



1ST
European Research
Consortium on ITP Meeting

INNOVATIONS IN IMMUNE THROMBOCYTOPENIA

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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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Disclosures of Guillaume Moulis

Company name	Research support	Employee	Consultant	Stockholder	Speakers bureau	Advisory board	Other
Alpine						X	
Amgen	X					X	X
Argenx	X					X	
Grifols	X					X	X
Novartis	X					X	X
Sanofi	X					X	
UCB						X	



ERCI initiative for the coordination of European ITP registries



1. description of existing registries

Registries in immune thrombocytopenia (ITP) in Europe: the European Research Consortium on ITP (ERCI) network

Br J Haematol 2022



**2. feasibility study
common extraction model**



**3. linkage
technical & regulatory aspects**



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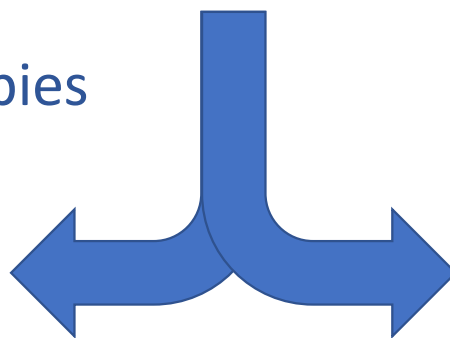


Research question

initial/emergency therapies

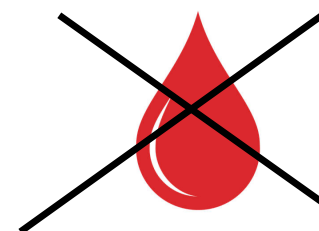


- ✓ steroids
- ✓ IVIg
- ✓ anti-D



maintenance therapies

- ✓ prevent bleeding
- ✓ avoid toxicity
- ✓ improve QoL
- ✓ ± « cure » ITP



- ✓ TPO-RAs
- ✓ B-cell targeted therapies
- ✓ anti-SYK
- ✓ immunosuppressants
- ✓ splenectomy
- ✓ dapsone
- ✓ ...



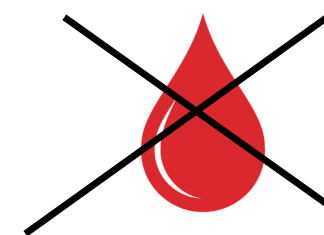
Research question

- ✓ discrepancies across countries
 - ✓ national regulations
 - ✓ national guidelines
 - ✓ reimbursement rules



maintenance therapies

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Aims

In adult patients with primary ITP

- ✓ to assess the proportion of patients requiring maintenance treatment
- ✓ to assess the use of the different maintenance therapies across countries in Europe
- ✓ to assess the variation of use of maintenance therapies by age, sex and time period



Methods

- ✓ Parallel study conducted in 6, multicenter, national, prospective registries
- ✓ Common protocol and statistical analysis plan
- ✓ Population: adult patients (≥ 18 years of age) with a diagnosis of pITP between 2010-2022
- ✓ Subgroup analyses: age (<40, 40-59, 60-79 and ≥ 80 years), sex and time period of ITP diagnosis (2010-2016 and 2017-2022)



ERCITP group – abstract session tomorrow



Results: patients (n=5189)



