



# 2<sup>ND</sup> meeting of the European Research Consortium on ITP

NEW INSIGHTS INTO IMMUNE  
THROMBOCYTOPENIA

Paris Crowne Plaza Paris République

April 23-24, 2026



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## Pharmacoeconomics of ITP: does cost matter?

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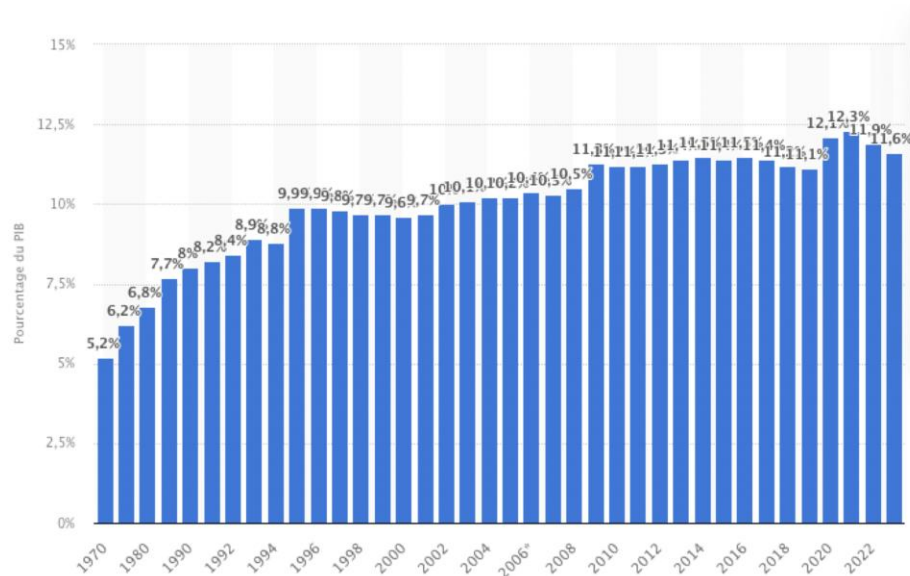
## Disclosures of GODEAU Bertrand, *January 2026*

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AMGEN	no	no	Yes	no	no	no	<ul style="list-style-type: none"> <li>• Communication during symposium</li> <li>• Funding for participation in conferences</li> </ul>
NOVARTIS	no	no	Yes	no	no	no	<ul style="list-style-type: none"> <li>• Communication during symposium</li> <li>• Funding for participation in conferences</li> </ul>
GRIFOLS	no	no	Yes	no	no	no	Communication during symposium
LFB	no	no	no	no	no	no	Funding for participation in conferences

# Issues

- Rising healthcare costs → Risk to the system's financial stability
- Therapeutic innovations play a major role in driving up healthcare costs
- Rising costs are a major concern for the ITP
- What can we do to combat this phenomenon without stifling therapeutic innovation?

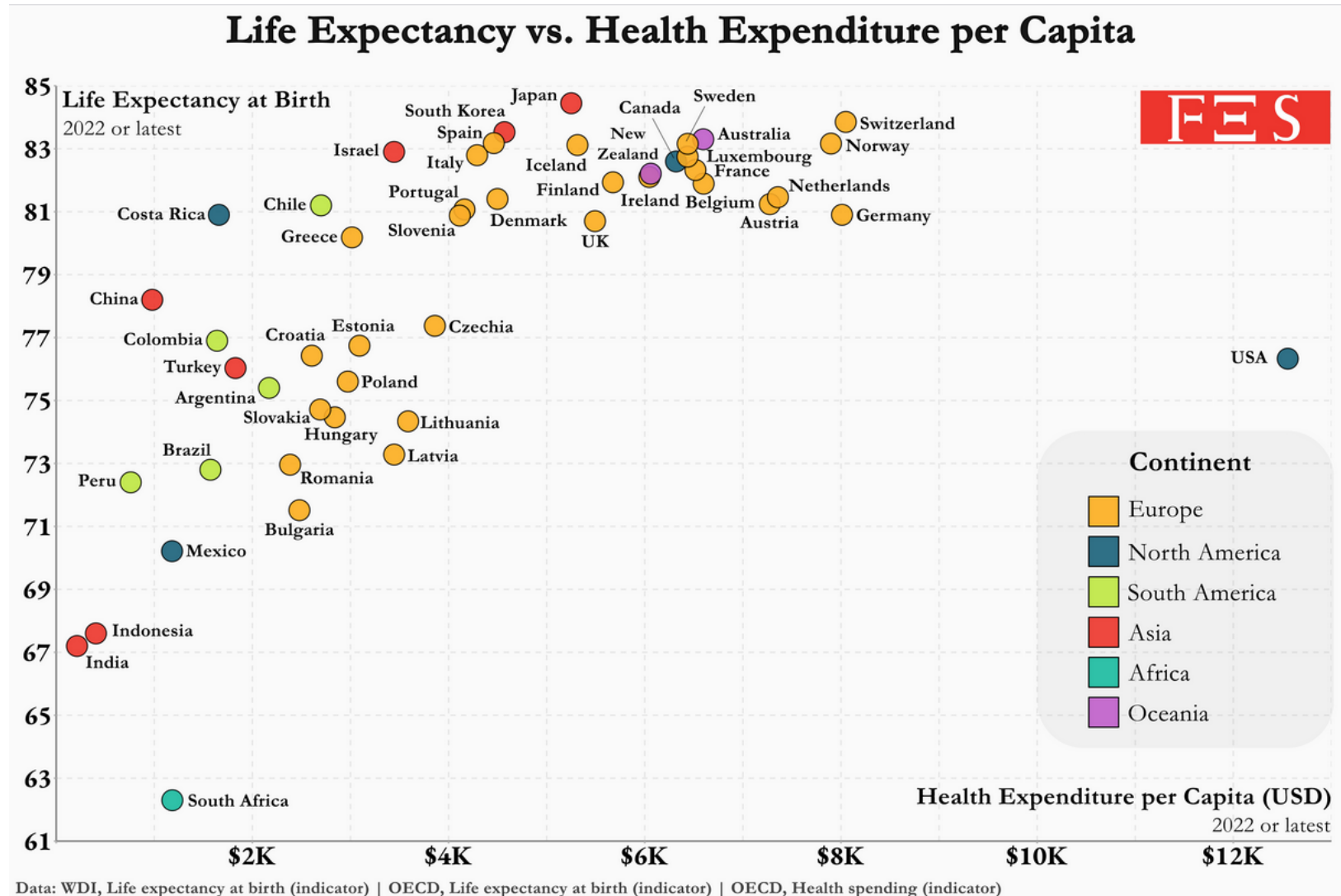
# Rising healthcare costs



**Healthcare spending as a percentage of Gross Domestic Product (GDP) in France between 1970 and 2023**

Country	% of gross domestic product in 2022
USA	16.5 %
UK	11.3 %
Germany	12.6 %
France	11.8 %
Italy	8.7 %
Spain	7.7 %

