2ND meeting of the European Research Consortium on ITP

NEW INSIGHTS INTO IMMUNE THROMBOCYTOPENIA

Paris Crowne Plaza Paris République

April 23-24, 2026

What's new in Childhood ITP - What are the Unmet Needs?

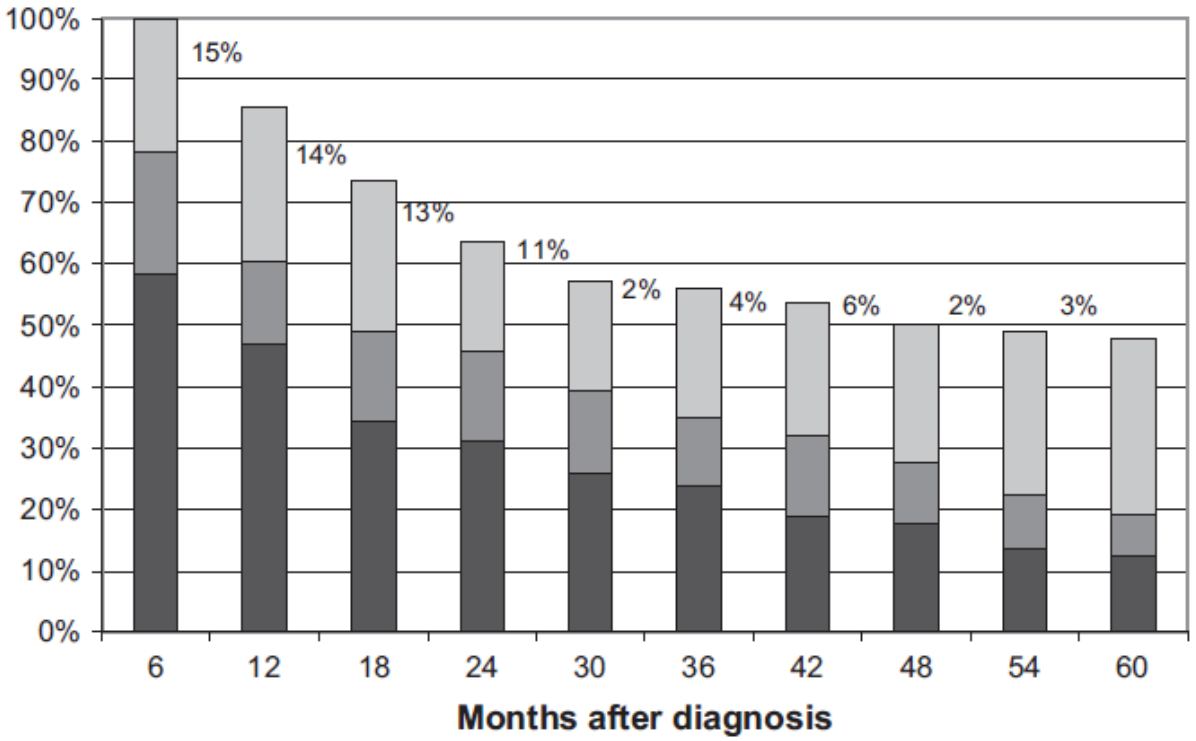
Thomas Kühne
University Children's Hospital Basel, Switzerland

Disclosures of
Thomas Kühne

Company name	Research support	Employee	Consultant	Stockholder	Speakers bureau	Advisory board	Other
Argenx						x	
Sobi			x				
Takeda						x	

Chronic ITP in Children

Recovery from thrombocytopenia in 96 children with continuing thrombocytopenia 6 months after diagnosis



Severe thrombocytopenia (<20x10^9/L, black columns)
Moderate thrombocytopenia (>20x10^9/L, dark grey columns)
Mild thrombocytopenia (>50x10^9/L, light grey columns)

This 5-year follow-up of a cohort of 96 children with ITP lasting >6 months indicates that:

- Spontaneous recovery can be expected in half
- Events requiring hospitalization will occur in less than half
- Serious bleeding episodes while the platelet count is <20x10^9/L will occur in <10%

«Adult-type ITP»

Rosthøj S et al. Acta Pædiatrica 2012;101:761–766

Children not responding to 2nd-Line Treatment

… who have no response to first TPO-RA or who lose an initial response

→ Switch to another TPO-RA
→ Combine TPO-RA with MMF or another immunosuppressant

… who do not respond to TPO-RA
→ Consider rituximab and dexamethasone, especially for adolescent females

Oved JH, Lee CSY, Bussel JB. J Pediatr 2017;191:225-231

Review, based on 4 evidence-based guidelines
2018 European guidelines (D, A, C, H) Matzdorff A et al. Oncol Res Treat. 2018;41 Suppl 5:1-30
2019 ASH guidelines: Neunert C et al. Blood Adv 2019;3(23):3829-3866
2019 Updated International Consensus Report Provan D et al. Blood Adv 2019;3(22):3780-3817
2021 Chinese guidelines Wu R et al. Pediatr Investig 2022;6(2):63–74

Criteria	Description
Population	Pediatric patients (< 18 years of age) with immune thrombocytopenia
Intervention	Q1: Dapsone Q2: Rituximab Q3: Thrombopoietin receptor agonists (e.g., romiplostim, eltrombopag)
Comparator	Not applicable
Outcomes	Q1: Recommendations regarding the use of dapsone for pediatric patients with immune thrombocytopenia Q2: Recommendations regarding the use of rituximab for pediatric patients with immune thrombocytopenia Q3: Recommendations regarding the use of thrombopoietin receptor agonists for pediatric patients with immune thrombocytopenia
Study designs	Evidence-based guidelines

Hui D et al. Canadian Agency for Drugs and Technologies in Health; 2022

2019 ASH Guidelines

Questions 14-18: In children with newly diagnosed ITP who have non-life-threatening bleeding and/or diminished HRQoL, the ASH guideline panel …

