# ITP Clinical Research Highlights: ISTH 2025

Shruti Chaturvedi, MBBS, MS

Assistant Professor of Medicine at Johns Hopkins

Baltimore, MD

# Continuing Education Information


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Learning Objective: Describe the latest research about ITP presented at ISTH 2025 and its clinical relevance in real-world settings.

Planner/Faculty Educator Dr. Chaturvedi discloses the following relevant financial relationships with ineligible companies:

- Scientific Advisory Board/Consultant: Sanofi, Takeda, Sobi, argenx, Star Pharma, RallyBio, Novartis, Alexion
- Grant/Research Support: Sanofi, Sobi, argenx

Planners and reviewers for this activity have no relevant financial relationships with any ineligible companies.

All relevant financial relationships listed have been mitigated. This activity will discuss off-label uses and investigational agents.

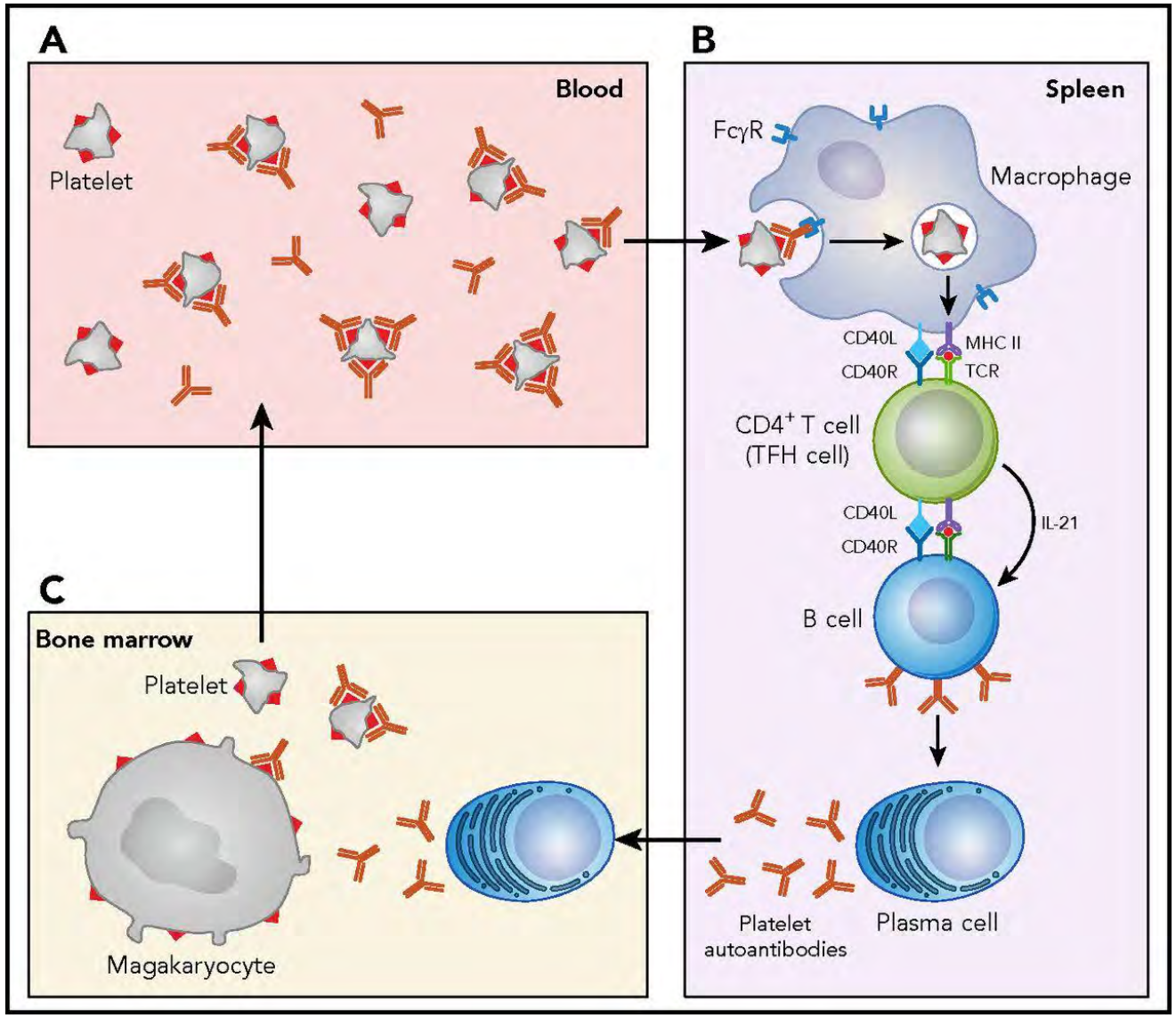
This accredited continuing education program is supported by an educational grant from Sanofi.

# Immune Thrombocytopenia (ITP)

- ITP is a common cause of isolated thrombocytopenia
- Caused by antibody mediated destruction of platelets
- ITP is a diagnosis of exclusion – need to rule out other causes of thrombocytopenia
  - History and physical
  - Labs
  - **No single diagnostic test**
- Several targeted therapies are in development (or recently approved), and their data was presented at ISTH 2025

# Pathogenesis of ITP

Antibody mediated phagocytosis and destruction of platelets



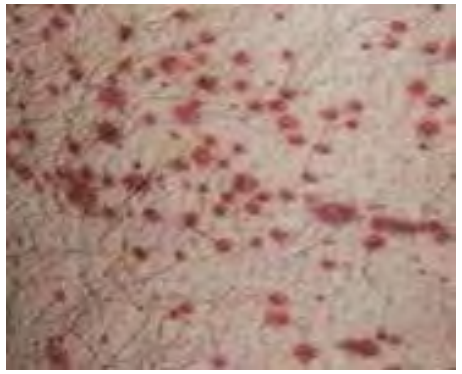

Some anti-platelet Ab can cause dysfunction and apoptosis of megakaryocytes in BM

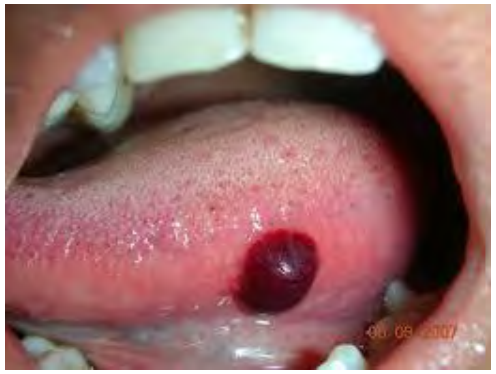
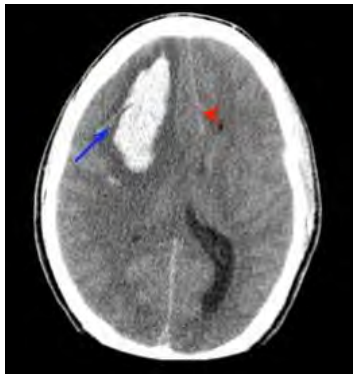
PLUS Autoreactive cytotoxic T cells

Chaturvedi et al. Blood 2018;131:1172-1182

# ITP – Clinical Presentation

- Bleeding is the primary symptom and depends on platelet count



- Some patients also report fatigue and poor health related quality of life

Newton et al. Eur J Haematol. 2011;86:420-429
Hill and Newland. British J Haematol. 2015;170:141

# Clinical Management

# ITP Treatment is Focused on Reducing Bleeding Risk

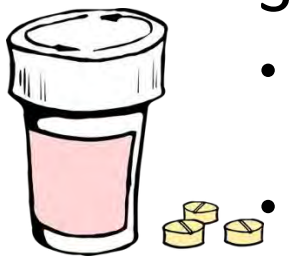
Factors that contribute to management decisions include:

- Extent of bleeding
- Comorbidities (e.g. cirrhosis) or medications predisposing to bleeding
- Potential interventions that may cause bleeding
- Side effects of specific therapies
- Activity and lifestyle
- Patient expectations/worry or anxiety about disease burden

Provan D, et al. *Blood.* 2010;115(2):168-186.