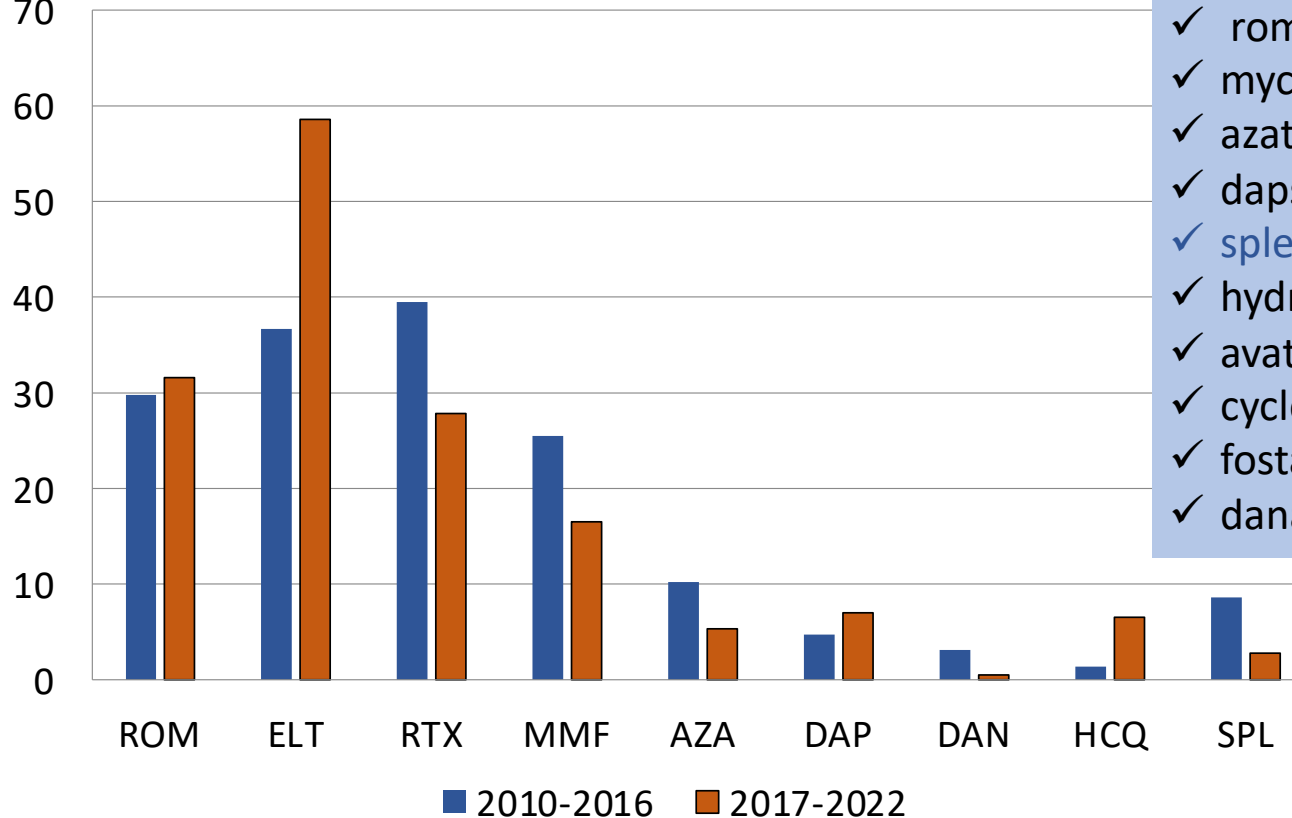
Results: patients (n=5189)

Characteristics	UK	France	Italy	Norway	Germany	Switzerland-Serbia
Number of patients with pITP	3020	1263	n/a	172	105	25
Median age (Q1-Q3) at ITP diagnosis, years	57 (36-70)	62 (39-75)	59 (39-72)	50 (33-65)	66 (46-75)	47 (30-60)
Women, n (%)	1613 (53.4%)	668 (52.9%)	336 (55.6%)	97 (56.4%)	43 (40.9%)	13 (52.0%)
Bleeding at ITP diagnosis, n (%)	1328 (44.0%)	763 (60.4%)	n/a	88 (51.2%)	37 (48.7%)	18 (72.0%)
Median platelet count at ITP diagnosis (Q1-Q3) x 10 ⁹ /L	19 (6-49)	18 (6-49)	19 (7-39)	22 (5-37)	27 (6-69)	13 (7-60)
Number of patients with pITP who needed treatment, n (%)	2467 (81.7%)	1072 (84.9%)	604 (n/a)	145 (84.3%)	65 (61.9%)	18 (72.0%)
Number of patients with pITP who required maintenance therapy (second-line), n (%)	1233 (40.8%)	576 (45.6%)	428 (n/a)	92 (53.4%)	31 (29.5%)	9 (36.0%)
Number of patients with pITP who required a second maintenance therapy (third line), n (%)	607 (20.1%)	272 (21.5%)	134 (n/a)	40 (23.3%)	7 (6.7%)	2 (8.0%)



