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

Data updates
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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 ≥30 x 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 China[1] • Children with primary ITP with ≥12 weeks of eltrombopag treatment and follow-up, receiving study drug between January 2020 and December 2022[1]	• **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 discontinuation**[1] • **Patients aged 2–6 months (n=5): CR, DR and TFR rates 100%; no patients relapsed; AEs in n=3 patients**[1]
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 10^9/L followed for 1 year[2] • Data collected May 2019 to January 2024[2]	• **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 SAEs[2]

*Total of patients who have achieved CR and R; †PC ≥30 x 10^9/L and at least doubling of the baseline count at 6 months; ‡PC ≥50 x 10^9/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 10^9/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 x10^9/L during weeks 6–12 without rescue treatment; ***PC ≥30 x 10^9/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 days[1] • **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 dose[2]

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 Europe[1] • Data cutoff 2 May 2024[1]	• **Primary outcome: Cumulative number of weeks with PC ≥30 x 10^9/L**: mean (SD) 45.9 (10.8) weeks; median (min, max) 50.4 (5.9, 51.4) weeks[1] • **Cumulative number of weeks with PC ≥50 x 10^9/L**: mean (SD) 43.5 (12.7) weeks; median (min, max) 47.2 (0.0, 51.4) weeks[1] • **PC ≥30 x 10^9/L and PC ≥50 x 10^9/L for ≥8 consecutive weeks:** n=17[1] • **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 -4.0[1]
Avatrombopag (N=72)[2]	• REAL-AVA 2.0 retrospective chart review study[2] • 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 2024[2] • Data cutoff 11 October 2024[2]	• **Primary outcome: 90% of patients achieved or maintained a PC ≥30 x 10^9/L** (median time to response 9.0 days) **or ≥50 x 10^9/L** (median time to response 13.0[2] days)**; 85% achieved or maintained a PC ≥100 x 10^9/L** (median time to response 21.0 days)[2] • **Mean duration of response** for all patients was >1 year at each PC threshold[2] • **Mean (SD) durability of response** for all patients was 90% (17%) at PC ≥30 x 10^9/L, 85% (22%) PC at ≥50 x 10^9/L and 71% (29%) at PC ≥100 x 10^9/L[2] • 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 initiation[2]

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 France[1] • Adult patients with persistent/chronic primary ITP who achieved CR* for >3 months on a TPO-RA[1] • Enrolment between September 2017 and February 2020[1]	• **Achieved SROT[†] at 12 months:** n=25/48[1] • 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 group[1] • **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 romiplostim[2]** • **Median time to relapse** 58 days[2] • **Only mild bleeding reported during/after tapering** in 41.2% (n=7/17) in patients who relapsed[2]

*PC >100 x 10^9/L for >2 months; [†]PC ≥30 x 10^9/L, no bleeding without ITP-specific medications; [‡]PC ≥100 x 10^9/L, no bleeding without ITP-specific medications; [§]PC >30 x 10^9/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 SAEs**[1] • **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 **sovleplenib** (N=179) vs crossover from placebo (n=53)[3]	• Long-term extension of ESLIM-01 (phase III)[3] • Adult patients[3] • Data cutoff 31 January 2024[3]	• **Overall response rate‡:** 81.0% vs 83.0% (all sovleplenib vs crossover)[3] • **Durable response rate§:** 51.4% vs 43.4% (all sovleplenib vs crossover)[3] • **Long-term durable response§:** 59.8% vs 64.2% (all sovleplenib vs crossover)[3] • **Most common TRAEs (≥grade 3):** ↑ ALT (2.2%); ↓ neutrophil count (1.7%); ↑ GGT (1.7%)[3]

*PC ≥50 x 10^9/L for ≥two-thirds of ≥8 of the last 12 of 24 weeks without receiving rescue therapy; †PC ≥50 x 10^9/L or ≥30–<50 x 10^9/L and doubled from baseline; ‡≥1 PC ≥50 x10^9/L with sovleplenib not impacted by rescue treatment; §PC ≥50 x10^9/L at ≥4 of 6 scheduled visits during weeks 14–24 in ESLIM-01 not impacted by rescue treatment, or PC ≥50 x10^9/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 months[1] • Core phase data previously reported (12 weeks)[1]	• **Primary endpoint: durable PR (previously reported)[1]** • **Clinically meaningful response (PC ≥30 x 10^9/L):** 92.6% vs 19.1%[1] • **Achieving a response (PC ≥50 x 10^9/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 splenectomy[2] • Data cutoff 12 June 2024[2]	• **Primary endpoint: ConfR* achieved in n=5/10 patients[2]** • **Achieved ConfR* and stable response†:** n=4[2] • **Median best post-baseline PC** in all 10 patients was 129.0 x 10^9/L (range 3–709)[2] • **Patients experiencing AEs / grade ≥3 AEs:** n=10 / n=3[2] • **Patients experiencing SAEs / grade ≥3 SAEs:** n=2 / n=2[2]

*Defined as a PC ≥50 x 10^9/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 10^9/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.