Questions 19-21: In children with ITP who have non-life-threatening mucosal bleeding and/or diminished HRQoL and do not respond to first-line treatment, the ASH guideline panel …

Question	Recommendation/Suggestion
14	…recommends against courses of corticosteroids longer than 7 days rather than courses 7 days or shorter
15	…suggests prednisone (2-4mg/kg per day; maximum, 120 mg daily, for 5-7 days) rather than dexamethasone (0.6 mg/kg per day; maximum, 40 mg daily, for 4 days)
16	…suggests corticosteroids rather than anti-D immunoglobulin
17	…suggests either anti-D immunoglobulin or IVIG
18	…suggests corticosteroids rather than IVIG
19	…suggests TPO-RAs rather than rituximab
20	…suggests TPO-RAs rather than splenectomy
21	…suggests rituximab rather than splenectomy

Neunert C et al. Blood Adv 2019;3(23):3829-3866

Quality of Life and Pediatric ITP

- Activities, sports, and limitations
- Cognition
- Fatigue
- Mental health (anxiety, continuous tension, stress, depression)

Quality of Life and Pediatric ITP

- Activities, sports, and limitations
- Cognition
- Fatigue
- Mental health (anxiety, continuous tension, stress, depression)

- *ICON Survey of 278 pediatric hematologists/oncologists regarding how children with ITP are counseled for participation in sports*
- *Substantial variation in physician perception of contact risk for different sports, and advice offered about restriction of sport activities*
- *"…that broad guidelines from an expert international panel might help reduce physician, patient, and parent anxiety, especially for popular moderate-risk and low-risk sports, and allow for more liberal sports participation, and therefore improved quality of life for our patients and their parents."*

ICON, ITP Consortium of North America

Kumar M et al. Pediatric blood & cancer 2015;62(12):2223-2225

Quality of Life and Pediatric ITP

- Activities, sports, and limitations
- Cognition
- Fatigue
- Mental health (anxiety, continuous tension, stress, depression)

- *From the baseline assessment (prior to treatment initiation) of participants enrolled in phase 1/2 LUNA 2 study*
- *49 adults with relapsed/refractory ITP*
- *"The severity of cognitive impairment was comparable to mild traumatic brain injury ...*
  Overall, these results warrant a clinical need to further consider the potential of cognitive dysfunction in assessing ITP patients."

Kuter DJ al. Br J Hematol 2024; 205(1):291-299

Quality of Life and Pediatric ITP

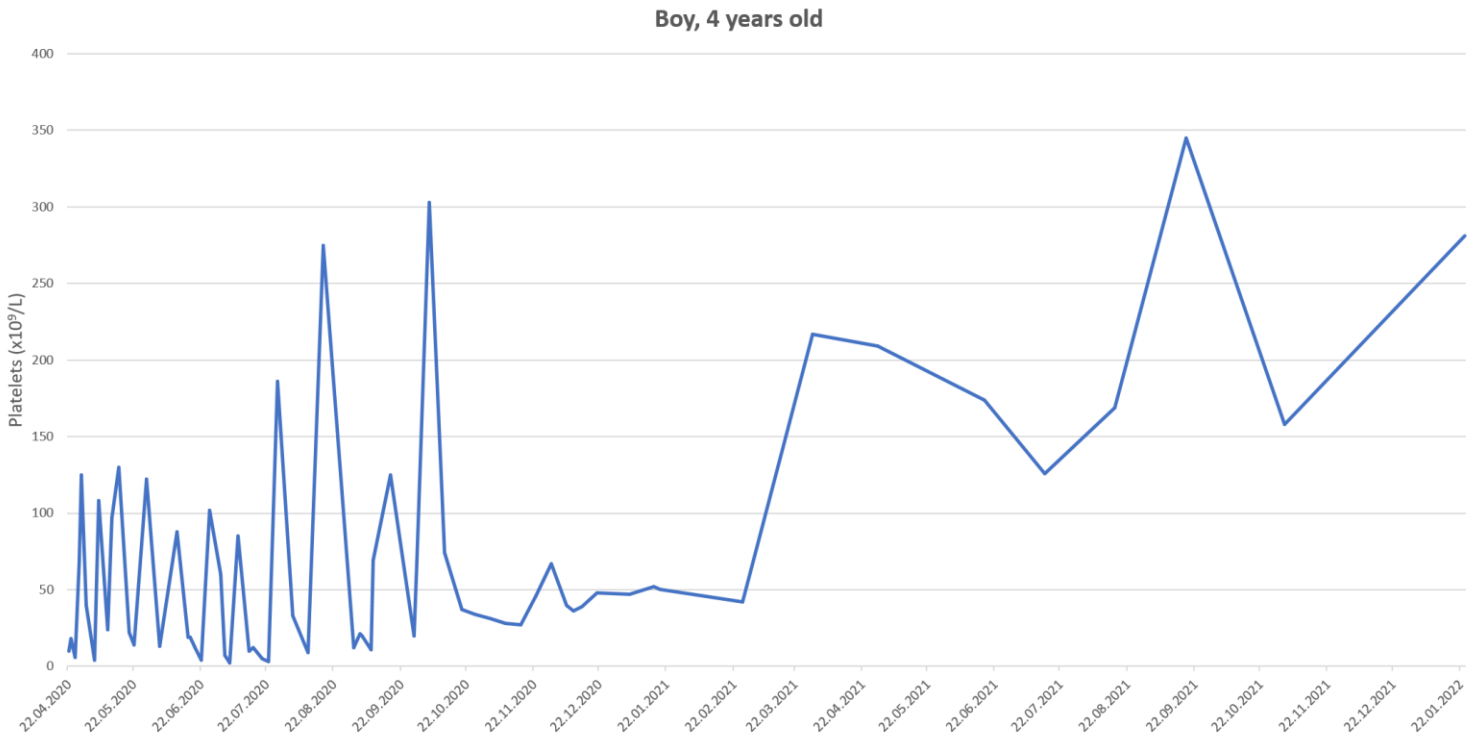
- Activities, sports, and limitations
- Cognition
- Fatigue
- Mental health (anxiety, continuous tension, stress, depression)

"Fatigue is common in children and adolescents with ITP and may benefit from ITP-directed treatment even in the absence of bleeding symptoms."

