

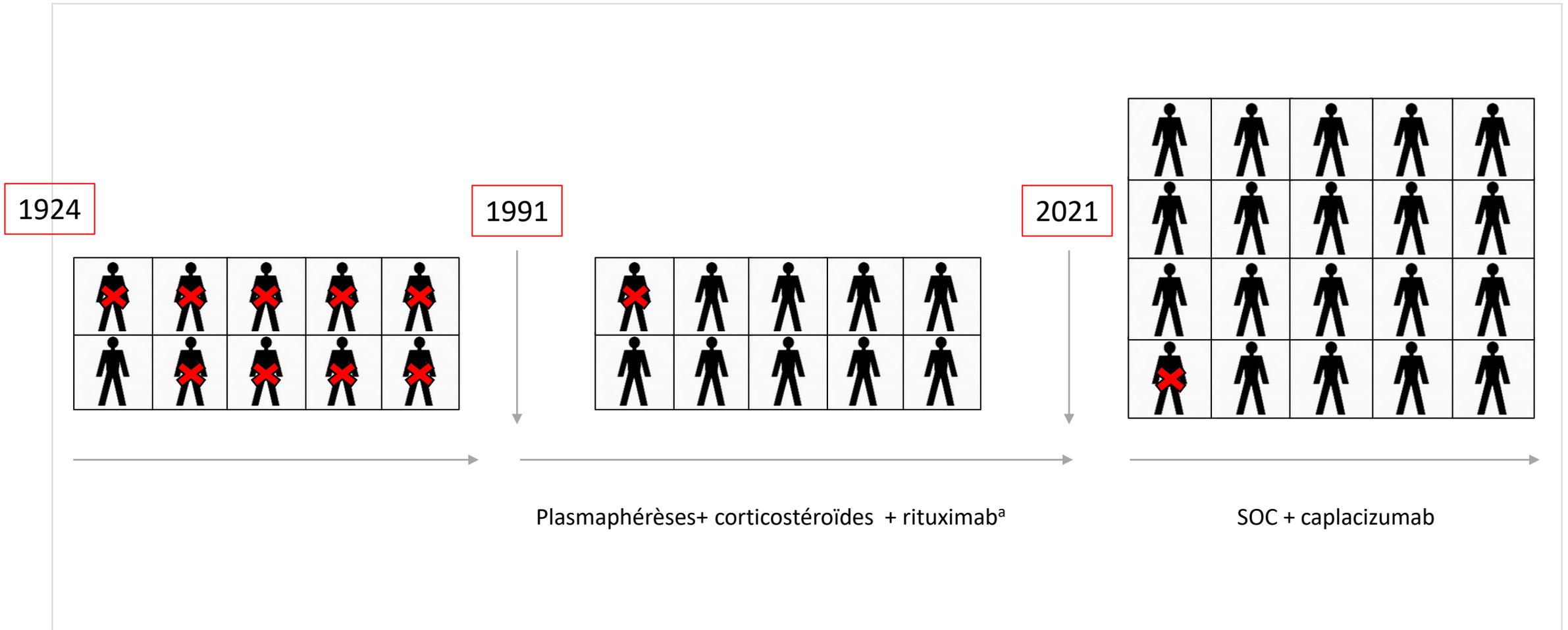
# PTTi en 2026: quand éviter les échanges plasmatiques



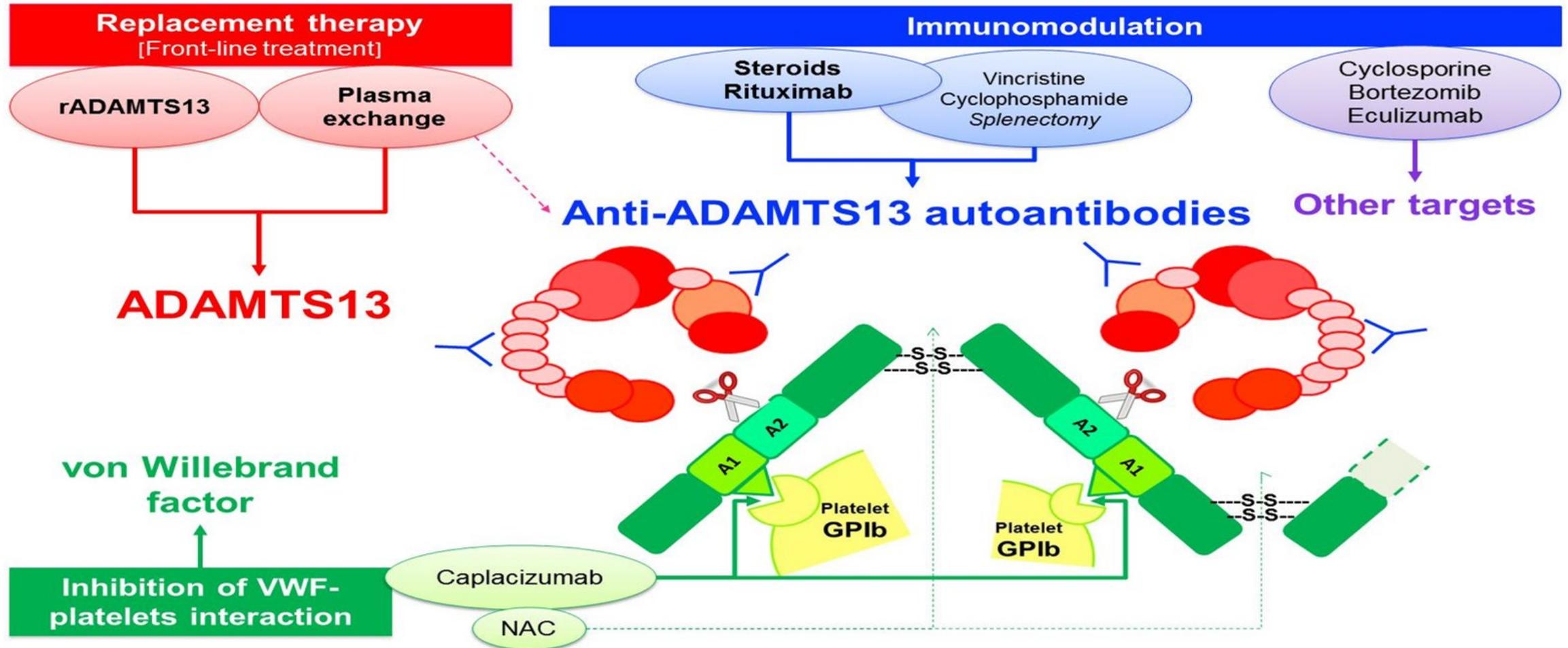
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Service de médecine interne  
Centre de compétence MAT  
CHU Edouard Herriot, Lyon