# Rising healthcare costs



# Innovative therapies play a significant role in driving up healthcare costs and often target small numbers of patients with rare diseases

- Infectious diseases: 22 Md\$
- Cancer: 50 Md\$
- Cardiovascular: 29 Md\$
- Metabolic: 19 Md\$
- Immunology: 22 Md\$

Rang	Nom	Laboratoire	Aire thérapeutique	Ventes en Md\$	Evolution vs 2023
5	Biktarvy* (bictegravir + emtricitabine + tenofovir alafenamide)	Gilead	● VIH	13,4	↗ +1
20	Cosentyx* (sécukinumab)	Novartis	● Dermatologie	6,1	↓ Entrée
7	Darzalex* (daratumumab)	J&J	● Cancer	11,67	↗ +3
4	Dupixent* (dupilumab)	Sanofi	● Immunologie	13,5	↗ +3
2	Eliquis* (apixaban)	BMS/Pfizer	● Cardiovasculaire	21	↔ Idem
15	Entresto* (sacubitril+valsartan)	Novartis	● Cardiovasculaire	7,8	↓ Entrée
10	Eylea* (afibercept)	Bayer/Regeneron	● Ophtalmologie	9,4	↗ +1
16	Farxiga*/Forxiga* (dapagliflozine)	AstraZeneca	● Diabète	7,7	↓ Entrée
13	Gardasil*	Merck & Co	● Vaccin HPV	8,6	↔ Idem
12	Humira* (adalimumab)	AbbVie	● Maladies auto-immunes	8,9	↘ -8
1	Keytruda* (pembrolizumab)	Merck & Co	● Cancer	29,5	↔ Idem
8	Mounjaro* (tirzépatide)	Eli Lilly	● Diabète/obésité	11,5	↓ Entrée
17	Ocrevus* (ocrelizumab)	Roche	● Maladies auto-immunes	7,4	↘ -2
11	Opdivo* (nivolumab)	BMS/Ono Pharmaceutical	● Cancer	9,38	↘ -2

Top 10 best-selling drugs worldwide in 2024

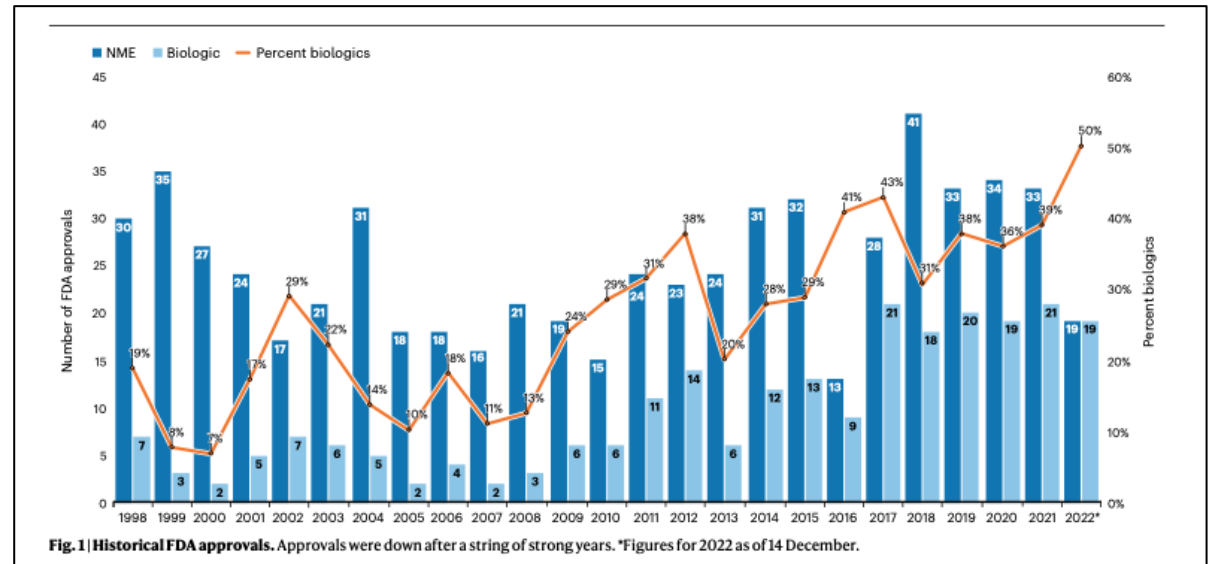
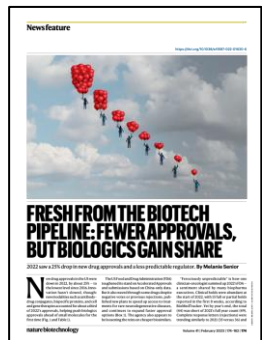
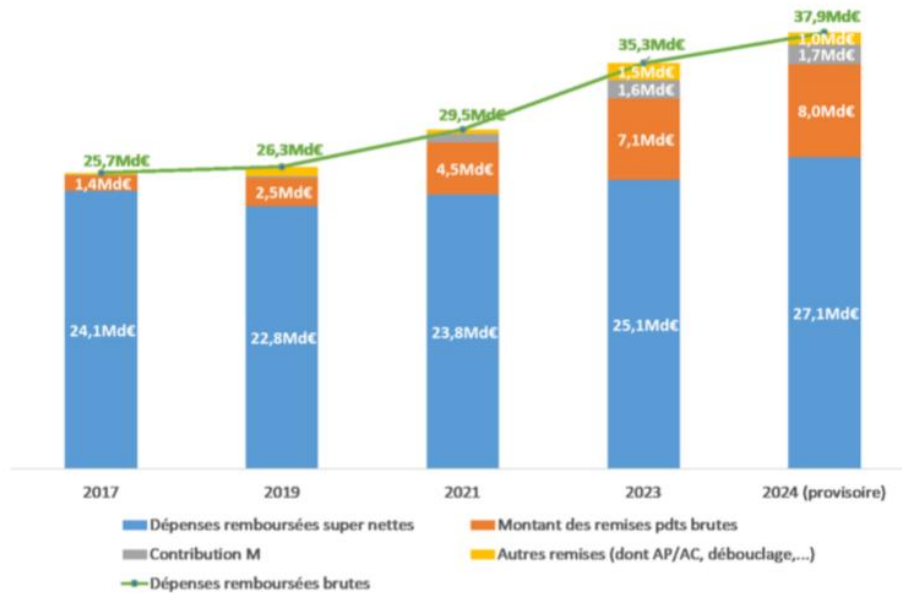


