What's new in primary ITP?
Key updates from ASH 2024

Data updates
March 2025

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New real-world data on avatrombopag

Study information	Outcomes
• AVAMAD multicentre observational study in Spain • Adult patients treated with **avatrombopag** from July 2022 to May 2023 (N=66)	• **Primary ITP:** n=55; **chronic ITP:** 72.7% • **Reason for starting treatment with AVA:** refractoriness to previous treatment/CS dependence (39.1%); loss of response (34.4%); patient preference (14.0%); side effects with previous treatment (4.7%) • 88% started 20 mg AVA QD, with 25.8% requiring 40 mg QD dose • **Patients responded to AVA:*** 86.4%; **median time from AVA initiation to response**: 2.0 weeks (IQR 1.29–4.29) • **Patients maintained PC ≥50 x 10^9/L at last visit:** 90.9% (n=50/55) • **Patients reduced/discontinued concomitant medication:** 56.0% (n=28/50) • **Patients experienced a side effect:** 19.7% • **Side effects reported:** Headache (15.2%), arthralgias (6.1%), GI intolerance (3.0%) • **Patients discontinued AVA:** 16.7%†

*Defined as a PC ≥50 x 10^9/L; †discontinued due to lack of response (n=6), AEs (n=3), following recommendation as bridge to surgery (n=1).
AVA, avatrombopag; BL, baseline; CS, corticosteroid; GI, gastrointestinal; IQR, interquartile range; ITP, immune thrombocytopenia; PC, platelet count; QD, once daily.
Pascual-Izquierdo C, et al. *Br J Haematol*. 2025;206:652–6.

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New data on emerging ITP treatments (1/3)

Treatment (N)	Study information	Outcomes
Rilzabrutinib N=26	• Part B of the open-label phase I/II LUNA2 study • Oral rilzabrutinib 400 mg BID • Data from patients aged 18–80 years with relapsed ITP* • 24-week main treatment period with either entrance to the LTE† or 4-week safety follow-up if ineligible for the LTE • Patients enrolled between 22 March 2018 and 31 January 2023	• **Treatment continuation:** 58% (n=15) completed main 24-week treatment period, 42% (n=11) met LTE criteria and were ongoing in the LTE **Data from 24-week treatment period** • **Primary outcome: Durable response‡** achieved in 35% of patients (95% CI 17–56%) • **Mean number of weeks with PC ≥50 x 10^9/L or ≥30 x 10^9/L and double BL:§** 9.3 weeks • **≥2 consecutive PCs ≥50 x 10^9/L and increased ≥20 x 10^9/L from BL:¶** 42% • **Received rescue medication:** 12% • **Mean change in IBLS score from BL to week 25:** –0.07 • **Median duration of treatment:** 167 days (IQR 112–168) • **Any-grade TRAE:** 62% (most grade 1) • **Most common TRAEs:** diarrhoea (35%), headache (23%), nausea (15%) **Data from LTE period** • **Eligible to enter LTE:** 42% • **Median PCs in LTE:** >80 x 10^9/L • **Received rescue medication:** 0 • **Median duration of LTE treatment:** 182 days (IQR 125–323) • **AE due to any cause:** 73%

*Patients had to have a response (PC ≥50 x 10^9/L) to corticosteroids or IVIg/anti-D that was not sustained and failed ≥1 other ITP therapy other than corticosteroids or IVIg);
†Eligible for entrance to LTE if PCs ≥50 x 10^9/L or ≥30 x 10^9/L and double baseline in ≥4 of the last 8 weeks of treatment without rescue medication;
‡PCs ≥50 x 10^9/L on ≥8 of the last 12 weeks of the 24-week treatment period without the use of rescue medication after 10 weeks of active treatment;
§in the absence of rescue therapy over the 24-week treatment period;
¶in 4 weeks prior to latest elevated PC (PCs separated by ≥5 days).
AE, adverse event; BID, twice daily; BL, baseline; CI, confidence interval; IBLS, ITP bleeding scale; IQR, interquartile range; ITP, immune thrombocytopenia; IVIg, intravenous immunoglobulin G; LTE, long-term extension; PC, platelet count; TRAE, treatment-related AE.
Cooper N, et al. *Am J Hematol*. 2025;100:439–49.

