was divided into two groups. The '**non-treatment**' group were managed with observation (*n* = 11). The **'treatment group**' required treatment due to bleeding symptoms (*n* = 17). In the 'treatment group', 11 are responding to a thrombopoietin receptor agonist (TPORA): seven to eltrombopag and four to romiplostim.

Six patients failed to respond to romiplostim and/or eltrombopag and are on a combination treatment including immunosuppression.

CONCLUSIONS

Lower platelet counts are a predictor of needing further treatment. Further analysis of lymphocyte subsets in peripheral blood and bone marrow could define better disease types and help determine response to treatment.

CONTACT INFORMATION

Shonnelly Novintan: 07738362140;
sn2315@ic.ac.uk

RESULTS

Initial platelet count was significantly higher in the non-treatment group *versus* the treatment group (21 × 10^9/l vs. 6 × 10^9/l) (Fig. 1). Patients needing treatment had lower CD56 + numbers (285 vs. 327 cells/μl) and higher IgG (10·65 vs. 7·95 g/l). Bone marrow biopsies of the treatment group had more megakaryocyte clustering (50% vs.14%).

Patients who failed to respond to TPORAs had the lowest CD56 + (Natural Killer, NK), CD4 + and CD8 + counts, although this did not achieve statistical significance in this small cohort. Both responders and non-responders had increased numbers of megakaryocytes (83% and 100%), which were small and hypolobated (83% and 77%) (Fig. 2).

Patients who responded to eltrombopag compared to romiplostim had a trend towards lower CD56 + (344 vs. 369 cells/μl), higher CD8 + (1137 vs. 578 cells/μl) and higher B-cell count (859 vs. 578 cells/μl) (Fig. 3).

	Treatment group (n=17)	Non-treatment group (n=11)	P value
Platelet count (x10^9/l)	21	6	0.01
CD56+ (cells/ul)	285	327	0.36
IgG (g/l)	10.65	7.95	0.06
Megakaryocyte clustering (%)	50	14	0.13

Figure 1. Biomarkers of treatment group compared to the non-treatment group.

	Eltrombopag responders	Romiplostim responders	P value
CD56+ (cells/ul)	344	369	0.07
CD8+ (cells/ul)	1137	578	0.23
B Cell count (cells/ul)	859	578	0.16

Figure 3. White cell subset populations in those who responded to the different TPORSAs

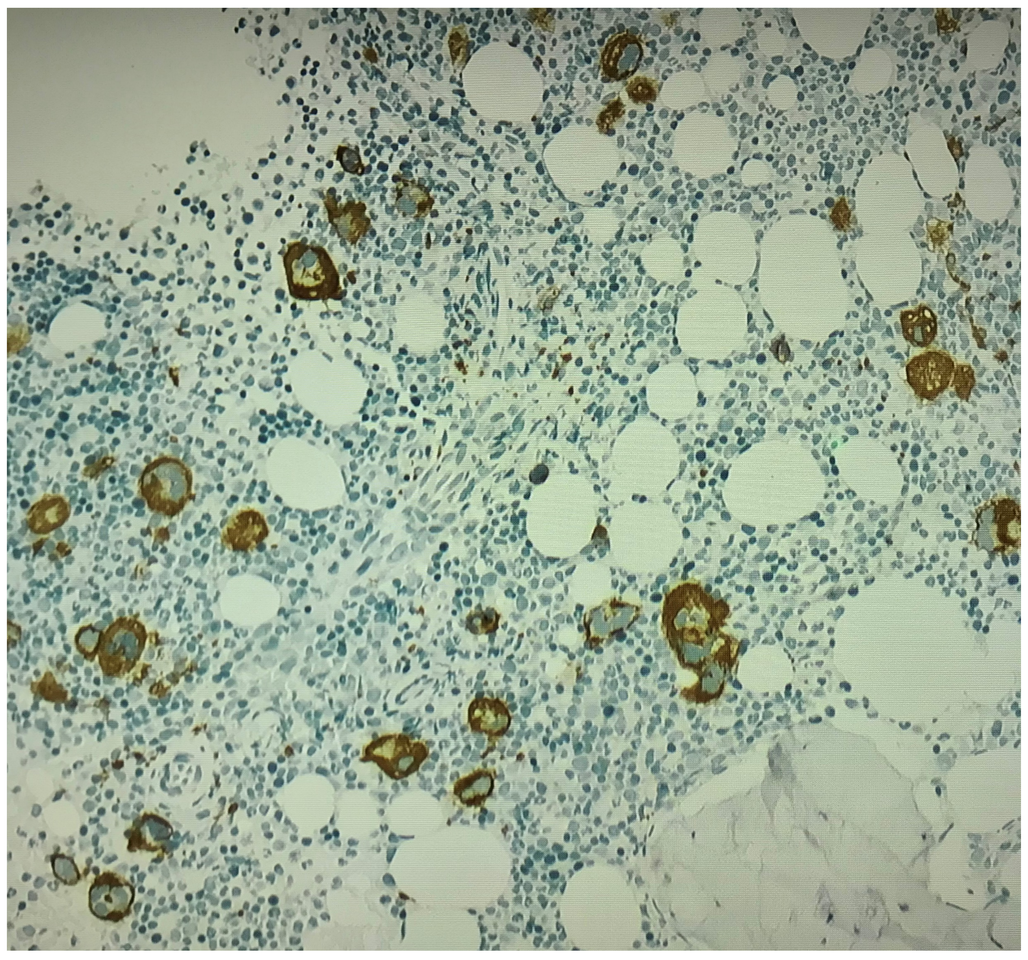

Figure 2. Bone marrow biopsy of a patient from the treatment group.