



2ND 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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Daratumumab can lead to long-lasting remissions in patients with refractory immune thrombocytopenia but with a high incidence of severe infections

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1.9. Créteil (F), 2. Toulouse (F), 3. Dijon (F), 4. 11. Amiens (F), 5. La Roche-sur-Yon (F), 6.14.17. Paris (F), 7. Colmar (F), 8. Marseille (F), 10. Clermont-Ferrand (F), 12. Le Kremlin-Bicêtre (F), 13. Pessac (F), 15. Reims (F), 16. Strasbourg (F)



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Disclosure

No conflicts of interest to disclose



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Introduction

The number of therapeutic options for immune thrombocytopenia (ITP) is increasing, but **refractory disease remains an unmet need in clinical practice**

Patients with multi-refractory ITP are a **highly vulnerable population**¹

Long-lived autoreactive plasma cells may explain ITP persistence in some of these patients²

Anti-CD38 monoclonal antibodies such as **daratumumab** are a **promising treatment in ITP**³, but data regarding anti-CD38 use in patients with refractory ITP remain limited

¹ Mahévas et al., *Blood* 2016

² Mahévas et al., *JCI* 2013

³ Tsykunova et al., *Blood Advances* 2025

Aims of the study

Assess **safety** and **efficacy** of daratumumab given for refractory ITP by :

- **Evaluating safety profile** during follow-up
- Investigating the immediate, intermediate and long-term **efficacy outcomes**



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Methods

- **Observational, retrospective** and **multicenter** study
- Performed throughout the network of the French reference center for adult immune cytopenias (CERECAI)

Inclusion criteria

- Age > 18 years-old
- ITP (primary or secondary)
- Compassionate off-label treatment by daratumumab for ITP between 01/20 and 01/26

- Exclusion of patients if Daratumumab was given to treat plasma cell malignancy



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Methods - Definitions

- Response was assessed starting from **week 6 after the first infusion** using the following criteria :
- **Complete response (CR)** : platelet count >100 G/L
- **Response (R)** : platelet count 30 to $100 \times 10^9/L$ with at least a 2-fold increase from baseline
- **Overall Response (OR)** : CR + R

- **Concomitant treatment** : treatment initiated **6 weeks before or after** the 1st daratumumab infusion
- **Background therapy** : treatment initiated within **> 6 weeks before** the 1st daratumumab infusion



Results – population characteristics at first infusion

	Total (n = 26)
Median age [IQR] (min-max) – years-old	68 [41.0-72.8] (21.0-88.0)
Sexe – n (%)	
Male	15 (57.7)
Female	11 (42.3)
Median ITP duration [IQR] (min-max) - months	76 [23.8-140.5] (1.0-594.0)
Secondary ITP – n (%)	11 (42.3)
Evans syndrome	6* (23.1)
Antiphospholipid syndrome	3* (11.5)
Rheumatoid arthritis	1 (3.8)
STIM1 mutation	1 (3.8)
Post allogeneic HSCT for myelodysplastic syndrome	1 (3.8)

* One patient had APS and Evans syndrome



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Results

Median number of ITP treatment lines (excluding corticosteroids and IVIg) [IQR] (min-max)	6 [4-7] (2-11)
Corticosteroids – n (%)	26 (100)
Intravenous immunoglobulins – n (%)	25 (96.2)
Eltrombopag – n (%)	24 (93.3)
Romiplostim – n (%)	23 (88.5)
Rituximab – n (%)	25 (96.1)
Dapsone – n (%)	7 (26.9)
Hydroxychloroquine – n (%)	4 (15.4)
Splenectomy – n (%)	16 (61.5)
Fostamatinib – n (%)	13 (50)
Mycophenolate mofetil – n (%)	19 (73.1)
Ciclosporin – n (%)	10 (38.5)
Azathioprine – n (%)	4 (15.9)
Sirolimus – n (%)	3 (11.5)
Obinutuzumab – n (%)	1 (3.8)
Cyclophosphamide – n (%)	1 (3.8)
Efgartigimod – n (%)	1 (3.8)
Corticosteroid-refractory - n (%)	17 (65.4)
IVIg-refractory - n (%)	15 (57.7)
Bleeding during the preceding month - n (%)	17 (65.4)
Median platelets count at baseline (Day 0) [IQR] (min-max)	14 [7.0-51.8] (2-294)



