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## Review Article

# Durability of Response to Eltrombopag in Chronic Immune Thrombocytopenia: A Five-Year Cohort

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## Abstract

**Background:** Thrombopoietin receptor agonists are a mainstay of second-line therapy for chronic immune thrombocytopenia (ITP), and eltrombopag now carries the longest continuous follow-up of any oral agent in the class, though no single prospective cohort spans a full five-year horizon.

**Objective:** To synthesize the pivotal trial and long-term extension-study literature on eltrombopag in chronic ITP and characterize what it says about durability of platelet response over years rather than weeks.

**Methods:** Narrative synthesis of placebo-controlled registration trials, open-label extension studies (including EXTEND), discontinuation cohorts, and comparative studies against romiplostim.

**Findings:** Once established within one to two months, platelet response is maintained for years in most responders; splenectomy status is the most consistent modifier of response and durability; loss of response after initial success is uncommon and non-progressive; and a minority of complete responders sustain normal counts after withdrawal.

**Conclusions:** Eltrombopag is a durable long-term option in chronic ITP with a monitoring burden distinct from, but durability broadly comparable to, romiplostim; the open question is less whether response persists and more whether its dosing and monitoring remain acceptable for however long that response holds.

**Keywords:** eltrombopag; immune thrombocytopenia; thrombopoietin receptor agonist; durability of response; romiplostim

## 1. Introduction

Immune thrombocytopenia is a diagnosis of exclusion, and for most adults who carry it into a chronic phase the practical question is not whether a drug can raise the platelet count but whether it can keep doing so for years. The disorder itself, driven by accelerated antibody-mediated platelet destruction alongside a blunted compensatory rise in production, has been reviewed extensively elsewhere [1]. Corticosteroids and intravenous immunoglobulin solve the short-term problem and do little for the long one; splenectomy solves the long-term problem for many but not all, and carries a cost that patients and physicians increasingly avoid as a second-line default [2,3]. Thrombopoietin receptor agonists were developed to fill that gap, and eltrombopag, an oral small molecule now in clinical use for close to two decades, has accumulated the longest observation windows of any agent in the class outside romiplostim [4,5,6].

The title borrows the framing of a five-year cohort deliberately rather than literally. No single dataset follows one prospectively enrolled eltrombopag cohort for a full five years with fixed visits and a pre-specified endpoint; what exists instead is open-label extension studies, discontinuation cohorts, and comparative series that, stitched together, cover exposures from six months to nearly nine years [6,7]. That composite literature is what this review synthesizes: not a new trial, but what the extension-study evidence says about durability, about the minority who lose response over time, and about how eltrombopag's trajectory compares with romiplostim, the other agent with comparably mature follow-up.

Durability matters beyond convenience: a drug that fails predictably invites a different treatment philosophy than one whose failures are scattered unpredictably. The extension-study literature on eltrombopag describes the first pattern more than the second, and understanding why requires looking at the pivotal trial evidence, the

open-label extensions that established persistence, and the mechanisms proposed to explain both the drug's staying power and its occasional loss.

## 2. Mechanism of thrombopoietin receptor agonism

Endogenous thrombopoietin binds the extracellular domain of c-Mpl and is, in ITP, often only modestly elevated despite the low platelet count, because platelet and megakaryocyte mass rather than production rate regulates its clearance [8]. Both licensed receptor agonists exploit the same receptor but not the same site. Romiplostim is a peptibody, an Fc fusion carrying TPO-mimetic peptide domains that engage the same extracellular region as native thrombopoietin and so behaves like a long-acting analogue of the natural ligand [9,10]. Eltrombopag is a small, orally bioavailable hydrazone derivative that instead binds the transmembrane domain of c-Mpl, triggering the same JAK2-STAT and MAPK cascade allosterically rather than orthosterically [11]. That difference is why the two drugs are not cross-resistant in practice and why eltrombopag raises no concern about neutralizing-antibody formation against an endogenous protein, unlike earlier peptide mimetics; romiplostim's own mechanism summary makes the ligand-mimetic point explicit [12]. The mechanism is not ITP-specific: eltrombopag also drives trilineage improvement in refractory aplastic anemia, a disease with essentially no autoantibody-mediated destruction, arguing that durability here is a question of receptor pharmacology rather than reversing ITP-specific autoimmunity [13].

