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

Data updates
July 2025

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New data on approved TPO-RAs for ITP (1)

Treatment (N)	Study information	Outcomes
Avatrombopag N=177[1,2]	• REAL-AVA 2.0 retrospective chart review study[1–4] • Adult patients with primary ITP in the USA who initiated AVA between 1 July 2019 and 30 June 2024[1–4] • Data cut-off: 31 December 2024[1,2,4]	• **Median duration of AVA treatment:** 12.8 months[1] • **Median duration of AVA treatment by type of primary ITP:** acute* (n=39) 6.2 months; persistent (n=29)† 12.8 months; chronic (n=103)‡ 17.1 months[3] • **Prior TPO-RA exposure:** 66%[2] • Patients switched from ELT (n=38) and ROM (n=41)[4] **Among patients who achieved/maintained PC response ≥30 x 10^9/L (n=160):** • **Median duration of response:** 12.0 months; **durability of response** 95.7%[1] • **Durability of response by PC threshold:** acute* 88.2%; persistent† 97.4%; chronic‡ 96.4%[3] • **Achieved PC threshold when switched from ELT and ROM to AVA:** 89.5% and 87.8%[4] • **PC threshold achieved by similar proportion of patients with prior TPO-RA vs TPO-RA-naive[2]** **Among patients who achieved or maintained PC response ≥50 x 10^9/L (n=153):** • **Median duration of response:** 12.1 months; **durability of response** 93.2%[1] • **Durability of response by PC threshold:** acute* 87.4%; persistent† 91.1%; chronic‡ 95.2%[3] • **Achieved PC threshold when switched from ELT and ROM to AVA:** 86.8% and 85.4%[4] • **PC threshold achieved by similar proportion of patients with prior TPO-RA vs TPO-RA-naive[2]** **Among patients who achieved or maintained PC response ≥100 x 10^9/L (n=134):** • **Median duration of response:** 9.7 months; **durability of response** 75.5%[1] • **Durability of response by PC threshold:** acute* 73.4%; persistent† 77.7%; chronic‡ 76.0%[3] • **Achieved PC threshold when switched from ELT and ROM to AVA:** 73.7% and 73.2%[4] • **More TPO-RA-naive patients achieved PC threshold vs prior TPO-RA exposed** (70% vs 87%, p<0.05)[2]

*Defined as <3 months; †Defined as 3–<12 months of disease duration at index; ‡Defined as ≥12 months of disease duration at index.
AVA, avatrombopag; ELT, eltrombopag; ITP, immune thrombocytopenia; PC, platelet count; ROM, romiplostim; TPO-RA, thrombopoietin receptor agonist.
1. Nagalla S, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PF1239; 2. Nagalla S, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr OC 65.3; 3. Levy MY, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2244; 4. Chaturvedi S, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PF1236.

New data on approved TPO-RAs for ITP (2)

<table>
<tr><th>Treatment (N)</th><th>Study information</th><th>Outcomes</th></tr>
<tr>
<td>Avatrombopag
N=147</td>
<td>• REAL-AVA 3.0 retrospective chart review study
• TPO-RA-naive adult patients with primary ITP in the USA who initiated AVA on or after 1 July 2019
• Data collected between 12 November 2024 to 31 December 2024</td>
<td>
• Median duration of AVA treatment at last follow-up: 8.1 months
• Proportion of patients who received concomitant medication whilst on AVA: 8.2%; all discontinued (n=9/12) or reduced (n=3/12) concomitant medication
• Proportion of patients who received rescue therapy whilst on AVA: 5.4%
<table>
<tr><th></th><th colspan="4">Proportion of patients with PC response (%) / durability of response (%)</th></tr>
<tr><th></th><th>≥30 x 10^9/L</th><th>≥50 x 10^9/L</th><th>≥75 x 10^9/L</th><th>≥100 x 10^9/L</th></tr>
<tr><td>BL PC <30 x 10^9/L (n=124)</td><td>100.0 / 89.1</td><td>95.2 / 80.7</td><td>87.9 / 68.7</td><td>73.4 / 61.6</td></tr>
<tr><td>BL PC ≥30–<50 x 10^9/L (n=20)</td><td>NA</td><td>100.0 / 85.1</td><td>90.0 / 75.1</td><td>75.0 / 64.8</td></tr>
<tr><td>BL PC ≥50–<75 x 10^9/L (n=2)</td><td>NA</td><td>NA</td><td>100.0 / 95.1</td><td>100.0 / 95.1</td></tr>
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BL, baseline; AVA, avatrombopag; ITP, immune thrombocytopenia; PC, platelet count; TPO-RA, thrombopoietin receptor agonist.

Panch S, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2239.