Fig. 1 | Historical FDA approvals. Approvals were down after a string of strong years. \*Figures for 2022 as of 14 December.

The percentage of biologics approved by the FDA

# Innovative therapies play a significant role in driving up healthcare costs and often target small numbers of patients with rare diseases



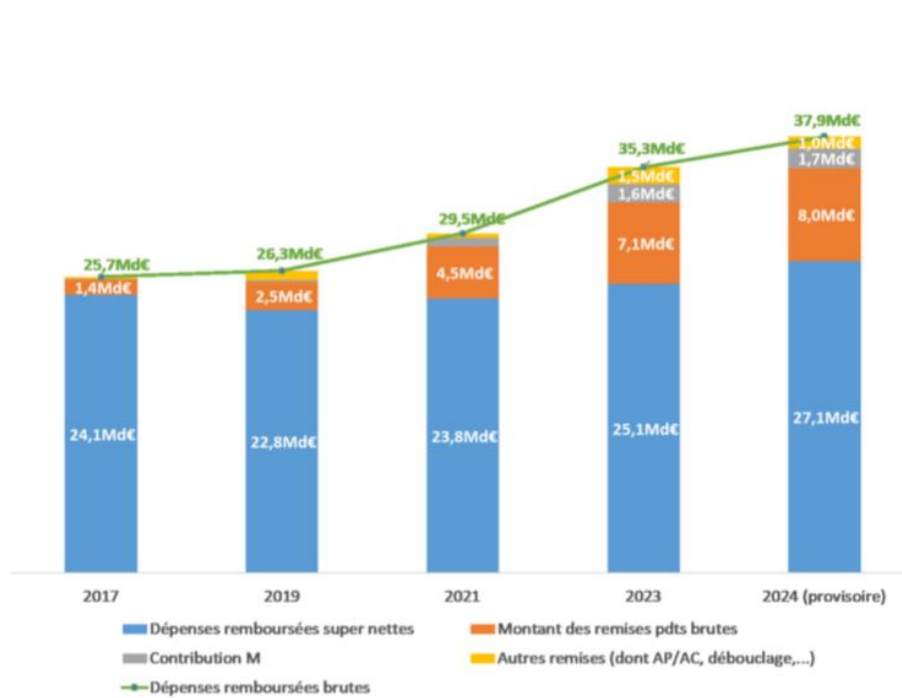
2021	2022	2023	2024	2025
100 Mds	102 Mds	105 Mds	105 Mds	110 Mds

Source : Medic'AM, Rétroced'AM, Scan Santé, CEPS (données provisoires pour l'année 2024) ; Traitements Cnam

Annual spending on medications in France

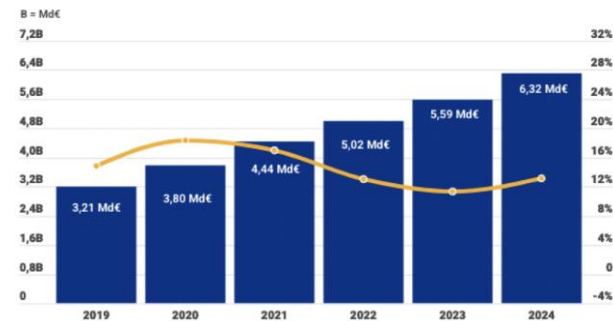
Annual hospital funding in France

# Innovative therapies play a significant role in driving up healthcare costs and often target small numbers of patients with rare diseases

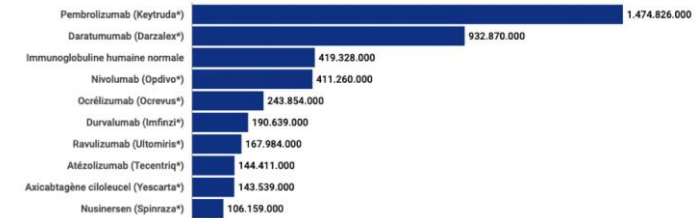


Source : Medic'AM, Rétroced'AM, Scan Santé, CEPS (données provisoires pour l'année 2024) ; Traitements Cnam

Annual spending on medications in France



Annual expenses for high-cost therapies with specific "add-on list" reimbursement



Top ten of high-cost therapies in hospital in France in 2024

2021	2022	2023	2024
+8.8%	+8%	+7.8%	+6.8%

Rising cost of high-cost therapies dispensed for out-patients

ITP: how much does it cost?

# 2010: before the era of TPO-RAs

Ann Hematol (2011) 90:441–446  
DOI 10.1007/s00277-010-1087-x

ORIGINAL ARTICLE

## Costs of managing severe immune thrombocytopenia in adults: a retrospective analysis

Mehdi Khellaf · Jean-Gabriel Le Moine · Priscille Poitrinal · Camille Francesconi · Albert Haddad · Philippe Bierling · Marc Michel · Laurent Eckert · Robert Launois · Bertrand Godeau

Received: 13 February 2010 / Accepted: 14 September 2010 / Published online: 5 October 2010  
© Springer-Verlag 2010

- Single-centre, single-arm, retrospective 1-year observational cohort study of adult patients with chronic ITP from a French hospital
- 57 patients (42F/15M) with a mean age of 48 years (SD=19) at ITP diagnosis
- Mean platelet count at diagnosis was 28 +/- 26 G/L
- Mean duration of ITP was 3.1 years (SD=2); 8 patients had undergone splenectomy at baseline.