# Traitement et Mortalité 2025



# Phase aigue : la « tripléte »



Effets indésirables des EP

# TRANSFUSION

IMMUNE HEMATOLOGIC DISEASE

## Deaths and complications associated with the management of acute immune thrombotic thrombocytopenic purpura

Meaghan Colling, Lova Sun, Vivek Upadhyay, Justine Ryu, Ang Li, Lynne Uhl, Richard M. Kaufman, Christopher P. Stowell, Walter H. Dzik, Robert S. Makar ✉, Pavan K. Bendapudi ✉

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- Rétrospective 101 prise en charge, 74 patients de 2004 à 2017
- 56/74 (76%) ont présenté une complication aux EP
- 7/101 (6,9%) cp sévère

Complication	Number (%) of events (incidence per course of TPE*)
Allergic reaction	53 (52)
Mild: hives requiring diphenhydramine alone	34 (34)
Moderate: symptoms requiring steroids/antihistamines	15 (15)
Severe: anaphylaxis requiring epinephrine	4 (4)
Moderate hypocalcemia	9 (9)
Line-associated complications	12 (12)
Thrombotic line complications	1 (1)
Line-associated infections	2 (2)
Obstructed/misplaced catheter	6 (6)
Catheter insertion site bleeding <sup>†</sup>	2 (2)
Catheter-induced supraventricular tachycardia	1 (1)
Non-line-associated infections	1 (1)
Non-allergic transfusion reaction	2 (2)

\* A total of 101 discrete courses of TPE comprising a total of 1497 procedures were included. Some courses of TPE were complicated by more than one adverse reaction.

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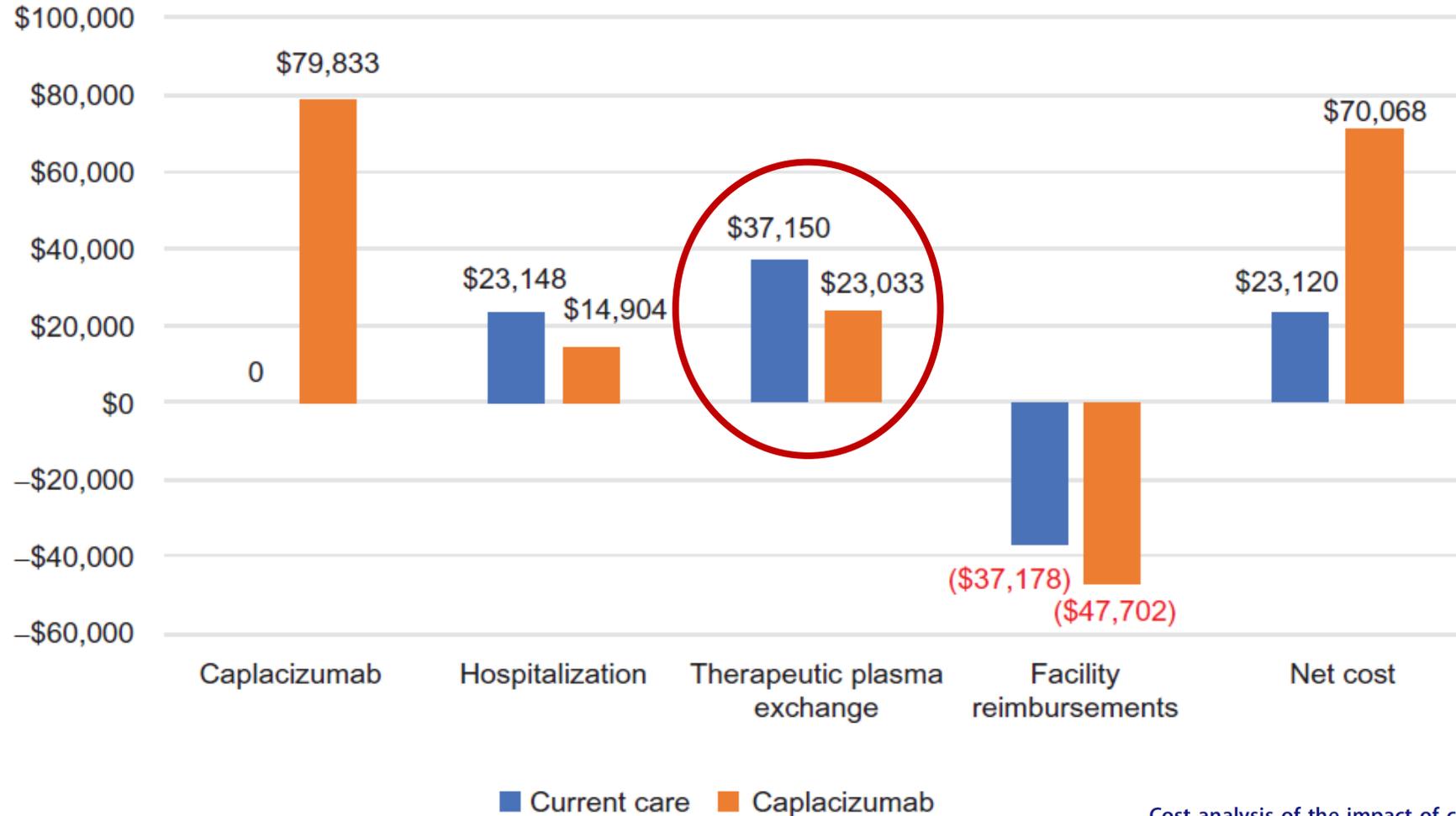
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- 1 décès consécutif à bactériémie sur infection de VVC