Eltrombopag activates human and chimpanzee Mpl far more potently than rodent or canine Mpl, so early pharmacology relied on human and xenograft systems rather than rodent models [11]. Clinically this means oral daily dosing with rapid onset, platelet counts typically rising within one to two weeks and peaking by four to six, consistent with the eight- to ten-day transit from megakaryocyte commitment to circulating platelet release

[4]. Absorption is reduced by co-administered polyvalent cations, so practical management is inseparable from counselling about dairy, antacids, and mineral supplements taken too close to the dose [5].

### 3. Pivotal trial evidence for eltrombopag in chronic ITP

The efficacy case was built across placebo-controlled trials completed between 2007 and 2011. The first dose-ranging study established that 50 mg daily produced platelet responses in most previously treated patients within six weeks, with lower response at 30 mg and a ceiling effect above 50 mg that justified the dose taken forward [4]. A subsequent placebo-controlled trial in patients with baseline counts below  $30 \times 10^9 / L$  confirmed that effect and also showed reduced clinically significant bleeding [14].

RAISE, the pivotal six-month, randomized, phase 3 study, is the reference trial for durability within a controlled setting. Patients on stable concurrent therapy were randomized 2:1 to eltrombopag or placebo for 26 weeks; the odds of achieving  $50\text{--}400 \times 10^9 / L$  over that period were roughly eight times higher with eltrombopag, bleeding was less frequent, and background medication was more often tapered [15]. RAISE matters here less for its endpoint than for the shape of response within it: once achieved, response was largely maintained across the full 26 weeks rather than eroding, the pattern motivating the extension studies below. The same pattern recurred in children in PETIT and its successor PETIT2, using the same  $50 \times 10^9 / L$  threshold [16,17]. Approval followed the adult trial sequence in 2008 and 2010, and the response criterion — at least  $50 \times 10^9 / L$  without rescue therapy — became the de facto outcome used by essentially every long-term study discussed here, aligned with the standardized ITP terminology the field adopted around the same period [18].

### 4. What long-term extension studies show about durability

The open-label EXTEND study is the closest

approximation to the five-year cohort implied by this review's title. Patients completing a qualifying trial could roll over into EXTEND on individually titrated doses; the interim analysis, at a median exposure of roughly two years, reported that about 80% of evaluable patients achieved a platelet count of at least  $50 \times 10^9 / L$  at some point, including many refractory to prior therapies, with response numerically lower among previously splenectomized patients [5]. Bleeding fell in parallel, and background medication use declined the longer patients remained on study [5].

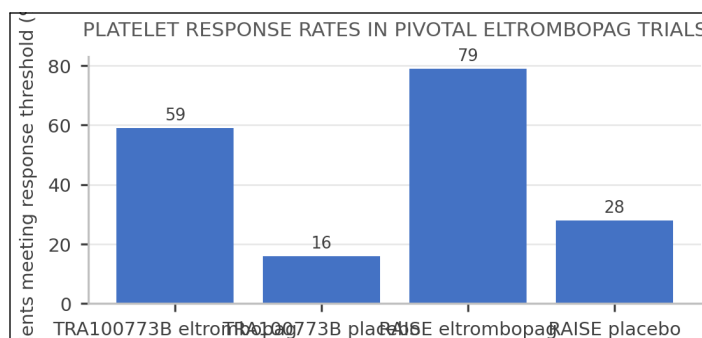


**Figure 1:** Platelet response rates in the two placebo-controlled adult trials establishing eltrombopag efficacy: 59% vs 16% achieved  $\geq 50 \times 10^9 / L$  by week 6 [14], and in RAISE roughly 79% vs 28% of assessed weeks met the  $50\text{--}400 \times 10^9 / L$  threshold [15].