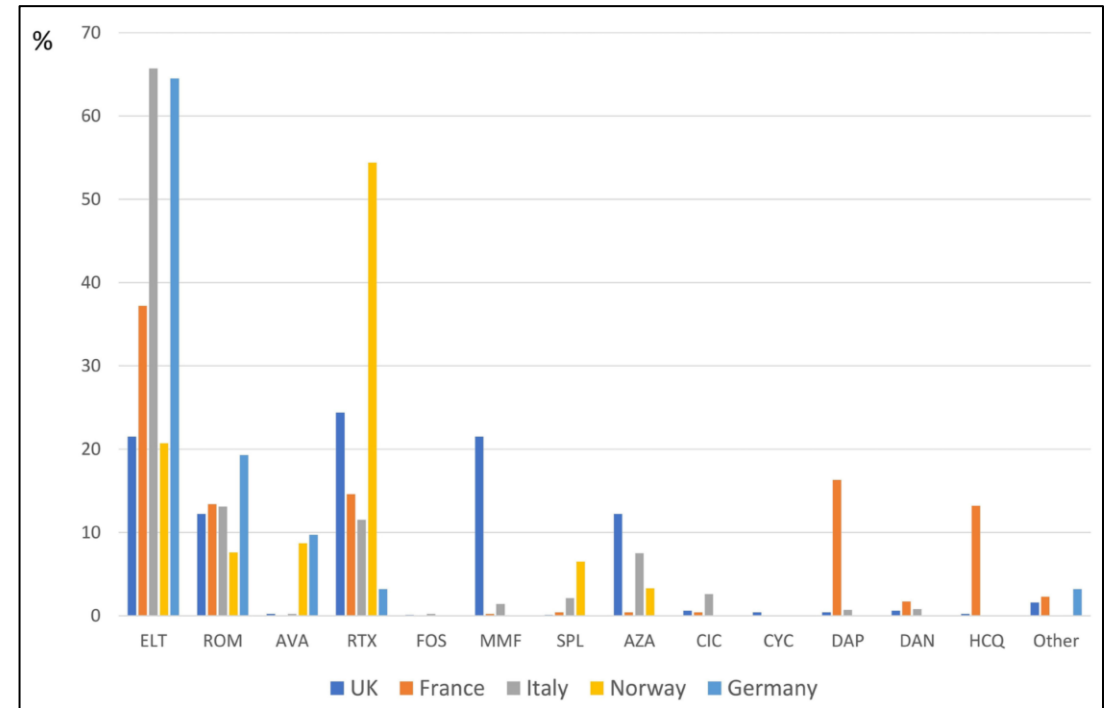
**Table 3** Costs during 1 year

Type of resource	All patients			Subgroup 1—hospitalisation			Subgroup 2—hospitalisation+IVIg		
	No. treated	Cost per consumer <sup>a</sup> (€) Mean (SD)	Cost per patient (€) Mean (SD)	No. treated	Cost per consumer <sup>a</sup> (€) Mean (SD)	Cost per patient (€) Mean (SD)	No. treated	Cost per consumer <sup>a</sup> (€) Mean (SD)	Cost per patient (€) Mean (SD)
Drugs not included in DRG									
Immunoglobulin	12	11,531 (24,142)	2,428 (11,704)	12	11,531 (24,142)	5,125 (16,753)	12	11,531 (24,142)	11,531 (24,142)
Rituximab	8	7,054 (2,288)	990 (2,601)	8	7,054 (2,288)	2,090 (3,490)	4	7,428 (2,712)	2,476 (3,922)
Type of visit									
Full hospitalisation	23	8,520 (10,110)	3,438 (7,612)	23	8,520 (10,110)	7,258 (9,798)	12	11,664 (13,259)	11,664 (13,259)
Brief hospitalisation	8	2,226 (1,134)	352 (924)	8	2,418 (1,044)	716 (1,249)	3	2,994 (1,595)	748 (1,515)
Outpatient visit	56	86 (37)	85 (39)	26	95 (39)	91 (42)	11	108 (32)	99 (44)
Total costs			7,294 (95% CI=3,369–13,584)			15,334 (95% CI=7,876–27,459)			26,581 (95% CI=12,241–50,578)

<sup>a</sup> A ‘consumer’ is a patient who actually received the drug or experienced a hospital or outpatient visit (a ‘consumer’ of healthcare resources). The cost per patient column shows the average cost per patient for the entire patient population, i.e. counting both patients who use healthcare resources and those who do not, i.e. ‘watch and wait’ patients

# The cost of ITP probably varies greatly from country to country

- Treatment strategies vary
- Significant variations in treatment costs
- Lack of robust health economic studies



**Proportion of first maintenance treatment in patients with primary ITP over the total number of patients treated with maintenance therapy in six European registries**

Moulis *et al*, Haematologica 2025

# Significant variations in treatment costs of ITP from country to country

## Why Italian Patients Are Choosing Turkey for Thrombocytopenia Treatment

Italian patients searching for Thrombocytopenia treatment price Italy often hit a cost wall, prompting many to look southward. High out-of-pocket expenses in Turin, combined with limited appointment slots, make the...

[Read More](#)



# Significant variations in treatment costs of ITP from country to country

The screenshot shows the Liv Hospital website's comparison tool. On the left, under the 'TURIN' header, the 'Average Thrombocytopenia Treatment' cost is listed as \$9,115. Below this, it notes 'High medical costs, long wait times for specialists, and complex insurance procedures.' In the center, a large 'VS' icon is positioned above a 'COMPARE NOW' button. On the right, under the 'ISTANBUL' header, the cost is \$2,260, with a 'Save 75%' badge. Below this, it states 'Same Treatment Quality' and 'JCI accredited hospitals, all-inclusive packages, VIP transfers, and 5-star accommodation.' At the bottom, the main heading reads 'Thrombocytopenia Treatment Cost in Turin vs Turkey', followed by a paragraph: 'Looking for the Thrombocytopenia treatment cost in Turin? Many Italians first check local prices before exploring alternatives abroad. In Italy, especially in Turin, the price level is generally higher due...' and a 'Read More' link.