# First Line / Rescue Treatments

IVIG 2 g/kg
over 2 or 4 days



Steroids

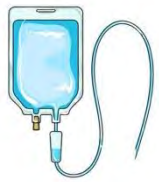
- Prednisone 1 mg/kg with taper
- Dexamethasone 40 mg daily x 4 days

- Response rates are > 70-90% : lack of response to IVIG or steroids should make you reconsider diagnosis
- IVIG may have slightly quicker response (1-3 days vs. 2-14 days)
- Can use together for critically low platelets or bleeding

Godeau et al. Lancet 2002;359(9300):23

# What If Steroids or IVIG Are Not Enough?

**Rituximab**

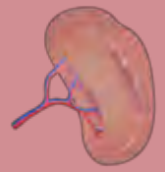


(anti-CD20 on B cells, antibody production)

**Thrombopoietin mimetic**

Increase platelet production

**Splenectomy**



Remove site of platelet destruction

All other immunosuppression, fostamatinib, etc.
Combination therapy

- No clear best treatment – no head to head comparison in RCTs
- Individualize treatment

Ghanima et al. Blood 2012;120:960-969

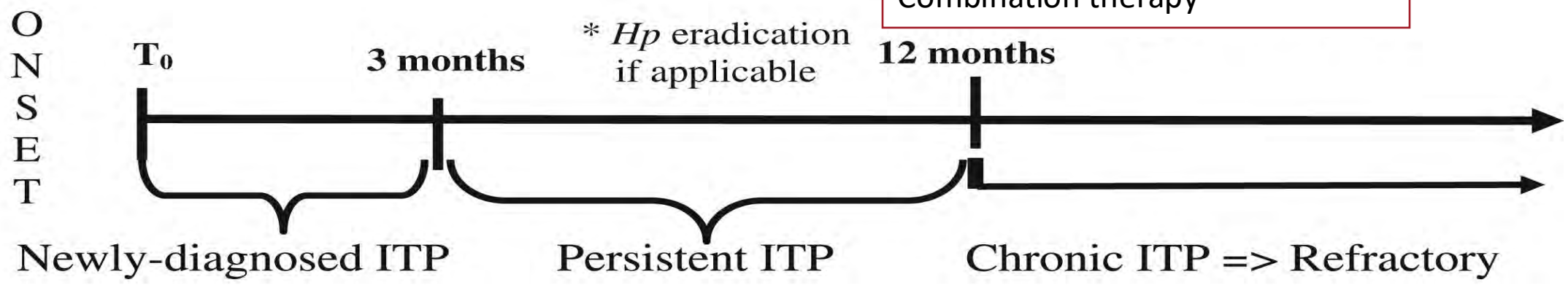
# Treatments Used in Different Phases of ITP

**Initial / rescue therapy**
Corticosteroids
Intravenous Ig
Anti-D
Splenectomy

**Usual next line treatments (ROBUST EVIDENCE)**
TPO-receptor agonists
Rituximab
Fostamatinib
Splenectomy (after 12 months)

**Salvage / refractory treatments (LESS ROBUST EVIDENCE)**
Azathioprine
Cyclosporin A
Cyclophosphamide
Danazol
Dapsone
HSCT
Mycophenolate mofetil
Vinca alkaloids
Combination therapy

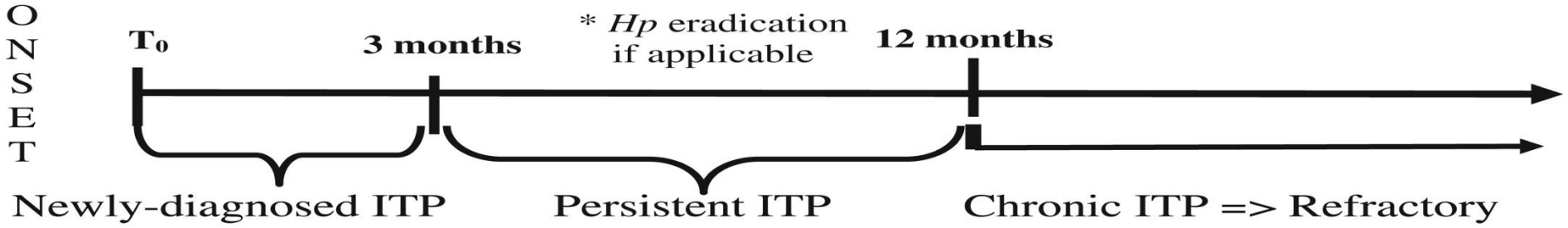
**Emerging therapipes**
Efgartigimod
**Rilzabrutinib***
Rozanolixizumab
Sutimlimab
Anti CD38




*Approved in August, 2025 for treating adults with ITP

Neunert CE, et al. *Blood*. 2011;117(16):4190-207. N eunert CE, et al. *Hematology Am Soc Hematol Educ Program.* 2018;2018(1):568-575. Provan D, et al. *Blood.* 2010;115(2):168-186. Salama A. *Expert Opin Emerg Drugs.* 2017;22(1):27-38. Dou X, Yang R. *Expert Rev Hematol.* 2019;12(9):723–732.

# Unmet Needs in Different Phases of ITP




- Steroid side effects
- Low rates of durable response with first line therapies

- Fatigue
- Poor quality of life

- Adverse effects of treatments

- Lack of well tolerated treatment options for refractory ITP that lead to durable responses

# Case Example

A 38 yo M presents with history of relapsed primary ITP, most recently treated with fostamatinib, presents with platelet count of 12 x $10^9$/L. He responds to corticosteroids and IVIG. He has previously received rituximab, romiplostim, eltrombopag, avatrombopag, mycophenolate, and cyclosporin without response. He prefers an oral treatment.

You recommend:

a) Splenectomy due to lack of effective oral agents

b) Rilzabrutinib (recently approved)

c) Lanalumab (once available)

d) Daratumumab

# Platelet responses per IWG criteria for LUNA3 rilzabrutinib vs placebo in primary ITP patients.

Ghanima W et al. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

Kuter DJ et al. Efficacy and safety of oral bruton tyrosine kinase inhibitor (BTKi) rilzabrutinib in adults with previously treated immune thrombocytopenia (ITP): A phase 3, placebo-controlled, parallel-group, multicenter study (LUNA 3). *Blood.* 2024;144 (Supplement 1): 5.

