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

Practice aid for ITP

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Guideline recommended treatments for ITP

Initial therapies

- **Corticosteroids**[1,2]
- IVIg[2]
- Anti-D Ig[2]

Second line onwards[1–3]

TPO-RAs
- Eltrombopag
- Romiplostim
- Avatrombopag

Anti-CD20
- Rituximab (off label)

Syk inhibitor
- Fostamatinib

Splenectomy

There are limited options for patients who are refractory/intolerant to standard therapies[4]

Factors to consider when selecting second- or later-line treatments

- Potential for a **durable treatment response**[1]
- **Risks and benefits** of withholding or administering treatment[5]
- Potential treatment **side effects**[5]
- Method of treatment **administration**[1,6]
- **Treatment adherence**[1]
- **Dietary restrictions** associated with any treatments[6]

Emerging therapies for ITP

Anti-CD38 mAb[4]
- Daratumumab
- CM313
- Mezagitamab

BAFF pathway inhibitor[4]
- Ianalumab
- Povetacicept

BTK inhibitor[4]
- Rilzabrutinib

Syk inhibitor[4]
- Cevidoplenib
- Sovleplenib

FcRn antagonist[4]
- Efgartigimod

Data presented at the 66th ASH Annual Meeting and Exposition

	VAYHIT3 (phase II)[7]	LUNA 3 (phase III)[8]	ESLIM-01 extension stage (phase III)[9]
Treatment arms	**Ianalumab** (N=10) Four doses: 9 mg/kg Q4W IV	Randomized 2:1 **rilzabrutinib** (n=133) vs placebo (n=69) 400 mg BID	All **sovleplenib** (n=179) vs crossover from placebo (n=53) 300 mg QD
Key efficacy results	• **ConfR:*** n=5 • **ConfR* + stable response:**† n=4 • **Median best post-BL PC:** 129.0 x 10^9/L	• **DR:**‡ 23% vs 0% (p<0.0001) • **Duration of PR:**§ longer with rilza vs PBO (p<0.0001) • **Rescue therapy required:** lower with rilza vs PBO (p=0.0007)	• **OR:**¶ 81.0% vs 83.0% • **DR:**‖ 51.4% vs 43.4% • **Long-term DR:**** 59.8% vs 64.2% • **Received rescue therapy:** 22.9% vs 18.9%
Key safety results	**Patients with:** **Any AE,** n=10; **grade ≥3,** n=3 **Any SAE,** n=2; **grade ≥3,** n=2	**Similar incidence of AEs and SAEs**	**Most common grade ≥3 TRAEs:** ↑ ALT (2.2%), ↓ neutrophil count (1.7%), ↑ GGT (1.7%)

Direct comparisons between trials should not be made due to differences in trial design.

*PC ≥50 x 10^9/L at ≥2 consecutive assessments ≥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; ‡PC ≥50 x 10^9/L for ≥two-thirds of ≥8 of the last 12 weeks of the 24-week blinded treatment period in the absence of rescue medication; §PC ≥50 x 10^9/L or 30–<50 x 10^9/L and >2 x BL; ¶≥1 PC ≥50 x 10^9/L with sovleplenib not impacted by rescue treatment; ‖PC ≥50 x10^9/L at ≥4 of 6 scheduled visits between weeks 14 and 24 in ESLIM-01 not impacted by rescue treatment, or PC ≥50 x 10^9/L at 2 or 3 protocol-defined visits during the second 12 weeks of 24 weeks in the open-label sub-study not impacted by rescue treatment; **after receiving sovleplenib for 12 weeks, PC ≥50 x 10^9/L at ≥2 of 3 of any 12-week consecutive protocol defined visits not impacted by rescue treatment.

The real-world impact of ITP

Symptomatic bleeding affects **60–70%** of patients with **chronic ITP** and **70–80%** of patients with **newly diagnosed ITP**[10]

ITP **impacts** patients' **psychological** and **emotional wellbeing**[13,14]

Patients may have **concerns over the risk of bleeding**[11] and **may have to alter their lifestyles** to reduce bleeding risk[10]

Patients can experience **fatigue and cognitive impairment** that can **decrease participation in activities and work**[13,15]

Heavy menstrual bleeding is common in female patients with ITP and **often impacts daily life**[12]

Adults living with chronic ITP have an **increased risk of thrombosis and thromboembolism** compared with the general population[16,17]

Platelet count does not fully correlate with disease burden[18]

Patient support groups can help educate patients with ITP, and provide resources and support[19–21]

- Platelet Disorder Support Association
- International ITP Alliance
- ITP Support Association

Abbreviations and references

Abbreviations

AE, adverse event; ALT, alanine aminotransferase; ASH, American Society of Hematology; BAFF-R, B-cell activating factor; BID, twice daily; BL, baseline; BTK, Bruton's tyrosine kinase; CD, cluster of differentiation; ConfR, confirmed response; DR, durable R; GGT, gamma-glutamyltransferase; Ig, immunoglobulin; ITP, immune thrombocytopenia; IV, intravenous; mAb, monoclonal antibody; OR, overall response; PBO, placebo; PC, platelet count; PR, platelet response; Q4W, once every 4 weeks; QD, once daily; Rilza, rilzabrutinib; SAE, serious AE; Syk, spleen tyrosine kinase; TPO-RA, thrombopoietin receptor agonist; TRAE, treatment-related AE.

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