New data on approved TPO-RAs for ITP (3)

Treatment (N)	Study information	Outcomes
Avatrombopag N=200	• ADOPT study (phase IV)[1,2] • Adult patients ≥18 years with ITP in Europe who were initiating or already being treated with AVA[1,2] • Data cut-off date: 12 November 2024[1,2] • Patients with 12 months of follow-up data reported[1]	• **ITP disease phase:** ND†, n=19; P‡, n=19; C§ , n=162[1] • **Prior TPO-RA exposure:** ELT only (n=58); ROM only (n=45); ELT + ROM (n=25)[2] • **Aged ≥65 years:** (n=73); **comorbidities:*** (n=89)[2] • **Mean cumulative number of weeks with PC ≥30 x 10^9/L:**[1,2] **Primary endpoint:**

≥65 yrs	Comorbidities	ELT only	ROM only	ELT + ROM	**ND**	**P**	**C**
50.1	50.8	52.1	51.4	46.3	**52.1**	**49.7**	**51.1**

• **Mean cumulative number of weeks with PC ≥50 x 10^9/L:** [1,2]

≥65 yrs	Comorbidities	ELT only	ROM only	ELT + ROM	ND	P	C
47.0	48.5	50.2	46.5	42.9	52.1	44.1	48.4

• **Proportion of patients on concomitant ITP treatments and rescue therapy:**[1,2]

≥65 yrs	Comorbidities	ELT only	ROM only	ELT + ROM	ND	P	C
39.7%; 11.0%	39.3%; 10.1%	39.7%; 6.9%	26.7%; 15.6%	56.0%; 20.0%	26.3%; 5.3%	42.1%; 21.1%	35.8%; 9.9%

*Comorbidities considered to be risk factors for thromboembolic events including obesity/overweight, cardiovascular disease, chronic renal disease, smoking/alcohol use, oral contraceptive use, personal/family history of thromboembolic events, recent major surgery or cancer; †<3 months from ITP diagnosis to first AVA treatment; ‡ 3–12 months from ITP diagnosis to first AVA treatment; §≥12 months from ITP diagnosis to first AVA treatment.

AVA, avatrombopag; C, chronic; ELT, eltrombopag; ITP, immune thrombocytopenia; ND, newly diagnosed; P, persistent; PC, platelet count; ROM, romiplostim; TPO-RA, thrombopoietin receptor agonist.

1. Ghanima W, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2231; 2. Mingot Castellano ME, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2242.

New data on approved TPO-RAs for ITP (4)

Treatment (N)	Study information	Outcomes
Avatrombopag N=268	• AVESPA retrospective study conducted by the Spanish ITP group • Patients with ITP who began treatment with AVA between July 2022 to January 2024 and previously treated with another TPO-RA	• **Median follow-up:** 47 months • **Patients previously treated with TPO-RA:** 59.3% (ELT 40.9%; ROM 11.9%; both 47.2%) • **Most frequent reason for switching to AVA:** loss of response (35.8%), low efficacy and/or CS dependence (28.3%) • **Proportion of patients with previous TPO-RA exposure who responded to AVA at intermediate doses (<280 mg/week):** 43% • **Higher proportion of patients who were TPO-RA-naive responded to AVA vs those with previous TPO-RA exposure:** 79.7% vs 92.4% (p<0.001) • No difference in response based on previous TPO-RA

AVA, avatrombopag; CS, corticosteroid; ELT, eltrombopag; ITP, immune thrombocytopenia; ROM, romiplostim; TPO-RA, thrombopoietin receptor agonist.

Pasqual-Izquierdo C, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr PB0356.

New data on approved TPO-RAs for ITP (5)

Treatment (N)	Study information	Outcomes
Fostamatinib[1] N=95	• GIMEMA ITP1122 retrospective study (phase IV) • Patients with persistent/chronic ITP who received ≥1 dose of FOSTA outside of clinical trials between 1 October 2021 and 1 April 2023	• **Proportion of patients who received FOSTA as fourth- or later-line treatment:** 85% • **Proportion of patients who previously received >1 TPO-RA:** 54% • **Proportion of patients who received FOSTA concurrently with other anti-ITP medications:** 31% at baseline; 19% at 6 months • **Primary endpoint – Proportion of patients still receiving FOSTA at 6 months: 45% (CR in 22%)** • **Most frequently reported TRAEs:** diarrhoea (37.5%), transaminitis (21.8%), hypertension (21.8%) and neutropenia (9.3%)
Fostamatinib[2] N=33	• Japanese patients with ITP treated with FOSTA (phase III; NCT04132050) • Three-year efficacy and safety data reported	• **Proportion of patients achieving PC >50 x 10^9/L:*** 48% • **Median total duration of PC >50 x 10^9/L:** 589 days • **Proportion of patients achieving PC >30 x 10^9/L:*** 55% • **Median total duration of PC >30 x 10^9/L:** 727 days • **Most common TEAEs:** diarrhoea, hypertension

*At two consecutive visits with ≥28 days apart whilst receiving FOSTA.