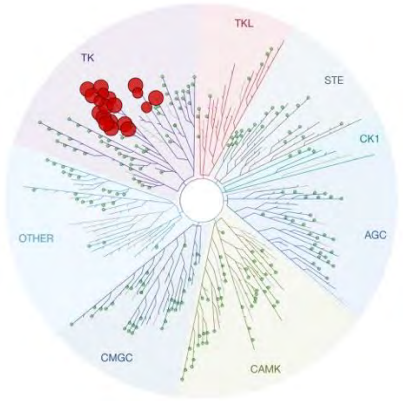
# Rilzabrutinib - Novel Oral BTK Inhibitor for ITP

**Pathophysiologic Mechanisms in ITP**

| | B cells, plasma cells | Monocyte, macrophage | Mast cells, basophils | Neutrophils | T cells |
|---|---|---|---|---|---|
| Effect of BTK Inhibition | Blocks B-cell receptor<br>Inhibits plasma cell differentiation and antibody production | Blocks IgG-mediated FcγR activation, phagocytosis, inflammatory mediators | Blocks IgE-mediated FcεR activation and degranulation | Inhibits activation, adhesion, recruitment, oxidative burst | No effect |
| | **BTK inhibition** | | | | |

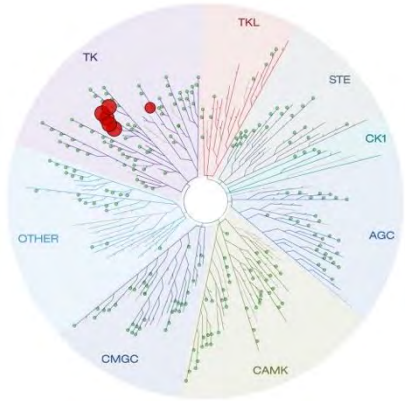
More selective – no inhibition of platelet function

**Ibrutinib Kinase Selectivity**




**21 kinases inhibited >90%**

**Rilzabrutinib Kinase Selectivity**

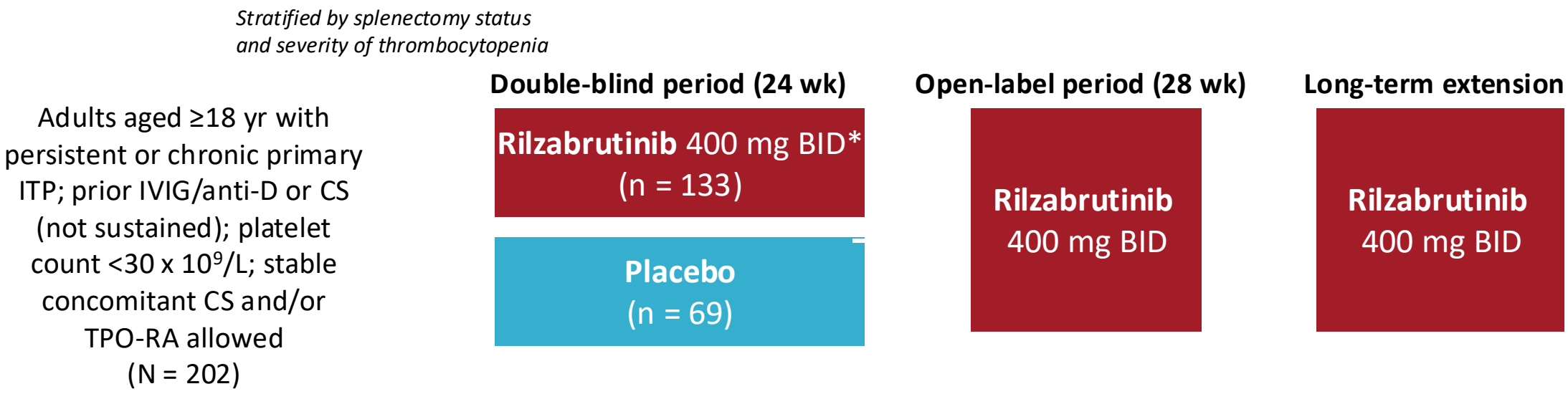



**6 kinases inhibited >90%**

Langrish C, et al. *JID (ESDR).* 2019;139:S216 (abstract 011). 2. Bradshaw JM, et al. *Nat Chem Biol.* 2015;11:525-531.

# LUNA 3: Study Design

- Multicenter, double-blind, placebo-controlled, randomized phase III trial




*At 13 wk, nonresponders could discontinue or proceed to open-label rilzabrutinib.

- **Primary endpoint:** durable response at 25 Wk (defined as plt count ≥50 x $10^9$/L for ≥2/3 of the last 12 weekly visits without rescue therapy
- **Key Secondary endpoints:** initial plt response (plt count ≥50 x $10^9$/L or ≥30 to <50 x $10^9$/L and doubled from baseline); use of ITP rescue therapy, effect on Wk 13 fatigue and Wk 25 bleeding, TEAE

Kuter DJ et al. *Blood.* 2024;144 (Supplement 1): 5.

# LUNA 3: Baseline Characteristics

| Characteristic | Rilzabrutinib (n = 133) | Placebo (n = 69) |
|---|---|---|
| Median age, yr (range) | 47 (18-80) | 46 (19-79) |
| Female, n (%) | 78 (59) | 49 (71) |
| Median duration of ITP, yr (range)* | 8.1 (0.3-52.2) | 6.2 (0.3-35.8) |
| Median baseline platelet count, x $10^9$/L (range) | 15 (1-32) | 15 (1-54) |
| Median number of prior ITP therapies, n (range)<br>▪ ≥5 prior therapies, n (%) | 4 (1-15)<br>57 (43) | 5 (1-12)<br>36 (52) |
| Prior splenectomy, n (%) | 37 (28) | 19 (28) |
| Prior TPO-RA, n (%) | 88 (66) | 51 (74) |

*Average first and second qualifying screening platelet counts and study Day 1 platelet count.

Kuter DJ et al. *Blood.* 2024;144 (Supplement 1): 5.

# LUNA 3: Efficacy

| Parameter | Rilzabrutinib (n = 133) | Placebo (n = 69) | *P* Value |
|---|---|---|---|
| Durable response at Wk 25 (platelet count ≥50 x $10^9$/L for ≥2/3 of the last 12 weekly visits without rescue therapy), % | 31 (23%) | 0 | <.0001 |
| Initial platelet response (platelet count ≥50 x $10^9$/L or ≥30 to <50 x $10^9$/L and doubled from baseline), n (%) | 85 (65%) | 23 (33%) | |
| Median time to platelet response, days | 15 | 50 | |
| Median duration of platelet response, wk | 12 | 2 | <.0001 |

Kuter DJ et al. *Blood.* 2024;144 (Supplement 1): 5.