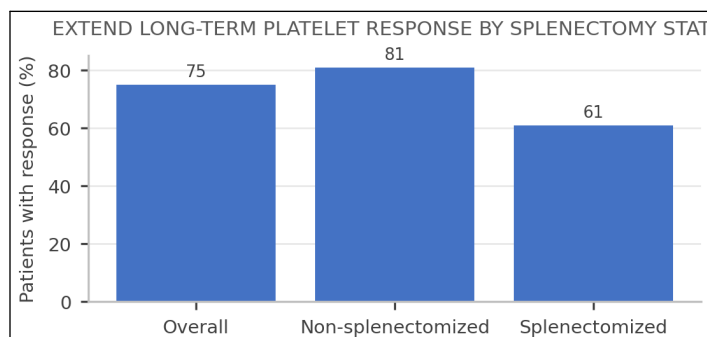


**Figure 2:** Platelet response ( $\geq 50 \times 10^9 / L$  on at least one occasion) in the final EXTEND study, overall and by splenectomy status; the lower splenectomized response recurs in romiplostim extensions too [6,7].

The final EXTEND report extended follow-up to just over 300 patients, median treatment duration exceeding two years and a maximum exposure approaching nine years — the longest continuous observation of any oral thrombopoietin receptor agonist in ITP published to date [6]. Roughly three-quarters achieved the response

threshold at least once, and among responders the median duration of response extended across years for most, with splenectomized patients again trailing non-splenectomized patients on both counts [6]. Discontinuation for lack of efficacy accounted for a minority of withdrawals; most patients who stopped did not stop because the drug had stopped working [6].

A parallel line of evidence asked what happens after eltrombopag is deliberately withdrawn. European cohorts identified a minority — roughly one in five to one in eight of those achieving stable complete remission — who sustained normal counts for a year or more after taper, a treatment-free remission pattern not generally expected from a purely stimulatory agonist [19,20]. For that minority, "durability of response" is not synonymous with "durability of drug exposure."

**5. Response loss, tachyphylaxis and dose adjustment**

Not every patient who responds keeps responding, and the literature is reasonably consistent about the shape of that failure. Loss of response in EXTEND and in comparable long-term romiplostim cohorts clusters in the first one to two years rather than accumulating late; patients maintaining response through eighteen to twenty-four months are unlikely to lose it purely through pharmacological exhaustion thereafter [6,7]. This argues against simple tachyphylaxis and instead suggests durable non-response reflects evolving underlying disease — new or unmasked secondary thrombocytopenia, a shifting autoantibody repertoire, or intercurrent illness — rather than desensitization of c-Mpl signalling itself [8].

Dose titration matters most at the individual rather than population level. The label calls for adjustment in 25 mg increments, and a substantial fraction of patients required increases toward the 75 mg ceiling to maintain response, while others needed reductions once concurrent immunosuppression was withdrawn [5,6]. Hepatic transaminase elevation, generally reversible with dose reduction, was the abnormality most often responsible for temporary interruption, and thromboembolic events, while uncommon, warranted monitoring in patients with cardiovascular risk [6]. None of these events increased in frequency the longer a patient remained on the drug [6]. Bone marrow reticulin deposition, a theoretical concern for any chronic megakaryopoiesis stimulant, was examined using paired biopsies; mild, non-progressive fibrosis was seen in a minority without the marrow failure syndrome feared, and reticulin grade did not predict subsequent loss of response — reassuring enough that routine surveillance biopsy is not part of standard monitoring [5,3].