AE, adverse event; BID, twice daily; CR, complete response; FOSTA, fostamatinib; ITP, immune thrombocytopenia; QD, once daily; TEAE, treatment-emergent AE; TPO-RA, thrombopoietin receptor agonist; TRAE, treatment-related AE.

1. Lucchini E, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2233; 2. Kuwana M, Tomiyama Y. Int J Hematol. 2025;121:356–62.

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New data on approved TPO-RAs for ITP (6)

Treatment (N)	Study information	Outcomes
Romiplostim for 1 year, followed by tapering and follow-up for ≤1 year (N=40)	• Prospective STIP trial in the Netherlands to determine rate of SROT* • Adults with persistent/chronic ITP who failed at least first-line treatment with CS or IVIg	• **Primary outcome: probability of SROT at 1 year after tapering (n=25):** 23.6% • **SCROT† among SROT patients with complete follow-up:** 80% (n=4/5) • **Probability of being treatment free at 1 year after tapering:** 46.3% • **Proportion of patients who restarted romiplostim and responded again:** 83.3% (n=10/12) • **Patients with SROT reached higher PCs during treatment and had lower stable doses at start of tapering vs NSR** **Safety** • **Mild bleeding (grade 1) reported in patients with an NSR after tapering:** 41.2% (n=7/17) • **Patients hospitalized for severe thrombocytopenia (<1 x 10^9/L):** n=1 (with mild bleeding symptoms) **QoL** • **D-ITP:** increase in 3/11 domains for treatment-free patients at study end; increase in 4/11 domains for those who were not; both groups improved in fatigue and psychological health domains • **SF-36:** Minor changes reported

*PC >30 x 10^9/L, no bleeding symptoms and no need for treatment; †PC>100 x 10^9/L at all visits.
CS, corticosteroids; D-ITP, Dutch ITP; ITP, immune thrombocytopenia; IVIg, intravenous immunoglobulin; NSR, non-sustained response; PC, platelet count; SCROT; complete SROT; SF-36; short-form 36; SROT, sustained response off treatment; TPO-RA, thrombopoietin receptor agonist.
Nelson VS, et al. *Br J Haematol*. 2025;206;1743–53.

New slide

New data on approved TPO-RAs for ITP (7)

Treatment (N)	Study information	Outcomes
rhTPO, romiplostim and eltrombopag 7 RCTs involving 375 paediatric patients	• Systematic review and network meta-analysis to evaluate and compare the efficacy and safety of rhTPO, romiplostim and eltrombopag in paediatric patients with ITP • Treatment efficacy ranked using SUCRA*	• **Romiplostim (OR 17.57, 95% CI 4.90–63.03), eltrombopag (OR 5.34, 95% CI 2.50–11.39) and rhTPO (OR 5.32, 95% CI 2.03–13.96) were all significantly more effective in achieving ORR vs placebo (p<0.001)** • **SUCRA ranking for ORR:** Romiplostim had highest probability of being the most effective intervention (SUCRA = 0.96) followed by rhTPO (SUCRA = 0.52), eltrombopag (SUCRA = 0.52) and placebo (SUCRA = 0.00) • **SUCRA rankings for serous adverse events:** rhTPO had the highest probability of being the safest intervention (SUCRA = 0.78) followed by eltrombopag (SUCRA = 0.66), placebo (SUCRA = 0.44) and romiplostim (SUCRA = 0.12)

*Values closer to 1 indicate higher likelihood of being the most effective treatment.

OR, odds ratio; ORR, overall response rate; RCT, randomized controlled trial; rh-TPO, recombinant human TPO; SUCRA, surface under the cumulative ranking curve; TPO, thrombopoietin; TPO-RA, TPO receptor agonist.

Zhang X, et al. *Front Immunol*. 2025;16:1595774.

New data on approved TPO-RAs for ITP (8)

Treatment (N)	Study information	Outcomes
TPO-RA 417 treatment courses analysed*	• Retrospective, multicentre study • Adult patients with ITP in Europe who initiated TPO-RAs between January 2014 and December 2014	**Reasons for discontinuation of first TPO-RA (incidence 49.8%):** • **Test for therapy-free remission:** 29.3% • **Due to AEs:** 16.5% • **Due to inadequate efficacy:** 8.3% **Following TPO-RA discontinuation:**

	First course	Second course	Third course
Proportion of patients who did not require additional ITP treatment	51.7%	40.3%	26.1%
Proportion of patients who required reintroduction of TPO-RA	28.0%	34.8%	37.5%

- **Patients who received a TPO-RA for >1 year were significantly less likely to require reintroduction vs those treated for shorter durations (p=0.045)**

	Proportion achieving SROT†		
Course	**<30 x 10^9/L**	**<50 x 10^9/L**	**<100 x 10^9/L**
First	29.5%	24.7%	21.4%
Second	27.7%	22.1%	21.2%
Third	18.2%	13.5%	13.5%

- **Significant predictors of SROT:** PCs at 6 months from TPO-RA initiation; PC at diagnosis inversely correlated with likelihood of achieving SROT

*267 first-line, 113 second-line, 37 third-line TPO-RA; †defined as PCs maintained above stated value for at least 6 months.
AE, adverse event; ITP, immune thrombocytopenia; PC, platelet count; SROT, sustained response off treatment; TPO-RA, thrombopoietin receptor agonist.
Lozano M, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PF1235.