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# Coût global de la prise en charge des PTT



Cost analysis of the impact of caplacizumab in the treatment of acquired thrombotic thrombocytopenic purpura from a US hospital perspective

# Conséquences psychologiques

## **Multiple major morbidities and increased mortality during long-term follow-up after recovery from thrombotic thrombocytopenic purpura**

Cassandra C. Deford,<sup>1,2</sup> Jessica A. Reese,<sup>1,2</sup> Lauren H. Schwartz,<sup>3</sup> Jedidiah J. Perdue,<sup>3,4</sup> Johanna A. Kremer Hovinga,<sup>5</sup> Bernhard Lämmle,<sup>5</sup> Deirdra R. Terrell,<sup>1</sup> Sara K. Vesely,<sup>1</sup> and James N. George<sup>1,2</sup>

BLOOD, 19 SEPTEMBER 2013 • VOLUME 122, NUMBER 12

### **Dépression :**

- 19% syndrome dépressif majeur selon PHQ-8 contre 6% attendus

## **Increased Morbidity During Long Term Follow Up of Survivors of Thrombotic Thrombocytopenic Purpura**

Shruti Chaturvedi, MD<sup>1</sup>, Hashim Abbas, MD<sup>2</sup>, and Keith McCrae, MD<sup>3,4</sup>

*Am J Hematol.* 2015 October ; 90(10): E208. doi:10.1002/ajh.24138.

### **Dépression :**

- Prévalence sur la période 18,8% contre 6,1% attendus

- Anxiété 9/53, maladie bipolaire 1/53, hystérie 1/53

Le souvenir de la pose du Kt et des EP ressort dans les questionnaires de vécu post PTT

EXPERIENCE ACTUELLE SANS EP

# Expérience allemande + autrichienne

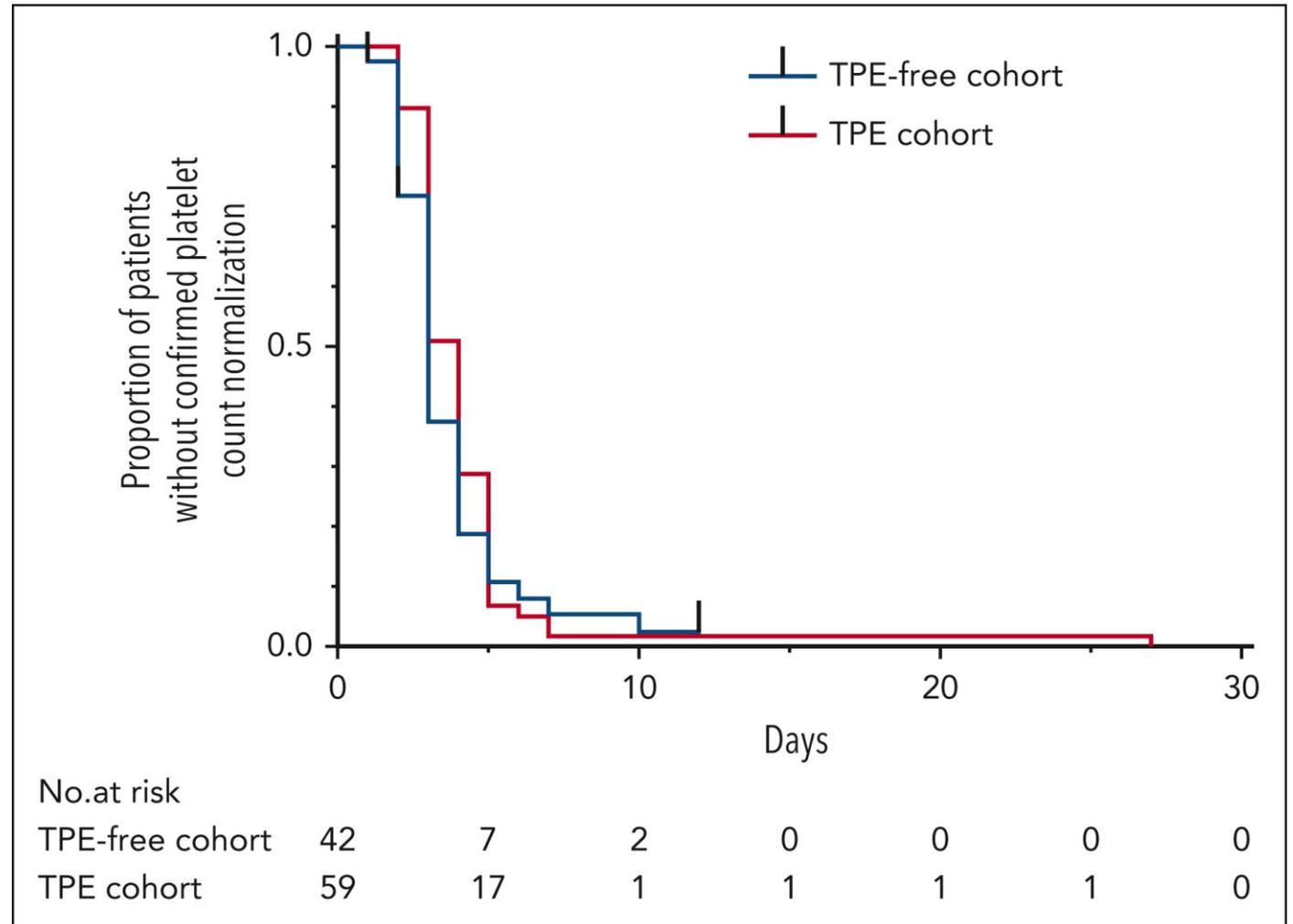
41 patients (42 épisodes) sans EP vs 59 avec EP

