

Insights from EHA and ISTH 2024: How can we optimize care for patients with chronic ITP?

**Data updates
March 2025**

New data on emerging ITP treatments

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 $\geq 50 \times 10^9/L$ or $\geq 30 \times 10^9/L$ and double BL:§ 9.3 weeks ≥ 2 consecutive PCs $\geq 50 \times 10^9/L$ and increased $\geq 20 \times 10^9/L$ 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: $>80 \times 10^9/L$ 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 $\geq 50 \times 10^9/L$) 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 $\geq 50 \times 10^9/L$ or $\geq 30 \times 10^9/L$ and double baseline in ≥ 4 of the last 8 weeks of treatment without rescue medication;

‡PCs $\geq 50 \times 10^9/L$ 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.

Data updates January 2025



New data on the burden of ITP

Patients (N)	Study information	Outcomes
Adults ≥18 years with persistent/chronic primary ITP initiating advanced therapies between October 2016 and April 2022 (n=1,140) matched to non-ITP patients (n=5,657)*	Observational retrospective matched cohort study in the USA to assess clinical burden of disease in patients with ITP	During average follow-up (2.3 vs 2.6 years), compared with matched population, the ITP cohort had a higher rate of: Bleed-related hospitalization (aRR 4.2 [95% CI 3.5–4.9]) Venous TE (aRR 1.7 [95% CI 1.4–2.1]) CNS arterial TEs (aRR 1.2 [95% CI 1.0–1.5]) Non-CNS arterial TEs (aRR 1.5 [95% CI 1.1–1.9]) Malignancies (RR 1.6 [95% CI 1.3–2.1]) Autoimmune conditions (RR 4.0 [95% CI 2.3–7.1]) Infections (RR 3.1 [95% CI 2.6–3.8]) New onset cognitive impairment/dementia (RR 1.7 [95% CI 1.3–2.2]) Death: 21% ITP vs 10% matched population. HR for death 1.5 (95% CI 1.2–1.7) after adjusting for potential confounders

*At baseline, ITP cohort had higher prevalence of solid tumours (15.4% vs 6.3%); infections (14.1% vs 2.7%); TEs (18.1% vs 5.5%); cardiovascular risk factors, i.e. smoking (31.6% vs 14.5%), obesity (26.8% vs 12.8%), diabetes (30.2% vs 14.3%), hypertension (65.4% vs 48.2%), CAD (25.2% vs 13.9%), and cerebrovascular disease (7.5% vs 2.6%); mental health issues (e.g. anxiety [19.7% vs 11.9%] and depression [17.7% vs 10.9%]) vs non-ITP cohort. aRR, adjusted RR; CAD, coronary artery disease; CI, confidence interval; CNS, central nervous system; HR, hazard ratio; ITP, immune thrombocytopenia; RR, rate ratio; TE, thromboembolism.

Kuter DJ, et al. Presented at: 66th ASH Annual Meeting and Exposition, 7–10 December 2024, San Diego, CA, USA. Abstr 3944.

New data on first-line treatments for ITP

Treatment (N)	Study information	Outcomes
8 RCTs with participants ≥ 16 years receiving dexamethasone (n=427) and prednisolone (n=404)	• Systematic review and meta-analysis • Search of RCTs comparing dexamethasone 40 mg/d for 4 days per cycle to prednisolone 0.5–2.0 mg/kg/d for 4 weeks	• Dexamethasone yielded higher IR rates vs prednisolone (RR 1.21, 95% CI 1.09–1.34; $I^2=52\%$, n=5 studies) • No improvement in ER, DR and PR • No significant difference in IR, DR or PR observed between 1–2 vs 3 cycles of dexamethasone • Higher frequency of AEs in dexamethasone vs prednisolone arm (n=141 vs n=71 events) • n=20 grade ≥ 3 AEs (dexamethasone n=7; prednisolone n=13) • Dexamethasone was discontinued in n=4 patients; prednisolone was discontinued in n=5 patients

Treatment response defined as platelet counts of $\geq 30 \times 10^9/L$ with at least a twofold increase of the baseline count in the absence of clinical bleeding. ER at 1 week; IR at 1 month; DR at 6 months; PR at 12 months. AE, adverse event; CI, confidence interval; DR, durable R; ER, early R; ITP, immune thrombocytopenia; IR, initial R; PR, persistent R; R, response; RCT, randomized controlled trial; RR, risk ratio.

Srisurapanont K, et al. Presented at: 66th ASH Annual Meeting and Exposition, 7–10 December 2024, San Diego, CA, USA. Abstr 2568.