**6. Comparative durability against romiplostim and other agents**

Romiplostim reached the market on a similar timeline and has its own mature long-term dataset, making it the natural durability comparator, since the two drugs are broadly similar on acute efficacy [21,15]. In the pivotal romiplostim trial, weekly subcutaneous injection produced a durable response — maintained across most weekly assessments in the second half of a six-month period — in a majority of splenectomized and non-splenectomized patients, with lower response in the splenectomized subgroup mirroring the pattern seen with eltrombopag [22]. The long-term extension, following patients for a median of roughly three years with some exceeding five, reported responses maintained in the large majority of treatment weeks once an effective dose was established, without evidence of declining potency over time [23,7].

| Table 1: Treatment-emergent findings during long-term eltrombopag exposure in the EXTEND cohorts, median exposure 2-2.4 years [5,6]. |            |                             |
|--------------------------------------------------------------------------------------------------------------------------------------|------------|-----------------------------|
| Finding                                                                                                                              | Proportion | Management                  |
| Dose titrated to 75 mg                                                                                                               | ~40-50%    | Maintained higher dose      |
| ALT/AST elevation                                                                                                                    | ~10-13%    | Reversible, dose reduced    |
| Thromboembolic event                                                                                                                 | ~2-3%      | Risk review, most continued |
| New reticulin fibrosis                                                                                                               | Minority   | No biopsy surveillance      |

| Table 2: Eltrombopag versus romiplostim, from each drug's own extension program; no head-to-head comparison exists [6,7,22,24]. |                        |                           |
|---------------------------------------------------------------------------------------------------------------------------------|------------------------|---------------------------|
| Feature                                                                                                                         | Eltrombopag            | Romiplostim               |
| Route                                                                                                                           | Oral, daily            | Subcutaneous, weekly      |
| Longest follow-up                                                                                                               | ~8.8 years             | Median ~3 years           |
| Response maintained                                                                                                             | Majority of responders | Majority of weeks         |
| Splenectomy response gap                                                                                                        | Present                | Present                   |
| Monitoring burden                                                                                                               | Liver enzymes, diet    | Injection site, overshoot |

Head-to-head randomized comparison over multi-year horizons does not exist; the comparative literature relies on indirect meta-analyses pooling each drug's own placebo-controlled trials. These have generally found the agents statistically similar on response rate and bleeding reduction, with the more consistent differences lying in administration — daily oral tablets against weekly injection — and in monitoring burden, eltrombopag carrying transaminase checks and dietary restrictions, romiplostim carrying injection-site reactions and more frequent thrombocytosis overshoot [24]. Smaller switch studies suggest patients who lose response to one agent not infrequently regain it on the other, consistent with the distinct binding sites [11,10]. Pediatric romiplostim data mirror the adult picture, with durable weekly responses in most children and the same splenectomy-linked response gap [25].

Set against fostamatinib, an oral spleen tyrosine kinase inhibitor with a different mechanism, and against splenectomy, the receptor agonist class occupies a middle position: more reliably sustained than most second-line immunosuppressive regimens, but without the single-procedure permanence a subset of splenectomized patients achieve [2,3]. Current ASH guidance favors thrombopoietin receptor agonists over splenectomy as an earlier-line option for many adults with persistent or chronic ITP, an update built substantially on the durability data summarized here [3].

7. Conclusion

No trial has followed one prospectively assembled eltrombopag cohort for a literal five years, but the composite of RAISE, the interim and final EXTEND analyses, and the smaller discontinuation cohorts describes a drug whose response, once established within the first one to two months, tends to persist for years rather than months [15,5,6]. Splenectomy status is the most consistent modifier of both likelihood and durability of response, loss of response after initial success is uncommon and does not show a clear time-dependent attrition curve, and a meaningful minority of well-responding patients sustain normal counts even after the drug is stopped [19,20]. Against romiplostim, the only agent with comparably long follow-up, eltrombopag looks less like an alternative with a different durability profile and more like one with a different administration route and monitoring burden [7,24]. For a clinician counselling a newly responding patient, the relevant question is rarely whether the response will last through year one — the literature says it usually will — but whether the patient can tolerate daily oral dosing, dietary timing, and periodic liver-enzyme monitoring for however many years that response holds.

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