Tous on reçu du capla

Groupes comparables

Temps médian normalisation  
plaquettaire:

3jsans EP vs 4j groupe EP p=0,31



# Expérience allemande + autrichienne (2)

Parameter	TPE-free cohort (n = 42)	TPE cohort (n = 69)	P value
<b>Key secondary outcomes</b>			
Patients achieving a clinical response (including patients with subsequent TPE in the TPE-free cohort), n (%)	41 (97.6)	57 (96.6)	>.99
Patients with a clinical exacerbation, n (%)	2 (4.8)	9 (15.3)	.12
Patients refractory to therapy, n (%)	0 (0)	1 (1.7)	>.99
TTP-related death, n (%)	0 (0)	1 (1.7)	>.99
Median time to platelet count doubling (range; IQR)	1 (1-7; 1-2)	1 (1-4; 1-2)	.88
<b>Other secondary outcomes</b>			
Median time to recovery of ADAMTS13 activity to $\geq 20\%$ after treatment initiation (IQR), d	25 (13-33)	37 (19-51)	.01
Patients without confirmed recovery of ADAMTS13 activity to $\geq 20\%$ at end of follow-up, n (%)	4 (9.5)	10 (16.9)	.39
Median days in hospital (IQR)	11 (5-15)	14 (9-21)	.02
Patients admitted to ICU, n (%)	11 (26.2)	42 (71.2)	<.01

Lucas Kühne et al, Management of immune thrombotic thrombocytopenic purpura without therapeutic plasma exchange, Blood, 2024, Figure 1.



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**Confirme la faisabilité d'un traitement sans EP chez certains patients**  
**« trop bon » résultats ?**

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# Etude Mayari: données non publiées(1)

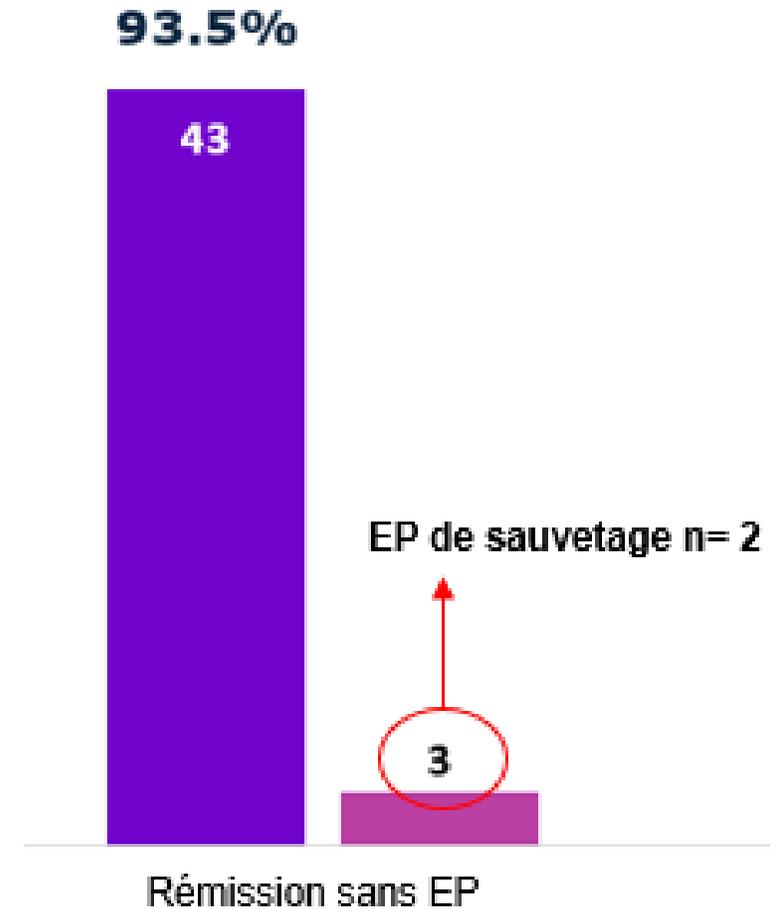
- étude internationale prospective (promoteur SANOFI) conduite sous la coordination du CNR-MAT en France.
- Objectif : évaluer la faisabilité et la sécurité d'une stratégie associant caplacizumab et immunosuppression, sans échanges plasmatiques en première intention.