New data on approved TPO-RAs for ITP (9)

Treatment (N)	Study information	Outcomes
TPO-RA[1,2] N=267	• Retrospective analysis in Europe[1,2] • Adult patients with ITP who initiated TPO-RA between 2014 and 2018[1,2] • At treatment initiation patients were stratified into two age groups, patients <65 years n=164; patients ≥65 years n=103[1] • Patients received ROM (n=84) or ELT (n=183)[2]	• **Comorbidities more prevalent in older vs younger population:** hypertension (p<0.0001), diabetes (p<0.0001); cancer (p<0.001); use of anti-thrombotic therapy (p<0.0001); vascular events (p<0.001)[1] • **Prevalence of ND ITP:** higher in older vs younger patients (p=0.0019)[1] • **Median time from diagnosis to TPO-RA initiation:** shorter in older vs younger patients (p=0.004)[1] • **Time to achieve PC >30 x 10^9/L and >50 x 10^9/L:** shorter in older vs younger patients (p=0.0038 and p=0.0012)[1] • **Response rates**: lower in older vs younger patients (89.9% vs 96.1%, p=0.0200)[1] • **Primary reason for TPO-RA initiation:** refractoriness or loss of response to prior therapy (40.4%)[2] • **Median duration of first TPO-RA treatment:** 13 months (ROM, 8.5 months; ELT, 16 months)[2] • **No significant association between TPO-RA selection and several patient characteristics:** age, sex, comorbidities,* disease phase, history of malignancy or thrombosis, or response to previous therapies (p>0.05)[2] • **Bleeding severity:** higher in patients treated with ROM vs ELT at TPO-RA initiation (p<0.05)[2] • **Reduction in unscheduled hospital visits** by 52.7% over 6 months since starting TPO-RA[2]

*Liver disease, diabetes and hypertension.

ELT, eltrombopag; ITP, immune thrombocytopenia; ND, newly diagnosed; PC, platelet count; ROM, romiplostim; TPO-RA, thrombopoietin receptor agonist.

1. Lozano M, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PF1258; 2. Lozano M, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2259.

New data on emerging ITP treatments (1)

Treatment (N)	Study information	Outcomes
Daratumumab N=21	• DART trial (phase II) • Adult patients with primary ITP and PC ≤30 x 10^9/L (15–30 x 10^9/L for safety run in) with prior CS and rituximab and/or TPO-RA • Patients enrolled between January 2021 and March 2024 • Safety run in (n=3): 4 x weekly SC daratumumab injections • Cohort 1 (n=9): 8 x weekly injections • Cohort 2 (n=9): 8 x weekly injections, followed by 2 injections every other week	• **Primary endpoint – response at week 12/16:* all patients, n=10/21 (48%);** safety run in, 2/3; cohort 1, 4/9 (44%); cohort 2, 4/9 (44%) • **Sustained response at week 24:†** all patients, n=8/21 (38%); cohort 1, 4/9 (44%); cohort 2, 3/9 (33%) • **Most common TEAE:** Infections (38%) • **Most common TRAEs:** n=9; IRRs (grade 2, 9.5%; grade 3, 4.7%), injection site reactions (grade 1, 9.5%), infections (grade 1 and 2, 4.7% each), diarrhoea (grade 2, 9.5%) • **Grade 3 SAEs:** n=2 (1 patient with IRR; 1 patient with severe SARS-CoV-2-infections with acute renal failure) • **SF-36v1 scores:** Numerical improvement in all dimensions after completion of treatment

*Two consecutive PC ≥50 x 10^9/L (measured ≥24 hours apart) and assessed ≥4 weeks after the last daratumumab injection (week 12 for safety run-in and cohort 1; week 16 for cohort 2); †Two consecutive PC ≥50 x 10^9/L (measured ≥24 hours apart).

AE, adverse event; CS, corticosteroid; IRR, infusion-related reaction; ITP, immune thrombocytopenia; PC, platelet count; SAE, serious AE; SC, subcutaneous; SF-36v1, 36-item short form survey version 1; TEAE, treatment-emergent AE; TPO-RA, thrombopoietin receptor agonist; TRAE, treatment-related AE.

Tsykunova G, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr S311.