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Results

Median number of 6 (2-11) daratumumab infusions

- 16 (62 %) SC 1800 mg
- 10 (38 %) IV 16 mg/kg

Median duration of follow-up after first daratumumab infusion : 14 months [0-73]



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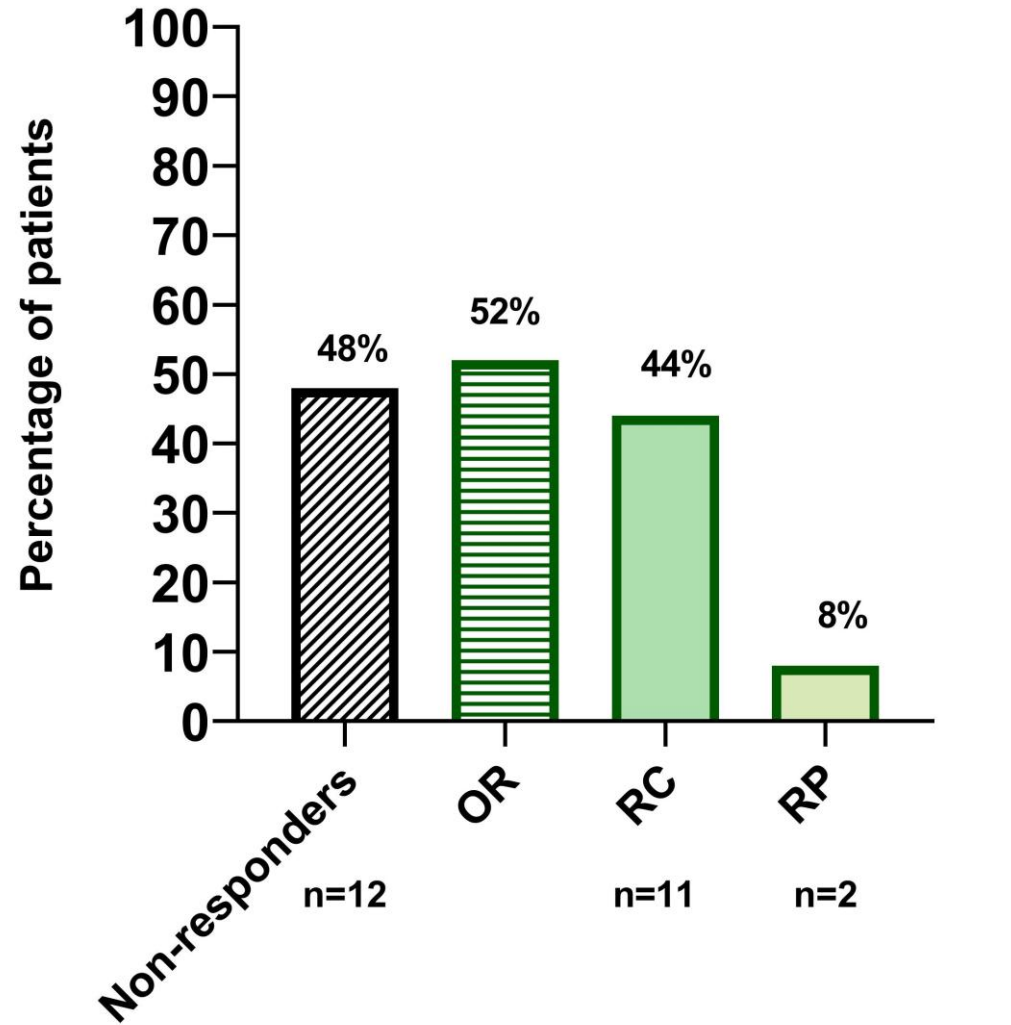
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Results – Response at 3 months