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- Objectif : évaluer la faisabilité et la sécurité d'une stratégie associant caplacizumab et immunosuppression, sans échanges plasmatiques en première intention.
- Le protocole reposait sur l'administration précoce du caplacizumab dès la suspicion clinique de PTTi, associée à une immunosuppression classique, les échanges plasmatiques n'étant réservés qu'en cas d'absence de réponse rapide.
- exclusion des patients les plus graves
  - atteinte neurologique à type de coma ou de déficit focal
  - élévation de la troponine à  $> 5$  fois la valeur normale supérieure.

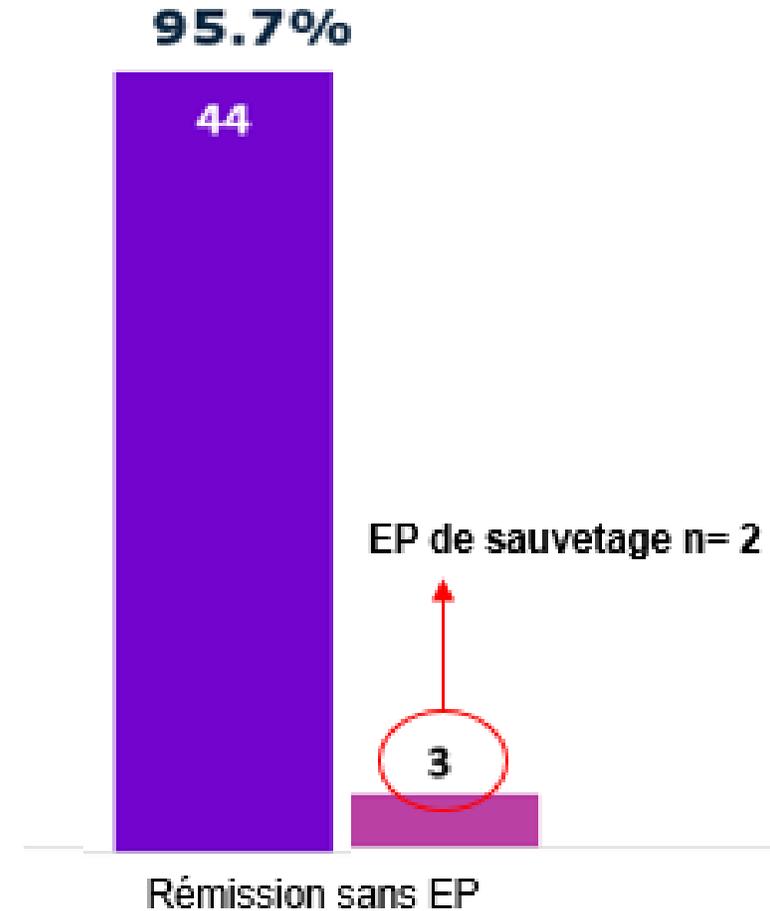
# Etude Mayari: données non publiées (2)

- 46 patients inclus
  - 9 français
- 45% récurrent, 55% inaugural
- tolérance satisfaisante