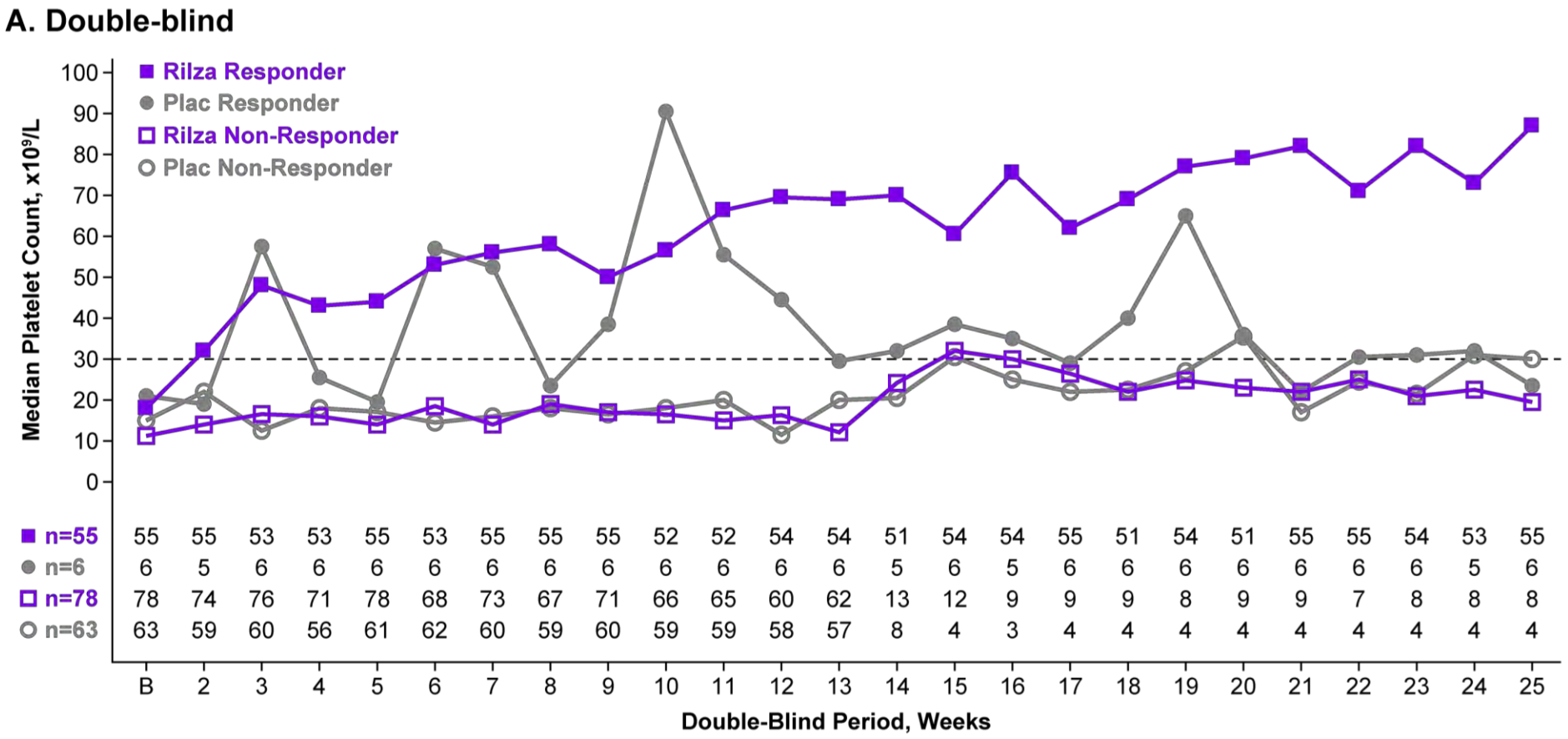
# Rilzabrutinib vs Placebo, IWG-Defined Platelet Response

- **Durable IWG response** = platelet count ≥30×$10^9$/L and at least doubled from baseline in the absence of bleeding for ≥50% of assessments during double-blind and open-label periods

| Double-Blind Period | Rilzabrutinib | Placebo |
|---|---|---|
| Double-blind treatment (p<0.0001) | 55/133 (41%) | 6/69 (9%) |
| Number of prior ITP therapies<br>1-4<br>≥5 | <br>16/34 (47%)<br>39/99 (39%) | <br>2/13 (15%)<br>4/56 (7%) |
| **Open-Label Period** | | |
| All open-label pts | 100/180 (56%) | - |
| Initial double-blind treatment<br>Rilzabrutinib<br>Placebo | <br>61/115 (53%)<br>39/65 (60%) | <br>-<br>- |
| Number of prior ITP therapies<br>1-4<br>≥5 | <br>23/40 (58%)<br>77/140 (55%) | <br>-<br>- |
| **Double-Blind or Open-Label Period** | | |
| Initial double-blind assignment<br>Rilzabrutinib | <br>73/115 (63%) | - |
| Number of prior ITP therapies<br>1-4<br>≥5 | <br>20/27 (74%)<br>53/88 (60%) | <br>-<br>- |

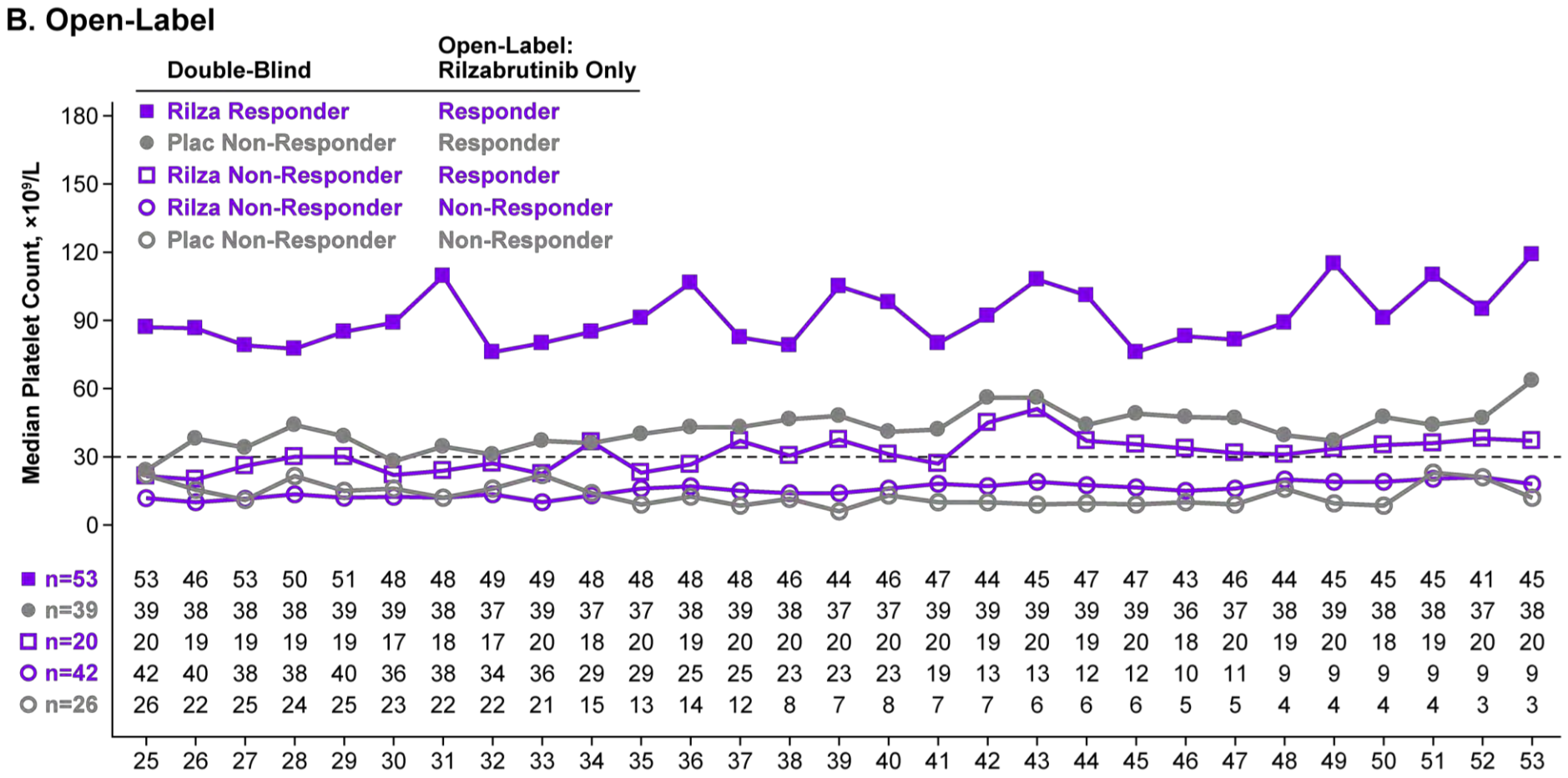
Kuter DJ et al. *Blood.* 2024;144 (Supplement 1): 5.
Ghanima W et al.. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Median Platelet Counts During the Double-blind Period (IWG Durable Response Status)




Ghanima W et al.. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Median Platelet Counts During the Open Label Period (IWG Durable Response Status)