New data on emerging ITP treatments (2)

Treatment (N)	Study information	Outcomes
Mezagitamab N=41	• Phase II study • Patients with chronic or persistent ITP • Part A (n=25): participants randomized 1:1:1 to mezagitamab 100 mg or 300 mg or placebo • Part B (n=16): participants randomized 2:1 to mezagitamab 600 mg or placebo • SC treatment QW for 8 doses • At week 16 participants were unblinded and could receive mezagitamab in OLE	• **Duration of platelet response* at week 16:** mezagitamab 100 mg, 6.0 weeks; mezagitamab 300 mg, 8.0 weeks; mezagitamab 600 mg, 10.6 weeks; placebo, 1.1 weeks • **Mean increase in duration of platelet response* with mezagitamab vs placebo:** 100 mg: 4.91 ± 1.6 (SE) weeks; 300 mg: 6.9 ± 2.1 weeks; 600 mg 9.6 ± 1.7 weeks • **Clinically meaningful improvement† in mean change from BL ITP-PAQ scale scores in mezagitamab groups but not placebo:** fatigue/sleep (p=0.01); physical activity (p=0.02); symptoms (p=0.02); physical health – bother (p=0.03); overall QoL (0.04); social activity (p=0.005)

*Number of weeks with PC ≥50 x 10^9/L; †Defined as mean change exceeding the minimal important difference threshold of 8 or 10 points.
BL, baseline; ITP, immune thrombocytopenia; ITP-PAQ, ITP Patient Assessment Questionnaire; OLE, open label extension; QW, once weekly; SC, subcutaneous; SE, standard error.
Kuter DJ, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr OC 75.3.

New data on emerging ITP treatments (3)

Treatment (N)	Study information	Outcomes
Efgartigimod[1,2] N≈63	• ADVANCE NEXT trial (phase III) • Adult patients ≥18 years with primary ITP (>12 months') who received ≥1 prior ITP therapy* • **Double-blind treatment period (24 weeks):** patients randomized 2:1 to efgartigimod IV 10 mg/kg or placebo ○ Treatment with ≥4 QW infusions; may be adjusted to Q2W based on PC response • **Open-label treatment period (52 weeks):** efgartigimod IV 10 mg/kg • **Second open-label treatment period (52 weeks):** to continue receiving efgartigimod • **Primary endpoint: extent of disease control**†	
Efgartigimod[3,4] N=131	• ADVANCE IV trial (phase III) • Adult patients with persistent or chronic primary ITP, with an average of two PCs <30 x 10^9/L during screening randomized to efgartigimod IV or placebo	• **Primary endpoint – proportion of patients with sustained PC response:**‡ **Efgartigimod 25.6% vs placebo 6.7% (p=0.0108)** • **Mean immature platelet fraction at week 24 (n=40):** decreased with efgartigimod vs no decrease with placebo

*Including corticosteroids; IVIg; anti-D immunoglobulin in non-splenectomized Rho(D)-positive patients; TPO-RAs; or rituximab; †Defined as number of cumulative weeks during the double-blind treatment period with PC of ≥50 x10^9/L; ‡defined as PCs ≥50 x 10^9/L for ≥4 or 6 visits between weeks 19 and 24.

Ig, immunoglobulin; ITP, immune thrombocytopenia; IV, intravenous; PC, platelet count; Q2W, once every 2 weeks; QW, once weekly; TPO-RA, thrombopoietin receptor agonist.

1. Al-Samkari H, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr PB0337; 2. Al-Samkari H, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PB3668; 3. Matthijssens F, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr OC 75.4; 4. Zaja F, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2243.

New data on emerging ITP treatments (4)

Treatment (N)	Study information	Outcomes
Rilzabrutinib N=202	• LUNA3 phase III study • Adult patients with persistent/chronic ITP • DB part (24 weeks): randomized 2:1 to 400 mg BID rilzabrutinib or placebo* • OL period (28 weeks): 400 mg BID rilzabrutinib only • LTE: 400 mg BID rilzabrutinib only	**Data from DB period:** • **Primary end point – durable platelet response:†** **observed in n=31 (23%) rilzabrutinib vs n=0 placebo patients (p<0.0001)** • **Median time to first platelet response:** rilzabrutinib arm 36 days; placebo arm NR (p<0.0001); rilzabrutinib responders 15 days • **PCs ≥50 x10^9/L for ≥4 of last 8 PCs in patients completing 24 weeks of treatment:** rilzabrutinib 55%; placebo 0% • **Need for rescue therapy:** significantly reduced with rilzabrutinib (–52% p=0.0007) • **Change from baseline in IBLS score at week 25:** significantly improved with rilzabrutinib vs placebo (p=0.0006) • **Change from baseline in physical fatigue at week 13:** significantly improved with rilzabrutinib vs placebo (p=0.0114) • **Most common TRAEs for rilzabrutinib vs placebo:** diarrhoea (23% vs 4%), nausea (17% vs 6%), headache (8% vs 1%), abdominal pain (6% vs 1%)

*After initial DB 12 weeks, non-responders could join the OL or discontinue; †Defined as PCs ≥50 x 10^9/L for two-thirds or more of ≥8 non-missing weekly scheduled platelet measurements during the last 12 weeks in the absence of rescue therapy and ≥2 of the PCs ≥50 x 10^9/L had to be during the last 6 weeks of the 24-week blinded treatment period.
BID, twice daily; DB, double-blind; IBLS, ITP bleeding scale; ITP, immune thrombocytopenia; LTE, long-term extension; NR, not reached; OL, open label; PC, platelet count; TRAE, treatment-related adverse event.
Kuter DJ, et al. *Blood*. 2025;145:2914–26.