The infographic is titled 'Average Costs Of Thrombopoietin Receptor Agonists (TPO-RAs)'. It explains that medications like eltrombopag and romiplostim stimulate platelet production in chronic immune thrombocytopenia patients unresponsive to first-line treatments, improving platelet counts and quality of life. A 'Save % 80' badge is present. Below, six city cards are shown, each with a flag, city name, cost, and duration:

City	Cost	Duration
Istanbul (Turkey)	\$1,200	5-6 Days
Turin (Italy)	\$8,500	5-6 Days
Munich (Germany)	\$15,000	6-7 Days
Vienna (Austria)	\$5,500	5-6 Days
Zagreb (Croatia)	\$4,800	5-6 Days
Budapest (Hungary)	\$2,850	5-6 Days

\*Based on Turkey-wide hospital averages.

# Significant variations in treatment costs of ITP from country to country

Country	Estimated price
Spain	300 €
UK	300-350 £
Italy	300-350 €
<b>USA</b>	<b>3000 to 5000 \$</b>
Germany	345 €
France	242 €

Catalog price Mabtera® and Rituxan® 500 mg

Country	Estimated price
Spain	1024 €
UK	1000 to 1800 £
Italy	900 to 1400 €
<b>USA</b>	<b>10 000 to 12 000 \$</b>
Germany	1000 €
France	923 €

Catalog price Revolade® 50mg, 28 tablets

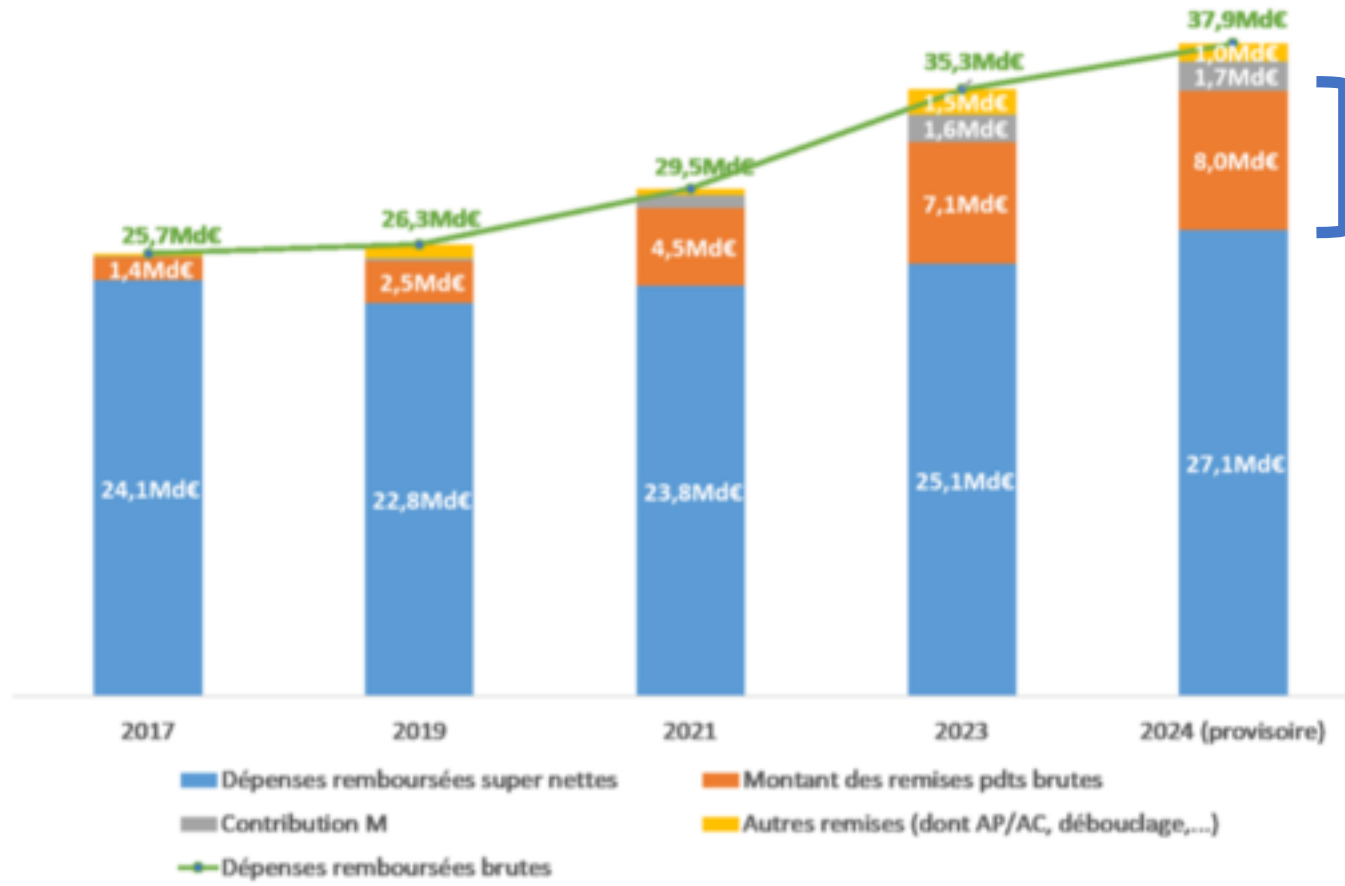
Country	Estimated price
Spain	?
UK	4000 £
Italy	4000 €
<b>USA</b>	<b>8400 \$</b>
Germany	?
France	4000 €

Catalog price Nplate® 500mcg, 4 bottles

There may be **significant differences between the list price and the price actually paid** by health authorities or insurance companies, with patients facing variable out-of-pocket costs depending on the country.