Ghanima W et al.. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# 2552 Improved Health-Related quality of Life (HRQoL) with Oral Bruton Tyrosine Kinase Inhibitor (BTKi) Rilzabrutinib Vs Placebo in Adults with Previously Treated Immune Thrombocytopenia (ITP): Phase 3 Luna 3 Multicenter Study

Program: Oral and Poster Abstracts
Session: 311. Disorders of Platelet Number or Function: Clinical and Epidemiological: Poster II
Hematology Disease Topics & Pathways:
Research, Bleeding and Clotting, Adult, Platelet disorders, Diseases, Thrombocytopenias, Immune mechanism, Biological Processes, Study Population, Human

Sunday, December 8, 2024, 6:00 PM-8:00 PM

***Waleed Ghanima, MD, PhD***[1], *Howard A. Liebman, MD, MA*[2], *Yu Hu*[3*], *Yoshitaka Miyakawa, MD*[4*], *Nichola Cooper, MD*[5*], *Güray Saydam, MD*[6*], *Marie Luise Hütter-Krönke, MD*[7*], *Sylvain Audia*[8*], *Mengjie Yao*[9*], *Ahmed Daak, MD, PhD, MSc, DPM/MFPM*[10], *Imene Gouia, PharmD, MSc Health Economics*[11*], *Matias Cordoba, MD*[12*] *and David J Kuter, MD, DPhil*[13]

# LUNA 3: Secondary Endpoints

- Other endpoints significantly improved with rilzabrutinib vs placebo:
  - Longer duration of platelet response in all patients (*P* <.0001) and in responders (*P* <.0001)
  - Less use of rescue therapy (*P* = .0007)
  - **Decreased IBLS bleeding score at 25 Wk (*P* = .0006)**
  - Improvements in fatigue

| Fatigue* | Rilzabrutinib | Placebo |
|---|---|---|
| Week 13 | +8.0 | –0.1% (p=0.01) |
| Week 25 | +4.7 | –7.3 (p=0.0003) |

*from baseline to week 25

Kuter DJ et al. *Blood.* 2024;144 (Supplement 1): 5.

# LUNA 3: Safety Summary

- Most safety events were of grade 1/2 (vs placebo)

| | | |
|---|---|---|
| Diarrhea | 23% | 4% |
| Nausea | 17% | 6% |

*from baseline to week 25

- Rilzabrutinib arm: 2 rilzabrutinib-related SAEs
  - 1 grade 4 neutropenia event lasting 14 days (no infection or change in treatment)
  - 1 grade 3 serious AE of a pulmonary embolism in the lower left leg in a patient with multiple risk factors (treatment discontinued)

Kuter DJ et al. *Blood.* 2024;144 (Supplement 1): 5.

# LUNA 3: Conclusions

- High response rate (65%) in previously treated ITP
  - 31% durable response at Wk 25 (plt count ≥50 x $10^9$/L for ≥2/3 of the last 12 weekly visits without rescue therapy) vs 0% placebo (*P* <.0001)
  - 15 days to response (median)
- 83% response rate in 2$^{nd}$ line setting (higher than reported for rituximab)
- Rilzabrutinib also showed improvement for all secondary endpoints – bleeding, fatigue, QOL!
- Well tolerated with no BTK inhibitor class effects in adults with difficult to treat ITP

Kuter DJ et al. *Blood.* 2024;144 (Supplement 1): 5.

# Real World

# Real-world outcomes of avatrombopag treatment in primary ITP stratified by prior TPO-RA exposure.

Nagalla S et al. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# TPO receptor agonists

| Agent | Romiplostim[1,2a] | Eltrombopag[3,4a] | Avatrombopag[5,6] |
|---|---|---|---|
| MOA | • Unique platform peptibody<br>• Binds to ligand binding site of TPO receptor<br>• Activates platelet production | • Small molecule<br>• Binds to transmembrane portion of TPO receptor<br>• Initiates signaling cascades that induce proliferation and differentiation from bone marrow progenitor cells | |
| Initial Dose | SC injection: 1 mcg/kg given weekly | PO: 50 mg once daily | PO: 20 mg once daily |
| Food interactions | N/A | Yes | No |
| Hepatotoxicity | No | Yes – monitor hepatic function | No |
| FDA approved | Aug 2008 | Nov 2008 | June 2019 |
| Indication | Immune thrombocytopenia (adults and children) | • Immune thrombocytopenia (adults and children)<br>• Hepatitis C-associated thrombocytopenia<br>• Severe aplastic anemia | • Periprocedural thrombocytopenia in CLD patients[b]<br>• Immune thrombocytopenia (adults)[c] |

CLD, chronic liver disease; MOA, mechanism of action; PO, orally by mouth; SC, subcutaneous; TPO, thrombopoietin. [a]Also indicated for pediatric patients 1 year and older. [b]FDA approval for CLD in May 2018. [c]FDA approval for chronic ITP in June 2019. 1. Bussel JB, et al. *N Engl J Med.* 2006;355(16):1672-1681. 2. Romiplostim Package Insert. 2019. 3. Bussel JB, et al. *N Engl J Med.* 2007;357(22):2237-2247. 4. Eltrombopag Package Insert. 2019. 5. Bussel JB. *Br J Haematol.* 2018;183(3):342-343. 6. Avatrombopag Package Insert. 2019.

# Avatrombopag Response in Primary ITP with vs without Prior TPO-RA Use

Assessed real-world avatrombopag (AVA) response in patients with primary ITP with or without prior TPO-RA exposure.

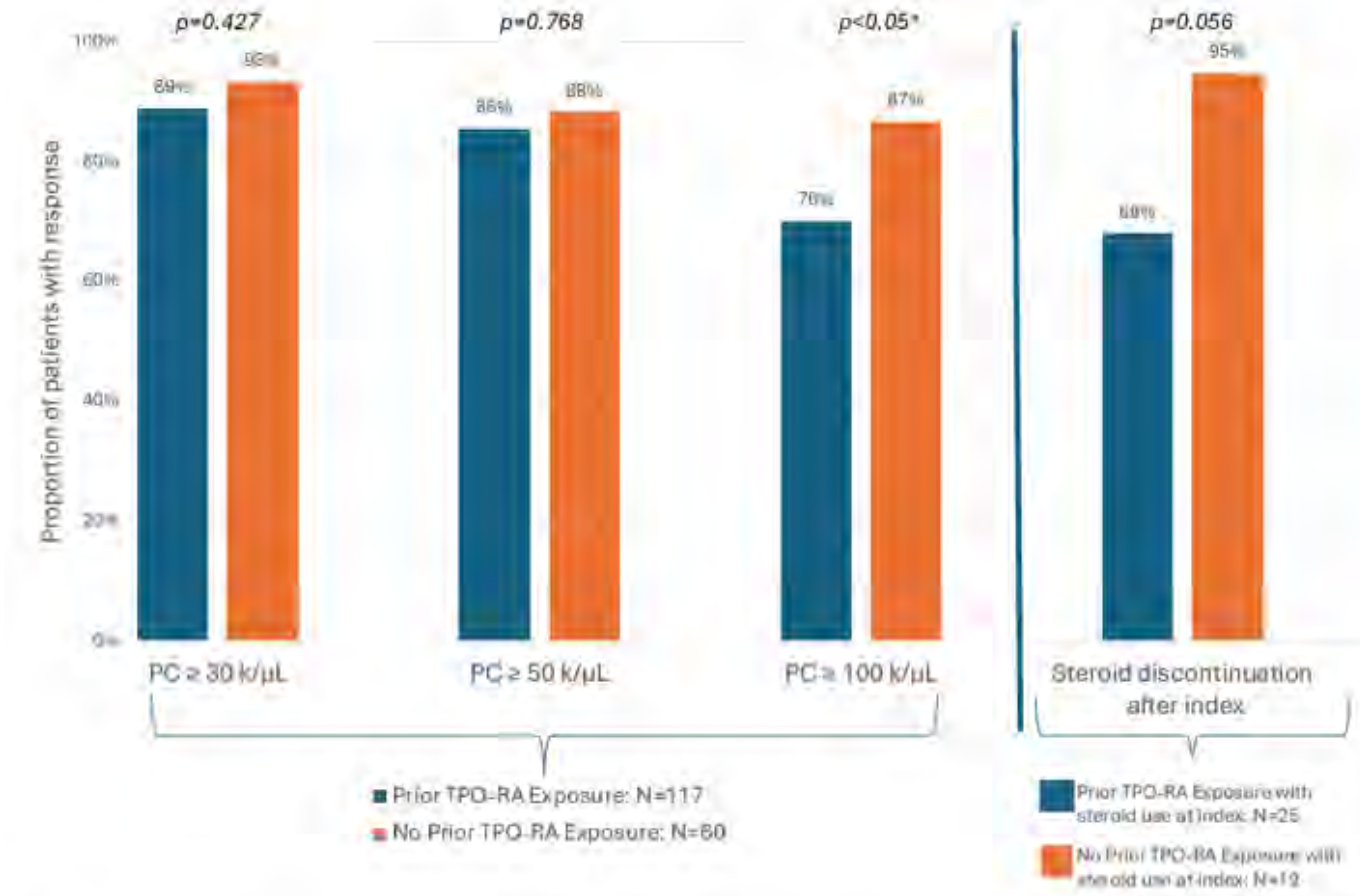
Retrospective multi-site chart review (n=177); evaluated platelet response thresholds and steroid discontinuation.