Grace RF et al. Br J Haematol 2020; 191(1):98-106

Quality of Life and Pediatric ITP

- Activities, sports, and limitations
- Cognition
- Fatigue
- Mental health (anxiety, continuous tension, stress, depression)



Unmet Needs for Children with ITP (incomplete list)

- «Adult-type ITP» in children and adolescents
- Novel treatments: Availability for children and adolescents
  - with newly diagnosed ITP
  - who do not respond to first-line/second-line treatments
- Prediction of persistent and chronic ITP

  Hypothesis: Patients at risk of developing chronic ITP may benefit from early use of platelet-enhancing therapies to prevent chronic ITP
- Health-related quality of life of children and adolescents

Menstrual Bleeding in Adolescents and Young Adult (AYAs) Females with Chronic Immune Thrombocytopenia

CARMEN-France (Guillaume Moulis)
OBS'CEREVANCE (Nathalie Aladjidi)
PARC-ITP Registry (Alexandra Schifferli, Thomas Kühne)

PD Dr. med. Alexandra Schifferli
Hematology/Oncology Department
University Children's Hospital Basel
Switzerland

Disclosures of

Alexandra (Name) Schifferli (Surname)

Company name	Research support	Employee	Consultant	Stockholder	Speakers bureau	Advisory board	Other
Novartis	X						
Sobi			X			X	
Amgen			X				
Basilea	x						
Novartis	X						

Heavy Menstrual Bleeding (HMB) in ITP

- HMB is common in women with bleeding disorder[1]
- **Up to 78% of women with ITP affected[2]**
  - 39% with *Pictorial Blood Assessment Chart* (PBAC) ≥100
  - **Not solely driven by platelet count**
    persists even at >50 × 10⁹/L
  - **Relevant clinical burden**
    ↓ quality of life, ↑ fatigue

➢ Aim:

To characterize females with chronic ITP and HMB in the AYA cohort

1: James AH. Women and bleeding disorders. Haemophilia. 2010;16(s5):160–7.
2 Menstrual Problems in Chronic Immune Thrombocytopenia: A monthly Challenge - van Dijk WEM, et al. British Journal of Haematology. 2022;198(4):753-764.

Female AYAs with Chronic ITP – Cohort Overview

- Multicenter registry analysis

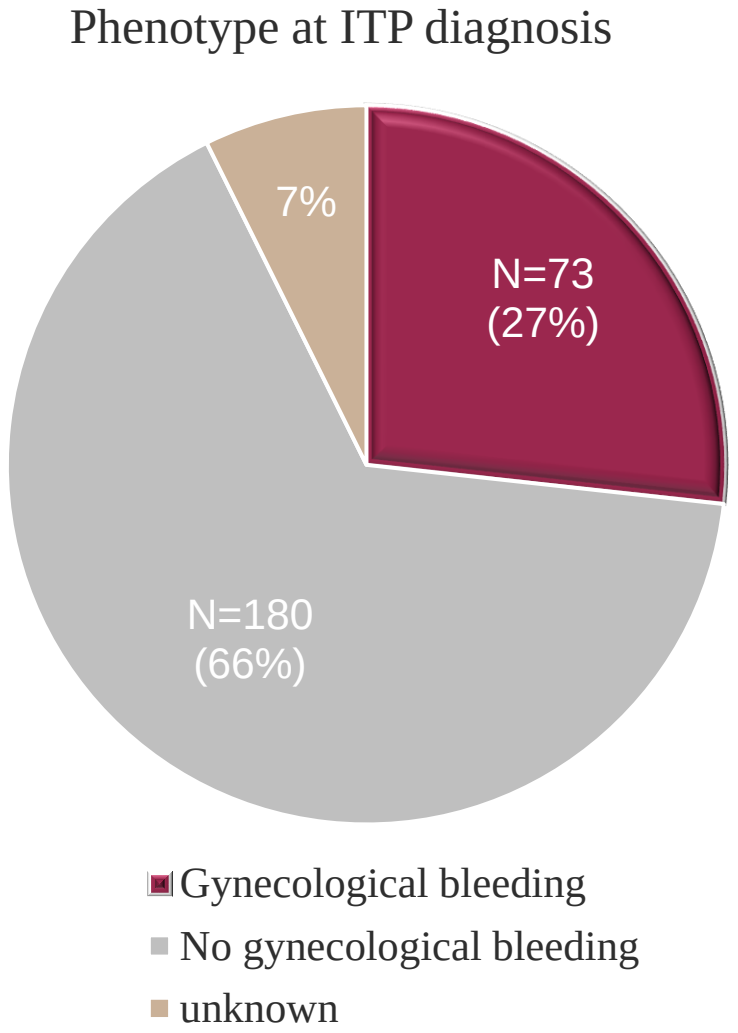
➢ *PARC-ITP, OBS'CEREVANCE, CARMEN-France*