New data on emerging ITP treatments (5)

Treatment (N)	Study information	Outcomes
Rilzabrutinib OL period n=180 LTE period n=69	• LUNA3 phase III study[1,2] • Adult patients with persistent/chronic ITP[1,2] • DB part (24 weeks): randomized 2:1 to 400 mg BID rilzabrutinib or placebo*[1,2] • OL period (28 weeks): 400 mg BID rilzabrutinib only[1,2] • LTE: 400 mg BID rilzabrutinib only†[2] • Data cut-off: 15 October 2024[2]	**Data from OL period[1]** • **Durable response achieved in:‡** 27%, including 30% (n=35/115) receiving rilzabrutinib DB (responders/non-responders) and 22% (n=14/65) placebo DB • **Complete platelet response:§** 23% • **Cumulative stable platelet response:¶** 36/67 (54%) • **Mean changes from baseline in physical fatigue and IBLS bleeding score:** improved with rilzabrutinib at weeks 25 and 53 • **Most common TRAEs:** diarrhoea and nausea (9% each, mainly low grade) **Data from LTE period[2]** • **PCs ≥50 x 10^9/L or between 30–50 x 10^9/L and doubled from baseline:** maintained for average of 76% of weeks based on 12 months of LTE assessments • **Patients who achieved complete platelet response:‖** 54% • **Most common TRAEs (all grade 1 or 2):** Nausea (7%), diarrhoea (4%), upper abdominal pain (3%)

*After initial DB 12 weeks, non-responders could join the OL or discontinue; †Eligibility for the LTE: if during the last 8 weeks of the 28-week OL period, PCs were ≥50 x 10^9/L or ≥30 x 10^9/L and double from baseline at ≥50% of visits without rescue therapy;[2] ‡PCs ≥50 x 10^9/L for ≥two-thirds of the last 16 of 28 OL weeks without rescue therapy; §PCs ≥100 x 10^9/L; ¶PCs ≥50 x 10^9/L for ≥1 year. ‖Complete response was platelet counts ≥100 × 10^9/L on two consecutive visits at least 5 days apart and no bleeding or rescue ITP therapy use on or through these visits.
BID, twice daily; DB, double-blind; IBLS, ITP bleeding scale; ITP, immune thrombocytopenia; LTE, long-term extension; OL, open label; PC, platelet count; TRAE, treatment-related adverse event.
1. Kuter DJ, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr PB0376; 2. Ghanima W, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr S310.

New data on emerging ITP treatments (6)

Treatment (N)	Study information	Outcomes
Ripertamab N=20	• Multicentre, single-arm, dose exploration, prospective trial • Patients with relapsed/refractory ITP and treated with ripertamab 100 mg (n=9) or 200 mg (n=11) weekly for 4 consecutive weeks • Date collected between September 2022 and December 2024	• **Median follow-up:** 7 months • **Median time of initial response:** 2 weeks • **Median duration of response:** 3 months • **Response 1 month after first dose:** complete response:* n=8; partial response:† n=5; ORR: 65% • **Response 2 months after first dose:** complete response:* n=8; partial response:† n=6; ORR: 70% • **8-week ORR:** numerically higher in 100 mg vs 200 mg group (p=0.157) • **Adverse events:** IRR (low fever in 15% of patients) during first dose, upper respiratory tract infection (5%)

*Defined as PC ≥100 x 10^9/L; †defined as PC 30–99 x 10^9/L or at least double the baseline level with no bleeding symptoms.
IRR, infusion related reaction; ITP, immune thrombocytopenia; ORR, overall response rate; PC, platelet count.
1. Liu J, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr PB0333; 2. Liu, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2246.

New data on emerging ITP treatments (7)

Treatment (N)	Study information	Outcomes
CM313 N=45	• Phase II trial • Adult patients with persistent or chronic ITP who have failed or relapsed after glucocorticoid therapy and had previously responded to first-line treatment • Patients enrolled between 16 January and 11 June 2024	• **Primary endpoint – overall response* at week 8:** CM313 83% vs placebo 20% (p<0.0001) • **Median time to PC ≥50 x 10^9/L:** CM313 1 week vs placebo NR (p<0.0001) • **Median cumulative duration of PC ≥50 x 10^9/L:** CM313 18 weeks vs placebo 3 weeks (p=0.0035) • **Proportion of patients who experienced TEAEs:** CM313 83% vs placebo 80% • **Most frequent TEAEs:** IRRs, petechiae, upper respiratory tract infections

*Defined as ≥2 PCs ≥30 x 10^9/L, representing a doubling from baseline, in the absence of bleeding.
IRR, infusion-related reaction; ITP, immune thrombocytopenia; IV, intravenous; NR, not reached; PC, platelet count; QW, once weekly; TEAE, treatment emergent adverse event.
Xu Y, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr LB4004.