| Measure | Prior TPO-RA (n=117) | No Prior TPO-RA (n=60) | P-value |
|---|---|---|---|
| Mean age (years) | 58 | 52 | <0.05 |
| Median ITP duration (years) | 2.8 | 0.4 | <0.001 |
| Platelet response ≥30k/µL (%) | 89% | 93% | 0.427 |
| Platelet response ≥50k/µL (%) | 86% | 88% | 0.768 |
| Platelet response ≥100k/µL (%) | 70% | 87% | <0.05 |
| Steroid discontinuation after AVA (%) | 68% | 95% | 0.056 |

Nagalla S et al. Real-world outcomes of avatrombopag treatment in primary ITP stratified by prior TPO-RA exposure. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

Avatrombopag was effective regardless of prior TPO-RA use; higher ≥100k/µL response in TPO-RA–naive patients.

**Figure: AVA Response by Prior TPO-RA Exposure Status**




***Abbreviations:*** AVA, avatrombopag; PC, platelet count; TPO-RA, thrombopoietin receptor agonist; k/µL, thousand per microliter.

***Note:***

1. P-values compared proportions of patients using Chi-square tests.

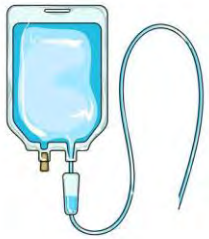
Nagalla S et al. Real-world outcomes of avatrombopag treatment in primary ITP stratified by prior TPO-RA exposure. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Stepwise treatment protocol vs. TPO-RA-based second-line therapy for severe immune thrombocytopenia

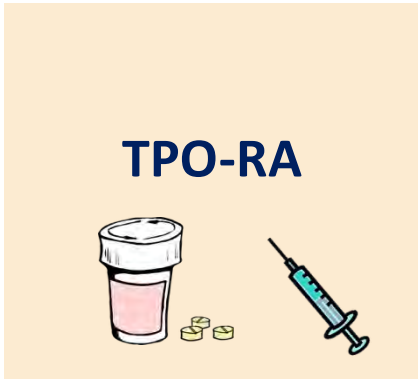
Lin X et al. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

## Stepwise treatment

High dose dexamethasone



Rituximab




## TPO-RA second line

High dose dexamethasone


- Compared individualized, stepwise response-guided protocol to TPO-RA-based second-line therapy in children with persistent/chronic ITP (n = 143)

# Stepwise Protocol vs TPO-RA for Pediatric p/cITP

- Stepwise protocol matched efficacy with fewer side effects, higher sustained off-treatment response, and lower cost.

| Measure | Stepwise Group (SG) | TPO-RA Group (TG) | P-value |
|---|---|---|---|
| **Response/remission rate** | Similar | Similar | >0.05 |
| **Bleeding control** | Similar | Similar | >0.05 |
| **Treatment-related side effects** | 9.0% (10 events in 111 pts) | 39% (13 events in 33 pts) | <0.001 |
| **Sustained response off-treatment (SRoT)** | 74% | 0% | — |
| **Treatment cost (12-month, per kg)** | $68.26 | $384.76 | <0.001 |

Lin X et al. Stepwise treatment protocol vs TPO-RA-based second-line therapy for severe immune thrombocytopenia. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Updates on Novel Treatments

# The Long-term Efficacy and Safety of Daratumumab in Children with chronic refractory ITP

Hu Y et al. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Evaluating Long-Term Daratumumab Efficacy in Pediatric Chronic Refractory ITP

- Single-center (n=11); daratumumab given weekly; follow-up up to 399 days; flow cytometry used to assess immune status.
- Daratumumab showed high initial and 3-month response rates, was well tolerated, and retreatment was effective after relapse.

| Outcome | Value |
|---|---|
| Initial response (Week 4) | 81.8% (9/11) |
| Response at 3 months | 90.9% (10/11) |
| Sustained response at 6 months | 42.8% (3/7) |
| Sustained response at 12 months | 66.7% (2/3) |
| Bleeding score >1 at baseline vs 1 month | 9 → 1 patient |
| Bleeding at 3 months | None |
| Relapse response to retreatment | Effective in 3/4 retreated pts |
| Safety | 81.8% mild-moderate AEs during first dose |

Hu Y et al. The long-term efficacy and safety of daratumumab in children with CRITP. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Short- and Long-Term Safety and Efficacy of CD38 Antibody Daratumumab in ITP