- Definitions

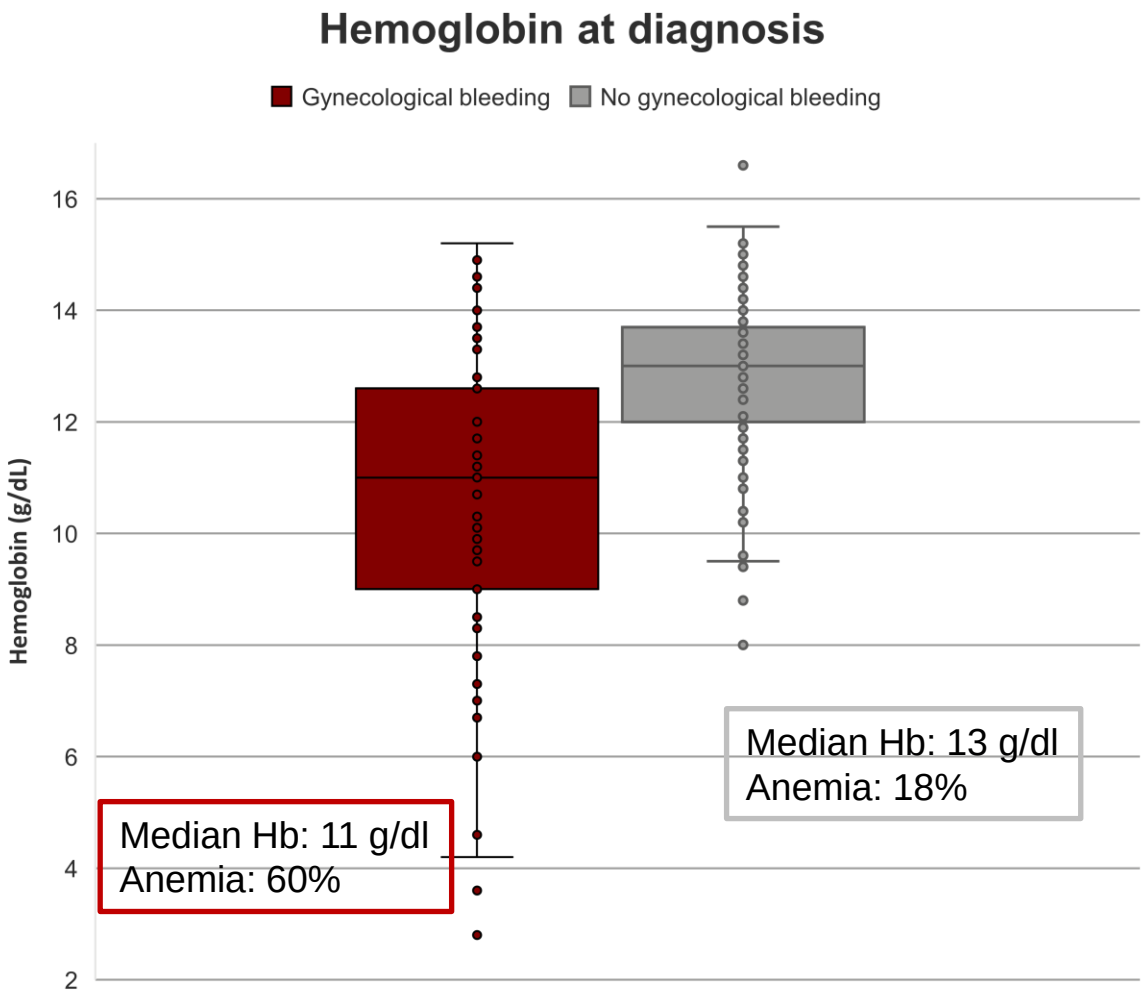
➢ AYAs = 12 – 25 years

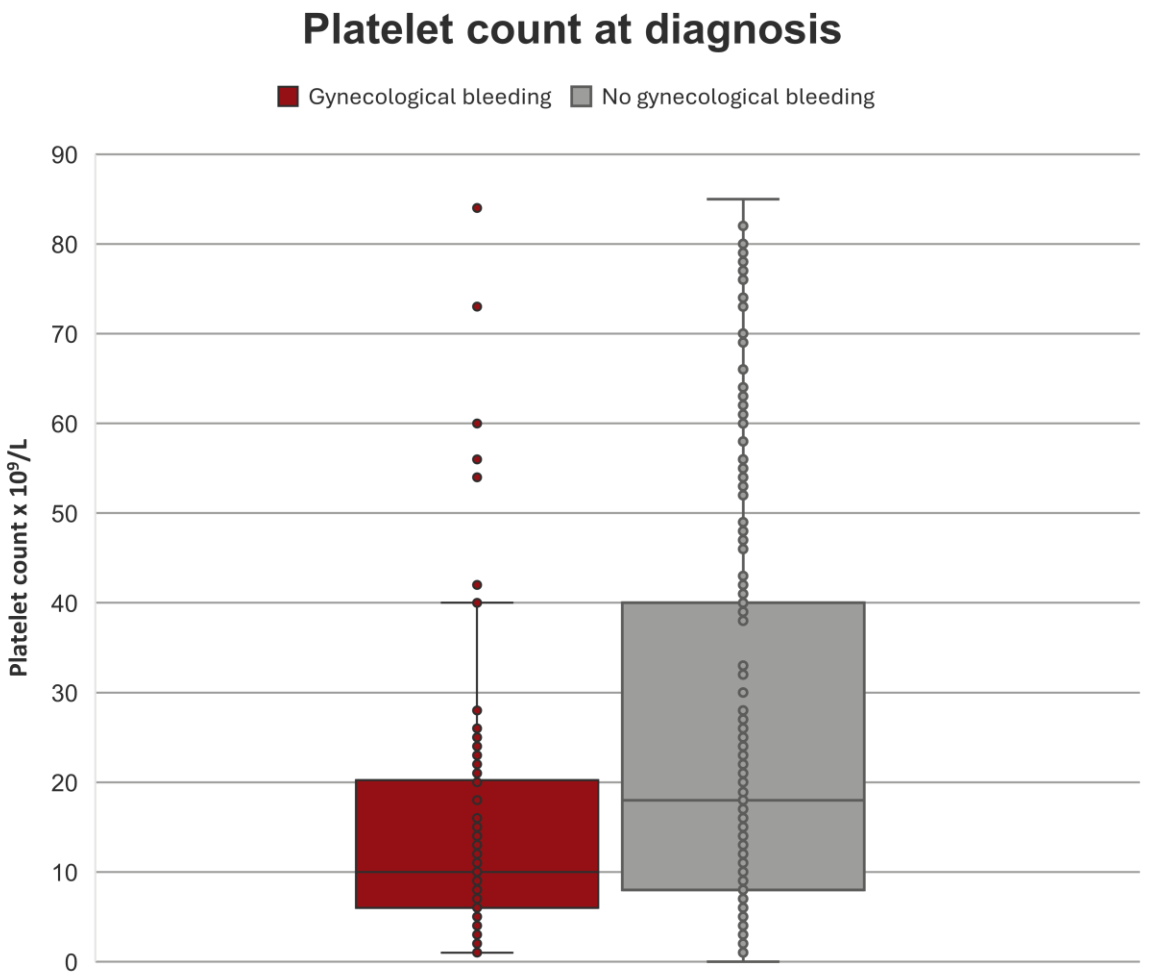
➢ chronic ITP: platelet count <100 ×10⁹/L
or ongoing treatment at 12 months

