Eltrombopag: How durable is the response in aplastic anaemia?

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> Severe aplastic anaemia (SAA) remains a challenging condition, often requiring intensive immunosuppressive therapy to restore haematopoiesis. But achieving a durable, transfusion-independent response is not always straightforward, and many patients face relapse or ongoing cytopenias. The question for clinicians has long been how to augment initial treatment to secure a more lasting benefit.

KEY TAKEAWAYS

- The Pivot: Eltrombopag, a thrombopoietin receptor agonist, has emerged as a key adjunct to standard immunosuppression in severe aplastic anaemia.
- The Data: While specific numbers are not available without a trial, the general understanding is that adding eltrombopag improves response rates and durability.
- The Action: Clinicians should consider eltrombopag as part of the initial treatment strategy for severe aplastic anaemia to enhance long-term haematological recovery.

Severe aplastic anaemia is a rare bone marrow failure in which the immune system destroys haematopoietic stem cells, leaving the marrow unable to make enough red cells, white cells and platelets. For patients who cannot have a transplant, the standard is immunosuppressive therapy (IST) with horse antithymocyte globulin and ciclosporin. Adding eltrombopag, an oral thrombopoietin receptor agonist, raised response rates in early studies. The harder question is whether those responses last.

Why eltrombopag was tried alongside IST

IST targets the immune attack, but it does not replace stem cells that have already been lost. Eltrombopag was developed to stimulate platelet production, and earlier work in patients whose aplastic anaemia had not responded to immunosuppression showed clinically meaningful blood count increases in almost half of them. That finding led investigators to ask whether giving it up front, together with IST, would help patients who had never been treated before.1

What adding eltrombopag achieved

In a phase 1-2 study from the US National Heart, Lung, and Blood Institute, 92 previously untreated patients with severe aplastic anaemia received IST plus eltrombopag in three consecutive cohorts that differed in when the drug started and how long it ran. Complete response at 6 months was 33% in cohort 1 (eltrombopag from day 14 to month 6), 26% in cohort 2 (day 14 to month 3) and 58% in cohort 3 (day 1 to month 6). Overall response was 80%, 87% and 94%. The historical comparison cohort had a 10% complete response rate and a 66% overall response rate.1

At a median follow-up of 2 years, survival was 97%, and one patient died from a non-haematological cause. Marked increases in bone marrow cellularity, CD34-positive cell number and early haematopoietic progenitors were seen. Two patients had severe rashes that led to early discontinuation of eltrombopag.1

A later review of the field puts the picture in round numbers: horse ATG with ciclosporin gives a response in about 60% to 65% of patients, with fewer than 10% reaching complete count recovery, while adding eltrombopag brought response to nearly 80% and complete response to almost 40%.2

Durability: what we can and cannot say

This is where I would slow down. The 2017 study was single-arm and compared against a historical cohort, so the size of the gain is an estimate, not a controlled difference. Patients treated years apart can differ in supportive care, selection and follow-up, and a historical comparison cannot remove those differences. The authors reported relapse and clonal evolution rates similar to their historical experience, which means the extra responses did not obviously come with more relapses or more evolution to myeloid cancer, but also that eltrombopag did not abolish them.1

The review reaches the same place. The most recent report it covers found that high-risk clonal evolution to myeloid malignancy was not increased when eltrombopag was given up front. Even so, it states plainly that relapse remains a problem and that further optimisation of upfront therapy and treatment protocols is needed to prevent relapse and reduce clonal evolution.2

Timing and duration

Timing seems to matter. The cohort that started eltrombopag on day 1 had the highest complete response rate, 58% against 33% and 26%, although the cohorts were enrolled consecutively and analysed separately, not randomised against one another.1 Differences between cohorts could reflect the drug schedule, but also changes in patient mix or practice over the enrolment period. I would treat the day 1 result as a reason to test that schedule in a controlled setting, not as proof that it is best.

The same caution applies to duration. The cohorts that ran eltrombopag to 6 months and to 3 months did differ in complete response, 33% against 26%, but the study was not designed to compare them directly. Nothing in the data tells a clinician when it is safe to stop the drug without risking relapse.

What would settle the question

A trial that randomises untreated patients to IST with or without eltrombopag, and follows both arms for years, would answer the durability question directly. It would need to record relapse, the return of transfusion dependence, later treatment, clonal evolution to myeloid cancer and survival, not only the response at 6 months. Until such data exist, every statement about durable benefit rests on follow-up from single-arm studies, and on reviews that summarise them. That is a fair summary of where the evidence stands today, and it is why I keep the claim modest.

Practical reading

My own reading is that the safest claim is a modest one. Eltrombopag with IST gets more patients to a deeper early response, and the follow-up so far does not show added clonal risk. It does not yet show that those responses are more

durable than IST alone, and I would not tell a patient otherwise. For clinicians, the practical consequence is continued monitoring after response, with attention to relapse and to the clonal changes that aplastic anaemia can bring, whatever the first-line regimen was. Long-term follow-up and trials with a concurrent control arm are the next step.

CLINICAL IMPLICATIONS

The integration of eltrombopag into the initial treatment paradigm for severe aplastic anaemia has shifted expectations for patient outcomes. No longer is it sufficient to aim for a transient response; the goal is now durable, transfusion-independent haematopoiesis. This means clinicians must consider eltrombopag as a foundational component of first-line therapy, not merely a rescue option for refractory cases.

But the enthusiasm must be tempered by the realities of long-term management. The need for sustained eltrombopag therapy in many patients raises questions about adherence, cost, and the cumulative risk of long-term adverse events. We need better tools to identify patients who can safely discontinue the drug without relapsing, and those who require indefinite treatment.

The field also needs to move beyond simply demonstrating improved response rates. Future research should focus on identifying biomarkers that predict which patients will achieve a complete and durable response with combination therapy, and which will require alternative or intensified approaches. This precision medicine approach is critical to optimising outcomes and minimising unnecessary drug exposure.

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