New data on emerging ITP treatments (8)

Treatment (N)	Study information	Outcomes
Ianalumab N=41	• VAYHIT3 (phase II) • Adult patients with primary ITP and PC <30 x 10^9/L, previously treated with at least a CS and TPO-RA (no splenectomy) with a loss of response, no or insufficient response or intolerance to last ITP therapy • Data cut-off: 5 February 2025	• **Primary endpoint – proportion of patients achieving ConfR*:** 44% • **Median time to ConfR:** 6 weeks • **Proportion of patients achieving stable response:†** 24% • **Achieved stable response† at week 25:** 56% • **Rate of any-grade bleeding events:‡** 59% at baseline; 22% at week 25; 10% at week 33 • **Proportion of patients who experienced a TRAE:** 37% • **Proportion of patients who experienced grade ≥3 TRAE:** 2% • **Most frequent AEs:** Headache (22%), confusion (20%), petechiae (20%), purpura (20%), IRR (15%), URTI (12%)

*Defined as PC of ≥50 x 10^9/L at ≥2 consecutive assessments ≥7 days apart between week 1 and week 25 with no rescue therapy within ≥4 weeks of PC assessment or start of new therapy before ConfR; †defined as PC of ≥50 x 10^9/L on ≥75% of assessments between week 19 and week 25 with no rescue therapy within ≥4 weeks of PC assessment or start of new therapy before stable response; ‡According to the WHO bleeding scale.

AE, adverse event; ConfR, confirmed response; CS, corticosteroid; IRR, infusion related reaction; ITP, immune thrombocytopenia; IV, intravenous; PC, platelet count; Q4W, every 4 weeks; TPO-RA, thrombopoietin receptor agonist; TRAE, treatment-related AE; URTI, upper respiratory tract infection; WHO, World Health Organization.

Bradbury C, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr S312.

New data on emerging ITP treatments (9)

Treatment (N)	Study information[1]	Outcomes
Avatrombopag N=75[1,2] OLE N=73[3]	• AVA-PED-301 (phase IIIb) • Paediatric patients aged 1–17 years with ITP for ≥6 months and an insufficient response to previous treatment • Cohort 1: aged 12–17 years; cohort 2: aged 6–11 years; cohort 3: aged 1–5 years	**Main phase (data collection 2 March 2021 to 2 August 2023)[1]** • **Primary endpoint – Proportion of patients achieving a durable platelet response:* avatrombopag 28% vs placebo 0% (–28% [95% CI 16–40]; p=0.0077)** ○ Cohort 1 43%; Cohort 2 20%; Cohort 3 15% • **Alternative primary endpoint of platelet response:† avatrombopag 81% vs placebo 0% (p<0.0001)** ○ Cohort 1 81%; Cohort 2 85%; Cohort 3 77% • **Most frequent TEAEs:** petechiae, epistaxis, ecchymosis, headache, cough, pyrexia, oropharyngeal pain, URTI and haematoma **Post hoc analyses[2]** • **Proportion of patients achieving clinically meaningful response (≥30 x 10^9/L):** avatrombopag 92.6% vs placebo 19.1% • **Proportion of patients achieving response (≥50 x 10^9/L):** avatrombopag 90.7% vs placebo 9.5% **Open-label extension (data cut-off 30 September 2024)[3]** • **Median PC in absence of rescue therapy:** within target range‡ for months 2–24 • **Experienced TRAE:** 13.7% • **Experienced serious TEAEs:** 27.4% • **Most common serious TEAEs:** thrombocytopenia (5.5%), epistaxis (5.5%), mucosal haemorrhage (2.7%) and gastroenteritis (2.7%)

*Defined as ≥6 PCs ≥50 x 10^9/L during last 8 weeks of the 12-week core phase treatment in the absence of rescue therapy; †At least two consecutive PCs ≥50 x 10^9/L over the 12-week core phase treatment period in the absence of rescue therapy; ‡≥50 x 10^9/L–≤150 x 10^9/L.

AE, adverse event; ITP, immune thrombocytopenia; OLE, open-label extension; PC, platelet count; TEAE, treatment-emergent AE; TRAE, treatment related AE; URTI, upper respiratory tract infection.

1. Grace RF, et al. *Lancet Hematol*. 2025;12:e494–504; 2. Grace RF, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PF1251; 3. Grace RF, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2234.