Median number of 6 (2-11) daratumumab infusions

- 16 (62 %) SC 1800 mg
- 10 (38 %) IV 16 mg/kg

Median duration of follow-up after first daratumumab infusion : 14 months [0-73]



n = 25 patients

1 patient excluded (death 3 weeks after 1st daratumumab infusion)



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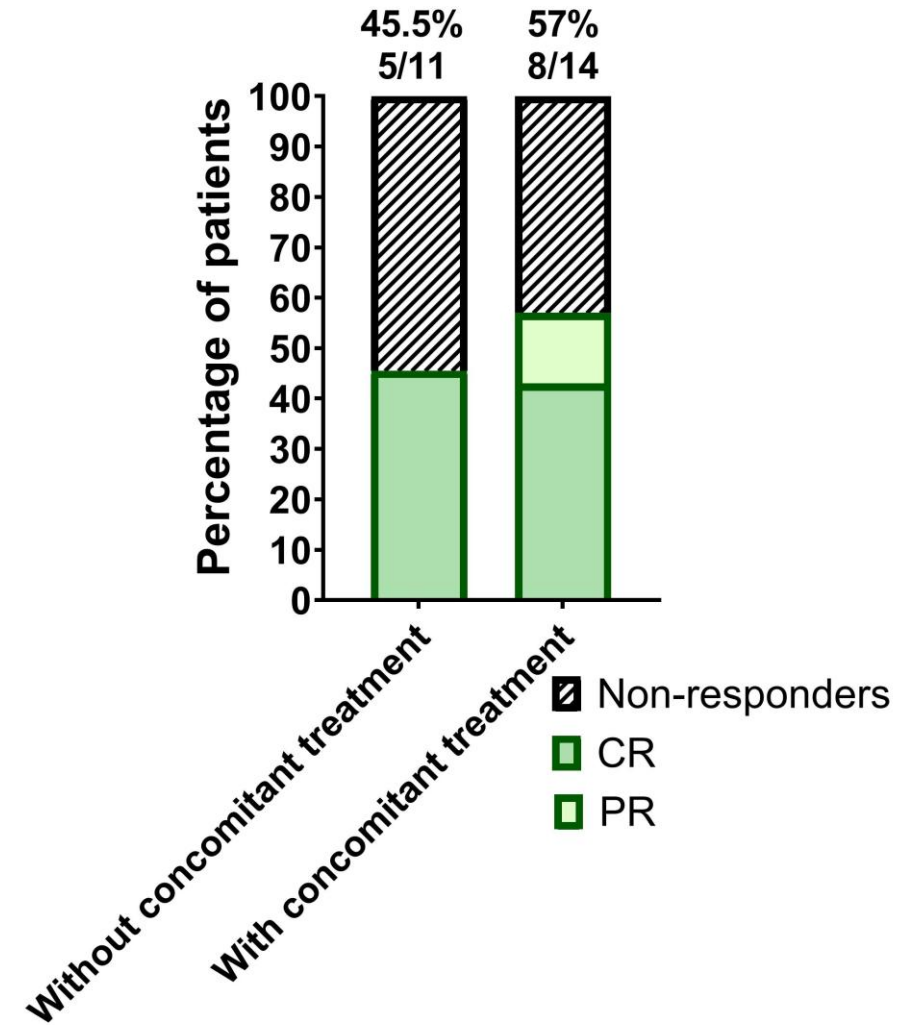
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- 16 (62 %) SC 1800 mg
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2 patients received neither maintenance therapy nor concomitant treatment, and both achieved CR