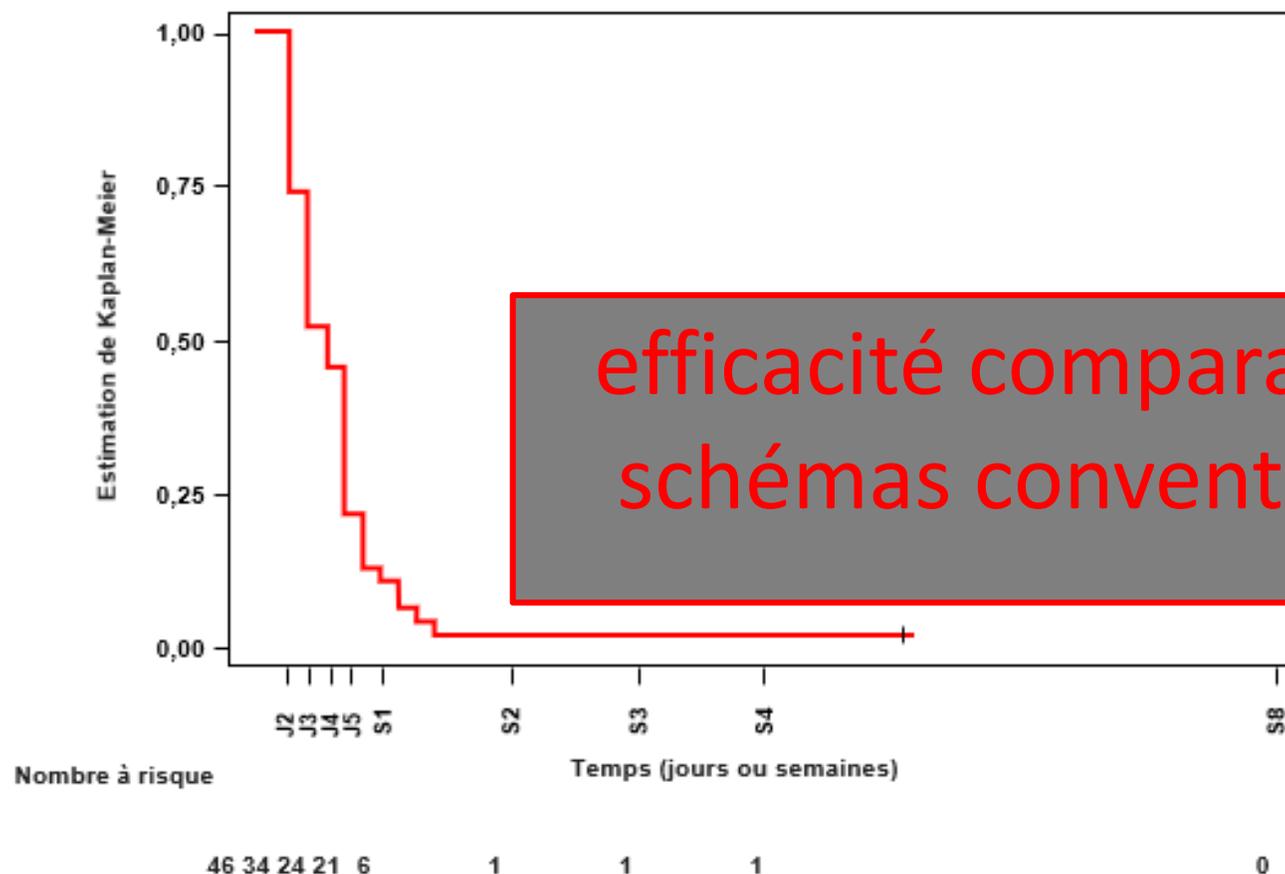


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# Etude Mayari: données non publiées (3)



efficacité comparable aux schémas conventionnels

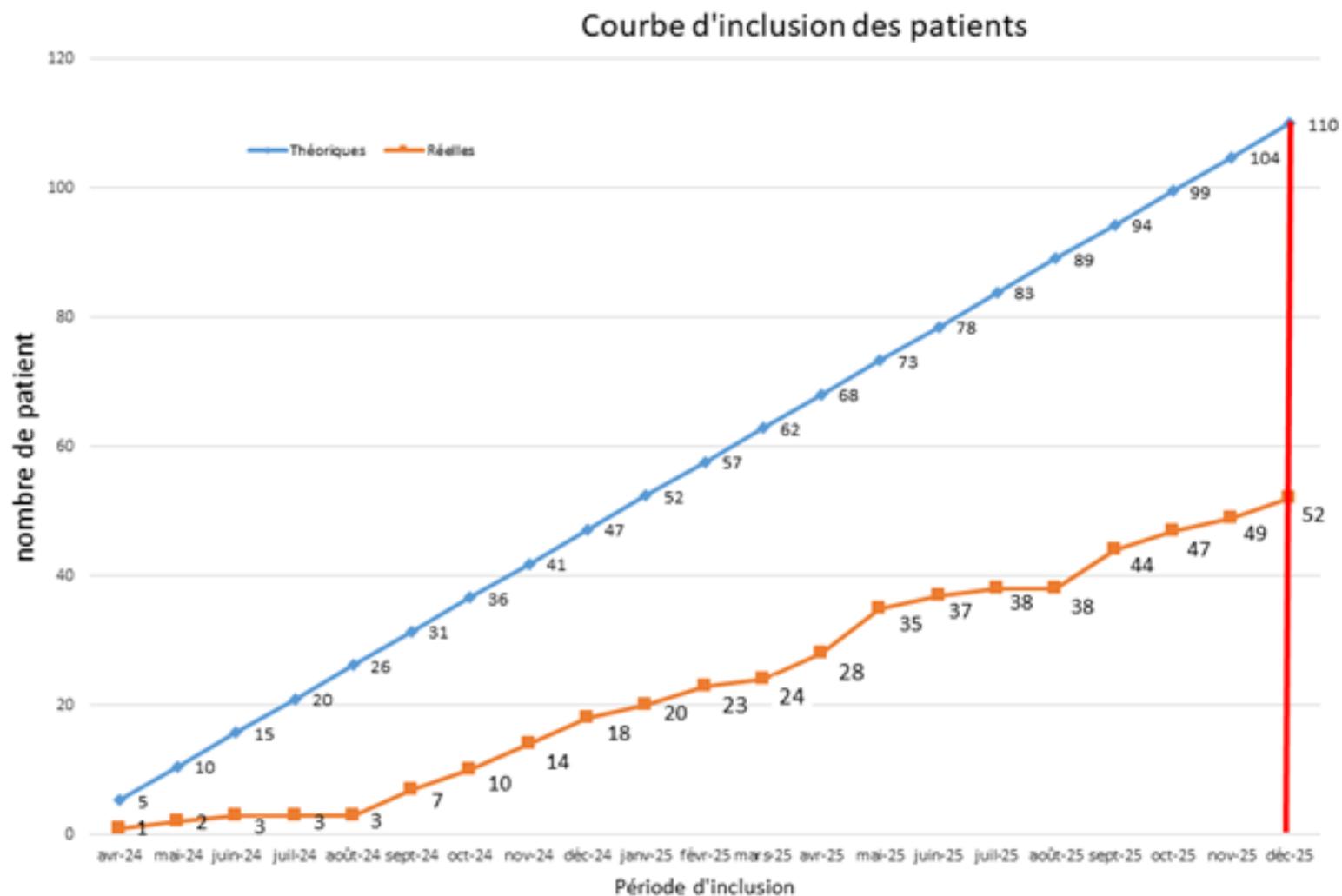
recupération plaquettaire rapide

- délai médian, 4 jours ;
- normalisation chez >75% des patients à J5 de la première dose de caplacizumab.