New data on emerging ITP treatments (2/3)

Treatment (N)	Study information	Outcomes	
Rozanolixizumab • TP0003 (n=33) • TP0006 (n=30) • TP0004 (n=43)*	**Phase III studies TP0003, TP0006 (24-week)** • Randomized 2:1 to receive RLZ or PBO • ≈15 mg/kg starting dose; ≈10 mg/kg maintenance dose Q2W • Data from patients ≥18 years with chronic or persistent primary ITP†,‡ and PC <30 x 10^9/L **TP0004 (1-year OLE)*** • Patients started on Q2W dosing but then switched to QW after protocol amendment **Studies terminated early; as termination was not due to safety concerns, ongoing participants could continue with trials.**	**Data from TP0003** • **Primary outcome: DCMPR§ in RLZ vs PBO groups:** 4/21 vs 0/12 pts • **Mean time PC ≥50 x 10^9/L RLZ vs PBO:** 7.6 vs 2.5 weeks • **Any TEAE in RLZ vs PBO**: 85.7% vs 75.0% • **TEAEs leading to discontinuation in RLZ vs PBO:** 4.8% vs 0% • **Most frequently reported TEAEs:** Headache, pyrexia, nausea	**Data from TP0006** • **Primary outcome: DCMPR§ in RLZ vs PBO groups:** 1/20 vs 0/10 pts • **Mean time PC ≥50 x 10^9/L RLZ vs PBO:** 4.0 weeks vs 1.4 weeks • **Any TEAE in RLZ vs PBO:** 95.0% vs 60.0% • **TEAEs leading to discontinuation in RLZ vs PBO:** 10.0% vs 0% • **Most frequently reported TEAEs:** Headache, pyrexia, nausea
		Data from TP0004 (1-year OLE) • **Primary endpoint: Any TEAE in QW vs Q2W group,** 59.1% vs 88.1% • **Primary endpoint: No TEAEs lead to treatment discontinuation** • **Mean PCs ≥50 x 10^9/L maintained during QW dosing but not Q2W dosing**	

*Patients who completed 24-week double-blind treatment period of TP0003 or TP0006 could enrol into TP0004; †Persistent ITP: 3–12 months duration, chronic ITP: >12 months duration; ‡Participants must have intolerance or insufficient response to ≥2 SoC ITP treatments and a history of response to previous ITP therapy; §PC ≥50 x 10^9/L for ≥8/12 visits during weeks 13–25.

DCMPR, durable clinically meaningful platelet response; ITP, immune thrombocytopenia; OLE, open-label extension; PBO, placebo; PC, platelet count; pts, patients; QW, once weekly, Q2W, once every 2 weeks; RLZ, rozanolixizumab; SoC, standard of care; TEAE, treatment-emergent adverse event.

Cooper N, et al. *Br J Haematol*. 2025;206:675–88.

New data on emerging ITP treatments (3/3)

Treatment (N)	Study information	Outcomes
• **Avatrombopag** N=94	• Real-world observational study in China • Children age <18 years with primary ITP treated with avatrombopag for ≥4 weeks from February 2020 to March 2024	• **Median effective dose:** 10 mg for children <6 years; 20 mg for children <18 years • **Overall response achieved:*** 72.3% within 4 weeks; 73.4% within 12 weeks • **Sustained response:†** 62.3% at 24 weeks; 51.6% at 48 weeks • Proportion of **bleeding symptoms** and **rate of bleeding events remained lower than BL** throughout the study • **Concomitant medications reduced throughout the study period:** BL 55.3%; 48 weeks 14.8% • **Most frequent AE:** thrombocytosis (PC ≥400 x 10^9/L) in 44 children, occurring 97 times

*The number of patients achieving complete response (at least one PC ≥100 x 10^9/L between 7 days and 4 weeks after initiation, without the need for rescue therapy) and platelet response (PC 30–100 x 10^9/L, with ≥2x increase in PC from baseline at least once between 7 days and 4 weeks after initiation, without the need for rescue therapy); †PCs >30 x 10^9/L at 75% of assessment points from initial response to 24 and 48 weeks of follow-up.

AE, adverse event; BL, baseline; ITP, immune thrombocytopenia; PC, platelet count.

Wang N, et al. *Br J Haematol*. 2025;206:935–43.

Critical bleeds in patients with ITP

Study information	Outcomes
• Systematic review of **treatments for critical bleeds** in patients with ITP • Information extracted for patients who received ≥1 intervention and for whom PC response, bleeding, disability (neurological sequalae or declining mental state) or death was reported • 49 eligible studies: N=112 critical bleed patients with ITP (n=66 children, n=36 adults, n=10 unreported age)	• **Most common interventions (n>10):** CS + platelet transfusion + splenectomy (n=13); CS + IVIg (n=13); splenectomy alone (n=13); IVIg alone (n=11) **Patient outcomes** • **Mortality:** 25.0% (adults 30.6%, children 19.7%) • **PC response*:** 83.6% (adults 92.6%, children 79.5%) • **Bleeding resolution achieved:** 81.7% (adults 84.6%, children 81.4%) • **Neurological sequalae or declining mental state:** 16.2% (adults 9.1%, children 19.2%) • **Mortality associated with ICH vs non-ICH bleeds:** 24.5% vs 28.6% • **Mortality with single intervention vs combination:** 30.2% vs 21.7%

*Achievement of any PC >30 x 10^9/L.

CS, corticosteroids; ICH, intracranial haemorrhage; ITP, immune thrombocytopenia; IVIg, intravenous immunoglobulin; PC, platelet count.

Chowdhury SR, et al. *Eur J Haematol*. 2025;144:458–68.