

Use of second-line treatments in adult patients with primary immune thrombocytopenia in Europe. A parallel study in six prospective multicenter national registries

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INTRODUCTION

In adult patients with primary immune thrombocytopenia (pITP), many options of second-line treatments are available. This study was aimed to describe the SLTs used in Europe in adult patients with pITP, overall and by age, sex, and time periods over the last decade.

METHODS

- This study is part of the European Research Consortium for ITP (ERCI) initiative to harmonize registries in Europe.
- It is a parallel study conducted in 6, multicenter, national, prospective registries of patients with ITP in Europe: the UK-ITP Registry, the CARMEN-France Registry, PARC-ITP registry restricted to patients from Switzerland and Serbia, the Italian, Norwegian, and German registries (Figure 1). Analyses were performed in each registry using a common protocol and statistical analysis plan.
- Adult patients (≥ 18 years of age) with a diagnosis of pITP between 2010 and 2022 were included.
- Second-line treatments were described overall, then, for those used in $>10\%$ of patients with pITP, by subgroups of age (<40 , 40-59, 60-79 and ≥ 80 years), sex and time period of ITP diagnosis (2010-2016 and 2017-2022).



Figure 1. Prospective registries as source of data in the present study.

RESULTS

- 5189 patients with pITP were selected (3020 from the UK, 1263 from France, 604 from Italy, 172 from Norway, 105 from Germany, 25 from Switzerland and Serbia).
- The median age at diagnosis of pITP ranged from 47 to 66 years; the proportion of women ranged from 40.9% to 56.4%.
- The proportion of patients requiring a second-line ranged from 29.5% (Germany) to 53.4% (Norway).
- The most commonly prescribed second-line treatment was rituximab in Norway (54.3% of second-line treatments) and the UK (24.6%), and eltrombopag in Germany (64.5%), Italy (61.2%) and France (55.4%) (Figure 2).
- No major difference was observed by age group except for rituximab in France (9.3% of SLTs in patients aged ≥ 80 years vs 14.0-17.0% in other age groups) and in Norway (no patient aged ≥ 80 years exposed to the drug) and the use of thrombopoietin receptor agonists (TPO-RAs) in Italy (45.1% in patients aged ≥ 80 years vs $>75\%$ in other age groups). In UK, azathioprine was used more frequently in women (14.8%) than in men (9.5%), like hydroxychloroquine in France (17.9% vs 8.0%). Over time, the use of TPO-RAs increased in all countries except in Norway; rituximab decreased in all countries but in Italy and Norway, and the use of dapsone decreased in France.

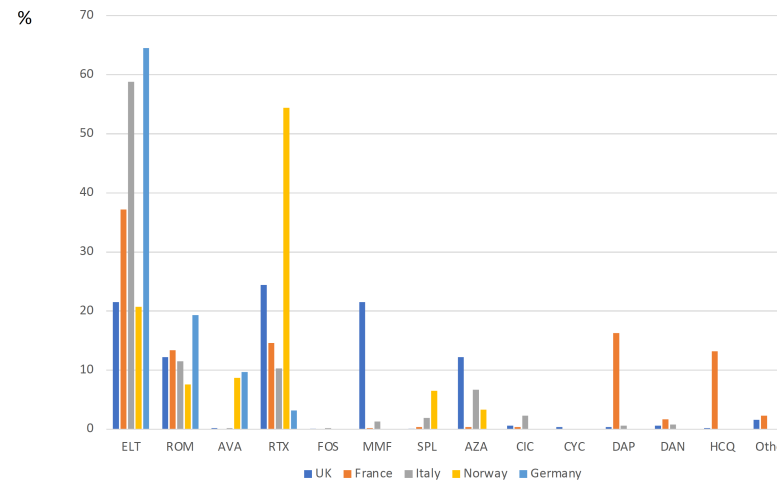


Figure 2. Proportion of second-line treatments in patients with pITP compared to the total number of patients treated with second-line therapy in each registry. The ITP-PARC registry (Switzerland-Serbia) was excluded due to insufficient number of selected patients (n=9). Abbreviations: AVA, avatrombopag; AZA, azathioprine; CIC, cyclosporine; CYC, cyclophosphamide; DAN, danazol; DAP, dapsone; ELT, eltrombopag; FOS, fostamatinib; HCC, hydroxychloroquine; MMF, mycophenolate mofetil; ROM, romiplostim; RTX, rituximab; SPL, splenectomy.

CONCLUSIONS

- This study shows similar populations treated for pITP across European countries, with small differences in the use of second-line treatments, explained by national regulations and guidelines. TPO-RAs were the preferred second-line therapy, with increased use over the last decade, while the use of rituximab, immunosuppressants and splenectomy decreased.
- With >5000 patients included, the combination of European registries is a powerful tool for conducting epidemiologic and pharmacoepidemiologic studies in a rare disease such as ITP.

DISCLOSURES (first author)

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