n = 25 patients

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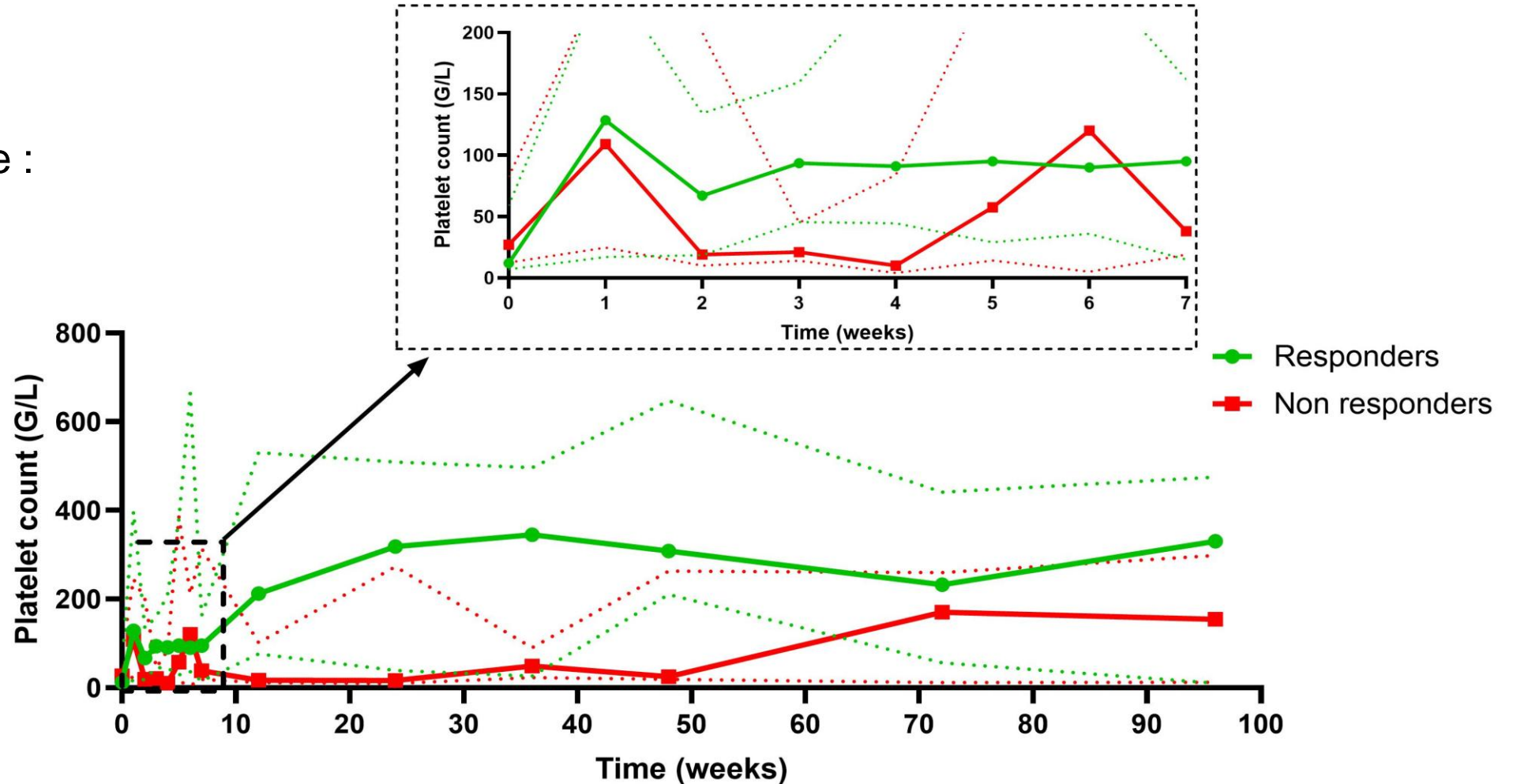
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Efficacy – Platelet counts

Median time to response :
29 days (range 7- 87)