Tsykunoiva G et al. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

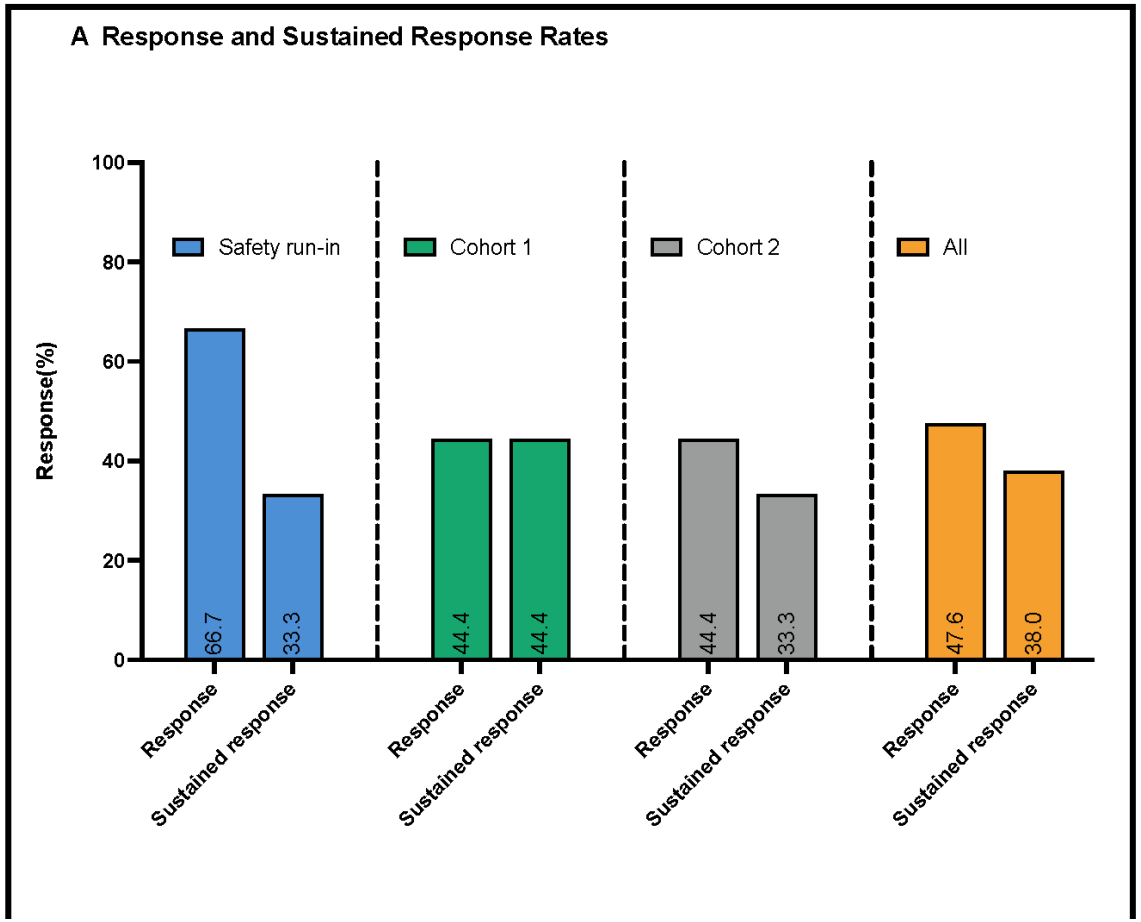
# Daratumumab for Refractory ITP: Phase 2 Efficacy and Safety

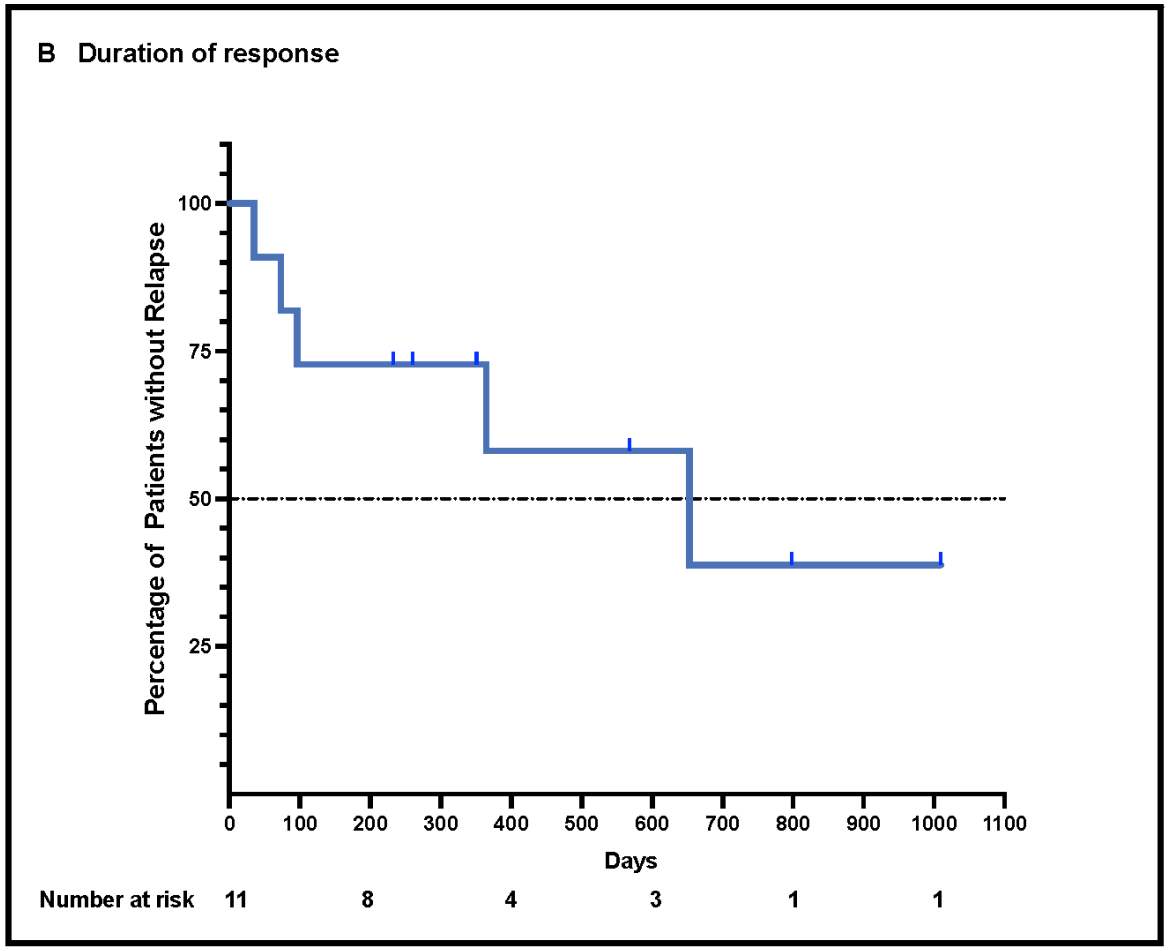
- a 3-patient safety run-in, 2 dosing cohorts received 8 and 10 daratumumab injections (1800 mg) for 8 or 12 weeks.
- Primary endpoint - two consecutive platelet counts ≥50x$10^9$/L at week 12 for safety run-in/Cohort 1, and week 16 for Cohort 2.

| | Total N = 21 |
|---|---|
| Median disease duration | 60.5 months |
| Median baseline platelet count | 17×$10^9$/L |
| Median prior therapies | 4 |
| Met primary endpoint | 52.4% (11/21) |
| Sustained response (Week 24) | 38% (8/21) |
| Median response duration | 351 days |
| Still in response at study end | 6 patients |
| Grade 3 AEs | 2 patients (infusion reaction, SARS-CoV-2) |
| Most common AEs | Infections (38%), all mostly grade 1–2 |
| Immunologic findings | ↓CD38+ cells; ↓Ig levels; no difference in autoantibodies |

Tsykunoiva G et al. Short- and long-term safety and efficacy of CD38 antibody daratumumab in immune thrombocytopenia. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Response Rates and Duration of Response




Tsykunoiva G et al. Short- and long-term safety and efficacy of CD38 antibody daratumumab in immune thrombocytopenia. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Results from a phase 2 study on mezagitamab for chronic/persistent primary ITP

Kuter D et al. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Phase 2 Results of Mezagitamab in Chronic ITP