**These discounts are confidential** and often impossible to ascertain.

**In France, the pharmaceutical industry pays € 7 Mds to health authorities out of a total budget of €35 Mds, representing 28%**



Pharmaceutical companies to cut prices by 8 billion, or 28%

Source : Medic'AM, Rétroced'AM, Scan Santé, CEPS (données provisoires pour l'année 2024) ; Traitements Cnam

## Annual spending on medications in France

# Changes in the price of rituximab since its launch and with the arrival of biosimilars

	Rituximab, Mabthéra® Bottle of 500 mg		Truxima® (biosimilar rituximab) Bottle of 500 mg	
	Reimbursement rate	Purchase price	Reimbursement rate	Purchase price
2005	1410 €	<b>The same</b>	--	<b>Confidential!</b> (negotiations between hospitals and pharmaceutical companies). Reimbursement to hospitals based on the National Health Service rate
2009	1318 €		--	
2016	1186 €		--	
2017	830 €		--	
<b>2019</b>	706 €		720€	
2022	494 €		494 €	
2025	346 €		345 €	
2026	242 €		242 €	

**Biosimilar available** →

# Changes in the cost of eltrombopag treatment following the arrival of generic versions in France

	28 tablets 50 mg	28 tablets 75 mg	] - 45%
Eltrombopag REVOLADE®	1024 €	1519 €	
Eltrombopag BIOGARAN®	552 €	812 €	

## A biosimilar of romiplostim exists but not available

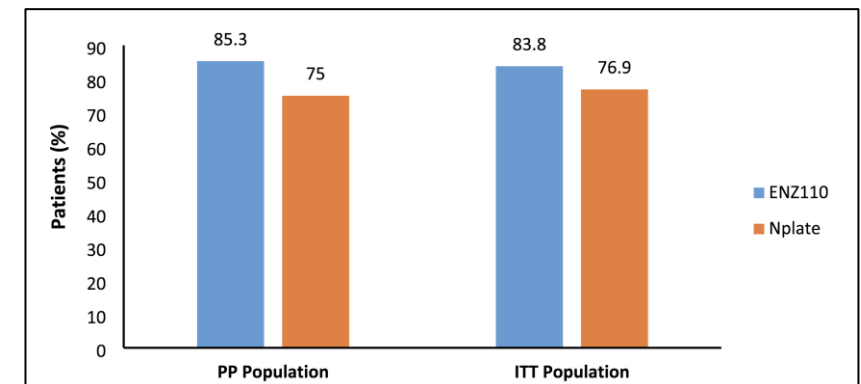
Indian J Hematol Blood Transfus (2023) 39:435–441  
<https://doi.org/10.1007/s12288-022-01602-5>

ORIGINAL ARTICLE

**Efficacy and Safety of Biosimilar Romiplostim Versus Innovator Romiplostim in Patients with Chronic Immune Thrombocytopenia**

S. Chandrakala<sup>1</sup> · Manoj Toshniwal<sup>2</sup> · Mitesh Halwawala<sup>3</sup> · Namita Padwal<sup>4</sup> · Neeraj Sidharthan<sup>5</sup> · Pankaj Malhotra<sup>6</sup> · B. Prashantha<sup>7</sup> · Riya Ballikar<sup>8</sup> · Sandip Shah<sup>9</sup> · Shashikant Apte<sup>10</sup> · T. Kasi Viswanathan<sup>11</sup> · Vijay Ramanan<sup>12</sup> · Akhilesh Sharma<sup>13</sup> · Dattatray Pawar<sup>13</sup> · Roshan Pawar<sup>13</sup> · Vinayaka Shahavi<sup>13</sup>

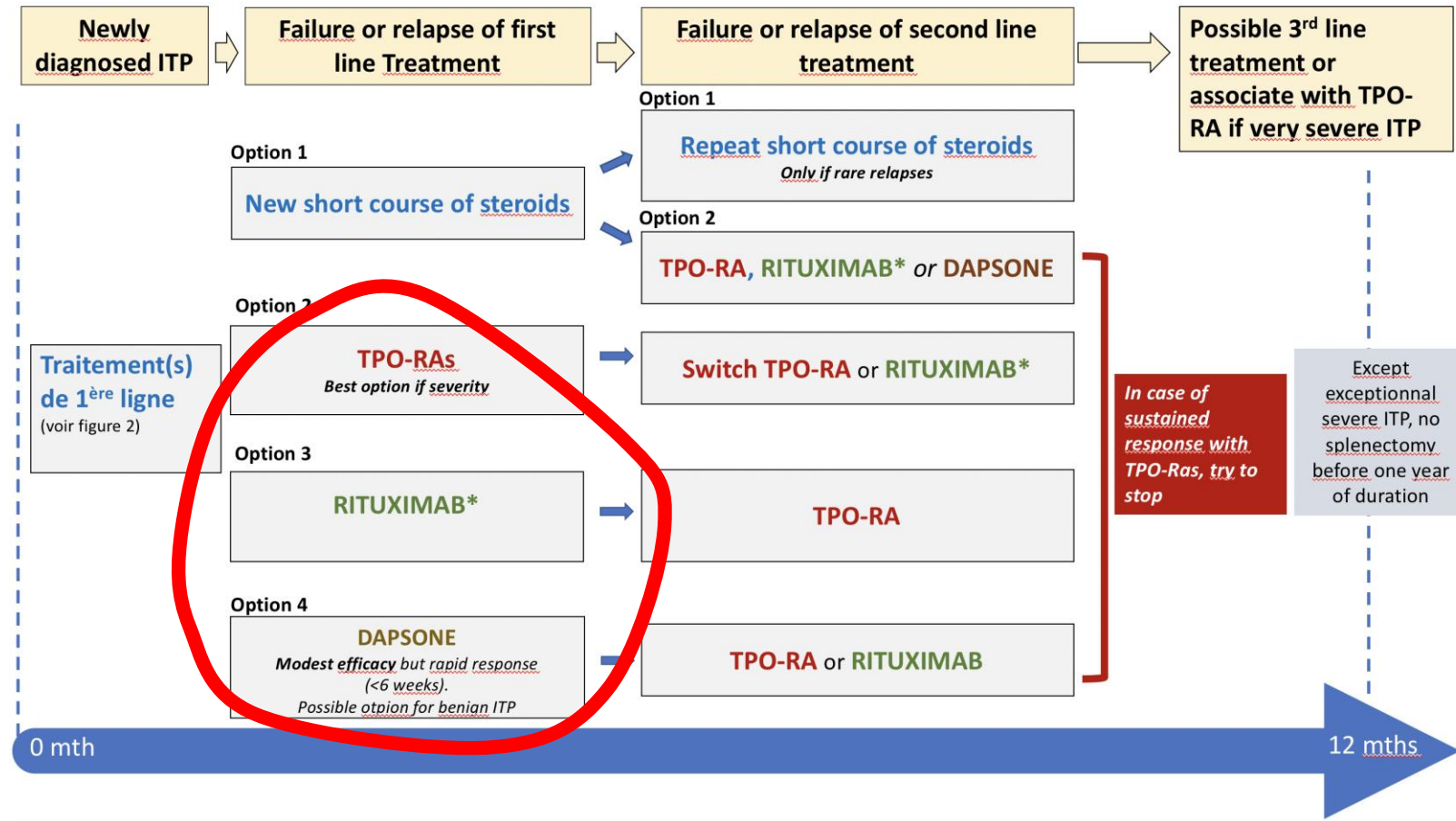
**Abstract** Romiplostim is a Food and Drug Administration (FDA)-approved therapy for immune thrombocytopenia (ITP). Biosimilar is a biological product that has no clinically meaningful difference from an existing FDA-approved reference product. It has a potential of lowering health-care-related cost. Biosimilar of romiplostim can be made available to patients with ITP at a low cost and can be beneficial in providing the best therapy. Thus, the efficacy and safety of biosimilar romiplostim (ENZ110) was compared with innovator romiplostim (Nplate) with respect to platelet response in patients with chronic ITP. This was a prospective, multicenter, randomized, and double-blind clinical trial.