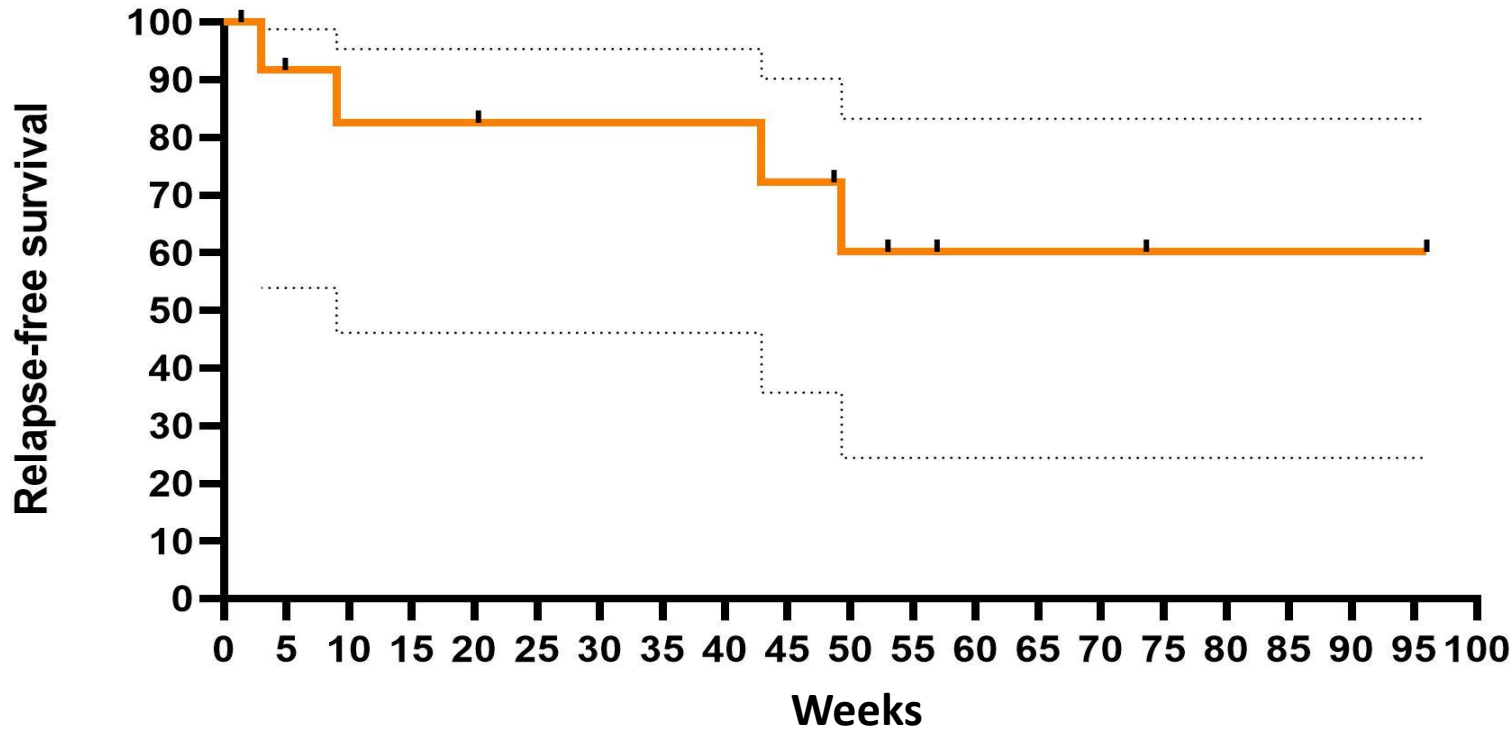
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Efficacy – Relapses in responders



Relapse-free survival at 24 months was **60 %**

4 patients had **long-lasting CR** without any other ITP treatment, with relapses in 2 (50%) after 10 and 32 months, respectively

Time (weeks)	0	1.4	3.0	4.9	9.0	20.3	42.9	48.7	49.3	53	56.9	73.6	96
n	13	13	12	11	10	9	8	7	6	5	4	3	2