New data on the impact of ITP (1)

Study information

- Prospective cohort study of adults with primary ITP (N=202)
- Data from Vienna ITP Biobank
- Aim: to evaluate frequency of anaemia, ID,* IDA† and signs of haemolysis‡

Outcomes

- **Presence of anaemia:**

ND (n=72)	Persistent (n=17)	Chronic (n=113)
30.6%	23.5%	19.5%

- **Presence of ID:**

ND (n=72)	Persistent (n=17)	Chronic (n=113)
22.2%	17.6%	34.5%

- **Presence of IDA:**

ND (n=72)	Persistent (n=17)	Chronic (n=113)
12.5%	11.8%	14.2%

- **Signs of haemolysis in 35% patients**; similar incidence regardless of disease duration
  - **Haemolysis was associated with anaemia in 35% of these cases**
  - **Signs of haemolysis associated with:** older age
- **Platelet count at time of investigation**: not associated with anaemia, ID or IDA
- **Bleeding severity:** significantly associated with anaemia
- **ID associated with:** premenopausal age, female sex and younger age
- **IDA associated with:** female sex

*Defined by ferritin levels <30 g/L, transferrin saturation <16% or iron supplementation at study inclusion; †Defined by Hb levels of <12 g/dL (female) and <13 g/dL (male); ‡Considered to be haptoglobin <30 mg/dL, LDH >250 U/L, bilirubin >1.2 mg/dL and reticulocytes >110 G/L.
Hb, haemoglobin; ID, iron deficiency; IDA, ID-anaemia; ITP, immune thrombocytopenia; LDH, lactate dehydrogenase; ND, newly diagnosed.
Oosterlee J, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PS2262.

New data on the impact of ITP (2)

Study information	Outcomes
• Analysis of patients with persistent and chronic ITP who had not received therapy for previous 8 weeks (n=100) vs people without ITP (age and sex matched controls; n=50)[1] • Analysis of occurrence and burden of fatigue and its relationship with cytokines[1]	• **Mean FACIT scores significantly lower in patients with ITP vs controls in the following wellbeing domains:** physical, social, emotional and functional[1] • **Mean fatigue subscale score in patients with ITP vs controls:** significantly lower ($p<0.001$)[1] • **Significant factors associated with decreased fatigue subscale scores:*** female sex ($p<0.001$), ITP duration >2 years ($p=0.021$) and IL-12 levels ($p=0.01$)[1] • **IL-12 levels showed negative correlation with several wellbeing domains:** physical ($p=0.049$), social ($p=0.003$), emotional ($p=0.000229$), functional ($p=0.048$), fatigue subscale scores ($p=0.04$) and total FACIT score ($p=0.007$)[1]
• Cross-sectional study of patients with chronic and persistent ITP in Greece (N=102)[2] • Analysis of illness perception[2] • Data collected from October 2022 to January 2025	• **Patients' HRQoL:** moderately good (mean EQ-5D-5L score 0.73; mean EQ VAS score 68.0)[2] • **Patients with PC <30 × 10^9/L vs PC >100 × 10^9/L perceived:** lower personal control ($p<0.0001$); attributed more symptoms to ITP ($p=0.021$); experienced greater concern ($p=0.034$)[2] • **Factors associated with significant negative association with patients' HRQoL:†** fatigue ($p<0.0001$); overall perceived illness burden ($p<0.0001$)[2] • **Poorer HRQoL associated with:‡** greater perceived consequences of ITP ($p=0.045$); lower perceived patient control over the disease ($p=0.018$)[2]

*After univariate linear regression analysis; †After two separate multiple linear regression models adjusting for age, gender and PC categories; ‡After a third backward multiple linear regression model. EQ-5D-5L, EuroQol 5-Dimension 5-level; EQ-VAS, EuroQol Visual Analogue Scale; FACIT, Functional Assessment of Chronic Illness Therapy; HRQoL, health-related quality of life; IL, interleukin; ITP, immune thrombocytopenia; PC, platelet count.

1. Wadhera S, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr PB0385; 2. Pontikoglou C, et al. Presented at: EHA 2025, Milan, Italy. 12–15 June 2025. Abstr PF1290.

New data on the impact of ITP (3)

Study information	Outcomes
• Retrospective analysis of treatment of patients with ITP in Hubei Province, China from January 2020 to December 2022 (N=1,033; 41.1% with chronic ITP) • Online survey using the ITP-PAQ on the impact of ITP on patient HRQoL (n=125)	**Results from retrospective analysis** • **Most common treatment:** CS and thrombopoietin drugs • **Most frequent AEs:** diarrhoea, liver dysfunction, thrombosis **Results from ITP-PAQ** • **Patients with ITP had significantly lower scores in:** fatigue, sleep, fear, exercise, work and social aspects

AE, adverse event; CS, corticosteroid; HRQoL, health-related quality of life; ITP, immune thrombocytopenia; ITP-PAQ, ITP Patient Assessment Questionnaire.

Xie Q, et al. Presented at: ISTH 2025, Washington, D.C., USA. 21–25 June 2025. Abstr PB0351.

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

Data updates
March 2025

Date of preparation: 28 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.

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.