n = 273 female AYAs, mean age 14.8 years (SD 2.9)
→ 1 in 4 females presents with heavy menstrual bleeding at initial diagnosis

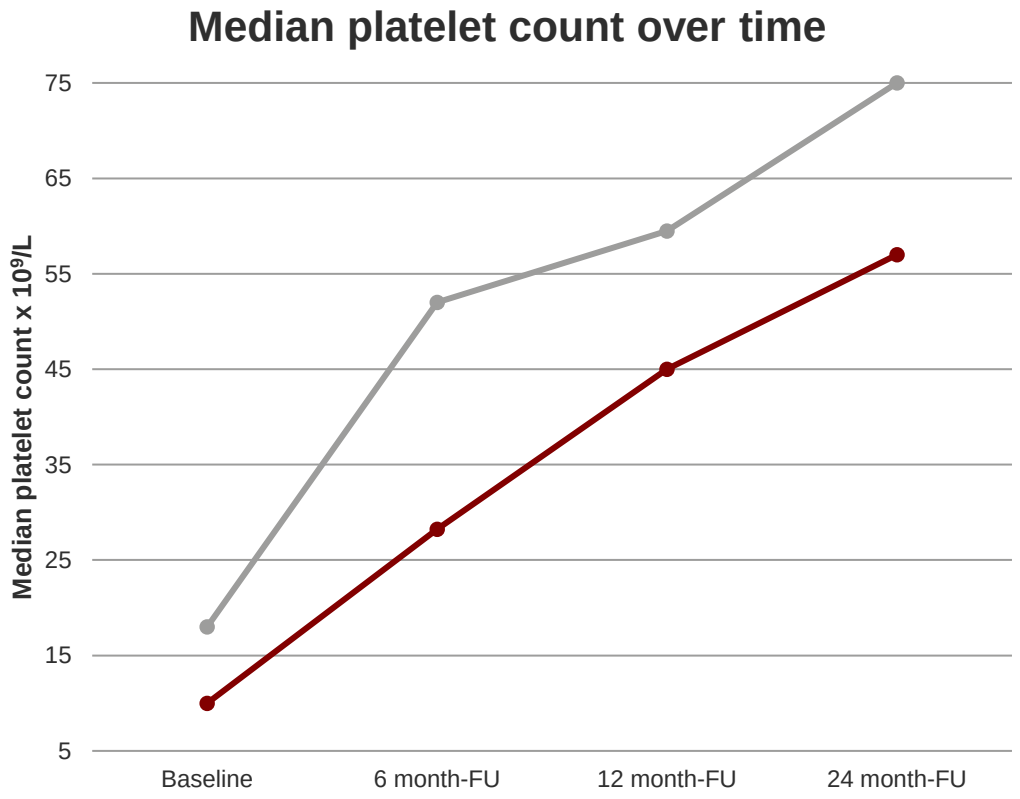