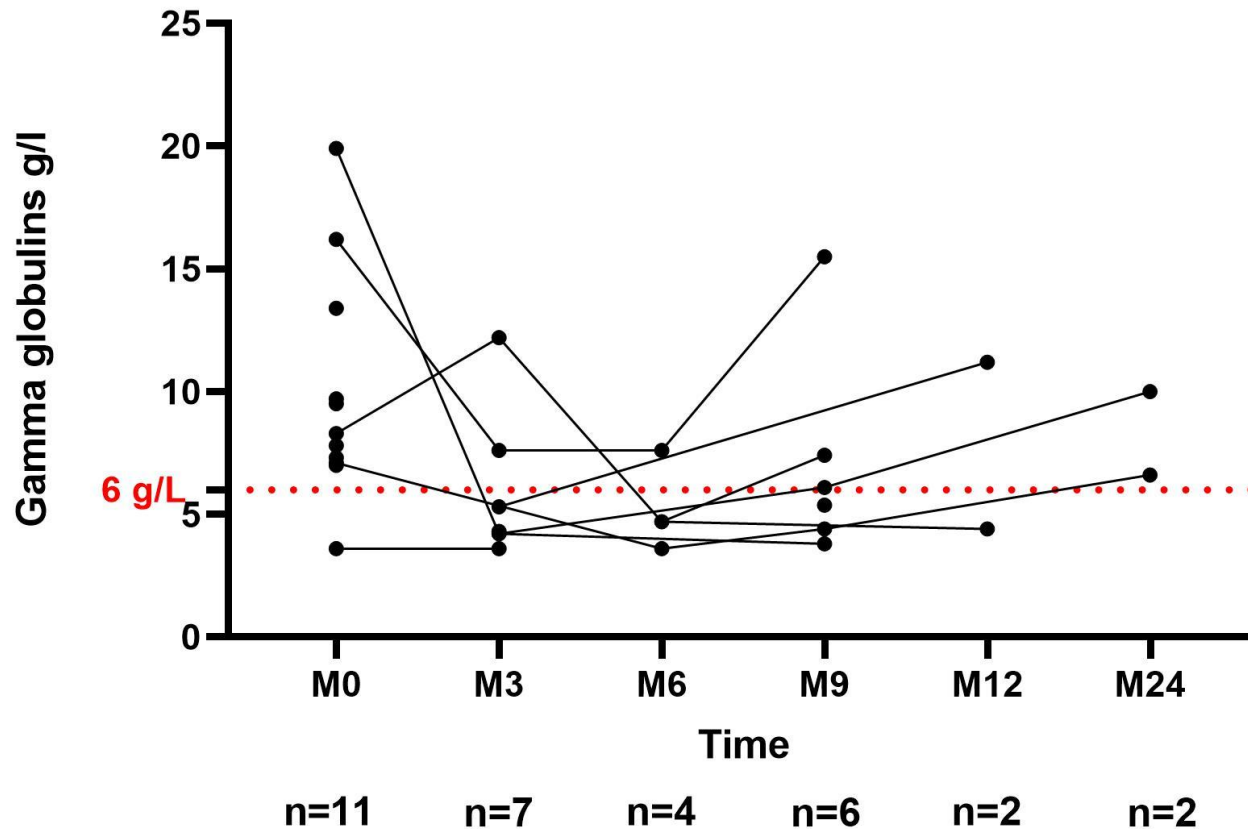
Safety

Adverse events (AE) occurred in 16 patients (62%)

- **8 patients (31%) had infectious AEs**, including 5 patients with grade 3–5 AEs
- **Other AEs included** 3 neutropenias, 3 infusion-related reactions, and 1 heart failure
- **6 patients (23%) died** : 2 from sepsis, 1 from heart failure, and 3 from hemorrhagic syndrome

Safety – gammaglobulins levels

56% of patients had gammaglobulins < 6 g/L during follow-up



Discussion – study limitations

- **Retrospective** and **observational** study design with **small sample size** (n = 26)
- **Premedication with dexamethasone** may have contributed to the observed responses (but 65% were refractory to corticosteroids)
- We plan to include additional patients from a german cohort

Ongoing European collaboration:

Dr Julian List

Dr Tim Strüßmann

Dr Reinhard Marks



Department of Hematology, Oncology and Stem Cell Transplantation, University of Freiburg Medical Center, Freiburg, Germany

Conclusions

In this retrospective study, overall response rate was 52% in patients with refractory ITP, and relapse-free survival probability at 24 months was 60% in responders

Adverse events were common, in particular infections, likely reflecting the greater frailty of our study population (more heavily pre-treated than in DART study)

- The optimal strategy regarding **daratumumab use in refractory ITP** remains to be determined
- Optimal **anti-infective prophylaxis strategies** still need to be established
- There is a need for additional studies focusing on clinical or biological biomarkers to **identify responder patients**

Acknowledgements



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