Results: overall maintenance therapies by time periods of ITP diagnosis

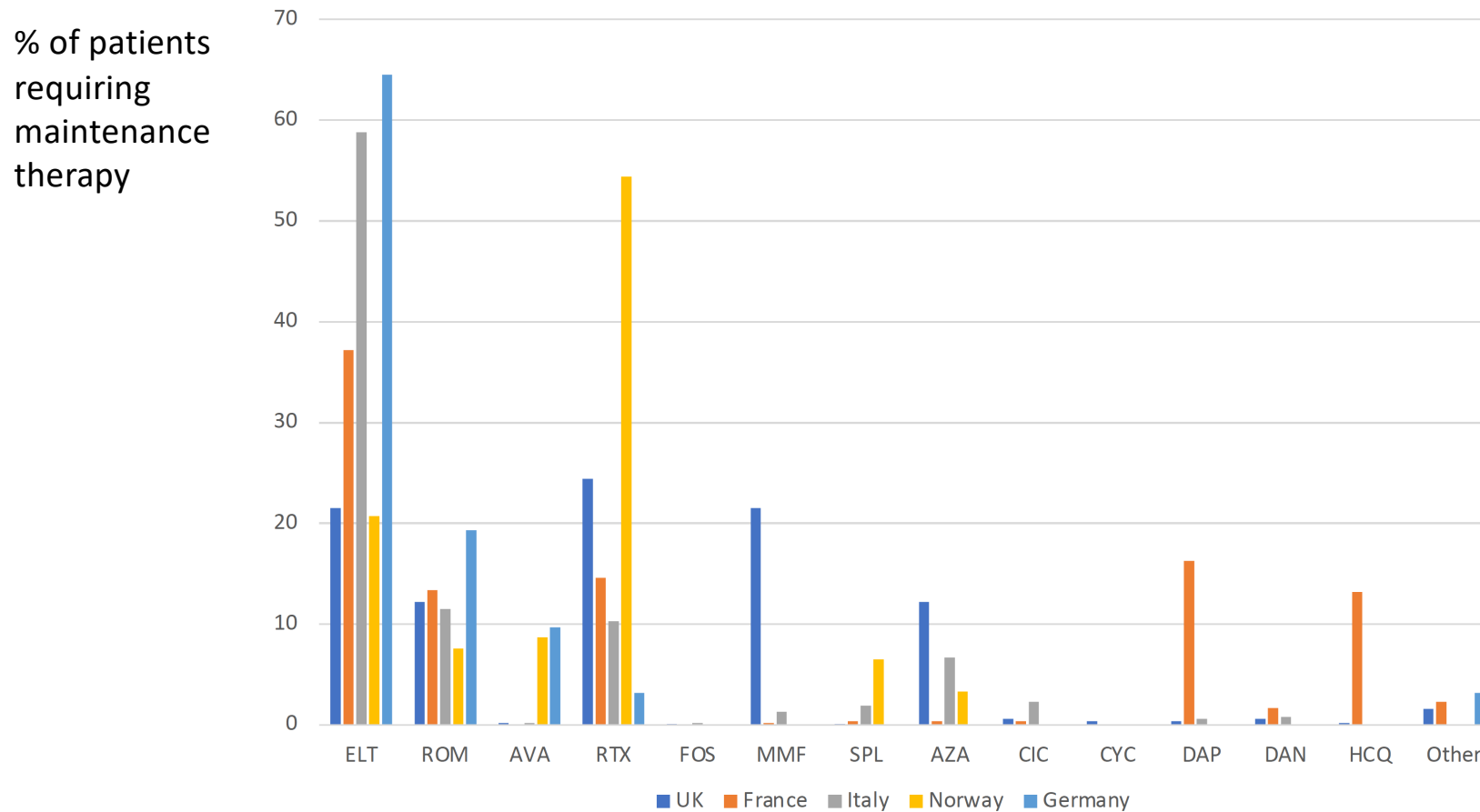
% of patients requiring maintenance therapy



- ✓ eltrombopag, n=1249
- ✓ rituximab, n=806
- ✓ romiplostim, n=698
- ✓ mycophenolate, n=482
- ✓ azathioprine, n=299
- ✓ dapsone, n=140
- ✓ splenectomy, n=140
- ✓ hydroxychloroquine, n=104
- ✓ avatrombopag, n=47
- ✓ cyclosporine, n=46
- ✓ fostamatinib, n=38
- ✓ danazol, n=38



Results: focus on the first maintenance therapy (i.e. second-line ITP therapy)



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✓ Subgroups by age

- rituximab less frequently used in elderly (≥ 80 years) in France and Norway
- TPO-RAs less frequently used in elderly (≥ 80 years) in Italy

✓ Subgroups by sex

- azathioprine was used more frequently in women in the UK
- hydroxychloroquine was used more frequently in women in France

✓ Subgroups by time periods of ITP diagnosis

- the use of TPO-RAs increased in all countries except Norway
- the use of rituximab decreased in all countries except Italy and Norway
- the use of dapson decreased in France



Discussion

STRENGTHS

- ✓ Large population, 6 clinical registries
- ✓ Recent data
- ✓ Common extraction model and analysis plan
- ✓ Measure of the impact of national regulation about restriction of use (new drugs) and the evolution of label (TPO-RAs)

LIMITATIONS

- ✓ Discrepancies about dates of registries (recent in Germany, Italy and Norway)
- ✓ Inclusion of registries with all treated pITP patients (not focused on a specific treatment like in Spain)
- ✓ Study period ended in 2022: the evolution of place in the strategy of new drugs need to be re-assessed in the coming years (avatrombopag, fostamatinib)



Conclusions

1. **Approximately 40% of patients with pITP required a maintenance (second-line) treatment and 20% required third-line treatment**
2. **There were few differences across Europe regarding the preferred maintenance therapies**
3. **Over the last decade, the use of TPO-RAs increased while the use of rituximab, immunosuppressants and splenectomy decreased**
4. **More generally, this study demonstrated the feasibility of conducting joint studies in ITP registries at the European level with >5000 patients included**



Thank you



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