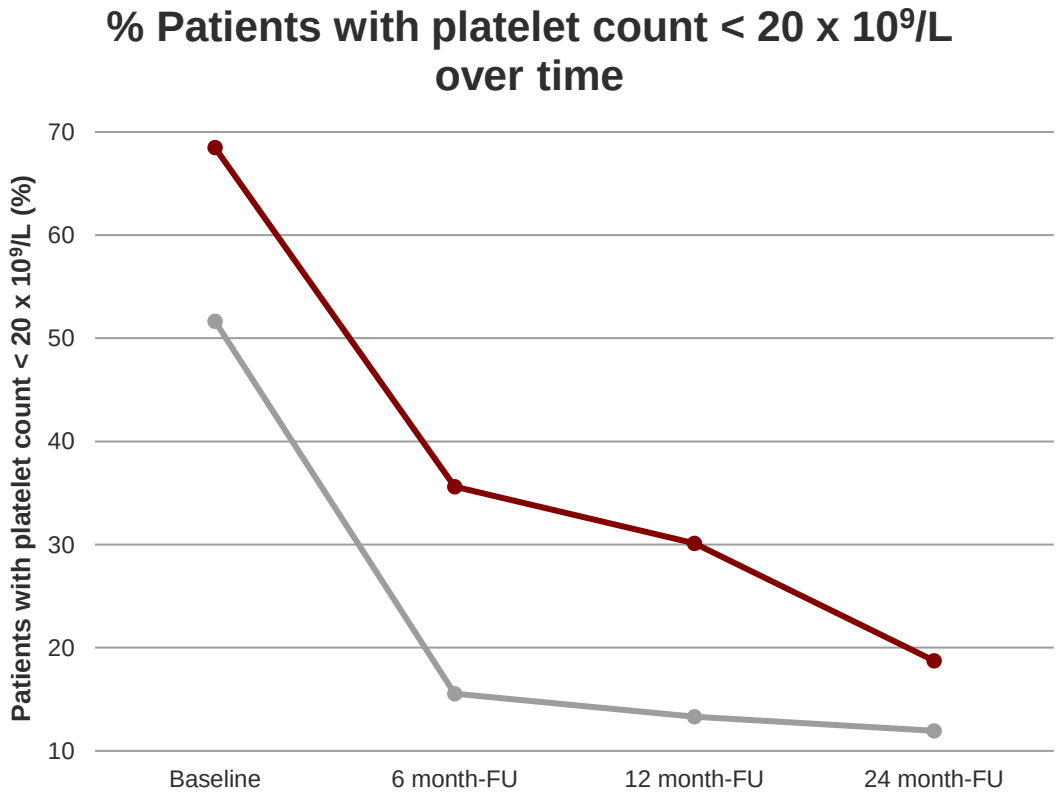
Initial Characteristica





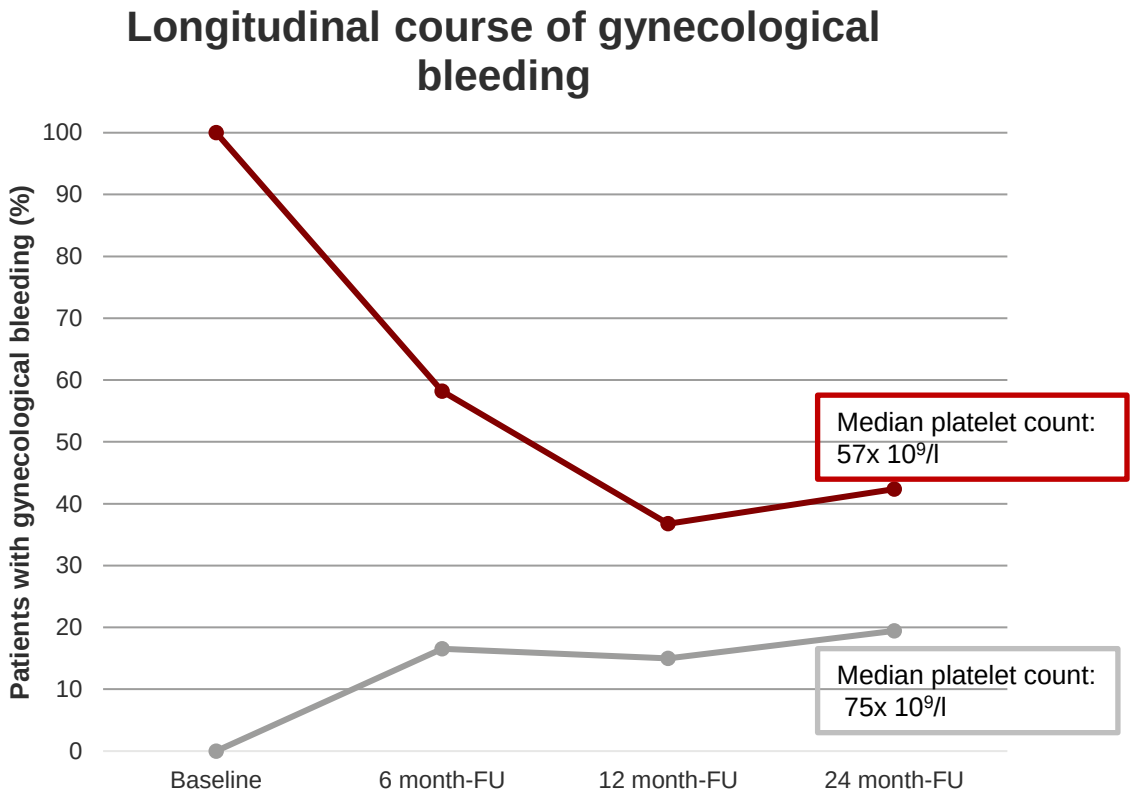
Differences persist over time: ▶ Platelet Count