Proportion of patients responding to the treatment during the 12 weeks of romiplostim treatment.

Do French doctors take cost into account when making treatment recommendations?

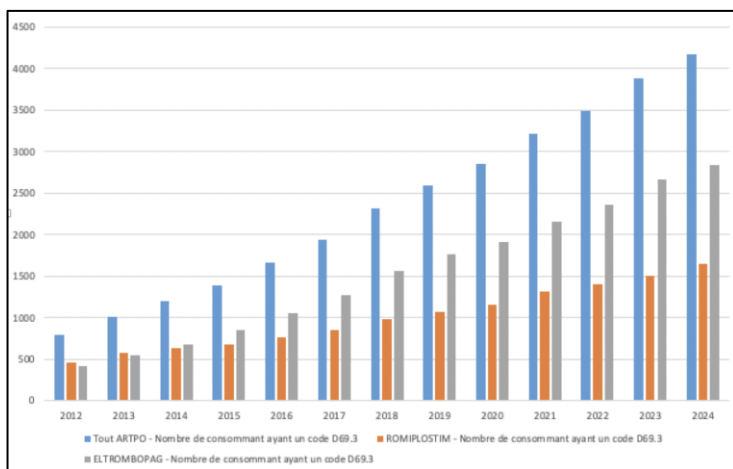
# Second line treatments, French guidelines



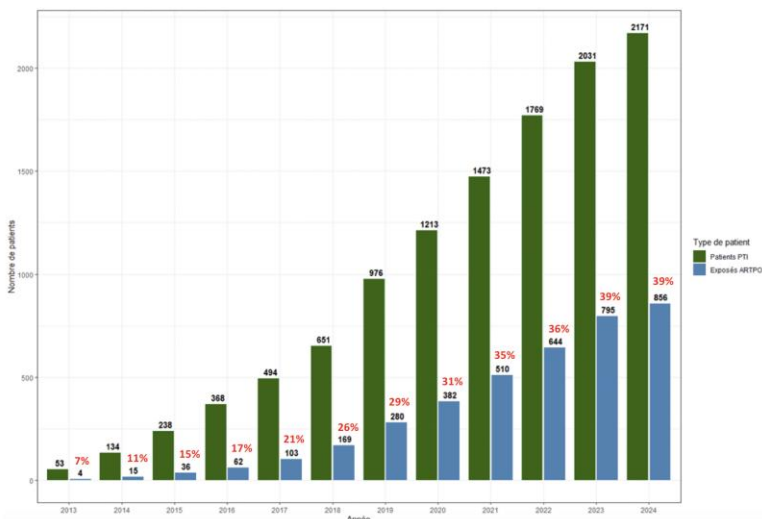
# Comparison of second line treatments in French guidelines

	TPO-RA		Rituximab	Dapsone
	Eltrombopag	Romiplostim		
Efficacy proved by controlled randomized study	Yes		No « Failure »	No « Failure »
Initial response	80%		60%	20-50%
Relapse if stopping	20-50%		30% à 5 ans	?
Side effect	Thrombosis		Infection	Skin eruption, Methemoglobinemy Hemolysis
Annual cost	<b>12 000 €</b> (50 mg/d)  6000 € for generic	<b>54 240 €</b> (70 kg 5 mcr/kg)	<b>400 € + 2600 €</b>	<b>40 €</b>

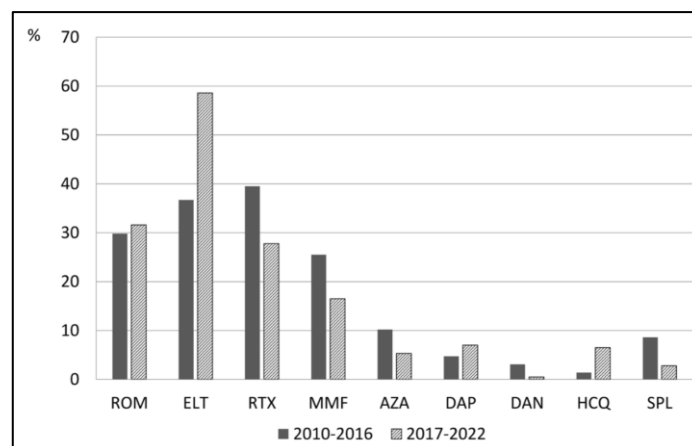
# More TPO-RAs, less rituximab in France and in Europe



Number of patients exposed to TPO-RAs in France



Number of patients exposed to TPO-RAs in CARMEN register



Proportions of patients initiating each major maintenance ITP treatment during 2010-2016 versus 2017-2022 in six European registries, Moulis *et al*, Haematologica 2025

Treatments	Number of patients (%)
Steroids	1500 (78%)
IVIg	737 (38%)
<b>TPO-RAs</b>	<b>584 (30%)</b>
Eltrombopag	464 (24%)
Romiplostim	282 (14%)
<b>Rituximab</b>	<b>318 (16%)</b>
Dapsone	136 (7%)
HCQ	136 (7%)
VBL	51 (3%)
MMF	33 (2%)
Splenectomy	21 (1%)