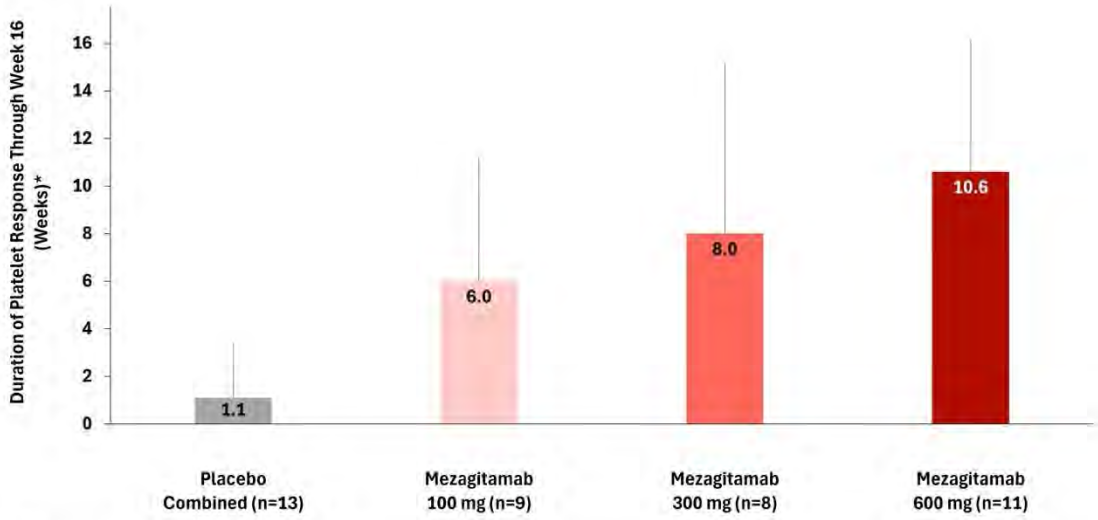
- Assessed efficacy, durability of response, and quality of life with mezagitamab in chronic/persistent ITP.
- Randomized, placebo-controlled phase 2 trial (n=41); weekly SC mezagitamab for 8 weeks.
  - 25 randomized 1:1 : 1 to placebo vs 100 vs 300 mg
  - 15 randomized to 2:1 to mezagitamab 600 mg vs placebo
- All doses showed favorable safety; background ITP therapy permitted.
- Mezagitamab improved platelet response duration and ITP-related quality of life in a dose-dependent manner.

Kuter D et al. Results from a phase 2 study on mezagitamab for chronic/persistent primary immune thrombocytopenia. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Results

| Group | Mean Increase in Platelet Response Duration vs Placebo (weeks) | Clinically Meaningful ITP-patient assessment questionnaire (PAQ) Change |
|---|---|---|
| Mezagitamab 100 mg | +4.9 | Yes |
| Mezagitamab 300 mg | +6.9 | Yes |
| Mezagitamab 600 mg | +9.6 | Yes |
| Placebo | — | No |

**Figure 1:** Duration of platelet response through week 16.

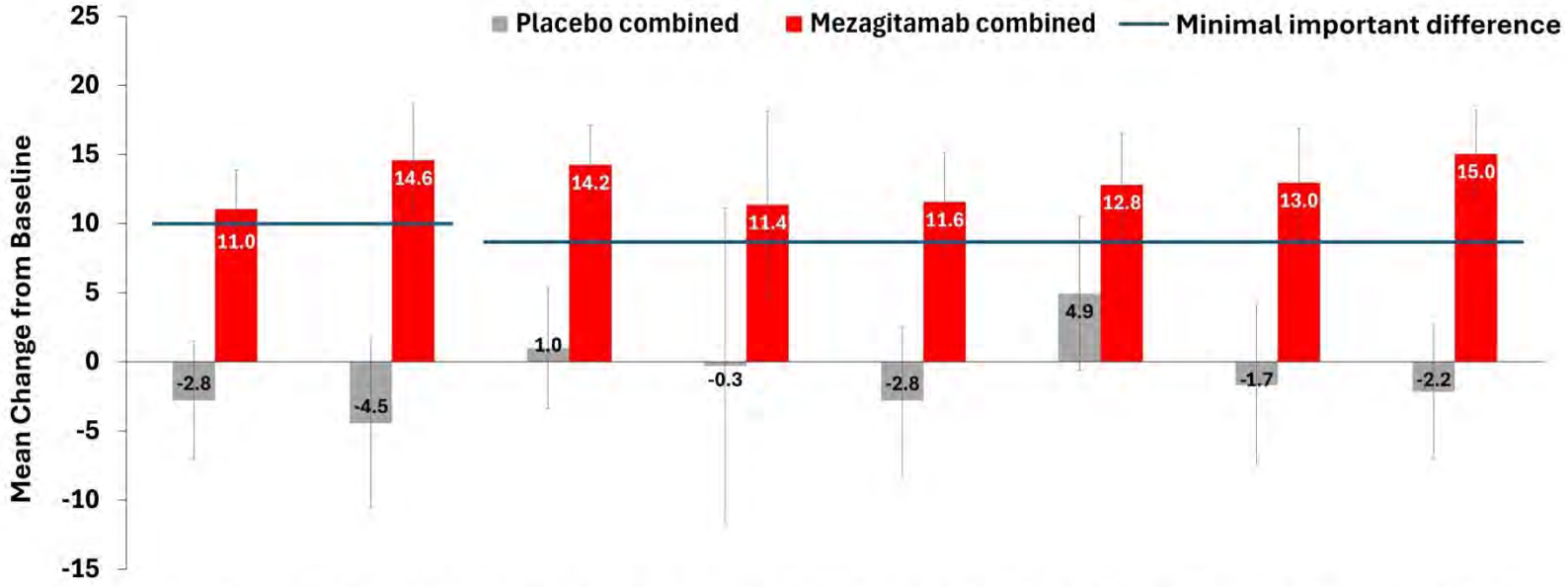



Data are presented as mean + SD.
* Duration of platelet response was defined as the number of weeks with platelet count ≥50,000/μL.

Kuter D et al. Results from a phase 2 study on mezagitamab for chronic/persistent primary immune thrombocytopenia. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# ITP Patient Assessment Questionnaire (ITP-PAQ) Score Change from Baseline to Week 16

**Figure 2:** Change from Baseline in ITP-PAQ Scale Scores at Week 16.




| | Fatigue/Sleep | Physical activity | Symptoms | Women's reproductive health | Physical health - Bother | Psychological health | Overall quality of life | Social activity |
|---|---|---|---|---|---|---|---|---|
| | n=10 n=20 | n=9 n=22 | n=10 n=22 | n=4 n=9 | n=10 n=22 | n=10 n=22 | n=10 n=22 | n=10 n=22 |
| Difference in least squares mean (95% CI vs placebo) | 13.9 (3.6, 24.3) | 19.2 (3.8, 34.7) | 13.0 (2.4, 23.6) | 10.3 (-17.7, 38.2) | 14.4 (1.5, 27.4) | 7.8 (-5.9, 21.6) | 14.7 (0.4, 29.0) | 17.2 (5.6, 28.8) |
| P value | 0.01 | 0.02 | 0.02 | 0.44 | 0.03 | 0.25 | 0.04 | 0.005 |

The graphed data are presented as model-based mean and standard error of the mean.

Kuter D et al. Results from a phase 2 study on mezagitamab for chronic/persistent primary immune thrombocytopenia. Presented at: 33rd Congress of the International Society on Thrombosis and Haemostasis; June 21–25, 2025; Washington, D.C.

# Clinical Pearls

- Rilzabrutinib demonstrated durable responses based on IWG criteria, particularly in patients with fewer prior ITP therapies.
- Avatrombopag was effective regardless of prior TPO-RA exposure; greater ≥100k/µL responses were seen in TPO-RA–naive patients.
- A stepwise, response-guided protocol offered comparable efficacy to TPO-RAs with fewer adverse effects, higher off-treatment response rates, and reduced cost.
- Daratumumab showed strong initial and 3-month efficacy in pediatric CRITP; retreatment was successful after relapse.
- In adults, 52% treated with daratumumab achieved response; durable outcomes lasted up to 1010 days with good tolerability.
- Mezagitamab extended platelet response duration and improved quality of life in a dose-dependent manner.