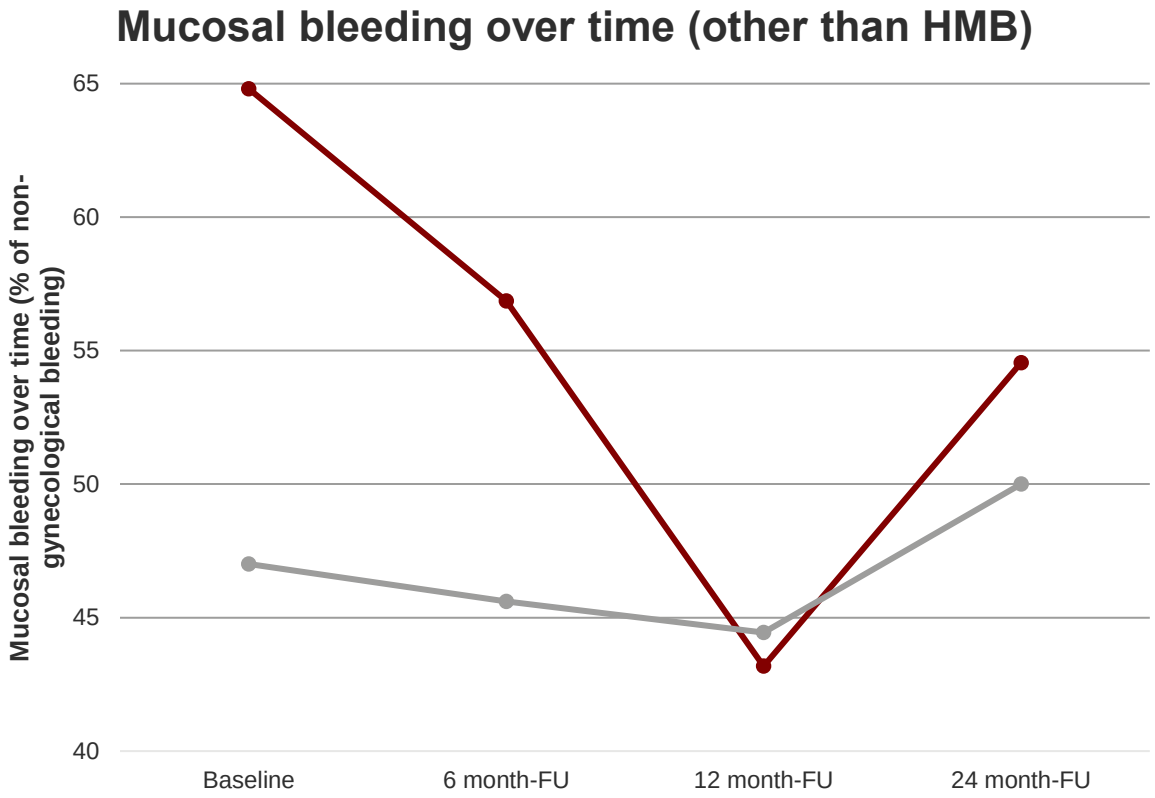




…this is not only a transient feature, but reflects a more persistent disease pattern

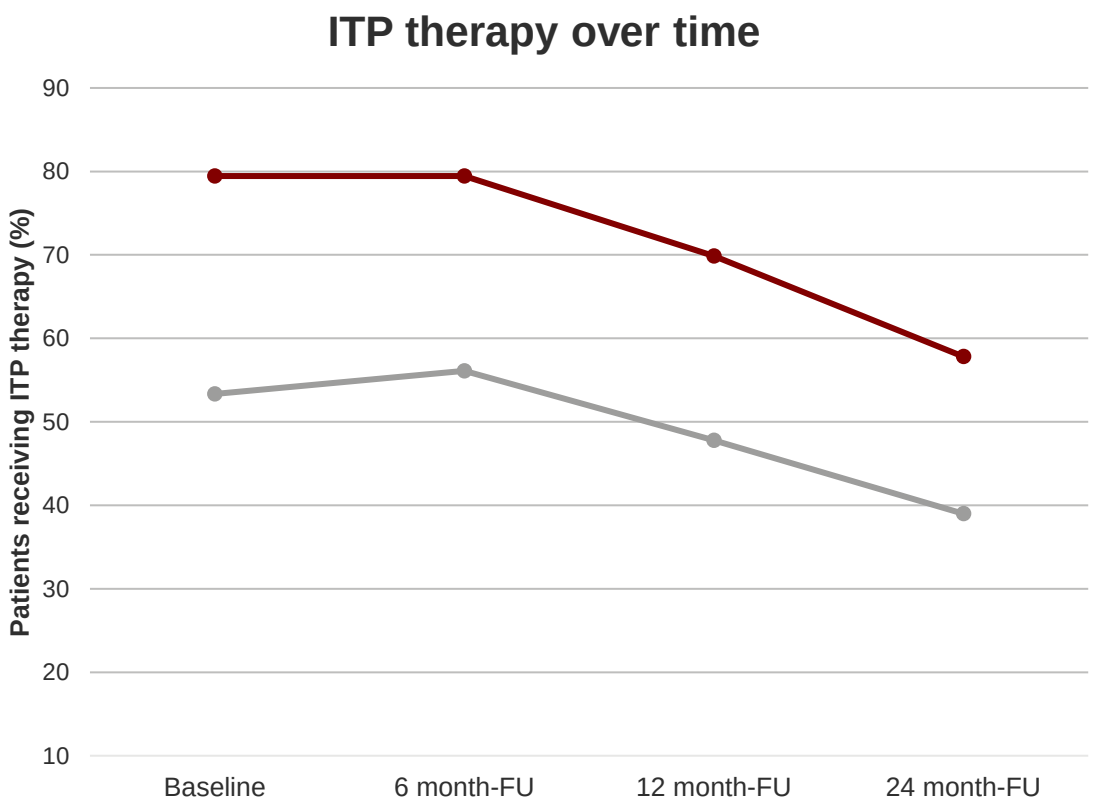
Differences persist over time: ▶ Bleeding