# Etude Pex free

- Etude Française prospective
- Objectif: évaluer efficacité régime associant CTC, Ritux, capla + perfusion plasma
- Exclusion forme neurologique grave, pas de critère cardiologique
  
- Schéma de l'étude:
  - 3 jours perfusion plasma 15ml/kg/j
  - Après 3j de tt:
    - Plaquettes X2 + baisse LDH + amélioration clinique → poursuite 10ml/kg/j
    - Sinon début EP

# Etude Pex free



Apport des nouvelles molécules:  
ADAMTS 13 recombinant

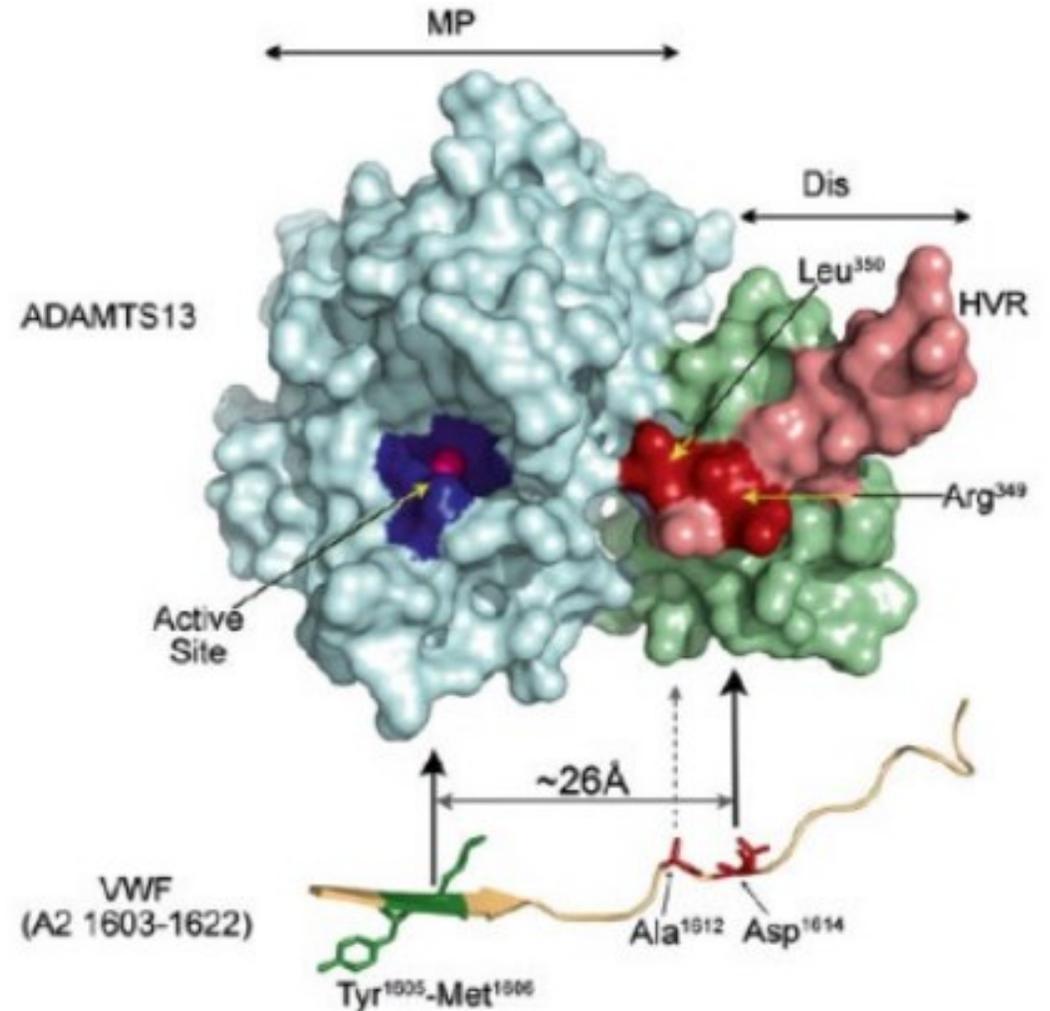
# Adamts 13r

- Fabriquée dans une lignée cellulaire génétiquement modifiée sans addition de protéine d'origine animale ou humaine

→ identique à l'ADAMTS 13 purifiée du plasma

- $\frac{1}{2}$  vie 59,2h
- Efficacité prouvée dans le PTTc

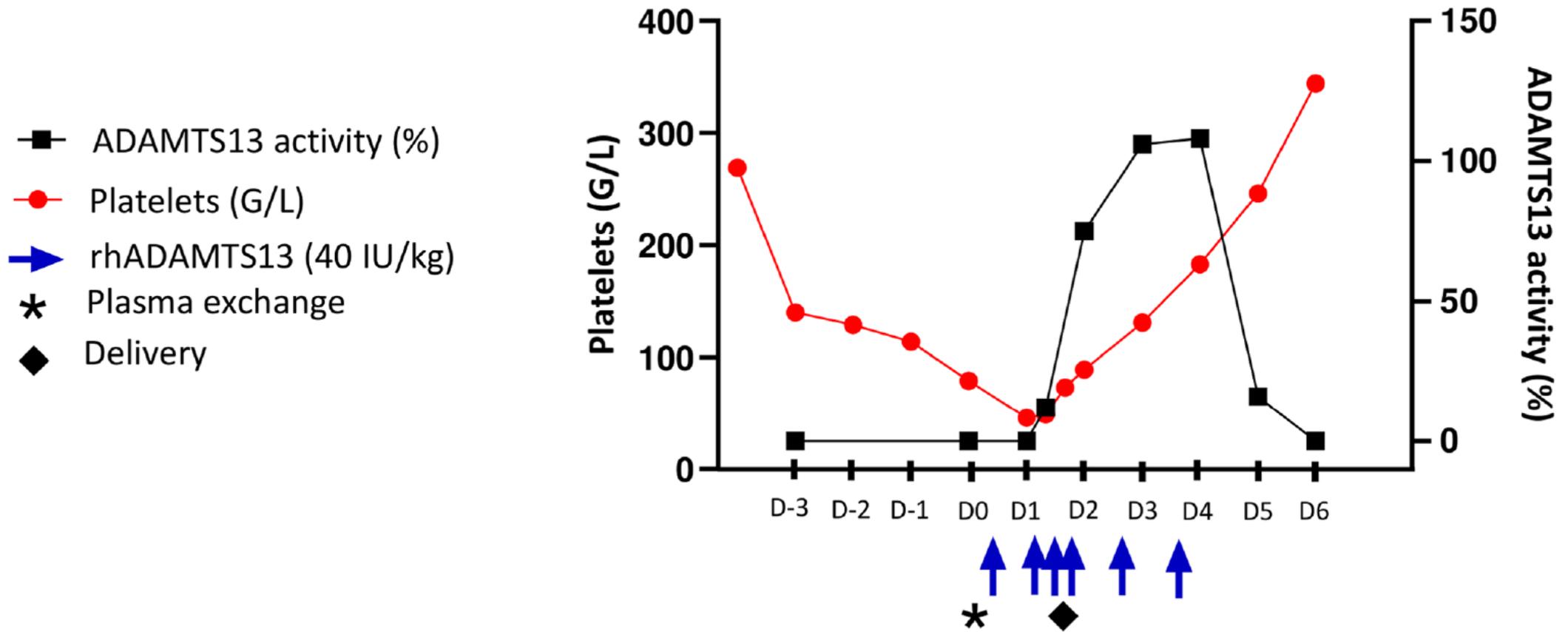
1. Foettinger-Vacha A et coll. GTH, 2013.
2. Zheng XL. Blood. 2009;113(22):5373-5374.