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

Treatment (N)	Study information	Outcomes
Eltrombopag (N=103) ¹	- Retrospective study in China¹ - Children with primary ITP with ≥12 weeks of eltrombopag treatment and follow-up, receiving study drug between January 2020 and December 2022¹	- OR rate*: 67%; CR rate: 55.3%; R rate: 11.7%; DR rate†: 56.3%; TFR rate‡: 60.0%; relapse rate§: 36.2%; NR rate : 33.0%¹ - DR and TFR rate were significantly higher in patients with newly diagnosed vs persistent/chronic ITP: DR, 68.8% vs 45.5% (p=0.017); TFR, 76.7% vs 35% (p=0.003)¹ - Relapse rate significantly higher in patients with persistent/chronic vs newly diagnosed ITP: 57.6% vs 16.7% (p=0.000)¹ - AEs in n=14 patients; no SAEs reported; no AEs led to treatment discontinuation¹ - Patients aged 2–6 months (n=5): CR, DR and TFR rates 100%; no patients relapsed; AEs in n=3 patients¹
Eltrombopag (n=78) vs SOC [¶] (n=40) ²	- Prospective PINES trial in the USA (phase III)² - Children aged 1–<18 years with ITP <3 months and PC <30 x 10⁹/L followed for 1 year² - Data collected May 2019 to January 2024²	Primary outcome: platelet response** achieved by 63% in eltrombopag arm vs 35% in SOC arm (n=108; p=0.0054)² - Rescue therapy received by 18% vs 38% in eltrombopag arm vs SOC arm (n=117; p=0.02)² - Composite endpoint*** at 12 weeks achieved by 66% vs 44% in eltrombopag vs SOC arms (n=117; p=0.03)² - Grade ≥3 AEs at 12 weeks: Eltrombopag, n=9 AEs and n=6 SAEs; SOC, n=3 AEs and n=3 SAEs²

*Total of patients who have achieved CR and R; †PC ≥30 x 10⁹/L and at least doubling of the baseline count at 6 months; ‡PC ≥50 x 10⁹/L and the maintenance time ≥6 months after discontinuation of eltrombopag and its accompanying treatment; §patients need rescue treatment including the infusion of platelet and IVIG infusion, and using glucocorticoid either during or after discontinuation of eltrombopag treatment; ||PC <30 x 10⁹/L, or less than a twofold increase in the baseline count, or bleeding events when the patient had received an appropriate dose of eltrombopag for 8 weeks.

¶Investigators choice of one of three standard therapies (prednisone, IVIG or anti-D); **≥3 of 4 PCs >50 x10⁹/L during weeks 6–12 without rescue treatment; ***PC ≥30 x 10⁹/L and two-fold increase and no bleeding. AE, adverse event; CR, complete R; DR, durable R; ITP, immune thrombocytopenia; NR, no R; OR, overall R; PC, platelet count; PR, persistent R; R, response; SAE, serious AE; SOC, standard of care; TFR, treatment-free remission.

1. Yang L, et al. *Ann Hematol.* 2024;103:2721–7; 2. Shimano KA, et al. Presented at: 66th ASH Annual Meeting and Exposition, 7–10 December 2024, San Diego, CA, USA. Abstr 709.

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

Treatment (N)	Study information	Outcomes
Switch from eltrombopag or romiplostim to avatrombopag (N=60; n=38 switched from eltrombopag and n=22 switched from romiplostim) ^{1,2}	• Prospective study in the USA (phase IV)^{1,2} • Patients receiving prior TPO-RA for ≥90 days with any PC response^{1,2} • Patients switched due to ineffectiveness (28%), convenience (63%) and AEs (13%)^{1,2}	• TEAEs in 25% (n=15/60); serious TEAE in 10% (n=6/60)¹ • PCs improved or maintained at 90 days¹ • Significant improvement in satisfaction (TSQM domain score mean difference from baseline to day 90/EOS): for effectiveness, convenience and global satisfaction (all p<0.001); for side effects (p=0.01)¹ • Post hoc analysis (n=55): Median TSQM scores increased for convenience, effectiveness and global satisfaction for eltrombopag switchers, and for convenience and global satisfaction for romiplostim switchers at Day 90 regardless of baseline dose²

AE, adverse event; EOS, end of study; ITP, immune thrombocytopenia; PC, platelet count; TEAE, treatment-emergent AE; TPO-RA, thrombopoietin receptor agonist; TSQM, Treatment Satisfaction Questionnaire for Medication.

1. Tarantino M, et al. Abstr 2560; 2. Tarantino M, et al. Abstr 2559. All data presented at: 66th ASH Annual Meeting and Exposition, 7–10 December 2024, San Diego, CA, USA.