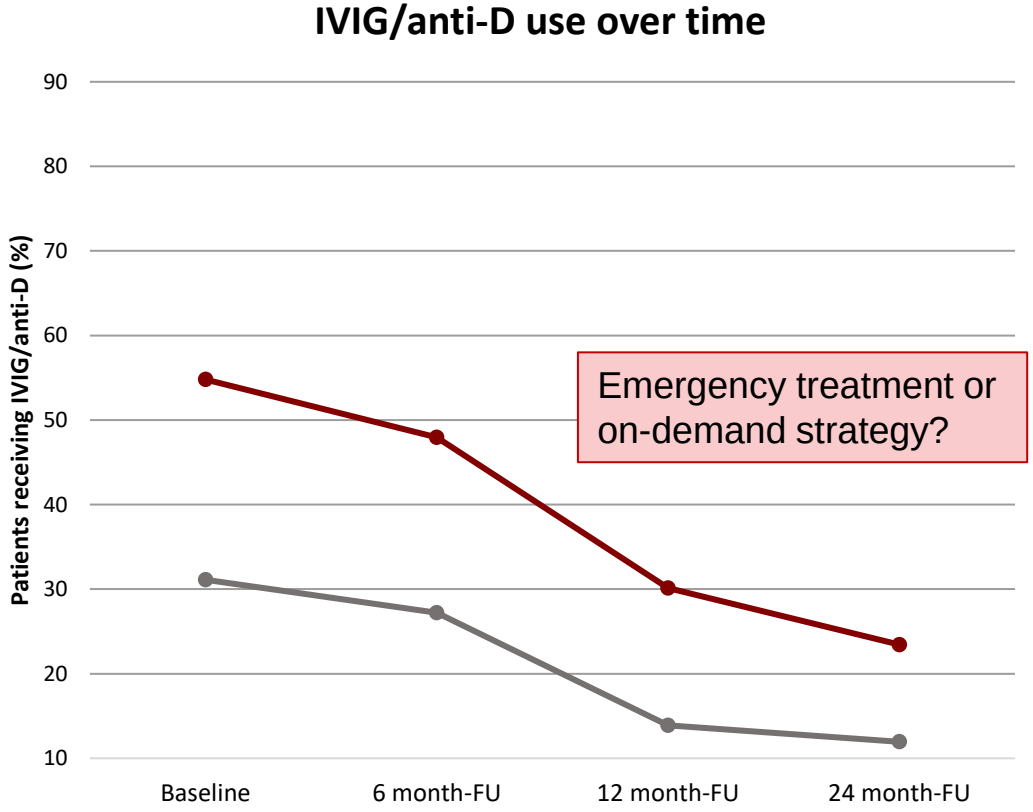


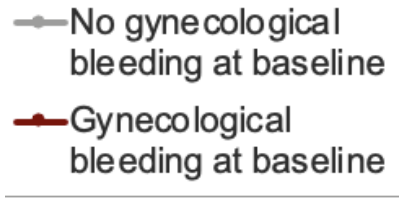


…overall women with HMB at initial diagnosis seem to present a more severe disease course

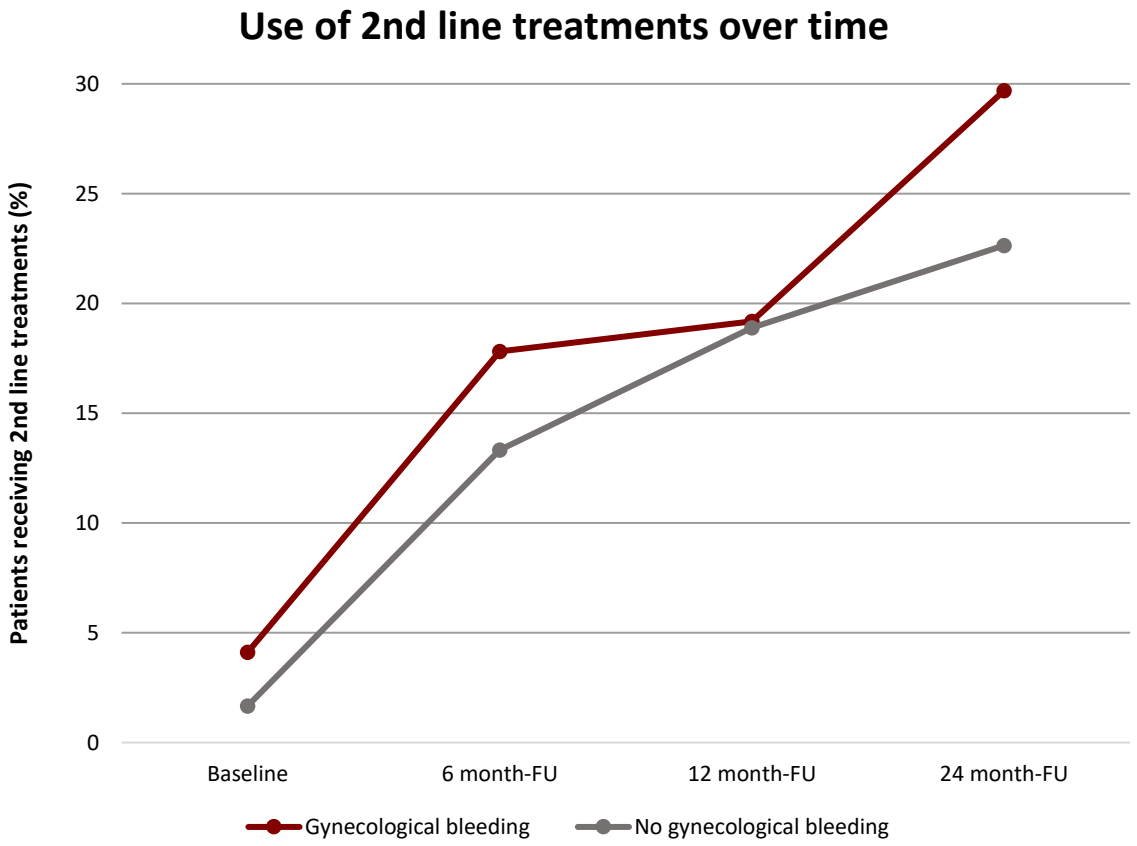
Differences persist over time: ▶ Treatment Need







Differences persist over time: ▶ Treatment escalation



Summary

Heavy menstrual bleeding at initial presentation identifies a distinct subgroup of females

with a persistently higher disease burden over at least 24 months

Summary

Heavy menstrual bleeding at initial presentation identifies a distinct subgroup of females

with a persistently higher disease burden over at least 24 months

1. more severe thrombocytopenia over all FU's

2. higher overall+mucosal bleeding burden

3. higher treatment need

Summary

Heavy menstrual bleeding at initial presentation identifies a distinct subgroup of females

with a persistently higher disease burden over at least 24 months

1. more severe thrombocytopenia over all FU's

2. higher overall+mucosal bleeding burden

3. higher treatment need

Distinct clinical phenotype with underlying biological differences?

Help us improve clinical care – your input matters

- Short survey on clinical practice (2 min)

THANK YOU!

Scan to participate