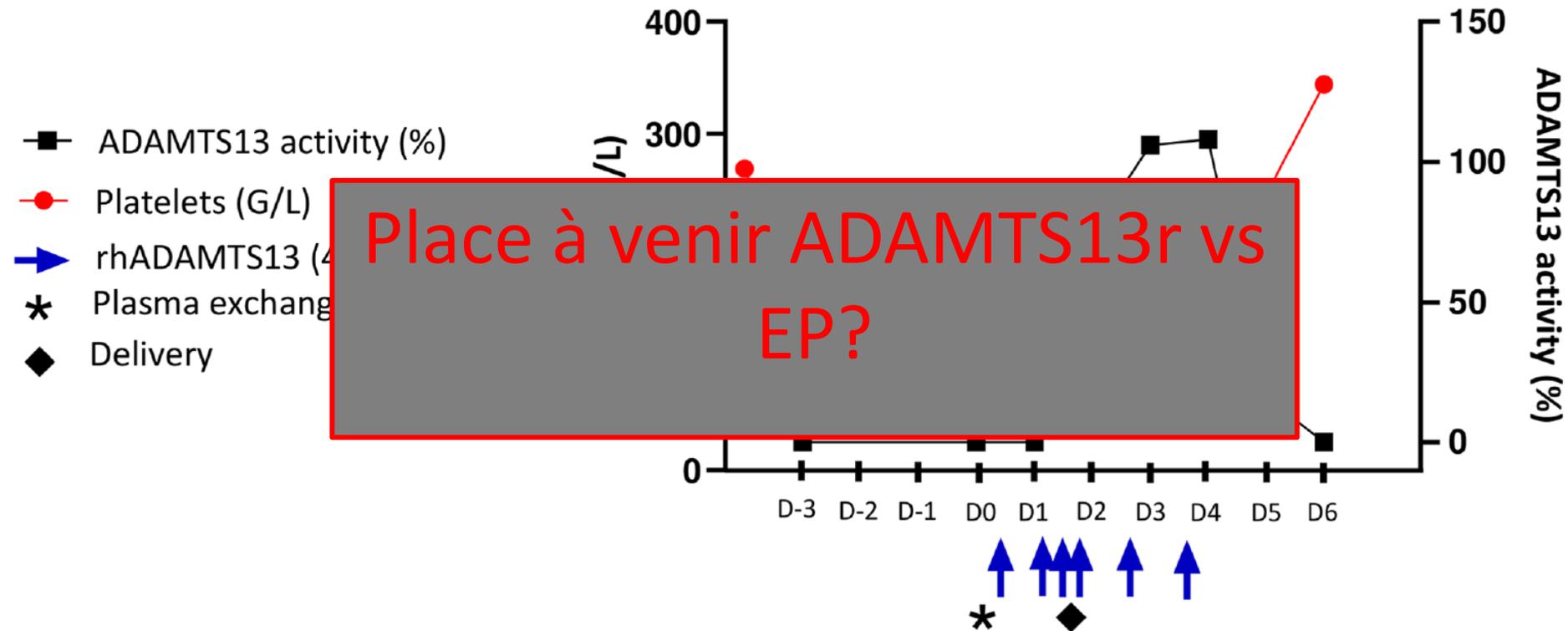
# Successful use of recombinant ADAMTS13 in a pregnant patient with immune-mediated thrombotic thrombocytopenic purpura

- Femme 36 ans
- 1ere poussée de PTTi il y'a 10 ans compliqué d'un AVC.
  - 23 EP pour obtenir rémission clinique
  - Persistance activité adamts 13 effondrée + anticorps élevés malgré multiples lignes d'IS
  - 33SA: plaquettes 46G/L + hémolyse méca
  - 1 EP → hypotension, vomissements
  - RCP MAT et grossesse: cADAMTS 13 40UI/kg X2/j après l'EP

# Successful use of recombinant ADAMTS13 in a pregnant patient with immune-mediated thrombotic thrombocytopenic purpura



# Successful use of recombinant ADAMTS13 in a pregnant patient with immune-mediated thrombotic thrombocytopenic purpura



Merci de votre attention



**Lucas Kühne et al, Management of immune thrombotic thrombocytopenic purpura without therapeutic plasma exchange, Blood, 2024, Figure 1.**