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

Treatment (N)	Study information	Outcomes
Avatrombopag (N=190 safety; n=18 effectiveness) ¹	- ADOPT study (phase IV)¹ - Adult patients ≥18 years with primary ITP in Europe¹ - Data cutoff 2 May 2024¹	Primary outcome: Cumulative number of weeks with PC ≥30 x 10⁹/L: mean (SD) 45.9 (10.8) weeks; median (min, max) 50.4 (5.9, 51.4) weeks¹ Cumulative number of weeks with PC ≥50 x 10⁹/L: mean (SD) 43.5 (12.7) weeks; median (min, max) 47.2 (0.0, 51.4) weeks¹ PC ≥30 x 10⁹/L and PC ≥50 x 10⁹/L for ≥8 consecutive weeks: n=17¹ All AEs / SAEs: n=29 patients / n=15 patients (n=2 discontinued treatment)¹ TRAEs: n=10 patients Improvement in HRQoL associated with treatment: Mean change in FACIT-F score at month 12 of -4.0¹
Avatrombopag (N=72) ²	- REAL-AVA 2.0 retrospective chart review study² - Adult patients with primary persistent (n=21) or chronic ITP (n=51) in the USA who initiated treatment with avatrombopag between July 2019 and June 2024² - Data cutoff 11 October 2024²	Primary outcome: 90% of patients achieved or maintained a PC ≥30 x 10⁹/L (median time to response 9.0 days) or ≥50 x 10⁹/L (median time to response 13.0² days); 85% achieved or maintained a PC ≥100 x 10⁹/L (median time to response 21.0 days)² Mean duration of response for all patients was >1 year at each PC threshold² Mean (SD) durability of response for all patients was 90% (17%) at PC ≥30 x 10⁹/L, 85% (22%) PC at ≥50 x 10⁹/L and 71% (29%) at PC ≥100 x 10⁹/L² 79% of patients on concomitant steroids at study initiation (n=15/19) discontinued their use after avatrombopag initiation; n=2/3 patients receiving concomitant immunosuppressants discontinued their use after avatrombopag initiation²

AE, adverse event; FACIT-F, Functional Assessment of Chronic Illness Therapy-Fatigue; HRQoL, health-related quality of life; PC, platelet count; ITP, immune thrombocytopenia; SAE, serious AE; SD, standard deviation; TRAE, treatment-related AE.

1. Mingot-Castellano ME, et al. Abstr 714; 2. Nagalla S, et al. Abstr 3700. All data presented at: 66th ASH Annual Meeting and Exposition, 7–10 December 2024, San Diego, CA, USA.

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

Treatment (N)	Study information	Outcomes
TPO-RA (N=48) ¹	- Open prospective, multicentre trial in France¹ - Adult patients with persistent/chronic primary ITP who achieved CR* for >3 months on a TPO-RA¹ - Enrolment between September 2017 and February 2020¹	Achieved SROT⁺ at 12 months: n=25/48¹ - Followed-up for a median of 5 years (range 4–6.3 years)¹ Achieved SROT⁺ and SCROT[‡] at the end of follow-up: 47.9% (n=23/48) and 39.6% (n=19/48), respectively, in the ITT group¹ Relapsed during extended follow-up: n=2 (no bleeds)¹
Romiplostim for 1 year, followed by tapering and follow-up for ≤1 year (N=39)	- Prospective STIP trial in the Netherlands to determine rate of SROT^{§2} - Adults with persistent/chronic ITP (77% with chronic ITP; 41% received ≥2 prior treatment lines)²	Primary outcome: SROT at 1 year after tapering (n=25): 23.6%² Patients with SROT had higher PCs and received lower doses of romiplostim² Median time to relapse 58 days² Only mild bleeding reported during/after tapering in 41.2% (n=7/17) in patients who relapsed²

*PC >100 x 10⁹/L for >2 months; [†]PC ≥30 x 10⁹/L, no bleeding without ITP-specific medications; [‡]PC ≥100 x 10⁹/L, no bleeding without ITP-specific medications; [§]PC >30 x 10⁹/L, no bleeding symptoms and no need for treatment. CR, complete response; ITP, immune thrombocytopenia; ITT, intent-to-treat; PC, platelet count; SCROT, sustained CR off-treatment; SROT, sustained rate of remission off-treatment; TPO-RA, thrombopoietin receptor agonist.

1. Cottu A, et al. *Blood*. 2024. DOI: 10.1182/blood.2024025707. Epub ahead of print; 2. Nelson VS, et al. Presented at: 66th ASH Annual Meeting and Exposition, 7–10 December 2024, San Diego, CA, USA. Abstr 2554.

