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Editorial

Romiplastim for the management of severe aplastic anemia

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Dear editor

Three or four decades ago, during medical rounds, if a professor asked students: “In a patient with pancytopenia but no organomegaly, would you prefer the bone marrow result to be aplastic anemia or acute leukemia?” many, despite the understandable fear of both diagnoses, would choose *aplastic anemia* with a sense of relative relief. However, the correct answer was acute leukemia, which was often a treatable disease, especially in children. Whereas for aplastic anemia, the only therapeutic management was bone marrow transplantation. Because of limited HLA matched donors, and about 25% mortality after transplantation, delays in definitive decisions can translate into life threatening infections, prolonged transfusion dependence, and profound psychosocial burden.

Severe aplastic anemia (SAA) remains one of the most demanding entities in pediatric hematology-oncology. The conventional pillars are allogeneic hematopoietic cell transplantation (HCT) when an optimal donor is available, and immunosuppressive therapy (IST); classically anti-thymocyte globulin (ATG) plus cyclosporine; for those without timely transplant options. Supportive care advances have improved survival, yet non-response, incomplete response, relapse, and prolonged cytopenia continue to shape real-world outcomes and decision-making (1).

In this context, thrombopoietin receptor agonists (TPO-RAs) altered the therapeutic narrative. Initially framed as platelet-raising agents, TPO-RAs demonstrated broader hematopoietic effects, supporting stem/progenitor survival and enabling the clinically meaningful possibility of trilineage recovery in a disease defined by global marrow failure (2, 3). This shift matters for pediatrics: “time gained”, and created a grate hope to live with fewer infections, fewer bleeds, fewer transfusions, and a credible path toward hematologic recovery or a safer bridge to transplant.

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Accumulating evidence; synthesized in the most recent systematic review and reinforced by prospective and real-world studies; suggests that romiplostim is moving from salvage use toward a more deliberate, protocolized role across multiple clinical settings. In IST-naïve patients, phase 2/3 data and cohort experiences indicate that adding romiplostim to standard IST can yield substantial hematologic response rates, with complete responses observed in a subset at defined timepoints (commonly around 6 months), and with signals of durability among responders on longer follow-up (5-7,22). Although most data are adult-dominant, the kinetics of response and the feasibility of dose-escalation strategies reported in these studies provide a practical scaffold for pediatric programs, particularly where immediate HCT is not feasible.

Romiplostim also appears relevant in refractory SAA, where prolonged severe cytopenia creates the highest-risk clinical trajectory. Multiple studies in IST-refractory aplastic anemia report meaningful overall responses and, importantly, trilineage responses over time, including in longer-term follow-up analyses (12-15). For pediatric clinicians, this is not merely a “last option”; it can be a rational attempt to reduce transfusion burden, mitigate infectious risk, and create a therapeutic bridge while donor searches, transplant logistics, or clinical stabilization proceed.

A particularly pragmatic point; highly relevant to routine practice; is sequencing after another TPO-RA (eltrombopag). Retrospective cohorts and real-world reports suggest that romiplostim can still induce responses in patients who were refractory to, intolerant of, or failed eltrombopag (17, 20). In health systems where drug availability, adherence interruptions, hepatic enzyme concerns, or procurement delays occur, having an evidence-supported alternative within the same class may meaningfully change the outcomes.

No discussion of TPO-RAs in aplastic anemia is complete without addressing clonal evolution. Reported clonal events are uncommon but not absent: transformation to myelodysplastic syndrome (MDS) has been reported in at least one first-line setting (6), and rare clonal expansions have been described in refractory cohorts with long follow-up (14). Conversely, several pediatric and mixed-age series report no clonal evolution within available follow-up (78,), but such reassurance must be interpreted through the lens of limited sample sizes and variable surveillance intensity. Therefore, the appropriate stance is neither complacency nor therapeutic paralysis: romiplostim should be considered a potentially disease-modifying adjunct, accompanied by disciplined monitoring plans (structured blood count follow-up; marrow reassessment when clinically indicated or at pre-specified milestones; and cytogenetics/NGS when available), aligned with established marrow failure practice (1, 6, 14).

In summary, romiplostim is increasingly supported as more than a symptomatic platelet strategy in SAA. Across first-line augmentation, refractory disease, and post-eltrombopag scenarios, the evolving literature suggests a credible potential for trilineage recovery and for reducing the morbidity of prolonged cytopenia (5-7, 12-15, 17, 20, 22). After a real-world study in Shiraz, I.R. Iran, focussing on pediatric age group, and many citations to it in the first year of publication in the Journal of Haematologica (8); the Pediatric Hematology Oncology Society now needs prospective pediatric-focused trials, harmonized endpoints, and long-term registries to define optimal patient selection, dosing paradigms, and surveillance schedules. Until then, the opportunity is to use romiplostim thoughtfully; within explicit algorithms; so that hope is translated into reproducible outcomes rather than anecdotal success.

Key Take Home Messages for Clinicians

In Iran, the “decision window” in SAA is often constrained by donor availability, transplant capacity, inter-center referral timelines, and variable access to medications and molecular monitoring. In such settings, romiplostim may be considered these three settings:

1- As an IST adjunct in selected first-line patients when timely HCT is not achievable,

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based on encouraging response signals and durability in published studies (5-8, 22).

2- As a structured option in IST-refractory SAA, to reduce transfusion dependence and create a safer bridge to transplant or longer-term management (12-15).

3- As a sequencing strategy after eltrombopag failure, in the appropriate candidates (17, 20). The most impactful next step is local standardization: each center should define eligibility criteria, escalation and response milestones (eg, 6 and 12 months), and a feasible clonal surveillance plan consistent with marrow failure standards (1, 6, 14). A national or multi-center registry would not only strengthen evidence for Iranian practice but could also clarify which children benefit most; an answer that the current literature still invites us to pursue.

Availability of Data

This is not a clinical trial. Data of researches outlined in this review are available in the Scientific Literature.

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Conflict of Interest

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M.S is the only Author.

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