Exposure to treatments over the course of the disease in 1,920 patients with primary ITP included in the CARMEN prospective registry

# TPO-RAs and fostamatinib expenditure in France

	2021	2022	2023	2024	2025
REVOLADE®	62.9	73.2	80.6	82.9	63.7
NPLATE®	56.7	60.5	65.7	72.4	80.6
TAVLESSE®	-	-	-	-	3.5



**144.3 M€**

# What is likely to happen in the near future?

Car T cells  
Bispecific  
Anti CD19  
Anti CD38  
Anti BAFF-R

FcRn  
Inhibitors

**Therapeutic  
innovations**



**Unsustainable  
rising costs ?**

Complement  
inhibitors

Anti SYK

BTK  
inhibitors

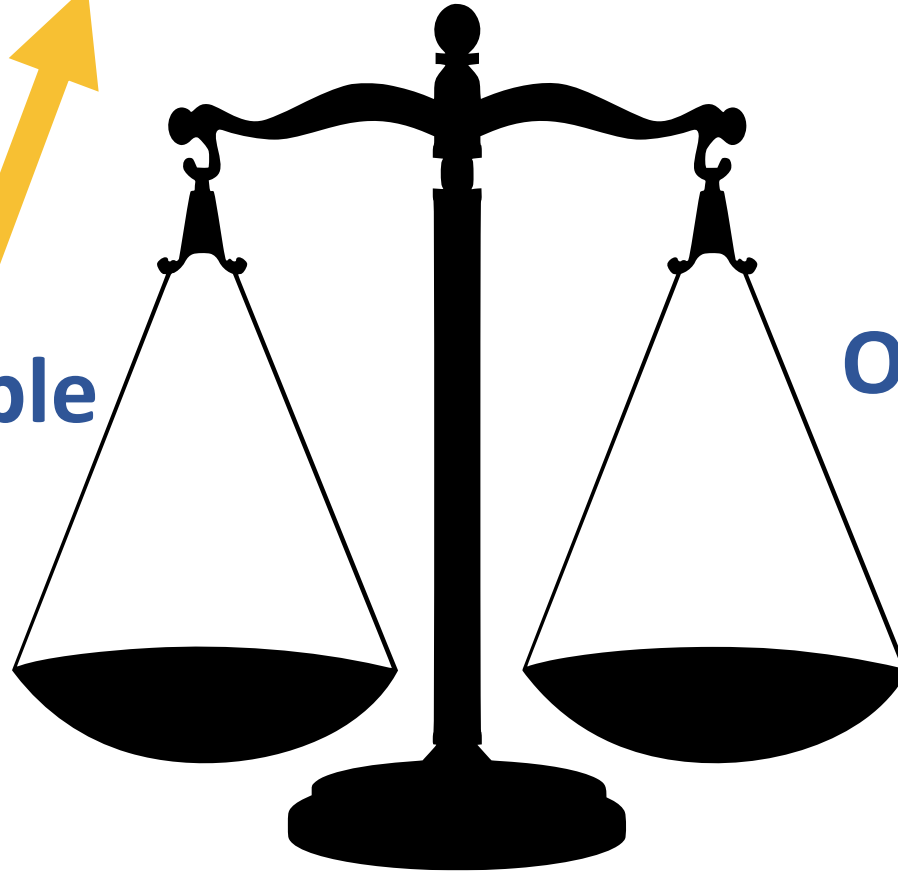
**Challenge for  
pharmaceutical  
companies**

**Return on investment**

**Number of eligible  
patients**

**Obtaining a sufficient  
reimbursement  
amount**

**To continue to invest in R&D**



# Innovative treatments For which patients?

## SHORT REPORT

Platelets, Thrombosis and Haemostasis



### Difficult-to-treat primary immune thrombocytopenia in adults: Prevalence and burden. Results from the CARMEN-France registry

Guillaume Moulis<sup>1,2</sup> | Manuela Rueter<sup>2</sup> | Aymeric Duvivier<sup>3</sup> | Matthieu Mahévas<sup>4</sup> |  
Jean-François Viillard<sup>5</sup> | Thibault Comont<sup>6</sup> | Stéphane Chèze<sup>7</sup> | Sylvain Audia<sup>8</sup> |  
Mikaël Ebbo<sup>9</sup> | Louis Terriou<sup>10</sup> | Jean-Christophe Lega<sup>11</sup> | Pierre-Yves Jeandel<sup>12</sup> |  
Ines Hemim<sup>3</sup> | Sylvie Bozzi<sup>3</sup> | Ahmed Daak<sup>13</sup> | Hikaru Okada<sup>14</sup> | Bernard Bonnotte<sup>8</sup> |  
Marc Michel<sup>4</sup> | Maryse Lapeyre-Mestre<sup>2,15</sup> | Bertrand Godeau<sup>4</sup> |  
the CARMEN-France Investigators Group

## Summary

The aim of this study was to assess the prevalence and the burden of difficult-to-treat primary ITP (pITP), defined by the need for another ITP treatment after romiplostim and eltrombopag. Adult patients were selected in the prospective, real-world CARMEN-France registry up to December 2021. Out of 821 adult patients with pITP, 29 had difficult-to-treat ITP (3.5%; 95% confidence interval [CI]: 2.3%–4.8% in total; 7.6%; 95% CI: 4.9%–10.2% of patients needing  $\geq 2$ nd line treatment). The 3-year cumulative incidence of bleeding, infection and thrombosis was 100%, 24.1% and 13.8% respectively. The median cumulative duration of hospital stays was 31 days (median follow-up: 30.3 months).

## KEYWORDS

difficult-to-treat, epidemiology, immune thrombocytopenia, infection, refractory, thrombosis

**Challenge for  
health care  
authorities**

- **maintain the system's financial stability**
- **ensure access to care**
- **not to limit therapeutic innovations**

Return on investment

To continue to invest in R&D

Number of  
patients

sufficient  
investment  
to

# CONCLUSIONS

- Physicians and patients should be aware that some treatment are very expensive
- Should we conduct medico-economic studies?
- Should we develop academical innovative treatment ? (Car-T ?)
- Sould we include the medico-economic dimension into our guidelines?
- To include the medico-economic dimension at the individual level is complicated and risky
- The high cost of a treatment must be justified by a major breakthrough: be wary of false progress