New data on emerging ITP treatments (1)

Treatment (N)	Study information	Outcomes
Rilzabrutinib n=133; placebo n=69 ^{1,2}	- LUNA 3 trial (phase III)^{1,2} - Data from patients ≥18 years with persistent/chronic primary ITP^{1,2} - Data cutoff 14 March 2024^{1,2}	Primary outcome: Durable response* at week 25 was met (23% difference between rilza vs placebo (95% CI 16–30%; p<0.0001))¹ Duration of PR†: significantly longer all patients and responders receiving rilza vs placebo (p<0.0001 for both)¹ Significantly less rescue therapy use associated with rilzabrutinib vs placebo (p=0.0007)¹ Similar proportion of AEs and SAEs¹
		Significant improvement in physical fatigue from baseline to week 13 (p=0.0114) and week 25 (p=0.0003) with rilza vs placebo (assessed by LS mean change)² Improvements in multiple measures of ITP-specific HRQoL at week 25 observed with rilza vs placebo (symptoms, bother-physical health, activity, psychological health, social activity and overall HRQoL)²
All soveplelenib (N=179) vs crossover from placebo (n=53) ³	- Long-term extension of ESLIM-01 (phase III)³ - Adult patients³ - Data cutoff 31 January 2024³	Overall response rate‡: 81.0% vs 83.0% (all soveplelenib vs crossover)³ Durable response rate§: 51.4% vs 43.4% (all soveplelenib vs crossover)³ Long-term durable response§: 59.8% vs 64.2% (all soveplelenib vs crossover)³ Most common TRAEs (≥grade 3): ↑ ALT (2.2%); ↓ neutrophil count (1.7%); ↑ GGT (1.7%)³

*PC ≥50 x 10⁹/L for ≥two-thirds of ≥8 of the last 12 of 24 weeks without receiving rescue therapy; †PC ≥50 x 10⁹/L or ≥30–<50 x 10⁹/L and doubled from baseline; ‡≥1 PC ≥50 x 10⁹/L with soveplelenib not impacted by rescue treatment; §PC ≥50 x 10⁹/L at ≥4 of 6 scheduled visits during weeks 14–24 in ESLIM-01 not impacted by rescue treatment, or PC ≥50 x 10⁹/L at 2 of 3 protocol-defined visits during the second 12 weeks of 24 weeks in the open-label sub-study not impacted by rescue treatment. AE, adverse event; ALT, alanine aminotransferase; CI, confidence interval; GGT, gamma-glutamyltransferase; HRQoL, health-related quality of life; ITP, immune thrombocytopenia; LS, least squares; PC, platelet count; PR, platelet response; SAE, serious AE; TRAE, treatment-related AE.

1. Kuter DJ, et al. Abstr 5; 2. Ghanima W, et al. Abstr 2552; 3. Hu Y, et al. Abstr 2558. All data presented at: 66th ASH Annual Meeting and Exposition, 7–10 December 2024, San Diego, CA, USA.

New data on emerging ITP treatments (2)

Treatment (N)	Study information	Outcomes
Avatrombopag (n=54) vs placebo (n=21) ¹	• AVA-PED-301 (phase III)¹ • Children aged 1–17 years with primary ITP ≥6 months¹ • Core phase data previously reported (12 weeks)¹	• Primary endpoint: durable PR (previously reported)¹ • Clinically meaningful response (PC ≥30 x 10⁹/L): 92.6% vs 19.1%¹ • Achieving a response (PC ≥50 x 10⁹/L) at any point in core phase: 88.9% vs 9.5%¹
Ianalumab (N=10) ²	• VAYHIT3 (phase II)² • Adults with primary ITP previously treated with at least a CS and a TPO-RA, with no prior splenectomy² • Data cutoff 12 June 2024²	• Primary endpoint: ConfR* achieved in n=5/10 patients² • Achieved ConfR* and stable response†: n=4² • Median best post-baseline PC in all 10 patients was 129.0 x 10⁹/L (range 3–709)² • Patients experiencing AEs / grade ≥3 AEs: n=10 / n=3² • Patients experiencing SAEs / grade ≥3 SAEs: n=2 / n=2²

*Defined as a PC ≥50 x 10⁹/L at two or more consecutive assessments at least 7 days apart between week 1 and week 25, in the absence of rescue treatment for ≥4 weeks prior to PC assessment and start of new ITP treatment before reaching a ConfR. †Stable response defined as proportion of patients with ≥75% PCs collected between study days 121 and 183 ≥50 x 10⁹/L in the absence of rescue treatment/new ITP treatment. AE, adverse event; ConfR, confirmed response; CS, corticosteroid; ITP, immune thrombocytopenia; PC, platelet count; PR, platelet response; SAE, serious AE; TPO-RA, thrombopoietin receptor agonist.

1. Grace RF, et al. Abstr 1191; 2. Kuter DJ, et al. Abstr 710. All data presented at: 66th ASH Annual Meeting and Exposition, 7–10 December 2024, San Diego, CA, USA.